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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

☑ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended: December 31, 2022

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

Commission file number: 001-35969

PTC THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**04-3416587**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
100 Corporate Court South Plainfield, NJ	**07080**
(Address of principal executive offices)	(Zip Code)

(908) 222-7000

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol (s)	Name of each exchange on which registered
Common Stock, $0.001 par value per share	PTCT	Nasdaq Global Select Market

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☑ No ☐

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☑

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☑ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☑ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☑	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☑

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☑

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). ☐

The aggregate market value of the Common Stock held by non-affiliates of the registrant, based upon the last sale price of the Common Stock reported on the Nasdaq Global Select Market on June 30, 2022, the last business day of the registrant's most recently completed second fiscal quarter, was $2,129,532,394. For purposes of this calculation, shares of Common Stock held by directors and officers have been treated as shares held by affiliates.

As of February 16, 2023, the registrant had 73,815,144 shares of Common Stock, $0.001 par value per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Part III of this Annual Report incorporates by reference information from the definitive Proxy Statement for the registrant's 2023 Annual Meeting of Shareholders which is expected to be filed with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2022.

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PTC Therapeutics, Inc.

FORWARD-LOOKING STATEMENTS

This Annual Report on Form10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Annual Report on Form 10-K, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Annual Report on Form 10-K include, among other things, statements about:

- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for our products or product candidates that we commercialize or may commercialize in the future;
- expectations with respect to our gene therapy platform, including our ability to commercialize Upstaza™ (eladocagene exuparvovec) for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency, in the European Economic Area, or EEA, any potential regulatory submissions and potential approvals for our product candidates, our manufacturing capabilities and the potential financial impact and benefits of our leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that we may be obligated to make;
- our ability to maintain our marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in the EEA, which is subject to the specific obligation to conduct and submit the results of Study 041 to the European Medicines Agency, or EMA, and annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA;
- our ability to utilize results from Study 041 to support a conversion of the conditional marketing authorization for Translarna for the treatment of nmDMD in the EEA to a standard marketing authorization and to support a marketing approval for Translarna for the treatment of nmDMD in the United States;
- the anticipated period of market exclusivity for Emflaza® (deflazacort) for the treatment of Duchenne muscular dystrophy in the United States under the Orphan Drug Act of 1983;
- our expectations with respect to the commercial status of Evrysdi® (risdiplam) and our program directed against spinal muscular atrophy in collaboration with F. Hoffmann La Roche Ltd and Hoffmann La Roche Inc. and the Spinal Muscular Atrophy Foundation and our estimates regarding future revenues from sales-based royalty payments or the achievement of milestones in that program;
- our expectations and the potential financial impact and benefits related to our Collaboration and License Agreement with a subsidiary of Ionis Pharmaceuticals, Inc. including with respect to the timing of regulatory approval of Tegsedi® (inotersen) and Waylivra™ (volanesorsen) in countries in which we are licensed to commercialize them, the commercialization of Tegsedi and Waylivra, and our expectations with respect to royalty payments by us based on our potential achievement of certain net sales thresholds;
- the timing and scope of our commercialization of our products and product candidates;
- our estimates regarding the potential market opportunity for our products or product candidates, including the size of eligible patient populations and our ability to identify such patients;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs for our products on adequate terms, or at all;

- our estimates regarding expenses, future revenues, third-party discounts and rebates, capital requirements and needs for additional financing, including our ability to maintain the level of our expenses consistent with our internal budgets and forecasts and to secure additional funds on favorable terms or at all;
- the timing and conduct of our ongoing, planned and potential future clinical trials and studies in our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications, including the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;
- our ability to realize the anticipated benefits of our acquisitions or other strategic transactions, including the possibility that the expected impact of benefits from the acquisitions or strategic transactions will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of operations and employees into our business, our ability to obtain marketing approval of our product candidates we acquired from the acquisitions or other strategic transactions and unknown liabilities;
- the rate and degree of market acceptance and clinical utility of any of our products or product candidates;
- the ability and willingness of patients and healthcare professionals to access our products and product candidates through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the timing of, and our ability to obtain additional marketing authorizations for our products and product candidates;
- the ability of our products and our product candidates to meet existing or future regulatory standards;
- our ability to complete Study 041, a multicenter, randomized, double-blind, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, according to the protocol agreed with the EMA;
- the potential receipt of revenues from future sales of our products or product candidates;
- our sales, marketing and distribution capabilities and strategy, including the ability of our third-party manufacturers to manufacture and deliver our products and product candidates in clinically and commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy their other obligations to us;
- our ability to establish and maintain arrangements for the manufacture of our products and product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our expectations with respect to the COVID-19 pandemic and related response measures and their effects on our business, operations, clinical trials, potential regulatory submissions and approvals, our collaborators, contract research organizations, suppliers and manufacturers;
- our ability to complete any post-marketing requirements imposed by regulatory agencies with respect to our products;
- our expectations with respect to the potential financial impact and benefits of our leased biologics manufacturing facility and our ability to satisfy our obligations under the terms of the lease agreement for such facility;
- our ability to satisfy our obligations under the terms of the credit agreement with funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit and Wilmington Trust, National Association, as the administrative agent;

- our ability to satisfy our obligations under the indenture governing our 1.50% convertible senior notes due September 15, 2026;
- our regulatory submissions, including with respect to timing and outcome of regulatory review;
- our plans to advance our earlier stage programs and pursue research and development of other product candidates, including our splicing, gene therapy, Bio-e, metabolic and oncology programs;
- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets and our ability to successfully develop or commercialize any assets to which we may gain rights pursuant to such business development opportunities;
- the potential advantages of our products and any product candidate;
- our intellectual property position;
- the impact of government laws and regulations;
- the impact of litigation that has been or may be brought against us or of litigation that we are pursuing against others; and
- our competitive position.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly under the heading "Summary of Risk Factors" and the risk factors detailed further in Part I, Item 1A. Risk Factors that we believe could cause actual results or events to differ materially from the forward-looking statements that we make.

Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

In this Annual Report on Form 10-K, unless otherwise stated or the context otherwise requires, references to "PTC," "PTC Therapeutics," "we," "us," "our," "the Company," and similar references refer to PTC Therapeutics, Inc. and, where appropriate, its subsidiaries. The trademarks, trade names and service marks appearing in this Annual Report on Form 10-K are the property of their respective owners.

All website addresses given in this Annual Report on Form 10-K are for information only and are not intended to be an active link or to incorporate any website information into this document.

SUMMARY OF RISK FACTORS

Below is a summary of the principal risk factors that make an investment in our common stock speculative or risky. This summary does not address all of the risks and uncertainties that we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Additional discussion of the risks summarized in this risk factor summary, and other risks that we face, can be found in Item 1A. Risk Factors, of this Annual Report on Form 10-K, and should be carefully considered, together with other information in this Annual Report on Form 10-K and our other filings with the Securities Exchange Commission, before making an investment decision regarding our common stock. The forward-looking statements discussed above are qualified by these risk factors. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Summary of Risk Factors

- We may be unable to continue to execute our commercial strategy for our products, fail to obtain renewal of, or satisfy the conditions of our marketing authorization for our products;
- Delays or failures in obtaining regulatory approval would materially impair our commercialization capabilities;
- We may not qualify for certain specialized pathways to develop our product candidates or to seek approval;
- We or our collaborators may experience any of a number of possible unforeseen events in connection with clinical trials related to our products and product candidates;
- Subgroup, retrospective, post-hoc, and certain statistical analyses may not be reliable and typically will not form the basis for regulatory approval;
- We may experience delays or difficulties in the enrollment of patients in our clinical trials;
- We may identify serious adverse side effects during the development or further development of any product or product candidate;
- Our products and product candidates may be difficult to manufacture;
- The marketing authorization granted by the European Commission for Translarna for the treatment of nmDMD is limited to ambulatory patients aged two years and older located in the EEA and is also subject to annual reassessment of the benefit-risk balance by the EMA as well as the specific obligation to conduct Study 041, and may be varied, suspended or withdrawn by the European Commission if we fail to satisfy those requirements;
- Our products and product candidates may fail to achieve market acceptance in the medical community;
- We may be unable to establish or maintain sales, marketing and distribution capabilities or enter into agreements with third parties to market, sell and distribute our products or product candidates;
- A substantial portion of our commercial sales currently occurs in territories outside of the United States which subjects us to additional business risks and laws and regulations, including those governing export restrictions and economic sanctions;
- We face substantial competition;
- Our products or product candidates may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives;
- We have incurred significant losses since our inception and expect to continue to incur significant operating expenses for the foreseeable future. We may need additional funding and we may never generate profits from operations or maintain profitability;
- Our credit agreement with funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit, collectively, Blackstone, may adversely affect our financial condition or restrict future operations;
- We may engage in strategic transactions to acquire assets, businesses, or rights to products, product candidates or technologies or from collaborations or make investments in other companies or technologies that could harm our business and dilute our stockholders' ownership;
- Raising additional capital may dilute our stockholders' ownership, restrict our operation or require us to relinquish rights to our technologies or product candidates;
- We may not be able to comply with applicable laws and regulations for our products or product candidates;
- We may not be able to obtain orphan drug exclusivity for our products or product candidates in either the United States or the EU;

- All pharmaceutical products for which marketing authorization has been granted are subject to extensive and rigorous regulation;
- Failure to obtain and maintain acceptable pricing and reimbursement terms for our products would delay our commercialization efforts;
- Legislative and regulatory changes affecting the pharmaceutical industry or the healthcare system more broadly may negatively affect our business;
- We may fail to properly allocate our resources;
- We face risks related to health epidemics and other widespread outbreaks of contagious disease, including COVID-19;
- We contract with third parties for the manufacture and distribution of our products and certain of our product candidates and these third parties may encounter issues that affect our business;
- We rely on third parties to conduct our preclinical and clinical trials and other essential services;
- We currently depend, and expect to continue to depend, on collaborations with third parties for the development and commercialization of some of our products and product candidates;
- Our business and operations would suffer in the event of computer system failures, cyber-attacks or a deficiency in our, or our collaborators' or third-party vendors', cyber-security;
- We may be subject to product liability and other civil lawsuits;
- We may be unable to retain our key executives;
- We may encounter difficulties in managing our growth as a company;
- We may be unable to obtain or maintain patent protection for our technology and products;
- We may become involved in lawsuits to protect or enforce our intellectual property or in connection with allegations that we are infringing on third party intellectual property rights;
- Without patent protection, our marketed products may face generic competition;
- We may not obtain or maintain adequate trademark protection for our brand names;
- Our rights to develop and commercialize Upstaza and our other gene therapy product candidates are subject, in part, to the terms and conditions of licenses granted to us by others;
- We may not have sufficient cash flow from our business to make payments on our debt; and
- The price of our common stock may be volatile and fluctuate substantially.

PART I

Item 1. Business

Overview

We are a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. Our ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. Our mission is to provide access to best-in-class treatments for patients who have little to no treatment options. Our strategy is to leverage our strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. We believe that this allows us to maximize value for all of our stakeholders.

Our Pipeline

We have a portfolio pipeline that includes several commercial products and product candidates in various stages of development, including clinical, pre-clinical and research and discovery stages, focused on the development of new treatments for multiple therapeutic areas for rare diseases relating to neurology, metabolism and oncology. The chart and the disclosure directly below summarizes the status of our clinical-stage programs and commercial products as of the date of this report, including those with our strategic partners:

- ***Global Commercial Footprint***
  - ***Global DMD Franchise –*** We have two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area, or EEA, for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients aged two years and older and in Russia for the treatment of nmDMD in patients aged two years and older. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for

continued treatment of patients that become non-ambulatory. Emflaza is approved in the United States for the treatment of DMD in patients two years and older.

- ***Upstaza™ (eladocagene exuparvovec)*** **–** Upstaza, a gene therapy for the treatment of Aromatic L-Amino Decarboxylase, or AADC, deficiency, a rare central nervous system, or CNS, disorder is approved for the treatment of AADC deficiency for patients 18 months and older within the EEA and the United Kingdom. We expect to submit a biologics license application, or BLA, for Upstaza for the treatment of AADC deficiency in the United States to the United States Food and Drug Administration, or FDA, in the first half of 2023.
- ***Tegsedi® (inotersen) and Waylivra® (volanesorsen)*** **–** We hold the rights for the commercialization of Tegsedi and Waylivra for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to our Collaboration and License Agreement with a subsidiary of Ionis Pharmaceuticals, Inc. Tegsedi has received marketing authorization in the United States, European Union, or EU, and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. We began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis and Waylivra for the treatment of familial chylomicronemia syndrome, or FCS, in Brazil in 2022. Waylivra is also approved in Brazil for the treatment of familial partial lipodystrophy, or FPL.
- ***Evrysdi® (risdiplam)*** **–** Evrysdi, a treatment for spinal muscular atrophy, or SMA, was approved by the FDA for the treatment of SMA in adults and children of all ages and by the European Commission for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi has also received marketing authorization for the treatment of SMA in Brazil and Japan. We expect the EMA to make a regulatory decision on approval for a label expansion for Evrysdi to include infants under two months old with SMA in 2023. Evrysdi is a product of our SMA program and our collaboration with F. Hoffman-La Roche Ltd. and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation.

- ***Diversified Development Pipeline***
  - ***Splicing Platform –*** In addition to our SMA program, our splicing platform also includes PTC518, which is being developed for the treatment of Huntington's disease, or HD. We initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. Enrollment in the Phase 2 study remains active and ongoing outside of the United States. Enrollment within the United States is paused as the FDA has requested additional data to allow the Phase 2 study to proceed; discussions are ongoing with the FDA to allow the resumption of U.S. enrollment. We expect data from the initial 12-week phase of the Phase 2 study in the second quarter of 2023.
  - ***Bio-e Platform*** **–** The two most advanced molecules in our Bio-e platform are vatiquinone and utreloxastat. We initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in children with mitochondrial disease associated seizures in the third quarter of 2020. We anticipate results from the Phase 2/3 trial to be available in the second quarter of 2023. We also initiated a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in the fourth quarter of 2020 and anticipate results from this trial to be available in the second quarter of 2023. We initiated a Phase 2 trial of utreloxastat for amyotrophic lateral sclerosis, or ALS, in the first quarter of 2022 and enrollment is ongoing.
  - ***Metabolic Platform –*** The most advanced molecule in our metabolic platform is sepiapterin. We initiated a registration-directed Phase 3 trial for sepiapterin for phenylketonuria, or PKU, in the third quarter of 2021 and now expect results from Part 2 of this trial to be available in May 2023 as the trial is overenrolled and additional time is required for the entirety of the primary analysis population to complete the study.
  - ***Gene Therapy Platform*** **–** In addition to Upstaza, our gene therapy platform includes an asset targeting Friedreich ataxia. We continue to work towards initiating a clinical study for this program. Additionally, the gene therapy platform includes our program targeting Angelman syndrome. We continue to work towards submitting a filing in support of the first-in-human study for this program.
  - ***Oncology Platform*** **–** Unesbulin is our most advanced oncology agent. We initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of leiomyosarcoma, or LMS, in the first quarter of 2022 and enrollment is ongoing. We expect to initiate a registration-directed Phase 2/3 trial of unesbulin for the treatment of diffuse intrinsic pontine glioma, or DIPG, in the fourth quarter of 2023.

- ***Multi-platform Discovery***
  - We continue to invest in our pre-clinical product pipeline across all of our platforms by committing significant resources to research and development programs and business development opportunities within our areas of scientific expertise, including potential collaborations, alliances, and acquisitions or licensing of assets that complement our strategic mission to provide access to best-in-class treatments for patients who have an unmet medical need.

Global Commercial Footprint

Global DMD Franchise

Duchenne muscular dystrophy (DMD)

Muscular dystrophies are genetic disorders involving progressive muscle wasting and weakness. DMD is the most common and one of the most severe types of muscular dystrophy. DMD occurs when a mutation in the dystrophin gene prevents the cell from making a functional dystrophin protein. Dystrophin is a muscle membrane associated protein and is critical to the structural and membrane stability of muscle fibers in skeletal, diaphragm and heart muscle. The absence of normally functioning dystrophin results in muscle fragility, such that muscle injury occurs when muscles contract or stretch during normal use. As muscle damage progresses, connective tissue and fat replace muscle fibers, resulting in inexorable muscle weakness.

Because the dystrophin gene is located on the X chromosome, DMD occurs primarily in young boys, although approximately 10% of female carriers show some disease symptoms. DMD is rare, and estimates of occurrence include approximately 1 in every 3,500 live male births, according to the National Organization for Rare Diseases and approximately 1 in every 5,000 live male births according to Ryder (2017) in the European Journal of Human Genetics. We estimate that there are between approximately 10,000 to 15,000 DMD patients in the United States. Several different types of mutation in the dystrophin gene can result in DMD, including deletion, duplication and nonsense mutations. A test known as multiplex ligation-dependent probe amplification (MLPA) can detect large deletions and duplications, which account for approximately 75% of all mutations. However, gene sequencing is required to identify small mutations such as nonsense mutations. We estimate that nonsense mutations account for approximately 13% of cases of DMD. Without treatment, patients with DMD typically lose walking ability by their early teens, require ventilation support in their late teens, and eventually experience premature death due to heart and lung failure. Even with medical care, most people with DMD die from cardiac or respiratory failure before or during their 30s.

Marketing authorization matters

Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy

European Economic Area

We received marketing authorization from the European Commission in August 2014 for Translarna for the treatment of nmDMD in ambulatory patients aged five years and older in the member states of the EEA, subject to annual renewal and other conditions. In July 2018, the European Commission approved a label-extension request to our marketing authorization for Translarna in the EEA to include patients from two to up to five years of age. In July 2020, the European Commission approved the removal of the statement “efficacy has not been demonstrated in non-ambulatory patients” from the indication statement for Translarna.

The marketing authorization is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of continued authorization, which we refer to as the annual EMA reassessment. In June 2022, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2023. In February 2023, we submitted a marketing authorization renewal request to the EMA.

This marketing authorization is further subject to a specific obligation to conduct and submit the results of an 18 month, placebo-controlled trial, followed by an 18 month open-label extension, which we refer to together as Study 041. Within the placebo-controlled trial, Translarna showed a statistically significant treatment benefit across the entire intent to treat population as assessed by the 6-minute walk test, assessing ambulation and endurance, and in lower-limb muscle function as assessed by the North Star Ambulatory Assessment, a functional scale designed for boys affected by DMD. Additionally, Translarna showed a statistically significant treatment benefit across the intent to treat population within the 10-meter run/walk and 4-stair stair climb, each assessing ambulation and burst activity, while also showing a positive trend in the 4-stair stair descend although not statistically significant. Within the primary analysis group, Translarna demonstrated a positive trend across all endpoints, however, statistical significance was not achieved. Translarna was also well tolerated. In September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. We expect an opinion from the Committee for Medicinal Products for Human Use, or CHMP, in the first half of 2023.

Marketing authorization is required in order for us to engage in any commercialization of Translarna in the EEA, including through participation in the market access process and related pricing and reimbursement negotiations, on a country-by-country basis with each country in the EEA, and is also required to make Translarna available under early access programs, or EAP programs. There is substantial risk that if we are unable to renew our EEA marketing authorization during any annual renewal cycle, if our product label is materially restricted, or if Study 041 does not provide the data necessary to maintain our marketing authorization, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna in the EEA and other territories.

See “Item 1. Business-Commercial Matters-Market Access Considerations” and “Item 1A. Risk Factors-Risks Related to the Development and Commercialization of our Product and our Product Candidates” and “-Risks Related to Regulatory Approval of our Product and our Product Candidates” for further information regarding the marketing authorization in the EEA, the market access process and related risks.

As the marketing authorization holder, we are obligated to monitor the use of Translarna for nmDMD to detect, assess and take required action with respect to information that could impact the safety profile of Translarna and to report this information, through pharmacovigilance submissions, to the EMA. Following its assessment of these submissions, the EMA can recommend to the European Commission actions ranging from the continued maintenance of the marketing authorization to its withdrawal.

United States

Translarna is an investigational new drug in the United States. During the first quarter of 2017, we filed a New Drug Application, or NDA, for Translarna for the treatment of nmDMD over protest with the FDA. In October 2017, the Office of Drug Evaluation I of the FDA issued a Complete Response Letter, or CRL, for the NDA, stating that it was unable to approve the application in its current form. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied our appeal of the CRL. In its response, the Office of New Drugs recommended a possible path forward for our ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness and safety of ataluren with new data to be generated on dystrophin production in nmDMD patients’ muscles. We followed the FDA’s recommendation and collected, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, and announced the results of Study 045 in February 2021. Study 045 did not meet its pre-specified primary endpoint. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Following this announcement, we submitted a meeting request to the FDA to gain clarity on the regulatory pathway for a potential re-submission of an NDA for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support NDA re-submission. We recently had an informal meeting with the FDA, during which we discussed the potential path to an NDA re-submission for Translarna. Based on the meeting discussion, we plan to request an additional Type C meeting with the FDA in the near future to review the totality of data collected to date, including dystrophin and other mechanistic data as well as additional analyses that could support the benefit of Translarna.

See “Item 1. Business-Government Regulation-The new drug and biologic approval process” below for further discussion with respect to the NDA process. See “Item 1. Business-Translarna (ataluren)” and “Item 1A. Risk Factors-Risks Related to the Development and Commercialization of our Product and our Product Candidates” and “-Risks Related to Regulatory Approval of our Product and our Product Candidates” for further detail regarding the results of our completed trials and studies of Translarna for the treatment of nmDMD, our regulatory strategy in the United States, our history with submissions to the FDA and the related risks to our business.

Other Territories

Translarna received marketing authorization for the treatment of nmDMD in Israel and South Korea in 2015, Chile in 2018, Brazil in 2019 and Russia in 2020, in addition to approvals from other countries, and these licenses are currently active. Many territories outside of the EEA, including Israel, South Korea and Chile, reference and depend on the determinations by the EMA when considering the grant of a marketing authorization. It is unlikely that we would be able to maintain our marketing authorizations in these regions in the event the EMA decides not to renew or otherwise modifies or withdraws our marketing authorization in the EEA. The marketing authorization for Translarna in Brazil and Russia are subject to renewal every five years. We have been pursuing and expect to continue to pursue marketing authorizations for Translarna for the treatment of nmDMD in other regions.

Emflaza for the treatment of Duchenne muscular dystrophy in the United States

Emflaza, both in tablet and suspension form, received approval from the FDA in February 2017 as a treatment for DMD in patients five years of age and older in the United States. In June 2019, the FDA approved our label expansion request for Emflaza for patients two to five years of age. We estimate that there are between approximately 10,000 and 15,000 DMD patients in the United States.

Emflaza has a seven-year exclusive marketing period in the United States for its approved indications, commencing on the date of FDA approval, under the provisions of the Orphan Drug Act of 1983, or the Orphan Drug Act. See “Item 1. Business-Government Regulation” for further discussion with respect to marketing protection we rely on.

Upstaza

Upstaza is a gene therapy for the treatment of AADC deficiency, a rare CNS disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. We are also preparing a BLA for Upstaza for the treatment of AADC deficiency in the United States and we expect to submit a BLA to the FDA in the first half of 2023.

AADC is the enzyme responsible for the conversion of L-dopa to dopamine. Dopamine is a key neurotransmitter that acts within the striatum (caudate and putamen), a component of the brain’s deep grey matter, to modulate output of neurons that project to the motor and premotor cortices of the brain that plan and execute normal motor function. Dopamine is required in the brain for humans to develop and maintain proper motor function.

AADC deficiency is a monogenic disorder of neurotransmitter synthesis that manifests in young children and most commonly results in profound developmental delay, often seen as complete arrest of motor development. AADC deficiency generally causes the inability to develop motor control, resulting in breathing, feeding, and swallowing problems, frequent hospitalizations, and the need for life-long care. On average, patients with AADC deficiency die in the first decade of life due to profound motor dysfunction and secondary complications such as choking, hypoxia, and pneumonia. Currently, no treatment options are available for the underlying cause of the disorder, and care is limited to palliative options with significant burden on caregivers.

The prevalence of AADC deficiency has been estimated to be approximately 5,000 patients worldwide, with a live-birth incidence of up to 1 in 40,000 worldwide. While several diagnostic tests for AADC deficiency are available, we believe the condition remains largely undiagnosed or misdiagnosed and may be confused with cerebral palsy.

Upstaza is an adeno-associated virus, or AAV, gene therapy, which has been assessed in two completed clinical trials, and one ongoing trial. The two completed trials include a total of 18 children with severe AADC deficiency who were treated with a one-time total dose of 1.8 x 10^{11} vg of Upstaza during a single procedure in which the gene therapy was administered directly to the region of the brain, called the putamen, where dopamine is made and released. The targeted micro-dosing approach administering small amounts of gene therapy directly to focal regions of affected cells in the putamen has the benefit of keeping the supply requirements for materials low, improving access of the therapeutic gene to key cells, potentially limiting immune and complement-mediated responses and reducing the risk of off-target uptake and excretion of the gene therapy by the liver and kidneys. To date, results from these trials suggest that patients may have a gain of motor functions and improvement in cognitive scales following gene therapy administration and have shown significant increases in motor function, which contrasts with the published natural history.

The two completed clinical trials, AADC-1601, a trial in which patients were enrolled under individual compassionate use consents, and AADC-010, were both single-arm, open-label, interventional trials that enrolled a total of 18 patients. The primary and secondary objectives of these trials were to assess the safety and efficacy of Upstaza administered via bilateral putaminal-infusions in patients with severe AADC deficiency at a total one-time dose of 1.8 x 10^{11} vg. Study enrollment required a diagnosis of AADC deficiency, defined as decreased homovanilic acid, or HVA, and ***5***-hydroxyindoleacetic acid, or 5-HIAA, and elevated levels of L-DOPA in the cerebrospinal fluid, or CSF, the presence of more than one DDC gene mutation, and the presence of clinical symptoms of AADC deficiency (including developmental delay, hypotonia, dystonia, and oculogyric crisis), and a patient age of older than 2 years.

Patients were evaluated monthly for safety assessments and every three months for efficacy assessments that included tests of motor developmental testing (Peabody Developmental Motor Scale, Second Edition, or PDMS-2, and Alberta Infant Motor Scale, or AIMS) through the first year after treatment with Upstaza and at periodic intervals thereafter through five years following treatment. The PDMS-2 and AIMS are validated scales used to assess motor skills in young children. Pharmacodynamic testing of CNS AADC activity over time included analyses of CSF neurotransmitter metabolites and F-DOPA PET imaging intervals, also through five years.

8 patients were enrolled in the AADC-1601 study. 10 patients were enrolled in the AADC-010 study. In both studies, the average age of patients was less than 5 years of age.

At baseline, patients had no functional movement and failed to achieve any motor milestones, including head control, sitting or standing capabilities, consistent with the published natural history of severe AADC deficiency. Compared to baseline, at one-year and at five-years after Upstaza administration, patients had objective evidence of de novo dopamine production as visualized by F-DOPA PET imaging of the brain, consistent with successful and stable gene expression and enzyme activity over time.

Based on preliminary analysis, following administration of Upstaza, the combined group of patients showed significant improvements from baseline capabilities at one-year post-treatment in functional motor skills assessed with the PDMS-2 total score, as well as on the locomotion, grasping, visual-motor integration and stationary subscales. Significant improvements from baseline at one-year post-treatment were also observed for the combined group of patients on the AIMS total score and on the prone, supine, sit and stand subscales.

Compared to published natural history data, patients in these trials showed statistically significant improvements at both two- and five-year post-treatment in achievement of motor milestones of full head control (at 2 and 5 years), sitting unassisted (at 2 and 5 years) and standing with support (at 5 years), reinforcing the clinical benefit and sustainability of functional motor improvements.

Surgical injection of Upstaza in both completed trials was well tolerated, with no adverse events occurring during the surgical procedure. Adverse events were generally associated with the disease state. The most frequent adverse event associated with Upstaza was dyskinesia and these events completely resolved over time. No serious adverse events have been attributed to Upstaza.

The ongoing clinical trial, AADC-011, is a single-center, open-label trial to assess the efficacy and safety of Upstaza in patients with AADC deficiency. The primary outcomes for this trial include assessing a change in the PDMS-2 score and

measuring the change in the neurotransmitter metabolite HVA or 5-HIAA in the cerebrospinal fluid. 10 patients have been enrolled and treated to date. With these 10 patients, we now have 28 patients from our three trials being evaluated in safety and efficacy studies.

An end-of-phase 2 meeting was held with the FDA in July 2017, and the clinical, non-clinical and chemistry, manufacturing and control, or CMC, data available to date from the two completed clinical trials were reviewed. The FDA provided feedback indicating that the clinical and non-clinical data available to date were sufficient to support the submission of a BLA without undertaking additional trials or studies at this time. In a late 2019 interaction with the FDA, the agency requested additional information concerning the use of the commercial delivery system for Upstaza in young patients. In response to the FDA's request, we provided additional information concerning the use of the commercial cannula for Upstaza in young patients. In October 2022, we held a type C meeting with the FDA to discuss the details of a potential submission package for Upstaza. At such meeting, the FDA asked for additional bioanalytical data in support of comparability between the drug product used in the clinical studies and the commercial drug product. We have completed these analyses and provided the results to the FDA for review. We expect to submit a BLA to the FDA in the first half of 2023.

Upstaza for the treatment of AADC deficiency has orphan drug designation in the United States and EU, and rare pediatric disease designation in the United States, and upon BLA approval the FDA may grant us a priority review voucher.

Due to its orphan medicinal product designation by the EMA, we rely on a ten-year exclusive marketing period for Upstaza in the EEA, which may potentially be extended for two additional years if we receive approval for a pediatric exclusivity incentive. If Upstaza for the treatment of AADC deficiency receives FDA approval, we expect that Upstaza would have a twelve-year exclusive marketing period in the United States for the approved indication, commencing on the date of FDA approval, under the provisions of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, as well as a concurrent seven-year exclusive marketing period, which would commence on the date of FDA approval, under the provisions of the Orphan Drug Act. We expect to rely on the twelve-year BPCIA regulatory exclusivity and concurrent seven-year Orphan Drug Act exclusivity to commercialize Upstaza in the United States, if it is approved.

See "Item 1. Business-Government Regulation-The new drug and biologic approval process" below for further discussion with respect to the BLA process.

Tegsedi and Waylivra

In August 2018 we entered into a Collaboration and License Agreement with Akcea Therapeutics, Inc., or Akcea, a subsidiary of Ionis Pharmaceuticals, Inc., or Ionis, for the commercialization by us of Tegsedi, Waylivra and products containing those compounds in countries in Latin America and the Caribbean, or the PTC Territory. See "Item 1. Business-Our Collaborations, License Agreements and Funding Arrangements-Tegsedi and Waylivra" below for further discussion with respect to this collaboration and license agreement.

Tegsedi

Tegsedi, a product of Ionis' proprietary antisense technology, is an antisense oligonucleotide, or ASO, inhibitor of human transthyretin, or TTR, production. Tegsedi is the world's first RNA-targeted therapeutic to treat patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. In October 2019, it received marketing authorization from ANVISA, the Brazilian health regulatory authority, for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hATTR amyloidosis in Brazil. Our marketing authorization for Tegsedi in Brazil is subject to renewal every five years. It has also received marketing authorization in the United States and EU for the same indication. We began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and we continue to make Tegsedi available in certain other countries within Latin America and the Caribbean through EAP programs.

hATTR amyloidosis is a progressive, systemic and fatal inherited disease caused by the abnormal formation of the TTR protein and aggregation of TTR amyloid deposits in various tissues and organs throughout the body, including in peripheral nerves, heart, intestinal tract, eyes, kidneys, central nervous system, thyroid and bone marrow. The progressive

accumulation of TTR amyloid deposits in these tissues and organs leads to sensory, motor and autonomic dysfunction often having debilitating effects on multiple aspects of a patient's life. Patients with hATTR amyloidosis often present with a mixed phenotype and experience overlapping symptoms of polyneuropathy and cardiomyopathy.

Ultimately, hATTR amyloidosis generally results in death within three to fifteen years of symptom onset. Therapeutic options for the treatment of patients with hATTR amyloidosis are limited and there are currently no disease-modifying drugs approved for the disease. There are an estimated 50,000 patients with hATTR amyloidosis worldwide, including approximately 6,000 patients with polyneuropathic hATTR amyloidosis in Latin America.

Waylivra

Waylivra is an ASO that has received marketing authorization in the EU for the treatment of FCS, subject to certain conditions. The United States and EU regulatory agencies have granted orphan drug designation to Waylivra for the treatment of FCS. In connection with the marketing approval for Waylivra in the EU, the European Commission is requiring Akcea to provide results of a study based on a registry of patients to investigate how blood checks and adjustments to frequency of injections are carried out in practice and how well they work to prevent thrombocytopenia and bleeding in FCS patients taking Waylivra. In August 2021, ANVISA approved Waylivra as the first treatment for FCS in Brazil and we began to make commercial sales of Waylivra in Brazil in the third quarter of 2022 while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP programs. Our marketing authorization for Waylivra in Brazil is subject to renewal every five years.

FCS is an ultra-rare disease caused by impaired function of the enzyme lipoprotein lipase, or LPL, and characterized by severe hypertriglyceridemia (>880mg/dL) and a risk of unpredictable and potentially fatal acute pancreatitis. Because of limited LPL function, people with FCS cannot break down chylomicrons, lipoprotein particles that are 90% triglycerides. In addition to pancreatitis, FCS patients are at risk of chronic complications due to permanent organ damage. They can experience daily symptoms including abdominal pain, generalized fatigue and impaired cognitions that affect their ability to work. People with FCS also report major emotional and psychosocial effects including anxiety, social withdrawal, depression and brain fog. There is no effective therapy for FCS currently available.

Additionally, we received approval of Waylivra for the treatment of FPL in Brazil in December 2022. FPL is a rare genetic metabolic disease characterized by selective, progressive loss of body fat (adipose tissue) from various areas of the body leading to ectopic fat deposition in liver and muscle and development of insulin resistance, diabetes, dyslipidemia and fatty liver disease. Individuals with FPL often have reduced subcutaneous fat in the arms and legs and the head and trunk regions may or may not have loss of fat. Conversely, affected individuals may also have excess subcutaneous fat accumulation in other areas of the body, especially the neck, face and intra-abdominal regions.

Evrysdi

Evrysdi was approved by the FDA in August 2020 for the treatment of SMA in adults and children two months and older and by the European Commission in March 2021 for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020 and Japan in June 2021. In May 2022, the FDA approved a label expansion for Evrysdi to include infants under two months old with SMA and we expect the EMA to make a regulatory decision on approval for a label expansion for Evrysdi to include infants under two months old with SMA in the 2023. Evrysdi is a product of our SMA program and our collaboration with Roche and the SMA Foundation. For additional information, see "Item 1. Business – Our Collaborations, License Agreements and Funding Arrangements – Roche and the SMA Foundation."

SMA is a genetic neuromuscular disease characterized by muscle wasting and weakness. The disease generally manifests early in life. SMA is caused by mutation or deletion of the Survival of Motor Neuron 1, or SMN1, gene that encodes the survival of motor neuron, or SMN, protein. The SMN protein is critical to the health and survival of the nerve cells in the spinal cord responsible for muscle contraction. A second gene, Survival of Motor Neuron 2, or SMN2, is very similar to SMN1, contains a T nucleotide at position 6 in exon 7 and produces low, insufficient levels of functional SMN protein due to alternative splicing of exon 7. According to the SMA Foundation, SMA is the leading genetic cause of death in

infants and toddlers. Approximately 1 in 10,000 children is born with the disease. We estimate that there are between 20,000 to 30,000 children and adults living with SMA in the United States, Europe and Japan.

Using our splicing technology and in collaboration with the SMA Foundation and Roche, we identified highly potent small molecule splicing modifiers that, in non-clinical studies in cultured cells derived from patients with SMA, increased both the inclusion of exon 7 in the SMN2 messenger RNA, or mRNA, transcript and the levels of SMN protein produced by the SMN2 gene. Importantly, in studies in transgenic mice carrying only the SMN2 gene, these orally bioavailable compounds penetrated the blood-brain barrier and increased the levels of full-length SMN2 mRNA and protein in brain, spinal cord, muscle and other tissues. In these same mouse studies, treatment with these compounds resulted in increased survival, restoration of body weight, prevention of motor neuron loss and improved motor function.

Diversified Development Pipeline

Our pipeline has a number of development programs in the clinical stages. These include splicing, Bio-e, metabolic, gene therapy and oncology programs as well as studies in our current commercial products for maintaining authorizations, label extensions and additional indications.

Splicing Platform

Our splicing platform focuses on the development of innovative therapies for diseases, such as SMA, that involve regulation of mRNA splicing in the cell.

In addition to Evrysdi and our SMA program, our splicing platform also includes PTC518, which is being developed for the treatment of HD. HD is a neurodegenerative and progressive brain disorder caused by a toxic gain-of-function triplet repeat expansion in the Huntingtin gene resulting in uncontrolled movements and cognitive loss. There are currently no disease-modifying therapies approved to delay the onset or slow the progression of HD. We believe that there are approximately 135,000 HD patients globally. PTC518 is an orally bioavailable molecule with broad central nervous system and systemic distribution that has been designed to target Huntingtin protein expression with high selectivity and specificity. We announced the results from our Phase 1 study of PTC518 in healthy volunteers in September 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid and protein levels, that PTC518 efficiently crosses blood brain barrier at significant levels and that PTC518 was well tolerated. We initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. Enrollment in the Phase 2 study remains active and ongoing outside of the United States. Enrollment within the United States is paused as the FDA has requested additional data to allow the Phase 2 study to proceed; discussions are ongoing with the FDA to allow the resumption of U.S. enrollment. We expect data from the initial 12-week phase of the Phase 2 study in the second quarter of 2023.

Our splicing platform clinical development program also includes several studies evaluating Evrysdi in a broad SMA patient population covering the ages from newborns to 60 years old that remain ongoing. In addition to the Firefish (infantile onset SMA; age at enrollment of one to seven months) and Sunfish (later onset SMA; age at enrollment of two to 25 years) studies that established the safety profile of Evrysdi, the Jewelfish (patients who previously received other SMA targeted therapies; age at enrollment of six months to 60 years) and Rainbowfish (pre-symptomatic patients; age at enrollment of newborns to 6 weeks) studies may potentially be used to support future applications for label extensions.

Jewelfish, an open-label study investigating the safety, tolerability, pharmacokinetics, and pharmacokinetics/pharmacodynamic relationship of Evrysdi in patients aged from 6 months to 60 years with SMA previously treated with one of several experimental or approved SMA therapies, initiated in the first quarter of 2017. Preliminary pharmacodynamic data from twelve Jewelfish patients presented in October 2018 at the World Muscle conference demonstrated sustained >2-fold increase in median SMN protein levels versus baseline over 12 months of treatment. Updated two-year results were presented in October 2022 at the World Muscle conference demonstrating an overall stabilization in the mean score of exploratory efficacy outcomes from baseline to Month 24. Also, Evrysdi was well tolerated, with no drug-related adverse events leading to withdrawal from the study. The study has completed recruitment.

Rainbowfish is an open-label, single-arm, multicenter study, investigating the efficacy, safety, pharmacokinetics and pharmacodynamics of Evrysdi in babies, from birth to six weeks of age (at first dose) with genetically diagnosed SMA who are not yet presenting with symptoms. The study has completed recruitment. Interim data from Rainbowfish showed that 80 percent of pre-symptomatic infants with SMA treated with Evrysdi for at least 12 months achieved motor milestones such as sitting without support, rolling, crawling, standing unaided, and walking independently.

Bio-e Platform

Our Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. Oxidation-reduction, or redox, reactions are an essential component of the generation and regulation of energy in living systems. These reactions are regulated through a set of enzymes known as oxidoreductase enzymes that uniquely require the transfer of an electron, or a redox chemical reaction, to affect their biological activity.

One of the advanced molecules in our Bio-e platform is vatiquinone. Vatiquinone is a small molecule orally bioavailable compound that has been in development for inherited mitochondrial diseases and related genetic disorders of oxidative stress. Vatiquinone targets 15-lipoxygenase, or 15-LO, a key regulator of oxidative stress, lipid-based neuro-inflammation, alpha-synuclein oxidation and aggregation and cell death. In the third quarter of 2020, we initiated a registration-directed Phase 2/3 randomized, placebo-controlled trial of vatiquinone in approximately 60 children with mitochondrial disease associated seizures, called MIT-E. We have completed enrollment in this trial after previously experiencing delays in enrollment due to the COVID-19 pandemic. All subjects were followed for one month to ensure a baseline seizure frequency, and have been randomized to receive vatiquinone or placebo for six months. We anticipate results from the Phase 2/3 trial to be available in the second quarter of 2023. Mitochondrial disease associated seizures is a highly morbid condition of refractory seizures in patients with inherited mitochondrial disease. We estimate that there are approximately 20,000 patients with mitochondrial disease associated seizures globally. The clinical rationale for the MIT-E trial is based on reports of decreased seizure frequency, disruption of status epilepticus and reduced mortality risk and disease-associated morbidity recorded through compassionate use studies of vatiquinone in mitochondrial disease patients conducted in the United States and EU.

Additionally, we initiated a registration-directed Phase 3 trial of vatiquinone in approximately 120 patients with Friedreich ataxia in the fourth quarter of 2020, called MOVE-FA. The MOVE-FA trial is an 18-month parallel arm, placebo-controlled study evaluating vatiquinone versus placebo in children and young adults with Friedreich ataxia. We have completed enrollment for the MOVE-FA trial and we anticipate results to be available in the second quarter of 2023. Friedreich ataxia is a rare and life-shortening neurodegenerative disease caused by a single defect in the FXN gene which causes reduced production of the frataxin protein. We believe that there are approximately 25,000 Friedreich ataxia patients globally. Vatiquinone has previously been studied in Friedreich ataxia patients in a Phase 2 trial that included a six-month placebo-controlled phase followed by an 18-month open label extension. In this trial, long-term vatiquinone treatment (18-24 months) was associated with an improvement in overall disease severity and neurological function relative to natural history. Vatiquinone has been dosed in over 500 subjects and has been generally well-tolerated in the clinic.

The other advanced molecule in our Bio-e platform is utreloxastat, a small molecule orally bioavailable compound that targets 15-LO and is in development for the potential treatment of adult CNS patients. In the third quarter of 2021, we completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of utreloxastat. Utreloxastat was found to be well-tolerated with no reported serious adverse events while demonstrating predictable pharmacology. We initiated a Phase 2 trial of utreloxastat for ALS in the first quarter of 2022 and enrollment is ongoing. ALS is a rapidly progressing neurodegenerative disease caused by oxidative damage which leads to neuronal cell death and muscular atrophy. We believe that there are approximately 150,000 ALS patients globally.

Metabolic Platform

Our metabolic platform seeks to identify potential treatments for metabolic disorders. The most advanced molecule in our metabolic platform is sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products. Sepiapterin has been pursued as a possible treatment for orphan metabolic diseases associated with defects in the tetrahydrobiopterin biochemical pathways, including

PKU. PKU is an inborn error of metabolism caused predominantly by mutations in the phenylalanine hydroxylase gene resulting in toxic buildup of the amino acid phenylalanine, or Phe, in the brain, and, if left untreated, severe and irreversible disabilities such as permanent intellectual disability, seizures, delayed development, behavioral problems and possibly psychiatric disorders can occur. We believe that there are approximately 58,000 PKU patients globally. In December 2019, it was announced that the Phase 2 trial for sepiapterin as a potential treatment for PKU met its primary and secondary endpoints, achieving statistically-significant and clinically-meaningful reduction in blood Phe levels compared to both baseline and an active control group. We initiated a registration-directed Phase 3 trial for sepiapterin for PKU in the third quarter of 2021 with the primary endpoint in the study of achieving statistically-significant reduction in blood Phe level. The primary analysis population includes those patients who have a greater than 30% reduction in blood Phe levels during the Part 1 run-in phase of the trial. In January 2023, we announced preliminary data from the Part 1 run-in phase of this trial, including that the mean reduction in blood Phe levels in an initial cohort of subjects during the Part 1 would be recognized as clinically meaningful if maintained in Part 2 of the trial. We now expect results from Part 2 of this trial to be available in May 2023 as the trial is overenrolled and additional time is required for the entirety of the primary analysis population to complete the study.

Gene Therapy Platform

Our gene therapy platform focuses on the development of innovative therapies for rare, debilitating diseases of the CNS. In addition to Upstaza, our gene therapy platform includes an asset targeting Friedreich ataxia continues to undergo preclinical studies. We continue to work towards initiating a clinical study for this program. Additionally, the gene therapy platform includes our program targeting Angelman syndrome, a rare, genetic, neurological disorder characterized by severe developmental delays. We continue to work towards submitting a filing in support of the first-in-human study for this program.

Oncology Platform

Unesbulin is our most advanced oncology agent. Unesbulin is a small molecule inhibitor of tubulin polymerization that is associated with cell cycle arrest. In addition, administration is associated with a hyperphosphorylation of tumor BMI1 protein that subsequently leads to BMI1 protein degradation and reduction in BMI1 protein function. We have assessed unesbulin in a Phase 1 multi-center study in patients with advanced solid tumors. Unesbulin is also being evaluated in LMS in patients who have relapsed or are refractory to current treatments. LMS is a type of sarcoma that manifests as malignant soft tissue tumors of muscle tissue. Preclinical evaluations suggested that unesbulin had synergistic effects in combination with dacarbazine. Approximately 4,000 patients are diagnosed with LMS annually in the United States. We completed a Phase 1 dose escalation study of unesbulin for LMS in the fourth quarter of 2021. Unesbulin in combination with dacarbazine was found to be well-tolerated and a dose was selected for subsequent trials. We initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022 and enrollment is ongoing.

We are also assessing unesbulin for the treatment of DIPG. DIPG is a rapidly fatal pediatric cancer with 90% of patients dying within two years of diagnosis. There are approximately 300 patients diagnosed annually in the United States. We completed a Phase 1 dose-escalation trial in DIPG patients in the fourth quarter of 2021. The initiation of our registration-directed Phase 2 trial of unesbulin for DIPG was delayed as we continued to track the progress of patients in our Phase 1 trial and analyze the corresponding data. We now expect to initiate a registration-directed Phase 2/3 trial of unesbulin for the treatment of DIPG in the fourth quarter of 2023.

While we have decided to deprioritize the emvododstat program for the treatment of acute myeloid leukemia, or AML, we are exploring other potential treatments for AML as part of our oncology platform.

We received grant funding of $5.4 million for our oncology platform from the Wellcome Trust. To the extent that we develop and commercialize certain program intellectual property on a for-profit basis ourselves or in collaboration with a partner (provided we retain overall control of worldwide commercialization), we may become obligated to pay to Wellcome Trust development and regulatory milestone payments. Our first such milestone payment of $0.8 million to Wellcome Trust occurred in the second quarter of 2016. During the year ended December 31, 2022, we incurred $2.5 million of development milestones in connection with the enrollment of patients in the registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS, which is recorded in other long-term liabilities on the balance sheet and will

be payable upon the earlier to occur of the first dose administered to the last patient enrolled in the study or the termination of dosing of all patients in the study. Additional milestone payments of up to an aggregate of $14.5 million may become payable by us to Wellcome Trust under this agreement. For additional information, see "Item 1. Business – Our Collaborations and Funding Arrangements."

As part of our continuous portfolio review, we have decided to deprioritize our program for emvododstat for the treatment of COVID-19.

Translarna (ataluren)

Mechanism of action

We discovered Translarna by applying our technologies to identify molecules that promote or enhance the suppression of nonsense mutations. Nonsense mutations are implicated in a variety of genetic disorders. Nonsense mutations create a premature stop signal in the translation of the genetic code contained in mRNA and prevent the production of full-length, functional proteins. Based on our research, we believe that Translarna interacts with the ribosome, which is the component of the cell that decodes the mRNA molecule and manufactures proteins, to enable the ribosome to read through premature nonsense stop signals on mRNA and allow the cell to produce a full-length, functional protein. As a result, we believe that Translarna has the potential to be an important therapy for genetic disorders which are the result of a nonsense mutation. Genetic tests are available for many genetic disorders, including those noted above, to determine if the underlying cause is a nonsense mutation. Translarna has been generally well-tolerated in all of our clinical trials to date, which have enrolled over 1,000 individuals to date.

Planned and ongoing clinical development of Translarna in nonsense mutation Duchenne muscular dystrophy

Study 041

Overview. As a specific obligation to our marketing authorization in the EEA, we are required to conduct and submit to the EMA the results of a three-year clinical trial to confirm the efficacy and safety of Translarna in the treatment of ambulatory patients with nmDMD aged five years or older. The trial is comprised of two stages: an 18-month randomized, double-blind, placebo controlled clinical trial followed by an 18-month open label extension period. We refer to the 18-month clinical trial portion as "Stage 1" and the 18-month extension period as "Stage 2". We refer to Stage 1 and Stage 2 together as Study 041. In September 2022, as part of our specific obligation, we submitted a report on Stage 1 and data from Stage 2 in connection with a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization.

For a discussion of the risks related to conducting clinical trials, in general, and Study 041, in particular, please see "Item 1A. Risk Factors-Risks Related to the Development and Commercialization of our Product and our Product Candidates" and "-Risks Related to Regulatory Approval of our Product and our Product Candidates".

Enrollment. According to the study protocol, Study 041 enrolled nmDMD patients aged five years and above who achieve a 6-minute walk distance, or 6MWD, equal to or greater than 150 meters at three pre-treatment evaluation times (screening, baseline day one and baseline day two), tested as set forth in the protocol. Qualified participants also needed to perform timed function tests of running/walking 10 meters, climbing/descending four stairs and standing from supine within 30 seconds at both screening and baseline, and meet the other criteria set forth in the protocol.

We completed enrollment of Study 041 in the fourth quarter of 2020. Of the 363 patients enrolled in Study 041, 185 patients meet the criteria for inclusion in the primary analysis population, which we refer to as the modified intention-to-treat population, or mITT. Patients included in the mITT must be at least 7, but less than 16, years old, with a 6MWD of equal to or greater than 300 meters and a stand from supine time of five seconds or more, each as tested at screening and baseline.

Objectives and endpoints. The primary objective of Study 041 is to evaluate the effect of Translarna on ambulation and endurance as assessed by the 6-minute walk test, or 6MWT. Based on the study protocol, the primary analysis of Stage 1

was to evaluate the difference in slope of change in 6MWD from baseline to week 72 between Translarna and placebo in the mITT population. Data from participants who did not qualify for inclusion in the mITT were used for summary and analysis of efficacy endpoints.

Slope of change in 6MWD over 144 weeks will also be assessed as a secondary endpoint at the conclusion of Stage 2, and the consistency of the results at 144 weeks against week 72 will be assessed. Changes in 6MWD from baseline to week 72 and week 144 respectively will also be assessed as secondary endpoints.

A secondary objective of Study 041 is to determine the effects of Translarna on ambulation and burst activity as assessed by timed function tests (10-meter run/walk, 4-stair stair-climb, and 4-stair stair descend). Each timed function test was analyzed as a secondary endpoint for both the mITT and ITT populations at the end of Stage 1 and will also be analyzed at the end of Stage 2. A separate analysis evaluates 10-meter run/walk results in participants with a baseline 6MWD below 300 meters. An additional analysis evaluates a composite endpoint of average change in times to run/walk 10 meters, climb 4 stairs, and descend 4 stairs. We also assess each of time to loss of ambulation, stair-climbing and stair-descending over 72 weeks and over 144 weeks.

Determination of the effects of Translarna on lower-limb muscle function as assessed by the North Star Ambulatory Assessment, or NSAA, a functional scale designed for boys affected by DMD, serves as an additional secondary objective. NSAA scores were analyzed as secondary endpoints for both the mITT and ITT populations at the end of Stage 1 and will also be analyzed as at the end of Stage 2. A separate analysis for Stage 2 will evaluate changes in total score in participants with a baseline 6MWD of equal to or greater than 400 meters and under 7 years of age. We also assess the risk of loss of NSAA items over 72 weeks and 144 weeks.

The safety profile of Translarna has been evaluated throughout Stage 1 and will continue to be evaluated for Stage 2 as a secondary objective.

Certain exploratory endpoints are also assessed in Study 041. In patients aged 7 years and above, change from baseline in upper limb function is assessed using both functional testing and parent/caregiver-reported questionnaires. In patients under 7 years of age, muscle strength is assessed by change from baseline in myometry parameters. At pre-qualified sites only, magnetic resonance imaging are used to assess change from baseline in muscle fat fraction. The effects of Translarna on pulmonary function are assessed by change from baseline in forced vital capacity. In addition, subject- and parent/caregiver-reported questionnaires and at-home diaries are assessed to evaluate the effect of Translarna on health-related quality of life (HRQL) changes from baseline.

Stratification. In Stage 1, participants were randomized 1:1 to placebo or Translarna (10, 10, 20 mg/kg). The randomization was stratified based on type of concomitant corticosteroid used at baseline (deflazacort versus prednisone/prednisolone), maximum of the two valid 6-minute walk tests performed at baseline day 1 and day 2 (<300 meters versus ≥300 to <350 meters, versus ≥350 to <400 meters, versus ≥400 meters), and time to stand from supine at baseline (<5 seconds versus ≥5 seconds).

Results. In June 2022, we announced top-line results from Stage 1. Within Stage 1, Translarna showed a statistically significant treatment benefit across the entire ITT population as assessed by the 6MWT as assessed by the NSAA. Additionally, Translarna showed a statistically significant treatment benefit across the ITT population within the 10-meter run/walk and 4-stair stair climb, while also showing a positive trend in the 4-stair stair descend although not statistically significant. Within the mITT population, Translarna demonstrated a positive trend across all endpoints, however, statistical significance was not achieved. Translarna was also well tolerated.

Observational study, data collection, and open label, extension trials of Translarna for treatment of nmDMD

We are undertaking a multi-center, observational post-approval study of patients receiving Translarna on a commercial basis, or Study 025o, as required by the Pharmacovigilance Risk Assessment Committee of the EMA and in collaboration with TREAT-NMD and the Cooperative International Neuromuscular Research Group. During the study we will gather data on the safety, effectiveness, and prescription patterns of Translarna in routine clinical practice. We have successfully enrolled more than 200 patients in Study 025o and we expect to follow their progress over five years.

Pursuant to a temporary managed access agreement entered into in July 2016 between us, the UK National Institute for Health and Care Excellence, or NICE, National Health Services England, or NHS England, and other interested parties, the NorthStar Network is collecting data on the efficacy of Translarna for the treatment of nmDMD as measured by the NorthStar Ambulatory Assessment test. Patients receiving Translarna will be compared to an historical natural history population as well as a matched control group in order to assess response to treatment over the period specified in the managed access agreement.

An open label, extension trial, Study 016, involving patients who participated in ACT DMD is also ongoing, across multiple sites in the United States and Canada with patients on commercial supply. We ended the two open label extension trials involving patients who had participated in our prior trials for nmDMD and have transitioned U.S. and Canadian patients from these trials to Study 016 while other patients have transitioned to commercial supply via commercial pathways or EAP programs.

Completed clinical trials of Translarna in nonsense mutation Duchenne muscular dystrophy

Phase 2 pediatric study

As part of our pediatric development commitments under our marketing authorization in the EEA and to support the potential expansion of the Translarna label to younger patients with nmDMD, we initiated a Phase 2 pediatric clinical study to evaluate the safety and pharmacokinetics of Translarna in patients two to five years of age. The study, initiated in June 2016, included a four-week screening period, a four-week study period, and a 48-week extension period for patients who complete the four-week study period (52 weeks total treatment). In July 2018, the EMA approved a label-extension request to our marketing authorization for Translarna in the EEA to include patients from two to up to five years of age, based on data from this study.

Phase 3 clinical trial of Translarna for nmDMD (ACT DMD)

In October 2015, we announced results from ACT DMD, also referred to as Study 020, our Phase 3, double-blind, placebo-controlled, 48-week clinical trial to evaluate the safety and efficacy of Translarna in patients with nmDMD. ACT DMD involved 228 patients at 53 sites across 18 countries.

In the overall intent-to-treat, or ITT, study population, the primary endpoint of change from baseline at week 48 in the 6MWT, showed a 15 meter benefit in favor of Translarna, which did not meet statistical significance.

A summary of the safety and efficacy results from ACT DMD is outlined below.

Safety and tolerability. The results of ACT DMD confirmed the favorable safety profile of Translarna seen in our 48-week, 174-patient Phase 2b double-blind, placebo controlled clinical trial evaluating the long-term safety and efficacy of Translarna in patients with nmDMD completed in 2009, or the Phase 2b trial.

Translarna was generally well tolerated at both dose levels in our Phase 2b clinical trial. There were no study discontinuations due to adverse events. Most treatment-emergent adverse events were mild or moderate in severity. Investigators' attributions of drug-related adverse effects were generally similar across the placebo and Translarna arms. The most common adverse events in this trial were vomiting (46.6% overall), headache (29.3%), diarrhea (24.1%), nasopharyngitis (20.7%), fever (19.0%), cough (19.0%) and upper abdominal pain (17.8%). These events were generally balanced across treatment arms and are typical of pediatric illnesses. Adverse events with at least a 10% incidence in any treatment arm that were seen with increased frequency from the placebo group to the Translarna 40 mg dose group to the Translarna 80 mg dose group were nausea (12.3% for placebo, 14.0% for the Translarna 40 mg group and 16.7% for the Translarna 80 mg group), abdominal pain (7.0% for placebo, 12.3% for the Translarna 40 mg group and 16.7% for the Translarna 80 mg group), pain in extremity (10.5% for placebo, 12.3% for the Translarna 40 mg group and 13.3% for the Translarna 80 mg group), flatulence (7.0% for placebo, 8.8% for the Translarna 40 mg group and 11.7% for the Translarna 80 mg group) and nasal congestion (7.0% for placebo, 8.8% for the Translarna 40 mg group and 10.0% for the Translarna 80 mg group). There were no serious adverse events observed during the trial that were considered possibly or probably

related to Translarna. Determination of relatedness of the serious adverse event to Translarna was made by the trial investigator, based on his or her judgment.

Translarna was generally well tolerated in ACT DMD. There were two study discontinuations due to adverse events, including one in the Translarna arm (constipation) and one in the placebo arm (disease progression). Most treatment-emergent adverse events were mild or moderate in severity. The most common adverse events in this trial were vomiting (20.4% overall), nasopharyngitis (20.0%), headache (18.3%), and fall (17.8%). These events were generally balanced across treatment arms and are typical of pediatric illnesses and/or patients with DMD. Adverse events with at least a 10% incidence in either treatment arm that were seen with increased frequency from the placebo group to the Translarna 40 mg dose group were vomiting (18.3% for placebo, 23.6% for the Translarna 40 mg group), nasopharyngitis (19.1% for placebo, 20.9% for the Translarna 40 mg group), fall (17.4% for placebo, 18.3% for the Translarna 40 mg group), cough (11.3% for placebo, 16.5% for the Translarna 40 mg group) diarrhea (8.7% for placebo, 17.4% for the Translarna 40 mg group), and pyrexia (10.4% for placebo, 13.9% for the Translarna 40 mg group). An overview of adverse events in this trial is shown in the table below.

Overview of treatment-emergent adverse events in Phase 3 clinical trial (as-treated population)

Parameter	Placebo N=115	Translarna 40 mg group N=115	All patients N=230
Patients with ≥1 adverse event	101 (87.8)%	10,3(89.6)%	20,4(88.7)%
Adverse events by severity			
Grade 1 (mild)	54 (47.0)%	6,1(53.0)%	11,5(50.0)%
Grade 2 (moderate)	37 (32.2)%	3,5(30.4)%	7,2(31.3)%
Grade 3 (severe)	9 (7.8)%	76(.1)%	167(.0)%
Grade 4 (life-threatening)	—	—	—
Adverse events by relatedness			
Unrelated	47 (40.9)%	4,4(38.3)%	9,1(39.6)%
Unlikely	30 (26.1)%	2,0(17.4)%	5,0(21.7)%
Possible	18 (15.7)%	2,7(23.5)%	4,5(19.6)%
Probable	6 (5.2)%	1,2(10.4)%	187(.8)%
Discontinuations due to adverse events	1 (0.9)%	10(.9)%	20(.9)%
Serious adverse events	4 (3.5)%	43(.5)%	83(.5)%
Deaths	—	—	—

There were no serious adverse events observed during the trial that were considered possibly or probably related to Translarna. Determination of relatedness of the serious adverse event to Translarna was made by the trial investigator, based on his or her judgment.

Intent to Treat (ITT) Population. The primary efficacy endpoint in ACT DMD was change in 6MWD from baseline to week 48. In the ITT population, a 15 meter benefit (p=0.213) was observed in the primary endpoint which did not meet statistical significance.

Secondary endpoints in the trial included the proportion of patients with at least 10% worsening in 6MWD at week 48 of the trial compared to baseline, or 10% 6MWD worsening, and change in timed function tests of time to run/walk 10 meters, climb four stairs and descend four stairs. The hazard ratio for Translarna versus placebo was 0.75 (p=0.160) for 10% 6MWD worsening. Benefits trended in favor of Translarna over placebo in the timed function tests in the ITT population, including observed results in time to run/walk 10 meters (1.2 seconds; p=0.117), time to climb four stairs (1.8 seconds; p=0.058), and time to descend four stairs (1.8 seconds; p=0.012).

Additional endpoints included the NSAA test and the Pediatric Outcomes Data Collection Instrument, or PODCI, a validated tool for measuring quality of life in pediatric patients with orthopedic conditions. These additional endpoints favored Translarna in the ITT population but did not meet statistical significance.

Pre-Specified Analyses. The statistical analysis plan submitted to the FDA for ACT DMD set forth pre-specified analyses of efficacy to be conducted, including subgroups of patients with baseline 6MWD less than 350 meters and patients with baseline 6MWD of greater than or equal to 300 and less than 400 meters, which we refer to as our key subgroups.

The pre-specification of our key subgroups was scientifically justified based upon knowledge of the biology and natural history of the disease and the evolving understanding of the of the six minute walk test as used to assess DMD patients. We considered the pre-specified less than 350 meter baseline 6MWD population as a key subgroup based on the knowledge that 350 meters represents a transition point for patients towards a more rapid decline in walking ability as supported by analysis from our Phase 2b trial. Furthermore, we considered the pre-specified 300 to 400 meter baseline 6MWD population as a key subgroup based on an increasing understanding of the sensitivity limitations of the six minute walk test as an endpoint in 48-week studies. Natural history data suggest that the 6MWT may not be the optimal tool to demonstrate efficacy in patients with either a baseline 6MWD of less than 300 meters, as these patients have significant muscle loss as monitored by magnetic resonance spectroscopy and are at high risk for losing ambulation regardless of treatment, or in high walking patients, such as those with a baseline 6MWD at or greater than 400 meters, as these patients are likely to remain stable over a 48 week testing period.

By defining these key subgroups, we thereby also defined corresponding subgroups of patients with baseline 6MWD greater than or equal to 350 meters, greater than or equal to 400 meters, and less than 300 meters. We also pre-specified a meta-analysis of the combined results from ACT DMD and the Phase 2b ambulatory decline phase patients.

Pre-specified sub-group analysis. We saw strong evidence of clinical benefit in the pre-specified subgroup of patients with baseline 6MWD between 300 and 400 meters. Specifically, we observed a benefit in Translarna-treated patients of 47 meters (nominal p=0.007) in the 6MWT in this subgroup. This was consistent with an observed benefit of 49 meters (nominal p=0.026) in our Phase 2b clinical trial in the 300 to 400 meters baseline 6MWD population. We also saw clinically meaningful benefit for Translarna over placebo in each of the timed function tests, including observed results in time to run/walk 10 meters (2.1 seconds; nominal p=0.066), time to climb four stairs (3.6 seconds; nominal p=0.003), and time to descend four stairs (4.3 seconds; nominal p<0.001). The hazard ratio for Translarna versus placebo was 0.79 (nominal p=0.418) for 10% 6MWD worsening. In addition, a benefit of 4.5 points over placebo (nominal p=0.041) was observed in the NSAA test, which we believe is clinically meaningful. We believe that the benefits observed in this key pre-specified subgroup support the use of the 6MWT in the patients with a walking ability in the 300 to 400 meters range and the understanding that the reliability of the 6MWT over a 48 week period was limited at both the lower and upper ends of our 6MWD enrollment range.

In the pre-specified subgroup of patients with baseline 6MWD less than 350 meters, we observed a benefit of 24 meters (nominal p=0.210) in favor of Translarna in the 6MWT. An analysis of the results from our Phase 2b clinical trial in the less than 350 meters baseline 6MWD population, defined post-hoc, demonstrated a 68 meter benefit in the 6MWT (nominal p=0.006). In the timed function tests for the subgroup of ACT DMD patients with baseline 6MWD less than 350 meters, we observed benefits for Translarna over placebo in time to run/walk 10 meters (2.3 seconds; nominal p=0.033), time to climb four stairs (4.2 seconds; nominal p=0.019) and time to descend four stairs (4.0 seconds; nominal p=0.007).

Typically, a trial result is statistically significant if the chance of it occurring when the treatment is like placebo is less than one in 20, resulting in a p-value of less than 0.05. A nominal p-value is the result of one particular comparison when more than one comparison is possible, such as when two active treatments are compared to placebo or when two or more subgroups are analyzed.

As described above, we believe the 6MWT lacks sensitivity to detect a clinical effect in patients with baseline less than 300 meters in a 48-week trial. However, the timed function tests trended in favor of patients treated with Translarna with a baseline 6MWD below 300 meters, including observed benefit over placebo in time to run/walk 10 meters (2.5 seconds; nominal p=0.066), time to climb four stairs (2.4 seconds; nominal p=0.790), and time to descend four stairs (2.1 seconds; nominal p=0.595). We believe the positive trends in this population reflect that short muscle burst activity tests may be a better clinical measure for patients that are at a more advanced stage of disease progression. Consistent with the natural history of ambulatory DMD patients with 6MWD greater than 400 meters, which indicates stability in walking ability over a 48 week period, we observed no meaningful difference in 6MWT between patient groups. Similarly, we observed no meaningful difference in 6MWT between patient groups with baseline 6MWD greater than 350 meters.

Pre-specified meta-analysis. The meta-analysis combined efficacy results from the ACT DMD ITT population and Phase 2b ambulatory decline phase subgroup. The Phase 2b ambulatory decline phase group includes the patients from our randomized, double-blind, placebo controlled, Phase 2b clinical trial in patients with nmDMD who would have met the enrollment criteria of ACT DMD.

Results from the meta-analysis showed a statistically significant 21 meter improvement in 6MWD ($p = 0.015$) favoring Translarna.

Additionally, the meta-analysis showed statistically significant benefit for Translarna over placebo across each timed function test including time to run/walk 10 meters (1.4 seconds; $p=0.025$), time to climb four stairs (1.6 seconds; $p =0.018$) and time to descend four stairs (2.0 seconds; $p=0.004$). The hazard ratio for Translarna versus placebo was 0.66 ($p=0.023$) for 10% 6MWD worsening. We believe that we are able to demonstrate a statistically significant outcome in the 6MWD in the meta-analysis, despite the significant variability in baseline 6MWD among patients in both ACT DMD and the Phase 2b trial's ambulatory decline phase, due to the substantially larger patient population available in the pooled analysis.

Retrospective Analysis. We also looked back at the observed results in the meta-analysis for all patients with a baseline 300 to 400 meter 6MWD from ACT DMD and the Phase 2b trial. The meta-analysis of these data demonstrated a 45 meter benefit (nominal $p<0.001$) in the 6MWT as well as clinically meaningful benefits across each secondary endpoint timed function test, including benefit over placebo in time to run/walk 10 meters (2.2 seconds; nominal $p=0.008$), time to climb four stairs (3.4 seconds; nominal $p<0.001$) and time to descend four stairs (4.3 seconds; nominal $p<0.001$). This meta-analysis of patients with baseline 6MWD of 300 to 400 meters was not pre-specified and is defined post-hoc.

A retrospective analysis performed after unblinding trial results can result in the introduction of bias if the analysis is inappropriately tailored or influenced by knowledge of the data and actual results. In addition, nominal p-values cannot be compared to the benchmark p-value of 0.05 to determine statistical significance without being adjusted for the testing of multiple dose groups or analyses of subgroups. Because of these limitations, regulatory authorities typically give greatest weight to results from pre-specified analyses and adjusted p-values and less weight to results from post-hoc, retrospective analyses and nominal p-values.

Statistical Considerations. The pre-specified meta-analysis results, which favored Translarna in the 6MWT and each of the timed function tests, are considered statistically significant. In the pre-specified subgroups of ACT DMD patients with a baseline 6MWD less than 350 meters and 300 to 400 meters, the p-values for the 6MWT and each of the timed function tests are considered nominal. For information with respect to the use of nominal p-values and post-hoc analyses, see Item 1A. Risk Factors, *"Subgroup, retrospective, post-hoc, and certain statistical analyses may not be reliable and typically will not form the basis for regulatory approval."*

Participation Criteria and Stratification. Certain key inclusion criteria were specified in the ACT DMD trial protocol for enrollment: the patient had to be 7 through 16 years of age; at the screening visit the patient had to be able to walk no more than 80% of predicted 6MWD compared to healthy boys matched for age and height, but had to be able to walk at least 150 meters during the 6MWT; and the patient must have used systemic corticosteroids for a minimum of six months prior to start of treatment. The ACT DMD trial protocol provided for the exclusion of patients from the trial if, among other things, they recently used systemic aminoglycoside antibiotics, recently initiated or changed corticosteroid therapy or previously received Translarna treatment. Patients enrolled in ACT DMD underwent 48 weeks of blinded treatment prior to the final analysis and the randomization was stratified based on age (<9 years versus ≥9), baseline 6MWD (<350 versus ≥350 meters), and duration of prior use of corticosteroids (<12 months versus ≥12 months).

Study 045

In the fourth quarter of 2018, following the FDA's recommendation to collect dystrophin data using validated quantification methods, we initiated Study 045, a Phase 2 open label clinical study of 20 boys with nmDMD from ages two to seven, to evaluate the ability of ataluren to increase dystrophin protein levels in boys with nmDMD. Study 045 did not meet its pre-specified primary endpoint. Patients received baseline biopsies prior to the initiation of treatment and follow-up biopsies scheduled at 40 weeks following the start of treatment. However, certain patients were delayed in obtaining the final study muscle biopsies performed at our clinical trial site at the University of California, Los Angeles

as a result of the COVID-19 pandemic. 8 of 20 patients were unable to undergo biopsies at week 40, and these patients had their second biopsies between 62 and 70 weeks of treatment. Full-length dystrophin levels were measured using both the Electrochemiluminescence, or ECL assay, as the primary endpoint and Immunohistochemistry, or IHC, assay as the secondary endpoint.

The ITT population included the 20 patients enrolled in the study. However, one subject was determined to be non-compliant, as he only took half of the study drug, and one subject did not have adequate biopsy samples to establish baseline levels. Therefore, 18 patients were compliant with the study drug and had evaluable biopsy samples. These 18 patients are considered the evaluable population. 10 of these 18 patients had their second biopsy at week 40 and 8 had their second biopsy between weeks 62 and 70. Patient characteristics, including age and steroid use were consistent across both cohorts.

Overall in the ITT population, there was an increase in dystrophin expression from baseline, on both ECL as the primary endpoint and IHC as the secondary endpoint, but these did not meet a p-value of <0.05. Nevertheless, when studying the 18 patients in the evaluable cohort, we identified a greater increase in dystrophin expression, and this increase did reach a nominal p-value of 0.04 in the analysis of the IHC assay. Also, over 80% of the evaluable subjects demonstrated an increase in dystrophin expression. 8 patients in the evaluable population had longer treatment exposure, ranging from 62-70 weeks, and these 8 patients had markedly greater levels of dystrophin increase with an average of approximately 24% in the ECL assay. We believe that these results suggest that longer duration of treatment resulted in greater biological effect, which is consistent with the long-term Translarna treatment benefit we have previously reported from our other clinical studies and our international drug registry for DMD patients receiving Translarna.

We also measured creatine kinase, or CK, levels of patients in Study 045 as an objective measure of muscle damage. Dystrophin acts as a shock absorber during a muscle contraction and would be expected to protect against muscle damage and therefore reduce CK levels. Consistent with an increase in the level of dystrophin, we observed a marked reduction of approximately 20% in creatine kinase and that longer treatment with Translarna was associated with a greater magnitude of biological effect.

Multi-platform Discovery

We continue to invest in our pre-clinical product pipeline by committing significant resources to research and development programs and business development opportunities within our areas of scientific expertise, including potential collaborations, alliances, and acquisitions or licensing of assets that complement our strategic mission to provide access to best-in-class treatments for patients who have an unmet medical need.

Our Approach

We use multiple drug discovery platforms to discover and develop therapies to target diseases with high-unmet need. Intervening at DNA, RNA and energy production pathways are post-transcriptional control processes, which are the events that occur in a cell following the transcription of DNA into RNA. These processes regulate, for example, how long RNA molecules exist in the cell, how precursor messenger RNA, or pre-mRNA, molecules are processed (spliced), and how efficiently mRNA molecules are translated into proteins. Additionally, several regions of mRNA do not code for the protein and are known as untranslated regions, or UTRs. They are unique to specific mRNAs or groups of mRNAs and are directly involved in the post-transcriptional control of protein production. Interactions of cellular factors with the UTRs in the mRNA determine when and how much protein is produced as well as how mRNA is degraded and eliminated from the cell.

Splicing

Post-transcriptional control processes are the events that occur in a cell following the transcription of DNA into RNA. These processes regulate, for example, how long RNA molecules last in the cell, how exons in precursor messenger RNA, or pre-mRNA, molecules are spliced, and how efficiently mRNA molecules are translated to proteins. In the majority of human protein-encoding genes, the sequence encoding the mature mRNA transcript is not contiguous in the pre-mRNA

but rather has intervening non-coding regions called introns that interrupt the coding sequences, called exons. These introns are removed from the final mRNA product by a process called splicing that also joins the exons together such that only the exons are retained in the mature mRNA.

We use our splicing technology to identify molecules that modulate splicing of the pre-mRNA. Pre-mRNA splicing is a series of highly organized biochemical reactions. Approximately 94% of all human genes encode pre-mRNAs that undergo splicing. In addition, through splicing, one gene can often generate several mRNA products that include a different set of exons through a process called alternative splicing which results in mature mRNA that encodes different, related proteins. Splicing can be therapeutically targeted, in many human diseases, including SMA, Huntington's disease, muscular dystrophy and various forms of cancer. We have developed several high-throughput drug discovery technology platforms that enable us to identify small molecule modifiers of pre-mRNA splicing. These technologies rely on sensitive quantification of pre-mRNA isoforms directly in human cells or tissue samples. Using this technology, we have successfully identified orally bioavailable small molecules that correct splicing of SMN2 mRNA. An example of one of these molecules is Evrysdi, which was approved in August 2020 by the FDA for the treatment of SMA in adults and children two months and older. Based on our knowledge of the mechanism of splicing and how small molecules interact with the cellular splicing machinery, we have identified additional small molecule drug candidates that modify splicing of pre-mRNA, promote inclusion of specific exons into mRNA, including pseudoexons, or force skipping of undesired exons from the mature mRNA. We believe that this technology is potentially widely applicable to a large number of target genes across many therapeutic areas.

Nonsense suppression

The protein coding region of mRNA contains the information for the amino acid sequence of the protein product, Additionally, certain sequences in the mRNA encode signals to start protein production and others to stop protein production. Mutations in DNA can result in stop signals within the mRNA that cause protein production to be stopped prematurely. These are termed premature stop codons.

We use our nonsense suppression technology to identify molecules that promote or enhance readthrough of premature stop codons in the mRNA. The presence of a premature stop codon results in translation termination before a full-length protein can be produced. Our nonsense suppression technologies identify small molecules that increase readthrough at the premature stop codon by facilitating the incorporation of a defined set of amino acids at the site of the premature stop codon resulting in the production of a full-length protein. We anticipate that this approach will be applicable to a wide variety of therapeutic areas.

In some instances, the nonsense, or premature stop, codon can cause the degradation of the mRNA through a process called nonsense-mediated decay. In addition to identifying molecules that increase readthrough, we are identifying molecules that can enhance the nonsense suppression effect of readthrough agents, such as Translarna, by preventing the decay of nonsense mutation containing mRNAs, a process known as nonsense mediated decay. We have developed a high throughput screen to identify molecules that increase the level of and stabilize premature stop codon-containing mRNAs. We can evaluate the effect of these molecules alone and in combination with Translarna in cell-based models of disease, identify lead compounds and initiate a chemical optimization program. We are currently in the process of evaluating compounds as single agents and in combination with readthrough compounds in preparation for an optimization program.

Gene therapy

Gene therapy is a technique that uses genes to treat or prevent disease through several approaches including 1) replacing a mutated gene that causes disease with a healthy copy of the gene, 2) inactivating, or "knocking out," a mutated gene that is functioning improperly or 3) introducing a new gene into the body to help fight a disease. Utilizing our CNS delivery strategy and technologies, we are focused on developing gene therapy product candidates that are engineered and optimized to provide durable treatments, and potentially functional cures, for CNS diseases for which there are currently no approved treatments. By directly administering low doses our therapies using non-pathogenic AAV to deliver therapeutic genes to the target non-dividing neuronal cells in the CNS, which we term targeted micro-dosing, we believe we maximize the probability of achieving a therapeutic benefit and mitigate systemic antibody, cellular immunity and complement-based reactions, minimize the stimulation of new immune responses, and reduce off-target effects.

We believe that our gene therapy platform will enable us to treat patients across a range of CNS disease indications. Our detailed knowledge and expertise in rare CNS diseases has enabled us to develop a gene therapy platform which we believe has important competitive advantages, is highly differentiated and provides practical approaches for delivery of gene therapies to the CNS in a range of disease indications. Our platform utilizes advanced, commercially-available delivery devices, instrumentation and software to optimize targeting to the region of the CNS known to be involved in the cause of the disease. Targeted micro-dosing ensures direct delivery to the CNS, thereby avoiding systemic administration, mitigating systemic immune and complement responses, minimizing the generation of newly mounted immunity to the gene therapy, and bypassing uptake and excretion of the gene therapy vector by organs such as the liver and kidney which further enhances safety. Our targeted micro-dosing strategy has the added benefit of requiring significantly lower gene therapy doses than systemic dosing would require. Our low dose requirements provide for efficient manufacturing approaches that reduce supply risks, enhance product quality, and lower production costs. Our direct delivery processes have also resulted in a deep understanding of routes of administration that result in effective gene therapy delivery to target cells.

Energy production and oxidative stress

Energy production in cells is critical to their survival. On the other hand, processes that induce oxidative stress in cells can negatively impact them. Energy production takes place in a part of the cell called mitochondria. The mitochondria use the transport of electrons via chemical reactions called redox reactions in their cell membranes to produce adenosine triphosphate, or ATP, which is the central energy molecule inside cells. This process of moving electrons to produce ATP is termed electron transfer or transport. The redox reactions, however, can also cause oxidative stress. We use our expertise in energy production via electron transfer chemical reactions and in oxidative stress to develop potentially first-in-class therapeutics for unmet medical needs. One area of our focus is on inherited mitochondrial diseases. Mitochondrial diseases often derive from defects in energy production and oxidative stress pathway. These diseases commonly result in severe neurological impairment and death at an early age. Through our screening processes, we have identified multiple drug targets which we are assessing in nonclinical studies with the aim of identifying additional product candidates to take into clinical development. Similar strategies potentially can be used for broader sets of diseases. We believe such approaches to these types of intractable diseases have the potential to lead to novel therapies to address areas of high unmet medical need.

Our Collaborations, License Agreements and Funding Arrangements

We currently have ongoing collaborations with Roche and the SMA Foundation for SMA, a license agreement with National Taiwan University, or NTU, for Upstaza, a collaboration and license agreement with Akcea for Tegsedi and Waylivra and a license agreement with Shiratori Pharmaceutical Co., Ltd., or Shiratori, relating to the manufacturing processes and technology for sepiapterin. We also have received grant funding from Wellcome Trust pursuant to funding agreements under which we have continuing obligations.

Roche and the SMA Foundation

Overview. In November 2011, we entered into a license and collaboration agreement with Roche and the SMA Foundation to further develop and commercialize compounds identified under our SMA sponsored research program with the SMA Foundation and to research other small molecule compounds with potential for therapeutic use in patients with SMA. The research term of this agreement was terminated effective December 31, 2014. The ongoing collaboration is governed by a joint steering committee consisting of an equal number of representatives of us, the SMA Foundation and Roche. We, the SMA Foundation and Roche have agreed to endeavor to make decisions by consensus, but if the joint steering committee cannot reach agreement after following a specified decision resolution procedure, Roche's decision will control. However, Roche may not exercise its final decision-making authority with respect to certain specified matters, including any decision that would increase our or the SMA Foundation's obligations, reduce our or the SMA Foundation's rights, expand Roche's rights, or reduce Roche's obligations under the license and collaboration agreement.

Commercialization. We have granted Roche worldwide exclusive licenses, with the right to grant sublicenses, to our patent rights and know-how with respect to such compounds and products. Roche is responsible for pursuing worldwide

clinical development of compounds from the research program and has the exclusive right to develop and commercialize compounds from the collaboration.

Payments and Contingent Payments. Pursuant to the license and collaboration agreement, Roche paid us an upfront non-refundable payment of $30.0 million. During the research term, which was terminated effective December 31, 2014, Roche provided us with funding, based on an agreed- upon full-time equivalent rate, for an agreed-upon number of full- time equivalent employees that we contributed to the research program. We are eligible to receive up to an aggregate of $135.0 million in payments if specified development and regulatory milestones are achieved and up to an aggregate of $325.0 million in payments if specified sales milestones are achieved. We are also entitled to tiered royalties ranging from 8% to 16% on worldwide net product sales of products developed pursuant to the collaboration. Roche's obligation to pay us royalties will expire generally on a country-by- country basis at the latest of the expiration of the last-to-expire patent covering a product in the given country, the expiration of regulatory exclusivity for that product in such country or 10 years from the first commercial sale of that product in such country. However, the royalties payable to us may be decreased in certain circumstances. For example, the royalty rate in a particular country is reduced if the product is not protected by patents in that country and no longer entitled to regulatory exclusivity in that country. We remain responsible for making any payments to the SMA Foundation that may become due under our pre-existing sponsored research agreement with the SMA Foundation.

As of December 31, 2022, we had recognized a total of $210.0 million in milestone payments and $172.9 million royalties on net sales pursuant to the SMA License Agreement. As of December 31, 2022, there are no remaining development and regulatory event milestones that we can receive. The remaining potential sales milestones as of December 31, 2022 are $250.0 million upon achievement of certain sales events.

In July 2020, we entered into a Royalty Purchase Agreement with RPI 2019 Intermediate Finance Trust, or RPI, and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, or the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, we sold to RPI 42.933%, or the Assigned Royalty Payment, of our right to receive sales-based royalty payments, or the Royalty, on worldwide net sales of Evrysdi and any other product developed pursuant to the SMA License Agreement. In consideration for the sale of the Assigned Royalty Payments, RPI paid us $650.0 million in cash consideration. We have retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the SMA License Agreement. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

Termination. Unless terminated earlier, the license and collaboration agreement will expire on the date when no royalty or other payment obligations are or will become due under the agreement. Roche's termination rights under the license and collaboration agreement include the right to terminate the agreement at any time after November 22, 2013 on a product-by-product and country-by-country basis upon three months' notice before the launch of the applicable product or upon nine months' notice thereafter; and the right to terminate the agreement in specified circumstances following a change of control of us. The license and collaboration agreement provides that we or Roche may terminate the agreement in the event of an uncured breach by the other party of a material provision of the agreement, or in the event of the other party's bankruptcy or insolvency. Upon termination of the collaboration agreement by Roche for convenience or termination by us as a result of Roche's breach, bankruptcy, change of control or patent challenge, we have the right to assume the development and commercialization of product candidates arising from the license and collaboration agreement. In that event, we may become obligated to pay royalties to Roche on sales of any such product.

SMA Foundation

Overview. In June 2006, we entered into a sponsored research agreement with the SMA Foundation under which we and the SMA Foundation have collaborated in the research and preclinical development of small molecule therapeutics for SMA. As discussed above, we are also collaborating with the SMA Foundation and Roche to further develop these compounds. Pursuant to the sponsored research agreement, as amended, the SMA Foundation provided us with $13.3 million in funding. The SMA Foundation is not obligated to provide any further funding under this agreement.

Continuing financial obligations. We may become obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that we successfully develop and subsequently commercialize or, with respect to collaboration products we outlicense, including Evrysdi, a specified percentage of certain payments we receive from our licensee. As discussed above, we have outlicensed rights to Roche pursuant to a license and collaboration agreement. We are not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. Since inception, the SMA Foundation has earned $28.5 million, $24.5 million which was paid and $4.0 million which was accrued as of December 31, 2022. Our obligation to make such payments would end upon our payment to the SMA Foundation of an aggregate of $52.5 million, which we refer to as the repayment amount.

Reversion rights. In specified circumstances, including those involving our decision to discontinue development or commercialization of a collaboration product, our uncured failure to meet agreed timelines or those that might arise following our change of control, we may be obligated to grant the SMA Foundation exclusive or non-exclusive sublicensable rights under our intellectual property, in certain collaboration products, among other rights, to assume the development and commercialization of such collaboration products and to provide the SMA Foundation with other transitional assistance, which we refer to as a reversion. In some such cases, we may be entitled to receive licensing fee payments from the SMA Foundation and single-digit royalties on sales of the applicable collaboration product, which amounts we collectively refer to as reversion payments. In other cases, the SMA Foundation is not required to make any payments to us in connection with the licenses it receives from us.

Termination. Unless terminated earlier, the sponsored research agreement will continue until the earliest of the SMA Foundation's receipt of the repayment amount or, if there was a reversion, either our receipt of all reversion payments that the SMA Foundation may be obligated to make to us or, if the SMA Foundation is not obligated to make reversion payments, the expiration of the last-to-expire patent we licensed to the SMA Foundation in connection with such reversion. The sponsored research agreement provides that either party may terminate the agreement in the event of an uncured material breach by the other party or in the event of the other party's bankruptcy or insolvency.

National Taiwan University

Overview. Pursuant to the license and technology transfer agreement, originally entered into between Agilis Biotherapeutics, Inc., or Agilis, NTU and Professor Wuh-Liang (Paul) Hwu, in December 2015, or the NTU Licensing Agreement., NTU granted to us an exclusive, perpetual license, with the right to grant sublicenses through all tiers, to research and use the intellectual property, data, chemistry, manufacturing and controls, or CMC, records, documents, confidential information, materials and know-how pertaining to the Research, including Upstaza for the treatment of AADC deficiency, under the NTU Collaboration Agreement (as defined below), or the Technology, and to develop, make, manufacture, use, sell, import and market the Technology and any other products made, invented, developed or incorporated by or with the Technology, or the Licensed Products. Subject to any regulatory delays or issues, we are obligated to research, use and develop the Technology to manufacture Licensed Products by December 23, 2025. Additionally, we are obligated to obtain marketing approval of Upstaza for the treatment of AADC deficiency, either by the FDA or by the EMA, by December 31, 2024. In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA, satisfying that obligation.

Funding Obligations. NTU received a lump sum of $100,000 upon execution of the NTU Licensing Agreement, as well as $2.0 million milestones payments based on the achievement of certain clinical and regulatory milestones, including $1.2 million that became due and payable in July 2022 upon the European Commission's approval of Upstaza for the treatment of AADC deficiency. Additionally, NTU will be entitled to receive contingent payments from us based on (i) annual license maintenance fees, (ii) a low double-digit percentage royalty of annual net sales of Licensed Products, and (iii) a percentage of sublicense revenue, ranging from low-twenties to mid-twenties. The annual license maintenance fees are non-refundable, but creditable against annual net sales payments.

Intellectual Property. All intellectual property relating to the manufacture, production, assembly, use or sale of Technology and any Licensed Products derived thereof are owned by NTU.

Termination. The NTU Licensing Agreement expires on December 23, 2035. Upon expiration, we will have a fully paid-up, perpetual, royalty-free exclusive license to the Technology. We may terminate the NTU Licensing Agreement upon

60 days' written notice to NTU in the event of (a) the failure of a pivotal clinical study, or serious adverse event in a clinical study, with respect to Upstaza for the treatment of AADC deficiency, that prevents continuing such clinical study under reasonable circumstances or (b) the rejection of a BLA with the FDA or an MAA with the EMA, or equivalent biologics approval application in another territory with respect to Upstaza for the treatment of AADC. In such termination event, we must pay $100,000 to NTU within 30 days of termination and NTU would retain all rights to the Technology. We may terminate the NTU Licensing Agreement for material breach by another party following a 30-day cure period. NTU may terminate the NTU Licensing Agreement for our failure to pay any undisputed license fees or net sales or sublicensing royalty fees within the applicable deadline following a 30-day cure period.

We are also a party to collaborative research agreements with NTU, or the NTU Collaboration Agreements, that govern the collaboration between us and NTU with respect to the research and clinical trials for AADC deficiency gene therapy. NTU is responsible for performing the research and clinical trials and we are responsible for providing related funding. As of December 31, 2022, an aggregate amount of $3.2 million in funding payments has been paid to NTU pursuant to the NTU Collaboration Agreements.

Tegsedi and Waylivra

Overview. PTC Therapeutics International Limited, our subsidiary, entered into a Collaboration and License Agreement, or the Tegsedi-Waylivra Agreement, dated August 1, 2018 by and between us and Akcea, for the commercialization by us of Tegsedi, Waylivra and products containing those compounds, which we refer to collectively as the Products, in countries in Latin America and the Caribbean, or the PTC Territory. In addition, Akcea has granted to us a right of first negotiation, or ROFN, to commercialize AKCEA-TTR-Lrx, a follow-on product candidate to inotersen, on an exclusive basis in the PTC Territory. We are responsible for all meetings, communications and other interactions with regulatory authorities in the PTC Territory. The activities of the parties pursuant to the Tegsedi-Waylivra Agreement is overseen by a Joint Steering Committee, composed of an equal number of representatives appointed by each of us and Akcea.

Commercialization. Under the terms of the Tegsedi-Waylivra Agreement, Akcea has granted to us an exclusive right and license, with the right to grant certain sublicenses, under Akcea's product-specific intellectual property to develop, manufacture and commercialize the Products in the PTC Territory. In addition, Akcea has granted to us a non-exclusive right and license, with the right to grant certain sublicenses, under Akcea's core intellectual property and manufacturing intellectual property to develop, manufacture and commercialize the Products in the PTC Territory and to manufacture the Products worldwide in accordance with a supply agreement with Akcea. Akcea has in-licensed certain of the Akcea intellectual property from its parent company, Ionis. Each party has agreed not to, independently or with any third party, commercialize any competing oligonucleotide product in the PTC Territory for the same gene target as inotersen.

Payments and Contingent Payments. We paid to Akcea an upfront licensing fee of $18.0 million, consisting of an initial payment of $12.0 million paid in connection with entering into the Tegsedi-Waylivra Agreement in August 2018, and a second payment of $6.0 million that was paid after Waylivra received regulatory approval from the EMA in May 2019. In addition, Akcea was eligible to receive milestone payments, on a Product-by-Product basis, of $4.0 million upon receipt of regulatory approval for a Product from ANVISA, subject to a maximum aggregate amount of $8.0 million for all such Products. We paid Akcea $4.0 million upon our receipt of marketing authorization from ANVISA in October 2019 for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hATTR amyloidosis in Brazil with Tegsedi and an additional $4.0 million upon our receipt of marketing authorization from ANVISA in August 2021 for the treatment of FCS. Akcea is also entitled to receive royalty payments in the mid-twenty percent range of net sales on a country-by-country and Product-by-Product basis, commencing on the earlier to occur of (1) 12 months after the first commercial sale of such Product in Brazil or (2) the date when we, our affiliates or sublicensees have recognized revenue of $10.0 million or more in cumulative net sales for such Product in the PTC Territory. The royalty payments are subject to reduction in certain circumstances as set forth in the Tegsedi-Waylivra Agreement.

Right of first negotiation. Akcea has granted to us a ROFN to commercialize AKCEA-TTR-Lrx in the PTC Territory, subject to negotiation of the terms of a definitive agreement and certain other terms and conditions. Such a definitive agreement would provide for a royalty rate to be paid by us for AKCEA-TTR-Lrx equal to the royalty rate we have agreed to pay for Tegsedi under the Tegsedi-Waylivra Agreement, or in the mid-twenty percent range of net sales, and the term of such royalty payments would be the same as the term of the Tegsedi royalty payments. During a specified period in the

Agreement, neither Akcea nor Ionis may enter into an agreement or grant any license to AKCEA-TTR-Lrx that is inconsistent with PTC's ROFN.

Termination. The Tegsedi-Waylivra Agreement will continue until the expiration of the last to expire royalty term with respect to all Products in all countries in the PTC Territory. Either party may terminate the Tegsedi-Waylivra Agreement on written notice to the other party if such other party is in material breach of its obligations thereunder and has not cured such breach within 30 days after notice in the case of a payment breach or 60 days after notice in the case of any other breach.

Shiratori

Overview. In connection with our acquisition of Censa Pharmaceuticals, Inc., or Censa, in May 2020, we became a party to a license agreement dated as of February 8, 2015, as amended, between Shiratori and Censa, or the Shiratori License Agreement. Pursuant to the Shiratori License Agreement, Shiratori granted Censa the sole and exclusive worldwide right and license, with the right to sublicense, under certain licensed know-how, or the Licensed Know-How, and licensed patents, or the Licensed Patents, relating to manufacturing processes and technology for sepiapterin, to research, have researched, develop, have developed, use, import, export, market, have marketed, offer for sale, sell and have sold, and otherwise commercialize any final pharmaceutical product in finished form containing sepiapterin as an active pharmaceutical ingredient, including sepiapterin, collectively the Sepiapterin Products, covered by the Licensed Patents or using the Licensed Know-How in all countries and territories of the world outside of Japan, or the Sepiapterin Territory.

Payments and Contingent Payments. Under the Shiratori License Agreement, we are obligated to pay to Shiratori a low single digit percentage of annual net sales of the Sepiapterin Products in each country in the Sepiapterin Territory until the expiration of the last-to-expire Licensed Patent controlled by Shiratori covering the relevant country followed by an obligation to pay a reduced royalty rate for a specified period of time thereafter. We are also obligated to pay to Shiratori certain regulatory and development milestones.

Termination. Unless earlier terminated, the Shiratori License Agreement will continue in full force and effect on a country-by-country and product-by-product basis until the obligation to pay royalties with respect to the sale of such Sepiapterin Product in such country expires. The parties may agree to mutually terminate the Shiratori License Agreement. Shiratori may elect to terminate the Shiratori License Agreement upon sixty days' prior written notice to us in the event that we fail to (i) achieve regulatory approval for a Sepiapterin Product in either the United States or EU by February 8, 2026 or (ii) commercially launch a Sepiapterin Product in the United States or European Union by February 8, 2027. We may elect to terminate the Shiratori License Agreement upon sixty days' prior written notice to Shiratori.

Wellcome Trust

We have two separate funding agreements with Wellcome Trust for the research and development of small molecule compounds in connection with our oncology platform and antibacterial program. Pursuant to the agreement relating to the antibacterial program, Wellcome Trust awarded us a $5.0 million grant of which we received $4.8 million between 2011 and 2015. We are no longer actively pursuing an antibacterial program and do not expect to receive additional funding under this agreement. The materials terms of these funding agreements are similar in substance, except as described below.

The other agreement, entered into in May 2010, relates to the research and development of certain small molecule compounds under our oncology platform, including unesbulin. Pursuant to this agreement, Wellcome Trust awarded us a $5.4 million grant, of which approximately $0.9 million was paid in connection with execution of the agreement and the balance of which was paid to us in 2010 and 2012 based on our achievement of specified milestones.

Development and commercialization. We own all intellectual property that arises from the conduct of the research programs under these funding agreements, which we refer to as program intellectual property, and are responsible for developing and commercializing the program intellectual property, including unesbulin (for our oncology platform), and other compounds. However, we will require Wellcome Trust's written consent prior to any such development or commercialization. If Wellcome Trust withholds such consent and we and Wellcome Trust are not able to resolve

Wellcome Trust's concerns, the parties have agreed to follow a specified dispute resolution procedure that gives neither party final decision-making authority.

Reversion rights. Under both funding agreements, if we fail to take reasonable steps to develop or commercialize program intellectual property during specified timeframes, we may be obligated to grant exclusive rights to Wellcome Trust or its nominee under the program intellectual property, along with non-exclusive rights under our background intellectual property, so that Wellcome Trust or its nominee can assume such development and commercialization. If we grant such a license, we would be entitled to a share of any consideration received by Wellcome Trust in connection with any subsequent development or commercialization of program intellectual property on a for-profit basis, which share would be proportionate to our contribution to the development and commercialization.

Continuing financial obligations-oncology platform. To the extent that we develop and commercialize certain program intellectual property on a for-profit basis ourselves or in collaboration with a partner (provided we retain overall control of worldwide commercialization), we may become obligated to pay to Wellcome Trust development and regulatory milestone payments and single-digit royalties on sales of any research program product under our oncology platform. We made the first development milestone payment of $0.8 million to Wellcome Trust under this agreement during the second quarter of 2016. During the year ended December 31, 2022, we incurred $2.5 million of development milestones in connection with the enrollment of patients in the registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS, which is recorded in other long-term liabilities on the balance sheet. Additional milestone payments of up to an aggregate of $14.5 million may become payable by us to Wellcome Trust under this agreement.

Additional continuing financial obligations. Our obligation to pay the royalties described above would continue on a country-by-country basis until the longer of the expiration of the last patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country. To the extent that we develop and commercialize program intellectual property on a for-profit basis through outlicensing, we will be obligated to pay to Wellcome Trust a specified share of any consideration we receive from our licensee, provided that Wellcome Trust would be entitled to receive a minimum amount equal to its original contribution. We would incur no payment obligations to Wellcome Trust to the extent that we elect to develop and commercialize program intellectual property on a non-profit basis.

Termination. Unless terminated earlier, each funding agreement will continue until we have received the full amount of the grant, the research program has ended, the last-to-expire of the patents in the program intellectual property has expired, any agreement entered into for the exploitation of the program intellectual property or our background intellectual property has expired, and there are no remaining payment obligations relating to the exploitation of the program intellectual property or our background intellectual property. Each funding agreement provides that either party may terminate the agreement in the event of an uncured material breach by the other party or in the event of the other party's bankruptcy or insolvency and that Wellcome Trust may terminate the agreement under specified circumstances, including, among others, in specified circumstances following a change in control of us or if Wellcome Trust believes that an uncorrected serious failure exists in the progress, management or conduct of the research program or that an act or omission by us is incompatible with or has an adverse effect on Wellcome Trust's charitable objectives or reputation.

If Wellcome Trust terminates either or both funding agreements in specified circumstances, including as a result of our material breach, bankruptcy or insolvency, or following our change of control, we may be obligated to assign to Wellcome Trust ownership of the applicable program intellectual property, grant to Wellcome Trust royalty-free non-exclusive rights under the applicable background intellectual property for the continuation of the research program (if applicable) and the development and commercialization of the applicable program intellectual property, and provide Wellcome Trust with other specified transitional assistance.

Certain specified rights and obligations of the parties will generally survive termination of the funding agreements, including Wellcome Trust's right to receive payments from us with respect to development and commercialization of program intellectual property on a for-profit basis.

If a funding agreement terminates prior to the end of a research program, we are obligated to return all funding we received from Wellcome Trust that is unspent at the date of termination (after deduction of costs and non-cancellable commitments incurred prior to such date).

Our Ongoing Acquisition-Related Obligations

From time to time, we have engaged in strategic transactions to expand and diversify our product pipeline, including through the acquisition of assets or businesses. In connection with these acquisitions, we have entered into agreements through which we have ongoing obligations, including obligations to make contingent payments upon the achievement of certain development, regulatory and net sales milestones or upon a percentage of net sales of certain products.

Complete Pharma Holdings, LLC

On April 20, 2017, we completed our acquisition of all rights to Emflaza, or the Emflaza Transaction. The Emflaza Transaction was completed pursuant to an asset purchase agreement, dated March 15, 2017, as amended on April 20, 2017, or the Emflaza Asset Purchase Agreement, by and between us and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon. The assets acquired by us in the Emflaza Transaction include intellectual property rights related to Emflaza, inventories of Emflaza, and certain contractual rights related to Emflaza. We assumed certain liabilities and obligations in the Emflaza Transaction arising out of, or relating to, the assets acquired in the Emflaza Transaction.

In addition to the upfront consideration paid to Marathon upon the closing of the Emflaza transaction, Marathon is entitled to receive contingent payments from us based on annual net sales of Emflaza, up to a specified aggregate maximum amount over the expected commercial life of the asset, subject to the terms and conditions of the Emflaza Asset Purchase Agreement. In 2022, we paid Marathon a single $50.0 million sales-based milestone in accordance with the Emflaza Asset Purchase Agreement.

Agilis Biotherapeutics, Inc.

On August 23, 2018, we completed our acquisition of Agilis pursuant to an Agreement and Plan of Merger, dated as of July 19, 2018, or the Agilis Merger Agreement, by and among us, Agility Merger Sub, Inc., a Delaware corporation and our wholly owned, indirect subsidiary, Agilis and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, or the Merger.

In addition to the upfront consideration paid to Agilis equityholders upon the closing of the Merger, Agilis equityholders may become entitled to receive contingent payments from us based on the achievement of certain development, regulatory and net sales milestones, as well as based upon a percentage of net sales of certain products.

On April 29, 2020, we, certain of the former equity holders of Agilis, or the Participating Rightholders, and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC, entered into a Rights Exchange Agreement, or the Rights Exchange Agreement. Pursuant to the Right Exchange Agreement, we issued 2,821,176 shares of our common stock and paid $36.9 million, in the aggregate, to the Participating Rightholders in exchange for the cancellation and forfeiture by the Participating Rightholders of their rights to receive certain milestone-based contingent payments under the Agilis Merger Agreement.

As of December 31, 2022, we have paid former equity holders of Agilis a total of $52.4 million in connection with the achievement of certain milestone-based contingent payments under the Agilis Merger Agreement. Our outstanding obligations under the Agilis Merger Agreement include obligations to pay up to an aggregate maximum amount of $20.0 million upon the achievement of certain development milestones, up to an aggregate maximum amount of $311.0 million upon the achievement of certain regulatory milestones, up to a maximum aggregate amount of $150.0 million upon the achievement of certain net sales milestones and a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2% to 6%, pursuant to the terms of the Agilis Merger Agreement. In October 2022, we paid the former Agilis equityholders $50.0 million in regulatory milestone payments as a result of the European Commission’s marketing approval of Upstaza for the treatment of AADC deficiency in July 2022.

We expect to pay the former Agilis equityholders an additional $20.0 million upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency, which we expect to occur in the first half of 2023.

BioElectron Technology Corporation

On October 25, 2019, we completed the acquisition of substantially all of the assets of BioElectron Technology Corporation, or BioElectron, pursuant to an Asset Purchase Agreement by and between the Company and BioElectron, dated October 1, 2019, or the BioElectron Asset Purchase Agreement.

In addition to the upfront consideration paid to BioElectron upon the closing of the asset acquisition, subject to the terms and conditions of the BioElectron Asset Purchase Agreement, BioElectron may become entitled to receive contingent milestone payments of up to $200.0 million (in cash or in shares of our common stock, as determined by us) from us based on the achievement of certain regulatory and net sales milestones. Subject to the terms and conditions of the BioElectron Asset Purchase Agreement, BioElectron may also become entitled to receive contingent payments based on a percentage of net sales of certain products.

Censa Pharmaceuticals, Inc.

On May 29, 2020, we acquired Censa pursuant to an Agreement and Plan of Merger, dated as of May 5, 2020, or the Censa Merger Agreement, by and among us, Hydro Merger Sub, Inc., our wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC, or the Censa Merger.

In addition to the upfront consideration paid to the Censa securityholders upon the closing of the Censa Merger, pursuant to the Censa Merger Agreement, Censa securityholders will be entitled to receive contingent payments from us based on (i) the achievement of certain development and regulatory milestones up to an aggregate maximum amount of $217.5 million for sepiapterin's two most advanced programs and receipt of a priority review voucher from the FDA as set forth in the Censa Merger Agreement, (ii) $109 million in development and regulatory milestones for each additional indication of sepiapterin, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $160.0 million, (iv) a percentage of annual net sales during specified terms, ranging from single to low double digits of the applicable net sales threshold amount, and (v) any sublicense fees paid to us in consideration of any sublicense of Censa's intellectual property to commercialize sepiapterin, on a country-by-country basis, which contingent payment will equal to a mid-double digit percentage of any such sublicense fees. In February 2023, we completed enrollment of our Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event, we are obligated to pay a $30.0 million development milestone to the former Censa securityholders, which we have the option to pay in cash or shares of our common stock. We also expect to make additional payments to the former Censa securityholders of $50.0 million in the aggregate upon the potential achievement in 2023 of certain development and regulatory milestones relating to sepiapterin.

Intellectual Property

Patents and trade secrets

Our success depends in part on our ability to obtain and maintain proprietary protection for our product candidates, technology and know-how, to operate without infringing the proprietary rights of others and to prevent others from infringing our proprietary rights. Our policy is to seek to protect our proprietary position by, among other methods, filing U.S. and certain ex-U.S. patent applications related to our proprietary technology, inventions and improvements that we believe are important to the development of our business, where patent protection is available. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position.

As of January 31, 2023, our patent portfolio included a total of 143 active U.S. patents and 63 pending U.S. non-provisional patent applications, including continuations and divisional applications, that are owned, co-owned, or exclusively in-licensed. Our patent portfolio also includes numerous International and ex-U.S. patents and patent applications. The patent

portfolio includes patents and patent applications with claims including composition of matter, pharmaceutical formulation and methods of use of our commercial products including ataluren, the active ingredient in the formulated product Translarna, and risdiplam, the active ingredient in the formulated product Evrysdi.

The patent rights relating to ataluren owned by us include 42 issued U.S. patents relating to composition of matter, methods of use, formulations, dosing regimens and methods of manufacture and multiple pending U.S. patent applications relating to methods of use, formulation, and dosing regimens. We do not license any material patent rights relating to ataluren to unaffiliated parties. The issued U.S. patents relating to composition of matter are currently scheduled to expire in 2024 and all U.S. patents that issue from U.S. patent applications arising from the composition of matter would also be expected to expire in 2024. Issued U.S. patents relating to therapeutic methods of use are currently scheduled to expire in 2026 and 2027, including patent term adjustment. Our patent rights relating to ataluren include granted patents or pending counterpart patent applications in a number of other jurisdictions, including Canada, certain South American countries, Europe, certain Middle Eastern countries, certain African countries, certain Asian countries and certain Eurasian countries. We own 15 European patents relating to composition of matter, uses, dosing regimens and methods of manufacture of ataluren, as well as multiple pending European patent applications relating to composition of matter, uses and formulations. Granted European patents will expire in 2024 for those patents drawn to composition of matter, in 2026 and 2027 for those patents drawn to dosing regimen, and in 2027 for those patents drawn to the manufacturing process. Except as indicated above, the anticipated expiration dates referred to above are without regard to potential patent term extension, patent term adjustment or other marketing exclusivities that may be available to us.

The patent rights relating to risdiplam co-owned by us and Roche include 4 issued U.S. patents relating to composition of matter, methods of use, and methods of manufacture and pending U.S. patent applications. We do not license any material patent rights relating to risdiplam to unaffiliated parties. The issued U.S. patents relating to composition of matter are currently scheduled to expire in 2033 and 2035. Our patent rights include granted patents or pending counterpart patent applications in a number of other jurisdictions, including Canada, certain South American countries, Europe, certain Middle Eastern countries, certain African countries, certain Asian countries and certain Eurasian countries. We co-own 2 European patents relating to composition of matter, and uses of risdiplam. The expiration dates of the granted European patents relating to composition of matter are currently scheduled to expire in 2033 and 2035. Except as indicated above, these anticipated expiration dates are without regard to potential patent term extension, patent term adjustment or other marketing exclusivities that may be available to us.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent's term may, in certain cases, be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent. The term of a U.S. patent that covers a drug, biological product or medical device approved pursuant to a pre-market approval, or PMA, may also be eligible for patent term extension when FDA approval is granted, provided statutory and regulatory requirements are met.

Analogous patent term extension provisions are available in Europe and certain other ex-U.S. jurisdictions to extend the term of a patent that covers an approved drug. One means of patent term extension in Europe after EMA approval is based on obtaining a Supplementary Protection Certificate, or SPC. We have applied for SPCs for ataluren in all applicable European countries in which we have a European patent and have obtained SPCs or expect to obtain SPCs in all applicable European countries. The maximum patent term extension provided by an SPC is a total of 5 years from the date of patent term expiration. For example, in jurisdictions where an SPC with maximum patent term extension has been granted, the ataluren composition of matter patent would be scheduled to expire in 2029. In the future, if and when our product candidates receive approval by the FDA or other non-European ex-U.S. regulatory authorities, we expect to apply for patent term extensions on issued patents covering those products, depending upon the length of the clinical trials for each drug and other factors.

We have no patents covering Emflaza or the approved use of Emflaza. We rely on non-patent market exclusivity periods under the Orphan Drug Act to commercialize Emflaza in the United States. See "Item 1. Business-Government Regulation" for further information regarding the exclusivity periods that we expect to rely on.

We rely on orphan drug exclusivity in the EEA for Upstaza for the treatment of AADC deficiency. If Upstaza is approved in the United States, we expect to rely on the non-patent market exclusivity periods under the Orphan Drug Act and the BPCIA to commercialize Upstaza in the United States. If approved elsewhere, we expect to rely on orphan drug exclusivity in other countries or regions where such exclusivity is available. See "Item 1. Business-Government Regulation" for further information regarding the exclusivity periods that we expect to rely on.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, using confidentiality agreements with our employees, consultants, scientific advisors, contractors and collaborators. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, such agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our employees, former employees, consultants, scientific advisors, contractors or collaborators use intellectual property owned by us or licensed to us by others in their work for us, trade secret disputes may arise. If such disputes arise in the U.S., we may protect our trade secrets and pursue remedies available under federal statute using either the Economic Espionage Act of 1996 and/or the Defend Trade Secrets Act of 2016 and, if necessary, under state law using either the Uniform Trade Secrets Act or other State law available in the applicable venue. If such disputes arise ex-US, we may protect our trade secrets and pursue remedies available under local or international law.

License agreements

We are a party to a number of license agreements under which we license patents, patent applications and other intellectual property from third parties. We enter into these agreements to augment our proprietary intellectual property portfolio. The licensed intellectual property covers some of the compounds that we are researching and developing, some post-transcriptional control targets and some of the scientific processes that we use. These licenses impose various diligence and financial payment obligations on us. We expect to continue to enter into these types of license agreements in the future.

We exclusively in-licensed know-how and materials related to the production and use of Upstaza. For a further discussion of the material agreements relating to our in-licensing of Upstaza for the treatment of AADC deficiency, see "Item 1. Business-Our Collaborations, License Agreements and Funding Arrangements-National Taiwan University." We also exclusively in-license or jointly own patent applications with claims directed to composition of matter, formulation and methods of use of other gene therapy products candidates currently in development.

Manufacturing

Other than as described below with respect to certain of our gene therapy product candidates, we do not currently own or operate functional manufacturing or distribution facilities for the production of clinical or commercial quantities of our products or product candidates or compounds that we are testing in our preclinical programs. We currently rely, and expect to continue to rely, on third parties for the manufacture, packaging, labeling and distribution of clinical and commercial supplies of our products or product candidates that we may develop, other than small amounts of compounds that we may synthesize ourselves for preclinical testing.

The active pharmaceutical ingredients in our products and product candidates are provided by third-parties. We currently rely on a single source for the production of some of our raw materials and we obtain our supply of the drug substance for Translarna from two third-party manufacturers.

We engage two separate manufacturers to provide bulk drug product for Translarna. We have a relationship with three manufacturers that are capable of providing fill and finish services for our finished commercial and clinical Translarna product.

We currently obtain our supplies of Translarna and most of our other products and product candidates from our third-party manufacturers pursuant to agreements that include specific supply timelines and volume expectations. If a manufacturer should become unavailable to us for any reason, we would seek to obtain supply from another manufacturer engaged by

us for the applicable product or service. In the event that we were unable to procure the applicable supply from a validated manufacturer, we believe that there are a number of potential replacements for each of our outsourced services, however we likely would experience delays in our ability to supply Translarna to patients or in advancing our clinical trials while we identify and qualify replacement suppliers.

We obtain our supply of the drug substance for Emflaza through a third-party manufacturer that is currently the only third-party manufacturer qualified to provide Emflaza drug substance for use in the United States. All of our drug product manufacturing, processing and packaging needs for Emflaza tablet and suspension product are fulfilled pursuant to two different exclusive supply agreements assumed by us in connection with our acquisition of Emflaza. We expect to fulfill all of our requirements for Emflaza tablets as well as secondary packaging of pre-filled Emflaza oral suspension bottles pursuant to one of these agreements, which has an automatic renewal provision subject to the termination rights of each party. We expect to fulfill all of our requirements for Emflaza suspension product pursuant to the other agreement. Through the seventh year anniversary of FDA approval of Emflaza, we are obligated to pay to the manufacturer of the Emflaza suspension product royalty payments, on a quarterly basis, based on a percentage (ranging from low to middle-low double digits) of, or a fixed payment with respect to, our annual net sales of suspension product in the United States, subject to reduction in accordance with the terms of the agreement. The royalty payments for the suspension product are subject to a minimum aggregate annual payment ranging from €0.5 million to €1.5 million per year.

If our drug substance provider or either of our drug product manufacturers was to be unable to provide drug substance or manufacture Emflaza product in sufficient quantities to meet projected demand, future sales could be adversely affected, which in turn could have a detrimental impact on our ability to maintain our marketing authorization in the United States and on our ability to commercialize Emflaza, which in turn would have a material adverse effect on our business, financial results and results of operations. Further, as we presently have no patent rights to protect the approved use of Emflaza, we rely on market exclusivity periods available to us under the Orphan Drug Act to commercialize Emflaza for DMD in the United States. As the holder of orphan exclusivity, we are required to assure the availability of sufficient quantities of Emflaza to meet the needs of patients. Failure to do so could result in loss of the drug's orphan exclusivity in the United States, which would have a material adverse effect on our ability to generate revenue from sales of Emflaza.

Translarna and Emflaza are manufactured in reliable and reproducible synthetic processes. Our raw materials are not scarce and are readily available subject to supply chain disruptions. We currently rely on a single source for the production of some raw materials and switching to an alternative source could, in some instances, take time and could lead to delays in manufacturing. We maintain inventories for such materials such that any delays with raw materials will not affect or delay our manufacturing. No material shortages or delays of raw materials were encountered in 2022 and no manufacturing delays are currently expected in 2023. The chemistry is amenable to scale up and does not require unusual equipment in the manufacturing process. We expect to continue to develop drug candidates that can be produced cost-effectively at contract manufacturing facilities or internally, in the case of our gene therapy platform.

We do not currently have any agreements with third-party manufacturers for the long-term commercial supply of Translarna or any of our product candidates, although we may seek to establish such arrangements in the future. In the event that we are unable to procure supply from a validated manufacturer, we would seek to identify and qualify replacement suppliers, however this process would likely delay our ability to supply Translarna to patients or advance our clinical trials. We may be unable to conclude agreements for commercial or clinical supply of Translarna with third-party manufacturers, or we may be unable to do so on acceptable terms.

We currently have contracts with multiple pharmacy and hospital distributors in the EU that distribute Translarna for limited commercial and EAP programs. We have engaged with third party logistic providers, or 3PLs, which distribute Translarna for the majority of our commercial and EAP programs on our behalf.

We utilize third parties for the commercial distribution of Emflaza, including a 3PL to warehouse Emflaza as well as specialty pharmacies to sell and distribute Emflaza to patients. The specialty pharmacies provide us with third-party call center services to provide patient support and financial services, prescription intake and distribution, reimbursement adjudication, and ongoing compliance support.

Pursuant to the Tegsedi-Waylivra Agreement, we have entered into a master supply agreement with Akcea whereby Akcea or its affiliates will manufacture and supply, or cause to be manufactured and supplied, Tegsedi and Waylivra in quantities sufficient to support the commercialization of Tegsedi and Waylivra in the PTC Territory. This is currently the only manufacturing and supply agreement that we have entered into for the drug substance of Tegsedi and Waylivra. If the master supply agreement is terminated and we are unable to find an alternative third party contractor, we may encounter delays in manufacturing Tegsedi and Waylivra.

We have a commercial manufacturing services agreement with MassBiologics of the University of Massachusetts Medical School, or MassBio, to provide sufficient quantities of our Upstaza program materials to meet anticipated clinical trial and commercial scale demands. We also manufacture clinical material at our leased biologics manufacturing and laboratory space located in Hopewell Township, New Jersey, or the Hopewell Facility, for certain of our gene therapy product candidates. We still rely on third-party manufacturers to complete product testing for all of our gene therapy product candidates that we manufacture at the Hopewell Facility as well as to provide sufficient quantities of certain program materials that we have not yet transitioned to the Hopewell Facility. We have personnel with manufacturing and quality experience to oversee our contract manufacturers.

We further utilize the Hopewell Facility to produce plasmid DNA and AAV vectors for gene therapy applications for external customers.

Manufacturers and suppliers of product candidates are subject to the FDA's current Good Manufacturing Practices, or cGMP, requirements, and other rules and regulations prescribed by ex-U.S. regulatory authorities. We depend on our third-party suppliers and manufacturers for continued compliance with cGMP requirements and applicable ex-U.S. standards.

Commercial Matters

Sales and marketing team

Our product revenue has primarily been attributable to sales of Translarna for the treatment of nmDMD in territories outside of the United States and to sales of Emflaza for treatment of DMD in the United States. We have employees across the globe, with the largest concentrations being in the United States, Latin America and Europe.

In addition, in select territories, we have engaged full time consultants, marketing partners and distribution partners to assist us with our international commercialization efforts for our products. We continue to evaluate new territories to determine in which geographies we might, if approved, choose to commercialize our products ourselves and in which geographies we might choose to collaborate with third parties. We expect that our internal team and partnership network will continue to grow, as needed, to maximize access to patients.

Customers

During 2022, our product revenue was primarily attributable to Translarna for the treatment of nmDMD and to Emflaza for treatment of DMD. Translarna for the treatment of nmDMD was available on a commercial basis or via reimbursed EAP programs in multiple territories outside of the United States. In some territories, orders for Translarna are placed directly with us and in other territories we have engaged with third-party distributors. As a result, orders for Translarna are generally received from hospital and retail pharmacies and, in some cases, one of our third-party partner distributors. Our third-party distributors act as intermediaries between us and end-users and do not typically stock significant quantities of Translarna. The ultimate payor for Translarna is typically a government authority or institution or a third-party health insurer. The payment terms are generally 30 to 90 days after receipt of products.

Emflaza for treatment of DMD is available on a commercial basis throughout the United States. We utilize six specialty pharmacies to sell and distribute Emflaza to patients. The specialty pharmacies receive prescription orders for Emflaza directly from physicians and ship Emflaza directly to the end-user upon fulfillment of the order. As such, there is very little inventory of Emflaza stocked. The ultimate payor for Emflaza is typically a state health insurance program or a third-party health insurer. The payment terms are generally 30 to 90 days after receipt of products.

During 2022, two of our distributors each accounted for over 10% of our net product sales. Financial information about our net product revenues and other revenues generated in the principal geographic regions in which we operate and our long-lived assets is set forth in our financial statements and in Note 16, “Geographic Information” to our consolidated financial statements included in this Annual Report on Form 10-K.

Translarna and Emflaza can generally only be returned if agreed upon in writing by us and the product is not opened nor in receipt by the final user, except in the case of quality issues associated with the product. Product is generally shipped when a specific patient is approved by the applicable government or insurer and an individual prescription has been written. The right of return is eliminated as a matter of course when the product is dispensed to patients. Other than in connection with our transition to a new third party distributor, we have never had a request for a return of a material amount of product for either Translarna or Emflaza.

In some countries, including those in Latin America, orders for named patient sales may be for multiple months of therapy, which can lead to an unevenness in orders which could result in significant fluctuations in quarterly net product sales. For example, the Brazilian Ministry of Health is continuing to experience significant administrative delays processing centralized group purchase orders. Almost all of our Brazilian product revenue for Translarna is attributable to such purchase orders. These centralized group purchase order delays have caused, and may continue to cause, fluctuations in our ability to generate revenue in Brazil. Similarly, Translarna orders placed through a distributor for the Ministry of Health of the Russian Federation are also intended to cover multiple months of therapy. Any fluctuations in quarterly net product sales in Russia resulting from these centralized group purchase orders may also be exacerbated by any delays.

Market Access Considerations

Translarna for the treatment of nmDMD is currently available on a commercial basis in multiple countries outside of the United States. We consider our products to be commercially available when we are permitted to market treatment to patients.

Translarna for the treatment of nmDMD is also currently available through EAP programs in select countries where funded named patient or cohort programs exist, both within the EEA and in other territories. These programs generally reference the EMA’s determinations with respect to our marketing authorization in the EEA. As of today, Translarna is available under EAP or similar styled programs in various countries outside of the United States. Generally, EAP programs allow for access to Translarna pursuant to a named patient program, under which a physician requests access to Translarna on behalf of the specific, or “named” patient or pursuant to a cohort program, which allows for a broader temporary authorization for use for nmDMD meeting the inclusion criteria. Our EAP programs are named patient or similar styled programs in all territories other than France, which is a cohort program.

Our ability to make Translarna available via commercial or EAP programs is largely dependent upon our ability to maintain our marketing authorization in the EEA for Translarna for the treatment of nmDMD in ambulatory patients aged two years and older. The marketing authorization is subject to annual review and renewal by the European Commission following reassessment by the EMA as well as the specific obligation to conduct and submit the results of Study 041. In September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on Stage 1 and data from Stage 2 of Study 041. We expect an opinion from the CHMP in the first half of 2023. Additionally, the marketing authorizations of Translarna in Brazil and Russia are subject to renewal every five years. See “Item 1. Business-Global commercial footprint-Global DMD franchise” and “Risk Factors-Risks Related to Regulatory Approval of our Product and our Product Candidates” for further information regarding the marketing authorization in the EEA and related risks.

Our future revenues from our products and any other product candidates we may develop, depends largely on our ability to obtain and maintain reimbursement from governments and third-party insurers. Each country in the EEA has its own pricing and reimbursement regulations and many countries in the EEA have other regulations related to the marketing and sale of pharmaceutical products in the applicable country. The pricing and reimbursement process varies from country to country and can take a substantial amount of time from initiation to completion. As a result, our commercial launches of products in the EEA has been and is expected to continue to be on a country-by-country basis and we generally will not be able to commence commercial sales of our products pursuant to our marketing authorizations in the EEA in any

particular member state of the EEA until we conclude the applicable pricing and reimbursement negotiations and comply with any licensing, employment or related regulatory requirements in that country. The price that is approved by local governmental authorities pursuant to commercial pricing and reimbursement processes may be lower than the price for purchases of product in that country pursuant to a reimbursed early access program.

In some instances, reimbursement may be subject to challenge, reduction or denial by the government and other payers. For example, in France, EAP programs and commercial sales of a product can begin while pricing and reimbursement rates are under discussion with the applicable government health programs. In the event that the negotiated price of the product is lower than the amount reimbursed for sales made prior to the conclusion of price negotiations, we may become obligated to repay such excess amount to the applicable government health program. Such retroactive reimbursement would be made following the conclusion of price negotiations with the applicable government health authority.

For Emflaza, which is approved in the United States, we are engaged in pricing, coverage and reimbursement discussions with third-party payors, such as state and federal governments, including Medicare and Medicaid, managed care providers, private commercial insurance plans and pharmacy benefit management plans. Decisions regarding the extent of coverage and the amount of reimbursement to be provided for Emflaza are made on a plan-by-plan, and in some cases, on a patient-by-patient basis. Coverage and reimbursement decisions by third-party payors, including the processing and adjudication of prescriptions, may vary from weeks to several months. Certain third-party payors routinely impose additional requirements before approving reimbursement of a prescription, including prior authorization and the requirement to try another therapy first. The specialty pharmacies we utilize provide patient services programs to support product access and, when eligible, out-of-pocket assistance.

We have generated revenue from net sales of Upstaza for the treatment of AADC deficiency in the EEA since May 2022. Upstaza is approved for the treatment of AADC deficiency for patients 18 months and older within the EEA and the United Kingdom. Our future revenues from Upstaza depends largely on our ability to obtain and maintain reimbursement from governments and third-party insurers as described above.

Tegsedi for the treatment of hATTR amyloidosis and Waylivra for the treatment of FCS are currently available on a commercial basis in multiple countries outside of the United States and we have the right to commercialize these products in the PTC Territory. We have received marketing authorization from ANVISA for Tegsedi for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hATTR amyloidosis in Brazil and Waylivra for the treatment of FCS and FPL in Brazil. We make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil and Waylivra for the treatment of FCS in Brazil. The marketing authorizations of Tegsedi and Waylivra in Brazil are subject to renewal every five years. We have also made both Tegsedi and Waylivra available in certain countries within the PTC Territory through EAP Programs. Our ability to make Tegsedi and Waylivra for the treatment of FCS available via EAP programs within the PTC Territory is largely dependent upon the maintenance of the marketing authorizations in the EU, which in the case of Waylivra for the treatment of FCS, is subject to certain conditions.

We record revenue net of estimated third party discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. These allowances are adjusted to reflect known changes in factors and may impact such allowances in the quarter those changes are known.

For important information regarding market access and pricing and reimbursement considerations see “Item 1. Business-Government Regulation-Pharmaceutical Pricing and Reimbursement” and “Item 1A. Risk Factors-Risks Related to the Development and Commercialization of our Product and our Product Candidates” and “-Risks Related to Regulatory Approval of our Product and our Product Candidates”.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technologies, knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including commercial pharmaceutical and biotechnology enterprises, academic institutions, government agencies and private and public research institutions. Any product candidates that we successfully develop and commercialize will

compete with existing therapies and new therapies that may become available in the future. In addition, other gene therapy companies may in the future decide to utilize existing technologies to address unmet needs that could potentially compete with our product candidates.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer side effects, are more convenient or are less expensive than any products that we may develop. In addition, our ability to compete may be affected because in some cases insurers or other third-party payors seek to encourage the use of generic products. This may have the effect of making branded products less attractive, from a cost perspective, to buyers.

The key competitive factors affecting the success of our products and product candidates are likely to be its efficacy, safety, convenience, price and the availability of coverage and reimbursement from government and other third-party payors.

The competition for our products and product candidates includes the following:

- ***Translarna for nmDMD.*** There is currently no marketed therapy, other than Translarna in the EEA, which has received approval for the treatment of the underlying cause of nmDMD. Sarepta Therapeutics, or Sarepta, has received approval in the United States for two treatments (Exondys 51 (eteplirsen) and Vyondys 53 (golodirsen)) addressing the underlying cause of disease for different mutations in the DMD gene. Additionally, the FDA granted accelerated approval to Viltepso (viltolarsen) from NS Pharma for the treatment of DMD in patients with exon 53 skipping and Sarepta (Casimersen (SRP 4045) for the treatment of DMD in patients with exon 45 skipping. Viltepso (viltolarsen) from NS Pharma is also approved in Japan. Other biopharmaceutical companies are developing treatments addressing the underlying cause of disease for different mutations in the DMD gene, including, Daiichi Sankyo (DS-5141)), Nippon Shinyaku (Viltolarsen (NS-065/NCNP-01) and NS-089/NCNP-02)), and Astellas (AT-702). Other pharmaceutical companies are developing micro dystrophin gene therapies for patients with DMD regardless of genotype, including Pfizer (PF-06939926), Solid Biosciences (SGT-001) and Sarepta (SRP-9001), whose gene therapy has been submitted for accelerated approval to the FDA.
- ***Emflaza for DMD.*** The FDA has not approved a corticosteroid specifically for DMD in the United States other than Emflaza. However, prednisone/prednisolone, which is not approved for DMD in the United States, is generically available and has been prescribed off label for DMD patients. ReveraGen BioPharma and Santhera are developing a glucocorticoid antagonist (vamorolone) for DMD patients. An NDA for vamorolone has been submitted to and accepted by the FDA, and the Prescription Drug User Fee Act, or PDUFA, date for a decision by the FDA is October 26, 2023.
- ***Upstaza.*** Currently, no other treatment options are available for the underlying cause of AADC deficiency. Additionally, we are not aware of any late-stage development product candidates for AADC deficiency.
- ***Waylivra for FCS.*** Waylivra faces competition from drugs like Myalept (metreleptin). Myalept, produced by Novelion Therapeutics, Inc., is currently approved for use in generalized lipodystrophy patients. Additionally, Ionis is developing AKCEA-APOCIII-LRx for the treatment of FCS.
- ***Waylivra for FPL.*** Currently, no other treatment options are available for the underlying cause of FPL. Additionally, we are not aware of any late-stage development product candidates for FPL.
- ***Tegsedi.*** Tegsedi faces competition from drugs like Onpattro (patisiran) which was launched by Alnylam in the United States in 2018 and received approval in Brazil for the treatment of hATTR amyloidosis in 2020 as was well as AMVUTTRA (vutrisiran) which Alnylam received approval for in the United States and Brazil in 2022 for the treatment of the polyneuropathy of hATTR amyloidosis in adults. Vyndaqel (tafamids meglumine) and Vyndamax (tafamidis) are commercialized in the United States, EU and some countries in Latin America by Pfizer. Other companies are also pursuing product candidates for the treatment of ATTR Amyloidosis with

polyneuropathy including BridgeBio Pharma (AG 10), Proclara Biosciences (NPT 189), Prothena (PRX 004) and SOM Biotech (tolcapone).

- ***Evrysdi.*** Evrysdi, an orally bioavailable treatment, faces competition from treatments that are not orally bioavailable, including Spinraza (nusinersen), a drug developed by Ionis and marketed by Biogen, which has received FDA approval to treat SMA and Zolgensma (onasemnogene abeparvovec), a gene therapy drug developed by AveXis, Inc., (acquired by Novartis in 2018), which is approved in the United States and Japan for the treatment of SMA in patients under 2 years of age and in Europe for babies and young children who weigh up to 21 kilograms. Other companies are also pursuing product candidates for the treatment of SMA, including Kowa (sodium valproate), Catalyst Pharmaceuticals (amifampridine), Scholar Rock (SRK 015), Roche Pharmaceuticals (RO7204239) and Cytokinetics (reldesemtiv).
- ***PTC518 for Huntington's disease.*** There are currently no disease-modifying therapies approved to delay the onset or slow the progression of Huntington disease. However, uniQure (AMT-130), Roche and Ionis (tominersen) and Wave Life Sciences (WVE-003) are all developing product candidates for treatment of Huntington disease.
- ***Vatiquinone for Friedreich ataxia.*** While there are currently no disease modifying treatment options available for Friedreich ataxia, omaveloxolone, which is being developed by Reata Pharmaceuticals is a late stage product candidate being investigated for the treatment of Friedreich ataxia. An NDA for omaveloxolone has been submitted to and accepted by the FDA, and the PDUFA date for a decision by the FDA is February 28, 2023.
- ***Vatiquinone for mitochondrial disease associated seizures.*** There are no disease modifying drugs approved for the treatment of mitochondrial disease associated seizures and we are not aware of any late-stage development product candidates for mitochondrial disease associated seizures.
- ***Utreloxastat for ALS.*** Current standard of care for ALS is Rilutek (riluzole), currently available as a generic and other formulations and Radicava (edaravone) and Relyvrio (AMX-0035) within the United States. Amylyx Pharmaceuticals (AMX-0035) has also submitted an MAA to EMA with a decision expected in the first half of 2023. There are multiple other late stage product candidates being developed for the treatment of ALS including Ionis (Jacifusen), Clene Nanomedicine (CNM-Au8), MediciNova (Ibudilast), AB Science (AB-1010 mastinib mesylate), Prilenia Therapeutics (Pridopidine) and Biogen (tofersen). An NDA for tofersen has been submitted to and accepted by the FDA, and the PDUFA date for a decision by the FDA is April 25, 2023.
- ***Sepiapterin for PKU.*** If approved, sepiapterin could face competition from Kuvan (sapropterin dihydrochloride), including generic versions, and Palynziq (pegvaliase-pqpz), each of which is approved for the treatment of PKU. Furthermore, Homology (HMI-102) and BioMarin (BMN 307) each are developing gene therapy product candidates for the treatment of PKU.
- ***Unesbulin for LMS.*** First line treatment for LMS is surgery where appropriate and then chemotherapy options including doxorubicin, gemcitabine, dacarbazine and docetaxel for unresectable metastatic disease. For second line treatment, two drugs are approved for soft tissue sarcoma including LMS and these are Yondelis (trabectedin) and Votrient (pazopanib). Most LMS patients require multiple lines of therapy.
- ***Unesbulin for DIPG.*** There is no approved treatment for DIPG and very little improvement have been observed over the past 40 years. The current standard of care is radiation therapy which can shrink the tumor, though response is transient.

Government Regulation

Government authorities in the United States, at the federal, state and local level, and in other countries extensively regulate, among other things, the research, development, testing, quality control, approval, manufacturing, labeling, post-approval monitoring and reporting, recordkeeping, packaging, promotion, storage, advertising, distribution, marketing and sales and export and import of biopharmaceutical products such as those we are developing. In addition, sponsors of biopharmaceutical products and drug products participating in Medicaid and Medicare are required to comply with mandatory price reporting, discount, and rebate requirements. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and ex-U.S. statutes and regulations require the expenditure of substantial time and financial resources. See "Item 1A. Risk Factors-Risks Related to Regulatory Approval of our Product and our Product Candidates" for important information regarding some of the risks to our business arising as a result of government regulation.

U.S. government regulation

In the United States, the FDA regulates drugs and biologic products, including gene therapy products, under the Federal Food, Drug, and Cosmetic Act, or the FDCA, the Public Health Service Act, or the PHSA, and regulations and guidance implementing these laws. The FDCA, PHSA and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving drugs and biologic products. Failure to comply with the applicable FDA requirements at any time pre- or post-approval may result in a delay of approval or administrative or judicial sanctions.

Regulatory requirements governing our business are also evolving. For example, the FDA has issued a growing body of guidance documents on CMC, clinical investigations and other areas of gene therapy development, all of which are intended to facilitate the industry's development of gene therapy products. Moreover, in light of the COVID-19 pandemic, the FDA issued a number of guidance documents to assist companies navigating product development and manufacturing concerns raised by COVID-19.

The new drug and biologic approval process

In the United States, an NDA is the vehicle through which the FDA approves a new pharmaceutical drug product for sale and marketing in the United States. A BLA is the vehicle through which the FDA approves a new biologic product for sale and marketing in the United States.

To market a new drug or biologic product in the United States, a sponsor generally must undertake the following:

- completion of nonclinical laboratory tests, animal studies and formulation studies under the FDA's Good Laboratory Practice, or GLP, regulations and other applicable laws or regulations;
- submission to the FDA of an investigational new drug application, or IND, for clinical testing, which must become effective before clinical trials may begin at United States clinical trial sites;
- approval by an independent Institutional Review Board, or IRB, and in the case of certain gene therapy studies, an Institutional Biosafety Committee, or IBC, prior to initiation and subject to continuing review;
- completion of adequate and well-controlled clinical trials to establish safety and efficacy, in the case of a drug product candidate, or safety purity, and potency, in the case of a biologic product candidate for its intended use, performed in accordance with Good Clinical Practices, or GCP, and the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use, or ICH, E6 GCP guidelines. Certain gene therapy research must also be conducted in accordance with the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, or NIH Guidelines;
- development of manufacturing processes to ensure the product candidate's identity, strength, quality, purity, and potency;
- submission and FDA acceptance of an NDA or BLA, and satisfactory completion of an FDA Advisory Committee meeting, if applicable;
- satisfactory completion of an FDA inspection or remote regulatory assessment of the manufacturing facility or facilities at which the product is produced to assess compliance with cGMPs, which require that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity, as well as satisfactory completion of an FDA inspection or remote regulatory assessment of selected clinical sites and selected clinical investigators to determine GCP compliance; and
  FDA review and approval of the NDA or BLA to permit commercial marketing for particular indications for use.

Nonclinical Studies and IND Submission

Nonclinical tests include laboratory evaluations of product chemistry, pharmacology, stability, toxicity and product formulation, as well as animal or other nonclinical studies to assess potential safety and efficacy. In order to begin clinical testing, a sponsor must submit an IND to the FDA, which includes, among other things, the results of the nonclinical tests, manufacturing information, analytical data, proposed clinical protocols, and any available clinical data or literature on the product candidate. Some nonclinical testing may continue after the IND is submitted. The IND must become effective before human clinical trials may begin. An IND will automatically become effective 30 days after receipt by the FDA,

unless before that time the FDA raises concerns or questions about issues such as the conduct of the trials as outlined in the IND. In that case, the IND sponsor and the FDA must resolve any outstanding FDA concerns or questions before clinical trials can proceed. Clinical holds also may be imposed by the FDA at any time before or during trials due to safety concerns or non-compliance.

Clinical Trials

Clinical trials involve the administration of an investigational product to human subjects under the supervision of qualified investigators. Clinical trials are conducted in accordance with protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety, the effectiveness criteria to be evaluated, and a statistical analysis plan. A protocol for each clinical trial and subsequent protocol amendments must be filed with the FDA as part of the IND. Sponsors must also provide FDA with diversity action plans. In accordance with GCP requirements, all research subjects or their legally authorized representatives must provide their informed consent in writing prior to their participation in a clinical trial. Each clinical trial must be reviewed and approved by an IRB and is subject to ongoing IRB monitoring. The IRB must approve the protocol, protocol amendments, the informed consent form, and communications to study subjects before a study commences at the site. An IRB considers among other things, whether the risks to individuals participating in the trials are minimized and are reasonable in relation to anticipated benefits, and whether the planned human subject protections are adequate. The IRB must continue to oversee the clinical trial while it is being conducted. Special clinical trial ethical considerations also must be taken into account if a study involves children. In the case of certain gene therapy studies, an IBC at the local level may also review and maintain oversight over the particular study, in addition to the IRB. If the product candidate is being investigated for multiple intended indications, separate INDs may also be required. Progress reports detailing the results of the clinical trials must be submitted at least annually to FDA and the IRB and more frequently if serious adverse events or other significant safety information is found. Certain reports may also be required to be submitted to the IBC.

Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group regularly reviews accumulated data and advises the study sponsor regarding the continuing safety of the trial. This group may also review interim data to assess the continuing validity and scientific merit of the clinical trial. The data safety monitoring board receives special access to unblinded data during the clinical trial and may advise the sponsor to halt the clinical trial if it determined there is an unacceptable safety risk for subjects or on other grounds, such as no demonstration of efficacy.

Information about certain clinical trials must be submitted within specific timeframes to the NIH to be publicly posted on the Clinicaltrials.gov website. Sponsors or distributors of investigational products for the diagnosis, monitoring, or treatment of one or more serious disease or conditions must also have a publicly available policy on evaluating and responding to requests for expanded access. Investigators must also provide certain information to clinical trial sponsors to allow the sponsors to make certain financial disclosures to the FDA.

The manufacture of investigational drugs and biologics for the conduct of human clinical trials is subject to cGMP requirements. Investigational drugs and biologics and active ingredients and therapeutic substances imported into the United States are also subject to regulation by the FDA. Further, the export of investigational products outside the United States is subject to regulatory requirements of the receiving country as well as U.S. export requirements under the FDCA.

In general, for the purposes of NDA and BLA approval, human clinical trials typically are conducted in three sequential phases, but the phases may overlap, be divided, or be combined. Phase 1 clinical trials may be conducted in patients or healthy volunteers to evaluate the product’s safety, dosage tolerance, structure-activity relationships, mechanism of action, absorption, metabolism distribution, excretion, and pharmacokinetics and, if possible, seek to gain an early indication of its effectiveness. Phase 2 clinical trials usually involve controlled trials in a larger but still relatively small number of subjects from the relevant patient population to evaluate dosage tolerance and appropriate dosage; identify possible short-term adverse effects and safety risks; and provide a preliminary evaluation of the efficacy of the drug or biologic product for specific indications. Phase 3 clinical trials usually further evaluate clinical efficacy and test further for safety in an expanded patient population at geographically dispersed clinical trial sites, to generate enough data to provide statistically significant evidence of clinical efficacy and safety of the product candidate for approval. These trials are well-controlled and are intended to establish the overall risk- benefit profile of the product or product candidate and provide an adequate

basis for physician labeling. Phase 3 clinical trials are usually larger, more time consuming, more complex and more costly than Phase 1 and Phase 2 clinical trials. The FDA typically requires that an NDA or BLA include data from two adequate and well-controlled clinical trials, but, in certain circumstances, approval may be based upon a single adequate and well-controlled clinical trial plus confirmatory evidence or a single large multicenter trial without confirmatory evidence. In some cases, the FDA may condition approval of an NDA or BLA on the applicant's agreement to conduct additional clinical trials to further assess the product's safety and effectiveness after NDA or BLA approval. Such post-approval trials are typically referred to as Phase 4 studies. The results of Phase 4 studies can confirm or refute the effectiveness of a product candidate, and can provide important safety information.

Additional kinds of data may also help support a BLA or NDA, such as patient experience data and real world evidence. Real world evidence may also be used to assist in clinical trial design or support an NDA for already approved products. For genetically targeted populations and variant protein targeted products intended to address an unmet medical need in one or more patient subgroups with a serious or life threatening rare disease or condition, the FDA may allow a sponsor to rely upon data and information previously developed by the sponsor or for which the sponsor has a right of reference, that was submitted previously to support an approved application for a product that incorporates or utilizes the same or similar genetically targeted technology or a product that is the same or utilizes the same variant protein targeted drug as the product that is the subject of the application. More recently, a program was established whereby a platform technology that is incorporated within or utilized by an approved drug or biologic product may be designated as a platform technology, provided that certain conditions are met, in which case development and approval of subsequent products using such technology may be expedited.

Concurrent with clinical trials, companies usually complete additional nonclinical studies and must also develop additional information about the physical characteristics of the drug or biologic product candidate as well as finalize a process for manufacturing the product candidate in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other requirements, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biologic product. Additionally, appropriate packaging must be selected and tested and adequate stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Additional FDA Expedited Review and Approval Programs

The FDA has various programs that are intended to expedite or simplify the process for the development and FDA review of certain products that are intended for the treatment of serious or life-threatening diseases or conditions, and demonstrate the potential to address unmet medical needs or present a significant improvement over existing therapy. The purpose of these programs is to provide important new therapeutics to patients earlier than under standard FDA review procedures.

To be eligible for a Fast Track designation, the FDA must determine, based on the request of a sponsor, that a product candidate is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. If Fast Track designation is obtained, sponsors may be eligible for more frequent development meetings and correspondence with the FDA. In addition, the FDA may initiate review of sections of an application before the application is complete. This ''rolling review'' is available if the applicant provides and the FDA approves a schedule for the remaining information. Applicable user fees must also be paid before the FDA will commence its review. In some cases, a Fast Track product may be eligible for accelerated approval or priority review.

The FDA may give a priority review designation to product candidates that are intended to treat serious conditions and, if approved, would provide significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of the serious condition. A priority review means that the goal for the FDA is to review an application within six months, rather than the standard review of ten months under current Prescription Drug User Fee Act, or PDUFA, guidelines.

The FDA's accelerated approval process allows for potentially faster development and approval of certain drugs or biologic products intended to treat serious or life-threatening illnesses that provide meaningful therapeutic benefit to patients over existing treatments. Under the accelerated approval process, the adequate and well-controlled clinical trials conducted with the drug or biologic product establish that the drug or biologic product has an effect on a "surrogate" endpoint that is reasonably likely to predict clinical benefit or on the basis of an effect on a clinical endpoint other than

survival or irreversible morbidity, that is reasonably likely to predict an effect on irreversible morbidity or mortality, taking into account the severity, rarity, or prevalence of the condition and availability or lack of alternative treatments. Drugs or biologic products approved through the accelerated approval process are subject to certain post-approval requirements, including completion of Phase 4 clinical trials to demonstrate clinical benefit. By the date of approval of an accelerated approval product, FDA must specify the conditions for the required post approval studies, including enrollment targets, the study protocol, milestones, and target completion dates. FDA may also require that the confirmatory Phase 4 studies be commenced prior to FDA granting a product accelerated approval. Reports on the progress of the required Phase 4 confirmatory studies must be submitted to FDA every 180 days after approval. If the trials fail to verify the clinical benefit of the drug or biologics product, the FDA may withdraw approval of the application through a statutorily defined streamlined process. Failure to conduct the required Phase 4 confirmatory studies or to conduct such studies with due diligence, as well as failure to submit the required update reports can subject a sponsor to penalties. Promotional materials for a drug or biologic approved under the accelerated approval pathway are subject to FDA prior review.

Sponsors can also request designation of a product candidate as a ‘‘breakthrough therapy.’’ A breakthrough therapy is defined as a product that is intended, alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Products designated as breakthrough therapies are eligible for intensive guidance on an efficient development program beginning as early as Phase 1 trials, a commitment from the FDA to involve senior managers and experienced review staff in a proactive collaborative and cross-disciplinary review, rolling review, and the facilitation of cross-disciplinary review.

Another expedited pathway is the Regenerative Medicine Advanced Therapy, or RMAT, designation. Qualifying products must be a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or a combination of such products, and not a product solely regulated as a human cell and tissue product. The product must be intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition, and preliminary clinical evidence must indicate that the product has the potential to address an unmet need for such disease or condition. Advantages of the RMAT designation include all the benefits of the Fast Track and breakthrough therapy designation programs, including early interactions with the FDA. These early interactions may be used to discuss potential surrogate or intermediate endpoints to support accelerated approval.

Companion Diagnostics and Other Combination Products

A drug or biologic product may be regulated as combination product if it is intended for use in conjunction with a medical device, such as a drug delivery device or in vitro diagnostic device, as further discussed below. In such cases, the use of the two products together (i.e., the drug/biological product and the device) must be shown to be safe and effective for the proposed intended use and the labeling of the two products must reflect their combined use. In some cases, the device component may require a separate premarket submission; for example, when the device component is intended for use with multiple drug products. Sponsors of clinical studies using investigational devices are required to comply with FDA’s investigational device exemption regulations. Once approved or cleared, the sponsor of the device component submission (or the combination product submission, if both components are covered by one premarket submission) would need to comply with FDA’s post-market device requirements, including establishment registration, device listing, device labeling, unique device identifier, quality system regulation, medical device reporting, and reporting of corrections and removals requirements.

If the safety or effectiveness of a drug or biologic product candidate for its proposed indication is dependent on the measurement or detection of specified biomarkers, the FDA may require the contemporaneous approval or clearance of an in vitro companion diagnostic device that measures such biomarkers, and require the labeling of both the drug/biological product and the companion diagnostic to including instructions for use of the two products together. The FDA has explained in guidance that in vitro diagnostic companion diagnostic devices may be used for a number of purposes, including identifying appropriate subpopulations for treatment. The type of premarket submission required for a companion diagnostic device will depend on the FDA classification of the device. A premarket approval, or PMA, application is required for high risk devices classified as Class III; a 510(k) premarket notification is required for moderate risk devices classified as Class II; and a de novo request may be used for novel devices not previously classified by FDA

that are low or moderate risk. The guidance states that the FDA generally will not approve a drug or biologic that is dependent upon the use of a companion diagnostic device if no such device is contemporaneously FDA-approved or -cleared for the relevant indication. According to the guidance, however, the FDA may approve such a drug/biologic product without an approved/cleared companion diagnostic when the drug/ biologic "is intended to treat a serious or life-threatening condition for which no satisfactory alternative treatment exists" and the FDA determines that the benefits from the use of the drug/biologic "are so pronounced as to outweigh the risks from the lack of an" approved/cleared companion diagnostic. Under these circumstances, the FDA expects that a companion diagnostic would be subsequently approved/cleared, and that the drug/biologic labeling would be revised "to stipulate the use of the" companion diagnostic device. The FDA would also consider whether additional protections, such as risk evaluation and mitigation strategies, or REMS, or post-approval requirements, are necessary.

In a separate guidance, specific to DMD and related dystrophinopathies, the FDA has stated that a sponsor should contemporaneously develop a companion diagnostic device in situations where (1) the safety or efficacy of the drug or biologic product "may be related to the patient's specific dystrophin mutation or to another type of finding related to a biomarker," and (2) a suitable companion diagnostic device is not currently available. However, given "the serious and life-threatening nature of dystrophinopathies and the lack of satisfactory alternative treatments that currently exist," the guidance further states that the FDA may approve a drug/biologic "even if a companion diagnostic device is not yet approved or cleared, if the benefits are so pronounced as to outweigh the risks from the lack of an approved or cleared in vitro companion diagnostic device." During the review, the "FDA will determine the need for clearance or approval of the device." The FDA guidance documents represent the FDA's current thinking on a topic but do not establish legally enforceable responsibilities.

FDA Approval Process

Assuming successful completion of the required clinical testing, the results of the preclinical studies and of the clinical trials, together with other detailed information, including proposed labeling and information on the chemistry, manufacture and composition of the product, are submitted to the FDA in the form of an NDA or BLA requesting approval to market the product for one or more indications. In most cases, the NDA or BLA must be accompanied by a substantial user fee, though a waiver of such fees may be obtained under certain limited circumstances. Product candidates that are designated as orphan products are not subject to application user fees unless the application includes an indication other than the orphan indication. The user fees must be paid at the time of the first submission of the application, even if the application is being submitted on a rolling basis. The FDA has 60 days from its receipt of an NDA or BLA to determine whether the application will be accepted for filing based on the FDA's threshold determination that it is sufficiently complete to permit a substantive review.

If the FDA determines that the NDA or BLA is incomplete, the FDA may refuse to file the application. If the FDA refuses to file an NDA or BLA, the applicant may refile the application with information addressing the FDA identified deficiencies, which refiling would be subject to FDA review before it is accepted for filing. After the NDA or BLA submission is accepted for filing, the FDA reviews the NDA or BLA to determine, among other things, whether a product meets FDA's approval standard and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. Under the goals and policies agreed to by the FDA under the PDUFA, the FDA has set the review goal of completing its review of 90% of all standard applications for new molecular entities and original BLAs within ten months of the 60 day filing date. Under the FDA's priority review program, however, the FDA set a review goal of completing its review of 90% of all applications within 6 months of the 60 day filing date. The FDA does not always meet its PDUFA goal dates. The review process and the PDUFA goal date may be extended by additional three-month review periods whenever the FDA requests or the NDA or BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission at any time during the review cycle.

NDAs or BLAs or supplements to NDAs or BLAs for a new active ingredient, dosage form, dosage regimen, or route of administration, unless subject to the below requirement for molecularly targeted cancer products, must contain data to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of data or full or partial waivers.

This requirement does not generally apply to products for an indication for which orphan designation has been granted. However, compliance may be required if approval is sought for other indications for which the product has not received orphan designation.

Product candidates intended for the treatment of adult cancer which are directed at molecular targets that the FDA determines to be substantially relevant to the growth or progression of pediatric cancer, must submit, with the marketing application, reports from molecularly targeted pediatric cancer investigations designed to yield clinically meaningful pediatric study data, gathered using appropriate formulations for each applicable age group, to inform potential pediatric labeling. The FDA may, on its own initiative or at the request of the applicant, grant deferrals or waivers of some or all of this data, as above. Orphan products are not exempt from this requirement.

The FDA will typically inspect or conduct a remote regulatory assessment of one or more clinical sites to assure compliance with GCP before approving an NDA or BLA. The FDA also will inspect or conduct a remote regulatory assessment of the facility or the facilities at which the product is manufactured before the NDA or BLA is approved. The FDA will not approve the product unless cGMP compliance is satisfactory.

The FDA may refer applications for novel drug products or biologic products to an advisory committee for recommendation as to whether the application should be approved and under what conditions. Specifically, for a product candidate for which no active ingredient (including any ester or salt of active ingredients) has previously been approved by the FDA, the FDA must either refer that product candidate to an advisory committee or provide in an action letter, a summary of the reasons why the FDA did not refer the product candidate to an advisory committee. The FDA may also refer other product candidates to an advisory committee if FDA believes that the advisory committee's expertise would be beneficial. The FDA is not bound by the recommendation of an advisory committee, but it considers such recommendations carefully, particularly any negative recommendations or limitations, when making drug or biologic product approval decisions.

After evaluating the marketing application and all related information, including the advisory committee recommendation, if any, and inspection or remote regulatory assessment reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a Complete Response Letter, or CRL. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A CRL indicates that the review cycle of the application is complete and the application is not ready for approval and describes all of the specific deficiencies that the FDA identified. A CRL generally contains a statement of specific conditions that must be met in order to secure final approval of the marketing application, and may require additional clinical or preclinical testing in order for the FDA to reconsider the application. The deficiencies identified may be minor, for example, requiring labeling changes; or major, for example, requiring additional clinical trials. If a CRL is issued, the applicant may either: resubmit the marketing application, addressing all of the deficiencies identified in the letter; withdraw the application; or request an opportunity for a hearing. The FDA has the goal of reviewing 90% of application resubmissions in either two or six months of the resubmission date, depending on the kind of resubmission. Even with submission of additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

Additional regulation for gene therapy clinical trials

In addition to the regulations discussed above, there are a number of additional standards that apply to clinical trials involving the use of gene therapy. The FDA has issued, and continues to issue, various guidance documents regarding the development and commercialization of gene therapies, which outline additional factors that the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical and nonclinical assessment of gene therapies; the design and conduct of clinical trials, the CMC information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and long term patient and clinical study subject follow up and regulatory reporting. The FDA also issued guidance documents that address gene therapy considerations during the COVID-19 pandemic and a guidance specific to the development of gene therapy products for neurodegenerative diseases.

Post-approval requirements

After FDA approval of a product is obtained, we are required to comply with a number of post-approval requirements, including, among other things, establishment registration and product listing, record-keeping requirements, reporting certain adverse reactions and production problems to the FDA, providing updated safety and efficacy information, and complying with requirements concerning advertising and promotional labeling. As a condition of approval of an NDA or BLA, the FDA may require the applicant to conduct additional clinical trials or other post market testing and surveillance to further monitor and assess the product's safety and efficacy. There also are continuing annual program user fee requirements for approved products, though orphan products may receive exemptions if certain criteria are met.

The FDA also has the authority to require a specific REMS to ensure that a product's benefits outweigh its risks. A REMS may be required to include various elements, such as a medication guide or patient package insert, a communication plan to educate healthcare providers of the product's risks, limitations on who may prescribe or dispense the product, or other measures that the FDA deems necessary to assure the safe use of the drug. The FDA may also impose a REMS requirement on an approved product if the FDA determines, based on new safety information, that a REMS is necessary to ensure that the product's benefits outweigh its risks.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Although physicians may prescribe a drug or biologic for off-label uses, manufacturers may only promote the product for the approved indications and in accordance with the approved labeling. All statements regarding products must be consistent with the FDA approved label, must be truthful and non-misleading, and must be adequately substantiated with a fair balance between product benefit claims and risks, among other requirements. This means, for example, that a manufacturer cannot make claims about the use of its marketed products or their relative benefits compared to other treatments outside of their FDA approved indications and label and without adequate comparative studies, and it would not be able to discuss or provide information on off-label uses or safety benefits of such products in a promotional context. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with the laws and regulations governing advertising and promotion can have negative consequences, including FDA and other governmental authority enforcement actions.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which, in addition to state requirements, regulates the distribution of samples at the federal level. Reports must also be submitted to FDA on sample distribution. The Drug Supply Chain Security Act, or DSCSA, added sections in the FDCA that require manufacturers, repackagers, wholesale distributors, dispensers, and third-party logistics providers to take steps to identify and trace certain prescription drugs and biologics to protect against the threats of counterfeit, diverted, stolen, contaminated, or otherwise harmful products in the supply chain. The DSCSA regulates the distribution of prescription pharmaceutical drugs and biologics, requiring passage of documentation to track and trace each prescription product at the saleable unit level through the distribution system. This documentation must be transferred electronically and will be required to enable interoperable electronic product tracing at the package level by November 2023. Products subject to the DSCSA must only be transferred to appropriately licensed purchasers. The DSCSA also requires manufacturers and repackagers to affix or imprint a unique product identifier on product packages in both a human-readable and on a machine-readable data carrier. The DSCSA also establishes several requirements relating to the verification of product identifiers. Further, under this legislation, sponsors have product investigation, quarantine, disposition, and notification responsibilities related to counterfeit, diverted, stolen, and intentionally adulterated products that would result in serious adverse health consequences or death to humans, as well as products that are the subject of fraudulent transactions or which are otherwise unfit for distribution such that they would be reasonably likely to result in serious health consequences or death.

Also, quality control and manufacturing procedures must continue to conform to cGMP after approval, including quality control and quality assurance and maintenance of records and documentation. Changes to the manufacturing process are strictly regulated and often require prior FDA approval or notification before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and specifications, and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use.

Manufacturers and others involved in the manufacture and distribution of such products also must register their establishments with the FDA and certain state agencies. Both domestic and ex-U.S. manufacturing establishments must register and provide additional information regarding manufactured products to the FDA upon their initial participation in the manufacturing process for a commercial product, as well as periodically during the year. The information that must be submitted to FDA regarding manufactured products was expanded through the Coronavirus Aid, Relief, and Economic Security, or CARES, Act to include the volume of drugs produced during the prior year.

Establishments may be subject to periodic, unannounced inspections or remote regulatory assessments by government authorities to ensure compliance with cGMP requirements and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance. The FDA may take into account results of inspections performed by certain counterpart ex-U.S. regulatory agencies in assessing compliance cGMPs. The FDA has entered into international agreements with ex-U.S. agencies, including the EU, in order to facilitate this type of information sharing.

Sponsors are further subject to various requirements related to FDA drug shortage and manufacturing volume reporting, supply chain security, such as risk management plan requirements, and the promotion of supply chain redundancy. Legislation and executive actions have also been issued to encourage domestic manufacturing.

Additional controls for biologics

To help reduce the risk of the introduction of adventitious agents or of causing other adverse events with the use of biologic products, the PHSA emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The PHSA also provides authority to the FDA to immediately suspend licenses in situations where there exists a danger to public health, to prepare or procure products in the event of shortages and critical public health needs, and to authorize the creation and enforcement of regulations to prevent the introduction or spread of communicable diseases in the United States and between states.

After a BLA is approved, the product may also be subject to official lot release as a condition of approval. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing the results of all of the manufacturer's tests performed on the lot. The FDA may also perform certain confirmatory tests on lots of some products before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

Orphan drug designation.

We have received orphan drug designation from the FDA for Translarna for the treatment of nmDMD, Emflaza for the treatment of DMD, Upstaza for the treatment of AADC deficiency, Evrysdi for the treatment of SMA, vatiquinone for the treatment of Friedreich ataxia and treatment of seizures in patients with mitochondrial disease, utreloxastat for the treatment of ALS, PTC-FA for the treatment of Friedreich ataxia, PTC-AS for the treatment of Angelman syndrome, sepiapterin for the treatment of hyperphenylalaninemia, including hyperphenylalaninemia caused by PKU and unesbulin for the treatment of LMS and DIPG. The FDA may grant orphan drug designation to drugs and biologics intended to treat a "rare disease or condition," which is defined as a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a product for this type of disease or condition will be recovered from sales in the United States for that product. Additionally, sponsors must present a plausible hypothesis for clinical superiority to obtain orphan designation if there is a product already approved by the FDA that that is considered by the FDA to be the same as the already approved product and is intended for the same indication. This hypothesis must be demonstrated to obtain orphan exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Orphan drug designation can provide opportunities for grant funding towards clinical trial costs, tax advantages and FDA user-fee benefits. In addition, if a product which has an orphan drug designation subsequently receives the first FDA approval for the indication for which it has such designation, the product is entitled to

orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug or biologic for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or the same drug or biologic for different indications. However, competitors may receive approval of different drugs or biologics for the indications for which the orphan product has exclusivity. Notably, a 2021 judicial decision, Catalyst Pharms., Inc. v. Becerra, challenged and reversed an FDA decision on the scope of orphan product exclusivity for the drug, Firdapse. Under this decision, orphan drug exclusivity for Firdapse blocked approval of another company's application for the same drug for the entire disease or condition for which orphan drug designation was granted, not just the disease or condition for which approval was received. In a January 2023 Federal Register notice, however, the FDA stated that it intends to continue to apply its regulations tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved. The exact scope of orphan drug exclusivity will likely be an evolving area.

Orphan product sameness decisions are also an evolving space. The FDA recently issued a final guidance document on how the agency will determine the "sameness" of gene therapy products. Pursuant to the guidance, "sameness" will depend on the products' transgene expression, viral vectors groups and variants, and other product features that may have a therapeutic effect. Generally, minor differences between gene therapy products will not result in a finding that two products are different. Any FDA sameness determinations could impact our ability to receive approval for our product candidates and to obtain or retain orphan drug exclusivity.

Rare Pediatric Disease Voucher Program

Under the FDCA, the FDA awards priority review vouchers to sponsors of rare pediatric disease products that meet certain criteria. To qualify, the rare disease must be serious or life-threatening in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. Also, the product must contain no active ingredient (including any ester or salt of the active ingredient) that has been previously approved in any other application and the application must meet certain additional qualifying criteria, including eligibility for FDA priority review. If FDA determines that a product is for a rare pediatric disease and the qualifying application criteria are met, upon a sponsor's request, FDA may award the sponsor a priority review voucher. This voucher may be redeemed to receive priority review (i.e., a review time of 6 months as compared to 10 months for standard review) of a subsequent marketing application for a different product. Use of a priority review voucher is subject to an FDA user fee. These vouchers are transferable. Accordingly, sponsors may sell these vouchers for substantial sums of money. Vouchers may also be revoked by FDA under certain circumstances and sponsors of approved rare pediatric disease products must submit certain reports to FDA.

Changes to the FDCA, however, have limited future pediatric priority review vouchers. Under the law's sunset provision, the drug or biologic must be designated by FDA for a rare pediatric disease no later than September 30, 2024, and approved no later than September 30, 2026, unless the law is reauthorized by Congress. Accordingly, while Upstaza currently has a rare pediatric disease designation, if we cannot secure FDA BLA approval prior to September 30, 2026, we may not be able to receive the benefit of such designation.

Hatch-Waxman Act for Drugs.

Section 505 of the FDCA describes three types of drug marketing applications that may be submitted to the FDA to request marketing authorization for a new drug. A Section 505(b)(1) NDA is an application that contains full reports of investigations of safety and efficacy. A 505(b)(2) NDA is an application that contains full reports of investigations of safety and efficacy but where at least some of the information required for approval comes from investigations that were not conducted by or for the applicant and for which the applicant has not obtained the right of reference or use from the person by or for whom the investigations were conducted. This regulatory pathway enables the applicant to rely, in part, on the FDA's prior findings of safety and efficacy for an existing product, or published literature, in support of its application. Section 505(j) establishes an abbreviated approval process for a generic version of approved drug products through the submission of an Abbreviated New Drug Application, or ANDA. An ANDA provides for marketing of a generic drug product that generally has the same active ingredients, dosage form, strength, route of administration, labeling, performance characteristics and intended use, among other things, to a previously approved product, called the reference listed drug. Certain differences, however, between the reference listed drug and ANDA product may be permitted pursuant to a suitability petition. Certain labeling differences may also be permitted if information in the reference listed

drug's label is protected by patent or exclusivities. ANDAs are termed "abbreviated" because they are generally not required to include nonclinical and clinical data to establish safety and efficacy. Instead, generic applications must scientifically demonstrate that their product is bioequivalent to, or performs in the same manner as, the innovator drug through in vitro, in vivo, or other testing. The generic version must deliver the same amount of active ingredients to the site of action in the same amount of time as the innovator drug and can often be substituted by pharmacists under prescriptions written for the reference listed drug. In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent with claims that cover the applicant's drug or a method of using the drug. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's list of Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential competitors in support of approval of an ANDA or 505(b)(2) NDA. In an effort to clarify which patents must be listed in the Orange Book, in January 2021, Congress passed the Orange Book Transparency Act of 2020, which largely codifies FDA's existing practices into the FDCA.

Upon submission of an ANDA or 505(b)(2) NDA, an applicant must certify to the FDA that (1) no patent information has been submitted to the FDA; (2) such patent has expired; (3) the date on which such patent expires; or (4) such patent is invalid or will not be infringed upon by the manufacturer, use or sale of the drug product for which the application is submitted. The applicant may also elect to submit a "section viii" statement certifying that its proposed label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. Generally, the ANDA or 505(b)(2) NDA approval cannot be made effective until all listed patents have expired, except where the ANDA or 505(b)(2) NDA applicant challenges a listed patent through the last type of certification, also known as a paragraph IV certification.

If the ANDA or 505(b)(2) NDA applicant has provided a paragraph IV certification to the FDA, the applicant must send notice of the certification to the NDA and patent holders. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the paragraph IV certification, in which case the FDA may not make an approval effective until the earlier of 30 months from the patent or application owner's receipt of the notice of the paragraph IV certification, the expiration of the patent, when the infringement case concerning each such patent is favorably decided in the applicant's favor or settled, or such shorter or longer period as may be ordered by a court. This prohibition is generally referred to as the 30 month stay. In instances where an ANDA or 505(b)(2) NDA applicant files a paragraph IV certification, the NDA holder or patent owner(s) regularly take action to trigger the 30 month stay. Thus, approval of an ANDA or 505(b)(2) NDA could be delayed for a significant period of time depending on the patent certification the applicant makes and the reference drug sponsor's decision to initiate patent litigation.

Exclusivity provisions under the FDCA can delay the submission or the approval of certain applications for competing products. The FDCA provides a five-year period of non-patent exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the therapeutic activity of the drug substance. During the exclusivity period, the FDA generally may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company that contains the new chemical entity. However, an ANDA or 505(b)(2) NDA may be submitted after four years if it contains a certification of patent invalidity or non-infringement.

The FDCA also provides a shorter three-year period of exclusivity for an NDA, 505(b)(2) NDA, or supplement to an existing NDA or 505(b)(2) NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application. Three-year exclusivity may be granted for example, for new indications, dosages, strengths or dosage forms of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and, as a general matter, does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for generic versions of the original, unmodified drug product. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

BPCIA Exclusivity

The 2010 Patient Protection and Affordable Care Act included the BPCIA as a subtitle. The BPCIA established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars. The FDA has issued a number of guidance documents outlining an approach to review and approval of biosimilars, including guidance documents on the demonstration of interchangeability and the licensure of biosimilar and interchangeable products for fewer than all of the reference product's licensed conditions of use.

Under the BPCIA, a manufacturer may submit an application for licensure of a biologic product that is "biosimilar to" or "interchangeable with" a previously approved biological product or "reference product." In order for the FDA to approve a biosimilar product, it must find that there is a high degree of similarity to the reference product, notwithstanding minor differences in clinically inactive components, and that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. Biosimilarity must be shown through analytical studies, animal studies, and at least one clinical trial, absent a waiver by the FDA. There must be no difference between the reference product and a biosimilar in mechanism of action, conditions of use, route of administration, dosage form, and strength. For the FDA to approve a biosimilar product as interchangeable with a reference product, the FDA must find that the biosimilar product can be expected to produce the same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date of approval of the reference product. The FDA may not approve a biosimilar product until 12 years from the date on which the reference product was approved. However, certain changes and supplements to an approved BLA, and subsequent applications filed by the same sponsor, manufacturer, licensor, predecessor in interest, or other related entity do not qualify for the 12 year exclusivity period. Even if a product is considered to be a reference product eligible for exclusivity, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor's own nonclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products.

The BPCIA also includes provisions to protect reference products that have patent protection. The biosimilar product sponsor and reference product sponsor may exchange certain patent and product information for the purpose of determining whether there should be a legal patent challenge. Based on the outcome of negotiations surrounding the exchanged information, the reference product sponsor may bring a patent infringement suit and injunction proceedings against the biosimilar product sponsor. The biosimilar applicant may also be able to bring an action for declaratory judgment concerning the patent.

The FDA maintains a publicly-available online database of licensed biological products, which is commonly referred to as the "Purple Book." The Purple Book lists product names, dates of licensure, and applicable periods of exclusivity. Further, the reference product sponsor must provide patent information and patent expiry dates to FDA following the exchange of patent information between biosimilar and reference product sponsors. This information is then published in the Purple Book.

In an effort to increase competition in the drug and biologic product marketplace, Congress, the executive branch, and FDA have taken certain legislative and regulatory steps. For example, measures have been proposed and implemented to facilitate product importation. Moreover, the 2020 Further Consolidated Appropriations Act included provisions requiring that sponsors of approved drug and biologic products, including those subject to REMS, provide samples of the approved products to persons developing 505(b)(2) NDA or ANDA drug products, or biosimilar products within specified timeframes, in sufficient quantities, and on commercially reasonable market-based terms. Failure to do so can subject the approved product sponsor to civil actions, penalties, and responsibility for attorney's fees and costs of the civil action. This same bill also includes provisions with respect to shared and separate REMS programs for reference and generic drug products.

Patent Term Restoration

If approved, drug and biologic products may also be eligible for periods of U.S. patent term restoration. If granted, patent term restoration extends the patent life of a single unexpired patent, that has not previously been extended, for a maximum of five years, and only those claims reading on the approved drug may be extended. The total patent life of the product with the extension also cannot exceed fourteen years from the product's approval date. Subject to the prior limitations, the period of the extension is calculated by adding half of the time from the effective date of an IND to the initial submission of a marketing application, and all the time between the submission of the marketing application and its approval. This period may also be reduced by any time that the applicant did not act with due diligence.

Pediatric exclusivity

Pediatric exclusivity is another type of non-patent market exclusivity in the United States and, if granted, provides for the attachment of an additional six months of market protection to the term of any existing Orange Book- listed patents or regulatory exclusivity, including the non-patent exclusivity periods described above. This six-month exclusivity may be granted based on the voluntary completion of a pediatric study or studies in accordance with an FDA-issued "Written Request" for such a study or studies within a specified timeframe prior to the expiration of the underlying patent or market exclusivity period to be extended.

Regulation outside the United States

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable regulatory authorities of ex-U.S. countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others. And, even if regulatory approval is granted, it may be withdrawn or limited under certain circumstances or post-approval requirements may be imposed by the applicable regulatory authority. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Regulation in the European Union

We have obtained an orphan medicinal product designation from the European Commission, following an evaluation by the EMA's Committee for Orphan Medicinal Products, for Translarna for the treatment of nmDMD, Upstaza for the treatment of AADC deficiency, Evrysdi for the treatment of SMA, vatiquinone for the treatment of Friedreich ataxia, utreloxastat for the treatment of ALS, PTC-AS for the treatment of Angelman syndrome, sepiapterin for the treatment of patients with hyperphenylalaninemia, including hyperphenylalaninemia caused by PKU, and unesbulin for the treatment of LMS. The European Commission can grant orphan medicinal product designation to products for which the sponsor can establish that it is intended for the diagnosis, prevention, or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in 10,000 people in the EU, or (2) a life threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives it is unlikely that sales of the drug in the EU would generate a sufficient return to justify the necessary investment. In addition, the sponsor must establish that there is no other satisfactory method approved in the EU of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients. Orphan drug designation is not a marketing authorization. It is a designation that provides a number of benefits, including fee reductions, regulatory assistance, and, in the event of a successful application for a centralized EU marketing authorization, 10 years of EU market exclusivity. During this market exclusivity period, neither the EMA, nor the European Commission nor any EU member states can accept an application or grant a marketing authorization for a "similar medicinal product." A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The market exclusivity period for the authorized

therapeutic indication may be reduced to six years if, at the end of the fifth year, it is established that the orphan designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. In addition, a competing similar medicinal product may in limited circumstances be authorized prior to the expiration of the market exclusivity period, including if it is shown to be safer, more effective or otherwise clinically superior to our product. Our product candidates can lose orphan designation, and the related benefits, prior to us obtaining a marketing authorization if it is demonstrated that the orphan designation criteria are no longer met.

Clinical Trial Developments. The structure and general regulation of clinical trials for both small molecule and biological medicines in the EU is similar to that in the United States. Separately, a new regulation, (EU) No.536/2014, regarding clinical trials of medicinal products for humans is included in the European regulatory framework and fills a series of regulatory gaps in the clinical trials regime through the creation of a uniform framework for the authorization of clinical trials by all interested EU member states with a single assessment of the results. The regulation (which came into effect on January 31, 2022) is thus intended to facilitate cross-border cooperation through streamlining of the rules on clinical trials across the EU, including by requiring the submission of clinical trial authorization applications via a new electronic EU portal.

Alongside the portal, a database is being created that will contain information on clinical trial data. The information on the database will be publicly accessible unless the trial data's confidentiality can be justified on the basis of protection of commercially confidential information, protection of personal data, protection of confidential communication between EU countries, or ensuring effective supervision of the conduct of clinical trials by EU countries. A sponsor of a trial conducted in the EU under the new regulation will be required to submit a summary of the clinical trial results to the EU database within a year of the end of the trial. In addition, where the trial was intended to be used for obtaining a marketing authorization (whether through the centralized procedure or via the national authorities), the applicant must submit the clinical study report within 30 days after the marketing authorization has been granted (or refused or withdrawn).

Overview of application process. To obtain regulatory approval of a drug under the EU's regulatory systems and authorization procedures, an applicant may submit marketing authorization applications under a centralized, decentralized, or national procedure. The centralized procedure is compulsory for certain medicinal products, including orphan medicinal products, like Translarna for the treatment of nmDMD, and medicinal products produced by certain biotechnological processes, and optional for certain other innovative products. The centralized procedure enables applicants to obtain a marketing authorization that is valid in all EU member states based on a single application. Under the centralized procedure, the EMA's Committee for Human Medicinal Products, or CHMP, is required to adopt an opinion on a valid application within 210 days, excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions.

More specifically, on day 120 of the procedure, once the CHMP has received the preliminary assessment reports and opinions from the rapporteur and co-rapporteur, it prepares a list of potential outstanding issues, referred to as "other concerns" or "major objections". These are sent to the applicant together with CHMP's recommendation. In addition, in relation to advanced therapy medicinal products, or ATMPs, which are medicines based on genes, cells or tissues, the Committee for Advanced Therapies, or CAT, EMA's committee responsible for assessing the quality, safety and efficacy of ATMPs, prepares a draft opinion on the ATMP application that is submitted to EMA before the CHMP adopts a final opinion on the marketing authorization of the applicable medicine. The CHMP can make one of two recommendations: (1) the marketing authorization could be granted provided that satisfactory answers are given to the "other concerns" and/or "major objections" identified and that all conditions outlined in the list of outstanding issues are implemented and complied with; or (2) the product is not approvable since there are "major objections".

Applicants have three months from the date of receiving the potential outstanding issues to respond to the CHMP, and can request a three-month extension if necessary. The granting of a marketing authorization will depend on the recommendations and potential major objections identified by the CHMP as well as the ability of the applicant to adequately respond to these findings. An accelerated assessment can be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, in particular from the viewpoint of therapeutic innovation. In this circumstance, the EMA ensures that the opinion of the CHMP is given within 150 days. After the adoption of the CHMP opinion, a decision on the marketing authorization application must be adopted by the European Commission, after consulting the EU member states, which in total can take more than 60 days.

An applicant for a marketing authorization application may request a re-examination in the event of a negative opinion, in connection with which CHMP appoints new rapporteurs. Within 60 days of receipt of the negative opinion, the applicant must submit a document explaining the basis for its request for re-examination. The CHMP has 60 days to consider the applicant's request for re-examination. The applicant may request an oral explanation before the CHMP, which is routinely granted, following which CHMP will adopt a final opinion. The final opinion, whether positive or negative, is published by the CHMP shortly following the CHMP meeting at which the oral explanation takes place. The EMA publishes a European Public Assessment Report, or EPAR, for every medicine granted a central marketing authorization by the European Commission following an assessment by the CHMP. EPARs are full scientific assessment reports of medicines authorized by the EMA.

Conditional marketing authorizations. In specific circumstances, as with Translarna for the treatment of nmDMD, EU legislation enables applicants to obtain a marketing authorization on a conditional basis prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional approvals may be granted for products designated as orphan medicinal products, if (1) the benefit-risk balance of the product is positive, (2) it is likely that the applicant will be in a position to provide the required comprehensive clinical trial data, (3) the product fulfills unmet medical needs, and (4) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the benefit-risk balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization. The granting of a conditional marketing authorization will depend on the applicant's ability to fulfill the conditions imposed within the agreed upon deadline.

For important information about matters that may adversely affect our ability to renew our conditional marketing authorization for Translarna, see "Item 1A. Risk Factors-Risks Related to the Development and Commercialization of our Product and our Product Candidates" and "Risks Related to Regulatory Approval of our Product and our Product Candidates."

Variations to conditional marketing authorizations. After the granting of a conditional marketing authorization, the marketing authorization holder may submit an application to vary the conditional marketing authorization under a variation procedure. In the case of the introduction of an additional therapeutic indication, the timeframe for the variation procedure for the initial assessment of the dossier is generally 90 days (plus up to 20 days for validation).

However, in the framework of a variation application assessment procedure, the EMA may send one or more requests for supplementary information to the marketing authorization holder, requiring that additional information be provided by the marketing authorization holder to support its variation application. Such supplementary requests will be sent together with a timetable stating the date by when the marketing authorization holder must submit the requested data and, where appropriate, the extended evaluation period to be applied to such variation procedure. The 90-day variation procedure may be suspended for up to three months for the marketing authorization holder to submit its responses to such supplementary requests. The marketing authorization holder will be notified of the outcome of the CHMP's assessment of the variation procedure within 15 days from the adoption of the CHMP opinion. If unfavorable, the CHMP opinion may be subject to a re-examination procedure upon the marketing authorization holder's request. This may imply an additional minimum two-month procedure. If the CHMP opinion is favorable, the European Commission will usually vary the marketing authorization to introduce the additional therapeutic indication within approximately two months from the receipt of the final CHMP opinion.

Exceptional Circumstances. Similarly, certain of our product candidates may be eligible for a marketing authorization under exceptional circumstances. Such an authorization may be granted where the applicant can demonstrate in its application that it is unable to provide comprehensive data on efficacy and safety under normal conditions of use, because: 1) the indications for which the product in question is intended are encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence; 2) in the present state of scientific knowledge, comprehensive information cannot be provided; or 3) it would be contrary to generally accepted principles of medical ethics to collect

such information. Authorizations under exceptional circumstances are annually reassessed and granted subject to a requirement for the applicant to implement certain procedures, in particular, competent authority notification in the event of any safety issue. After 5 years, the authorization is renewed under exceptional circumstances for an unlimited period, unless European Medicines Agency decides, on justified grounds relating to pharmacovigilance, to proceed with one additional five-year renewal. A marketing authorization under exceptional circumstances will not be granted when a conditional marketing authorization is more appropriate. Orphan products are further eligible for approval under exceptional circumstances only if the criteria considered for the approval under exceptional circumstances are fulfilled.

Additional requirements and considerations. Prior to obtaining a marketing authorization in the EU, applicants have to demonstrate compliance with all measures included in an EMA-approved Pediatric Investigation Plan, or PIP, covering all subsets of the pediatric population, unless the EMA has granted (1) a product-specific waiver, (2) a class waiver, or (3) a deferral for one or more of the measures included in the PIP. In the case of orphan medicinal products, completion of an approved PIP can result in an extension of the aforementioned market exclusivity period from ten to twelve years.

In the EU there is also a procedure which allows member states to authorize the distribution of an unauthorized medicinal product in response to the spread of pathogens. The UK (but no EU countries) used this procedure with two COVID-19 vaccines during December 2020. Notwithstanding the UK's subsequent full departure from the EU, the EU provision is mirrored in UK medicines legislation.

In the EU, for a period of eight years from the grant of a marketing authorization of an innovative product (the "reference medicinal product"), competent authorities may not accept marketing authorization applications from applicants seeking to market "generic medicinal products" where such applications rely on the data in the marketing authorization dossier of the reference product. Moreover, generic medicinal products that rely on the independently generated data of the reference product may not be placed on the market for 10 years from the granting of the initial marketing authorization for that reference medicinal product. This is extended to a maximum of 11 years if, during the first eight years of those 10 years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications considered to offer a significant clinical benefit in comparison with existing therapies. These periods of data exclusivity do not prevent other companies from obtaining a marketing authorization based on their own independently generated data.

If a marketing authorization is granted in the EEA for a medicinal product, such as the marketing authorization granted for Translarna for the treatment of nmDMD by the European Commission, the marketing authorization holder is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of the medicinal products that are in addition to the other conditions of the marketing authorization described above. The marketing authorization holder must, for example, comply with the EU's stringent pharmacovigilance or safety reporting rules, pursuant to which post- authorization studies and additional monitoring obligations can be imposed. Other requirements relate to, for example, the manufacturing of products and active pharmaceutical ingredients in accordance with good manufacturing practice standards. Competent authorities of EU member states may conduct inspections to verify compliance with applicable requirements, and the marketing authorization holder will have to continue to expend time, money and effort to remain compliant. Non-compliance with EU requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties in the EU Similarly, failure to comply with the EU's requirements regarding the protection of individual personal data can also lead to significant penalties and sanctions. Individual EU member states may also impose various sanctions and penalties in case we do not comply with locally applicable requirements. The CAT is involved in any procedure regarding the provision of advice on the conduct of efficacy follow-up, pharmacovigilance and risk management systems of ATMPs as provided for in ATMP legislation.

Off-label promotion of medicinal products is prohibited in the EU. The applicable laws at EU level and in the individual EU member states also prohibit the direct-to-consumer advertising of prescription-only medicinal products. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict our promotional activities with healthcare professionals. In addition, legislation adopted at the EU level and by individual EU member states require that promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics, or SmPC, as approved by the competent authorities. The SmPC is the document that provides information to physicians concerning the

safe and effective use of the medicinal product. Promotion of indications not covered by the SmPC is specifically prohibited. ATMP legislation lays down certain minor extra labelling requirements for ATMPs.

The EMA is responsible for coordinating inspections to verify compliance with the principles of GCP, good manufacturing practice, or GMP, GLP, and good pharmacovigilance practice. These inspections are also intended to verify compliance with other aspects of the supervision of authorized medicinal products in use in the EU. The EMA coordinates any inspection by the relevant member state regulatory authority as requested by the CHMP in connection with the assessment of marketing authorization applications or matters referred to these committees. Inspections may be routine or triggered by issues arising during the assessment of the dossier or by other information, such as previous inspection experience. Inspections usually are requested during the initial review of a marketing authorization application, but could arise post-authorization.

Inspectors are drawn from the regulatory authorities of member states of the EU and the EEA. Following an inspection, the inspectors provide a written inspection report to the inspected site or applicant and provide an opportunity for response. Some inspection reports require follow-up and may result in additional adverse consequences due to critical or major findings. The inspectors and the CHMP will comment on any response from an inspected site or applicant and may monitor future compliance with any proposed corrective action plan.

In the GCP area, inspectors grade their findings according to the following scale:

- Critical: Conditions, practices or processes that adversely affect the rights, safety or well-being of the subjects or the quality and integrity of data. Observations classified as critical may include a pattern of deviations classified as major.
- Major: Conditions, practices or processes that might adversely affect the rights, safety or well-being of the subjects and/or the quality and integrity of data. Observations classified as major may include a pattern of deviations or numerous minor observations.
- Minor: Conditions, practices or processes that would not be expected to adversely affect the rights, safety or wellbeing of the subjects or the quality and integrity of data. Minor observations indicate the need for improvement of conditions, practices and processes.
- Comments: Suggestions on how to improve quality or reduce the potential for a deviation to occur in the future.

Possible consequences of critical and major findings include rejection of clinical trial data, causing significant delays in obtaining final marketing authorization, or other direct action by national regulatory authorities.

Falsified Medicines Directive – As of February 2019, new legislation required manufacturers of marketed prescription medicines to place safety features on all medicines and contribute financially to the establishment of a verification system allowing the authenticity of a medicine to be assessed at the time of supply to the patient. Under the legislation, all packages of prescription medicines placed on the market in Europe have to bear two safety features: a unique identifier in the form of a two-dimensional data matrix (barcode) and an anti-tamper device. In addition, ATMP legislation requires a procedure for tracing the product and its starting and raw materials from its source to the site where the product is used.

Early access programs

Many jurisdictions allow the supply of unauthorized medicinal products in the context of strictly regulated and exceptional EAP programs, and some countries may provide reimbursement for drugs provided in the context of such programs. In the EU, the legal basis for EAP programs, also referred to as named-patient and compassionate use programs, is set out in the EU legislation regulating the authorization, manufacture, distribution and marketing of medicinal products. Detailed regulatory requirements applicable to EAP programs have been adopted and implemented by EU member states in their national laws. The promotion, advertising and marketing of unauthorized medicinal products is generally prohibited, and authorization for EAP programs must generally be obtained from national competent authorities, which might not grant such authorization. Obtaining authorization for an EAP program in one country does not ensure that authorization will be obtained in another country.

U.S. law permits “expanded access” (also known as compassionate use and treatment use) for certain patients with serious diseases who have no comparable alternative treatment options. The potential patient benefit must justify the potential risks of the treatment use and those potential risks must not be unreasonable in the context of the disease or condition to be treated. Moreover, providing the investigational drug or biologic for the requested use must not interfere with the initiation, conduct, or completion of clinical investigations that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use. Additional requirements apply depending on the size of the expanded access population. To provide expanded access, sponsors, including individual physicians, must submit detailed regulatory information to the FDA and receive the agency’s approval for the use. However, if there is an emergency that requires that a patient be treated before a written submission can be made, the FDA may authorize the expanded access use by telephone. In such a case, a written expanded access submission must be submitted to the FDA within fifteen working days of the FDA’s authorization. Following approval for expanded access use, both the sponsor of the use and the investigator (i.e., physician) must comply with certain FDA requirements. Sponsors may not promote products as safe or effective for expanded-access uses.

Pharmaceutical Pricing and Reimbursement

The containment of healthcare costs has become a priority of federal, state and ex-U.S. governments, and the prices of pharmaceuticals have been a focus of this effort. Ex-U.S. governments, the U.S. government, and state legislatures have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, increases in rebates paid, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs.

In some countries, particularly the countries of the EU, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing and reimbursement negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product and there is only limited EU-level control over the decision-making autonomy of the government authorities including in relation to timing, justification and the ability to challenge such decisions. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. In some countries, governments can set conditions that must be satisfied for prices to be set at a certain value. Political, economic and regulatory developments may further complicate pricing and reimbursement negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU member states, and parallel distribution (arbitrage between low-priced and high- priced member states), can further reduce prices. In some countries we may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product or product candidate to other available therapies in order to obtain reimbursement or pricing approval.

In the United States, federal price reporting laws require manufacturers to calculate and report complex pricing metrics used to determine prescription rebates paid under the Medicaid Drug Rebate Program and amounts reimbursed pharmacies and other providers by the Medicaid and Medicare programs. Various state healthcare programs similarly obligate us to report drug pricing information that is used as the basis for their reimbursement of pharmacies and other healthcare providers and the negotiation of supplemental rebates. Payment for a manufacturer’s drugs by these programs is conditioned on submission of this pricing information. Some government healthcare programs impose penalties if drug price increases exceed specified percentages or inflation rates, and these penalties can result in mandatory penny prices for certain federal and 340B program customers. States, such as California, have also enacted transparency laws that require manufacturers to report price increases and related information, and may cap price increases, or require negotiation of supplemental rebates for new drugs entering the market at price points determined to be high. Refusal to negotiate supplemental rebates can negatively affect market access and provider reimbursement. Failure to comply with the rules for calculating and submitting pricing information or otherwise overcharging the government or its beneficiaries may result in criminal, civil, or administrative sanctions or enforcement actions, and expose us to federal civil False Claims Act, or the False Claims Act, liability.

The Veterans Health Care Act of 1992 requires, as a condition of payment by certain federal agencies and the Medicaid program, that manufacturers of “covered drugs” (including all drugs approved under an NDA) enter into a Master Agreement and Federal Supply Schedule (FSS) contract with the Department of Veterans Affairs through which their covered drugs must be offered for sale at a mandatory calculated ceiling price to certain federal agencies, including the

VA and Department of Defense. FSS contracts require compliance with applicable federal procurement laws and regulations, including disclosure of commercial prices during contract negotiations and maintenance of price relationships during the term of the contract, and subject manufacturers to contractual remedies as well as administrative, civil, and criminal sanctions. The Veterans Health Care Act also requires manufacturers to enter into pricing agreements with the Department of Health and Human Services to charge no more than a different ceiling price (derived from the Medicaid rebate percentage) to covered entities participating in the 340B drug discount program. Failure to accurately report drug pricing or to provide the mandatory discount may subject the manufacturer to specific civil monetary penalties. Termination of either of these agreements also jeopardizes payment by Medicaid and Medicare for the manufacturer's drugs in an outpatient setting. Certain states have also enacted drug price transparency laws that require reporting of pricing information, including certain increases in a drug's wholesale acquisition cost and the reasons causing the price increase.

Coverage policies, third-party reimbursement rates and drug pricing regulation may change at any time. For example, in the United States, healthcare reform measures under the Affordable Care Act, contain provisions that may affect the profitability of drug products. However, since its passage, Congress has repealed and amended certain provisions of the Affordable Care Act, repeal efforts may occur again, and legal challenges to the Affordable Care Act may contribute to the uncertainty of the ongoing implementation and impact of the Affordable Care Act and underscore the potential for additional reform going forward. Certain provisions of enacted or proposed legislative changes may negatively impact coverage and reimbursement of, or rebates paid by manufacturers for, healthcare items and services. We cannot assure that the Affordable Care Act, as currently enacted or as amended in the future, will not adversely affect our business and financial results and we cannot predict how future federal or state legislative or administrative changes relating to healthcare reform will affect our business.

Legislators and regulators at both the federal and state level are increasingly focused on containing the cost of drugs, and there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, penalize companies that do not agree to cap prices paid for certain drugs, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. For example, in 2016, the Centers for Medicare and Medicaid Services, or CMS, issued a final rule regarding the Medicaid drug rebate program, which among other things, revises the manner in which the "average manufacturer price" or AMP is to be calculated by manufacturers participating in the program and implements certain amendments to the Medicaid rebate statute created under the Affordable Care Act, or ACA. More recently, Congress amended the Medicaid statute, effective October 1, 2019, to exclude prices paid by secondary manufacturers for an authorized generic drug (but not a product approved under the BLA process) from the NDA holder's AMP for the brand, thereby increasing the rebate amount and the 340B price for the brand. This was implemented by CMS in a final rule issued December 31, 2020. The rule also expanded the definition of products identified as "line extensions" and, in certain circumstances, required inclusion of patient copay assistance in Medicaid best price (effective January 1, 2023), thereby potentially increasing Medicaid rebates paid by manufacturers for such drugs. 340B program guidance regulations on civil monetary penalties for statutory violations, which had been finalized in early 2017 but deferred, also recently went into effect.

On November 27, 2020, CMS issued an interim final rule implementing a Most Favored Nation payment model under which reimbursement for certain Medicare Part B drugs and biologicals will be based on a price that reflects the lowest per capita Gross Domestic Product-adjusted (GDP-adjusted) price of any non-U.S. member country of the Organisation for Economic Co-operation and Development (OECD) with a GDP per capita that is at least sixty percent of the U.S. GDP per capita. This rule now has been rescinded, but other efforts to address the costs of pharmaceuticals have been adopted, including the Inflation Reduction Act of 2022, or the IRA. These and any additional healthcare reform measures could further constrain our business or limit the amounts that federal and state governments will pay for healthcare products and services, which could result in additional pricing pressures.

Any regulatory approval of a product is limited to specific diseases and indications for which such product has been deemed safe and effective by the FDA. Coverage by federal healthcare programs, however, may be more limited than the indications for which a drug is approved by the FDA or comparable ex-U.S. regulatory authorities' coverage of the same products. Sales of any products for which we may receive regulatory approval for commercial sale will depend in part on the extent to which the costs of the products will be covered and reimbursed by third-party payors, including government

healthcare programs (such as, in the United States, Medicare and Medicaid), private health insurers and other organizations. Obtaining reimbursement for orphan drugs may be particularly difficult because of the significant research and development challenges and costs and resulting pricing considerations typically associated with drugs developed to treat conditions that affect a small population of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement in connection with drugs that are perceived as having high costs. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors.

The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third- party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the approved products for a particular indication. Third-party payors are increasingly challenging the price and examining the cost-effectiveness of medical products and services. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our product or product candidates or conduct direct head-to-head studies to demonstrate clinical superiority and cost-effectiveness. Our products and product candidates may not be considered clinically superior and cost-effective to competitor products.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and other third-party payors fail to provide adequate coverage and reimbursement.

Freedom of Information Requests and Affirmative Disclosures

We are also subject, in the U.S. and many other countries, to various regulatory schemes that require disclosure of clinical trial data or allow access to our data via freedom of information requests. We have been and may, from time to time, be notified by regulators, such as the EMA or the competent authorities of EU member states that they have received a freedom of information request for documents that they hold relating to our company, including information related to our product or our product candidates. For example, in 2015, we were notified by the EMA that it had received from another pharmaceutical company a request under Regulation (EC) No 1049/2001 seeking access to aspects of our marketing authorization application for Translarna for the treatment of nmDMD. Following the decision of the EMA to release such documentation with only minimal redactions we initiated litigation before the General Court of the EU to prevent disclosure of this information. In the first quarter of 2018, the Court ruled in favor of the EMA, allowing the EMA to release the documentation. We appealed the General Court's decision to the Court of Justice of the EU, or CJEU, but the CJEU dismissed our appeal in January 2020 and released the information to the requester. In addition, under policies recently adopted in the EU, clinical trial data submitted to the EMA in MAAs that were traditionally regarded as confidential commercial information is now subject to automatic public disclosure. Further, under the Clinical Trials Regulation 536/2014, the sponsor of an EU trial must submit a summary of the results to an EU database within a year of the end of the trial. In addition, where the trial was intended to be used for obtaining a marketing authorization the applicant must submit the clinical study report 30 days after MA has been granted, refused or withdrawn. Subject to our limited ability to review and redact a narrow sub-set of confidential commercial information, these new EU policies will result in the EMA's public disclosure of certain of our clinical study reports, clinical trial data summaries and clinical overviews for recently completed and future MAA submissions. The move toward public disclosure of development data could adversely affect our business in many ways, including, for example, resulting in the disclosure of our confidential methodologies for development of our products, preventing us from obtaining intellectual property right protection for innovations, requiring us to allocate significant resources to prevent other companies from violating our intellectual property rights, adding even more complexity to processing health data from clinical trials consistent with applicable data privacy regulations, and enabling competitors to use our data to gain approvals for their own products.

Fraud and Abuse Laws

Any present or future arrangements or interactions with third-party payors, healthcare professionals, healthcare organizations, patients and other customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may restrict certain marketing and contracting practices. These laws include, and are not limited to, anti-kickback and false claims statutes.

Both the federal Foreign Corrupt Practices Act, or FCPA, and the UK Bribery Act of 2010, or Bribery Act are broad in scope and will require companies to make and keep books and records that accurately and fairly reflect the transactions of the company and to devise and maintain an adequate system of internal accounting controls. The FCPA prohibits the offering, promising, giving, or authorizing others to give anything of value, either directly or indirectly, to a non-U.S. government official, political party or candidate for public office in order to improperly influence any act or decision, secure any other improper advantage, or obtain or retain business. The FCPA also prohibits any U.S. person from corruptly acting outside the U.S. in furtherance of such offer, promise or payment. Under the UK Bribery Act, companies which carry on a business or part of a business in the United Kingdom may be held liable for bribes given, offered or promised to any person, including non-UK government officials and private persons, by employees and persons associated with the company in order to obtain or retain business or a business advantage for the company. Similar statutes have been adopted, or may be adopted in the future, by other countries in which we operate and with which we are or may be required to comply.

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, in cash or kind, to induce or reward either the referral of an individual for, or the purchase, or order or recommendation of, any good or service, for which payment may be made in whole or in part under federal and state healthcare programs such as Medicare and Medicaid. This statute imposes criminal penalties and has been broadly interpreted to apply to manufacturer arrangements with prescribers, purchasers and formulary managers, among others. Although a number of statutory exemptions and regulatory safe harbors exist to protect certain common activities from prosecution, the exemptions and safe harbors for this statute are narrow, and practices that involve compensation intended to induce prescriptions, purchases, or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. HHS recently promulgated a regulation that is effective in two phases. First, the regulation excludes from the definition of “remuneration” limited categories of (a) PBM rebates or other reductions in price to a plan sponsor under Medicare Part D or a Medicaid Managed Care Organization plan reflected in point-of sale reductions in price and (b) PBM service fees. Second, the regulation expressly provides that rebates to plan sponsors under Medicare Part D either directly to the plan sponsor under Medicare Part D, or indirectly through a pharmacy benefit manager will not be protected under the anti-kickback statute discount safe harbor. Recent legislation delayed implementation of this portion of the rule until January 1, 2026, and further proposed legislation would permanently prohibit implementation of the rule beyond 2026. Our practices may not always meet all of the criteria for safe harbor protection. A person or entity need not have knowledge of the statutes or the specific intent to violate it in order to have committed a violation. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act. Federal enforcement agencies have shown increased interest under the federal Anti-Kickback Stature and the federal civil False Claims Act in pharmaceutical companies’ product and patient assistance programs, including reimbursement and co-pay support services and donations to independent charitable patient assistance programs. A number of investigations into these programs have resulted in significant civil and criminal settlements. Most states have adopted laws similar to the federal Anti-Kickback Statute, which apply to items and services reimbursed under Medicaid and other state programs; furthermore, in several states, these statutes and regulations apply regardless of the payor, including to commercial plans. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a manufacturer and its products from participation in federal healthcare programs, debarment from federal government procurement and non-procurement programs, criminal fines, and imprisonment. Several other countries, including the United Kingdom, have enacted similar anti-kickback, fraud and abuse laws and regulations.

The federal civil False Claims Act imposes civil liability and penalties on individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent, knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim, or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. Claims under the federal civil False Claims Act may be initiated by whistleblowers, who receive substantial financial incentives to come forward, through “qui tam” actions that can be pursued by the whistleblower even if the government declines to prosecute the case. Intent to deceive is not necessary to establish civil liability, which may be predicated on deliberate indifference or reckless disregard for the truth. The federal government continues to use the False Claims Act, and the accompanying threat of significant liability, in investigations against pharmaceutical and healthcare companies. These investigations have involved, for example, allegations of improper financial relationships with referral sources, providing free product to customers with the expectation that the customers would bill federal programs for the free product, as well as the promotion

of products for unapproved uses and reporting false pricing information. A violation of the federal Anti-Kickback Statute is a per se violation of civil False Claims Act. Potential liability under the federal civil False Claims Act includes treble damages and significant per claim penalties. The criminal federal False Claims Act imposes criminal fines or imprisonment against individuals or entities who make or present a claim to the government knowing such claim to be false, fictitious or fraudulent. Conviction or civil judgment for violation of the False Claims Act can also result in debarment from federal government procurement and non-procurement programs and exclusion from participation in federal healthcare programs. The majority of states also have statutes or regulations similar to the federal False Claims Act, which apply to items and services reimbursed under Medicaid and other state programs.

The Affordable Care Act included a provision requiring certain providers and suppliers of items and services to federal healthcare programs to report and return overpayments within sixty days after they are "identified" (the "Overpayment Statute"), after which the recipient of the overpayment incurs federal civil False Claims Act liability. The law prohibits a recipient of a payment from the government from keeping an overpayment when the government mistakenly pays more than the amount to which the recipient is entitled even if the overpayment is not caused by any conduct of the recipient. In 2014 and 2016, the CMS released regulatory guidance (in the form of final rules) to Medicare providers, suppliers and managed care and prescription drug plans regarding how to comply with the Overpayment Statute. Although these Medicare providers, suppliers and plans have faced federal False Claims Act liability since 2010 for failures to comply with the Overpayment Statute, these final rules interpreting the Overpayment Statute provide guidance regarding how to comply with applicable obligations, and guidance to government regulators and enforcement authorities regarding monitoring and prosecuting suspected violations. These final rules are not directly applicable to manufacturers, unless a manufacturer is a direct recipient of payment by an agency such as a research grant, but may impact a manufacturer's customers and potential customers who are Medicare providers, suppliers, and plans. In a proposed rule issued on December 27, 2022, CMS is proposing to revise the standard for "identification" which could significantly reduce the time to investigate and report any possible overpayment, thereby increasing the risk of incurring federal civil False Claims Act liability for healthcare providers and suppliers.

The federal Physician Payments Sunshine Act, enacted as part of the Affordable Care Act, and its implementing regulations, require manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to certain payments and other transfers of value made to or at the request of covered recipients, such as, but not limited to, physicians, physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and certified nurse midwives licensed in the United States and to US teaching hospitals, as well as ownership and investment interests held by physicians and members of their immediate family. Payments made to physicians, other principal investigators and certain research institutions for research, including clinical trials, are included within the ambit of this law. Such information is made publicly available by CMS in a searchable format, with data collected in each calendar year published the following June. Failure to submit required information may result in civil monetary penalties, with increased penalties for "knowing failures," for each payment, transfer of value or ownership or investment interest not timely and accurately reported in an annual submission. If not preempted by this federal law, several states currently require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products and to report gifts and payments to healthcare professionals in those states. Depending on the state, legislation may prohibit various marketing-related activities, such as gift bans, or require the posting of information relating to clinical studies and their outcomes. In addition, certain states, such as California, Nevada, Connecticut and Massachusetts, require pharmaceutical companies to implement compliance programs or marketing codes of conduct and several other states are considering similar proposals. Manufacturers that fail to comply with these state laws can face civil penalties.

Statutory requirements to disclose publicly payments made to healthcare professionals and healthcare organizations have also been enacted in certain European Union member states. In addition, self-regulatory bodies of the pharmaceuticals industry, such as the European Federation of Pharmaceutical Industries and Associations, or EFPIA, have published codes of conduct to which its members have agreed to abide, that require the public disclosure of payments made to healthcare professionals and healthcare organizations. In some countries (including France, Denmark and Portugal) such requirements are enforceable by law.

The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, also created federal criminal statutes that prohibit, among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, a healthcare benefit program, regardless of whether the payor is public or private, in connection with the delivery of, or payment for, healthcare benefits, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense and knowingly and willfully falsifying, concealing, or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items, or services relating to healthcare matters. Additionally, the Affordable Care Act amended the intent requirement of certain of these criminal statutes under HIPAA so that a person or entity no longer needs to have actual knowledge of the statute, or the specific intent to violate it, to have committed a violation.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH Act, and similar state laws also impose obligations on certain entities with respect to safeguarding the privacy, security and transmission of certain individually identifiable health information, known as protected health information. Among other things, the HITECH Act and its implementing regulations make HIPAA's security and certain privacy standards directly applicable to "business associates," defined as persons or organizations of covered entities, other than members of the covered entity's workforce, that create, receive, maintain or transmit protected health information on behalf of a covered entity for a function or activity regulated by HIPAA. The HITECH Act also strengthened the civil and criminal penalties that may be imposed against covered entities, business associates and individuals, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, other federal and state laws, such as the California Consumer Privacy Act, may regulate the privacy and security of information that we maintain, many of which may differ from each other in significant ways and may not be preempted by HIPAA.

Outside of the U.S., additional privacy and data protection laws may apply to our operations. For example, the European General Data Protection Regulation, or GDPR, United Kingdom's implementation of the GDPR and equivalent Swiss legislation may apply to some or all of the clinical or other protected data obtained, transmitted, or stored from those territories. These laws require specific, freely given and fully informed consent to be obtained from patients or clinical study participants. The consent must also be capable of being withdrawn. There are also other requirements for lawful processing, including transparency obligations, data minimization requirements, data transfer restrictions and compliance obligations with individuals' stringent rights to access their personal data and to otherwise control the processing of their personal data. There are data breach notification obligations, to supervisory authorities and to individuals, where there are potential risks to them arising from the data breach. These laws impose high regulatory fines in the event of breach of processing requirements of up to 4% of global annual turnover or EUR 20 million (whichever is the higher amount). The European, UK and Swiss legislation only permits data export to countries where there is adequate protection or where other mechanisms are in place such as data transfer agreements in the approved form such as standard contractual clauses or the UK approved clauses. In July 2020, the European Court declared the EU-US data 'Privacy Shield' invalid meaning that data transfers to the United States require other lawful data transfer mechanisms. Further certain privacy laws and genetic testing laws may apply directly to our operations and/or those of our collaborators and may impose restrictions on our use and dissemination of individuals' health information.

In addition, interactions between pharmaceutical companies and physicians are also governed by industry self-regulation codes of conduct and physicians' codes of professional conduct. In the United States, some state laws require pharmaceutical companies to comply with these industry and physician codes and the relevant compliance guidance for pharmaceutical manufacturers promulgated by the federal government. The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the EU. The provision of benefits or advantages to physicians is also governed by the national laws of the EU member states, as well as codes of conduct issued by self-regulatory industry bodies. Moreover, agreements with physicians must often be the subject of prior notification and approval by the physician's employer, their competent professional organization, and the competent authorities of the individual EU member states. These requirements are provided in the national laws, industry codes, or professional codes of conduct, applicable in the EU member states.

Any continuing efforts to modify, repeal, or otherwise invalidate all, or certain provisions of, the Affordable Care Act, could have an impact on fraud and abuse provisions and other requirements, including the Physician Payments Sunshine Act, that were authorized and enacted under the Affordable Care Act.

Human Capital Resources

As of December 31, 2022, we had 1,410 employees, of whom 1,402 were employed on a full-time basis, as well as 128 consultants and contractors, of whom 113 were full-time. None of our U.S. based employees are represented by labor unions or covered by collective bargaining agreements, although certain international employees are covered by collective labor agreements established under local law. We consider our relationship with our employees to be good.

We believe that our growth and success is dependent on the contributions of our employees, as led by our executive officers. We focus significant attention on attracting, retaining, engaging and further developing talented and experienced individuals to manage and support our operations. In particular, recruiting and retaining qualified scientific, clinical, manufacturing, commercial, marketing and support personnel is critical to our success. Competition for these skilled personnel is high. We believe that our strong culture of teamwork and desire to be ever better helps us to attract and retain employees. Our employees complete Gallup, Inc.'s CliftonStrengths talent assessment and attend related training sessions. These tools have been implemented to help our employees identify their core strengths and learn how to use these strengths to become more engaged and productive at work as well as to lead an overall more satisfied and healthier lifestyle. Our Brazilian office was recognized as a "great place to work" by the Great Place to Work Institute in 2021 and 2022.

Based on external benchmarks, we offer employees a number of additional resources and tools to help in their personal and professional development, including career coaching, targeted leadership development for identified current and emerging leaders, internal and external development programs, professional assessment tools, a paid subscription to a digital on-demand career and management learning solutions platform and a wellness website through which employees may access information regarding scheduled healthy lifestyle activities, articles and other beneficial resources. To help newly hired employees, our global onboarding team conducts monthly surveys and focus groups and each newly hired employee is paired with a "buddy" to assist in their transition. Additionally, we require specialized leadership training for all employees that are responsible for the management of others within our organization. Our executive team routinely reviews employee turnover throughout the organization to monitor employee satisfaction.

We believe that we provide a competitive total rewards offering to our employees, with market competitive cash compensation, equity, and industry competitive company-paid benefits, including subsidized medical, and dental insurance and retirement plans, as well as group vision insurance, tuition reimbursement, fitness reimbursement and benefits and policies to support parental leave, mental health and wellness, family planning and child bonding. Total rewards offerings are established by employee positions, skill levels, experience, knowledge, and geographic location. We also provide flexible work arrangements to our employees, including remote work options when practicable. In addition, to assist our employees during times of personal disasters that impact them and their families, we have established an employee relief program that is funded by our employees with corporate matches.

We are committed to hiring, developing and supporting a diverse and inclusive workplace, and continue to focus on extending our equality, diversity and inclusion initiatives across our workforce. All of our employees are required to adhere to our Code of Business Conduct and Ethics, and all relevant country regulations which sets forth the high level of integrity, legal compliance and patient-centric focus expected of all our employees. We have a Chief Culture and Community Officer who oversees our culture and community team. The mission of our culture and community team is to collaborate with cross functional partners and create intentional efforts to connect and engage with employees who want to find community and apply their passion to make a difference. A core element of this mission is our equality, diversity and inclusion, or ED&I, program which is managed by an ED&I professional, who routinely meets with our executive committee. Our ED&I program seeks to enable all employees to feel a sense of purpose and belonging through their connections with our internal communities. This program is guided by a steering committee comprised of senior leaders, volunteer ED&I ambassadors and representatives from our seven Employee Resource Groups, or ERGs, each of which associates with a different underrepresented community. Our ERGs meet monthly and serve to offer a safe place for our employees to have conversations about social issues, celebrate cultural observances and to grow as individuals. We believe that our ERGs

and our ED&I program help our employees to better understand and celebrate each other, resulting in a more cohesive work environment.

We continue to provide opportunities for talented individuals through our global Talent Pipeline Program, or the TPP. The TPP is a global fellowship program aimed at providing recent diverse graduates real-world experience in the biopharmaceutical industry and related professions, including research, clinical, finance, commercial, marketing, compliance, quality, legal, information technology, human resources, government affairs, and communications. Participants are recruited from a global diverse group of institutions and networks and are provided mentorship, job coaching, career counseling, and leadership training for one year. Participants from the TPP are often offered full-time positions based upon our workforce needs. The TPP was originally established in 2020 to benefit students that graduated during the COVID-19 pandemic.

We have continued to maintain a COVID-19 task force as COVID-19 outbreaks continue to manifest throughout the world. Our COVID-19 task force consists of senior leaders from various departments within our organization and is responsible for the safety of our employees, consultants and contractors throughout the world and for the maintenance of our business continuity. Additionally, our COVID-19 task force continues to monitor and evaluate safety protocols and procedures to protect our workers as well as business essential operations. Our COVID-19 task force periodically provides updates to our executive team and our board of directors and provides timely communications to our employees. We have encouraged and, where possible, required all employees to be fully vaccinated against COVID-19.

Our Corporate Information

Our principal executive offices are located at 100 Corporate Court, South Plainfield, New Jersey 07080. Our telephone number is (908) 222-7000. We maintain a website at www.ptcbio.com.

Additional Information

We make available, free of charge on our website, www.ptcbio.com, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, and all amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, as soon as reasonably practicable after we electronically file those reports with, or furnish them to, the Securities and Exchange Commission, or SEC. We also make available, free of charge on our website, the reports filed with the SEC by our executive officers, directors and 10% stockholders pursuant to Section 16 under the Exchange Act as soon as reasonably practicable after copies of those filings are provided to us by those persons. Such reports, proxy statements and other information may be obtained through the SEC's website (www.sec.gov). The information contained on, or that can be accessed through, our website is not a part of or incorporated by reference in this Annual Report on Form 10-K.

Item 1A. Risk Factors

The following risk factors and other information included in this Annual Report on Form 10-K should be carefully considered. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Please see page 1 of this Annual Report on Form 10-K for a discussion of some of the forward-looking statements that are qualified by these risk factors. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

Risks Related to the Development and Commercialization of our Products and our Product Candidates

If we are unable to continue to execute our commercial strategy for our products, fail to obtain renewal of, or satisfy the conditions of our marketing authorization for our products, or if we experience significant delays in accomplishing such goals, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources to bring our products to market through research and development, collaborations and acquisitions. Our ability to continue to generate product revenues will depend heavily on the successful commercialization of our products.

If we do not successfully maintain our marketing authorizations for our products and obtain new marketing authorizations for our product candidates and new uses of our approved products, our ability to generate additional revenue will be jeopardized and, consequently, our business will be materially harmed. The success of our products will depend on a number of additional factors, including the following:

- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms on a timely basis, or at all;
- the timing, scope and outcome of commercial launches;
- the maintenance and expansion of a commercial infrastructure capable of supporting product sales, marketing and distribution;
- the implementation and maintenance of marketing and distribution relationships with third parties in territories where we do not pursue direct commercialization;
- our ability to establish and maintain commercial manufacturing arrangements with third-party manufacturers;
- our ability or the ability of our third-party manufacturers to successfully produce commercial and clinical supply of drug on a timely basis sufficient to meet the needs of our commercial and clinical activities;
- successful identification of eligible patients;
- acceptance of the drug as a treatment for the approved indication by patients, the medical community and third-party payors;
- effectively competing with other therapies;
- global trade policies;
- a continued acceptable safety profile of the drug;
- the costs, timing and outcome of post-marketing studies and trials required for our products, including, with respect to Translarna, Study 041;
- protecting our rights in our intellectual property portfolio, obtaining and maintaining regulatory exclusivity and whether we are able to maintain market exclusivity periods under the Orphan Drug Act or equivalent protections in other jurisdictions;
- whether negative results from our clinical or pre-clinical trials of a product for one indication affect the perception of such product in another indication, including with respect to determinations by regulators, including the FDA and EMA, with respect to our ongoing or future regulatory submissions for marketing authorization of our products for any indication;
- whether, with respect to Translarna, we are able to continue to satisfy our obligations under, and maintain, the marketing authorization in the EEA for Translarna for the treatment of nmDMD, including whether the EMA determines on an annual basis that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA, on the current approved label;
- whether, and within what timeframe, we are able to advance Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for our products on adequate terms;
- our ability to successfully prepare and advance regulatory submissions for marketing authorizations for our products in additional territories and for additional or expanded indications and whether and in what timeframe we may obtain such authorizations; and

- the ability and willingness of patients and healthcare professionals to access our products through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to continue to commercialize our products, either of which would have a material adverse effect on our business, results of operations and financial condition.

Delays or failures in obtaining regulatory approval would prevent us from commercializing our product candidates in the applicable territory and our ability to generate revenue will be materially impaired. Moreover, should we need to conduct additional development work, other than those we have planned, we expect to incur significant costs, which may have a material adverse effect on our business and results of operations.

There is significant risk that we will be unable to obtain approval for our product candidates on a timely basis or at all, and we may be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost. Product development is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. This is especially true for rare and/or complicated diseases. A failure of one or more clinical or preclinical trials, or manufacturing development can occur at any stage. Preclinical and clinical studies may also reveal unfavorable product candidate characteristics, including safety concerns, or may not demonstrate product candidate efficacy. In some instances, there can be significant variability in results between different clinical trials of the same product candidate due to numerous factors. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing authorization of their products.

The approval process is also subject to the substantial discretion of regulatory authorities and the approval procedures vary among countries, can involve additional testing, and the time for approval may materially differ and be subject to administrative delays that we cannot control. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, the failure to obtain approval in one jurisdiction may compromise our ability to obtain approval elsewhere.

In response to changes in the regulatory environment or requests from regulators, we may elect, or be obliged, to postpone a regulatory submission to include additional analyses, which could cause delays in getting our products to market and substantially increase our costs. Securing marketing authorization also requires the submission of information about the product manufacturing process to, and inspection or conduct of remote regulatory assessments of manufacturing facilities by, the regulatory authorities. Changes to manufacturers, product candidate formulation, manufacturing processes and other product candidate attributes, such as the method of delivery, during product candidate development may also require additional studies to demonstrate the comparability of the product candidate using prior processes, formulation, or manufacturers, or with the prior attributes, to the product candidate using new the processes, formulation, or manufacturers, or with the new attributes.

For example, we have been seeking FDA approval for Translarna for nmDMD with the FDA since 2010 and the FDA has repeatedly disagreed with our interpretation of our results. In October 2017, the Office of Drug Evaluation I of the FDA issued a Complete Response Letter for the NDA, stating that it was unable to approve the application in its current form. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied our appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients' muscles. We followed the FDA's recommendation and collected, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, and announced the results of Study 045 in February 2021. Study 045 did not meet its pre-specified primary endpoint. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Following this announcement, we submitted a meeting request to the FDA to gain clarity on the regulatory pathway for a potential re-

submission of an NDA for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support NDA re-submission. We recently had an informal meeting with the FDA, during which we discussed the potential path to an NDA re-submission for Translarna. Based on the meeting discussion, we plan to request an additional Type C meeting with the FDA in the near future to review the totality of data collected to date, including dystrophin and other mechanistic data as well as additional analyses that could support the benefit of Translarna.

With respect to Upstaza, in a late 2019 interaction with the FDA, the FDA requested additional information concerning the use of the commercial delivery system for Upstaza in young patients. In response to the FDA's request, we provided additional information concerning the use of the commercial cannula for Upstaza in young patients. In October 2022, we held a type C meeting with the FDA to discuss the details of a potential submission package for Upstaza. At such meeting, the FDA asked for additional bioanalytical data in support of comparability between the drug product used in the clinical studies and the commercial drug product. We have completed these analyses and provided the results to the FDA for review. We expect to submit a BLA to the FDA in the first half of 2023.

There is no guarantee that we will be able to achieve our milestones at all or within our anticipated timeframes, or that regulators may have additional questions to which we will need to respond. There is also substantial risk that the results of our future or current studies will not ultimately support the approval of a product candidate. Any delays in obtaining regulatory approval, or if we never obtain regulatory approval, could have a material adverse effect on our business, financial condition and results of operations.

We may use certain specialized pathways to develop our product candidates or to seek approval. We may not qualify for these pathways or such pathways may not ultimately speed the time to approval or result in product candidate approval.

In the United States, we may pursue the accelerated approval pathway for certain of our product candidates, such as Translarna. However, the FDA may find that our product candidates do not qualify for accelerated approval. Moreover, even if we do ultimately receive accelerated approval, we would need to meet certain post approval requirements, such as completing a post-approval study confirming our product candidates' clinical benefit that may require substantial time, effort, and funds. Under a newly enacted law, the FDA must specify the conditions for the required post approval studies, including enrollment targets, the study protocol, milestones, and target completion dates, by the time of approval and the FDA may require that the post-approval studies be commenced before the date of approval. If this study does not confirm the product's clinical benefit or if the study is not conducted in accordance with the FDA's requirements, it would be subject to the risk of expedited FDA withdrawal. Additional regulatory requirements also include the pre-submission of promotional materials to the FDA and potential restrictions, such as distribution restrictions, to assure the product's safe use. In recent years, the accelerated approval pathway has come under significant FDA and public scrutiny. Accordingly, depending on the results of our studies, the FDA may be more conservative in granting accelerated approval or, if granted, may be more apt to withdrawal approval if clinical benefit is not confirmed. Due to these and other uncertainties, we are unable to estimate the timing or potential for product candidates for which we may use the accelerated approval pathway or the cost or effort required to receive FDA approval. Further, even if we receive accelerated approval, there is no guarantee that we would be able to maintain such approval.

For our gene therapy product candidates, we may pursue an exceptional circumstances marketing authorization from the EMA. If a product candidate is eligible for marketing authorization under exceptional circumstances, the authorization would be subject to a requirement for the applicant to implement specific procedures, in particular related to notification of the competent authorities of any safety issue. Such exceptional circumstance marketing authorizations are annually reassessed and after five years, the authorization may be renewed under exceptional circumstances for an unlimited period, or the EMA may decide, on justified grounds relating to pharmacovigilance, to proceed with one additional five-year renewal. If any product we have is approved under the exceptional circumstances process, there is no guarantee that we will be able to maintain such approval. Moreover, our product candidates may not be eligible for exceptional circumstances marketing authorization.

If we or our collaborators experience any of a number of possible unforeseen events in connection with clinical trials related to our products or our product candidates, maintenance of our existing marketing authorization for our products and any additional potential marketing authorization or commercialization of our products or our product candidates could be delayed or prevented.

We or our collaborators may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing authorization or commercialize our products or our product candidates, including:

- clinical trials may produce negative or inconclusive results, regulators may disagree with our interpretation of results, our studies may fail to reach the necessary level of statistical significance, or we may not be able to demonstrate that our product candidates are safe, effective, or provide an advantage over current standard of care or other therapies;
- our clinical trials may not meet their primary endpoints. For example, for Translarna, the primary efficacy endpoint in the intent to treat, or ITT, population did not achieve statistical significance in the Phase 2b trial (completed in 2009), Phase 3 trial in ACT DMD (completed in 2015), or Study 045 (completed in 2021);
- there may be flaws in our clinical trials' design that may not become apparent until the clinical trials are well advanced or regulators may not agree with the design of our studies or our analysis of the resulting data;
- clinical trial sites or enrolled patients may be negatively affected by outbreaks of COVID-19 or other outbreaks of contagious disease, resulting in delays and disruptions in completing clinical trials, such as the delays we experienced in enrolling our registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in children with mitochondrial disease associated seizures trial as some patients were unable or hesitant to travel to clinical trial sites due to the COVID-19 pandemic;
- we may be unable to enroll a sufficient number of patients in our clinical trials, the number of patients required for clinical trials may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials, not comply with trial procedures, misrepresent their eligibility, or be lost to follow-up at a higher rate than we anticipate;
- we may enroll patients in foreign countries in which clinical sites may have less experience with studies or the disease at issue, or may use a different standard of care; regulatory authorities may not accept the data generated at foreign sites;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all, or we may be required to engage in additional clinical trial site monitoring;
- regulators, institutional review boards, institutional biosafety committees, or independent ethics committees may not authorize us or our investigators to commence or continue a clinical trial, may require additional data or studies, or may require changes to our studies, including applications and protocols;
- we may be unable to engage trial sites and contract research organizations or they may withdraw from our studies;
- we, regulators, institutional review boards, institutional biosafety committees, or independent ethics committees may require the suspension or termination of studies for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our products or our product candidates may be greater than we anticipate or we may have insufficient funds for a clinical trial or to pay the substantial user fees required by the FDA upon the filing of a marketing application;
- the supply or quality of our products or our product candidates or other materials necessary to conduct clinical trials of our products or our product candidates may be insufficient or inadequate;
- regulators may require us to perform additional or unanticipated studies, develop additional manufacturing information, or make changes to our manufacturing process to obtain approval;
- there may be changes in the applicable regulatory authorities' approval requirements, which may render our data insufficient to obtain marketing approval;
- the FDA or comparable regulatory authorities may disagree with our intended indications;
- regulators may fail to approve or subsequently find fault with the manufacturing processes or facilities for clinical and future commercial supplies;
- the FDA or comparable regulatory authorities may take longer than we anticipate to make a decision on our product candidates; or

- we may decide to abandon the development of a product candidate or development program.

These risks may be increased for product candidates intended for the treatment of diseases for which there is little clinical experience, where we are using new endpoints or methodologies, or where the product candidates are new or novel. For example, there are no marketed therapies approved to treat the underlying cause of nmDMD and there is limited clinical trial experience with respect to drugs to treat nmDMD and other diseases that we are studying or have studied. As a result, the design and conduct of clinical trials for these diseases, particularly for drugs to address the underlying nonsense mutations causing these diseases in some subsets of patients, is subject to increased risk. Furthermore, the regulatory requirements regarding gene therapies are continually evolving and regulatory authorities have only approved a limited number of gene therapies. Moreover, because gene therapy products are a relatively new development, less is known about such products and product candidates and, accordingly there is an increased risk that such products may not perform as expected. Regulatory review agencies and the requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval studies, limitations or restrictions.

We may also experience increased risks to the extent that product candidates require a specialized delivery device or method. For example, Upstaza is administered directly to the putamen in the brain using stereotactic surgery, a brain surgery requiring significant skill and training. There is little experience with such surgeries being used to deliver drugs and for such surgeries being performed on children. We may need to train sufficient brain surgeons to perform the procedure properly, which may expose us to additional regulatory risks as our interactions with such healthcare providers must comply with all applicable laws and regulations. As a result, we will need to invest significant resources to ensure all personnel and contractors are adequately trained on these requirements and to monitor their conduct. Delivery of Upstaza to the putamen also requires certain medical devices, which may result in our product candidate being deemed to be a combination product by the FDA, requiring compliance with the FDA's device regulations and collaboration with medical device manufacturers.

Our product development costs will increase if we experience delays in testing or marketing authorizations, and we may not have sufficient funding to complete the testing and approval process for any of our product candidates. We may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of our products and product candidates. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our products or our product candidates and allow our competitors to bring products to market before we do or impair our ability to successfully commercialize our products or our product candidates, and so may harm our business, results of operations and financial condition.

Subgroup, retrospective, post-hoc, and certain statistical analyses may not be reliable and typically will not form the basis for regulatory approval.

In the event that a study's primary endpoint is not met, companies may undertake certain analyses to further understand the data and potential reasons for the study results, including retrospective, post-hoc, and subgroup analyses. Because these analyses are not pre-planned and studies may not be adequately designed for these analyses, they may not be reliable and typically will not form the basis for regulatory approval. For example, after determining that we did not achieve the primary efficacy endpoint with the pre-specified level of statistical significance in our completed ACT DMD and Phase 2b clinical trials of Translarna for the treatment of nmDMD, we performed subgroup, retrospective, and meta-analyses. We submitted these analyses to the FDA as part of our NDA, taking the position that the totality of clinical data from these trials support the clinical benefit of Translarna for the treatment of nmDMD. The FDA, however, did not agree that these analyses supported approval.

Some of our favorable statistical data from these trials also are based on nominal p-values. Nominal p-values are subject to certain limitations, and which, because of these limitations, regulatory authorities typically give less weight to nominal p-values, compared to regular p-values. For example, the p-values in ACT DMD for change from baseline at week 48 in the 6-minute walk test, or 6MWT (which we also refer to as 6-minute walk distance, or 6MWD) and each secondary end

point timed function test were nominal p-values. The FDA found that certain post-hoc adjustments, our retrospective analyses and our reliance on nominal p-values for some of our statistical data did not support approval.

An unfavorable view of our data and analyses by regulatory authorities has and could continue to negatively impact our ability to obtain or maintain marketing authorizations, which would have a material adverse effect on our revenue and would materially harm our business, financial results and results of operations.

If we experience delays or difficulties in the enrollment of patients in our clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates, including clinical trials due to the inability to enroll a sufficient number of patients. Patient enrollment is affected a number of factors including:

- the size of the patient population (many of our studies concern rare conditions with small patient populations);
- the availability of approved treatments;
- severity of the disease under investigation;
- eligibility criteria for the study in question;
- perceived benefits and risks of the product candidate under study;
- disruptions caused by and the willingness of patients to enroll in a clinical trial during outbreaks of COVID-19;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- competition from other clinical trials;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

For example, we previously experienced delays in enrolling our registration-directed Phase 2/3 trial of vatiquinone in children with mitochondrial disease associated seizures as some patients were unable or hesitant to travel to clinical trial sites due to the COVID-19 pandemic. We anticipate results from the Phase 2/3 trial to be available in the second quarter of 2023.

Enrollment delays in our clinical trials may result in increased development costs for our product candidates. Our inability to enroll, timely or at all, a sufficient number of patients in our clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

If serious adverse side effects are identified during the development of any product candidate or for any product for which we have or may obtain marketing approval, we may need to abandon or limit our development and/or marketing of that product or product candidate.

If our products or our product candidates are associated with undesirable side effects or have characteristics that are unexpected, regulatory authorities, institutional review boards, institutional biosafety committees, or independent ethics committees may place our studies on clinical hold, withdraw or suspend study approvals, or require that we modify our protocols. We may also need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a benefit-risk perspective. Adverse events or side effects may also result in study recruitment challenges, marketing authorization denial, limitations on the indicated use of a product, the inclusion of warnings, contraindications, or precautions on the label of any approved products, or significant conditions imposed on any approval, including the requirement of a risk evaluation and mitigation strategies, or REMS, costly post-marketing studies or clinical trials and surveillance to monitor the safety of the product. Adverse effects may also prevent the adoption of a product, if it is approved. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause side effects that prevented further development of the compound. Furthermore, we may be sued and held liable for harm caused by our products to patients as a result of the identification of undesirable side effects, which may cause reputational harm.

For example, although we did not observe a pattern of liver enzyme elevations in our Phase 2 or Phase 3 clinical trials of Translarna, we did observe modest elevations of liver enzymes in some subjects in one of our Phase 1 clinical trials. These elevated enzyme levels did not require cessation of Translarna administration, and enzyme levels typically normalized after completion of the treatment phase. We did not observe any increases in bilirubin, which can be associated with serious harm to the liver, in the Phase 1 clinical trial.

In addition, in Study 009, our first Phase 3 clinical trial of Translarna for the treatment of nmCF, five adverse events in the Translarna arm of the trial that involved the renal system led to discontinuation. As compared to the placebo group, the Translarna treatment arm also had a higher incidence of adverse events of creatinine elevations, which can be an indication of impaired kidney function. In the Translarna treatment arm, more severe clinically meaningful creatinine elevations were reported in conjunction with cystic fibrosis pulmonary exacerbations. These creatinine elevations were associated with concomitant treatment with antibiotics associated with impaired kidney functions, such as aminoglycosides or vancomycin. This led to the subsequent prohibition of concomitant use of Translarna and these antibiotics, which was successful in addressing this issue in the clinical trial.

The risk of finding adverse side effects may be particularly heightened in the case of gene therapies. For instance, new gene copies may produce too much or too little of the desired protein or RNA, or the production of the desired protein or RNA may change over time. Because the treatment is irreversible, there may be challenges in managing side effects. Adverse effects would not be able to be reversed or relieved by stopping dosing and might require us to develop additional clinical safety procedures. Furthermore, new gene copies may disrupt other normal biological molecules and processes. Adverse side effects may also be experienced by patients as a result of the process for administering the therapy or related procedures.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia, immune- and complement-mediated responses, and death seen in other trials using other vectors. While new recombinant vectors have been developed to potentially reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. For instance, possible adverse side effects that could occur include an immunologic or complement-mediated reactions early after administration which, could substantially limit the effectiveness of the treatment. Depending on the vector, additional manufacturing, clinical, and preclinical testing may be required, as well as additional analyses, assessments, and potential long-term patient and clinical study subject monitoring and sample testing and associated regulatory reporting. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could further adversely impact our product candidates in the form of increased government regulation, unfavorable public perception, potential regulatory delays, stricter labeling requirements, and a decrease in demand.

If, following approval, we or others identify previously unknown side effects, if such side-effects are severe, or if known side effects are more frequent or severe than in the past then our marketing authorizations may be restricted or withdrawn, changes may be required to the product's label, sales may be adversely impacted, we may be required to undertake additional studies or trials, and government investigations or litigation, including product liability claims, may be brought against us. Additionally, if the safety warnings in our product labels are not followed, adverse medical situations in patients may arise, resulting in negative publicity and potential lawsuits. Any of these occurrences would limit or prevent us from commercializing our products, which would have a material adverse effect on our business, financial results and operations.

Certain of our products and product candidates, such as our gene therapies and other biologic product candidates, may be difficult to produce, presenting manufacturing challenges that may delay product development and regulatory approval.

Manufacturers of pharmaceutical products must comply with strictly enforced manufacturing and quality requirements, including cGMP requirements, state and federal regulations, as well as ex-U.S. requirements when applicable. These may be particularly difficult to meet for complex products such as biologic and gene therapy products. Any failure to meet the applicable manufacturing and quality requirements could lead to a delay or interruption in development programs, delays in receiving regulatory approval, and consequences should we receive marketing approval.

The manufacture of biologic and gene therapy products is technically complex, requires extreme precision to meet specification requirements and necessitates substantial expertise and capital investment. Production difficulties caused by unforeseen events, even if seemingly minimal, may delay the availability of material for clinical studies and commercial product. For example, given the nature of biologics manufacturing, there is a risk of contamination. Any contamination could materially adversely affect our ability to produce our gene therapy product candidates on schedule and could, therefore, harm our results of operations and cause reputational damage.

In addition, gene therapy products have only in limited cases been manufactured at scales sufficient for pivotal trials and commercialization. Few pharmaceutical contract manufacturers specialize in gene therapy products and those that do are still developing appropriate processes, controls and facilities for large-scale production. While we believe that there are alternative sources of supply that can satisfy our clinical and commercial requirements, we cannot be certain that we will be able to identify and establish relationships with such sources, if necessary, in a timely manner or at all, and what the terms and costs of such new arrangements would be, or that such alternative suppliers would be able to supply our potential commercial needs. To the extent that we decide to manufacture our own clinical and commercial supply as an alternative source of supply, there is no guarantee that we will be able to cost effectively produce sufficient quantities of our program material. Any switch from our current manufacturer would result in a significant delay, would require regulatory authority approval, and cause material additional costs.

Furthermore, some of the raw materials and other components required in our manufacturing process are derived from diverse biologic sources that may be difficult to procure and may be subject to contamination or recall. Any material shortage, supply chain disruption, contamination recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the production and commercialization of products.

In 2021, we began cGMP manufacturing of clinical material at the Hopewell Facility for certain of our gene therapy product candidates other than Upstaza. We still rely on third-party manufacturers to complete product testing for all of our gene therapy product candidates that we manufacture at the Hopewell Facility as well as to provide sufficient quantities of certain program materials that we have not yet transitioned to the Hopewell Facility. To the extent we rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers.

With respect to the Hopewell Facility, we have limited experience conducting our own manufacturing and could encounter problems and delays. The Hopewell Facility requires substantial investment and significant expertise, and our management devotes substantial time to its operation. There is substantial competition for skilled personnel within gene therapy manufacturing and we may not be able to attract and retain these personnel on acceptable terms. Moreover, operating a manufacturing facility may cost more than we currently anticipate. If we experience any problems or delays with the Hopewell Facility, we may need to rely on contract manufacturers for the manufacturing of program materials that we intended to produce ourselves, which may not be available or on acceptable terms.

Additionally, we have limited experience producing plasmid DNA and AAV vectors for third party customers, and we have yet to manufacture cGMP gene therapy product materials for our own clinical trials or commercialization. If we are unable to manufacture these product materials to the required specifications for the third parties we contract with, our business, financial condition, and results of operations could be materially adversely affected and we may become subject to regulatory or contractual actions, may need to expend significant time and costs to remedy issues, and we may forgo sales, incur liabilities or lose customers, which would materially adversely affect our business, financial condition and results of operations.

Finally, we and our third party manufacturers may experience any number of unforeseen issues, unforeseen delays, including equipment failure, labor shortages, natural disasters, power failures, transportation difficulties, quality control or other issues, including those resulting from compliance with regulatory requirements, as further described in these risks, that could prevent us from realizing the intended benefits of our manufacturing strategy.

The marketing authorization granted by the European Commission for Translarna for the treatment of nmDMD is limited to ambulatory patients aged two years and older located in the EEA, which significantly limits an already small treatable patient population, which reduces our commercial opportunity and is also subject to annual reassessment of the benefit-risk balance by the EMA as well as the specific obligation to conduct Study 041, and may be varied, suspended or withdrawn by the European Commission if we fail to satisfy those requirements.

The marketing label for Translarna approved by the European Commission is limited to ambulatory nmDMD patients aged two years and older who have been identified through genetic testing as having a nonsense mutation in the dystrophin gene. Prevalence estimates for rare diseases are uncertain due to the uncertainties associated with the methodologies used to derive estimates, such as epidemiology assumptions. It can take many years of experience in rare disease market places before prevalence becomes well characterized. Our estimates of both the number of people who have DMD caused by a nonsense mutation, as well as the subset of people with nmDMD who are ambulatory and at least two years old, are based on our beliefs and estimates derived from a variety of sources and may prove to be either incorrect or subject to additional refinement or characterization on a country specific basis over the coming years. If the market opportunities for Translarna for the treatment of nmDMD are smaller than we believe they are, our business and anticipated revenues will be negatively impacted. If we decide to seek to expand the approved product label of Translarna for the treatment of nmDMD in the future, the timing of, and our ability to generate, the necessary data or results required to obtain expanded regulatory approval is currently uncertain. Given the small number of patients who have nmDMD, and the smaller number of patients who meet the criteria for treatment under our current marketing authorization, our commercial opportunity is limited. It is critical to the commercial success of Translarna for nmDMD that we successfully identify and treat these patients.

In order to continue to generate revenue from Translarna, we must maintain our current marketing authorizations in a number of countries and we also may need to receive or maintain marketing authorizations in other territories. The marketing authorization in the EEA is conditional and subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, which we refer to as the annual EMA reassessment. In June 2022, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2023. This marketing authorization is further subject to a specific obligation to conduct and submit the results of Study 041. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. In September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. We expect an opinion from the Committee for Medicinal Products for Human Use in the first half of 2023.

If the EMA determines in any annual renewal cycle that the balance of benefits and risks of using Translarna for the treatment of nmDMD has changed materially or that we have not or are unable to comply with any conditions that have been or may be placed on the marketing authorization, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or require the imposition of other conditions or restrictions. As such, there is ongoing risk to our ability to maintain our marketing authorization in the EEA. If we are unable to renew our marketing authorization in the EEA during any annual renewal cycle, or if our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program, and in all territories, which would have a material adverse effect on our business, results of operations and financial condition.

Any of our products or any other product candidate that receives marketing authorization, if any, may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

Even if we are successful in obtaining and maintaining marketing authorizations, our products may not gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Third-party payors may require prior authorizations or failure on another type of treatment before covering a particular drug, particularly with respect to higher-priced drugs. Decreases in third-party reimbursement for a product or a decision by a third-party payor to not cover a product could reduce physician usage of the product. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenues or any profits from operations.

The degree of market acceptance of our products or product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages, as well as cost effectiveness compared to alternative treatments;
- the prevalence and severity of any side effects, as well as perceived safety;
- limitations or warnings contained in, as well as permitted claims based on the product's FDA-approved labeling;
- distribution and use restrictions imposed by the FDA or which we voluntarily implement;
- the ability to offer our products or product candidates for sale at competitive prices;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies. For example, gene therapy remains a novel technology, must be administered directly to the brain via a surgery and public perception may be influenced by claims that gene therapy is unsafe, which may cause gene therapy to not gain acceptance by the public or the medical community;
- the convenience and ease of administration compared to alternative treatments;
- the strength of marketing and distribution support;
- sufficient third-party coverage or reimbursement and, where applicable, our ability to obtain pricing approvals which is separate from the marketing authorization process;
- adverse publicity about our and our competitors' products or product candidates or favorable publicity about competitive products or product candidates. For example, earlier gene therapy trials conducted by other organizations have led to several well-publicized adverse events, including cases of leukemia, immune- and complement-mediated adverse events, and death seen in other such organizations' trials using vectors;
- the results of studies of the product in other indications or similar products; and
- any restrictions on concomitant use of other medications.

Obtaining coverage and reimbursement for a product from third-party payers is a time-consuming and costly process. Failure to obtain adequate reimbursement may significantly impact the adoption and sale of products. Market acceptance and obtaining reimbursement coverage may be particularly challenging in the case of gene therapies, where the cost of a single administration may be substantial and adequate coverage and reimbursement will be essential for patients to afford the treatment. Payors may require us to provide supporting scientific, clinical and cost-effectiveness data, which we may not be able to provide. Moreover, ethical, social and legal concerns about certain treatments, such as gene therapy, could result in additional regulations restricting or prohibiting sale of our products.

In the United States, third-party payers, including government payers such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. Expensive specialty drugs in particular are often subject to restriction. The Medicare and Medicaid programs increasingly are used as models for how private payers and government payers develop their coverage and reimbursement policies. We cannot be assured that Medicare or Medicaid will cover our product candidates that may be approved or provide reimbursement without restriction and at adequate levels to realize a sufficient return on our investment. Our rebate payments may increase or our prices be adjusted under value-based purchasing arrangements based on evidence-based measures or outcomes-based measures for a patient or beneficiary based on use of our drug. Moreover, reimbursement agencies in the EU may be more conservative than CMS. It is difficult to predict what third-party payers will decide with respect to the coverage and reimbursement for our products for which we obtain marketing approval. Additionally, within Europe, each country has its own reimbursement regime employing various health technology assessment approaches to assess the cost-effectiveness of the product (in the United Kingdom a HTA assessment is conducted by NICE) which may significantly affect the effective access to the market.

Our ability to negotiate, secure and maintain third-party coverage and reimbursement may also be affected by political, economic and regulatory developments. Governments continue to impose cost containment measures, and third-party payors are increasingly challenging prices charged for medicines and examining their cost effectiveness, in addition to their safety and efficacy. These and other similar developments could significantly limit the degree of market acceptance of our products or any of our other product candidates that receive marketing authorization.

If we are unable to establish or maintain sales, marketing and distribution capabilities or enter into agreements with third parties to market, sell and distribute our products or product candidates, we may not be successful in our continuing efforts to commercialize our products or any other product candidate if and when they are approved.

Our ongoing commercial strategy for our products and any other product candidate that may receive marketing authorization involves the development of a commercial infrastructure that spans multiple jurisdictions and is heavily dependent upon our ability to continue to build an infrastructure that is capable of implementing our global commercial strategy. The establishment and development of our commercial infrastructure will continue to be expensive and time consuming, and we may not be able to develop our commercial organizations in all intended territories, including in the United States, in a timely manner or at all. Doing so will require a high degree of coordination and compliance with laws and regulations in numerous territories, including restrictions on advertising practices, enforcement of intellectual property rights, restrictions on pricing or discounts, transparency laws and regulations, and unexpected changes in regulatory requirements and tariffs. If we are unable to effectively coordinate such activities or comply with such laws and regulations, our ability to commercialize our products or any other product candidates that may receive marketing authorization will be adversely affected. If we are unable to establish and maintain adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue consistent with our expectations and may not become profitable.

There are risks involved with establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training an internal commercial team is expensive and time consuming and could delay commercialization efforts. If a commercial launch for any product or product candidate for which we recruit a commercial team and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition such personnel.

The arrangements that we have entered into, or may enter into, with third parties to perform sales and marketing services will generate lower product revenues or profitability of product revenues to us than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively.

If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our products or product candidates. Factors that may materially affect our efforts to commercialize our products include:

- our ability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- our ability to monitor the legal and regulatory compliance of sales and marketing personnel;
- an inability to secure adequate coverage and reimbursement by government and private health plans;
- reduced realization on government sales from mandatory discounts, rebates and fees, and from price concessions to private health plans and pharmacy benefit managers necessitated by competition for access to managed formularies;
- the clinical indications for which the products are approved and the claims that we may make for the products;
- limitations or warnings, including distribution or use restrictions, contained in the products' approved labeling;
- any distribution and use restrictions imposed by the FDA or to which we agree as part of a mandatory REMS or voluntary risk management plan;
- liability for sales or marketing personnel who fail to comply with the applicable legal and regulatory requirements;
- our ability to implement third-party marketing and distribution relationships on favorable terms, or at all, in territories where we do not pursue direct commercialization;
- the ability of our commercial team to obtain access to or persuade adequate numbers of physicians to prescribe our current or any future products;

- the lack of complementary products to be offered by our commercial team, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent commercial organization.

Any of these factors, individually or as a group, if not resolved in a favorable manner may have a material adverse effect on our business and results of operations. Similar risks apply in those territories where any of our products are available on a reimbursed basis under an EAP program.

A substantial portion of our commercial sales currently occurs in territories outside of the United States which subjects us to additional business risks that could adversely affect our revenue and results of operations.

We commercialize Translarna, Upstaza, Tegsedi and Waylivra outside of the United States. We have operations in multiple European countries, Latin America and other territories. We expect that we will continue to expand our international operations in the future, including in emerging growth markets, pending successful completion of the applicable regulatory processes. International operations inherently subject us to a number of risks and uncertainties, including:

- political, regulatory, compliance and economic developments that could restrict our ability to manufacture, market and sell our products, including the Russia-Ukraine conflict and related sanctions that have been imposed by various countries in response thereto;
- financial risks such as longer payment cycles, difficulty collecting accounts receivable, potentially high inflation rates and exposure to fluctuations in foreign currency exchange rates;
- difficulty in staffing and managing international operations;
- various effects and responsive measures relating to COVID-19 outbreaks;
- potentially negative consequences from changes in or interpretations of tax laws;
- changes in international medical reimbursement policies and programs;
- unexpected changes in healthcare policies of ex-U.S. jurisdictions;
- trade protection measures, including import or export licensing requirements and tariffs;
- our ability to develop relationships with qualified local distributors and trading companies;
- political and economic instability in particular ex-U.S. economies and markets, in particular in emerging markets, for example in Brazil;
- diminished protection of intellectual property in some countries outside of the United States;
- differing labor regulations and business practices; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors' and service providers' activities that may fall within the purview of the Foreign Corrupt Practices Act, UK Bribery Act or similar local regulation.

For example, the Brazilian Ministry of Health is continuing to experience significant administrative delays processing centralized group purchase orders. Almost all of our product revenue for Translarna in Brazil is attributable to such purchase orders. These centralized group purchase order delays have caused, and may continue to cause, fluctuations in our ability to generate revenue in Brazil.

In addition, some countries in which a product candidate is not approved allow patients access to the product candidate through other legal mechanisms, including court intervention or EAP programs, if the product is approved in another jurisdiction. The price that is ultimately approved by governmental authorities in any country pursuant to commercial pricing and reimbursement processes may be significantly lower than the price we are able to charge for sales under such legal mechanisms and we may become obligated to repay such excess amount.

Some of the countries in which our products are available for sale are in emerging markets. Some countries within emerging markets, including those in Latin America, may be especially vulnerable to periods of global or regional financial instability or may have very limited resources to spend on. We also may be required to increase our reliance on third-party agents within less developed markets. In addition, many emerging market countries have currencies that fluctuate substantially and if such currencies devalue and we cannot offset the devaluations, our financial performance within such countries could be adversely affected.

Furthermore, in some countries, including Brazil and Russia, orders for named patient sales may be for multiple months of therapy, which can lead to an unevenness in orders which could result in significant fluctuations in quarterly net product sales. Other factors may also contribute to fluctuations in quarterly net product sales including a product's availability in any particular territory, government actions, economic pressures, political unrest and other factors. Net product sales are impacted by factors such as the timing of decisions by regulatory authorities and our ability to successfully negotiate favorable pricing and reimbursement processes on a timely basis in the countries in which we have or may obtain regulatory approval, including the United States, EEA and other territories.

Any of these factors may, individually or as a group, have a material adverse effect on our business and results of operations. As we continue to expand our existing international operations, we may encounter new risks.

Laws and regulations governing export restrictions and economic sanctions may preclude us from developing and selling certain products, generating revenue from such products, and manufacturing certain materials outside of the United States.

Many countries, including the United States, restrict the export or import of products to or from certain countries through, for example, bans, sanction programs, and boycotts. Such restrictions may preclude us from supplying products or generating revenue in certain countries or may require an export license prior to the export of the controlled item. Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Furthermore, if we, or third parties acting on our behalf, do not comply with these restrictions, we may be subject to substantial civil and criminal penalties and suspension or debarment from government contracting.

Our activities outside of the United States, require that we dedicate resources to comply with these laws. Many of our customers and suppliers are ex-U.S. entities or have significant ex-U.S. operations. Although these restrictions have not affected our operations in the past, there is a risk that they could do so in the future as additional geographic regions and entities may become subject to such restrictions. The imposition of new or additional economic and trade sanctions against our major customers or suppliers or financial counterparties or intermediaries could result in our inability to sell to, and generate revenue from such customers or purchase materials from such suppliers. For example, we make sales of Translarna through a distributor to the Ministry of Health of the Russian Federation to access Russian nmDMD patients. Our ability to generate and realize revenue in Russia may be materially and adversely impacted as many countries, including the United States, have imposed and may continue to consider imposing additional enhanced export controls on certain products and sanctions on certain industry sectors and parties in Russia in connection with the Russia-Ukraine conflict. We also contract with government-owned hospitals and third-party manufacturers located in China, which has recently been involved in political conflict with the United States. This conflict has increased the likelihood of restrictions that could materially and adversely affect our clinical trial sites located in China, our ability to obtain certain supplies, our ability to manufacture certain product candidates and our ability to potentially commercialize products in China. If our activities are affected because of these or other such restrictions, sanctions, or controls, our business, financial condition and results of operations could be materially and adversely affected. As a result of restrictive export laws, our customers may also seek to obtain a greater supply of similar or substitute products from our competitors that are not subject to these restrictions, which could materially and adversely affect our business, financial condition and results of operations.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current products and product candidates and any products we may seek to develop or commercialize in the future from major pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies worldwide. Other gene therapy companies may in the future decide to utilize existing technologies to address unmet needs that could potentially compete with our product candidates.

There is currently no marketed therapy, other than Translarna in the EEA, which has received approval for the treatment of the underlying cause of nmDMD. Sarepta recently received approval in the United States for two treatments (Exondys

51 (eteplirsen) and Vyondys 53 (golodirsen)) addressing the underlying cause of disease for different mutations in the DMD gene. Additionally, the FDA granted accelerated approval to Viltepso (viltolarsen) from NS Pharma for the treatment of DMD in patients with exon 53 skipping and Sarepta (Casimersen (SRP 4045) for the treatment of DMD in patients with exon 45 skipping. Viltepso (viltolarsen) from NS Pharma is also approved in Japan. Other biopharmaceutical companies are developing treatments for the underlying cause of disease for different mutations in the DMD gene, Daiichi Sankyo (DS-5141), Nippon Shinyaku (Viltolarsen (NS-065/NCNP-01) and NS-089/NCNP-02), and Astellas (AT-702). Other pharmaceutical companies are developing micro dystrophin gene therapies for patients with DMD regardless of genotype, including Pfizer (PF-06939926), Solid Biosciences (SGT-001) and Sarepta (SRP-9001), whose gene therapy has been submitted for accelerated approval to the FDA.

Although the FDA has not approved a corticosteroid specifically for DMD in the United States other than Emflaza, we face competition in the U.S. DMD market from prednisone/prednisolone, which, while not approved for DMD in the United States, is generically available and has been prescribed off label for DMD patients. ReveraGen BioPharma and Santhera are developing a glucocorticoid antagonist (vamorolone) for DMD patients. An NDA for vamorolone has been submitted to and accepted by the FDA, and the Prescription Drug User Fee Act, or PDUFA, date for a decision by the FDA is October 26, 2023.

Currently, no other treatment options are available for the underlying cause of AADC deficiency. Additionally, we are not aware of any late-stage development product candidates for AADC deficiency.

There are several pharmaceutical and biotechnology companies engaged in the development or commercialization of products against targets that are also targets of Tegsedi and Waylivra. For example, Waylivra for FCS faces competition from drugs like Myalept (metreleptin). Myalept, produced by Novelion Therapeutics, Inc., is currently approved for use in generalized lipodystrophy patients. Additionally, Ionis is developing AKCEA-APOCIII-LRx for the treatment of FCS. Currently, no other treatment options are available for the underlying cause of FPL. Additionally, we are not aware of any late-stage development product candidates for FPL. Tegsedi also faces competition from drugs like Onpattro (patisiran), which was launched by Alnylam in the United States in 2018 and received approval in Brazil for the treatment of hATTR amyloidosis in 2020 as well as AMVUTTRA (vutrisiran) which Alnylam received approval for in the United States and Brazil in 2022 for the treatment of the polyneuropathy of hATTR amyloidosis in adults. Vyndaqel (tafamidis meglumine) and Vyndamax (tafamidis) are commercialized in the United States, EU and some other countries in Latin America by Pfizer. Other companies are also pursing product candidates for the treatment of ATTR Amyloidosis with polyneuropathy including BridgeBio Pharma (AG-10), Proclara Biosciences (NPT-189), Prothena (PRK-004) and SOM Biotech (tolcapone).

Further, Tegsedi and Waylivra are delivered by injection, which may render them less attractive to patients than non-injectable products offered by our current or future competitors. If Tegsedi or Waylivra cannot compete effectively with these and other products with common or similar indications, we may not be able to generate substantial revenue from our product sales.

Evrysdi, an orally bioavailable treatment, faces competition from treatments that are not orally bioavailable, including Spinraza (nusinersen), a drug developed by Ionis and marketed by Biogen, which has received FDA approval to treat SMA and Zolgensma (onasemnogene abeparvovec), a gene therapy drug developed by AveXis, Inc., (acquired by Novartis in 2018), which is approved in the United States and Japan for the treatment of SMA in patients under 2 years of age and in Europe for babies and young children who weigh up to 21 kilograms. Other companies are also pursuing product candidates for the treatment of SMA, including Kowa (sodium valproate), Catalyst Pharmaceuticals (amifampridine), Scholar Rock (SRK 015), Roche Pharmaceuticals (RO7204239) and Cytokinetics (reldesemtiv)..

For additional discussion regarding the competition we face with respect to our current product candidates, see “Item 1. Business-Competition.”

Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are marketing or developing or that would render our products or product candidates obsolete or non-competitive. Our competitors may also obtain marketing authorization for their products more rapidly than we may obtain approval for our

products and product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market.

We believe that many competitors are attempting to develop therapeutics for the target indications of our products and product candidates, including academic institutions, government agencies, public and private research organizations, large pharmaceutical companies and smaller more focused companies.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to or necessary for our programs.

Our products or product candidates may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

We may not obtain adequate coverage or reimbursement for our products, or we may be required to sell our products at an unsatisfactory price. In addition, obtaining pricing, coverage and reimbursement approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we do not receive these approvals on a timely basis.

The regulations and practices that govern marketing authorizations, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries, including almost all of the member states of the EEA, require approval of the sale (list) price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some ex-U.S. markets, including the European market, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing authorization for a product in a particular country, but then be subject to price regulations, in some countries at national as well as regional levels, that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more products or other product candidates, even following marketing authorization.

Our ability to successfully commercialize our products or product candidates that may receive marketing authorization will depend in large part on the extent to which coverage and reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers, managed healthcare organizations and other third-party payors and organizations. Government authorities and other third-party payors, such as private health insurers and managed healthcare organizations, decide which medications they will pay for and establish reimbursement conditions and rates. A primary trend in the EU and U.S. healthcare industries and elsewhere is cost containment. Government authorities, including the United States government and state legislatures, and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Prices at which our products are reimbursed can be subject to challenge, reduction or denial by the government and other payers. Increasingly, third-party payors are requiring that drug companies provide them with discounts off the products' sale (list) prices and are challenging the prices manufacturers charge for medical products. We cannot be sure that coverage will be available for any product or product candidate that we may commercialize and, if coverage is available, the level of reimbursement is also uncertain.

Reimbursement levels may impact the demand for, or the price of, any product or product candidate for which we obtain marketing authorization. Obtaining reimbursement for our products has been and is expected to continue to be, particularly difficult due to price considerations typically associated with drugs that are developed to treat conditions that affect a small

population of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement of a higher priced drug, such as prior authorization and the requirement to try other therapies first, or high co-payments which can result in patient rejection. Decreases in third-party reimbursement for a product or a decision by a third-party payor to not cover a product could reduce physician usage of the product. If reimbursement is not available or is available only on a limited basis, we may not be able to successfully commercialize any product or product candidate for which we have obtained or may obtain marketing authorization.

There may be significant delays in obtaining coverage for newly approved drugs, and coverage may be more limited than the drug's approved indications as determined by the applicable regulatory authority. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent, and programs intended to provide patient assistance until coverage is established can be very costly. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Further, coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws, enforcement policies or administrative determinations with respect to the importation of drugs into the United States from other countries where they may be sold at lower prices.

In the United States, third-party payors include federal healthcare programs, such as Medicare, Medicaid, TRICARE, and Veterans Health Administration programs; managed care providers, private health insurers and other organizations. Several of the U.S. federal healthcare programs establish ceiling prices or require that drug manufacturers extend discounts or pay rebates to certain programs in order for their products to be covered and reimbursed. For example, the Medicaid Drug Rebate Program requires pharmaceutical manufacturers of covered outpatient drugs to enter into and have in effect a national rebate agreement with the federal government as a condition for coverage of the manufacturer's covered outpatient drug(s) by state Medicaid programs. The amount of the rebate for each product is based on a statutory formula and may be subject to an additional discount if certain pricing increases more than inflation. State Medicaid programs and Medicaid managed care plans can seek additional "supplemental" rebates from manufacturers in connection with states' establishment of preferred drug lists. A further requirement for Medicaid coverage is that manufacturers of single source and innovator multiple source drugs enter into a Master agreement and Federal Supply Schedule, or FSS, agreement with the Secretary for Veterans Affairs and charge no more than statutory ceiling prices to the Department of Veteran Affairs, the Department of Defense and certain other federal agencies.

Similarly, in order for a covered outpatient drug to receive federal reimbursement under the Medicare Part B and Medicaid programs, the manufacturer must extend discounts on the covered outpatient drug to entities that are enrolled and participating in the 340B drug pricing program, which is a federal program that requires manufacturers to provide discounts to certain statutorily-defined safety-net providers. The 340B discount for each product is calculated based on certain Medicaid Drug Rebate Program metrics that manufacturers are required to report to CMS.

Emflaza is also eligible for reimbursement under the Medicare Part D program. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities, which will provide coverage of outpatient prescription drugs. Part D prescription drug formularies are required to include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any negotiated prices for our products covered by a Part D prescription drug plan likely will be lower than the prices we might otherwise obtain, and payment of Medicare Coverage Gap discounts may further reduce realization on Part D drugs. Further, CMS is proposing to relax Part D coverage requirements to give plans more leverage in negotiating their formularies.

With respect to drugs eligible for reimbursement under Medicare Part B, on November 27, 2020, CMS issued an interim final rule implementing a Most Favored Nations payment model under which reimbursement for certain Medicare Part B drugs and biologicals will be based on a price that reflects the lowest per capita Gross Domestic Product-adjusted (GDP-adjusted) price of any non-U.S. member country of the Organisation for Economic Co-operation and Development

(OECD) with a GDP per capita that is at least sixty percent of the U.S. GDP per capita. This rule now has been rescinded but other measures, including the Inflation Reduction Act of 2022, or IRA, have been enacted to address the costs of pharmaceuticals. Such rules and any additional healthcare reform measures could further constrain our business or limit the amounts that federal and state governments will pay for healthcare products and services, which could result in additional pricing pressures.

In addition, U.S. private health insurers often rely upon Medicare coverage policies and payment limitations in setting their own coverage and reimbursement policies. Any such coverage or payment limitations may result in a similar reduction in payments from non-governmental payors. Payment by private payors is also subject to payor-determined coverage and reimbursement policies that vary considerably and are subject to change without notice. We expect that coverage and reimbursement of Emflaza in the United States will vary from commercial payor to commercial payor. Many commercial payors, such as managed care plans, manage access to prescription drugs partly to control costs to their plans, and may use drug formularies and medical policies to limit their exposure. Exclusion from policies can directly reduce product usage in the payor's patient population and may negatively impact utilization in other payor plans, as well.

There has been recent negative publicity and increasing legislative and public scrutiny around pharmaceutical drug pricing in the U.S., in particular with respect to orphan drugs and specifically with respect to Emflaza. Moreover, U.S. government authorities and third-party payors are increasingly attempting to limit or regulate drug prices and reimbursement, often with particular focus on orphan drugs. These dynamics may give rise to heightened attention and potential negative reactions to pricing decisions for Emflaza and products for which we may receive regulatory approval in the future, possibly limiting our ability to generate revenue and attain profitability.

Moreover, in 2017, the U.S. Congress modified and amended certain provisions of the 2010 U.S. healthcare reform legislation (the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, known collectively as the Affordable Care Act), which could have an impact on coverage and reimbursement for healthcare items and services covered by the federal and state healthcare programs as well as plans in the private health insurance market. The so-called "individual mandate" was repealed as part of tax reform legislation adopted in December 2017. Legal challenges to the Affordable Care Act continue to arise and there may be future efforts to modify, repeal, or otherwise invalidate all, or certain provisions of the Affordable Care Act. The Biden administration is expected to continue to take measures to further facilitate the implementation of the Affordable Care Act. We cannot assure that the Affordable Care Act, as currently enacted or as amended in the future, will not adversely affect our business and financial results and we cannot predict how future federal or state legislative or administrative changes relating to healthcare reform will affect our business.

Additionally, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. Failure of the Joint Select Committee on Deficit Reduction to reach required deficit reduction goals triggered the legislation's automatic reduction to several government programs. This legislation resulted in aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2031. However, pursuant to the CARES Act and subsequent legislation, these Medicare sequester reductions were suspended through the end of March 2022 and from April 2022 through June 2022, a 1% cut was in effect, with the full 2% cut remaining thereafter. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidates is prescribed or used.

In the EU, reference pricing systems and other measures may lead to cost containment and reduced prices with respect to Translarna for the treatment of nmDMD, Upstaza for the treatment of AADC deficiency and other product candidates that might receive marketing authorization in the future. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for our product or any of our product candidates that may receive marketing authorization, or a reduction in coverage for payment rates for our product or any such product candidates, could have a material adverse effect on our business, results of operations and financial condition. In addition, in the EU, an authorized trader, such as a wholesaler, can purchase a medicine in one EU member state and obtain a license to import the product into another EU member state. This process is called "parallel distribution". As a result, a purchaser in one EU

member state may seek to import Translarna from another EU member state where Translarna is sold at a lower price. This could have a negative impact on our business, financial condition, results of operations and growth.

Similarly, sales of Emflaza in the United States could also be reduced if deflazacort is imported into the United States from lower-priced markets, whether legally or illegally. For example, in the United States, prices for pharmaceuticals are generally higher than in the bordering nations of Mexico and Canada. In October 2020, the Department of Health and Human Services, or HHS, and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. The final rule is currently the subject of ongoing litigation, but at least six states (Vermont, Colorado, Florida, Maine, New Mexico, and New Hampshire) have passed laws allowing for the importation of drugs from Canada with the intent of developing SIPs for review and approval by the FDA. Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The implementation of the rules has been delayed by the Biden administration from January 1, 2022 to January 1, 2023 in response to ongoing litigation. The rule also creates a safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufactures, the implementation of which has been delayed until January 1, 2026 by the Infrastructure Investment and Jobs Act.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception and based on our current commercial, research and development plans, we expect to continue to incur significant operating expenses for the foreseeable future. We may never generate profits from operations or maintain profitability.

Since inception, we have incurred significant operating losses. As of December 31, 2022, we had an accumulated deficit of $2,657.0 million. We have historically financed our operations primarily through the issuance and sale of our common stock in public offerings, our “at the market offerings” of our common stock, our initial public offering, proceeds from the Royalty Purchase Agreement, net proceeds from our borrowings under the Credit Agreement, or the Blackstone Credit Agreement, dated as of October 27, 2022, among us, as the Borrower, the subsidiaries of the Borrower from time to time party thereto, as Guarantors, the Lenders from time to time party thereto and Wilmington Trust, National Association, as Administrative Agent, the private placements of our preferred stock and common stock, collaborations, bank and institutional lender debt, other convertible debt, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. We have relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States since 2014, Emflaza for the treatment of DMD in the United States since 2017, and Upstaza for the treatment of AADC deficiency in the EEA since May 2022. We have also relied on revenue associated with milestone and royalty payments from Roche pursuant to the SMA License Agreement under our SMA program. We also began to recognize revenue generated from net sales of Tegsedi for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hATTR amyloidosis in 2019 and Waylivra for the treatment of FCS in 2020 in Latin America and the Caribbean. Based on our current commercial, research and development plans, we expect to continue to incur significant operating expenses for the foreseeable future, which we anticipate will be partially offset by revenues generated from the sale of our products and our collaboration and royalty revenues. We expect to continue to generate operating losses through 2023 and, while we anticipate that operating losses generated in future periods should decline versus prior periods, we may never generate profits from operations or maintain profitability. The net losses we incur may fluctuate significantly from period to period.

From time to time, we have engaged in strategic transactions to expand and diversify our product pipeline, including through the acquisition of assets or businesses. In connection with these acquisitions, we have entered into agreements through which we have ongoing obligations, including obligations to make contingent payments upon the achievement of certain development, regulatory and net sales milestones or upon a percentage of net sales of certain products. See “Item 1. Business-Our Ongoing Acquisition-Related Obligations” for further information regarding our acquisitions and our ongoing obligations. We may engage in additional strategic transactions to expand and diversify our product pipeline, including through the acquisition of assets, businesses, or rights to products, product candidates or technologies or through strategic alliances or collaborations and we may incur expenses, including with respect to transaction costs, subsequent

development costs or any upfront, milestone or other payments or other financial obligations associated with any such transaction.

Our current ability to generate revenue from sales of Translarna is dependent upon our ability to maintain our marketing authorizations in the EEA for Translarna for the treatment of nmDMD in ambulatory patients aged two years and older, in Russia for the treatment of nmDMD in patients aged two years and older and in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. The marketing authorization in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is further subject to a specific obligation to conduct and report the results of Study 041, a multi-center, randomized, double-blind, 18-month, placebo-controlled trial, followed by an 18-month open-label extension, according to an agreed protocol, in order to confirm the efficacy and safety of Translarna. Enrolling, conducting and reporting a clinical trial is a time-consuming, expensive and uncertain process that takes years to complete, and we expect that we will incur material costs related to the implementation and conduct of Study 041. We may experience unknown complications with Study 041 and may not achieve the pre-specified endpoint with statistical significance, which would have a material adverse effect on our ability to maintain our marketing authorization in the EEA.

If, in any annual renewal cycle, the EMA determines that the balance of benefits and risks of using Translarna for the treatment of nmDMD has changed materially or that we have not or are unable to comply with the specific obligation to complete Study 041 or any other requirement that has been or may be placed on the marketing authorization, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or impose other specific obligations or restrictions, which would have a materially adverse effect on our business. We expect to incur significant costs in connection with our efforts to maintain our marketing authorization in the EEA. If our marketing authorization in the EEA is not renewed, or our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or a reimbursed early access program, or EAP program, and throughout all territories. We also expect that our efforts to advance Translarna for the treatment of nmDMD in the United States will be time-consuming and may be expensive.

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with ongoing, planned and potential future clinical trials and studies in our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications. We have begun seeking and intend to continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories outside of the EEA, Brazil and Russia. We are also preparing a BLA for Upstaza for the treatment of AADC deficiency in the United States and we anticipate submitting a BLA to the FDA in the first half of 2023. These efforts may significantly impact the timing and extent of our commercialization expenses.

In addition, the clinical and regulatory developments noted in this risk factor may exacerbate the risks related to our commercialization efforts set forth under the heading "Risks Related to the Development and Commercialization of our Products and our Product Candidates," which could increase the costs associated with our commercial activities or have a negative impact on our revenues.

We may seek to continue to expand and diversify our product pipeline through opportunistically in-licensing or acquiring the rights to products, product candidates or technologies and we may incur expenses, including with respect to transaction costs, subsequent development costs or any upfront, milestone or other payments or other financial obligations associated with any such transaction, which would increase our future capital requirements.

With respect to our outstanding 1.50% convertible senior notes due September 15, 2026, or the 2026 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.3 million annually. With respect to borrowings under the Blackstone Credit Agreement, cash interest payments are payable on the applicable interest payment dates for each loan thereunder. In addition, we will be required under conditions specified in the Blackstone Credit Agreement to fund a reserve account up to certain amounts specified therein. The funds in the reserve

account are available to prepay the Loans at any time at our option, and are, if funded, subject to release upon certain further conditions. Upon any such release, such funds are freely available for use by us subject to the generally applicable terms and conditions of the Blackstone Credit Agreement. Furthermore, the Blackstone Credit Agreement covenant requiring us to have consolidated liquidity of at least $100.0 million as of the last day of each fiscal quarter will be increased to $200.0 million if we consummate acquisitions meeting certain consideration thresholds described in the Blackstone Credit Agreement.

In February 2023, we completed enrollment of our Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event and in accordance with the Agreement and Plan of Merger, dated as of May 5, 2020, or the Censa Merger Agreement, by and among us, Hydro Merger Sub, Inc., our wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa Pharmaceuticals, Inc., or Censa, Shareholder Representative Services LLC, we are obligated to pay a $30.0 million development milestone to the former Censa securityholders, which we have the option to pay in cash or shares of our common stock. We also expect to make additional payments to the former Censa securityholders of $50.0 million in the aggregate upon the potential achievement in 2023 of certain development and regulatory milestones relating to sepiapterin. Furthermore, we expect to pay the former equityholders of Agilis an additional $20.0 million upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency, which we expect to occur in the first half of 2023.

In addition, our expenses will increase if and as we:

- seek to satisfy contractual and regulatory obligations that we assumed through our acquisitions and collaborations;
- execute our commercial strategy for our products, including initial commercialization launches of our products, label extensions or entering new markets;
- are required to complete any additional clinical trials, non-clinical studies or CMC assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- are required to take other steps, in addition to Study 041, to maintain our current marketing authorization in the EEA, Brazil and Russia for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications;
- utilize the Hopewell Facility to manufacture program materials for certain of our gene therapy product candidates as well as program materials for third parties;
- initiate or continue the research and development of our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

Our expenses may also increase as a result of economic conditions, such as potentially high inflation rates within the jurisdictions that we operate or unfavorable fluctuations in foreign currency exchange rates.

Our ability to generate profits from operations and become and remain profitable depends on our ability to successfully develop and commercialize drugs that generate significant revenue. This will require us to be successful in a range of challenging activities, including:

- commercializing and marketing all of our products and products candidates;
- negotiating, securing, and maintaining adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for our products and product candidates;
- maintaining the marketing authorization of Translarna for the treatment of nmDMD in the EEA, including successfully obtaining annual renewals of the marketing authorization, fulfilling the specific obligation to conduct

and report the results of Study 041 to the EMA, and meeting any ongoing requirements related to the marketing authorization;
- advancing Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA re-submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- maintaining orphan exclusivity in the United States for Emflaza;
- successfully completing any post-marketing requirements imposed by regulatory agencies with respect to our products;
- expanding the territories in which we are approved to market our products;
- successfully advancing our other programs and collaborations, including our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for additional indications;
- maintaining a global commercial infrastructure, including the sales, marketing and distribution capabilities to effectively market and sell our products and product candidates throughout the world;
- implementing marketing and distribution relationships with third parties in territories where we do not pursue direct commercialization;
- identifying patients eligible for treatment with our products and product candidates;
- successfully developing or commercializing any product candidate or product that we may in-license or acquire;
- protecting our rights to our intellectual property portfolio related to Translarna and other products and product candidates; and
- contracting for the manufacture and distribution of commercial quantities of our products and product candidates.

We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to generate profits from operations. Even if we do generate profits from operations, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to generate profits from operations and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investment in our company.

We may need additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

As noted in the prior risk factor, we expect to incur significant expenses related to our clinical, regulatory, commercial, legal, research and development, and other business efforts. We believe that our cash flows from product sales, together with existing cash and cash equivalents, will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next twelve months. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect.

Our future capital requirements will depend on many factors, including:

- our ability to commercialize and market our products and product candidates;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for our products and product candidates;
- our ability to maintain the marketing authorization in the EEA for Translarna for the treatment of nmDMD, including whether the EMA determines on an annual basis that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA, on the current approved label;
- the timing and outcome of Study 041;
- our ability to obtain marketing authorization for Upstaza for the treatment of AADC deficiency in the United States;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA re-submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;

- our ability to maintain orphan exclusivity in the United States for Emflaza;
- our ability to successfully complete any post-marketing requirements imposed by regulatory agencies with respect to our products;
- the progress, results and costs of our activities under our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for our products and for any of our other product candidates that may receive marketing authorization or any additional indications or territories in which we receive authorization to market our products;
- our ability to utilize the Hopewell Facility to manufacture program materials for certain of our gene therapy product candidates as well as program materials for third parties;
- the costs, timing and outcome of regulatory review of our other product candidates, including those in our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;
- our ability to satisfy our obligations under the Blackstone Credit Agreement;
- our ability to satisfy our obligations under the indenture governing our 2026 Convertible Notes;
- the timing and scope of growth in our employee base;
- revenue received from commercial sales of or products or any of our other product candidates;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for our products and product candidates on adequate terms, or at all;
- the ability and willingness of patients and healthcare professionals to access our products and product candidates through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products, product candidates, and technologies, including the success of any acquisition, in-licensing or other strategic transaction we may pursue, and the costs of subsequent development requirements and commercialization efforts, including with respect to our acquisitions of Emflaza, Agilis, Censa and of BioElectron's assets, and our licensing of Tegsedi and Waylivra; and
- our ability to establish and maintain collaborations, including our collaborations with Roche and the SMA Foundation, and our ability to obtain research funding and achieve milestones under these agreements.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales for certain product candidates or indications. In addition, our products and product candidates, if approved, may not achieve sustained commercial success. Likewise, if we fail to maintain our marketing authorization or lose non-patent market exclusivity for our products and product candidates, we will be unable to commercialize and generate revenue from the sales of those products.

Accordingly, we may need to continue to rely on additional financing in connection with our continuing operations and to achieve our business objectives. In addition, we may seek additional capital due to favorable market conditions or based on strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. Additional financing may not be available to us on acceptable terms or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or our commercialization efforts.

Our indebtedness under the Blackstone Credit Agreement could adversely affect our financial condition or restrict future operations.

On October 27, 2022, or the Closing Date, we entered into the Blackstone Credit Agreement for fundings of up to $950.0 million consisting of a committed loan facility of $450.0 million and further contemplating the potential for up to $500.0

million of additional financing, to the extent that we request such additional financing and subject to the lenders' agreement to provide such additional financing and to mutual agreement on terms.

The Blackstone Credit Agreement provides for a senior secured term loan facility funded on the Closing Date in the aggregate principal amount of $300.0 million, or the Initial Loans, and a committed delayed draw term loan facility of up to $150.0 million, or the Delayed Draw Loans and, together with the Initial Loans, the Loans, to be funded at our request within 18 months of the Closing Date subject to specified conditions. In addition, the Blackstone Credit Agreement contemplates the potential for further financings by Blackstone, by providing for incremental discretionary uncommitted further financings of up to $500.0 million.

The Loans mature on the date that is seven years from the Closing Date. Borrowings under the Blackstone Credit Agreement bear interest at a variable rate equal to, at our option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively.

All obligations under the Blackstone Credit Agreement are secured by security interests in certain of our assets, including (1) intellectual property and other assets related to Translarna, Emflaza, Upstaza, sepiapterin and, until certain release conditions are met, vatiquinone, in each case, together with any other forms, formulations, or methods of delivery of any such products, and regardless of trade or brand name, (2) future acquired intellectual property (but not internally developed intellectual property unrelated to other intellectual property collateral) and other related assets, and (3) the equity interests held by us in certain of our subsidiaries. The Blackstone Credit Agreement contains certain negative covenants with which we must remain in compliance. The Blackstone Credit Agreement also requires that we maintain consolidated liquidity of at least $100.0 million as of the last day of each fiscal quarter, which shall be increased to $200.0 million upon our consummating acquisitions meeting certain consideration thresholds described therein. In addition, we will be required under conditions specified in the Blackstone Credit Agreement to fund a reserve account up to certain amounts specified therein. The funds in the reserve account are available to prepay the Loans at any time at our option, and are, if funded, subject to release upon certain further conditions. Upon any such release, such funds are freely available for use by us subject to the generally applicable terms and conditions of the Blackstone Credit Agreement. The Blackstone Credit Agreement contains certain customary representations and warranties, affirmative covenants and provisions relating to events of default.

In the event of an acceleration of amounts due under the Blackstone Credit Agreement as a result of an event of default, we may not have sufficient funds or may be unable to arrange for additional financing to repay the Loans or to make any accelerated payments, and the lenders could seek to enforce security interests in the collateral securing the Loans, which would have a material adverse effect on our business, financial condition and results of operations.

In addition, our indebtedness under the Blackstone Credit Agreement could have significant adverse consequences, including, among other things:

- requiring us to dedicate a substantial portion of cash and cash equivalents and marketable securities to the payment of interest on, and principal of, the Loans, which will reduce the amounts available to fund working capital, capital expenditures, product development efforts and other general corporate purposes;
- obligating us to negative covenants restricting our activities, including limitations on dispositions, mergers or acquisitions, encumbering our intellectual property, incurring indebtedness or liens, paying dividends, making investments and engaging in certain other business transactions;
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry;
- placing us at a competitive disadvantage compared to our competitors who have less debt or competitors with comparable debt at more favorable interest rates; and

- limiting our ability to borrow additional amounts for working capital, capital expenditures, research and development efforts, acquisitions, debt service requirements, execution of our business strategy and other purposes.

Any of these factors could materially and adversely affect our business, financial condition and results of operations.

We intend to satisfy our current and future debt obligations with our existing cash, cash equivalents and available for sale securities, potential future product revenue and funds from external sources. However, our inability to satisfy such obligations for any reason would have a material adverse effect on our business, financial condition and results of operations.

We may engage in strategic transactions to acquire assets, businesses, or rights to products, product candidates or technologies or form collaborations or make investments in other companies or technologies that could harm our operating results, dilute our stockholders' ownership, increase our debt, or cause us to incur significant expense.

As part of our business strategy, we may engage in additional strategic transactions to expand and diversify our product pipeline, including through the acquisition of assets, businesses, or rights to products, product candidates or technologies or through strategic alliances or collaborations, similar to our acquisitions of Emflaza, Agilis, Censa and BioElectron's assets and the Tegsedi-Waylivra Agreement. We may not identify suitable strategic transactions, or complete such transactions in a timely manner, on a cost-effective basis, or at all. Moreover, we may devote resources to potential opportunities that are never completed, or we may incorrectly judge the value or worth of such opportunities. Even if we successfully execute a strategic transaction, we may not be able to realize the anticipated benefits of such transaction, may incur additional debt or assume unknown or contingent liabilities in connection therewith, and may experience losses related to our investments in such transactions. Integration of an acquired company or assets into our existing business may not be successful and may disrupt ongoing operations, require the hiring of additional personnel and the implementation of additional internal systems and infrastructure, and require management resources that would otherwise focus on developing our existing business. Even if we are able to achieve the long-term benefits of a strategic transaction, our expenses and short-term costs may increase materially and adversely affect our liquidity. Any of the foregoing could have a detrimental effect on our business, results of operations and financial condition.

In addition, future strategic transactions may entail numerous operational, financial and legal risks, including:

- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions;
- exposure to known and unknown liabilities, including possible intellectual property infringement claims, violations of laws, tax liabilities and commercial disputes;
- higher than expected acquisition and integration costs;
- difficulty in integrating operations and personnel of any acquired business;
- increased amortization expenses or, in the event that we write-down the value of acquired assets, impairment losses;
- impairment of relationships with key suppliers or customers of any acquired business due to changes in management and ownership;
- inability to retain personnel, customers, distributors, vendors and other business partners integral to an in-licensed or acquired product, product candidate or technology;
- potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges;
- entry into indications or markets in which we have no or limited direct prior development or commercial experience and where competitors in such markets have stronger market positions; and
- other challenges associated with managing an increasingly diversified business.

If we are unable to successfully manage any strategic transaction in which we may engage, our ability to develop new products and continue to expand and diversify our product pipeline may be limited.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate enough product revenues to cover our expenses, we expect to supplement our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates; marketing, distribution, licensing or other arrangements.

To the extent that we raise additional capital through the sale of equity or convertible debt securities, our shareholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Our senior secured term loan facility with Blackstone as well as any additional debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, entering into agreements involving licenses to our intellectual property, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates; or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Our ability to use our net operating losses and certain other tax attributes to offset potential taxable income and related income taxes that would otherwise be due is subject to limitation under the provisions of Sections 382 and 383 of the Internal Revenue Code as a result of ownership changes of the Company and could be subject to further annual limitations under such provisions. In addition, we may not generate sufficient future taxable income to use our net operating losses and certain other tax attributes.

If a corporation undergoes an "ownership change" within the meaning of Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or Sections 382 and 383, the corporation's ability to utilize any net operating losses, or NOLs, and certain tax credits and other tax attributes generated before such an ownership change, is limited. We believe that we have in the past experienced ownership changes within the meaning of Sections 382 and 383 that have resulted in limitations under Sections 382 and 383 (and similar state provisions) on the use of our NOLs and other tax attributes.

Sections 382 and 383 are extremely complex provisions with respect to which there are many uncertainties, and we have not requested a ruling from the United States Internal Revenue Service, or IRS, to confirm our analysis of the ownership change limitations related to the NOLs and other tax attributes generated by us. Therefore, we have not established whether the IRS would agree with our analysis regarding the application of Sections 382 and 383. We continue to fully evaluate the impact of a limitation on the use of our NOLs and other tax attributes under Sections 382 and 383.

Moreover, our ability to use these NOLs to offset potential future taxable income and related income taxes that would otherwise be due is dependent upon our generation of future taxable income. We generated taxable income that is subject to income tax in 2022, but continue to maintain NOLs from previous years that will be carried forward.

Changes in our effective income tax rates and future changes to U.S. and non-U.S. tax laws could adversely affect our results of operations.

We are subject to income taxes in the Unites States and various ex-U.S. jurisdictions. Taxes will be incurred as income is earned in these different jurisdictions. Various factors may have favorable or unfavorable effects on our effective income tax rate. These factors include, but are not limited to, interpretations of existing tax laws, changes in tax laws and rates, the accounting for stock options and other share-based compensation, changes in accounting standards, future levels of research and development spending, changes in the mix and level of pre-tax earnings by taxing jurisdiction, the outcome of examinations by the IRS and other jurisdictions, the accuracy of our estimates for unrecognized tax benefits, the realization of deferred tax assets, or by changes to our ownership or capital structure. The impact on our income tax

provision resulting from the above-mentioned factors and others may be significant and could adversely affect our results of operations.

Changes in tax laws or regulations, including further regulatory developments arising from U.S. tax reform legislation as well as multi-jurisdictional changes enacted in response to the action items provided by the Organization for Economic Cooperation and Development (OECD), may increase tax uncertainty and the amount of tax we pay.

On December 22, 2017, the United States government enacted the 2017 Tax Act, which significantly reformed the U.S. Internal Revenue Code of 1986, as amended, or the Code. The Tax Act, among other things, contained significant changes to corporate taxation. As part of Congress's response to the COVID-19 pandemic, economic relief was enacted in 2020 and 2021. Such legislation contains numerous tax provisions. In addition, the IRA was signed into law in August 2022. The IRA introduced new tax provisions, including a 1% excise tax imposed on certain stock repurchases by publicly traded corporations. The 1% excise tax generally applies to any acquisition by the publicly traded corporation (or certain of its affiliates) of stock of the publicly traded corporation in exchange for money or other property (other than stock of the corporation itself), subject to a *de minimis* exception. Thus, the excise tax could apply to certain transactions that are not traditional stock repurchases.

Regulatory guidance under the 2017 Tax Act, the IRA, and such additional legislation is and continues to be forthcoming, and such guidance could ultimately increase or lessen the impact of these laws on our business and financial condition. In addition, it is uncertain if and to what extent various states will confirm the IRA, the Tax Act, and additional tax legislation.

Regulatory guidance under the 2017 Tax Act, which was enacted on December 22, 2017, the FFCR Act, the CARES Act, the CAA, and the ARPA is and continues to be forthcoming, and such guidance could ultimately increase or lessen the impact of these laws on our business and financial condition. It is also possible that Congress will enact additional legislation in connection with the COVID-19 pandemic, and as a result of the changes in the U.S. presidential administration and control of the U.S. Senate, additional tax legislation may also be enacted.

Although we monitor actual and potential changes to the tax laws in the United States and other jurisdictions, it is very difficult to assess to what extent these changes may impact the way in which we conduct our business or our effective tax rate due to the unpredictability and interdependency of these changes. Changes in tax laws and related regulations and practices could have a material adverse effect on our business operations, cash flows, effective tax rate, financial position and results of operations.

Risks Related to Regulatory Approval of our Products and our Product Candidates

Our marketing authorization in the EEA for Translarna for the treatment of nmDMD is a "conditional marketing authorization" that requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, and, as such, there is ongoing risk that we may be unable to maintain such authorization. If we are unable to obtain renewal of such marketing authorization in any future renewal cycle, we could lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program, which would have a material adverse effect on our business, financial performance and results of operations.

Our marketing authorization in the EEA for Translarna for the treatment of nmDMD is a "conditional marketing authorization" that requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is further conditioned upon the conduct of Study 041. We received initial marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged five years and older from the European Commission in August 2014 as a "conditional marketing authorization." In July 2018, the European Commission approved a label-extension request to our marketing authorization for Translarna in the EEA to include patients from two to up to five years of age. In July 2020, the European Commission approved the removal of the statement "efficacy has not been demonstrated in non-ambulatory patients" from the indication statement for Translarna. The marketing authorization is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Within the placebo-controlled trial, Translarna showed a statistically significant treatment

benefit across the entire intent to treat population as assessed by the 6-minute walk test, assessing ambulation and endurance, and in lower-limb muscle function as assessed by the North Star Ambulatory Assessment, a functional scale designed for boys affected by DMD. Additionally, Translarna showed a statistically significant treatment benefit across the intent to treat population within the 10-meter run/walk and 4-stair stair climb, each assessing ambulation and burst activity, while also showing a positive trend in the 4-stair stair descend although not statistically significant. Within the primary analysis group, Translarna demonstrated a positive trend across all endpoints, however, statistical significance was not achieved. Translarna was also well tolerated. In September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. We expect an opinion from the Committee for Medicinal Products for Human Use in the first half of 2023. Given that statistical significance was not achieved in the placebo-controlled portion of Study 041 within the primary analysis group, the EMA may deny our request for conversion and our annual reassessment by the EMA of the benefit-risk balance of the authorization may be negatively impacted as well.

We may also still experience unknown complications with open-label extension period Study 041, which would have a materially adverse effect on our ability to maintain our marketing authorization in the EEA. We are further required to implement measures, including pharmacovigilance plans, which are detailed in the risk management plan.

If we fail to satisfy our obligations under the marketing authorization, or if it is determined in any annual renewal cycle that the balance of benefits and risks of using Translarna has changed materially, the European Commission could, at the EMA's recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna. The EMA may also impose other new conditions to our marketing authorization (in addition to Study 041), and may make other recommendations, including new label restrictions. In the event that we do secure annual renewal of the marketing authorization for any given annual renewal cycle, the EMA could nevertheless later determine that we have not complied, or are unable to comply, with any conditions that have been or may be placed on the marketing authorization, including those related to Study 041, which could result in the withdrawal of our marketing authorization or other outcome that would have a materially adverse effect on our business, results of operations and financial condition.

If our marketing authorization in the EEA is not renewed, or our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna, whether pursuant to a commercial or an EAP program and throughout all territories, which would have a material adverse effect on our business, results of operations and financial condition.

We may not be able to obtain orphan drug exclusivity for our products or product candidates in either the United States or the EU.

Regulatory authorities in some jurisdictions, including the EU and the United States, may designate drugs for relatively small patient populations as orphan drugs. We have obtained orphan drug designations from the EMA and from the FDA for Translarna for the treatment of nmDMD, Upstaza for the treatment of AADC, Evrysdi for the treatment of SMA, PTC-AS for the treatment of Angelman syndrome, sepiapterin for the treatment of patients with hyperphenylalaninemia, including hyperphenylalaninemia caused by PKU, vatiquinone for the treatment of Friedreich ataxia, utreloxastat for the treatment of ALS and unesbulin for the treatment of LMS. The FDA has also granted an orphan drug designation to Emflaza for the treatment of DMD, vatiquinone for the treatment of seizures in patients with mitochondrial disease, unesbulin for the treatment of DIPG and PTC-FA for the treatment of Friedreich ataxia. We may also seek orphan drug exclusivity for other product candidates, if we believe that the product candidate may qualify. We, however, may not be able to obtain orphan drug designation in the future for any of our other product candidates. Obtaining orphan drug exclusivity, both in the EU and in the United States, may be important to a product candidate's future success.

In the EU, if an orphan designated product subsequently receives the first marketing authorization for the indication for which it has received such a designation, the product is entitled to 10 years of market exclusivity, which, subject to certain exceptions, precludes the EMA from accepting another marketing application for a similar medicinal product, even if the new marketing application relies on independently generated data submitted as part of a full marketing authorization application dossier. The EU exclusivity period can be reduced to six years, at the end of the fifth year, if a drug no longer meets the criteria for orphan drug designation, including if the drug is sufficiently profitable so that market exclusivity is

no longer justified. In addition, a competing similar medicinal product may in limited circumstances be authorized prior to the expiration of the market exclusivity period, including if it is shown to be safer, more effective or otherwise clinically superior to the orphan product. In this context, a "similar medicinal product" is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. Product candidates can also lose orphan designation, and the related benefits, prior to obtaining a marketing authorization if it is demonstrated that the orphan designation criteria are no longer met.

In the United States, under FDA's current policy, if a product with an orphan drug designation subsequently receives the first marketing authorization for the indication for which it has such designation, the product is entitled to seven years of market exclusivity which precludes the FDA from approving another marketing application for the "same drug" for the same orphan designated approved indication for that time period. When determining whether a drug is the "same drug" as an orphan designated product, the FDA looks to the products' molecular features and use. The specific sameness criteria, however, varies based on whether the product is composed of small or large molecules and if the product is a gene therapy. Moreover, for gene therapies, the sameness criteria is currently evolving. For example, the FDA recently issued a final guidance document specific to sameness determinations. Depending on product characteristics, sameness may be determined by the FDA on a case by case basis, making it difficult to predict when FDA may approve a product and whether periods of exclusivity will effectively block competitors seeking to market products that are the same or similar to ours for the same intended use. Moreover, following the Catalyst Pharms., Inc. v. Becerra and FDA's subsequent statement that it intends to continue to apply its regulations tying the scope of orphan-drug exclusivity to the uses or indications for which a drug is approved, as further described in this filing, the exact scope of orphan drug exclusivity may be an evolving space. Accordingly, whether any of our products or product candidates will be deemed to be the same as another product or product candidate is uncertain and the scope of any potential or received orphan drug exclusivity period may be subject to revision.

Obtaining orphan drug designation does not guarantee that we will be able to receive ultimate marketing approval. Orphan drug designation neither shortens the development time or regulatory review time of a product candidate nor gives the product candidate any advantage in the regulatory review or approval process. Moreover, the FDA may grant orphan drug designation to multiple products that are considered to be the "same drug" for the same indication. If a competitor obtains an orphan drug designation for and approval of a product with orphan drug exclusivity for the same indication as one of our product candidates before we do and if the competitor's product is the same drug, in the United States or a similar medicinal product, in the EU, as ours, we could be excluded from the market for a period of time.

We also may not be able to maintain any orphan drug designations or exclusivities. For instance, orphan drug designations may be revoked if the FDA finds that the request for designation contained an untrue statement of material fact or omitted material information, or if the FDA finds that the product candidate was not eligible for designation at the time of the submission of the request. Even if we are able to receive and maintain orphan drug designations, we may ultimately not receive any period of regulatory exclusivity if our product candidates are approved. For instance, we may not receive orphan product regulatory exclusivity if the indication for which we receive FDA approval is broader than the orphan drug designation. Orphan exclusivity may also be lost for the same reasons that designation may be lost. Orphan exclusivity may further be lost if we are unable to assure a sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

Further, even if we do receive orphan drug exclusivity upon approval of a product candidate, this exclusivity is not absolute. For example, if a competitive product that is the same drug or a similar medicinal product as one of our approved products with orphan exclusivity is shown to be "clinically superior" to our product candidate as determined by the FDA or EMA, respectively, any orphan drug exclusivity we have obtained will not block the approval of such competitive product. Orphan exclusivity also would not block FDA from approving a drug that is the same as our product candidates for different indications or products that are different from ours for the same indication. Moreover, marketing exclusivity would not prevent a provider from prescribing or using another drug off-label and third-party payors may reimburse for products off-label even if not indicated for the orphan condition.

For certain of our products, periods of orphan drug exclusivity are important. For instance, for Emflaza, we rely on non-patent market exclusivity periods under the Orphan Drug Act to commercialize Emflaza in the United States. Emflaza's seven-year period of orphan drug exclusivity related to the treatment of DMD in patients five years and older expires in

February 2024 while its orphan drug exclusivity related to the treatment of DMD in patients two years of age to less than five expires in June 2026. If we are not able to maintain orphan drug exclusivity for Emflaza or if another company is able to overcome this exclusivity, we may be materially harmed.

The respective orphan designation and exclusivity frameworks in the United States and in the EU are subject to change, and any such changes may affect our ability to obtain, or the impact of obtaining, EU or United States orphan designations in the future.

All pharmaceutical products for which marketing authorization has been granted are subject to extensive and rigorous governmental regulation and could be subject to restrictions or withdrawal from the market. We may also be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved, as well as our product candidates during development.

We, our products and product candidates, our operations, our facilities, our suppliers and our contract manufacturers, distributors, contract research organizations, clinical trial sites and contract testing laboratories are subject to extensive regulation by governmental authorities in the EEA, the United States, and other territories, with regulations differing from country to country.

We are not permitted to market our product candidates in the EEA, the United States, or other territories until we have received requisite regulatory approvals. In order to receive and maintain such approvals, and to be compliant with regulatory authority requirements, we and our third-party service providers must comply on a continuous basis with a broad array of regulations and requirements. Depending on the stage of product development and whether a product is approved these requirements may relate to establishment registration and product listing, the payment of user fees, manufacturing processes, risk management measures, quality and pharmacovigilance systems (including reporting of manufacturing deviations and adverse events), pre- and post-approval clinical and pre-clinical data, labeling, packaging, advertising, marketing and promotional activities (including product sampling), record keeping, distribution, storage, and import and export of pharmaceutical products. Any regulatory approval of any of our products or product candidates, once obtained, may be withdrawn. For example, our marketing authorization for Translarna for the treatment of nmDMD in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, as well as the specific obligation to conduct and report the results of Study 041.

After approving a drug, the FDA may withdraw product approval if compliance with regulatory standards is not maintained or if safety problems occur after the product reaches the market. Requirements for additional clinical trials and studies to confirm safety and effectiveness may be imposed as a condition of marketing approval. In addition, the FDA requires surveillance programs to monitor approved products that have been commercialized, as well as REMS, and the agency has the power to require changes in labeling or to prevent further marketing and distribution of a product. For example, we were obligated to perform certain FDA post-marketing requirements in connection with our marketing authorization for Emflaza in the United States, including pre-clinical and clinical safety studies. Additionally, our marketing authorizations for Translarna, Tegsedi and Waylivra in Brazil and our marketing authorization for Translarna in Russia are subject to renewal every five years. There is no guarantee that we will be able to complete our post-marketing obligations in accordance with the established timetables. Failure to complete the required studies in accordance with the established timetables or failure to provide the requisite periodic reports on the status of post-marketing studies in the absence of good cause could result in an enforcement action. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing and distribution.

Regulatory authorities conduct ongoing reviews and inspections or remote regulatory assessments of marketed products, as well as sponsors and manufacturing facilities. Regulatory authorities also conduct inspections of manufacturing facilities and clinical trial sites before approving a product, which can delay approval. If compliance issues are found, it could also result in refusal to approve marketing applications, disruption of production or distribution of a product or product candidate, disruption, cancellation, or suspension of a study, or require substantial resources to correct.

Even if marketing authorization of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed, the product may have labeling that includes significant restrictions, warnings, including black box warnings, and contraindications, the regulatory authorities may not approve label claims

necessary for successful product marketing, or the approval may be subject to significant conditions of approval, including the requirement of a REMS. A regulatory authority also may impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. In addition, the competent authorities of each EU member state and the FDA closely regulate the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling and regulatory requirements. Such regulatory authorities can and do impose stringent restrictions on our communications regarding off-label use and if we do not comply with the laws governing promotion of approved drugs, we may be subject to enforcement action for off-label promotion. For example, violations of the FDCA relating to the promotion of prescription drugs may lead to civil and criminal penalties, investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, both before and after product approval, may yield various results which could negatively affect our business, including:

- restrictions on such products, manufacturers or manufacturing processes;
- changes to or restrictions on the labeling or marketing of a product;
- modifications to promotional pieces;
- issuance of corrective information;
- clinical holds or termination of clinical trials;
- changes in the way a product is administered;
- liability for harm caused to patients or subjects;
- adverse publicity, reputational harm, or the product becoming less competitive;
- regulatory authority issuance of safety alerts, Dear Healthcare Provider letters, press releases, or other communications containing warnings or other safety information about the product;
- restrictions on product distribution or use;
- requirements to implement a REMS;
- requirements to conduct post-marketing studies or clinical trials;
- warning, cyber or untitled letters;
- withdrawal of the products from the market or marketing suspensions;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing authorizations;
- refusal to permit the import or export of our products;
- product seizure or detention;
- injunctions;
- the imposition of civil or criminal penalties; or
- FDA debarment, suspension and debarment from government contracts, and refusal of orders under existing government contracts, exclusion from federal healthcare programs, consent decrees, or corporate integrity agreements.

Non-compliance with regulatory requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with regulatory requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Not only will we be responsible for our own conduct, but we will also be responsible for the conduct of our employees, independent contractors, consultants, commercial partners, manufacturers, investigators, and contract research organizations. To the extent that any of these third parties engage in intentional, reckless, negligent, or unintentional failures to comply applicable legal and regulatory requirements, we may be subject to regulatory enforcement action, legal actions and liability, and serious harm to our reputation. Moreover, it is possible for a whistleblower to pursue a False

Claims Act case against us as a result of such third party conduct, even if the government considers the claim unmeritorious and declines to intervene, which could require us to incur costs defending against such a claim.

Any of the above events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, or could substantially increase the costs and expenses of developing and commercializing such product, which in turn could delay or prevent us from generating significant revenues from its sale. Any of these events could further have other material and adverse effects on our operations and business and could adversely impact our stock price and could significantly harm our business, financial condition, results of operations, and prospects.

We may face competition from biosimilar, generic, and similar products approved through abbreviated pathways, as well as products approved pursuant to full applications.

Our approved products may face competition from products approved via abbreviated pathways as well as products approved pursuant to full applications. For example, our biologic products may face competition from biosimilar or interchangeable products. Sponsors seeking approval of biosimilar or interchangeable products to ours would reference our product in their applications. The applicable laws, however, establish certain protections for reference biologic products. For example, there is a complex and involved framework for sponsors to bring patent infringement actions and actions for declaratory judgment. Accordingly, we may need to pursue costly and time-consuming patent infringement actions, which may include certain statutorily specified regulatory steps before an infringement action may be brought. We may also need to spend time and money defending an action for declaratory judgement that is brought by the biosimilar product sponsor.

Another protection established for biologic products is a period of 12 years of exclusivity for reference products that begins on the date that the reference product was first licensed by the FDA. During this time, the FDA may not make the licensure of a biosimilar product effective. Biosimilar applications can, however, be submitted for FDA review beginning four years after the date of the reference product's first licensure. This exclusivity period, however, is subject to certain limitations. For example, certain changes and supplements to an approved BLA, and certain subsequent applications filed by the same sponsor, manufacturer, licensor, predecessor in interest, or other related entity do not qualify for the 12-year exclusivity period. Moreover, there have been legislative efforts to decrease this period of exclusivity to a shorter timeframe. Future proposed budgets, international trade agreements and other arrangements or proposals may affect periods of exclusivity. Further, even if our biologic product candidates qualify for biologic exclusivity, there is a risk that the FDA will not consider our product candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Additionally, this period of regulatory exclusivity does not apply to companies pursuing regulatory approval via their own traditional BLA, rather than via the abbreviated pathway. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet fully clear, and will depend on a number of marketplace and regulatory factors that are still developing. It is also possible that payers will give reimbursement preference to biosimilars, even over reference biologics, absent a determination of interchangeability. Similarly, in the EU, another company could gain approval for a competing product based on an MAA with a completely independent data package that includes pharmaceutical tests, preclinical tests and clinical trials.

For small molecule drug products, a pharmaceutical manufacturer may file an abbreviated new drug application, or ANDA, seeking approval of a generic copy of one of our approved products. A manufacturer could also submit an NDA under section 505(b)(2), referencing the FDA's finding of safety and efficacy for one of our drug products, while also conducting its own studies to support any product changes. Any ANDA or 505(b)(2) NDA products referencing our approved products would be required to submit patent certifications to the FDA. Unless the applicant does not seek approval until any of our Orange Book listed patents expire or, to the extent possible, carve out any of our Orange Book listed method of use patents, such an applicant would be required to submit what is known as a "Paragraph IV certification," challenging the validity or enforceability of, or claiming non-infringement of, the listed patent or patents. This would provide us with an opportunity to sue to enforce our patents, which would stay any FDA approval for 30 months from the patent or application owner's receipt of the notice of the paragraph IV certification, the expiration of the patent, when the infringement case concerning each such patent is favorably decided in the applicant's favor or settled, or such shorter or longer period as may be ordered by a court. While this would delay the approval of the generic or 505(b)(2) product, such actions would require significant time and cost.

Our small molecule drug products may also be eligible for certain periods of regulatory exclusivity (e.g., five years for new chemical entities, three years for changes to an approved drug requiring a new clinical study, and seven years for orphan drugs), which preclude FDA approval (or in some circumstances, FDA filing and review of) an ANDA or 505(b) (2) NDA relying on the FDA's finding of safety and effectiveness for the innovative drug. These exclusivities, however, are also subject to certain limitations. For instance, they would not block FDA acceptance and approval of full NDA applications.

Even with the various protections in place, we may not be successful in securing or maintaining proprietary patent protection for our products and product candidates necessary to prevail should we need to bring any challenges under the above FDA regulatory structures. We may also not receive any anticipated periods of regulatory exclusivity. Competition that our products may face from biosimilar, interchangeable, generic, or 505(b)(2) NDA products could materially and adversely impact our future revenue, profitability, and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates. In the United States, this risk has increased in recent years as the FDA and the U.S. government have taken steps to encourage increased drug and biologic competition in the market, in an effort to bring down the cost of pharmaceutical products.

Commercialization of Translarna and Upstaza has been in, and is expected to continue to take place in, countries that tend to impose strict price controls, which may adversely affect our revenues. Failure to obtain and maintain acceptable pricing and reimbursement terms for Translarna for the treatment of nmDMD or Upstaza for the treatment of AADC deficiency in the EEA and other countries where Translarna is available would delay or prevent us from marketing our product in such regions, which would adversely affect our business, results of operations, and financial condition.

In some countries, particularly the member states of the EEA, the pricing of prescription pharmaceuticals is subject to strict governmental control. Each country in the EEA has its own pricing and reimbursement regulations and may have other regulations related to the marketing and sale of pharmaceutical products in the country. We generally will not be able to commence commercial sales of Translarna for the treatment of nmDMD or Upstaza for the treatment of AADC deficiency pursuant to the marketing authorization granted by the European Commission in any particular member state of the EEA until we conclude the applicable pricing and reimbursement negotiations and comply with any licensing, employment or related regulatory requirements in that country. In some countries we may be required to conduct additional clinical trials or other studies of our product, including trials that compare the cost-effectiveness of our product to other available therapies in order to obtain reimbursement or pricing approval. We may not be able to conclude pricing and reimbursement negotiations or comply with additional regulatory requirements in the countries in which we seek to commercialize Translarna or Upstaza on a timely basis, or at all.

The pricing and reimbursement process varies from country to country and can take a substantial amount of time from initiation to completion. Pricing negotiations may continue after reimbursement has been obtained. We cannot predict the timing of Translarna's or Upstaza's commercial launch in countries where we are awaiting pricing and reimbursement guidelines. While we have submitted pricing and reimbursement dossiers with respect to Translarna for the treatment of nmDMD and Upstaza for the treatment of AADC deficiency in many EEA countries, we have only received both pricing and reimbursement approval on terms that are acceptable to us in a limited number of countries.

The price that is approved by governmental authorities in any country pursuant to commercial pricing and reimbursement processes may be significantly lower than the price we are able to charge for sales under our reimbursed EAP programs and various forms of national "market access agreements" may need to be entered into to achieve reimbursement. In some instances, reimbursement may be subject to challenge, reduction or denial by the government and other payors.

For example, in France, EAP and commercial sales of a product can begin while pricing and reimbursement rates are under discussion with the applicable government health programs. In the event that the negotiated price of the product is lower than the amount reimbursed for sales made prior to the conclusion of price negotiations, we may become obligated to repay such excess amount to the applicable government health program. We will make such retroactive reimbursement, if any, following the conclusion of price negotiations with the applicable government health authority.

Further, based on unsustainable economics imposed by the arbitration board in Germany upon the conclusion of an arbitration process in 2016 with us and the German Federal Association of the Statutory Health Insurances, we delisted

Translarna from the German pharmacy ordering system, effective April 1, 2016. While some patients and healthcare professionals in Germany have been able to access Translarna through a reimbursed importation pathway possible under German law, there can be no assurance that other patients or healthcare professionals in Germany will be successful doing so or, if initially successful, that any or all will continue to be successful. We were required to reimburse payors in Germany the difference between the commercial price of Translarna and the price established by the arbitration board in Germany for sales made in Germany after December 2015, other than sales made pursuant to the reimbursed importation pathway.

Political, economic and regulatory developments may further complicate pricing and reimbursement negotiations and there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. In addition, adverse clinical and regulatory developments may exacerbate these risks.

Reference pricing used by various EU member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices and revenues. Publication of discounts by third-party payors or authorities may lead to further pressure on prices or reimbursement levels within the country of publication and other countries.

If we fail to successfully secure and maintain pricing and reimbursement coverage for Translarna or Upstaza or are significantly delayed in doing so or if burdensome conditions are imposed by private payers, government authorities or other third-party payors on such reimbursement, planned launches in the affected countries will be delayed and our business, results of operations and financial condition could be adversely affected.

Our relationships with customers, healthcare providers and professionals, patients, patient organizations, and third-party payors are or will be subject to applicable anti-kickback, fraud and abuse, transparency and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare professionals and third-party payors play a primary role in the recommendation and prescription of any products or product candidates. Our arrangements with customers, healthcare professionals and third-party payors may expose us to broadly applicable fraud and abuse, transparency and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing authorization. Failure to maintain a comprehensive and effective compliance program, and to integrate the operations of any acquired businesses into a combined comprehensive and effective compliance program on a timely basis, could result in business practices and operations that expose us to a range of regulatory actions that could adversely affect our ability to commercialize our products and could harm or prevent sales of the affected products, or could substantially increase the costs and expenses of commercializing and marketing our products.

There are numerous restrictions and reporting requirements under applicable U.S. federal and state healthcare laws and regulations, and equivalent laws and regulations in the EU and other countries in which we operate, as well as self-regulatory codes. Efforts to ensure that we and our business arrangements with third parties will comply with applicable healthcare laws, regulations, transparency requirements and self-regulatory codes have and will continue to involve substantial costs. We cannot guarantee that we, our employees, our consultants, our third-party contractors, or the healthcare professionals or entities with whom we expect to do business, are or will be in compliance with all federal, state and ex-U.S. regulations and codes. It is possible that governmental authorities could conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, reputational harm, and the curtailment or restructuring of our operations. Exclusion, suspension and debarment from government funded healthcare, procurement and non-procurement programs would adversely affect, perhaps materially, our ability to commercialize, sell or distribute any drug. Even if we were not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant time and resources and generate negative publicity, which could also have an adverse effect on our business, financial condition and results of operations.

Legislative and regulatory changes affecting the pharmaceutical industry or the healthcare system more broadly may increase the difficulty and cost for us to obtain or maintain marketing authorization of and commercialize our products and product candidates and affect the coverage and reimbursement we may obtain.

Our industry is highly regulated and changes in law may adversely impact our business, operations, or financial results. In the United States and some ex-U.S. jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing authorization of our products or product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any products or product candidates for which we have obtained, or may obtain, marketing authorization.

Certain provisions of enacted or proposed legislative changes may negatively impact coverage and reimbursement of healthcare items and services. For example, in the United States, the Medicare Modernization Act requires manufacturers to calculate and report a drug's Average Sales Price used to reimburse providers for physician-administered drugs under Medicare Part B and changed the way Medicare covers and pays for pharmaceutical products. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and reimbursement that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own policies. Therefore, any restrictions to coverage or reductions in reimbursement that result from the Medicare Modernization Act may result in a similar coverage restriction or reimbursement reduction from private payors. In addition, private payors may implement coverage restrictions or payment reductions independently from federal programs such as Medicare.

Similarly, in the United States, the Affordable Care Act contains provisions that may reduce the profitability of drug products. However, legal challenges to the Affordable Care Act may contribute to the uncertainty of the ongoing implementation and impact of the Affordable Care Act and also underscore the potential for additional reform going forward. The Biden administration is expected to continue to take measures to further facilitate the implementation of the Affordable Care Act. We cannot assure that the Affordable Care Act, as currently enacted or as amended in the future, will not adversely affect our business and financial results.

Promulgated and proposed regulatory changes could also affect coverage or reimbursement of our products and in 2016, CMS issued a final rule regarding the Medicaid drug rebate program, which among other things, revises the manner in which the "average manufacturer price" is to be calculated by manufacturers participating in the program and implements certain amendments to the Medicaid rebate statute created under the ACA. More recently, Congress amended the Medicaid statute, effective October 1, 2019, to exclude prices paid by secondary manufacturers for an authorized generic drug (but not a product approved under the BLA process) from the NDA holder's AMP for the brand, thereby increasing the rebate amount and the 340B price for the brand. This was implemented by CMS in a final rule issued December 31, 2020. The rule also expanded the definition of products identified as "line extensions" and, in certain circumstances, required inclusion of patient copay assistance in Medicaid best price (effective January 1, 2023), thereby potentially increasing Medicaid rebates paid by manufacturers for such drugs. 340B program guidance regulations on civil monetary penalties for statutory violations, which had been finalized in early 2017 but deferred, recently also went into effect.

In 2020, the Trump administration issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, CMS issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

More recently, on August 16, 2022, the IRA was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program

with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Nonetheless, since CMS may establish a maximum price for these products in price negotiations, we would be fully at risk of government action if our products are the subject of Medicare price negotiations. Moreover, given the risk that could be the case, these provisions of the IRA may also further heighten the risk that we would not be able to achieve the expected return on our drug products or full value of our patents protecting our products if prices are set after such products have been on the market for nine years.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

We anticipate that the U.S. Congress, administrative agencies, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures may include:

- controls on government funded reimbursement for drugs;
- caps or mandatory discounts under certain government sponsored programs;
- controls on healthcare providers;
- challenges to the pricing of drugs or limits on reimbursement of specific products through other means;
- reform of drug importation laws and policies;
- expansion of use of managed care systems in which the healthcare providers contract to provide comprehensive healthcare for a fixed cost per person; and
- requirements or restrictions related to direct-to-consumer advertising or drug marketing practices.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third-party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. In particular, we are unable to predict what changes the Biden administration will implement through the U.S. Congress or future executive orders and how these would impact us. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted, could significantly decrease the available coverage and the price we might establish for our products, which would have an adverse effect on our net revenues and operating results. Changes in FDA laws, regulations, and policies may also make it more difficult to obtain and maintain marketing authorizations.

In the EU, similar political, economic and regulatory developments may affect our ability to profitably commercialize Translarna, Upstaza and our product candidates. In addition to continuing pressure on prices and cost containment measures, legislative developments at the EU or member state level may result in significant additional requirements or obstacles that may increase our operating costs. We cannot predict how future changes relating to healthcare reform in the EU, the United States, or other territories, will affect our business.

Legislative and regulatory proposals have also been made to expand post-approval requirements, limit regulatory exclusivity periods or the applicability of such exclusivity periods, restrict sales and promotional activities for pharmaceutical products and to otherwise encourage competition in the market and bring down drug prices, including proposals related to drug importation. We cannot be sure whether additional legislative or regulatory changes will be enacted in any territory in which we are authorized, or become authorized, to market our products or product candidates,

or whether applicable regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing authorizations of our products or product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process or by comparable ex-U.S. bodies overseeing regulatory authorities in other territories may significantly delay or prevent marketing authorization, as well as subject us to more stringent product labeling and post-marketing testing and other requirements. We cannot predict how future changes relating to pre- and post-marketing approval and requirements will affect our business.

Risks Related to Our Business

We may expend our resources to pursue a particular product, product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

We focus on products, research programs and product candidates for specific indications. As a result, we may forgo or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential.

For example, in connection with our acquisition of Censa, we paid to the Censa securityholders (i) cash consideration of $15.0 million, which consisted of an upfront payment of $10.4 million and an additional $4.6 million for the net assets on Censa's opening balance sheet as of the date of the acquisition, and (ii) 845,364 shares of our common stock. Censa securityholders may also be entitled to receive contingent consideration payments from us in the future. We may never realize the anticipated benefits of the acquisition of Censa and by investing our resources in sepiapterin, we may be required to forgo or delay other opportunities.

In addition, we have previously commenced clinical trials that were not successful for a number of reasons, including inconsistent or negative data and difficulties identifying qualified patients. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products.

Notwithstanding our large investments to date and anticipated future expenditures in proprietary technologies for both small-molecule and gene therapy drug discovery, to date we have been granted marketing authorization for a limited number of commercial products and have not achieved profitability. We may never realize a return on investment. We may not be able to successfully renew or satisfy the ongoing requirements of our current marketing authorizations for our current products and we may never successfully develop any other marketable drugs or indications using our scientific approach. As a result of pursuing the development of product candidates using our proprietary technologies, we may fail to develop product candidates or address indications based on other scientific approaches that may offer greater commercial potential or for which there is a greater likelihood of success. Research programs to identify new product candidates require substantial technical, financial and human resources. These research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development.

If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We face risks related to health epidemics and other widespread outbreaks of contagious disease, which have previously, and may once again, delay our ability to complete our ongoing clinical trials and initiate future clinical trials, disrupt regulatory activities and have other adverse effects on our business and operations, including the novel coronavirus (COVID-19) pandemic, which disrupted, and may continue to disrupt, our operations and may significantly impact our operating results. In addition, the COVID-19 pandemic has caused substantial disruption in the financial markets and economies, which could result in adverse effects on our business and operations.

Significant outbreaks of contagious diseases, and other adverse public health developments, could have a material impact on our business operations and operating results. In December 2019, a strain of novel coronavirus, COVID-19, causing

respiratory illness emerged in the city of Wuhan in the Hubei province of China. Since that time, multiple other countries throughout the world, including the United States, have been affected by the spread of the virus. To date, responsive measures such as social distancing, vaccine mandates, travel bans and quarantines have been put into place in many countries throughout the world, including the United States. These responsive measures have had a significant impact, both direct and indirect, on business and commerce worldwide, as worker shortages have occurred, supply chains have been disrupted and facilities and production have been suspended or curtailed.

The spread of COVID-19 and the responsive measures taken to date have limited our access to our facilities, the access of trial participants to clinical sites and, at one point in time, caused the majority of our employees to work from home. We continue to monitor the global spread and response of international, national and local authorities of COVID-19 and have put in place and will continue to put in place measures as appropriate and necessary for our business and the safety of our employees. While we expect the pandemic to continue to have an adverse effect on our business and operations, and the pandemic may have an adverse effect on our financial condition and results of operations, we are unable to predict the extent or nature of the future progression of the COVID-19 pandemic or its effects on our business, operations, financial condition and results of operations at this time.

Furthermore, we have clinical trial sites located in countries that have been affected by COVID-19 that have been and may continue to be disrupted, including the United States. The disruption of our clinical trial sites has had an adverse impact on our clinical trial plans and timelines. The COVID-19 pandemic has also adversely affected our ability to timely enroll patients for our clinical trials which may delay the completion of clinical trials. For example, we previously experienced delays in enrolling our registration-directed Phase 2/3 randomized, placebo-controlled trial of vatiquinone in children with mitochondrial disease associated seizures as some patients were unable or hesitant to travel to clinical trial sites due to the COVID-19 pandemic. We anticipate results from the Phase 2/3 trial to be available in the second quarter of 2023. Such disruptions could result in significant delays or could require us to abandon a clinical trial altogether. For additional information, see the risk factor under “Risks Related to the Development and Commercialization of our Products and our Product Candidates” titled, *“If we experience delays or difficulties in the enrollment of patients in our clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.”*

Our ability to market and promote our products, as well as patient demand for our products may also be impacted. Because access to healthcare providers and institutions has been limited in certain regions of the world, we have had to transition to virtual and online promotion to reach existing and potential customers in those areas. Healthcare provider and institution restrictions and closures, as well as patient reticence to visit their physicians may also result in a decrease in product prescribing.

Significant suppliers and manufacturing located in countries that have been affected by COVID-19 may also be disrupted, which may affect our ability to procure items that are essential for our research and development activities and may cause disruptions or delays in our sales and commercialization efforts of approved products and clinical trials with respect to product candidates. For example, in response to the COVID-19 pandemic, China has at times imposed complete lockdowns of cities that have experienced a high number of COVID-19 cases. We contract with third-party manufacturers located in China that may be forced to shut down for an unknown amount of time if the Chinese government determines that there is a COVID-19 outbreak where they are located. Additionally, we have experienced delays in certain of our preclinical programs due to a shortage in non-human primates. Many manufacturers have also experienced shortages of key equipment and ingredients needed for product manufacturing. The response to the COVID-19 pandemic may also redirect resources with respect to regulatory matters in a way that would adversely impact our ability to progress to regulatory approval. In response to the global uncertainty caused by the COVID-19 pandemic, we may also choose to redirect our own resources in a way that may adversely impact or delay certain of our programs.

Furthermore, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions. For example, due to delays related to responsive measures to the COVID-19 pandemic taken in Europe in 2021, including travel bans and quarantines, the CHMP required additional time to complete its pre-approval inspections and imposed a clock stop extension with respect to our MAA for the treatment of AADC deficiency in the EEA. To the extent that inspections of facilities by governmental authorities are required, the review of our marketing applications or supplements may further be delayed as regulatory authorities, such as FDA, have significantly limited facility inspections during the pandemic.

We cannot predict the severity and duration of future COVID-19 outbreaks or other widespread outbreaks of contagious diseases. If COVID-19 outbreaks or other outbreaks are not effectively and timely controlled, we may experience further or prolonged disruption of our clinical trials, third-party suppliers or contract manufacturers, extended closures of facilities, such as clinical trial sites, suppliers, manufacturers and distributors, including single source suppliers, and further delays with respect to regulatory approvals or the commercialization of any current or future products. Such events may materially and adversely affect our business operations and financial condition. Additionally, the COVID-19 pandemic has caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could impact our ability to raise additional funds and has also impacted, and may continue to impact, the volatility of our stock price and trading in our stock. Moreover, the COVID-19 pandemic has significantly impacted economies worldwide, which could result in adverse effects on our business and operations. We cannot be certain what the overall impact of the COVID-19 pandemic or other future potential outbreaks of contagious diseases will have on our business and their potential to materially adversely affect our business, financial condition, results of operations, and prospects.

We contract with third parties for the manufacture and distribution of our products and certain of our product candidates, which may increase the risk that we will not have sufficient quantities of our products or product candidates, such quantities may not meet the applicable regulatory quality standards, or such quantities at an acceptable cost, which could delay, prevent or impair our commercialization or development efforts. For certain of our product candidates, we may also directly engage in manufacturing, which will require significant expenditures and compliance with the FDA's manufacturing requirements.

We have limited personnel with experience in drug manufacturing and currently rely on third parties to manufacture our products and certain product candidates on a clinical or commercial scale. We currently rely on third parties for supply of the active pharmaceutical ingredients used in all of our products and product candidates. We outsource most of the manufacturing, packaging, labeling and distribution of our products and certain of our product candidates to third parties, including our commercial supply of Translarna, Emflaza and Upstaza. In 2021, we began cGMP manufacturing of clinical material at the Hopewell Facility for certain of our gene therapy product candidates. We also utilize the Hopewell Facility to produce plasmid DNA and AAV vectors for gene therapy applications for external customers. We still rely on third-party manufacturers to complete product testing for all of our gene therapy product candidates that we manufacture at the Hopewell Facility as well as to provide sufficient quantities of certain program materials that we have not yet transitioned to Hopewell. With respect to the Hopewell Facility, we are required to directly comply with the applicable regulatory authorities' manufacturing requirements and are subject to inspection in the same way that our contract manufacturers are. Utilizing our own manufacturing will require a significant continued investment and we may not be successful in maintaining our own manufacturing capacity, especially given the complexities of gene therapy manufacturing. For additional information, see the risk factor under "Risks Related to the Development and Commercialization of our Products and Product Candidates" titled, *"Certain of our products and product candidates, such as our gene therapies and other biologic product candidates, may be difficult to produce, presenting manufacturing challenges that may delay product development and regulatory approval."*

We do not directly control manufacturing for most of our products and product candidates and we are dependent on and will continue to be dependent on, our contract manufacturers for compliance with cGMP or good distribution practice, or GDP, or similar regulatory requirements outside the EU and the United States for manufacture of both active drug substances and finished drug products. Should we or our contract manufacturers fail to comply with these requirements, we and they could face significant regulatory and commercial consequences. For example, regulatory authorities routinely inspect manufacturing and other drug/biologic facilities. Our manufacturers and manufacturing facilities must also be approved by such regulatory authorities pursuant to inspections that will be conducted after we submit our marketing applications and will be subject to continuing regulatory authority inspections should we receive marketing approval. If we or our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the EU member state regulatory authorities, FDA, or other ex-U.S. regulatory agencies, we or they will not be able to secure and/or maintain regulatory approval for the manufacturing facilities, and we would not be able to secure and/or maintain, or may be delayed in securing regulatory approval of marketing applications or supplements for the applicable products or product candidates. In addition, we or third-party manufacturers or distributors may not be able to comply with generally accepted worker safety standards, cGMP, GDP or similar regulatory requirements outside the EU and the United States. Our failure, or the failure of our third-party manufacturers or distributors, over whom we have no direct control, to comply with applicable regulations could result in sanctions being

imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, clinical holds or termination of clinical studies, warning or untitled letters, regulatory communications warning the public about safety issues with a product, import or export refusals, license revocation, seizures, detentions, or recalls of product candidates or product, operating restrictions, criminal prosecutions or debarment, suits under the civil False Claims act, corporate integrity agreements, or consent decrees, any of which could significantly and adversely affect our reputation and supplies of our products or product candidates and our business, results of operations and financial condition could be materially adversely affected.

In addition, we have no direct control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. Furthermore, all of our contract manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes our manufacturers to regulatory risks for the production of such other materials and products. As a result, failure to meet the regulatory requirements for the production of those materials and products may generally affect the regulatory status of our contract manufacturers' facilities. If the FDA, EU member state regulatory authorities or a comparable ex-U.S. regulatory agency do not approve these or our facilities for the manufacture of our products or product candidates or if it withdraws its approval in the future, we may need to find alternative manufacturing facilities, which would negatively impact our ability to develop, obtain regulatory approval for or market our products or product candidates, if approved. There is also no guarantee that we would be able to find alternative manufacturing facilities or enter into agreements with alternative manufacturers on favorable terms. There may be limited manufacturers who would have the ability to manufacture our products and product candidates, especially our gene therapy product candidates, particularly as the pharmaceutical manufacturing industry becomes increasingly more consolidated. To the extent that we decide to manufacture our own clinical and commercial supply of Upstaza as an alternative source of supply, there is no guarantee that we will be able to cost-effectively produce sufficient quantities of our program materials. Moreover, any alternative manufacturers would need to be approved by the relevant regulatory authority, which approval is not guaranteed. We, accordingly, may not be able to make alternative manufacturing arrangements, which could adversely affect our products, product candidates, and our business, results of operations and financial condition. See "Item 1. Business—Manufacturing" for additional information regarding the manufacturing of our products and product candidates.

Even if we are able to establish and maintain arrangements with third-party manufacturers, distributors and other third parties, reliance on such third parties entails additional risks, including:

- reliance on the third party for regulatory compliance and quality assurance;
- the possible breach of the agreements by the third party;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how;
- the possibility of commercial supplies of our products not being distributed to commercial vendors or end users in a timely manner, resulting in lost sales;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions;
- the possibility of third-party resources not being devoted in the manner necessary to satisfy our requirements within the expected time frame;
- the possibility of third parties not providing us with accurate or timely information regarding their inventories, the number of patients who are using our products, or serious adverse events and/or product complaints regarding our products;
- the possibility of third parties being unable to satisfy their financial obligations to us or to others; and
- the possible termination or nonrenewal of a critical agreement by the third party at a time that is costly or inconvenient to us.

Many additional factors could cause production or distribution interruptions with the manufacture and distribution of any of our products and product candidates, including human error, natural disasters, labor disputes, acts of terrorism or war, equipment malfunctions, contamination, supply chain disruption, including disruptions caused by the COVID-19 pandemic, or raw material and component shortages. We have previously experienced delays in receiving certain raw materials in connection with supply chain disruptions caused by the COVID-19 pandemic, however, these delays did not affect or delay our manufacturing given our inventories for such materials at the time. If future supply chain disruptions

create prolonged delays, the supplies of our products or products candidates may be significantly and adversely affected and our business, results of operations and financial condition could be materially adversely affected.

Our products and product candidates and any other products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. In addition, changes in cGMP regulations could negatively impact our ability or the ability of our contract manufacturers to complete the manufacturing process of our products and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively.

If we or the third parties that we engage to manufacture product for our commercial sales, preclinical tests and clinical trials should, prior to the time that we have validated alternative providers, cease to continue to do so for any reason, we likely would experience delays in our ability to supply our products or product candidates to patients or in our ability to advance our clinical trials while we identify and qualify replacement suppliers and we may be unable to obtain replacement supplies on terms that are favorable to us. In addition, if we are not able to obtain adequate supplies of our products or product candidates or the drug substances used to manufacture them, we will lose commercial sales revenue and it will be more difficult for us to develop our product candidates and compete effectively.

In addition, to the extent that any contract manufactures that we engage develop proprietary manufacturing processes or procedures, should we need to change manufacturers, we may not be able to transfer know-how to a new manufacturer. In such a case, the new manufacturer would need to invest substantial time, money, and effort to develop its own processes and procedures, which would require regulatory authority approval.

Third parties might illegally distribute and sell counterfeit or unfit versions of our products that do not meet our rigorous manufacturing and testing standards. A patient who receives a counterfeit or unfit drug may be at risk for a number of dangerous health consequences. Our reputation and business could suffer harm as a result of counterfeit or unfit drugs sold under our brand name. In addition, thefts of inventory at warehouses, plants or while in-transit, which are not properly stored and which are sold through unauthorized channels, could adversely impact patient safety, our reputation and our business.

Our current and anticipated future dependence upon others for the manufacture and distribution of Translarna, Emflaza, Upstaza, Tegsedi, Waylivra and certain of our product candidates may adversely affect our business, financial condition, results of operations and limit our ability to grow including our ability to develop product candidates and commercialize our products that receive regulatory approval on a timely and competitive basis.

We rely on third parties to conduct our preclinical and clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We do not independently conduct preclinical or clinical trials for our products or product candidates. We rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to perform this function. While we have agreements governing the activities of such third parties, we have limited influence and control over their actual performance and activities. For instance, our third-party service providers are not our employees, and except for remedies available to us under our agreements with such third parties we cannot control whether or not they devote sufficient time and resources to our ongoing clinical, non-clinical, and preclinical programs. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our preclinical studies or clinical trials in accordance with regulatory requirements or our stated protocols, if they need to be replaced or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements or for other reasons, our trials may be repeated, extended, delayed, or terminated, we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates, we may not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates, or we or they may be subject to regulatory enforcement actions. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed. To the extent we are unable to successfully identify and manage the performance of third-party service providers in the future, our

business may be materially and adversely affected. Further, any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it will delay our product development activities.

Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as GCP for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. In addition, we will be required to report certain financial interests of our third-party investigators if these relationships exceed certain financial thresholds or meet other criteria. The FDA or comparable ex-U.S. regulatory authorities may question the integrity of the data from those clinical trials conducted by investigators who may have conflicts of interest. We must further ensure that our preclinical trials are conducted in accordance with good laboratory practices, or GLPs, as appropriate. Regulatory authorities enforce these requirements through periodic inspections or remote regulatory assessments of trial sponsors, clinical and preclinical investigators, and trial sites. Similar GCP and transparency requirements apply in the EU. Failure to comply with the applicable regulatory requirements, including with respect to clinical trials conducted outside the EU and United States, can also lead regulatory authorities to refuse to accept into account clinical trial data submitted as part of a marketing application, as well as other regulatory consequences, as further described above.

Furthermore, third parties that we rely on for our clinical development activities may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing authorizations for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Our product development costs will increase if we experience delays in testing or obtaining marketing authorizations.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing authorizations of our products or product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

We currently depend, and expect to continue to depend, on collaborations with third parties for the development and commercialization of some of our products and product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these products and product candidates.

For each of our product candidates, we plan to evaluate the merits of retaining commercialization rights for ourselves or entering into selective collaboration arrangements with leading pharmaceutical or biotechnology companies, such as our collaborations with Roche and the SMA Foundation, for our spinal muscular atrophy program, including Evrysdi. We have entered into arrangements with certain third parties to market or distribute Translarna for the treatment of nmDMD in certain countries and, as we continue to implement our commercialization plans for Translarna, we anticipate that we will engage additional third parties to perform these functions for us in other countries. We generally plan to seek collaborators for the development and commercialization of product candidates that have high anticipated development costs, are directed at indications for which a potential collaborator has a particular expertise, or involve markets that require a large sales and marketing organization to serve effectively. Our likely collaborators for any marketing, distribution, development, licensing or broader collaboration arrangements may include: large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and/or biotechnology companies.

We will have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates and our collaborators will be subject to the same product development and commercialization risks that we are subject to. Our ability to generate revenues from these arrangements will depend on our collaborators' desire and ability to successfully perform the functions assigned to them in these arrangements. In particular, the commercial success of Evrysdi will depend on the success of Roche's commercialization program.

Furthermore, the successful development of another product candidate from our spinal muscular atrophy program will depend on the success of our collaborations with the SMA Foundation and Roche, including whether Roche pursues clinical development of any other compounds identified under the collaborations.

Collaborations involving our products and product candidates, including our collaborations with the SMA Foundation and Roche, pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of our products and product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that replace or compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators may fail to comply with the applicable regulatory requirements, subjecting them or us to potential regulatory enforcement action;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborator and us as to the ownership of intellectual property arising during the collaboration;
- we may grant exclusive rights for our products or product candidates to our collaborators, which would prevent us from collaborating with others, or from using our products or product candidates ourselves;
- disputes may arise between the collaborators and us that result in the delay or termination of the collaboration, which may include ending research, development or commercialization activities for our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

We may rely on third parties to perform many essential services for any products that we commercialize, including services related to warehousing and inventory control, distribution, government price reporting, customer service, accounts receivable management, cash collection, and pharmacovigilance and adverse event reporting. If these third parties fail to perform as expected or to comply with legal and regulatory requirements, our ability to commercialize our product candidates will be significantly impacted and we may be subject to regulatory sanctions.

We may retain third-party service providers to perform a variety of functions related to the sale and distribution of our product candidates, key aspects of which will be out of our direct control. These service providers may provide key services related to warehousing and inventory control, distribution, customer service, accounts receivable management, and cash collection. If we retain a service provider, we will substantially rely on it as well as other third-party providers that perform

services for us, including entrusting our inventories of products to their care and handling. If these third-party service providers fail to comply with applicable laws and regulations, fail to meet expected deadlines, or otherwise do not carry out their contractual duties to us, or encounter physical or natural damage at their facilities, our ability to deliver product to meet commercial demand would be significantly impaired and we may be subject to regulatory enforcement action.

In addition, we may engage third parties to perform various other services for us relating to pharmacovigilance and adverse event reporting, safety database management, fulfillment of requests for medical information regarding our product candidates and related services. If the quality or accuracy of the data maintained by these service providers is insufficient, or these third parties otherwise fail to comply with regulatory requirements, we could be subject to regulatory sanctions.

Additionally, we may contract with a third party to calculate and report pricing information mandated by various government programs. If a third party fails to timely report or adjust prices as required, or errors in calculating government pricing information from transactional data in our financial records, it could impact our discount and rebate liability, and potentially subject us to regulatory sanctions or False Claims Act lawsuits.

Our business and operations would suffer in the event of computer system failures, cyber-attacks or a deficiency in our, or our collaborators' or third-party vendors', cyber-security.

We collect, store and transmit large amounts of confidential information, including personal information, operational and financial transactions and records, clinical trial data and information relating to intellectual property, on internal information systems and through the information systems of collaborators and third-party vendors with whom we contract. Despite our implementation of security measures, including implementing the National Institute of Standards and Technology cybersecurity framework, instituting a training and compliance program on cybersecurity for all employees and doing a yearly external audit and penetration test, these information systems are vulnerable to damage from computer viruses, malware, natural disasters, terrorism, war, telecommunication and electrical failures, cyber-attacks or cyber-intrusions over the Internet or other mechanisms, attachments to emails, persons inside our organization, or persons with access to systems inside our organization. No such security measures can eliminate the possibility of the information systems' improper functioning or the improper access or disclosure of confidential or personally identifiable information such as in the event of cyber-attacks. The risk of a security breach or disruption, particularly through cyber-attacks or cyber-intrusion, including by computer hackers, criminals, ex-U.S. governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. Additionally, outside parties may attempt to fraudulently induce employees, collaborators, or other third-party vendors to disclose sensitive information or take other actions, including making fraudulent payments or downloading malware, by using "spoofing" and "phishing" emails or other types of attacks. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our clinical and commercialization activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy, despite our having a security risk insurance policy and disaster recovery and incident response plans. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur material legal claims and liability, damage to our reputation, suffer loss or harm to our intellectual property rights, face significant financial exposure, including incurring significant costs to remediate possible injury to the affected parties and the further research, development and commercial efforts of our products and product candidates could be delayed.

Product liability and other civil lawsuits against us could cause us to incur substantial liabilities and to limit clinical trials or commercialization of any current or future products. Our insurance program may not be extensive enough to adequately protect us against these risks.

We face an inherent risk of product liability exposure related to the commercialization of our products and any product candidate that we may market or commercialize, any gene therapy product materials that we manufacture for third parties at the Hopewell Facility and in connection with the human clinical trials testing of our products and product candidates. If we cannot successfully defend ourselves against claims that our product candidates, products or gene therapy product

materials caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- reduced resources of our management to pursue our business strategy;
- decreased demand for our products or any product candidates that we may develop;
- decreased demand for the gene therapy product materials that we manufacture for third parties at the Hopewell Facility;
- injury to our reputation and significant negative media attention;
- the inability to continue current clinical trials or begin planned clinical trials;
- withdrawal or reduced enrollment of clinical trial participants;
- significant costs to defend the related claims/litigation;
- increased insurance costs, or an inability to maintain appropriate insurance coverage;
- substantial monetary awards to trial participants, patients and/or their families;
- loss of revenue;
- the inability to commercialize or to continue commercializing any products or product candidates;
- initiation of investigations and enforcement actions by regulators; and
- the withdrawal of products from the market, product recalls, or the cessation of development or regulatory disapproval of product candidates or withdrawal of approvals, as well as labeling, marketing, or promotional restrictions.

We have a broad insurance program covering risks appropriate to our research and development activities, clinical programs, and aggregate annual limits of $25.0 million covering our products and sales. We also have industry standard insurance policies covering other aspects of our business and operations based on our locations, activities and other relevant factors. With respect to all insurance matters, we are advised by our insurance brokers and our insurance advisor, who we retain and compensate on a non-commission basis. However, our insurance program may not adequately cover the risks that we face for a variety of reasons, including:

- certain risks and related losses, such as delays to our clinical and development programs, are too speculative or unquantifiable for us to adequately insure against;
- if we were to face multiple claims, renewing or replacing our insurance may become more expensive, the terms (including deductibles and limits) we receive may worsen, and we may even have difficulty securing any coverage at all;
- our insurance limits may not be adequate to cover all liabilities and defense costs that we may incur; and
- we may need to further increase our insurance coverage if we commercialize our current products in additional jurisdictions, our sales increase, or we commercialize new products.

The cost of insurance coverage is highly variable, based on a wide range of factors. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability or defense costs that may arise.

In addition, we could be subject to other costly civil litigation, including contractual claims with respect to our expected manufacturing of gene therapy product materials for potential external customers. If our customers believe that we have violated our contractual terms, they may seek reimbursement for the cost of our gene therapy product materials or other related losses, the cost of which could be significant.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures, manufacturing and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations currently, and may in the future, involve the use of hazardous and flammable materials, including chemicals and medical and biological materials, and produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and wastes, we cannot eliminate the risk of contamination or injury from these materials. In the

event of contamination or injury resulting from our use of hazardous materials or disposal of hazardous wastes, we could be held liable for any resulting damages, and any liability could exceed our resources.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or manufacturing and distribution efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Our future success depends on our ability to retain our chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on Dr. Stuart W. Peltz, our co-founder and Chief Executive Officer, and the other principal members of our executive, commercial and scientific teams. Although we have formal employment agreements with each of our executive officers, these agreements do not prevent our executives from terminating their employment with us at any time. We do not maintain "key person" insurance on any of our executive officers. The loss of the services of any of these persons might impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. Additionally, because the field of gene therapies and gene therapy manufacturing is new and complex, we might face a shortage of skilled individuals with substantial gene therapy and gene therapy manufacturing experience. As a result, competition for skilled personnel, including in gene therapy research and gene therapy manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

We are in the process of expanding our development, regulatory, and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

In connection with our commercialization plans and business strategy, including our continued commercialization of Translarna, Emflaza, Upstaza, Tegsedi and Waylivra and, if approved, other product candidates, we have experienced and may to continue to experience significant growth in our employee base for sales, marketing, operational, managerial, financial, human resources, drug development, quality, regulatory and medical affairs and other areas. This growth has imposed and will continue to impose significant added responsibilities on members of management, including the need to recruit, hire, retain, motivate and integrate additional employees, including employees who joined us in connection with any of our acquisitions or other strategic transactions. Also, our management may have to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities, including any applicable integration. To manage our recent and anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. In addition, we may need to adjust the size of our workforce as a result of changes to our expectations for our business, which can result in diversion of management attention, disruptions to our business, and related expenses. For example, following our receipt of the Refuse to File letter from the FDA in 2016, we implemented a reorganization of our operations in March 2016 that resulted in a one-time charge for the related work-force reduction. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to our Intellectual Property

If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection or other intellectual property rights with respect to our proprietary technology and products. One primary way that we seek to protect our proprietary position is by filing patent applications in the United States and in certain ex-U.S. jurisdictions related to our proprietary technology and products. This process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications. It is also possible that we will fail to file a patent application on patentable aspects of our research and development. Moreover, if we license technology or product candidates from third parties, these license agreements may not permit us to control the filing and prosecution of patent applications, or to maintain or enforce the patents. These agreements could also give our licensors the right to enforce the licensed patents without our involvement, or to decide not to enforce the patents at all. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which prevent others from commercializing competitive technologies and products. Changes in patent laws or their interpretation in the United States and other countries may diminish the value of our patents.

The laws of ex-U.S. countries may not protect our rights to the same extent as the laws of the United States. For example, patent law in many countries restricts the patentability of methods of treatment of the human body more than U.S. law does. In addition, we may not pursue or obtain or be able to pursue or obtain patent protection in all major markets. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. In addition, the Leahy-Smith America Invents Act of 2011, or the Act, which reformed certain patent laws in the U.S., may create additional uncertainty. The significant changes engendered by the Act include switching from a “first-to-invent” system to a “first-to-file” system, and the implementation of new procedures that permit competitors to challenge our patents in the USPTO after grant, including inter partes review and post grant review.

Moreover, we may be subject to a third party prior art submissions in a patent office, or may become involved in patent office proceedings, including oppositions, derivation proceedings, reexamination, inter partes review, post grant review, interference proceedings, or litigation, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection or prevent competitors from competing with us. Our competitors may be able to circumvent our owned or licensed patents by developing alternative technologies or products in a non-infringing manner. Other companies may also attempt to circumvent any regulatory data protection or market exclusivity that we obtain under applicable legislation, which may require us to allocate significant resources to prevent such circumvention. Legal and regulatory developments in the European Union, or EU, and elsewhere may also result in clinical trial data and other information, that would ordinarily be treated as trade secret, submitted as part of a marketing authorization application becoming publicly available.

The EMA Policy on publication of clinical data and other such information, as well as the current application of EU freedom of information regulations, could impact our proprietary information (comprising both clinical and non-clinical data and other information) that would normally be maintained by a regulatory body as commercially confidential. Such developments could enable other companies to circumvent our intellectual property rights and use our clinical trial data or other information to obtain marketing authorizations in the EU and in other jurisdictions where we have not been able to obtain any intellectual property or regulatory protection, resulting in loss of market share. Such developments may also require us to allocate significant resources or engage in litigation to prevent other companies from circumventing or violating our intellectual property rights. Our attempts to prevent third parties from circumventing or violating our intellectual property and other rights may ultimately be unsuccessful. We may also fail to take the required actions to maintain our patents.

For example, during 2015, we were notified by the EMA that it had received from another pharmaceutical company a request under Regulation (EC) No 1049/2001 seeking access to aspects of our marketing authorization for Translarna for the treatment of nmDMD. Following the decision of the EMA to release such documentation with only minimal redactions we initiated litigation before the General Court of the EU to prevent disclosure of this information. In the first quarter of 2018, the Court ruled in favor of the EMA, allowing the EMA to release the documentation. We appealed the General Court's decision to the Court of Justice of the EU, or CJEU, but the CJEU dismissed our appeal in January 2020 and released the information to the requester.

An issued patent may be challenged, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

We may become involved in lawsuits to protect or enforce our intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our intellectual property. To counter infringement or unauthorized use, we may be required to file a lawsuit and claims for damages, which can be expensive and time consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their intellectual property or defenses, such that they do not infringe our intellectual property or that our intellectual property is invalid or unenforceable. In addition, in a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or may refuse to stop the other party from using the technology at issue.

Third parties may initiate legal proceedings alleging that our patents are invalid and unenforceable or that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our products and our product candidates and use our proprietary technologies without infringing the intellectual property and other proprietary rights of third parties. We may not be aware of all intellectual property rights potentially relating to our product and our product candidates. Typically, patent applications in the United States and other jurisdictions are not published until 18 months after filing, or in some cases not at all, and new patent applications are continuously publishing. Thus, we may not be aware of patents or patent applications relating to our product or our product candidates. There may be pending or future patent applications that, if issued, would block us from commercializing our products. Thus, we do not know with certainty whether any of our products or product candidates, or our commercialization thereof, would or would not infringe any third party's intellectual property.

We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights or other proprietary with respect to our products and technology. Third parties may assert infringement claims

against us based on existing or future intellectual property rights. We may allege that a third party patent we are alleged to infringe is invalid and/or we may be able to avail ourselves in the United States of the safe harbor exemption provided by the Hatch-Waxman Act as a basis for non-infringement. In order to successfully challenge the validity of a third party issued U.S. patent that we are alleged to infringe, we would need to overcome that patent's presumption of validity in district court or prove unpatentability by a preponderance of the evidence before the USPTO in a post grant proceeding. There is no assurance that a court or the USPTO would find these claims to be invalid or unpatentable, respectively.

If we are found to infringe a third party's intellectual property rights, or in order to avoid or settle litigation, we may seek to obtain a license to continue developing and marketing our products and technology. However, we may not be able to obtain any such license on commercially reasonable terms or at all. Also, any license obtained may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us, and could require us to make substantial payments. We could be forced, including by court order, to cease commercializing an alleged infringing technology or product. In addition, we could be found liable for monetary damages if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from commercializing our products or our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees were previously employed at universities or other companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while we typically require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation could cause us to spend substantial resources and could distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of such proceedings. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development, sales, marketing or distribution activities. We may not have sufficient resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

Without patent protection, our marketed products may face generic competition.

Certain of the products we market have no or limited patent protection and, as a result, potential competitors face fewer regulatory barriers in introducing competing products. Without patent protection or other regulatory exclusivity, we may

not be able to exclude others from, among other things, selling or importing similar products in any jurisdiction. In some instances, we may rely on trade secrets and other unpatented proprietary information to protect our commercial position, although we may be unable to provide adequate protection for our commercial position via these means. In other instances, we may need to rely on regulatory exclusivity to protect our commercial position.

Furthermore, generic competition against a branded product often results in decreases in the prices at which the branded product can be sold, particularly when there is more than one generic product available in the marketplace. Third-party companies could also develop products that are similar, but not identical, to our marketed products, such as an alternative formulation of our product or an alternative formulation combined with a different delivery technology, and seek approval in the United States by referencing our products and relying, to some degree, on the FDA's finding that our products are safe and effective in their approved indications. In addition, legislation enacted in the United States allows for, and in a few instances, in the absence of specific instructions from the prescribing physician, mandates the dispensing of generic products rather than branded products where a generic version is available.

On February 9, 2017, the FDA approved the corticosteroid Emflaza for the treatment of patients 5 years and older with DMD. Although approved for other indications outside of the United States, this was the first approval for deflazacort in the United States and the first approval in the United States for the use of a corticosteroid to treat DMD. Emflaza's seven-year period of orphan drug exclusivity related to the treatment of DMD in patients five years and older expires in February 2024. Emflaza's orphan drug exclusivity related to the treatment of DMD in patients two years of age to less than five expires in June 2026.

We rely on regulatory exclusivity for Emflaza and currently have no issued patents that could prevent a third-party company from seeking to introduce a generic Emflaza formulation in the United States for the treatment of DMD or another indication, and we may never be able to obtain such patent protection. Such third-party companies may also obtain patents covering a new deflazacort formulation or method of use.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents and regulatory exclusivity for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. More particularly, we may rely on trade secrets and other unpatented proprietary information to protect our competitive position related to our products and product candidates, especially when patent protection is not obtainable. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors, partners and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. However, we cannot guarantee that we have executed these agreements with each party that may have or have had access to our trade secrets or that the agreements we have executed will provide adequate protection. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be obtained or independently developed by a competitor, our competitive position would be harmed. If our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, employees, consultants, advisors, partners and other third parties develop new inventions or processes related to our products independently, or jointly with us, that may be applicable to our products under development, disputes may arise about ownership or proprietary rights to those inventions and processes. Enforcing a claim that a third party illegally obtained and is using any of our inventions or trade secrets is expensive and time-consuming, and the outcome is unpredictable. In addition, courts outside of the United States are sometimes less willing to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how.

We have not yet registered our trademarks in all of our potential markets, and failure to secure those registrations could adversely affect our business.

Our trademark applications may be refused registration, or our registered trademarks may not be maintained or may be found to be unenforceable. During trademark examination proceedings, our trademark applications may be rejected. Although we are given an opportunity to respond to those rejections in most jurisdictions, we may not be able to successfully overcome them. In addition, in the U.S. Patent and Trademark Office and Trademark Offices in many other jurisdictions, third parties are given an opportunity to oppose pending trademark applications or to seek cancellation of registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Further, if we do not secure registrations for our trademarks, we may encounter difficulty enforcing our trademark rights against third parties in the jurisdictions where we do not have registered rights.

If we are not able to obtain adequate trademark protection or regulatory approval for our brand names, we may be required to re-brand affected products, which could cause delays in getting such products to market and substantially increase our costs.

To protect our rights in any trademark we intend to use for our products or product candidates, we may seek to register such trademarks. Trademark registration is territory-specific and we must apply for trademark registration in the United States as well as any other country where we intend to commercialize our product or product candidates. Failure to obtain trademark registrations may place our use of the trademarks at risk or make them subject to legal challenges, which could force us to choose alternative names for our product or product candidates. In addition, the FDA, and other regulatory authorities outside the United States, conduct an independent review of proposed product names for pharmaceuticals, including an evaluation of the potential for confusion with other pharmaceutical product names for medications, which could result in medication errors in prescribing, dispensing and consumption. These regulatory authorities may also object to a proposed product name if they believe the name inappropriately makes or implies a therapeutic claim. If the FDA or other regulatory authorities outside the United States object to any of our proposed product names, we may be required to adopt alternative names for our product or product candidates. If we adopt alternative names, either because of our inability to obtain a trademark registration or because of objections from regulatory authorities, we would lose the benefit of our existing trademark applications and the rights attached thereto. Consequently, we may be required to expend significant additional resources in an effort to adopt a new product name that would be registrable under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA and other regulatory authorities, which could cause delays in getting our products to market and substantially increase our costs. Furthermore, in the United States and many other jurisdictions, a trademark registration may be cancelled through cancellation or forfeiture proceedings brought by a third party or from non-use of the trademark in that jurisdiction. We may not be able to build a successful brand identity for a new trademark in a timely manner or at all, which would limit our ability to commercialize our product or our product candidates.

Our rights to develop and commercialize Upstaza and our other gene therapy product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.

We depend upon the intellectual property rights granted to us under licenses from third parties that are important or necessary to the development of Upstaza for the treatment of AADC deficiency and our other gene therapy product candidates. In particular, we have in-licensed certain intellectual property rights and know-how from National Taiwan University, or NTU, relevant to Upstaza for the treatment of AADC deficiency. Any termination of these licenses could result in the loss of significant or all rights licensed to us and could harm or prevent our ability to commercialize Upstaza for the treatment of AADC deficiency and our other gene therapy product candidates. Each of our existing gene therapy licensing agreements are exclusive but are limited to particular fields, such as AADC deficiency and are subject to certain retained rights.

Our current gene therapy license agreements, including our agreement with NTU pursuant to which we have in-licensed certain intellectual property rights and know-how relevant to Upstaza for the treatment of AADC deficiency, impose various obligations, including certain payment obligations, including contingent payments to be made upon reaching certain development and regulatory milestones. If we fail to satisfy our obligations, the licensor may have the right to terminate the agreement. Disputes may arise between us and any of our licensors regarding intellectual property subject to

such agreements and other issues. Such disputes over intellectual property that we have licensed or the terms of our license agreements, including with respect to Upstaza for the treatment of AADC deficiency, may prevent or impair our ability to maintain our current arrangements on acceptable terms, or at all, or may impair the value of the arrangement to us. Any such dispute could have a material adverse effect on our business and our ability to realize the anticipated benefits of our acquisition of Agilis. If we cannot maintain a necessary license agreement, including with respect to Upstaza for the treatment of AADC deficiency, or if the agreement is terminated, we may be unable to successfully develop and commercialize the affected product candidates.

If we fail to comply with our obligations in our intellectual property licenses and funding arrangements with third parties, we could lose rights that are important to our business.

We are a party to a number of license agreements and expect to enter into additional licenses in the future. Our existing licenses impose, and we expect that future licenses will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, the licensor may have the right to terminate the license, in which event we might not be able to market any product that is covered by these agreements, which could materially adversely affect the value of the product candidate being developed under such license agreement. Termination of these license agreements or reduction or elimination of our licensed rights may result in our having to negotiate new or reinstated licenses with less favorable terms or cause us to lose rights in important intellectual property or technology.

We have also received grant funding for some of our development programs from philanthropic organizations and patient advocacy groups pursuant to agreements that impose development and commercialization diligence obligations on us. If we fail to comply with these obligations, the applicable organization could require us to grant to the organization exclusive rights under certain of our intellectual property, which could materially adversely affect the value to us of product candidates covered by that intellectual property even if we are entitled to a share of any consideration received by such organization in connection with any subsequent development or commercialization of the product candidates.

Some of our patented technology was developed with U.S. federal government funding. When new technologies are developed with U.S. government funding, the government obtains certain rights in any resulting patents, including a nonexclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise “march-in” rights to use or allow third parties to use our patented technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S. industry. In addition, U.S. government-funded inventions must be reported to the government and U.S. government funding must be disclosed in any resulting patent applications. Furthermore, our rights in such inventions are subject to government license rights and certain restrictions on manufacturing products outside the United States.

Risks Related to our Common Stock

Servicing the 2026 Convertible Notes requires a significant amount of cash. We may not have sufficient cash flow from our business to make payments on our debt, and we may not have the ability to raise the funds necessary to settle conversions of, or to repurchase, the 2026 Convertible Notes upon a fundamental change, which could adversely affect our business, financial condition and results of operations.

In September 2019, we incurred indebtedness in the amount of $287.5 million in aggregate principal with additional accrued interest under the 2026 Convertible Notes, for which interest is payable semi-annually in arrears on March 15 and September 15 of each year, beginning on March 15, 2020. Our ability to make scheduled payments of the principal of, to pay interest on or to refinance the 2026 Convertible Notes depends on our future performance, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate cash flow from operations in the future sufficient to service our debt, including the 2026 Convertible Notes. If we are unable to generate cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be unfavorable to us or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at the time we seek to refinance such indebtedness. We may

not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

Upon conversion of the 2026 Convertible Notes, unless we elect to deliver solely shares of our common stock to settle such conversion (other than paying cash in lieu of delivering any fractional shares), we will be required to make cash payments in respect of the 2026 Convertible Notes being converted. However, we may not have enough available cash or be able to obtain financing at the time we are required to repurchase 2026 Convertible Notes, to pay the 2026 Convertible Notes at maturity or to pay cash upon conversions of 2026 Convertible Notes. In addition, our ability to repurchase 2026 Convertible Notes or to pay cash upon conversions of 2026 Convertible Notes may be limited by law, by regulatory authority or by agreements governing our future indebtedness. Our failure to repurchase 2026 Convertible Notes at a time when the repurchase is required by the indenture, to make interest payments on the 2026 Convertible Notes when due under the indenture or to pay any cash payable on future conversions of the 2026 Convertible Notes as required by the indenture would constitute a default under each indenture governing the 2026 Convertible Notes and the Blackstone Credit Agreement. An event of default under the applicable indenture governing the 2026 Convertible Notes or the fundamental change itself could also lead to a default under agreements governing our future indebtedness. If the repayment of any such related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness, repurchase the 2026 Convertible Notes, make interest payments on the 2026 Convertible Notes or make cash payments upon conversions of the 2026 Convertible Notes.

Even if holders of the 2026 Convertible Notes do not elect to convert their 2026 Convertible Notes, we could be required to reclassify all of the outstanding principal of the 2026 Convertible Notes as a current rather than long-term liability in accordance with applicable accounting rules, which would result in a material reduction of our net working capital. Any of these factors could materially and adversely affect our business, financial condition and results of operations.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- provide for a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock and lawsuits against us and our officers and directors.

Our stock price has been and will likely continue to be volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price at which they purchased it. The market price for our common stock may be influenced by many factors, including:

- expectations with respect to our gene therapy platform, including any potential regulatory submissions and potential approvals, including those related to Upstaza;
- any developments related to our ability or inability to execute our commercialization strategy for any of our products;
- our ability to resolve the matters set forth in the FDA's denial of our appeal to the Complete Response Letter we received from the FDA in connection with our NDA for Translarna for the treatment of nmDMD, and our ability to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost;
- our ability to maintain our marketing authorization for Translarna for the treatment of nmDMD in Brazil, Russia and in the EEA, which is subject to the specific obligation to conduct Study 041 and is also subject to annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA;
- any developments related to Study 041, including with respect to design, timing and conduct, and developments with respect to any clinical or non-clinical trial required by other regulatory agencies, including the FDA for Translarna for the treatment of nmDMD;
- the commercialization of Evrysdi and the development of the SMA program with Roche and the SMA Foundation;
- results of clinical trials of any other product candidate that we develop;
- announcements by us or our competitors of significant acquisitions, licenses, strategic collaborations, joint ventures, collaborations or capital commitments;
- negative publicity around our products or product candidates;
- other developments concerning our regulatory submissions;
- whether regulators in other territories agree with our interpretation of the results of ACT DMD;
- the success of competitive products or technologies;
- results of clinical trials of product candidates of our competitors, including negative results that investors may associate with our product candidates;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- our ability to realize the benefits of our acquisitions or other business combinations;
- the recruitment or departure of key personnel;
- the loss of distributors, suppliers or manufacturers;
- the level of expenses related to any of our products, product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- announcements with respect to litigation;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions, including potentially high inflation rates; and
- the other factors described in this "Risk Factors" section.

Companies that have experienced volatility in the market price of their stock have frequently been the subject of securities class action and shareholder derivative litigation. For example, in 2018 we settled a securities class action lawsuit initiated against us and certain of our current and former executive officers during 2016, as well as derivative lawsuits brought against us, as a nominal defendant, certain of our current and former executive officers and certain of our current and

former directors during 2017. We could be the target of other such litigation in the future. Class action and derivative lawsuits, whether successful or not, could result in substantial costs, damage or settlement awards and a diversion of our management's resources and attention from running our business, which could materially harm our reputation, financial condition and results of operations.

Because we do not anticipate paying any cash dividends on our capital in the foreseeable future, capital appreciation, if any, will be our stockholders' sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the development and growth of our business. The terms of the Blackstone Credit Agreement preclude us from paying dividends, other than permitted dividends set forth in the agreement. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be our stockholders' sole source of gain for the foreseeable future.

The issuance of additional shares of our common stock or the sale of shares of our common stock by our stockholders could dilute our stockholders' ownership interest in the Company and could significantly reduce the market price of our common stock.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

We have issued a significant number of equity awards under our equity compensation plans or as inducement grants to new hire employees pursuant to Nasdaq rules. The shares underlying these awards are registered on a Form S-8 registration statement. As a result, upon vesting these shares can be freely exercised and sold in the public market upon issuance, subject to volume limitations applicable to affiliates. The exercise of options and the subsequent sale of the underlying common stock or the sale of restricted stock upon vesting could cause a decline in our stock price. These sales also might make it difficult for us to sell equity securities in the future at a time and at a price that we deem appropriate.

Certain of our employees, executive officers and directors have entered or may enter into Rule 10b5-1 plans providing for sales of shares of our common stock from time to time. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the employee, director or officer when entering into the plan, without further direction from the employee, officer or director. A Rule 10b5-1 plan may be amended or terminated in some circumstances. Our employees, executive officers and directors may also buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

Additionally, certain shares that we issued in connection with our acquisitions or other strategic transactions have not yet been sold and are currently restricted as a result of securities laws. These shares may be freely sold in the public market subject to any requirements and restrictions, including any applicable volume limitations, imposed by Rule 144 under the Securities Act. The sale or resale of these shares in the public market, or the market's expectation of such sales, may result in an immediate and substantial decline in our stock price. Such a decline will adversely affect our investors and also might make it difficult for us to sell equity securities in the future at a time and at a price that we deem appropriate.

Sales of substantial amounts of shares of our common stock or other securities by our stockholders or by us, including sales made under the Sales Agreement, pursuant to which we may offer and sell shares of our common stock having an aggregate offering price of up to $125 million from time to time, through the Sales Agent by any method that is deemed to be an "at the market" offering as defined in Rule 415(a)(4) promulgated under the Securities Act, or the issuance of shares of our common stock upon conversion of our outstanding 2026 Convertible Notes or any future securities convertible or exchangeable into our common stock or in connection with a strategic transaction or otherwise, could dilute our stockholders, lower the market price of our common stock and impair our ability to raise capital through the sale of equity securities.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

Our principal facilities consist of approximately 126,000 square feet of research and office space located at 100, 200, 250 and 400 Corporate Court, Middlesex Business Center, South Plainfield, New Jersey, that we occupy under leases that expire in 2024, with two consecutive five-year renewal options to renew the leases after 2024. Additionally, we entered into a lease agreement for approximately 103,000 square feet of laboratory and office space in Bridgewater, New Jersey. The rental term for such facility commenced on May 1, 2020 with an initial term of seven years and two consecutive five year renewal periods at our option. We entered into a lease agreement for approximately 220,500 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey. The rental term for such facility commenced on July 1, 2020, with an initial term of fifteen years and two consecutive 10-year renewal periods at our option. We also lease two entire buildings comprised of approximately 360,000 square feet of shell condition, modifiable space at a facility located in Warren, New Jersey. The rental term for such facility commenced on June 1, 2022, with an initial term of seventeen years followed by three consecutive five-year renewal periods at our option. We lease approximately 6,500 square feet of office space in Dublin, Ireland, that we occupy under a lease that expires in 2024. Additionally, we lease approximately 5,000 square feet of office space in Sao Paulo, Brazil, that we occupy under a lease that expires in 2024. We also lease additional office space in the U.S. and other countries to support our operations as a global organization, but these leases are not material to us.

Item 3. Legal Proceedings

From time to time in the ordinary course of our business, we are subject to claims, legal proceedings and disputes. We are not currently aware of any material legal proceedings which we are a party to or of which any of our property is the subject.

Item 4. Mine Safety Disclosures

None.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuers Purchases of Equity Securities

Market Information

Our common stock has been publicly traded on the Nasdaq Global Select Market under the symbol "PTCT" since June 20, 2013. Prior to that time, there was no public market for our common stock.

Holders

As of February 17, 2023, there were 98 holders of record of our common stock. This number does not include beneficial owners whose shares are held in street name.

Recent Sales of Unregistered Securities

We did not sell any of our equity securities or any options, warrants, or rights to purchase our equity securities during the period covered by this Annual Report on Form 10-K that were not registered under the Securities Act of 1933, as amended, or the Securities Act, and that have not otherwise been described in a Current Report on Form 8-K or a Quarterly Report on Form 10-Q.

Purchase of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

Item 6. [Reserved]

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis is meant to provide material information relevant to an assessment of the financial condition and results of operations of our company, including an evaluation of the amounts and certainty of cash flows from operations and from outside resources, so as to allow investors to better view our company from management's perspective. The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Annual Report on Form 10-K. This discussion contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth in Part I, Item 1A. Risk Factors, of this Annual Report on Form 10-K, our actual results may differ materially from those anticipated in these forward-looking statements.

We are a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. Our ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. Our mission is to provide access to best-in-class treatments for patients who have little to no treatment options. Our strategy is to leverage our strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. We believe that this allows us to maximize value for all of our stakeholders. We have a portfolio pipeline that includes several commercial products and product candidates in various stages of development, including clinical, pre-clinical and research and discovery stages, focused on the development of new treatments for multiple therapeutic areas for rare diseases relating to neurology, metabolism and oncology.

We have two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area, or EEA, for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients

aged two years and older and in Russia for the treatment of nmDMD in patients aged two years and older. In July 2020, the European Commission approved the removal of the statement “efficacy has not been demonstrated in non-ambulatory patients” from the indication statement for Translarna. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. During the year ended December 31, 2022, we recognized $288.6 million in sales of Translarna. We hold worldwide commercialization rights to Translarna for all indications in all territories. Emflaza is approved in the United States for the treatment of DMD in patients two years and older. During the year ended December 31, 2022, Emflaza achieved net sales of $218.3 million.

Our marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the European Medicines Agency, or EMA, of the benefit-risk balance of the authorization, which we refer to as the annual EMA reassessment. In June 2022, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2023. This marketing authorization is further subject to a specific obligation to conduct and submit the results of an 18-month, placebo-controlled trial, followed by an 18-month open-label extension, which we refer to together as Study 041. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Within the placebo-controlled trial, Translarna showed a statistically significant treatment benefit across the entire intent to treat population as assessed by the 6-minute walk test, assessing ambulation and endurance, and in lower-limb muscle function as assessed by the North Star Ambulatory Assessment, a functional scale designed for boys affected by DMD. Additionally, Translarna showed a statistically significant treatment benefit across the intent to treat population within the 10-meter run/walk and 4-stair stair climb, each assessing ambulation and burst activity, while also showing a positive trend in the 4-stair stair descend although not statistically significant. Within the primary analysis group, Translarna demonstrated a positive trend across all endpoints, however, statistical significance was not achieved. Translarna was also well tolerated. In September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. We expect an opinion from the Committee for Medicinal Products for Human Use in the first half of 2023.

Each country, including each member state of the EEA, has its own pricing and reimbursement regulations. In order to commence commercial sale of product pursuant to our Translarna marketing authorization in any particular country in the EEA, we must finalize pricing and reimbursement negotiations with the applicable government body in such country. As a result, our commercial launch will continue to be on a country-by-country basis. We also have made, and expect to continue to make, product available under early access programs, or EAP programs, both in countries in the EEA and other territories. Our ability to negotiate, secure and maintain reimbursement for product under commercial and EAP programs can be subject to challenge in any particular country and can also be affected by political, economic and regulatory developments in such country.

There is substantial risk that if we are unable to renew our EEA marketing authorization during any annual renewal cycle, or if our product label is materially restricted, or if Study 041 does not provide the data necessary to maintain our marketing authorization, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna in the EEA and other territories.

Translarna is an investigational new drug in the United States. During the first quarter of 2017, we filed a New Drug Application, or NDA, for Translarna for the treatment of nmDMD over protest with the United States Food and Drug Administration, or FDA. In October 2017, the Office of Drug Evaluation I of the FDA issued a Complete Response Letter for the NDA, stating that it was unable to approve the application in its current form. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied our appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients’ muscles. We followed the FDA’s recommendation and collected, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, and announced the results of Study 045 in February 2021. Study 045 did not meet its pre-specified primary endpoint. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Following this announcement, we submitted a meeting request to the FDA to gain clarity on the regulatory pathway for a potential re-submission of an NDA for

Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support NDA re-submission. We recently had an informal meeting with the FDA, during which we discussed the potential path to an NDA re-submission for Translarna. Based on the meeting discussion, we plan to request an additional Type C meeting with the FDA in the near future to review the totality of data collected to date, including dystrophin and other mechanistic data as well as additional analyses that could support the benefit of Translarna.

We have a pipeline of gene therapy product candidates for rare monogenic diseases that affect the CNS, including Upstaza for the treatment of Aromatic L-Amino Decarboxylase, or AADC, deficiency, a rare central nervous system, or CNS, disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene, for patients 18 months and older within the EEA. In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. We are also preparing a biologics license application, or BLA, for Upstaza for the treatment of AADC deficiency in the United States. In October 2022, we held a type C meeting with the FDA to discuss the details of a potential submission package for Upstaza. At such meeting, the FDA asked for additional bioanalytical data in support of comparability between the drug product used in the clinical studies and the commercial drug product. We have completed these analyses and provided the results to the FDA for review. We expect to submit a BLA to the FDA in the first half of 2023.

We hold the rights for the commercialization of Tegsedi and Waylivra for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to a Collaboration and License Agreement, or the Tegsedi-Waylivra Agreement, dated August 1, 2018, by and between us and Akcea Therapeutics, Inc., or Akcea, a subsidiary of Ionis Pharmaceuticals, Inc. Tegsedi has received marketing authorization in the United States, European Union, or EU, and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. We began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and we continue to make Tegsedi available in certain other countries within Latin America and the Caribbean through EAP programs. In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome, or FCS, in Brazil and we began to make commercial sales of Waylivra in Brazil in the third quarter of 2022 while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP programs. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy, or FPL. Waylivra has also received marketing authorization in the EU for the treatment of FCS.

We also have a spinal muscular atrophy, or SMA, collaboration with F. Hoffman-La Roche Ltd. and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. The SMA program has one approved product, Evrysdi® (risdiplam), which was approved by the FDA in August 2020 for the treatment of SMA in adults and children two months and older and by the European Commission in March 2021 for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020 and Japan in June 2021. In May 2022, the FDA approved a label expansion for Evrysdi to include infants under two months old with SMA and we expect the EMA to make a regulatory decision on approval for a label expansion for Evrysdi to include infants under two months old with SMA in 2023. In addition to our SMA program, our splicing platform also includes PTC518, which is being developed for the treatment of Huntington's disease, or HD. We announced the results from our Phase 1 study of PTC518 in healthy volunteers in September 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid and protein levels, that PTC518 efficiently crosses blood brain barrier at significant levels and that PTC518 was well tolerated. We initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. Enrollment in the Phase 2 study remains active and ongoing outside of the United States. Enrollment within the United States is paused as the FDA has requested additional data to allow the Phase 2 study to proceed; discussions are ongoing with the FDA to allow the resumption of U.S. enrollment. We expect data from the initial 12-week phase of the Phase 2 study in the second quarter of 2023.

Our Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. The two most advanced molecules in our Bio-e platform are vatiquinone and utreloxastat. We initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in children with mitochondrial disease associated seizures in the third quarter of 2020. We have completed enrollment in this trial after previously experiencing delays in enrollment due to the COVID-19 pandemic. We anticipate results from the Phase 2/3 trial to be available in the second quarter of 2023. We also initiated a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in the fourth quarter of 2020 and anticipate results from this trial to be available in the second quarter of 2023. In the third quarter of 2021, we completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of utreloxastat. Utreloxastat was found to be well-tolerated with no reported serious adverse events while demonstrating predictable pharmacology. We initiated a Phase 2 trial of utreloxastat for amyotrophic lateral sclerosis in the first quarter of 2022 and enrollment is ongoing.

The most advanced molecule in our metabolic platform is sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products, for orphan diseases. We initiated a registration-directed Phase 3 trial for sepiapterin for PKU in the third quarter of 2021 with the primary endpoint in the study of achieving statistically-significant reduction in blood Phe level. The primary analysis population includes those patients who have a greater than 30% reduction in blood Phe levels during the Part 1 run-in phase of the trial. In January 2023, we announced preliminary data from the Part 1 run-in phase of this trial, including that the mean reduction in blood Phe levels in an initial cohort of subjects during the Part 1 would be recognized as clinically meaningful if maintained in Part 2 of the trial. We now expect results from Part 2 of this trial to be available in May 2023 as the trial is overenrolled and additional time is required for the entirety of the primary analysis population to complete the study.

Unesbulin is our most advanced oncology agent. We completed our Phase 1 trials evaluating unesbulin in leiomyosarcoma, or LMS, and diffuse intrinsic pontine glioma, or DIPG, in the fourth quarter of 2021. We initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022 and enrollment is ongoing. The initiation of our registration-directed Phase 2/3 trial of unesbulin for DIPG was delayed as we continued to track the progress of patients in our Phase 1 trial and analyze the corresponding data. We now expect to initiate a registration-directed Phase 2/3 trial of unesbulin for the treatment of DIPG in the fourth quarter of 2023.

In addition, we have a pipeline of product candidates and discovery programs that are in early clinical, pre-clinical and research and development stages focused on the development of new treatments for multiple therapeutic areas for rare diseases.

COVID-19 Impact

The global pandemic caused by a strain of novel coronavirus, COVID-19, has impacted the timing of certain of our clinical trials and regulatory submissions as well as other aspects of our business operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to materially adversely affect our business, financial condition, results of operations, and prospects. For additional information, see "Item 1A. Risk Factors - *We face risks related to health epidemics and other widespread outbreaks of contagious disease, which have previously, and may once again, delay our ability to complete our ongoing clinical trials and initiate future clinical trials, disrupt regulatory activities and have other adverse effects on our business and operations, including the novel coronavirus (COVID 19) pandemic, which disrupted, and may continue to disrupt, our operations and may significantly impact our operating results. In addition, the COVID 19 pandemic has caused substantial disruption in the financial markets and economies, which could result in adverse effects on our business and operations.*"

Overview—Funding

The success of our products and any other product candidates we may develop, depends largely on obtaining and maintaining reimbursement from governments and third-party insurers. During 2022, our revenues were primarily generated from sales of Translarna for the treatment of nmDMD in countries where we were able to obtain acceptable commercial pricing and reimbursement terms and in select countries where we are permitted to distribute Translarna under our EAP programs, and from sales of Emflaza for the treatment of DMD in the United States. We also generated revenue

from sales of Upstaza for the treatment of AADC deficiency in the EEA and have recognized revenue associated with milestone and royalty payments from Roche pursuant to a License and Collaboration Agreement, or the SMA License Agreement, by and among us, Roche and, for the limited purposes set forth therein, the SMA Foundation, under our SMA program.

See "Item 1. Business—Commercial Matters—Market Access Considerations" for additional information and "Item 1A. Risk Factors—*Commercialization of Translarna and Upstaza has been in, and is expected to continue to take place in, countries that tend to impose strict price controls, which may adversely affect our revenues. Failure to obtain and maintain acceptable pricing and reimbursement terms for Translarna for the treatment of nmDMD or Upstaza for the treatment of AADC deficiency in the EEA and other countries where Translarna is available would delay or prevent us from marketing our product in such regions, which would adversely affect our business, results of operations, and financial condition.*"

In August 2019, we entered into an At the Market Offering Sales Agreement, or the Sales Agreement, with Cantor Fitzgerald and RBC Capital Markets, LLC, or together, the Sales Agents, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended, or the Securities Act. During the year ended December 31, 2020, we issued and sold an aggregate of 542,470 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $53.37 per share. We received net proceeds of $28.1 million after deducting agent discounts and commissions and other offering expenses payable by us. We did not issue or sell any shares of common stock pursuant to the Sales Agreement during the years ending December 31, 2021 and December 31, 2022. The remaining shares of our common stock available to be issued and sold, under the Sales Agreement, have an aggregate offering price of up to $93.0 million as of December 31, 2022.

In September 2019, we issued $287.5 million aggregate principal amount of 1.50% convertible senior notes due September 15, 2026, or the 2026 Convertible Notes, which included an option to purchase up to an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes, which was exercised in full by the initial purchasers. We received net proceeds of $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us. See "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and capital resources—Sources of Liquidity" for additional information.

On April 29, 2020, we entered into a Rights Exchange Agreement, or the Rights Exchange Agreement, pursuant to which we issued 2,821,176 shares of our common stock and paid $36.9 million, in the aggregate, to certain former equityholders, or the Participating Rightholders, of Agilis Biotherapeutics, Inc., or Agilis, in exchange for the cancellation and forfeiture by the Participating Rightholders of their rights to receive certain milestone-based contingent payments under the Agreement and Plan of Merger, dated as of July 19, 2018 by and among us, Agility Merger Sub, Inc. and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, or the Agilis Merger Agreement.

On May 29, 2020, we acquired Censa Pharmaceuticals, Inc., or Censa, for total upfront consideration composed of (i) cash consideration of $15.0 million, which consisted of an upfront payment of $10.4 million and an additional $4.6 million for the net assets on Censa's opening balance sheet as of the date of the acquisition, and (ii) 845,364 shares of our common stock, which were valued at $42.9 million based on the closing stock price on the acquisition date. The number of shares issued was determined using a 30-day VWAP pursuant to the Agreement and Plan of Merger, dated as of May 5, 2020, or the Censa Merger Agreement, by and among us, Hydro Merger Sub, Inc., our wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC.

In July 2020, we entered into a Royalty Purchase Agreement, or the Royalty Purchase Agreement, with RPI 2019 Intermediate Finance Trust, or RPI, pursuant to which we sold to RPI 42.933%, or the Assigned Royalty Payment, of our right to receive sales-based royalty payments, or the Royalty, on worldwide net sales of Evrysdi and any other product developed pursuant to the SMA License Agreement. In consideration for the sale of the Assigned Royalty Payments, RPI paid us $650.0 million in cash consideration. The Royalty Purchase Agreement will terminate 60 days following the earlier

of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

In June 2021, we filed a Certificate of Amendment to our Restated Certificate of Incorporation, which increased the number of authorized shares of our common stock from 125,000,000 to 250,000,000 shares.

In October 2022, we entered into a credit agreement, or the Blackstone Credit Agreement, for fundings of up to $950.0 million consisting of a committed loan facility consisting of a senior secured term loan facility funded on October 27, 2022, or the Closing Date, in the aggregate principal amount of $300.0 million, and a delayed draw term loan facility of up to $150.0 million to be funded at our request within 18 months of the Closing Date subject to specified conditions, and further contemplating the potential for up to $500.0 million of additional financing, to the extent that we request such additional financing and subject to the Lenders' agreement to provide such additional financing and to mutual agreement on terms among us and certain of our subsidiaries, or, collectively with us, the Loan Parties, and funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit, or collectively, Blackstone, and such lenders, together with their permitted assignees, the Lenders, and Wilmington Trust, National Association, as the administrative agent for the Lenders. See "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and capital resources—Sources of Liquidity" for additional information.

In connection with the execution of the Blackstone Credit Agreement, we and certain entities affiliated with the Lenders, or the Purchasers, also entered into a stock purchase agreement on the Closing Date for the sale and issuance of 1,095,290 shares of common stock to the Purchasers at a price of $45.65 per share, for an aggregate purchase price of approximately $50.0 million. The per share price represents the closing price of our common stock on the Nasdaq Global Select Market on October 26, 2022.

To date, we have financed our operations primarily through our offering of the 2026 Convertible Notes, our public offerings of common stock in February 2014, in October 2014, in April 2018, in January 2019, and in September 2019, the common stock issued in our "at the marketing offering", our initial public offering of common stock in June 2013, proceeds from the Royalty Purchase Agreement, net proceeds from our borrowings under the Blackstone Credit Agreement, private placements of our convertible preferred stock and common stock, collaborations, bank and institutional lender debt, other convertible debt, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. We have relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States since 2014, Emflaza for the treatment of DMD in the United States since 2017 and Upstaza for the treatment of AADC deficiency in the EEA since May 2022. We have also relied on revenue associated with milestone and royalty payments from Roche pursuant to the SMA License Agreement, under our SMA program.

As of December 31, 2022, we had an accumulated deficit of $2,657.0 million. We had a net loss of $559.0 million, $523.9 million, and $438.2 million for the fiscal years ended December 31, 2022, 2021, and 2020, respectively.

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing, including expanding our direct manufacturing capabilities at our leased biologics manufacturing facility and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur ongoing research and development expenses for our products and product candidates, including our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in. We are also preparing a BLA for Upstaza for the treatment of AADC deficiency in the United States and we anticipate submitting a BLA to the FDA in the first half of 2023. These efforts may significantly impact the timing and extent of our commercialization expenses.

We may seek to expand and diversify our product pipeline through opportunistically in-licensing or acquiring the rights to products, product candidates or technologies and we may incur expenses, including with respect to transaction

costs, subsequent development costs or any upfront, milestone or other payments or other financial obligations associated with any such transaction, which would increase our future capital requirements.

With respect to our outstanding 2026 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.3 million annually. On August 15, 2022, we repaid the outstanding principal amount and accrued interest, totaling $152.3 million, of the 3.00% convertible senior notes due August 15, 2022, or the 2022 Convertible Notes, that was due upon maturity in accordance with the terms of the 2022 Convertible Notes.

Borrowings under the Blackstone Credit Agreement bear interest at a variable rate equal to, at our option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively.

In October 2022, we paid the former equityholders of Agilis $50.0 million in regulatory milestone payments as a result of the European Commission's marketing approval of Upstaza for the treatment of AADC deficiency in July 2022. In 2022, we also paid Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, a single $50.0 million sales-based milestone in accordance with the asset purchase agreement, dated March 15, 2017, as amended on April 20, 2017, or the Emflaza Asset Purchase Agreement, by and between us and Marathon.

In February 2023, we completed enrollment of our Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event and in accordance with the Censa Merger Agreement, we are obligated to pay a $30.0 million development milestone to the former Censa securityholders, which we have the option to pay in cash or shares of our common stock.

We expect to make additional payments to the former Censa securityholders of $50.0 million in the aggregate upon the potential achievement in 2023 of certain development and regulatory milestones relating to sepiapterin. Furthermore, we expect to pay the former equityholders of Agilis an additional $20.0 million upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency, which we expect to occur in the first half of 2023.

We have never been profitable and we will need to generate significant revenues to achieve and sustain profitability, and we may never do so. Accordingly, we may need to obtain substantial additional funding in connection with our continuing operations. Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or our commercialization efforts.

Financial operations overview

To date, our net product revenues have consisted primarily of sales of Translarna for the treatment of nmDMD in territories outside of the United States, and sales of Emflaza for the treatment of DMD in the United States. Our process for recognizing revenue is described below under "Critical accounting policies and significant judgments and estimates—Revenue recognition".

Roche and the SMA Foundation Collaboration. In November 2011, we entered into the SMA License Agreement pursuant to which we are collaborating with Roche and the SMA Foundation to further develop and commercialize compounds identified under our SMA program with the SMA Foundation. The research component of this agreement terminated effective December 31, 2014. We are eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of specified sales events, and up to double digit royalties on worldwide annual net sales of a commercial product. As of December 31, 2022, we had recognized a total of $210.0 million in milestone payments and $172.9 million royalties on net sales pursuant to the SMA License Agreement. As of December 31, 2022, there are no remaining research and development event milestones that we can receive. The remaining potential sales milestones as of December 31, 2022 are $250.0 million upon achievement of certain sales events.

Pursuant to the Royalty Purchase Agreement, we sold to RPI the Assigned Royalty Payment, in consideration for $650.0 million. We have retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the License Agreement. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

Research and development expense

Research and development expenses consist of the costs associated with our research activities, as well as the costs associated with our drug discovery efforts, conducting preclinical studies and clinical trials, manufacturing development efforts and activities related to regulatory filings. Our research and development expenses consist of:

- external research and development expenses incurred under agreements with third-party contract research organizations and investigative sites, third-party manufacturing organizations and consultants;
- employee-related expenses, which include salaries and benefits, including share-based compensation, for the personnel involved in our drug discovery and development activities; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, IT, human resources, and other support functions, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

We use our employee and infrastructure resources across multiple research projects, including our drug development programs. We track expenses related to our clinical programs and certain preclinical programs on a per project basis.

We expect our research and development expenses to fluctuate in connection with our ongoing activities, particularly in connection with Study 041 and other studies for Translarna for the treatment of nmDMD, our activities under our splicing, gene therapy, Bio-e, metabolic and oncology programs and performance of any post-marketing requirements imposed by regulatory agencies with respect to our products. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our products or product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs, and product and product candidate manufacturing costs.

The following table provides research and development expense for our most advanced principal product development programs, for the years ended December 31, 2022, 2021, and 2020.

	Year ended December 31,		
	2022	2021	2020
	(in thousands)		
Global DMD Franchise	$ 78,544	$ 83,791	$ 80,742
Metabolic	81,810	49,458	59,135
Gene Therapy	183,487	150,566	213,206
Bio-e	67,209	60,964	29,322
Oncology	34,395	18,618	16,467
Splicing	76,208	53,429	18,567
Emvododstat for COVID-19	12,667	38,348	13,590
Discovery	117,176	85,510	46,614
Total research and development	$ 651,496	$ 540,684	$ 477,643

The successful development of our product and product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the scope, rate of progress and expense of our clinical trials and other research and development activities;

- the potential benefits of our product and product candidates over other therapies;
- our ability to market, commercialize and achieve market acceptance for our products or any of our product candidates that we are developing or may develop in the future, including our ability to negotiate pricing and reimbursement terms acceptable to us;
- clinical trial results;
- the terms and timing of regulatory approvals; and
- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of any of our products or product candidates could mean a significant change in the costs and timing associated with the development of that product candidates. For example, if the EMA or FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of any of our products or product candidate or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. In addition, the uncertainty with respect to the duration, nature and extent of negative impacts of the COVID-19 pandemic and responsive measures relating thereto on our ability to successfully enroll our current and future clinical trials, has caused us to experience delays, and may cause us to experience further delays, in our clinical trials and regulatory submissions.

Selling, general and administrative expense

Selling, general and administrative expenses consist primarily of salaries and other related costs for personnel, including share-based compensation expenses, in our executive, legal, business development, commercial, finance, accounting, information technology and human resource functions. Other selling, general and administrative expenses include facility-related costs not otherwise included in research and development expense; advertising and promotional expenses; costs associated with industry and trade shows; and professional fees for legal services, including patent-related expenses, accounting services and miscellaneous selling costs.

We expect that selling, general and administrative expenses will increase in future periods in connection with our continued efforts to commercialize our products, including increased payroll, expanded infrastructure, commercial operations, increased consulting, legal, accounting and investor relations expenses.

Interest expense, net

Interest expense, net consists of interest expense from the liability for the sale of future royalties related to the Royalty Purchase Agreement, the 2026 Convertible Notes outstanding, the Blackstone Credit Agreement, the 2022 Convertible Notes that we repaid in August 2022 and from our credit and security agreement, or the MidCap Credit Agreement, with MidCap Financial Trust that was terminated in July 2020 offset by interest income earned on investments.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. Actual results may differ from these estimates under different assumptions or conditions.

Of our policies, the following are considered critical to an understanding of our consolidated financial statements as they require the application of the most subjective and complex judgment, involving critical accounting estimates and assumptions impacting our consolidated financial statements:

- Revenue recognition related to net product revenue

- Liability for sale of future royalties
- Contingent consideration from business combinations
- Indefinite-lived intangible assets annual impairment assessment

Revenue recognition related to net product revenue

Our net product revenue primarily consists of sales of Translarna in territories outside of the U.S. and sales of Emflaza in the U.S., both for the treatment of DMD. We recognize revenue when performance obligations with customers have been satisfied. Our performance obligations are to provide products based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligations are satisfied at a point in time when our customer obtains control of the product, which is typically upon delivery. We invoice customers after the products have been delivered and invoice payments are generally due within 30 to 90 days of invoice date. We determine the transaction price based on fixed consideration in its contractual agreements. Contract liabilities arise in certain circumstances when consideration is due for goods not yet provided. As we have identified only one distinct performance obligation, the transaction price is allocated entirely to the product sale. In determining the transaction price, a significant financing component does not exist since the timing from when we deliver product to when the customers pay for the product is typically less than one year. Customers in certain countries pay in advance of product delivery. In those instances, payment and delivery typically occur in the same month.

We record product sales net of any variable consideration, which includes discounts, allowances, rebates related to Medicaid and other government pricing programs, and distribution fees. We use the expected value or most likely amount method when estimating variable consideration, unless discount or rebate terms are specified within contracts. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained.

During the years ended December 31, 2022, 2021, and 2020, net product sales in the United States were $218.3 million, $187.3 million, and $139.0 million, respectively, consisting solely of sales of Emflaza, and net product sales outside of the United States were $316.9 million, $241.6 million, and $194.4 million respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net product revenues made up $288.6 million, $236.0 million, and $191.9 million of the net product sales outside the United States for the years ended December 31, 2022, 2021, and 2020, respectively. During the year ended December 31, 2022, two countries, the United States and Russia, accounted for at least 10% of our net product sales, representing $218.3 million and $59.7 million, respectively. During the years ended December 31, 2021 and 2020, only the United States accounted for at least 10% of our net product sales.

In relation to customer contracts, we incur costs to fulfill a contract but do not incur costs to obtain a contract. These costs to fulfill a contract do not meet the criteria for capitalization and are expensed as incurred. We consider any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise. Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Liability for sale of future royalties

In July 2020, we entered into the Royalty Purchase Agreement with RPI, pursuant to which we sold to RPI the Assigned Royalty Payment. In consideration for the sale of the Assigned Royalty Payments, RPI paid us $650.0 million in cash consideration. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

The cash consideration obtained pursuant to the Royalty Purchase Agreement is classified as debt and is recorded as “liability for sale of future royalties-current” and “liability for sale of future royalties-noncurrent” on our consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of

future royalties at the time of the transaction was based on our estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability is amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance. We utilize the prospective method to account for subsequent changes in the estimated future payments to be made to RPI.

Contingent consideration from business combinations

The consideration for our business acquisitions may include future payments that are contingent upon the occurrence of a particular event or events. The obligations for such contingent consideration payments are recorded at fair value on the acquisition date. The contingent consideration obligations are then evaluated each reporting period. Changes in the fair value of contingent consideration, other than changes due to payments, are recognized as a gain or loss and recorded within the change in the fair value of deferred and contingent consideration in the consolidated statements of operations. The fair value of development and regulatory milestones are estimated utilizing a probability adjusted, discounted cash flow approach. The discount rates are estimated utilizing Corporate B rated bonds maturing in the years of expected payments based on our estimated development timelines for the acquired product candidate. The fair value of the net sales milestones and royalties is based on probability adjusted sales estimates and estimated discount rates and utilizes an option pricing model with Monte Carlo simulation to simulate a range of possible payment scenarios, and the average of the payments in these scenarios is then discounted to calculate present fair value.

Indefinite-lived intangible assets annual impairment assessment

Indefinite-lived intangible assets consist of IPR&D acquired in business combinations. Intangible assets with indefinite lives, including IPR&D, are tested for impairment if impairment indicators arise and, at a minimum, annually. The indefinite-lived intangible asset impairment test consists of a one-step analysis that compares the fair value of the intangible asset with its carrying amount. If the carrying amount of an intangible asset exceeds its fair value, an impairment loss is recognized in an amount equal to that excess. Several methods may be used to determine the estimated fair value of the IPR&D. We utilize the “income method”, and use estimated future net cash flows that are derived from projected sales revenues and estimated costs. These projections are based on factors such as relevant market size, patent protection, and expected pricing and industry trends. The estimated future net cash flows are then discounted to the present value using an appropriate discount rate. The estimated fair value is then compared to the carrying value of IPR&D.

We performed an annual impairment test for our indefinite-lived intangible assets as of October 1, 2022. In the fourth quarter of 2022, we recorded a partial impairment on the Upstaza indefinite lived intangible asset of $33.4 million, which is recorded as intangible asset impairment in the statement of operations. The impairment was related to a decrease in projected cash flows due to refinements in current market assumptions and the timing of patient treatments. To calculate the impairment amount, we utilized a discounted cash flow model under the income method, which primarily utilized Level 3 fair value inputs. Some of the more significant assumptions inherent in the development of the model included the estimated annual cash flows, particularly net revenues and operations costs, the appropriate discount rate to select in order to measure the risk inherent in the future cash flows, and the probability of success. Refer to note 18 for further information regarding our intangible assets.

For a description of our significant accounting policies, see note 2 to our consolidated financial statements.

Year ended December 31, 2022 compared to year ended December 31, 2021

The following table summarizes revenues and selected expense and other income data for the year ended December 31, 2022 and 2021:

(in thousands)	Year ended December 31, 2022	2021	Change 2022 vs. 2021
Net product revenue	$ 535,228	$ 428,904	$ 106,324
Collaboration revenue	50,052	55,046	$ (4,994)
Royalty revenue	113,521	54,643	$ 58,878
Cost of product sales, excluding amortization of acquired intangible assets	44,678	32,328	$ 12,350
Amortization of acquired intangible assets	116,554	54,751	$ 61,803
Research and development expense	651,496	540,684	$ 110,812
Selling, general and administrative expense	325,998	285,773	$ 40,225
Change in the fair value of deferred and contingent consideration	(25,900)	(500)	$ (25,400)
Intangible asset impairment	33,384	—	$ 33,384
Interest expense, net	(90,871)	(86,022)	$ (4,849)
Other expense, net	(49,207)	(57,875)	$ 8,668
Income tax benefit (expense)	28,470	(5,561)	$ 34,031

Net product revenue. Net product revenue was $535.2 million for the year ended December 31, 2022, an increase of $106.3 million, or 25%, from net product revenue of $428.9 million for the year ended December 31, 2021. Translarna net product revenues were $288.6 million for the year ended December 31, 2022, an increase of $52.6 million, or 22%, compared to $236.0 million for the year ended December 31, 2021. These results were driven by treatment of new patients in existing geographies and continued geographic expansion. Emflaza net product revenues were $218.3 million for the year ended December 31, 2022, an increase of $31.0 million, or 17%, compared to $187.3 million for the year ended December 31, 2021. These results were driven by new patient prescriptions, continued high compliance, and appropriate weight-based dosing. The remaining increase of $22.7 million was due to an increase in net product sales of Tegsedi, Waylivra, and Upstaza.

Collaboration revenue. Collaboration revenue was $50.1 million for the year ended December 31, 2022, a decrease of $5.0 million, or 9%, from collaboration revenue of $55.0 million for the year ended December 31, 2021. The decrease is due to a decrease in actual milestones that were achieved in the year ended December 31, 2022 compared to the year ended December 31, 2021, respectively. A sales milestone of $50.0 million was recognized for the achievement of $750.0 million in worldwide annual net sales from Evrysdi in the year ended December 31, 2022. In March 2021, the first commercial sale of Evrysdi in the EU was made. This event triggered a $20.0 million milestone payment to us from Roche. Additionally, in June 2021, the Japanese Ministry of Health, Labor and Welfare approved Evrysdi for the treatment of SMA in Japan. In August 2021, the first commercial sale of Evrysdi in Japan triggered a $10.0 million milestone payment to us from Roche. In December 2021, we recorded our first sales milestone of $25.0 million for the achievement of $500.0 million in worldwide annual net sales from Evrysdi.

Royalty revenue. Royalty revenue was $113.5 million for the years ended December 31, 2022, an increase of $58.9 million, or over 100%, from $54.6 million for the years ended December 31, 2021. The increase in royalty revenue was due to higher Evrysdi sales in the year ended December 31, 2022 as compared to the year ended December 31, 2021. In accordance with the SMA License Agreement, we are entitled to royalties on worldwide annual net sales of the product.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, was $44.7 million for the year end December 31, 2022, an increase of $12.4 million, or 38%, from $32.3 million for the year ended December 31, 2021. Cost of product sales consist primarily of royalty payments associated with Emflaza, Translarna and Upstaza net product sales, excluding contingent payments to Marathon, costs associated with Emflaza, Translarna and Upstaza product sold during the period, and royalty expense related to royalty revenues and collaboration milestone revenues. The increase in cost of product sales, excluding

amortization of acquired intangible asset, is primarily due to the increases in net product revenue, royalty revenues, and collaboration milestone revenue.

Amortization of acquired intangible asset. Amortization of acquired intangible asset was $116.6 million for the year ended December 31, 2022, an increase of $61.8 million, or over 100%, from $54.8 million for the year ended December 31, 2021. These amounts are related to the Emflaza rights acquisition, as well as the Waylivra, Tegsedi, and Upstaza intangible assets, which are all being amortized on a straight-line basis over their estimated useful lives. With the approval of Upstaza by the European Commission in July 2022, $89.6 million was reclassified from indefinite lived intangible assets to definite lived intangible assets, which is being amortized over its expected useful life. The amortization increase is primarily related to the additional Marathon contingent payments, which includes a $50.0 million contingent payment made in the year ended December 31, 2022.

Research and development expense. Research and development expense was $651.5 million for the year ended December 31, 2022, an increase of $110.8 million, or 20%, compared to $540.7 million for the year ended December 31, 2021. The increase in research and development expenses is primarily related to increased investment in research programs and advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $326.0 million for the year ended December 31, 2022, an increase of $40.2 million, or 14%, from $285.8 million for the year ended December 31, 2021. The increase reflects our continued investment to support our commercial activities including our expanding commercial portfolio.

Change in the fair value of deferred and contingent consideration. Change in the fair value of deferred and contingent consideration was a gain of $25.9 million for the year ended December 31, 2022, a change of $25.4 million, or over 100%, from a gain of $0.5 million for the year ended December 31, 2021. The change is related to the fair valuation of the potential future consideration to be paid to former equityholders of Agilis as a result of our merger with Agilis which closed in August 2018. Changes in the fair value were due to the re-calculation of discounted cash flows for the passage of time and changes to certain other estimated assumptions.

Intangible asset impairment. Intangible asset impairment was $33.4 million for the year ended December 31, 2022, an increase of $33.4 million, 100%, from intangible asset impairment of $0.0 million for the year ended December 31, 2021. The increase was due to a decrease in projected cash flows for the Upstaza indefinite lived intangible asset due to refinements in current market assumptions and the timing of patient treatments, resulting in a partial impairment.

Interest expense, net. Interest expense, net was $90.9 million for the year ended December 31, 2022, an increase of $4.8 million, 6%, from interest expense, net of $86.0 million for the year ended December 31, 2021. The increase in interest expense, net was primarily due to interest expense recorded from the senior secured term loan, partially offset by lower interest expense due to the repayment of the 2022 Convertible Notes.

Other expense, net. Other expense, net was $49.2 million for the year ended December 31, 2022, a decrease of $8.7 million, 15%, from other expense, net of $57.9 million for the year ended December 31, 2021. The decrease in other expense, net resulted primarily from an unrealized foreign exchange loss of $14.4 million and a non cash foreign currency remeasurement loss of $16.9 million from the remeasurement of our intercompany loan for the year end December 31, 2022, as compared to an unrealized foreign exchange loss of $41.0 million from the remeasurement of our intercompany loan for the year ended December 31, 2021. In addition, we had unrealized losses on our equity investments and convertible debt security in ClearPoint Neuro, Inc. (formerly MRI Interventions, Inc.), or ClearPoint, of $3.5 million and $5.8 million, respectively, for the year ended December 31, 2022, as compared to unrealized losses on our equity investments and convertible debt security in ClearPoint of $6.1 million and $8.3 million, respectively, for the year ended December 31, 2021.

Income tax benefit (expense). Income tax benefit was $28.5 million for the year ended December 31, 2022, a change of $34.0 million, or over 100%, from income tax expense of $5.6 million for the year ended December 31, 2021. The change in income tax benefit (expense) is primarily attributable to the partial amortization of an intangible which had

previously been classified as an indefinite lived intangible, and the subsequent release of a portion of the valuation allowance associated with this asset.

Year ended December 31, 2021 compared to year ended December 31, 2020

The following table summarizes revenues and selected expense and other income data for the years ended December 31, 2021 and 2020:

(in thousands)	Year ended December 31, 2021	2020	Change 2021 vs. 2020
Net product revenue	$ 428,904	$ 333,401	$ 95,503
Collaboration revenue	55,046	42,579	$ 12,467
Royalty revenue	54,643	4,786	$ 49,857
Cost of product sales, excluding amortization of acquired intangible assets	32,328	18,942	$ 13,386
Amortization of acquired intangible assets	54,751	36,892	$ 17,859
Research and development expense	540,684	477,643	$ 63,041
Selling, general and administrative expense	285,773	245,164	$ 40,609
Change in the fair value of deferred and contingent consideration	(500)	23,280	$ (23,780)
Settlement of deferred and contingent consideration	—	10,613	$ (10,613)
Interest expense, net	(86,022)	(56,352)	$ (29,670)
Other (expense) income, net	(57,875)	85,188	$ (143,063)
Income tax expense	(5,561)	(35,228)	$ 29,667

Net product revenue. Net product revenue was $428.9 million for the year ended December 31, 2021, an increase of $95.5 million, or 29%, from net product revenue of $333.4 million for the year ended December 31, 2020. Translarna net product revenues were $236.0 million for the year ended December 31, 2021, an increase of $44.1 million, or 23%, compared to $191.9 million for the year ended December 31, 2020. These results were driven by treatment of new patients, continued high compliance, and geographic expansion. Emflaza net product revenues were $187.3 million for the year ended December 31, 2021, an increase of $48.3 million, or 35%, compared to $139.0 million for the year ended December 31, 2021. These results were driven by continued new prescriptions, continued high compliance, and more favorable access. The remaining increase of $3.1 million was due to an increase in net product sales of Tegsedi and Waylivra.

Collaboration revenue. Collaboration revenue was $55.0 million for the year ended December 31, 2021, an increase of $12.5 million, or 29%, from collaboration revenue of $42.6 million for the year ended December 31, 2020. The increase is primarily related to three milestones that were triggered from Roche in the years ended December 31, 2021. In March 2021, the first commercial sale of Evrysdi in the EU was made. This event triggered a $20.0 million milestone payment to us from Roche. Additionally, in June 2021, the Japanese Ministry of Health, Labor and Welfare approved Evrysdi for the treatment of SMA in Japan. In August 2021, the first commercial sale of Evrysdi in Japan triggered a $10.0 million milestone payment to us from Roche. In December 2021, we recorded our first sales milestone of $25.0 million for the achievement of $500.0 million in worldwide annual net sales from Evrysdi. Comparatively, in the year ended December 31, 2020, the FDA approved Evrysdi for the treatment of SMA in adults and children two months and older in August 2020. The first commercial sale of Evrysdi in the United States was made in August 2020. This event triggered a $20.0 million milestone payment to us from Roche. In August 2020, the EMA accepted the MAA filed by Roche for Evrysdi for the treatment of SMA, which triggered a $15.0 million milestone payment to us from Roche. In October 2020, Chugai filed an NDA in Japan for Evrysdi for the treatment of SMA, which triggered a $7.5 million milestone payment to us from Roche.

Royalty revenue. Royalty revenue was $54.6 million for the years ended December 31, 2021, an increase of $49.9 million, or over 100%, from $4.8 million for the years ended December 31, 2020. The increase in royalty revenue was due to the FDA approval of Evrysdi in August 2020. In accordance with the SMA License Agreement, we are entitled to royalties on worldwide annual net sales of the product.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, was $32.3 million for the year end December 31, 2021, an increase of $13.4 million, or 71%, from $18.9 million for the year ended December 31, 2020. Cost of product sales consist primarily of royalty payments associated with Emflaza and Translarna net product sales, excluding contingent payments to Marathon, costs associated with Emflaza and Translarna product sold during the period, and royalty expense related to royalty revenues and collaboration milestone revenues. The increase in cost of product sales, excluding amortization of acquired intangible asset, is primarily due to the increases in net product revenue, royalty revenues, and collaboration milestone revenue.

Amortization of acquired intangible asset. Amortization of acquired intangible asset was $54.8 million for the year ended December 31, 2021, an increase of $17.9 million, or 48%, from $36.9 million for the year ended December 31, 2020. These amounts are related to the acquisition of all rights to Emflaza acquired in May 2017, Marathon contingent payments, and our Waylivra and Tegsedi intangible assets. The increase is primarily related to additional Marathon contingent payments. The amount allocated to the Emflaza intangible asset is amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of the acquisition of all rights to Emflaza, the period of estimated future cash flows. The Marathon contingent payments are amortized prospectively as incurred, straight-line, over the remaining useful life of the Emflaza intangible asset. The Waylivra and Tegsedi assets are amortized on a straight-line basis over their estimated useful life of approximately ten years, respectively. Additionally, in August 2021, we made a $4.0 million milestone payment to Akcea upon regulatory approval of Waylivra from ANVISA. In accordance with the guidance for an asset acquisition, we recorded the milestone payment when it became payable to Akcea, and it increased the cost basis for the Waylivra intangible asset. This payment is being amortized to cost of product sales over the expected remaining useful life of the Waylivra asset on a straight line basis.

Research and development expense. Research and development expense was $540.7 million for the year ended December 31, 2021, an increase of $63.0 million, or 13%, compared to $477.6 million for the year ended December 31, 2020. The increase in research and development expenses is primarily related to increased investment in research programs and advancement of the clinical pipeline. This increase was partially offset by one time charges in the year ended December 31, 2020 of $53.6 million for our Censa Merger, as well as $41.4 million for our commercial manufacturing service agreement with MassBio related to dedicated manufacturing space for our lead gene therapy program, AADC deficiency.

Selling, general and administrative expense. Selling, general and administrative expense was $285.8 million for the year ended December 31, 2021, an increase of $40.6 million, or 17%, from $245.2 million for the year ended December 31, 2020. The increase reflects our continued investment to support our commercial activities including our expanding commercial portfolio, including an increase in rent and related expenses associated with entering into a long term lease for the Hopewell Facility that commenced on July 1, 2020.

Change in the fair value of deferred and contingent consideration. Change in the fair value of deferred and contingent consideration was a gain of $0.5 million for the year ended December 31, 2021, a change of $23.8 million, or over 100%, from a loss of $23.3 million for the year ended December 31, 2020. The change is related to the fair valuation of the potential future consideration to be paid to former equityholders of Agilis as a result of our merger with Agilis which closed in August 2018. Changes in the fair value were due to the re-calculation of discounted cash flows for the passage of time and changes to certain other estimated assumptions.

Settlement of deferred and contingent consideration. Settlement of deferred and contingent consideration was $0.0 million for year ended December 31, 2021, a decrease of $10.6 million, or 100%, from $10.6 million for the year ended December 31, 2020. The settlement of deferred and contingent consideration for the year ended December 31, 2020 is related to a loss upon the settlement of the deferred and contingent consideration liabilities as a result of the Rights Exchange Agreement with certain former equityholders of Agilis, whereby we exchanged their pro rata share of specific future cash milestone payments in the aggregate amount of $225.0 million for a combination of cash and equity. We paid $36.9 million in cash and issued 2,821,176 shares of common stock in exchange for the cancellation and forfeiture of the Participating Rightholders' rights to receive (i) $174.0 million, in the aggregate, of potential milestone payments based on the achievement of certain regulatory milestones and (ii) $37.6 million, in the aggregate, of $40.0 million in development

milestone payments that would have been due upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the milestones are achieved.

Interest expense, net. Interest expense, net was $86.0 million for the year ended December 31, 2021, an increase of $29.7 million, 53%, from interest expense, net of $56.4 million for the year ended December 31, 2020. The increase in interest expense, net was primarily due to interest expense recorded from the liability for the sale of future royalties related to the Royalty Purchase Agreement, partially offset by a decrease in interest expense recorded from the 2022 and 2026 Convertible Notes as a result of the adoption of ASU 2020-06 and interest income from our investments.

Other (expense) income, net. Other expense, net was $57.9 million for the year ended December 31, 2021, a change of $143.1 million, over 100%, from other income, net of $85.2 million for the year ended December 31, 2020. The change in other (expense) income, net resulted primarily from an unrealized foreign exchange loss of $41.0 million from the remeasurement of our intercompany loan, which is recorded on a non-U.S. subsidiary and denominated in U.S. dollars, and unrealized losses on our equity investments and convertible debt security in ClearPoint Neuro, Inc. (formerly MRI Interventions, Inc.), or ClearPoint, of $6.1 million and $8.3 million, respectively.

Income tax expense. Income tax expense was $5.6 million for the year ended December 31, 2021, a decrease of $29.7 million, or 84%, from income tax expense of $35.2 million for the year ended December 31, 2020. We recorded a state income tax provision for the year ended December 31, 2020, which is attributable to the taxable income from the sale of our right to receive sales-based royalty payments on Roche's worldwide net sales of Evrysdi. We also incurred income tax expense in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses.

As a growing commercial-stage biopharmaceutical company, we are engaging in significant commercialization efforts for our products while also devoting a substantial portion of our efforts on research and development related to our products, product candidates and other programs. To date, our product revenue has primarily consisted of sales of Translarna for the treatment of nmDMD in territories outside of the United States and from Emflaza for the treatment of DMD in the United States. Our ongoing ability to generate revenue from sales of Translarna for the treatment of nmDMD is dependent upon our ability to maintain our marketing authorizations in Brazil, Russia and in the EEA and secure market access through commercial programs following the conclusion of pricing and reimbursement terms at sustainable levels in the member states of the EEA or through EAP programs in the EEA and other territories. The marketing authorization requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is subject to the specific obligation to conduct Study 041. Our ability to generate product revenue from Emflaza will largely depend on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors.

We have historically financed our operations primarily through the issuance and sale of our common stock in public offerings, our "at the market offering" of our common stock, proceeds from the Royalty Purchase Agreement, net proceeds from our borrowings under the Blackstone Credit Agreement, the private placements of our preferred stock and common stock, collaborations, bank and institutional lender debt, convertible debt financings and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. We expect to continue to incur significant expenses and operating losses for at least the next fiscal year. The net losses we incur may fluctuate significantly from quarter to quarter.

In August 2015, we closed a private placement of $150.0 million in aggregate principal amount of 3.00% convertible senior notes due 2022 including the exercise by the initial purchasers of an option to purchase an additional $25.0 million in aggregate principal amount of the 2022 Convertible Notes. On August 15, 2022, we repaid the outstanding principal amount and accrued interest, totaling $152.3 million, of the 2022 Convertible Notes that was due upon maturity

in accordance with the terms of the notes. While outstanding, the 2022 Convertible Notes bore cash interest at a rate of 3.00% per year, payable semi-annually on February 15 and August 15 of each year, beginning on February 15, 2016.

In August 2019, we entered into the Sales Agreement, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act. See "Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations—Overview—Funding" for additional information regarding the transactions described in this paragraph.

In September 2019, we issued $287.5 million aggregate principal amount of 2026 Convertible Notes, which included an option to purchase up to an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes, which was exercised in full by the initial purchasers. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. We received net proceeds of $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us.

Holders may convert their 2026 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding March 15, 2026 only under the following circumstances: (1) during any calendar quarter commencing on or after December 31, 2019 (and only during such calendar quarter), if the last reported sale price of our common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day; (2) during the five business day period after any five consecutive trading day period, or the measurement period, in which the trading price (as defined in the 2026 Convertible Notes Indenture) per $1,000 principal amount of 2026 Convertible Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price of our common stock and the conversion rate on each such trading day; (3) during any period after we have issued notice of redemption until the close of business on the scheduled trading day immediately preceding the relevant redemption date; or (4) upon the occurrence of specified corporate events. On or after March 15, 2026, until the close of business on the business day immediately preceding the maturity date, holders may convert their 2026 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, we will pay or deliver, as the case may be, cash, shares of our common stock or any combination thereof at our election.

The conversion rate for the 2026 Convertible Notes was initially, and remains, 19.0404 shares of our common stock per $1,000 principal amount of the 2026 Convertible Notes, which is equivalent to an initial conversion price of approximately $52.52 per share of our common stock. The conversion rate may be subject to adjustment in some events but will not be adjusted for any accrued and unpaid interest.

We are not permitted to redeem the 2026 Convertible Notes prior to September 20, 2023. We may redeem for cash all or any portion of the 2026 Convertible Notes, at our option, if the last reported sale price of its common stock has been at least 130% of the conversion price then in effect on the last trading day of, and for at least 19 other trading days (whether or not consecutive) during, any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which we provide notice of redemption, at a redemption price equal to 100% of the principal amount of the 2026 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the 2026 Convertible Notes, which means that we are not required to redeem or retire the 2026 Convertible Notes periodically.

If we undergo a "fundamental change" (as defined in the 2026 Convertible Notes Indenture), subject to certain conditions, holders of the 2026 Convertible Notes may require us to repurchase for cash all or part of their 2026 Convertible Notes at a repurchase price equal to 100% of the principal amount of the 2026 Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

The 2026 Convertible Notes represent senior unsecured obligations and will rank senior in right of payment to our future indebtedness that is expressly subordinated in right of payment to the notes, equal in right of payment to our existing

and future unsecured indebtedness that is not so subordinated, effectively junior in right of payment to any of our secured indebtedness to the extent of the value of the assets securing such indebtedness, and structurally subordinated to all existing and future indebtedness and other liabilities (including trade payables) incurred by our subsidiaries. The 2026 Convertible Notes Indenture contains customary events of default with respect to the 2026 Convertible Notes, including that upon certain events of default (including our failure to make any payment of principal or interest on the 2026 Convertible Notes when due and payable) occurring and continuing, the 2026 Convertible Notes Trustee by notice to us, or the holders of at least 25% in principal amount of the outstanding 2026 Convertible Notes by notice to us and the Convertible Notes Trustee, may, and the 2026 Convertible Notes Trustee at the request of such holders (subject to the provisions of the 2026 Convertible Notes Indenture) will, declare 100% of the principal of and accrued and unpaid interest, if any, on all the 2026 Convertible Notes to be due and payable. In case of certain events of bankruptcy, insolvency or reorganization, involving us or a significant subsidiary, 100% of the principal of and accrued and unpaid interest on the 2026 Convertible Notes will automatically become due and payable. Upon such a declaration of acceleration, such principal and accrued and unpaid interest, if any, will be due and payable immediately.

In July 2020, we entered into the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, we sold to RPI the Assigned Royalty Payment in consideration for $650.0 million. See “Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations—Overview—Funding” for additional information regarding this transaction.

In October 2022, we entered into the Blackstone Credit Agreement for fundings of up to $950.0 million consisting of a committed loan facility of $450.0 million and further contemplating the potential for up to $500.0 million of additional financing, to the extent that we request such additional financing and subject to the Lenders’ agreement to provide such additional financing and to mutual agreement on terms.

The Blackstone Credit Agreement provides for a senior secured term loan facility funded on the Closing Date in the aggregate principal amount of $300.0 million and a committed delayed draw term loan facility of up to $150.0 million to be funded at the Company’s request within 18 months of the Closing Date subject to specified conditions. In addition, the Blackstone Credit Agreement contemplates the potential for further financings by Blackstone, by providing for incremental discretionary uncommitted further financings of up to $500.0 million. We will be required under conditions specified in the Blackstone Credit Agreement to fund a reserve account up to certain amounts specified therein.

The Loans mature on the date that is seven years from the Closing Date. Borrowings under the Blackstone Credit Agreement bear interest at a variable rate equal to, at our option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively. Payment of the Loans are subject to certain premiums specified in the Blackstone Credit Agreement, in each case, from the date the applicable Loan is funded.

All obligations under the Blackstone Credit Agreement are secured, subject to certain exceptions and specified inclusions, by security interests in certain assets of the Loan Parties, including (1) intellectual property and other assets related to Translarna, Emflaza, Upstaza, sepiapterin and, until certain release conditions are met, vatiquinone, in each case, together with any other forms, formulations, or methods of delivery of any such products, and regardless of trade or brand name, (2) future acquired intellectual property (but not internally developed intellectual property unrelated to other intellectual property collateral) and other related assets, and (3) the equity interests held by the Loan Parties in certain of their subsidiaries. The Blackstone Credit Agreement contains certain negative covenants with which we must remain in compliance. The Blackstone Credit Agreement also requires that we maintain consolidated liquidity of at least $100.0 million as of the last day of each fiscal quarter, which shall be increased to $200.0 million upon our consummating acquisitions meeting certain consolidated thresholds described therein. In addition, we will be required under conditions specified in the Blackstone Credit Agreement to fund a reserve account up to certain amounts specified therein. The funds in the reserve account are available to prepay the Loans at any time at our option, and are, if funded, subject to release upon certain further conditions. Upon any such release, such funds are freely available for our use subject to the generally applicable terms and conditions of the Blackstone Credit Agreement. The Blackstone Credit Agreement contains certain customary representations and warranties, affirmative covenants and provisions relating to events of default. In connection with the execution of the Blackstone Credit Agreement, we and the Purchasers also entered into the Stock Purchase

Agreement for the sale and issuance of 1,095,290 shares of common stock to the Purchasers at a price of $45.65 per share, for an aggregate purchase price of approximately $50.0 million. The per share price represents the closing price of our common stock on the Nasdaq Global Select Market on October 26, 2022.

Cash flows

As of December 31, 2022, we had cash and cash equivalents and marketable securities of $410.7 million.

The following table provides information regarding our cash flows and our capital expenditures for the periods indicated.

	Years ended December 31,		
(in thousands)	**2022**	**2021**	**2020**
Cash (used in) provided by:			
Operating activities	$ (356,654)	$ (251,332)	$ (194,071)
Investing activities	$ 290,181	$ 219,182	$ (561,548)
Financing activities	$ 167,952	$ 20,877	$ 668,715

Net cash used in operating activities was $356.7 million, $251.3 million, and $194.1 million for the years ended December 31, 2022, 2021, and 2020, respectively. The cash used in operating activities primarily related to supporting clinical development, including the manufacture of drug product, commercial activities for Emflaza, Translarna, and Upstaza, and costs associated with the expansion of our international infrastructure for the years ended December 31, 2022, 2021, and 2020.

Net cash provided by investing activities was $290.2 million and 219.2 million for the years ended December 31, 2022 and 2021, respectively. Net cash used in investing activities was $561.5 million for the year ended December 31, 2020. The cash provided by investing activities for the years ended December 31, 2022 and December 31, 2021 were primarily related to net sales and redemptions of marketable securities, partially offset by purchases of marketable securities, purchases of fixed assets and the acquisition of product rights. The cash used in investing activities for the year ended December 31, 2020 was primarily related to purchases of marketable securities, the acquisition of product rights, purchases of fixed assets, and our purchase of convertible debt security, partially offset by net sales and redemptions of marketable securities.

Net cash provided by financing activities was $168.0 million, $20.9 million, and $668.7 million for the years ended December 31, 2022, 2021 and 2020, respectively. The cash provided by financing activities for the year ended December 31, 2022 is primarily attributable to the proceeds from the issuance of the senior secured term loan under the Blackstone Credit Agreement, the Stock Purchase Agreement, the exercise of options, and issuance of stock under our Employee Stock Purchase Plan, or ESPP, offset by the repayment of the 2022 Convertible Notes, payments on contingent consideration obligation, and payments on our finance lease principal. The cash provided by financing activities for the year ended December 31, 2021 is primarily attributable to the exercise of options, and issuance of stock under our ESPP offset by payments on our finance lease principal. Net cash provided by financing activities for the year ended December 31, 2020 is primarily attributable to proceeds from the Royalty Purchase Agreement, net proceeds received from our “at the market offering” of our common stock, the exercise of options, and issuance of stock under our ESPP, partially offset by repayment on our senior secured term loan, payments on deferred consideration obligation, and payments on our finance lease principal.

Funding requirements

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with the research and development of our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products

for maintaining authorizations, including Study 041, label extensions and additional indications. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in. We are also preparing a BLA for Upstaza for the treatment of AADC deficiency in the United States and we expect to submit a BLA to the FDA in the first half of 2023. These efforts may significantly impact the timing and extent of our commercialization expenses.

In addition, our expenses will increase if and as we:

- seek to satisfy contractual and regulatory obligations that we assumed through our acquisitions and collaborations;
- execute our commercialization strategy for our products, including initial commercialization launches of our products, label extensions or entering new markets;
- are required to complete any additional clinical trials, non-clinical studies or Chemistry, Manufacturing and Controls, or CMC, assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- are required to take other steps, in addition to Study 041, to maintain our current marketing authorization in the EEA, Brazil and Russia for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications;
- utilize the Hopewell Facility to manufacture program materials for certain of our gene therapy product candidates as well as program materials for third parties;
- initiate or continue the research and development of our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

We believe that our cash flows from product sales, together with existing cash and cash equivalents, including our offering of the 2026 Convertible Notes, public offerings and private placements of common stock, our “at the market offering” of our common stock, proceeds from the Royalty Purchase Agreement, net proceeds from our borrowings under the Blackstone Credit Agreement and marketable securities, will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next twelve months. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect.

Our future capital requirements will depend on many factors, including:

- our ability to commercialize and market our products and product candidates that may receive marketing authorization;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for our products and products candidates;

- our ability to maintain the marketing authorization for our products, including in the EEA for Translarna for the treatment of nmDMD and whether the EMA determines on an annual basis that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA, on the current approved label;
- the costs, timing and outcome of Study 041;
- our ability to obtain marketing authorization for Upstaza for the treatment of AADC deficiency in the United States;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA re-submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- unexpected decreases in revenue or increase in expenses resulting from the COVID-19 pandemic or other potential widespread outbreaks of contagious disease;
- our ability to maintain orphan exclusivity in the United States for Emflaza;
- our ability to successfully complete all post-marketing requirements imposed by regulatory agencies with respect to our products;
- the progress and results of activities under our splicing, gene therapy, Bio-e, metabolic and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for any of our products and for any of our other product candidates that may receive marketing authorization or any additional territories in which we receive authorization to market Translarna;
- the costs, timing and outcome of regulatory review of our splicing, gene therapy, Bio-e, metabolic and oncology programs and Translarna in other territories;
- our ability to utilize the Hopewell Facility to manufacture program materials for certain of our gene therapy product candidates and program materials for third parties;
- our ability to satisfy our obligations under the Blackstone Credit Agreement;
- our ability to satisfy our obligations under the indentures governing the 2026 Convertible Notes;
- the timing and scope of growth in our employee base;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates, including those in our splicing, gene therapy, Bio-e, metabolic and oncology programs;
- revenue received from commercial sales of our products or any of our product candidates;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;
- the ability and willingness of patients and healthcare professionals to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products, product candidates, and technologies, including the success of any acquisition, in-licensing or other strategic transaction we may pursue, and the costs of subsequent development requirements and commercialization efforts, including with respect to our acquisitions of Emflaza, Agilis, our Bio-E platform and Censa and our licensing of Tegsedi and Waylivra; and
- our ability to establish and maintain collaborations, including our collaborations with Roche and the SMA Foundation, and our ability to obtain research funding and achieve milestones under these agreements.

With respect to our outstanding 2026 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.3 million annually. On August 15, 2022, we repaid the outstanding principal amount and accrued interest, totaling $152.3 million, of the 3.00% convertible senior notes due August 15, 2022, or the 2022 Convertible Notes, that was due upon maturity in accordance with the terms of the 2022 Convertible Notes.

Borrowings under the Blackstone Credit Agreement bear interest at a variable rate equal to, at our option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively.

In October 2022, we paid the former equityholders of Agilis $50.0 million in regulatory milestone payments as a result of the European Commission's marketing approval of Upstaza for the treatment of AADC deficiency in July 2022. In 2022, we also paid Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, a single $50.0 million sales-based milestone in accordance with the Emflaza Asset Purchase Agreement.

In February 2023, we completed enrollment of our Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event and in accordance with the Censa Merger Agreement, we are obligated to pay a $30.0 million development milestone to the former Censa securityholders, which we have the option to pay in cash or shares of our common stock.

We expect to make additional payments to the former Censa securityholders of $50.0 million in the aggregate upon the achievement of certain development and regulatory milestones in 2023 relating to sepiapterin. We also expect to pay the former equityholders of Agilis an additional $20.0 million upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency, which we expect to occur in the first half of 2023. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such including significant legal, accounting, investor relations and other expenses.

We also have certain significant contractual obligations and commercial commitments that require funding. We lease office space for our principal office in South Plainfield, New Jersey and we occupy under leases that expire in 2024, with two consecutive five-year renewal options to renew the leases after 2024. Additionally, we entered into a lease agreement for approximately 103,000 square feet of laboratory and office space in Bridgewater, New Jersey. The rental term for such facility commenced on May 1, 2020 with an initial term of seven years and two consecutive five year renewal periods at our option. We have significant lease obligations that stem from our lease of office, manufacturing, and laboratory space in Hopewell, New Jersey. The rental term for such facility commenced on July 1, 2020, with an initial term of fifteen years and two consecutive 10-year renewal periods at our option. We also lease two entire buildings comprised of approximately 360,000 square feet of shell condition, modifiable space at a facility located in Warren, New Jersey. The rental term for such facility commenced on June 1, 2022, with an initial term of seventeen years followed by three consecutive five-year renewal periods at our option. In addition, we lease office space, vehicles and equipment in various other locations in the U.S. and other countries for our employees and operations. We have a total of $268.0 million in obligations that stem from our operating leases.

We have a total of $30.0 million in obligations that stem from a commercial manufacturing services agreement entered into with MassBio on June 19, 2020, for a term of 12.5 years. Pursuant to the terms of the agreement, MassBio agreed to provide us with four dedicated rooms for our Upstaza program.

Under an Exclusive License and Supply Agreement, or the Faes Agreement, with Faes Farma, S.A., or Faes, we are required to pay royalties as a percentage of or as a fixed payment with respect to net product sales by us allocable to the Emflaza oral suspension product. We are required to pay Faes an annual minimum royalty during the first seven calendar years with a fixed percentage royalty during the remainder of the Faes Agreement term. The minimum royalty based on the euro to U.S. dollar exchange rate as of December 31, 2022 is $1.6 million, with the last minimum royalty payment due in 2023.

Under various agreements, we will be required to pay royalties and milestone payments upon the successful development and commercialization of products, including the following agreements with The Wellcome Trust Limited, or Wellcome Trust, and the SMA Foundation.

We have entered into funding agreements with Wellcome Trust for the research and development of small molecule compounds in connection with our oncology platform and antibacterial program. As we have discontinued development under our antibacterial program, we do not expect that milestone and royalty payments from us to Wellcome Trust will apply under that agreement. Under our oncology platform funding agreement, to the extent that we develop and commercialize certain program intellectual property on a for-profit basis ourselves or in collaboration with a partner (provided we retain overall control of worldwide commercialization), we may become obligated to pay to Wellcome Trust development and regulatory milestone payments and single-digit royalties on sales of any research program product. Our obligation to pay such royalties would continue on a country-by-country basis until the longer of the expiration of the last

patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country. We made the first development milestone payment of $0.8 million to Wellcome Trust under the oncology platform funding agreement during the second quarter of 2016. During the year ended December 31, 2022, the Company incurred $2.5 million of development milestones in connection with the enrollment of patients in the registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS, which is recorded in other long-term liabilities on the balance sheet and will be payable upon the earlier to occur of the first dose administered to the last patient enrolled in the study or the termination of dosing of all patients in the study. Additional milestone payments of up to an aggregate of $14.5 million may become payable by the Company to Wellcome Trust under this agreement.

We have also entered into a sponsored research agreement with the SMA Foundation in connection with our spinal muscular atrophy program. We may become obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that we successfully develop and subsequently commercialize or, with respect to collaboration products we outlicense, including Evrysdi, a specified percentage of certain payments we receive from our licensee. We are not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. Since inception, the SMA Foundation has earned $28.5 million, $24.5 million which was paid and $4.0 million which was accrued as of December 31, 2022. Our obligation to make such payments would end upon our payment to the SMA Foundation of an aggregate of $52.5 million.

Additionally, we have employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such, including significant legal, accounting, investor relations and other expenses.

We have never been profitable and we will need to generate significant revenues to achieve and sustain profitability, and we may never do so. We may need to obtain substantial additional funding in connection with our continuing operations. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs primarily through a combination of equity offerings, debt financings, collaborations, strategic alliances, grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product and product candidates and marketing, distribution or licensing arrangements. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our shareholders ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

If we are unable to raise additional funds through equity, debt or other financings when needed or on attractive terms, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Item 7A. Quantitative and Qualitative Disclosures about Market Risk

We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. At any time, sharp changes in interest rates can affect the fair value of the investment portfolio and its interest earnings. There were no investments classified as long-term at December 31, 2022. At December 31, 2022, we held $410.7 million in cash and cash equivalents and short-term investments. After a review of our marketable investment securities, we believe that in the event of a hypothetical ten percent increase in interest rates, the resulting decrease in fair value of our marketable investment securities would be insignificant to the consolidated financial statements.

Currently, we do not hedge these interest rate exposures. We maintain an investment portfolio in accordance with our investment policy. The primary objectives of our investment policy are to preserve principal, maintain proper liquidity and to meet operating needs. Although our investments are subject to credit risk, our investment policy specifies credit quality standards for our investments and limits the amount of credit exposure from any single issue, issuer or type of investment. Our investments are also subject to interest rate risk and will decrease in value if market interest rates increase. However, due to the conservative nature of our investments and relatively short duration, interest rate risk is mitigated. We do not own derivative financial instruments. Accordingly, we do not believe that there is any material market risk exposure with respect to derivative or other financial instruments.

As a result of our ex-U.S. operations, we face exposure to movements in foreign currency exchange rates, including the British Pound, Euro, Brazilian Real, Swiss Franc, and Russian Ruble against the U.S. dollar. The current exposures arise primarily from cash, accounts receivable, intercompany receivables and payables, intercompany loans and product sales denominated in foreign currencies. Both positive and negative impacts to our international product sales from movements in foreign currency exchange rates may be partially mitigated by the natural, opposite impact that foreign currency exchange rates have on our international operating expenses. For the year ended December 31, 2022, we recognized realized foreign currency transaction losses, net, of $19.6 million, which is recorded within other income, net on the Statement of Operations. A hypothetical ten percent increase or decrease in the exchange rate between the U.S. dollar and the British Pound, Euro, Brazilian Real, Swiss Franc, or Russian Ruble from the December 31, 2022 rate would not have a significant impact on our cash flows. We are not currently engaged in any foreign currency hedging activities. We will evaluate the use of derivative financial instruments to hedge our exposure as the needs and risks should arise.

In September 2019, we issued $287.5 million of 1.50% convertible senior notes due September 15, 2026, or the 2026 Convertible Notes. We do not have economic interest rate exposure on the 2026 Convertible Notes as they have a fixed annual interest rate of 1.50%. However, the fair value of the 2026 Convertible Notes is exposed to interest rate risk. We do not carry the 2026 Convertible Notes at fair value on our balance sheet but present the fair value of the principal amount for disclosure purposes. Generally, the fair value of the 2026 Convertible Notes will increase as interest rates fall and decrease as interest rates rise. The 2026 Convertible Notes are also affected by the price and volatility of our common stock and will generally increase or decrease as the market price of our common stock changes. The estimated fair value of the Convertible Notes was approximately $281.7 million as of December 31, 2022.

Item 8. Financial Statements and Supplementary Data

Index to consolidated financial statements

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of PTC Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of PTC Therapeutics, Inc. (the Company) as of December 31, 2022 and 2021, the related consolidated statements of operations, comprehensive loss, stockholders' (deficit)/ equity and cash flows for each of the three years in the period ended December 31, 2022, and the related notes (collectively referred to as the “consolidated financial statements”). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2022 and 2021, and the consolidated results of its operations and its cash flows for each of the three years in the period ended December 31, 2022, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2022, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) and our report dated February 21, 2023 expressed an unqualified opinion thereon.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company’s financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Adoption of ASU No. 2020-06

As discussed in Note 8 to the consolidated financial statements, the Company changed its method of accounting for convertible notes in 2021 due to the adoption of Accounting Standards Update (ASU) No. 2020-06, Debt— (Subtopic 470-20 & 815-40), and the related amendments.

Critical Audit Matters

The critical audit matters communicated below are matters arising from the current period audit of the financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matters below, providing separate opinions on the critical audit matters or on the accounts or disclosures to which they relate.

Variable consideration in contracts with customers

Description of the Matter

As discussed in Note 2 of the consolidated financial statements, the Company's revenues for product sold to its customers within the United States reflect discounts mandated by the Medicaid Drug Rebate Program. The Company includes an estimate of this variable consideration in its transaction price at the time of sale when control of the product transfers to the customer. The Company uses the expected value or most likely amount method when estimating variable consideration, unless discount or rebate terms are specified within contracts. The estimates for variable consideration are adjusted to reflect known changes.

Auditing the amount of consideration to be paid under the Medicaid Drug Rebate Program (Medicaid) was complex and highly judgmental due to significant uncertainty about the volume of expected claims from governmental entities, the estimated amount of shipments from wholesalers that will be dispensed to eligible benefit plan participants, as well as the complexity of governmental pricing calculations in various jurisdictions. Governmental pricing calculations are complex as a result of assumptions such as patient mix, the average manufacturer price, best price, and the unit rebate amount. The reductions to gross product revenues are sensitive to these significant estimates and calculations.

How We Addressed the Matter in Our Audit

We identified, evaluated and tested controls over management's review of the calculated reductions to gross product prices related to Medicaid and the significant assumptions and data inputs utilized in the calculations.

To test the revenue adjustments related to Medicaid our audit procedures included, among others, evaluating the methodology used as well as testing the significant estimates discussed above and the underlying assumptions and data used by the Company in its analysis. We compared the assumptions used by management to historical claims and payment trends, evaluated pricing adjustments recorded in the current period, and assessed the historical accuracy of management's estimates against actual results. In addition, we involved an internal governmental pricing specialist to assist with our evaluation of management's methodology and the calculations made to measure the estimated Medicaid rebates.

Valuation of acquisition-related contingent consideration liability

Description of the Matter

As discussed in Note 2 to the consolidated financial statements under the caption "Business combinations and asset acquisitions," the Company recognizes contingent consideration liabilities at their estimated fair values on the acquisition date. Subsequent changes to the fair values of the contingent consideration liabilities are recorded within the consolidated statement of operations in the period of change. At December 31, 2022, the Company recorded $164.0 million in total contingent consideration liabilities related to development, regulatory and net sales milestones.

The fair value of the contingent consideration is estimated using a combination of a probability adjusted, discounted cash flow approach and an option pricing model with Monte Carlo simulation. Certain assumptions, including development timelines, probabilities of success, and certain inputs to the weighted average cost of capital are highly subjective and the fair value estimate is sensitive to these assumptions. Auditing the valuation of contingent consideration liabilities was complex and required significant auditor judgment due to the high degree of subjectivity in evaluating these assumptions and the method used for the calculation.

How We Addressed the Matter in Our Audit

We obtained an understanding, evaluated the design and tested the operating effectiveness of controls over the Company's contingent consideration liabilities process including management's process to establish the significant assumptions and measure the liability.

To test the estimated fair value of the contingent consideration liabilities, our audit procedures included, among others, assessing the fair value methodology and testing the significant assumptions discussed above and the underlying data used in management's analyses. We evaluated the assumptions and judgments in light of observable industry and economic trends and standards, external data sources and regulatory factors. Estimated amounts of future sales and probabilities of achieving milestones were evaluated in relation to internal and external analyses, clinical development progress and timelines, probability of success benchmarks, and regulatory notices. Additionally, we compared the weighted average cost of capital that was adjusted for the Company's credit risk, to those of comparable guideline companies. Our procedures also included evaluating the data sources used by management in determining its assumptions and, where necessary, included an evaluation of available information that either corroborated or contradicted management's conclusions. We involved valuation specialists to assist with our assessment of the Company's fair value measurement methodology and to perform corroborative fair value calculations.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2010.

Iselin, New Jersey

February 21, 2023

PTC Therapeutics, Inc.

Consolidated Balance Sheets

In thousands, except shares

	December 31, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 279,834	$ 189,718
Marketable securities	130,871	583,658
Trade and royalty receivables, net	155,614	110,455
Inventory, net	21,808	15,856
Prepaid expenses and other current assets	105,658	54,681
Total current assets	693,785	954,368
Fixed assets, net	72,590	52,585
Intangible assets, net	705,891	724,841
Goodwill	82,341	82,341
Operating lease ROU assets	102,430	77,421
Deposits and other assets	48,582	46,500
Total assets	$ 1,705,619	$ 1,938,056
Liabilities and stockholders' (deficit) equity		
Current liabilities:		
Accounts payable and accrued expenses	320,366	288,784
Current portion of long-term debt	—	149,540
Deferred revenue	1,351	—
Operating lease liabilities- current	9,370	7,273
Finance lease liabilities- current	3,000	3,000
Liability for sale of future royalties- current	72,149	59,291
Other current liabilities	—	1,460
Total current liabilities	406,236	509,348
Long-term debt	571,722	281,894
Contingent consideration payable	164,000	239,900
Deferred tax liability	102,834	137,110
Operating lease liabilities- noncurrent	100,860	73,619
Finance lease liabilities- noncurrent	18,675	20,053
Liability for sale of future royalties- noncurrent	685,737	674,694
Other long-term liabilities	2,641	—
Total liabilities	2,052,705	1,936,618
Stockholders' (deficit) equity:		
Common stock, $0.001 par value. Authorized 250,000,000 shares; issued and outstanding 73,104,692 shares at December 31, 2022. Authorized 250,000,000 shares; issued and outstanding 70,828,226 shares at December 31, 2021.	72	71
Additional paid-in capital	2,305,020	2,123,606
Accumulated other comprehensive income (loss)	4,796	(24,282)
Accumulated deficit	(2,656,974)	(2,097,957)
Total stockholders' (deficit) equity	(347,086)	1,438
Total liabilities and stockholders' (deficit) equity	$ 1,705,619	$ 1,938,056

See accompanying consolidated notes.

PTC Therapeutics, Inc.

Consolidated Statements of Operations

In thousands, except shares and per share data

	Year ended December 31,		
	2022	2021	2020
Revenues:			
Net product revenue	$ 535,228	$ 428,904	$ 333,401
Collaboration revenue	50,052	55,046	42,579
Royalty revenue	113,521	54,643	4,786
Total revenues	698,801	538,593	380,766
Operating expenses:			
Cost of product sales, excluding amortization of acquired intangible assets	44,678	32,328	18,942
Amortization of acquired intangible assets	116,554	54,751	36,892
Research and development	651,496	540,684	477,643
Selling, general and administrative	325,998	285,773	245,164
Change in the fair value of deferred and contingent consideration	(25,900)	(500)	23,280
Intangible asset impairment	33,384	—	—
Settlement of deferred and contingent consideration	—	—	10,613
Total operating expenses	1,146,210	913,036	812,534
Loss from operations	(447,409)	(374,443)	(431,768)
Interest expense, net	(90,871)	(86,022)	(56,352)
Other (expense) income, net	(49,207)	(57,875)	85,188
Loss before income tax expense	(587,487)	(518,340)	(402,932)
Income tax benefit (expense)	28,470	(5,561)	(35,228)
Net loss attributable to common stockholders	$ (559,017)	$ (523,901)	$ (438,160)
Weighted-average shares outstanding:			
Basic and diluted (in shares)	71,728,634	70,466,393	66,027,908
Net loss per share—basic and diluted (in dollars per share)	$ (7.79)	$ (7.43)	$ (6.64)

See accompanying consolidated notes.

PTC Therapeutics, Inc.

Consolidated Statements of Comprehensive Loss

In thousands

	Year ended December 31,		
	2022	2021	2020
Net loss	$ (559,017)	$ (523,901)	$ (438,160)
Other comprehensive income (loss):			
Unrealized gain (loss) on marketable securities, net of tax	108	(2,502)	1,145
Foreign currency translation gain (loss), net of tax	28,970	39,177	(51,518)
Comprehensive loss	$ (529,939)	$ (487,226)	$ (488,533)

See accompanying consolidated notes.

PTC Therapeutics, Inc.

Consolidated Statements of Stockholders' Equity/ (Deficit)

In thousands, except shares

	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity (deficit)
	Shares	Amount				
Balance, December 31, 2019	61,935,870	$ 62	$ 1,795,351	$ (10,584)	$ (1,190,499)	$ 594,330
Issuance of common stock related to equity offerings	542,470	1	28,091	—	—	28,092
Issuance of common stock related to acquisition	845,364	1	42,868	—	—	42,869
Issuance of common stock related to rights exchange	2,821,176	3	150,525	—	—	150,528
Exercise of options	3,268,452	3	70,176	—	—	70,179
Restricted stock vesting and issuance, net	180,028	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	124,736	—	5,303	—	—	5,303
Share-based compensation expense	—	—	70,325	—	—	70,325
Receivable from investor	—	—	9,107	—	—	9,107
Other	—	—	—	—	(218)	(218)
Net loss	—	—	—	—	(438,160)	(438,160)
Comprehensive loss	—	—	—	(50,373)	—	(50,373)
Balance, December 31, 2020	69,718,096	$ 70	$ 2,171,746	$ (60,957)	$ (1,628,877)	$ 481,982
Exercise of options	635,871	1	17,308	—	—	17,309
Restricted stock vesting and issuance, net	307,658	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	166,601	—	5,792	—	—	5,792
Share-based compensation expense	—	—	103,513	—	—	103,513
Receivable from investor	—	—	483	—	—	483
Adjustment for the adoption of ASU 2020-06	—	—	(175,236)	—	54,796	(120,440)
Other	—	—	—	—	25	25
Net loss	—	—	—	—	(523,901)	(523,901)
Comprehensive income	—	—	—	36,675	—	36,675
Balance, December 31, 2021	70,828,226	$ 71	$ 2,123,606	$ (24,282)	$ (2,097,957)	$ 1,438
Issuance of common stock related to stock purchase agreement	1,095,290	1	49,999	—	—	50,000
Exercise of options	496,863	—	14,632	—	—	14,632
Restricted stock vesting and issuance, net	490,008	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	194,305	—	6,450	—	—	6,450
Share-based compensation expense	—	—	110,333	—	—	110,333
Net loss	—	—	—	—	(559,017)	(559,017)
Comprehensive income	—	—	—	29,078	—	29,078
Balance, December 31, 2022	73,104,692	$ 72	$ 2,305,020	$ 4,796	$ (2,656,974)	$ (347,086)

See accompanying consolidated notes.

PTC Therapeutics, Inc.

Consolidated Statements of Cash Flows

In thousands

	Year ended December 31,		
	2022	2021	2020
Cash flows from operating activities			
Net loss	$ (559,017)	$ (523,901)	$ (438,160)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	128,836	64,134	43,490
Non-cash operating lease expense	9,884	7,386	6,084
Non-cash finance lease amortization expense	—	—	41,382
Non-cash royalty revenue related to sale of future royalties	(48,738)	(23,460)	(2,055)
Non-cash interest expense on liability related to sale of future royalties	72,639	77,683	31,817
Intangible asset impairment	33,384	—	—
Change in valuation of deferred and contingent consideration	(25,900)	(500)	23,280
Settlement of deferred and contingent consideration	—	—	10,613
Non-cash stock consideration, acquisition	—	—	42,869
Unrealized loss (gain) on ClearPoint Equity Investments	3,560	6,078	(14,310)
Unrealized loss (gain) on ClearPoint convertible debt security	5,740	8,281	(19,252)
Unrealized loss (gain) on marketable securities- equity investments	7,992	(1,673)	—
Disposal of asset	80	—	16
Deferred income taxes	(34,276)	377	5,872
Amortization of premiums on investments, net	1,713	5,299	409
Amortization of debt issuance costs	1,901	1,848	1,020
Share-based compensation expense	110,333	103,513	70,325
Non-cash interest expense	—	—	22,598
Unrealized foreign currency transaction losses (gains), net	13,263	47,391	(56,980)
Non-cash foreign currency remeasurement loss on intercompany loan	16,887	—	—
Changes in operating assets and liabilities:			
Inventory, net	(6,668)	1,800	1,841
Prepaid expenses and other current assets	(51,621)	(15,310)	(12,621)
Trade and royalty receivables, net	(48,468)	(44,991)	(10,483)
Deposits and other assets	(2,913)	(232)	(662)
Accounts payable and accrued expenses	27,542	45,659	71,963
Other liabilities	(4,558)	(6,704)	(3,930)
Deferred revenue	1,351	(4,010)	(8,032)
Payments on contingent consideration	(9,600)	—	(1,165)
Net cash used in operating activities	(356,654)	(251,332)	(194,071)
Cash flows from investing activities			
Purchases of fixed assets	(32,016)	(28,213)	(17,843)
Purchases of marketable securities- available for sale	(52,764)	(333,148)	(1,439,665)
Purchase of convertible debt security	—	—	(10,000)
Purchases of marketable securities- equity investments	(22,787)	(210,018)	—
Sale and redemption of marketable securities- available for sale	405,234	843,498	944,094
Sale and redemption of marketable securities- equity investments	112,958	4,281	—
Acquisition of product rights and licenses	(120,444)	(57,118)	(38,134)
Purchase of equity investment in ClearPoint	—	(100)	—
Net cash provided by (used in) investing activities	290,181	219,182	(561,548)
Cash flows from financing activities			
Proceeds from exercise of options	14,632	17,309	70,179
Termination and exit fees related to payoff of senior secured term loan	—	—	(597)
Net proceeds from public offerings	—	—	28,092
Debt issuance costs related to senior secured term loan	(11,454)	—	—
Proceeds from issuance of senior secured term loan	300,000	—	—
Repayment of Convertible Notes	(150,000)	—	(28,333)
Payments on deferred and contingent consideration obligation	(40,400)	—	(38,100)
Proceeds from employee stock purchase plan	6,450	5,792	5,303
Payment of finance lease principal	(1,276)	(2,224)	(17,829)
Proceeds from stock purchase agreement	50,000	—	—
Cash consideration received from Royalty Purchase Agreement	—	—	650,000
Net cash provided by financing activities	167,952	20,877	668,715
Effect of exchange rate changes on cash	(2,772)	(7,821)	7,688
Net increase (decrease) in cash and cash equivalents	98,707	(19,094)	(79,216)
Cash and cash equivalents, and restricted cash beginning of period	197,218	216,312	295,528
Cash and cash equivalents, and restricted cash end of period	$ 295,925	$ 197,218	$ 216,312

Supplemental disclosure of cash information			
Cash paid for interest	$ 18,463	$ 9,588	$ 9,802
Cash paid for income taxes	$ 4,922	$ 7,708	$ 26,397
Supplemental disclosure of non-cash investing and financing activity			
Unrealized gain (loss) on marketable securities, net of tax	$ 108	$ (2,502)	$ 1,145
Right-of-use assets obtained in exchange for operating lease obligations	$ 35,817	$ 645	$ 76,811
Right-of-use assets obtained in exchange for finance lease obligations	$ —	$ —	$ 41,382
Acquisition of product rights and licenses	33,239	22,294	14,191
Issuance of common stock related to rights exchange	$ —	$ —	$ 150,528
Debt issuance costs related to senior secured term loan	159	—	—
Capital expenditures unpaid at the end of period	$ 308	$ —	$ 1,060

See accompanying consolidated notes.

PTC Therapeutics, Inc.

Notes to consolidated financial statements

December 31, 2022

(In thousands except share and per share amount)

1. The Company

PTC Therapeutics, Inc. (the "Company" or "PTC") is a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes that this allows it to maximize value for all of its stakeholders.

The Company has two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy ("DMD"), a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area (the "EEA") for the treatment of nonsense mutation Duchenne muscular dystrophy ("nmDMD") in ambulatory patients aged 2 years and older and in Russia for the treatment of nmDMD in patients aged two years and older. In July 2020, the European Commission approved the removal of the statement "efficacy has not been demonstrated in non-ambulatory patients" from the indication statement for Translarna. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. Emflaza is approved in the United States for the treatment of DMD in patients two years and older.

The Company's marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, which the Company refers to as the annual EMA reassessment. The marketing authorization in the EEA was last renewed in June 2022 and is effective, unless extended, through August 5, 2023. In September 2022, the Company submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. The Company expects an opinion from the Committee for Medicinal Products for Human Use in the first half of 2023.

Translarna is an investigational new drug in the United States. Following the Company's announcement of top-line results from the placebo-controlled trial of Study 041 in June 2022, the Company submitted a meeting request to the U.S. Food and Drug Administration ("FDA") to gain clarity on the regulatory pathway for a potential re-submission of New Drug Application ("NDA") for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support NDA re-submission. The Company recently had an informal meeting with the FDA, during which the Company discussed the potential path to an NDA re-submission for Translarna. Based on the meeting discussion, the Company plans to request an additional Type C meeting with the FDA in the near future to review the totality of data collected to date, including dystrophin and other mechanistic data as well as additional analyses that could support the benefit of Translarna.

The Company has a pipeline of gene therapy product candidates for rare monogenic diseases that affect the central nervous system ("CNS") including Upstaza (eladocagene exuparvovec) for the treatment of Aromatic L-Amino Acid Decarboxylase ("AADC") deficiency ("AADC deficiency"), a rare CNS disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. The Company is also preparing a biologics license application

PTC Therapeutics, Inc.

Notes to consolidated financial statements (Continued)

December 31, 2022

(In thousands except share and per share amount)

("BLA") for Upstaza for the treatment of AADC deficiency in the United States and it expects to submit a BLA to the FDA in the first half of 2023.

The Company holds the rights for the commercialization of Tegsedi® (inotersen) and Waylivra® (volanesorsen) for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to the Collaboration and License Agreement (the "Tegsedi-Waylivra Agreement"), dated August 1, 2018, by and between the Company and Akcea Therapeutics, Inc. ("Akcea"), a subsidiary of Ionis Pharmaceuticals, Inc. Tegsedi has received marketing authorization in the United States, the European Union (the "EU") and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis ("hATTR amyloidosis"). The Company began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and it continues to make Tegsedi available in certain other countries within Latin America and the Caribbean through early access programs ("EAP Programs"). In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome ("FCS") in Brazil and the Company began to make commercial sales of Waylivra in Brazil in the third quarter of 2022 while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP Programs. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy, or FPL. Waylivra has also received marketing authorization in the EU for the treatment of FCS.

The Company also has a spinal muscular atrophy ("SMA") collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc. (referred to collectively as "Roche") and the Spinal Muscular Atrophy Foundation ("SMA Foundation"). The SMA program has one approved product, Evrysdi® (risdiplam), which was approved by the FDA in August 2020 for the treatment of SMA in adults and children two months and older and by the European Commission in March 2021 for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020 and Japan in June 2021. In May 2022, the FDA approved a label expansion for Evrysdi to include infants under two months old with SMA and the Company expects the European Medicines Agency ("EMA") to make a regulatory decision on approval for a label expansion for Evrysdi to include infants under two months old with SMA in 2023. In addition to the Company's SMA program, the Company's splicing platform also includes PTC518, which is being developed for the treatment of Huntington's disease ("HD"). The Company initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. Enrollment in the Phase 2 study remains active and ongoing outside of the United States. Enrollment within the United States is paused as the FDA has requested additional data to allow the Phase 2 study to proceed; discussions are ongoing with the FDA to allow the resumption of U.S. enrollment. The Company expects data from the initial 12-week phase of the Phase 2 study in the second quarter of 2023.

The Company's Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. The two most advanced molecules in the Company's Bio-e platform are vatiquinone and utreloxastat. The Company initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in children with mitochondrial disease associated seizures in the third quarter of 2020. The Company has completed enrollment in this trial after previously experiencing delays in enrollment due to the COVID-19 pandemic. The Company anticipates results from the Phase 2/3 trial to be available in the second quarter of 2023. The Company also initiated a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in the fourth quarter of 2020 and anticipates results from this trial to be available in the second quarter of 2023. In the third quarter of 2021, the Company completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of utreloxastat. Utreloxastat was found to be well-tolerated with no

reported serious adverse events while demonstrating predictable pharmacology. The Company initiated a Phase 2 trial of utreloxastat for amyotrophic lateral sclerosis in the first quarter of 2022 and enrollment is ongoing.

The most advanced molecule in the Company's metabolic platform is sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products. The Company initiated a registration-directed Phase 3 trial for sepiapterin for PKU in the third quarter of 2021 with the primary endpoint in the study of achieving statistically-significant reduction in blood Phe level. The primary analysis population includes those patients who have a greater than 30% reduction in blood Phe levels during the Part 1 run-in phase of the trial. In January 2023, the Company announced preliminary data from the Part 1 run-in phase of this trial, including that the mean reduction in blood Phe levels in an initial cohort of subjects during the Part 1 would be recognized as clinically meaningful if maintained in Part 2 of the trial. The Company now expects results from Part 2 of this trial to be available in May 2023 as the trial is overenrolled and additional time is required for the entirety of the primary analysis population to complete the study.

Unesbulin is the Company's most advanced oncology agent. The Company completed its Phase 1 trials evaluating unesbulin in leiomyosarcoma ("LMS") and diffuse intrinsic pontine glioma ("DIPG") in the fourth quarter of 2021. The Company initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022 and enrollment is ongoing. The initiation of the Company's registration-directed Phase 2/3 trial of unesbulin for DIPG was delayed as it continued to track the progress of patients in its Phase 1 trial and analyze the corresponding data. The Company now expects to initiate a registration-directed Phase 2/3 trial of unesbulin for the treatment of DIPG in the fourth quarter of 2023.

In addition, the Company has a pipeline of product candidates and discovery programs that are in early clinical, pre-clinical and research and development stages focused on the development of new treatments for multiple therapeutic areas for rare diseases.

As of 2022, the Company had an accumulated deficit of approximately $2,657.0 million. The Company has financed its operations to date primarily through the private offerings in September 2019 of 1.50% convertible senior notes due 2026 (see Note 8), public offerings of common stock in February 2014, October 2014, April 2018, January 2019, and September 2019, "at the market offering" of its common stock, its initial public offering of common stock in June 2013, proceeds from the Royalty Purchase Agreement dated as of July 17, 2020, by and among the Company, RPI 2019 Intermediate Finance Trust ("RPI"), and, solely for the limited purposes set forth therein, Royalty Pharma PLC (the "Royalty Purchase Agreement") (see Note 2), net proceeds from our borrowings under our credit agreement with Blackstone (see Note 8), private placements of its convertible preferred stock and common stock, collaborations, bank and institutional lender debt, other convertible debt, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company's product candidates. On August 15, 2022, the Company repaid the outstanding principal amount and accrued interest, totaling $152.3 million, of the 3.00% convertible senior notes due August 15, 2022 (the "2022 Convertible Notes") that was due upon maturity in accordance with the terms of the notes. The Company has also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States since 2014, Emflaza for the treatment of DMD in the United States since 2017 and Upstaza for the treatment of AADC deficiency in the EEA since May 2022. The Company has also relied on revenue associated with milestone and royalty payments from Roche pursuant to the License and Collaboration Agreement (the "SMA License Agreement") dated as of November 23, 2011, by and among the Company, Roche and, for the limited purposes set forth therein, the SMA Foundation, under its SMA program. The Company expects that cash flows from the sales of its products, together with the Company's cash, cash equivalents and marketable securities, will be sufficient to fund its operations for at least the next twelve months.

PTC Therapeutics, Inc.

Notes to consolidated financial statements (Continued)

December 31, 2022

(In thousands except share and per share amount)

2. Summary of significant accounting policies

Basis of presentation

The accompanying consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP") and include all adjustments necessary for the fair presentation of the Company's financial position for the periods presented. Certain prior period balances have been reclassified to conform to the current period presentation. These reclassifications did not have a material impact on the consolidated statements of operations, consolidated balance sheets, consolidated statements of cash flows, or notes to the consolidated financial statements.

Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Significant estimates in these consolidated financial statements have been made in connection with the calculation of net product sales, royalty revenue, certain accruals related to the Company's research and development expenses, valuation procedures for liability for sale of future royalties, valuation procedures for the convertible notes, indefinite lived intangible assets annual impairment assessment, fair value of the contingent consideration, and the provision for or benefit from income taxes. Actual results could differ from those estimates. Changes in estimates are reflected in reported results in the period in which they become known.

Restricted Cash

Restricted cash included in deposits and other assets on the consolidated balance sheet relates to an unconditional, irrevocable and transferable letter of credit that was entered into during the twelve-month period ended December 31, 2019 in connection with obligations under a facility lease for the Company's leased biologics manufacturing facility in Hopewell Township, New Jersey. The amount of the letter of credit is $7.5 million, is to be maintained for a term of not less than five years and has the potential to be reduced to $3.8 million if after five years the Company is not in default of its lease. Restricted cash also contains an unconditional, irrevocable and transferable letter of credit that was entered into during June 2022 in connection with obligations for the Company's new facility lease in Warren, New Jersey. The amount of the letter of credit is $8.1 million and has the potential to be reduced to $4.1 million if after five years the Company is not in default of its lease. Both amounts are classified within deposits and other assets on the consolidated balance sheet due to the long-term nature of the letter of credit. Restricted cash also includes a bank guarantee of $0.5 million denominated in a foreign currency.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheet that sum to the total of the same amounts shown in the statement of cash flows:

	End of period- December 31, 2022	Beginning of period- December 31, 2021
Cash and cash equivalents	$ 279,834	$ 189,718
Restricted cash included in deposits and other assets	16,091	7,500
Total Cash, cash equivalents and restricted cash per statement of cash flows	$ 295,925	$ 197,218

PTC Therapeutics, Inc.

Notes to consolidated financial statements (Continued)

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Consolidation

The consolidated financial statements include the accounts of PTC Therapeutics, Inc. and its wholly owned subsidiaries. All inter-company accounts, transactions, and profits have been eliminated in consolidation.

Segment and geographic information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating and reporting segment.

Cash equivalents

The Company considers all highly liquid investments with a maturity of 90 days or less at the time of purchase to be cash equivalents. Cash equivalents are carried at cost which approximates fair value due to their short-term nature.

Marketable securities

The Company's marketable securities consists of both debt securities and equity investments. The Company considers its investments in debt securities with original maturities of greater than 90 days to be available for sale securities. Securities under this classification are recorded at fair value and unrealized gains and losses within accumulated other comprehensive income. The estimated fair value of the available for sale securities is determined based on quoted market prices or rates for similar instruments. In addition, the cost of debt securities in this category is adjusted for amortization of premium and accretion of discount to maturity. For available for sale debt securities in an unrealized loss position, the Company assesses whether it intends to sell or if it is more likely than not that the Company will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value. If the criteria are not met, the Company evaluates whether the decline in fair value has resulted from a credit loss or other factors. In making this assessment, management considers, among other factors, the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating agency, and adverse conditions specifically related to the security. If this assessment indicates that a credit loss exists, the present value of cash flows expected to be collected from the security are compared to the amortized cost basis of the security. If the present value of the cash flows expected to be collected is less than the amortized cost basis, a credit loss exists and an allowance for credit losses is recorded for the credit loss, limited by the amount that the fair value is less than the amortized costs basis. Any impairment that has not been recorded through an allowance for credit losses is recognized in other comprehensive income. For the years ended December 31, 2022 and 2021, no allowance was recorded for credit losses.

Marketable securities that are equity investments are measured at fair value, as it is readily available, and as such are classified as Level 1 assets. Unrealized holding gains and losses for these equity investments are components of other (expense) income, net within the consolidated statement of operations.

Concentration of credit risk

The Company's financial instruments that are exposed to credit risks consist primarily of cash and cash equivalents, available-for-sale marketable securities and accounts receivable. The Company maintains its cash and cash equivalents in bank accounts, which, at times, exceed federally insured limits. The Company has not experienced any credit losses in

(In thousands except share and per share amount)

these accounts and does not believe it is exposed to any significant credit risk on these funds. The Company's investment policy includes guidelines on the quality of the financial institutions and financial instruments the Company is allowed to invest in, which the Company believes minimizes the exposure to concentration of credit risk.

The Company is subject to credit risk from its accounts receivable related to its product sales. The payment terms are predetermined and the Company evaluates the creditworthiness of each customer or distributor on a regular basis. The Company reserves all uninsured amounts billed directly to a patient until the time of cash receipt as collectability is not reasonably assured at the time the product is received. To date, the Company has not incurred any material credit losses.

Fixed assets

Fixed assets are stated at cost. Depreciation is computed starting when the asset is placed into service on a straight-line basis over the estimated useful life of the related asset as follows:

Leasehold improvements	Lesser of useful life or lease term
Computer equipment and software	3 years
Machinery and lab equipment	7 years
Furniture and fixtures	7 years

Inventory and cost of product sales

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis by product. The Company capitalizes inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Products which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes. Inventory used for marketing efforts are charged to selling, general and administrative expense. Amounts related to clinical development programs and marketing efforts are immaterial.

The following table summarizes the components of the Company's inventory for the periods indicated:

	December 31, 2022	December 31, 2021
Raw materials	$ 1,078	$ 1,418
Work in progress	14,074	7,721
Finished goods	6,656	6,717
Total inventory	$ 21,808	$ 15,856

The Company periodically reviews its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. The Company recorded write downs of $1.7 million and $2.2 million for the years ended December 31, 2022 and 2021, respectively, primarily related to product approaching expiration. Additionally, though the Company's product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of product sales. For the years ended December 31, 2022 and December 31, 2021, these amounts were immaterial.

Cost of product sales

Cost of product sales consists of the cost of inventory sold, manufacturing and supply chain costs, storage costs, amortization of the acquired intangible asset, royalty payments associated with net product sales, and royalty payments to collaborative partners associated with royalty revenues and collaboration revenue related to milestones. Production costs are expensed as cost of product sales when the related products are sold or royalty revenues and collaboration revenue milestones are earned.

Accumulated other comprehensive income (loss)

Accumulated other comprehensive income (loss) consists of unrealized gains or losses on marketable securities and foreign currency translation adjustments.

Revenue recognition

Net product revenue

The Company's net product revenue primarily consists of sales of Translarna in territories outside of the U.S. for the treatment of nmDMD and sales of Emflaza in the U.S. for the treatment of DMD. The Company recognizes revenue when its performance obligations with its customers have been satisfied. The Company's performance obligations are to provide products based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligations are satisfied at a point in time when the Company's customer obtains control of the product, which is typically upon delivery. The Company invoices its customers after the products have been delivered and invoice payments are generally due within 30 to 90 days of the invoice date. The Company determines the transaction price based on fixed consideration in its contractual agreements. Contract liabilities arise in certain circumstances when consideration is due for goods the Company has yet to provide. As the Company has identified only one distinct performance obligation, the transaction price is allocated entirely to product sales. In determining the transaction price, a significant financing component does not exist since the timing from when the Company delivers product to when the customers pay for the product is typically less than one year. Customers in certain countries pay in advance of product delivery. In those instances, payment and delivery typically occur in the same month.

The Company records product sales net of any variable consideration, which includes discounts, allowances, rebates related to Medicaid and other government pricing programs, and distribution fees. The Company uses the expected value or most likely amount method when estimating its variable consideration, unless discount or rebate terms are specified within contracts. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained.

During the years ended December 31, 2022, 2021, and 2020, net product sales in the United States were $218.3 million, $187.3 million, and $139.0 million, respectively, consisting solely of sales of Emflaza, and net product sales outside of the United States were $316.9 million, $241.6 million, and $194.4 million respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net product revenues made up $288.6 million, $236.0 million, and $191.9 million of the net product sales outside of the United States for the years ended December 31, 2022, 2021, and 2020, respectively. During the year ended December 31, 2022, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $218.3 million and $59.7 million of net product sales,

respectively. During the years ended December 31, 2021 and 2020, only the United States accounted for at least 10% of the Company's net product sales.

In relation to customer contracts, the Company incurs costs to fulfill a contract but does not incur costs to obtain a contract. These costs to fulfill a contract do not meet the criteria for capitalization and are expensed as incurred. The Company considers any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise. Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Collaboration and royalty revenue

The terms of these agreements typically include payments to the Company of one or more of the following: nonrefundable, upfront license fees; milestone payments; research funding and royalties on future product sales. In addition, the Company generates service revenue through agreements that generally provide for fees for research and development services and may include additional payments upon achievement of specified events.

At the inception of a collaboration arrangement, the Company needs to first evaluate if the arrangement meets the criteria in Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 808 "Collaborative Arrangements" to then determine if ASC Topic 606 is applicable by considering whether the collaborator meets the definition of a customer. If the criteria are met, the Company assesses the promises in the arrangement to identify distinct performance obligations.

For licenses of intellectual property, the Company assesses, at contract inception, whether the intellectual property is distinct from other performance obligations identified in the arrangement. If the licensing of intellectual property is determined to be distinct, revenue is recognized for nonrefundable, upfront license fees when the license is transferred to the customer and the customer can use and benefit from the license. If the licensing of intellectual property is determined not to be distinct, then the license will be bundled with other promises in the arrangement into one distinct performance obligation. The Company needs to determine if the bundled performance obligation is satisfied over time or at a point in time. If the Company concludes that the nonrefundable, upfront license fees will be recognized over time, the Company will need to assess the appropriate method of measuring proportional performance.

For milestone payments, the Company assesses, at contract inception, whether the development or sales-based milestones are considered probable of being achieved. If it is probable that a significant revenue reversal will occur, the Company will not record revenue until the uncertainty has been resolved. Milestone payments that are contingent upon regulatory approval are not considered probable of being achieved until the applicable regulatory approvals or other external conditions are obtained as such conditions are not within the Company's control. If it is probable that a significant revenue reversal will not occur, the Company will estimate the milestone payments using the most likely amount method. The Company will re-assess the development and sales-based milestones each reporting period to determine the probability of achievement. The Company recognizes royalties from product sales at the later of when the related sales occur or when the performance obligation to which the royalty has been allocated has been satisfied. If it is probable that a significant revenue reversal will not occur, the Company will estimate the royalty payments using the most likely amount method.

The Company recognizes revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. The Company records these reimbursements as revenue and not as a reduction of research and development expenses as the Company has the risks and rewards as the principal in the research and development activities.

For the years ended December 31, 2022, 2021, and 2020, the Company has recognized $50.1 million, $55.0 million, and $42.6 million of collaboration revenue, respectively, related to the SMA License Agreement with Roche.

For the years ended December 31, 2022, 2021 and 2020, the Company has recognized $113.5 million, $54.6 million, and $4.8 million of royalty revenue, respectively, related to Evrysdi.

Allowance for doubtful accounts

The Company maintains an allowance for estimated losses resulting from the inability of its customers to make required payments. The Company estimates uncollectible amounts based upon current customer receivable balances, the age of customer receivable balances, the customer's financial condition and current economic trends. The Company also assesses whether an allowance for expected credit losses may be required which includes a review of the Company's receivables portfolio, which are pooled on a customer basis or country basis. In making its assessment of whether an allowance for credit losses is required, the Company considers its historical experience with customers, current balances, levels of delinquency, regulatory and legal environments, and other relevant current and future forecasted economic conditions. For the years ended December 31, 2022 and 2021, no allowance was recorded for credit losses. The allowance for doubtful accounts was $0.3 million as of December 31, 2022 and $0.1 million as of December 31, 2021. For the years ended December 31, 2022, 2021 and 2020, bad debt expense was immaterial.

Liability for sale of future royalties

On July 17, 2020, the Company, RPI Intermediate Finance Trust ("RPI"), and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, entered into a Royalty Purchase Agreement (the "Royalty Purchase Agreement"). Pursuant to the Royalty Purchase Agreement, the Company sold to RPI 42.933% (the "Assigned Royalty Payment") of the Company's right to receive sales-based royalty payments (the "Royalty") on worldwide net sales of Evrysdi and any other product developed pursuant to the License and Collaboration Agreement (the "License Agreement"), dated as of November 23, 2011, by and among the Company, Roche and, for the limited purposes set forth therein, the SMA Foundation under the SMA program. In consideration for the sale of the Assigned Royalty Payments, RPI paid the Company $650.0 million in cash consideration. The Company has retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the License Agreement, which remaining milestone payments equal $250.0 million in the aggregate as of December 31, 2022. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

The cash consideration obtained pursuant to the Royalty Purchase Agreement is classified as debt and is recorded as "liability for sale of future royalties-current" and "liability for sale of future royalties-noncurrent" on the Company's consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of future royalties at the time of the transaction was based on the Company's estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability is being amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance. The Company utilizes the prospective method to account for subsequent changes in the estimated future payments to be made to RPI. Refer to Note 8 for further details.

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Notes to consolidated financial statements (Continued)

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(In thousands except share and per share amount)

Leases

The Company determines if an arrangement is a lease at inception. This determination generally depends on whether the arrangement conveys to the Company the right to control the use of an explicitly or implicitly identified fixed asset for a period of time in exchange for consideration. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. The Company has lease agreements which include lease and non-lease components, which the Company accounts for as a single lease component for all leases. Operating and finance leases are classified as right of use ("ROU") assets, short term lease liabilities, and long term lease liabilities. Operating and finance lease ROU assets and lease liabilities are recognized at the commencement date based on the present value of lease payments over the lease term. ROU assets are amortized and lease liabilities accrete to yield straight-line expense over the term of the lease. Lease payments included in the measurement of the lease liability are comprised of fixed payments.

Variable lease payments associated with the Company’s leases are recognized when the event, activity, or circumstance in the lease agreement on which those payments are assessed occurs. Variable lease payments are presented in the Company’s consolidated statements of operations in the same line item as expense arising from fixed lease payments for operating leases.

Leases with an initial term of 12 months or less are not recorded on the consolidated balance sheet and the Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company applies this policy to all underlying asset categories.

A lessee is required to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. As most of the Company’s leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at the commencement date in determining the present value of lease payments. The Company gives consideration to its recent debt issuances as well as publicly available data for instruments with similar characteristics when calculating its incremental borrowing rates.

The lease term for all of the Company’s leases includes the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor. Leasehold improvements are capitalized and depreciated over the lesser of useful life or lease term. See Note 6 Leases for additional information.

Research and development costs

Research and development expenses include the clinical development costs associated with the Company’s product development programs and research and development costs associated with the Company’s discovery programs. These expenses include internal research and development costs and the costs of research and development conducted on behalf of the Company by third parties, including sponsored university-based research agreements and clinical study vendors. All research and development costs are expensed as incurred. Costs incurred in obtaining technology licenses are charged immediately to research and development expense if the technology licensed has not reached technological feasibility and has no alternative future uses.

Advance payments made for goods and services that will be used in future research and development activities are deferred if the contracted party has not yet performed the related activities. The amount deferred is then recognized as expense when the research and development activities are performed. The short term deferred research and development advance payments were $2.4 million and $1.4 million and are classified as prepaid expenses and other current assets on

the consolidated balance sheet as of December 31, 2022 and 2021, respectively. The long term deferred research and development advance payments were $3.9 million and $1.8 million and are classified as deposits and other assets on the consolidated balance sheet as of December 31, 2022 and 2021, respectively.

Fair value of financial instruments

The Company follows the fair value measurement rules, which provides guidance on the use of fair value in accounting and disclosure for assets and liabilities when such accounting and disclosure is called for by other accounting literature. These rules establish a fair value hierarchy for inputs to be used to measure fair value of financial assets and liabilities. This hierarchy prioritizes the inputs to valuation techniques used to measure fair value into three levels: Level 1 (highest priority), Level 2, and Level 3 (lowest priority).

- Level 1—Unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the balance sheet date.

- Level 2—Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs include quoted prices for similar assets and liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active, inputs other than quoted prices that are observable for the asset or liability (i.e., interest rates, yield curves, etc.), and inputs that are derived principally from or corroborated by observable market data by correlation or other means (market corroborated inputs).

- Level 3—Inputs are unobservable and reflect the Company's assumptions as to what market participants would use in pricing the asset or liability. The Company develops these inputs based on the best information available.

Cash equivalents, marketable securities, and equity investments are reflected in the accompanying financial statements at fair value. The carrying amount of receivables and accounts payable and accrued expenses approximates fair value due to the short-term nature of those instruments.

Share-based compensation

The Company measures the cost of employee services received in exchange for an award of equity instruments based on the grant date fair value of the award. Restricted stock awards are measured based on the fair market values of the underlying stock on the dates of grant. For service type awards, share-based compensation expense is recognized on a straight-line basis over the period during which the employee is required to provide service in exchange for the entire award.

The fair value of options is calculated using the Black-Scholes option pricing model to determine the fair value of stock options on the date of grant based on key assumptions such as expected volatility and expected term. The Company estimates the expected volatility of options utilizing the Company's historical stock volatility. The Company estimates the expected term of options utilizing the Company's historical exercise data. The risk-free rate of the option is based on U.S. Government Securities Treasury Constant Maturities yields at the date of grant for a term similar to the expected term of the option. In connection with the adoption of FASB Accounting Standards Update ("ASU") 2016-9, the Company made a policy election to continue its methodology for estimating its forfeiture rate.

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Income taxes

On March 27, 2020, the United States enacted the Coronavirus Aid, Relief, and Economic Security Act, referred to herein as the CARES Act, as a response to the economic uncertainty resulting from a strain of novel coronavirus, COVID-19. The CARES Act includes modifications for net operating loss carryovers and carrybacks, limitations of business interest expense for tax, immediate refund of alternative minimum tax (“AMT”) credit carryovers as well as a technical correction to the 2017 Tax Cuts and Jobs Act ("the 2017 Tax Act") for qualified improvement property. On December 27, 2020, the Coronavirus Response and Relief Supplemental Appropriations Act of 2021 – a $900 billion relief package to deliver the second round of economic stimulus for individuals, families, and businesses was signed into law. The bill provides relief through multiple measures and expands many of the provisions already put into place under the CARES Act. As of December 31, 2022, the Company expects that these provisions will not have a material impact. Tax provisions of the CARES Act also include the deferral of certain payroll taxes, relief for retaining employees, and other provisions. The relief for retaining employees was not material to the financial statements and there were no deferrals of certain payroll taxes as of December 31, 2022, which is no longer accrued in other current liabilities on the consolidated balance sheet.

Additionally, the Organization for Economic Co-operation and Development, or OECD, the EC, and individual taxing jurisdictions where the Company and its affiliates do business have recently focused on issues related to the taxation of multinational corporations. The OECD has released its comprehensive plan to create an agreed set of international rules for fighting base erosion and profit shifting. In addition, the OECD, the EC, and individual counties are examining changes to how taxing rights should be allocated among countries considering the digital economy. As a result, the tax laws in the U.S. and other countries in which PTC and its affiliates do business could change on a prospective or retroactive basis and any such changes could materially adversely affect the Company’s business.

On December 22, 2017, the U.S. government enacted the 2017 Tax Cuts and Jobs Act (“TCJA”), which significantly revised U.S. tax law by, among other provisions, lowering the U.S. federal statutory income tax rate to 21%, imposing a mandatory one-time transition tax on previously deferred foreign earnings, and eliminating or reducing certain income tax deductions. The Global Intangible Low-tax Income ("GILTI") provisions of the TCJA require the Company to include in its U.S. income tax return foreign subsidiary earnings in excess of an allowable return on the foreign subsidiary’s tangible assets. The Company has elected to account for GILTI tax in the period in which it is incurred, and therefore has not provided any deferred tax impacts of GILTI in its consolidated financial statements for the period ended December 31, 2022.

Starting in 2022, TCJA amendments to IRC Section 174 will no longer permit an immediate deduction for research and development (R&D) expenditures in the tax year that such costs are incurred. Instead, these IRC Section 174 development costs must now be capitalized and amortized over either a five- or 15-year period, depending on the location of the activities performed. The new amortization period begins with the midpoint of any taxable year that IRC Section 174 costs are first incurred, regardless of whether the expenditures were made prior to or after July 1 and runs until the midpoint of year five for activities conducted in the United States or year 15 in the case of development conducted on foreign soil. This tax law change resulted in an increased current taxable income of the Company by $450.1 million for the year ended December 31, 2022.

In December 2017, the SEC staff issued Staff Accounting Bulletin No. 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act (SAB 118), which allowed the Company to record provisional amounts during a measurement period not to extend beyond one year of the enactment date. As a result of the reduction in the U.S. corporate income tax rate, the Company revalued its ending net deferred tax assets as of December 31, 2017. In the fourth quarter of 2018, the Company completed its analysis to determine the effect of the Tax Act and recorded no further adjustments.

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Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and net operating loss and credit carryforwards. Deferred tax assets and liabilities are measured at rates expected to apply to taxable income in the years in which those temporary differences and carryforwards are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date. A valuation allowance is recorded when it is not more likely than not that all or a portion of the net deferred tax assets will be realized.

On August 23, 2018, the Company completed its acquisition of Agilis Biotherapeutics, Inc. (“Agilis”), pursuant to an Agreement and Plan of Merger, dated as of July 19, 2018 (the “Agilis Merger Agreement”), by and among the Company, Agility Merger Sub, Inc., a Delaware corporation and the Company’s wholly owned, indirect subsidiary, Agilis and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, (the “Agilis Merger”). The Company recorded a deferred tax liability in conjunction with the Agilis Merger of $122.0 million in 2018, related to the tax basis difference in the In-Process Research and Development, or IPR&D, indefinite-lived intangibles acquired. The Company’s policy is to record a deferred tax liability related to acquired IPR&D which may eventually be realized either upon amortization of the asset when the research is completed and a product is successfully launched or the write-off of the asset if it is abandoned or unsuccessful. In July 2022, the Company received EMEA approval for a portion of the IPR&D assets, and thus, began the amortization of the intangible.

Foreign currency

The functional currencies of the Company’s foreign subsidiaries primarily are the local currencies of the country in which the subsidiary operates. The Company also has an intercompany loan which is recorded on a non-U.S. subsidiary and was formally denominated in U.S. dollars and was remeasured into local currency using the exchange rate as of the balance sheet date. During the year ended December 31, 2022, the loan agreement was amended to change the denomination from U.S. dollars to local currency, and the Company recorded a non-cash realized foreign exchange loss of $16.9 million, which is recorded within other (expense) income, net on the consolidated statement of operations. The Company’s asset and liability accounts, including the intercompany loan, are translated using the current exchange rate as of the balance sheet date. Stockholders’ equity accounts are translated using historical rates at the balance sheet date. Revenue and expense accounts are translated using a weighted average exchange rate over the period ended on the balance sheet date. Adjustments resulting from the translation of the financial statements of the Company’s foreign subsidiaries into U.S. dollars are accumulated as a separate component of stockholders’ equity within other comprehensive income. Gains or losses resulting from transactions denominated in foreign currencies are included in other income or expense, within the consolidated statements of income.

Net (loss) income per share

Basic net (loss) income per share is calculated by dividing the net (loss) income attributable to common stockholders by the weighted average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net income per share is calculated by dividing the net income attributable to common stockholders by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method and the if-converted method. During periods in which the Company incurs net losses, both basic and diluted loss per share is calculated by dividing the net loss by the weighted average shares outstanding—potentially dilutive securities are excluded from the calculation because their effect would be anti-dilutive. Dilutive common stock equivalents are comprised of options and unvested restricted stock outstanding under the Company’s stock option plans.

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Business combinations and asset acquisitions

The Company evaluates acquisitions of assets and other similar transactions to assess whether or not the transaction should be accounted for as a business combination or asset acquisition by first applying a screen to determine if substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar identifiable assets. If the screen is met, the transaction is accounted for as an asset acquisition. If the screen is not met, further determination is required as to whether or not the Company has acquired inputs and processes that have the ability to create outputs, which would meet the requirements of a business. If determined to be a business combination, the Company accounts for the transaction under the acquisition method of accounting as indicated in ASU 2017-01, “Business Combinations”, which requires the acquiring entity in a business combination to recognize the fair value of all assets acquired, liabilities assumed, and any non-controlling interest in the acquiree and establishes the acquisition date as the fair value measurement point. Accordingly, the Company recognizes assets acquired and liabilities assumed in business combinations, including contingent assets and liabilities, and non-controlling interest in the acquiree based on the fair value estimates as of the date of acquisition. In accordance with ASC 805, the Company recognizes and measures goodwill as of the acquisition date, as the excess of the fair value of the consideration paid over the fair value of the identified net assets acquired.

The consideration for the Company’s business acquisitions may include future payments that are contingent upon the occurrence of a particular event or events. The obligations for such contingent consideration payments are recorded at fair value on the acquisition date. The contingent consideration obligations are then evaluated each reporting period. Changes in the fair value of contingent consideration, other than changes due to payments, are recognized as a gain or loss and recorded within the change in the fair value of deferred and contingent consideration in the consolidated statements of operations.

If determined to be an asset acquisition, the Company accounts for the transaction under ASC 805-50, which requires the acquiring entity in an asset acquisition to recognize assets acquired and liabilities assumed based on the cost to the acquiring entity on a relative fair value basis, which includes transaction costs in addition to consideration given. No gain or loss is recognized as of the date of acquisition unless the fair value of noncash assets given as consideration differs from the assets’ carrying amounts on the acquiring entity’s books. Consideration transferred that is noncash will be measured based on either the cost (which will be measured based on the fair value of the consideration given) or the fair value of the assets acquired and liabilities assumed, whichever is more reliably measurable. Goodwill is not recognized in an asset acquisition and any excess consideration transferred over the fair value of the net assets acquired is allocated to the identifiable assets based on relative fair values.

Contingent consideration payments in asset acquisitions are recognized when the contingency is resolved and the consideration is paid or becomes payable (unless the contingent consideration meets the definition of a derivative, in which case the amount becomes part of the basis in the asset acquired). Upon recognition of the contingent consideration payment, the amount is included in the cost of the acquired asset or group of assets.

Finite-lived intangible assets

The Company records the fair value of purchased intangible assets with finite useful lives as of the transaction date of a business combination or asset acquisition. Purchased intangible assets with finite useful lives are amortized to their estimated residual values over their estimated useful lives.

Impairment of long-lived assets

The Company monitors its long-lived assets and finite-lived intangibles for indicators of impairment. If such indicators are present, the Company assesses the recoverability of affected assets by determining whether the carrying value of such assets is less than the sum of the undiscounted future cash flows of the assets. If such assets are found not to be recoverable, the Company measures the amount of such impairment by comparing the carrying value of the assets to the fair value of the assets, with the fair value generally determined based on the present value of the expected future cash flows associated with the assets. The Company believes that no impairment of long-lived assets exists as of December 31, 2022.

Indefinite-lived intangible assets

Indefinite-lived intangible assets consist of IPR&D. IPR&D acquired directly in a transaction other than a business combination is capitalized if the projects will be further developed or have an alternative future use; otherwise they are expensed. The fair values of IPR&D projects and license agreement assets acquired in business combinations are capitalized. Several methods may be used to determine the estimated fair value of the IPR&D and license agreement asset acquired in a business combination. The Company utilizes the "income method" and uses estimated future net cash flows that are derived from projected sales revenues and estimated costs. These projections are based on factors such as relevant market size, patent protection, and expected pricing and industry trends. The estimated future net cash flows are then discounted to the present value using an appropriate discount rate. These assets are treated as indefinite-lived intangible assets until completion or abandonment of the projects, at which time the assets are amortized over the remaining useful life or written off, as appropriate. Intangible assets with indefinite lives, including IPR&D, are tested for impairment if impairment indicators arise and, at a minimum, annually. However, an entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that an indefinite-lived intangible asset's fair value is less than its carrying amount. Otherwise, no further impairment testing is required. The indefinite-lived intangible asset impairment test consists of a one-step analysis that compares the fair value of the intangible asset with its carrying amount. If the carrying amount of an intangible asset exceeds its fair value, an impairment loss is recognized in an amount equal to that excess. The Company considers many factors in evaluating whether the value of its intangible assets with indefinite lives may not be recoverable, including, but not limited to, expected growth rates, the cost of equity and debt capital, general economic conditions, the Company's outlook and market performance of the Company's industry and recent and forecasted financial performance.

The Company performed an annual test for its indefinite-lived intangible assets as of October 1, 2022. In the fourth quarter of 2022, the Company recorded a partial impairment on the Upstaza indefinite lived intangible asset of $33.4 million, which is recorded as intangible asset impairment in the statement of operations. The impairment was related to a decrease in projected cash flows due to refinements in current market assumptions and the timing of patient treatments. To calculate the impairment amount, the Company utilized a discounted cash flow model under the income method, which primarily utilized Level 3 fair value inputs. Some of the more significant assumptions inherent in the development of the model included the estimated annual cash flows, particularly net revenues and operations costs, the appropriate discount rate to select in order to measure the risk inherent in the future cash flows, and the probability of success. Refer to Note 18 for further information regarding the Company's intangible assets.

Goodwill

Goodwill represents the amount of consideration paid in excess of the fair value of net assets acquired as a result of the Company's business acquisitions accounted for using the acquisition method of accounting. Goodwill is not amortized and is subject to impairment testing at a reporting unit level on an annual basis or when a triggering event occurs that may

indicate the carrying value of the goodwill is impaired. The Company reassess its reporting units as part of its annual segment review. As of December 31, 2022, the Company concluded that it continues to operate as one reporting unit. An entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that the fair value of the reporting unit is less than its carrying amount. The Company performed an annual test for goodwill as of October 1, 2022. The Company's single reporting unit had a negative carrying value and thus the Company determined there was no impairment of goodwill.

3. Acquisitions

Censa Asset Acquisition

On May 29, 2020, the Company completed its acquisition of Censa Pharmaceuticals, Inc. ("Censa") pursuant to an Agreement and Plan of Merger, dated as of May 5, 2020 (the "Censa Merger Agreement"), by and among the Company, Hydro Merger Sub, Inc., the Company's wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC (the "Censa Merger").

Upon the closing of the Censa Merger, the Company paid to the Censa securityholders (i) cash consideration of $15.0 million, which consisted of an upfront payment of $10.4 million and an additional $4.6 million for the net assets on Censa's opening balance sheet as of the date of the acquisition, and (ii) 845,364 shares of the Company's common stock, which were valued at $42.9 million based on the closing stock price on the acquisition date. The number of shares issued was determined using a 30-day volume weighted average price ("VWAP") pursuant to the Censa Merger Agreement.

The Company determined that substantially all of the fair value is concentrated in sepiapterin, formerly known as PTC923, and accounted for the transaction as an asset acquisition under ASC 805-50. The purchase price consisted of the cash consideration of $15.0 million and $42.9 million in the Company's common stock, in addition to $0.7 million of acquisition costs. As such, the total consideration transferred was determined to be $58.6 million. The opening balance sheet net assets of $4.6 million, which consisted of cash of $3.8 million and other current assets of $0.8 million, were determined to be non-qualifying assets and recorded at their fair values, respectively. The remaining consideration of $54.0 million was allocated to sepiapterin. As sepiapterin is an IPR&D asset, the Company concluded that it did not have any alternative future use, and accordingly, the fair value amount allocated to the IPR&D was expensed. Of the $54.0 million, $53.3 million is included in research and development expense and the $0.7 million related to the acquisition costs, is included in selling, general, and administrative expense within the Company's statement of operations for the year ended December 31, 2020. Refer to Note 15 for to the terms and conditions of the Censa Merger Agreement.

4. Fair value of financial instruments and investments

The Company follows the fair value measurement rules, which provide guidance on the use of fair value in accounting and disclosure for assets and liabilities when such accounting and disclosure is called for by other accounting literature. Cash equivalents, marketable securities, and equity investments are reflected in the accompanying financial statements at fair value. The carrying amount of receivables and accounts payable and accrued expenses approximate fair value due to the short-term nature of those instruments.

The Company uses the market approach to measure fair value for its marketable securities. The market approach uses prices and other relevant information generated by market transactions involving identical or comparable assets. The

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Company's marketable securities are classified as Level 2 as they primarily utilize broker quotes in a nonactive market to value these securities.

The Company owns common stock in ClearPoint Neuro, Inc. ("ClearPoint") (formerly MRI Interventions, Inc.), a publicly traded medical device company. The ClearPoint equity investments (collectively, the "ClearPoint Equity Investments") represent financial instruments, and therefore, are recorded at fair value, which is readily determinable. The ClearPoint Equity Investments are components of deposits and other assets on the consolidated balance sheet. During the years ended December 31, 2022 and 2021, the Company recorded unrealized losses of $3.5 million and $6.1 million, respectively, which are components of other (expense) income, net within the consolidated statement of operations. The fair value of the equity investments was $11.0 million and $14.5 million as of December 31, 2022 and 2021, respectively. The Company classifies its equity investments in ClearPoint as a Level 1 asset within the fair value hierarchy, as the value is based on a quoted market price in an active market, which is not adjusted.

In January 2020, the Company purchased a $10.0 million convertible note from ClearPoint that the Company can convert into ClearPoint shares at a conversion rate of $6.00 per share at any point throughout the term of the loan, which matures five years from the purchase date. The Company determined that the convertible note represents an available for sale debt security and the Company has elected to record it at fair value under ASC 825. The Company classifies its ClearPoint convertible debt security as a Level 2 asset within the fair value hierarchy, as the value is based on inputs other than quoted prices that are observable. The fair value of the ClearPoint convertible debt security is determined at each reporting period by utilizing a Black-Scholes option pricing model, as well as a present value of expected cash flows from the debt security utilizing the risk free rate and the estimated credit spread as of the valuation date as the discount rate. During the years ended December 31, 2022 and 2021, the Company recorded unrealized losses of $5.8 million and $8.3 million, respectively. These unrealized losses are components of other (expense) income, net within the consolidated statement of operations. The fair value of the convertible debt security was $15.2 million and $21.0 million as of December 31, 2022 and 2021, respectively. The convertible debt security is considered to be long term and is included as a component of deposits and other assets on the consolidated balance sheet. Other than the equity investment and the convertible debt security, no other items included in deposits and other assets on the consolidated balance sheets are fair valued.

The Company has investments in mutual funds, including one that is denominated in a foreign currency. All of these are equity investments and are classified as marketable securities on the Company's consolidated balance sheets. These equity investments are reported at fair value, as it is readily available, and as such are classified as Level 1 assets. Unrealized holding gains and losses for these equity investments are included as components of other (expense) income, net within the consolidated statement of operations. During the years ended December 31, 2022 and 2021, the Company had $8.0 million unrealized losses and $1.7 million unrealized net gains, respectively, relating to the equity investments still held at the reporting date. During the years ended December 31, 2022 and 2021, the Company had redemptions of $113.0 million and $4.3 million, respectively. During the years ended December 31, 2022 and 2021, the Company had $0.5 million and $0.4 million foreign currency unrealized losses, respectively, relating to these equity investments.

Fair value of marketable securities that are classified as available for sale debt securities is based upon market prices using quoted prices in active markets for identical assets quoted on the last day of the period. In establishing the estimated fair value of the remaining available for sale debt securities, the Company used the fair value as determined by its investment advisors using observable inputs other than quoted prices.

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The following represents the fair value using the hierarchy described in Note 2 for the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis as of December 31, 2022 and 2021:

	December 31, 2022			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities - available for sale	$ 22,610	$ —	$ 22,610	$ —
Marketable securities - equity investments	$ 108,261	$ 108,261	$ —	$ —
ClearPoint Equity Investments	$ 10,965	$ 10,965	$ —	$ —
ClearPoint convertible debt security	$ 15,231	$ —	$ 15,231	$ —
Contingent consideration payable- development and regulatory milestones	$ 82,500	$ —	$ —	$ 82,500
Contingent consideration payable- net sales milestones and royalties	$ 81,500	$ —	$ —	$ 81,500

	December 31, 2021			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities - available for sale	$ 376,685	$ —	$ 376,685	$ —
Marketable securities - equity investments	$ 206,973	$ 206,973	$ —	$ —
ClearPoint Equity Investments	$ 14,525	$ 14,525	$ —	$ —
ClearPoint convertible debt security	$ 20,971	$ —	$ 20,971	$ —
Contingent consideration payable- development and regulatory milestones	$ 139,300	$ —	$ —	$ 139,300
Contingent consideration payable- net sales milestones and royalties	$ 100,600	$ —	$ —	$ 100,600

No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the years ended December 31, 2022 and 2021.

The following is a summary of marketable securities accounted for as available for sale debt securities at December 31, 2022 and 2021:

	December 31, 2022			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 12,419	$ 5	$ —	$ 12,424
Corporate debt securities	10,685	—	(499)	10,186
Total	$ 23,104	$ 5	$ (499)	$ 22,610

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	December 31, 2021			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 75,275	$ 5	$ (1)	$ 75,279
Corporate debt securities	268,246	81	(644)	267,683
Asset-backed securities	15,287	16	(5)	15,298
Government obligations	18,479	5	(59)	18,425
Total	$ 377,287	$ 107	$ (709)	$ 376,685

For available for sale debt securities in an unrealized loss position, the Company assesses whether it intends to sell or if it is more likely than not that the Company will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value. For the years ended December 31, 2022 and 2021, no write downs occurred. The Company does not intend to sell the investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost basis, which may be maturity. The Company also reviews its available for sale debt securities in an unrealized loss position and evaluates whether the decline in fair value has resulted from credit losses or other factors. This review is subjective, as it requires management to evaluate whether an event or change in circumstances has occurred in that period that may be related to credit issues. For the years ended December 31, 2022 and 2021, no allowance was recorded for credit losses. Unrealized gains and losses are reported as a component of accumulated other comprehensive (loss) income in stockholders' equity.

For the year ended December 31, 2022, the Company had $4.0 million of realized losses from the sale of available for sale debt securities. For the year ended December 31, 2021, the Company had $0.8 million of realized gains from the sale of available for sale debt securities. Realized gains and losses are reported as a component of interest expense, net in the consolidated statement of operations.

The unrealized losses and fair values of available for sale debt securities that have been in an unrealized loss position for a period of less than and greater than or equal to 12 months as of December 31, 2022 are as follows:

	December 31, 2022					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than or equal to 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Corporate debt securities	$ —	—	(499)	10,186	(499)	$ 10,186
Total	$ —	$ —	$ (499)	$ 10,186	$ (499)	$ 10,186

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The unrealized losses and fair values of available for sale debt securities that have been in an unrealized loss position for a period of less than and greater than or equal to 12 months as of December 31, 2021 are as follows:

	December 31, 2021					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than or equal to 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Commercial paper	$ (1)	12,992	—	—	(1)	$ 12,992
Corporate debt securities	(608)	217,540	(36)	4,985	(644)	222,525
Asset-backed securities	(5)	10,786	—	—	(5)	10,786
Government obligations	(59)	15,483	—	—	(59)	15,483
Total	$ (673)	$ 256,801	$ (36)	$ 4,985	$ (709)	$ 261,786

Available for sale debt securities on the balance sheet at December 31, 2022 and 2021 mature as follows:

	December 31, 2022	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 12,424	$ —
Corporate debt securities	—	10,186
Total	$ 12,424	$ 10,186

	December 31, 2021	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 75,279	$ —
Corporate debt securities	131,606	136,077
Asset-backed securities	8,724	6,574
Government obligations	6,002	12,423
Total	$ 221,611	$ 155,074

The Company classifies all of its marketable securities as current as they are all either available for sale debt securities or equity investments and are available for current operations.

Convertible senior notes

In August 2015, the Company issued $150.0 million of 3.0% convertible senior notes due August 15, 2022 (the “2022 Convertible Notes”). In September 2019, the Company issued $287.5 million of 1.5% convertible senior notes due September 15, 2026 (the “2026 Convertible Notes,” together with the “2022 Convertible Notes,” the “Convertible Notes”). On August 15, 2022, the Company repaid the outstanding principal amount and accrued interest, totaling $152.3 million, of the 2022 Convertible Notes that was due upon maturity in accordance with the terms of the notes. The fair value of the Convertible Notes, which differs from their carrying values, is influenced by interest rates, the Company’s stock price and stock price volatility and is determined by prices for the Convertible Notes observed in market trading which are Level 2 inputs. As the 2022 Convertible Notes have been repaid, they are no longer on the balance sheet as of December 31, 2022. The estimated fair value of the 2022 Convertible Notes at December 31, 2021 was $158.3 million. The estimated fair value of the 2026 Convertible Notes at December 31, 2022 and December 31, 2021 was $281.7 million and $305.3 million, respectively.

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Level 3 valuation

The contingent consideration payable is fair valued each reporting period with the change in fair value recorded as a gain or loss in the consolidated statements of operations. The fair value of the development and regulatory milestones are estimated utilizing a probability adjusted, discounted cash flow approach. The discount rates are estimated utilizing Corporate B rated bonds maturing in the years of expected payments based on the Company's estimated development timelines for the acquired product candidate. On December 31, 2022, the weighted average discount rate for the development and regulatory milestones was 7.8% and the weighted average probability of success was 35%. The fair value of the net sales milestones and royalties is determined utilizing an option pricing model with Monte Carlo simulation to simulate a range of possible payment scenarios, and the average of the payments in these scenarios is then discounted to calculate present fair value. On December 31, 2022, the weighted average discount rate for the net sales milestones and royalties was 11.5% and the weighted average probability of success for the net sales milestones was 50%.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuation for the contingent consideration payables for the years ended December 31, 2022, and 2021:

	Contingent consideration payable-development and regulatory milestones - Agilis	Contingent consideration payable-net sales milestones and royalties - Agilis
Beginning balance as of December 31, 2020	$ 139,200	$ 101,200
Additions	—	—
Change in fair value	100	(600)
Payments	—	—
Ending balance as of December 31, 2021	$ 139,300	$ 100,600
Additions	—	—
Change in fair value	(6,800)	(19,100)
Payments	(50,000)	—
Ending balance as of December 31, 2022	$ 82,500	$ 81,500

In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA, which triggered a $50.0 million milestone payment to the former equityholders of Agilis in accordance with the terms of the Agilis Merger Agreement. In accordance with ASC 230, the portion of the $50.0 million milestone payment up to the acquisition date fair value of the contingent consideration liability is classified as a financing outflow and the amount paid in excess of the acquisition date fair value of that liability is classified as an operating outflow on the consolidated statements of cash flows.

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The following significant unobservable inputs were used in the valuation of the contingent consideration payables for the years ended December 31, 2022 and 2021:

	December 31, 2022			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$82,500	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $331 million 25% - 92% 6.2% - 8.3% 2023 - 2029
Contingent considerable payable- net sales milestones and royalties	$81,500	Option-pricing model with Monte Carlo simulation	Potential net sales milestones Probabilities of success Potential percentage of net sales for royalties Discount rate Projected years of payments	$0 - $150 million 25% - 100% 2% - 6% 11.5% 2025 - 2041

	December 31, 2021			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$139,300	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $381 million 25% - 94% 1.7% - 4.7% 2022 - 2028
Contingent considerable payable- net sales milestones and royalties	$100,600	Option-pricing model with Monte Carlo simulation	Potential net sales milestones Probabilities of success Potential percentage of net sales for royalties Discount rate Projected years of payments	$0 - $150 million 25% - 94% 2% - 6% 11.0% 2023 - 2040

The contingent consideration payables are classified Level 3 liabilities as their valuation requires substantial judgment and estimation of factors that are not currently observable in the market. If different assumptions were used for the various inputs to the valuation approaches, including but not limited to, assumptions involving probability adjusted sales estimates for the gene therapy platform and estimated discount rates, the estimated fair value could be significantly higher or lower than the fair value determined.

5. Fixed assets

Fixed assets, net were as follows at December 31, 2022 and 2021:

	December 31,	
	2022	2021
Leasehold improvements	$ 28,969	$ 19,937
Computer equipment and software	15,332	13,812
Machinery and lab equipment	47,496	33,187
Furniture and fixtures	3,812	3,805
Assets in process	14,349	9,017
	109,958	79,758
Less accumulated depreciation	(37,368)	(27,173)
Total	$ 72,590	$ 52,585

Depreciation expense was approximately $12.3 million, $9.4 million, and $6.6 million for the years ended December 31, 2022, 2021, and 2020, respectively.

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6. Leases

The Company leases office space in South Plainfield, New Jersey for its principal office under two noncancelable operating leases through August 2024, in addition to office and laboratory space in Bridgewater, New Jersey and office space in various countries for international employees primarily through workspace providers.

The Company also leases approximately 220,500 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey (the "Campus") pursuant to a Lease Agreement (the "Lease") with Hopewell Campus Owner LLC (the "Landlord"). The rental term of the Lease commenced on July 1, 2020 and has an initial term of fifteen years (the "Initial Term"), with two consecutive ten year renewal periods, each at the Company's option. The aggregate rent for the Initial Term will be approximately $111.5 million. The rental rate for the renewal periods will be 95% of the Prevailing Market Rate (as defined in the Lease) and determined at the time of the exercise of the renewal. The Company is also responsible for maintaining certain insurance and the payment of proportional taxes, utilities and common area operating expenses. The Lease contains customary events of default, representations, warranties and covenants.

Subject to the terms of the Lease, the Company has a right of first refusal to rent certain other space of the Campus, which would be triggered upon the Landlord's issuance of a second round proposal or letter of intent to another tenant for such space. The Company also may seek to build a new separate building on the Campus, which may not contain less than 75,000 square feet (the "New Building"). Upon receipt of notice of the Company's intention to build the New Building, the Landlord may, in its sole discretion, construct and lease the New Building to the Company or enter into a ground lease with the Company permitting the Company to construct the New Building. Rent terms for the New Building would be determined based on the land value, construction and project costs subject to whether the Landlord or Company constructs the New Building.

In May 2022, the Company entered into a Lease Agreement (the "Warren Lease") with Warren CC Acquisitions, LLC (the "Warren Landlord") relating to the lease of two entire buildings comprised of approximately 360,000 square feet of shell condition, modifiable space (the "Warren Premises") at a facility located in Warren, New Jersey. The rental term of the Warren Lease commenced on June 1, 2022 (the "Commencement Date"), with an initial term of seventeen years (the "Warren Initial Term"), followed by three consecutive five-year renewal periods at the Company's option. The aggregate base rent for the Warren Initial Term will be approximately $163.0 million; provided, however, that if the Company is not subject to an Event of Default (as defined in the Warren Lease), the Company will be entitled to a base rent abatement over the first three years of the Warren Initial Term of approximately $18.6 million, reducing the Company's total base rent obligation to $144.4 million. The rental rate for the renewal periods will be at the Fair Market Rental Value (as defined in the Warren Lease) and determined at the time of the exercise of the renewal. Beginning in the second lease year, the Company is also responsible for the payment of all taxes and operating expenses for the Warren Premises. As a result, the Company recorded an operating lease ROU asset of $28.9 million and an operating lease ROU liability of $28.9 million as of the Commencement Date.

The Company plans on developing the Warren Premises into office and laboratory space. The Company is entitled to an allowance of approximately $36.2 million to be provided by the Warren Landlord to be used towards such improvements. The Landlord is providing the allowance to cover those assets that are real property improvements, such as structural components, roofs, flooring, etc., whose useful lives are typically longer in nature. Upon the first issuance of a temporary certificate of occupancy for the Warren Premises, the Company will receive $5.0 million from the Landlord, which the Company has committed to fund into the construction account. The Company evaluated the leasehold improvements under ASC 842 and determined that the Company will be the owner of the improvements, and therefore the $36.2 million allowance and $5.0 million due from the Landlord were treated as lease incentives at the commencement of the lease and included in the calculation of the lease ROU asset and lease ROU liability, effectively reducing both at

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Commencement Date. In connection with the execution of the Warren Lease, the Company also committed to fund a construction account with $3.6 million to go towards the Company's improvements of the Warren Premises. Subject to the terms of the Warren Lease, the Company has a right of first offer to purchase the Warren Premises if the Warren Landlord receives a bona fide third party offer to purchase the Warren Premises or the Warren Landlord decides to sell the Warren Premises.

On June 19, 2020, the Company entered into a commercial manufacturing service agreement for a term of 12.5 years with MassBiologics of the University of Massachusetts Medical School ("MassBio"). The Company determined that the agreement was a finance lease, for which the Company recorded a finance lease ROU asset for $41.4 million and corresponding finance lease liability for $41.4 million at the onset of the lease agreement. Given that the leased asset is designed for the production of PTC's AADC program and would not have an alternate use outside the PTC gene therapy platform without incurring significant costs, the Company determined that the lease should be treated as research and development expense under ASC 730. Accordingly, the full $41.4 million relating to the finance lease ROU asset was written off and expensed to research and development during the year ended December 31, 2020. As of December 31, 2022, the balance of the finance lease liabilities-current and finance lease liabilities-non-current are $3.0 million and $18.7 million, respectively, and are directly related to the Company's MassBio agreement. As of December 31, 2021, the balance of the finance lease liabilities-current and finance lease liabilities non-current were $3.0 million and $20.1 million, respectively. Additionally, during the years ended December 31, 2022 and December 31, 2021, the Company recorded finance lease costs of $1.6 million and $1.7 million, respectively, related to interest on the lease liability.

The Company also leases certain vehicles, lab equipment, and office equipment under operating leases. The Company's operating leases have remaining lease terms ranging from 1.2 years to 16.4 years and certain leases include renewal options to extend the lease for up to 15 years. Rent expense was approximately $25.2 million, $21.4 million, and $15.3 million for the years ended December 31, 2022, 2021 and 2020.

The components of lease expense were as follows:

	Year Ended December 31, 2022	Year Ended December 31, 2021	Year Ended December 31, 2020
Operating Lease Cost			
Fixed lease cost	$ 19,804	$ 16,411	$ 12,368
Variable lease cost	4,557	4,361	2,448
Short-term lease cost	808	656	450
Total operating lease cost	$ 25,169	$ 21,428	$ 15,266

Total operating lease cost is a component of operating expenses on the consolidated statements of operations.

Supplemental lease term and discount rate information related to leases was as follows:

	December 31, 2022	December 31, 2021
Weighted-average remaining lease terms - operating leases (years)	11.61	10.87
Weighted-average discount rate - operating leases	8.61 %	8.91 %
Weighted-average remaining lease terms - finance lease (years)	10.01	11.00
Weighted-average discount rate - finance lease	7.80 %	7.80 %

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Supplemental cash flow information related to leases was as follows:

	Year Ended December 31,		
	2022	2021	2020
Cash paid for amounts included in the measurement of lease liabilities:			
Operating cash flows from operating leases	$ 14,736	$ 13,683	$ 8,462
Financing cash flows from finance lease	1,276	2,224	17,829
Operating cash flows from finance leases	1,724	776	171
Right-of-use assets obtained in exchange for lease obligations:			
Operating leases	$ 35,817	$ 645	$ 76,811
Finance lease	—	—	41,382

Future minimum lease payments under non-cancelable leases as of December 31, 2022 were as follows:

	Operating Leases	Finance Lease
2023	$ 15,373	$ 3,000
2024	18,456	3,000
2025	20,431	3,000
2026	19,985	3,000
2027 and thereafter	193,792	18,000
Total lease payments	268,037	30,000
Less: Imputed Interest expense	157,807	8,325
Total	$ 110,230	$ 21,675

7. Accounts payable and accrued expenses

Accounts payable and accrued expenses at December 31, 2022 and 2021 consist of the following:

	December 31,	
	2022	2021
Employee compensation, benefits, and related accruals	$ 62,669	$ 55,733
Income tax payable	4,712	1,287
Consulting and contracted research	38,882	26,434
Professional fees	3,093	3,547
Sales allowance	63,787	61,662
Sales rebates	67,355	68,770
Royalties	40,546	35,679
Accounts payable	27,268	23,033
Other	12,054	12,639
Total	$ 320,366	$ 288,784

8. Debt

Liability for sale of future royalties

In July 2020, the Company entered into the Royalty Purchase Agreement. As RPI's interest is explicitly limited, the $650.0 million cash consideration was classified as debt and is recorded as "liability for sale of future royalties-current"

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and “liability for sale of future royalties-noncurrent” on the Company’s consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of future royalties at the time of the transaction was based on the Company’s estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability will be amortized using the effective interest method over the life of the arrangement, in accordance ASC 470 and ASC 835. The initial annual effective interest rate was determined to be 11.0%. The Company utilizes the prospective method to account for subsequent changes in the estimated future payments to be made to RPI and updates the effective interest rate on a quarterly basis. Issuance costs related to the transaction were determined to be immaterial.

The following table shows the activity within the “liability for sale of future royalties- current” and “liability for sale of future royalties- noncurrent” accounts for the year ended December 31, 2022:

Liability for sale of future royalties- (current and noncurrent)	Year Ended December 31, 2022
Beginning balance as of December 31, 2021	$ 733,985
Less: Non-cash royalty revenue payable to RPI	(48,738)
Plus: Non-cash interest expense recognized	72,639
Ending balance	$ 757,886
Effective interest rate as of December 31, 2022	8.7 %

Non-cash interest expense is recorded in the statement of operations within “Interest expense, net”.

Senior Secured Term Loan

On October 27, 2022 (the “Closing Date”), the Company entered into a credit agreement (the “Blackstone Credit Agreement”) for fundings of up to $950.0 million consisting of a committed loan facility of $450.0 million and further contemplating the potential for up to $500.0 million of additional financing, to the extent that the Company requests such additional financing and subject to the Lenders’ agreement to provide such additional financing and to mutual agreement on terms, among the Company, certain subsidiaries of the Company (together with the Company, the “Loan Parties”) and funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit (collectively, “Blackstone”, and such lenders, together with their permitted assignees, the “Lenders” and each a “Lender”) and Wilmington Trust, National Association, as the administrative agent for the Lenders.

The Blackstone Credit Agreement provides for a senior secured term loan facility funded on the Closing Date in the aggregate principal amount of $300.0 million (the “Initial Loans”) and a committed delayed draw term loan facility of up to $150.0 million (the “Delayed Draw Loans” and, together with the Initial Loans, the “Loans”) to be funded at the Company’s request within 18 months of the Closing Date subject to specified conditions. In addition, the Blackstone Credit Agreement contemplates the potential for further financings by Blackstone, by providing for incremental discretionary uncommitted further financings of up to $500.0 million. The Company capitalized approximately $11.6 million of debt issuance costs which are presented on the balance sheet as a direct deduction from the debt liability and are being amortized over the term of the senior secured term loan facility using the effective interest rate method.

The Loans mature on the date that is seven years from the Closing Date. Borrowings under the Blackstone Credit Agreement bear interest at a variable rate equal to, at the Company’s option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent

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(1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively. Payment of the Loans are subject to certain premiums specified in the Blackstone Credit Agreement, in each case, from the date of the applicable Loan funded.

All obligations under the Blackstone Credit Agreement are secured, subject to certain exceptions and specified inclusions, by security interests in certain assets of the Loan Parties, including (1) intellectual property and other assets related to Translarna, Emflaza, Upstaza, sepiapterin and, until certain release conditions are met, vatiquinone, in each case, together with any other forms, formulations, or methods of delivery of any such products, and regardless of trade or brand name, (2) future acquired intellectual property (but not internally developed intellectual property unrelated to other intellectual property collateral) and other related assets, and (3) the equity interests held by the Loan Parties in certain of their subsidiaries. The Blackstone Credit Agreement contains certain negative covenants with which the Company must remain in compliance. The Blackstone Credit Agreement also requires that the Company maintains consolidated liquidity of at least $100.0 million as of the last day of each fiscal quarter, which shall be increased to $200.0 million upon the Company consummating acquisitions meeting certain consolidated thresholds described therein. In addition, the Company will be required under conditions specified in the Blackstone Credit Agreement to fund a reserve account up to certain amounts specified therein. The funds in the reserve account are available to prepay the Loans at any time at the Company's option, and are, if funded, subject to release upon certain further conditions. Upon any such release, such funds are freely available for use by the Company subject to the generally applicable terms and conditions of the Blackstone Credit Agreement. The Blackstone Credit Agreement contains certain customary representations and warranties, affirmative covenants and provisions relating to events of default.

The following table sets forth total interest expense recognized related to the senior secured term loan:

	Year ended December 31, 2022
Contractual interest expense	$ 6,069
Amortization of debt issuance costs	290
Total	$ 6,359
Effective interest rate	12.2 %

2026 Convertible Notes

In September 2019, the Company issued, at par value, $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026, which included an option to purchase up to an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes, which was exercised in full by the initial purchasers. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. The net proceeds to the Company from the offering were $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by the Company.

The 2026 Convertible Notes are governed by an indenture (the "2026 Convertible Notes Indenture") with U.S Bank National Association as trustee (the "2026 Convertible Notes Trustee").

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Holders of the 2026 Convertible Notes may convert their 2026 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding March 15, 2026 only under the following circumstances:

- during any calendar quarter commencing on or after December 31, 2019 (and only during such calendar quarter), if the last reported sale price of the Company's common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day;

- during the five business day period after any five consecutive trading day period (the "measurement period") in which the trading price (as defined in the 2026 Convertible Notes Indenture) per $1,000 principal amount of 2026 Convertible Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price of the Company's common stock and the conversion rate on each such trading day;

- during any period after the Company has issued notice of redemption until the close of business on the scheduled trading day immediately preceding the relevant redemption date; or

- upon the occurrence of specified corporate events.

On or after March 15, 2026, until the close of business on the business day immediately preceding the maturity date, holders may convert their 2026 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, the Company will pay or deliver, as the case may be, cash, shares of the Company's common stock or any combination thereof at the Company's election.

The conversion rate for the 2026 Convertible Notes was initially, and remains, 19.0404 shares of the Company's common stock per $1,000 principal amount of the 2026 Convertible Notes, which is equivalent to an initial conversion price of approximately $52.52 per share of the Company's common stock. The conversion rate may be subject to adjustment in some events but will not be adjusted for any accrued and unpaid interest.

The Company is not permitted to redeem the 2026 Convertible Notes prior to September 20, 2023. The Company may redeem for cash all or any portion of the 2026 Convertible Notes, at its option, if the last reported sale price of its common stock has been at least 130% of the conversion price then in effect on the last trading day of, and for at least 19 other trading days (whether or not consecutive) during, any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption, at a redemption price equal to 100% of the principal amount of the 2026 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the 2026 Convertible Notes, which means that the Company is not required to redeem or retire the 2026 Convertible Notes periodically.

If the Company undergoes a "fundamental change" (as defined in the 2026 Convertible Notes Indenture), subject to certain conditions, holders of the 2026 Convertible Notes may require the Company to repurchase for cash all or part of their 2026 Convertible Notes at a repurchase price equal to 100% of the principal amount of the 2026 Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

The 2026 Convertible Notes represent senior unsecured obligations and will rank senior in right of payment to the Company's future indebtedness that is expressly subordinated in right of payment to the notes, equal in right of payment to the Company's existing and future unsecured indebtedness that is not so subordinated, effectively junior in right of

payment to any of the Company's secured indebtedness to the extent of the value of the assets securing such indebtedness, and structurally subordinated to all existing and future indebtedness and other liabilities (including trade payables) incurred by the Company's subsidiaries. The 2026 Convertible Notes Indenture contains customary events of default with respect to the 2026 Convertible Notes, including that upon certain events of default (including the Company's failure to make any payment of principal or interest on the 2026 Convertible Notes when due and payable) occurring and continuing, the 2026 Convertible Notes Trustee by notice to the Company, or the holders of at least 25% in principal amount of the outstanding 2026 Convertible Notes by notice to the Company and the Convertible Notes Trustee, may, and the 2026 Convertible Notes Trustee at the request of such holders (subject to the provisions of the 2026 Convertible Notes Indenture) will, declare 100% of the principal of and accrued and unpaid interest, if any, on all the 2026 Convertible Notes to be due and payable. In case of certain events of bankruptcy, insolvency or reorganization, involving the Company or a significant subsidiary, 100% of the principal of and accrued and unpaid interest on the 2026 Convertible Notes will automatically become due and payable. Upon such a declaration of acceleration, such principal and accrued and unpaid interest, if any, will be due and payable immediately.

Prior to the adoption of ASU 2020-06, the Company accounted for the 2026 Convertible Notes as a liability and equity component where the carrying value of the liability component was valued based on a similar instrument. In accounting for the issuance of the 2026 Convertible Notes, the Company separated the 2026 Convertible Notes into liability and equity components. The carrying amount of the liability component was calculated by measuring the fair value of a similar liability that did not have an associated convertible feature. The carrying amount of the equity component representing the conversion option was determined by deducting the fair value of the liability component from the par value of the 2026 Convertible Notes as a whole. The excess of the principal amount of the liability component over its carrying amount, referred to as the debt discount, was amortized to interest expense over the seven-year term of the 2026 Convertible Notes. The equity component was not re-measured as long as it continued to meet the conditions for equity classification. The equity component recorded at issuance related to the 2026 Convertible Notes was $123.0 million and was recorded in additional paid-in capital.

In accounting for the transaction costs related to the issuance of the 2026 Convertible Notes, the Company allocated the total costs incurred to the liability and equity components of the 2026 Convertible Notes based on their relative values. Transaction costs attributable to the liability component were amortized to interest expense over the seven-year term of the 2026 Convertible Notes, and transaction costs attributable to the equity component were netted with the equity components in stockholders' equity. Additionally, the Company initially recorded a net deferred tax liability of $25.3 million in connection with the 2026 Convertible Notes.

Effective January 1, 2021 the Company adopted ASU 2020-06. After adoption, the Company now accounts for the 2026 Convertible Notes as a single liability measured at amortized cost. As the equity component is no longer required to be split into a separate component, the Company recorded an adjustment for the initial $123.0 million that was allocated to additional paid in capital and $16.1 million of life to date interest expense recorded as amortization of debt discount. Additionally, the net deferred tax liability recorded for the 2026 Convertible Notes was reversed. The principal amount of the liability over its carrying amount is amortized to interest expense over the seven-year term of the 2026 Convertible Notes. Since the 2026 Convertible Notes are classified as a single liability, there is no debt discount required to be amortized.

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The 2026 Convertible Notes consist of the following:

	Year ended December 31,	
Liability component	**2022**	**2021**
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(4,456)	(5,606)
Net carrying amount	$ 283,044	$ 281,894

As of December 31, 2022, the remaining contractual life of the 2026 Convertible Notes is approximately 3.7 years.

The following table sets forth total interest expense recognized related to the 2026 Convertible Notes:

	Year ended December 31,	
	2022	**2021**
Contractual interest expense	$ 4,313	$ 4,313
Amortization of debt issuance costs	1,150	1,128
Total	$ 5,463	$ 5,441
Effective interest rate	1.9 %	1.9 %

In April 2022, under the terms of the 2026 Convertible Notes Indenture, the Company paid additional interest on the 2026 Convertible Notes at a rate equal to 0.5% per annum, for a total interest payment of approximately $2.1 million, for the period beginning September 25, 2020 and ending March 14, 2022. In September 2022, under the terms of the 2026 Convertible Notes Indenture, the Company paid additional interest on the 2026 Convertible Notes at a rate equal to 0.5% per annum, for a total interest payment of approximately $0.1 million, for the period beginning March 15, 2022 and ending April 8, 2022. These amounts are not included in the table above, but were recorded as interest expense, net within the statement of operations for the year ended December 31, 2022.

2022 Convertible Notes

In August 2015, the Company issued, at par value, $150.0 million aggregate principal amount of 3.00% convertible senior notes due 2022. On August 15, 2022, the Company repaid the outstanding principal amount and accrued interest, totaling $152.3 million, of the 2022 Convertible Notes that was due upon maturity in accordance with the terms of the notes.

The 2022 Convertible Notes consisted of the following:

	Year ended December 31,	
Liability component	**2022**	**2021**
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	—	(460)
Repayment of Convertible Notes	(150,000)	—
Net carrying amount	$ —	$ 149,540

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The following table sets forth total interest expense recognized related to the 2022 Convertible Notes:

	Year ended December 31,	
	2022	2021
Contractual interest expense	$ 2,800	$ 4,500
Amortization of debt issuance costs	460	720
Total	$ 3,260	$ 5,220
Effective interest rate	3.5 %	3.5 %

9. Capital structure

Common stock

In August 2019, the Company entered into an At the Market Offering Sales Agreement (the "Sales Agreement") with Cantor Fitzgerald and RBC Capital Markets, LLC (together, the "Sales Agents"), pursuant to which, the Company may offer and sell shares of its common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended.

During the year ended December 31, 2020, the Company issued and sold an aggregate of 542,470 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $53.37 per share. During the year ended December 31, 2020, the Company received net proceeds of $28.1 million after deducting agent discounts and commissions and other offering expenses payable by the Company. No shares were sold during the years ended December 31, 2021 and 2022. The remaining shares of the Company's common stock available to be issued and sold, under the Sales Agreement, have an aggregate offering price of up to $93.0 million as of December 31, 2022.

On April 29, 2020, the Company, certain of the former equity holders of Agilis ("the Participating Rightholders"), and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC, entered into a Rights Exchange Agreement (the "Rights Exchange Agreement"). As a result of the Rights Exchange Agreement, during the year ended December 31, 2020, the Company issued 2,821,176 shares of its common stock to Participating Rightholders. The shares had a fair value of $150.5 million upon issuance.

As a result of the Censa Merger, during the year ended December 31, 2020, the Company issued 845,364 shares of the Company's common stock to Censa securityholders, which were valued at $42.9 million based on the closing stock price on the acquisition date. The number of shares issued was determined using a 30-day VWAP pursuant to the Censa Merger Agreement.

In June 2021, the Company filed a Certificate of Amendment to its Restated Certificate of Incorporation, which increased the number of authorized shares of the Company's common stock from 125,000,000 to 250,000,000 shares.

In connection with the execution of the Blackstone Credit Agreement, the Company and certain entities affiliated with the Lenders (the "Purchasers") also entered into a stock purchase agreement (the "Stock Purchase Agreement") on the Closing Date for the sale and issuance of 1,095,290 shares of common stock (the "Shares") to the Purchasers at a price of $45.65 per share, for an aggregate purchase price of approximately $50.0 million. The per share price represents the closing price of the Company's common stock on the Nasdaq Global Select Market on October 26, 2022.

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Under the Stock Purchase Agreement, the Company agreed to register the resale of the Shares on a registration statement to be filed with the Securities and Exchange Commission within 60 days of the Closing Date. The Company has agreed to keep such registration statement effective for a period of six months following the Closing Date. In addition, subject to certain conditions, the Purchasers will be entitled to participate in registered underwritten public offerings by the Company during such period.

Pursuant to the terms of the Stock Purchase Agreement, the Purchasers and certain of their controlled affiliates have agreed not to, without the prior written approval of the Company and subject to specified conditions, directly or indirectly acquire shares of the Company's outstanding common stock in excess of specified thresholds, seek or propose any acquisition of all or substantially all of the assets of the Company, seek or propose a merger or other business combination involving the Company, solicit proxies or consents with respect to any securities of the Company, seek to influence the management, board of directors or policies of the Company, or undertake other specified actions related to the potential acquisition of additional equity interests in the Company, or to encourage others to do any of the above (the "Standstill Restrictions"). The Standstill Restrictions terminate on the earliest of (i) the commencement of a tender offer or exchange offer by a third party unaffiliated with the Purchasers for more than 50% of the Company's outstanding common stock, (ii) the public announcement by the Company of a written agreement to consummate a change of control of the Company, (iii) the public announcement of the Company's voluntary or involuntary bankruptcy and (iv) the termination of the Blackstone Credit Agreement.

The Purchasers have also agreed not to sell or transfer the Shares without the prior written approval of the Company for a period of 90 days following the Closing Date, subject to certain exceptions.

As of December 31, 2022, the Company's number of authorized shares of common stock was 250,000,000.

10. Net loss per share

Basic and diluted net loss per share is computed by dividing net loss available to common stockholders by the weighted-average number of common shares outstanding. Potentially dilutive securities were excluded from the diluted calculation because their effect would be anti-dilutive.

The following table sets forth the computation of basic and diluted net loss per share for common stockholders:

	Year ended December 31,		
	2022	2021	2020
Numerator			
Net loss	$ (559,017)	$ (523,901)	$ (438,160)
Denominator			
Denominator for basic and diluted net loss per share	71,728,634	70,466,393	66,027,908
Net loss per share:			
Basic and diluted	$ (7.79)*	$ (7.43)*	$ (6.64)*

* For the years ended December 31, 2022, 2021, and 2020, the Company experienced a net loss and therefore did not report any dilutive share impact.

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The following table shows historical dilutive common share equivalents outstanding, which are not included in the above historical calculation, as the effect of their inclusion is anti-dilutive during each period.

	As of December 31,		
	2022	2021	2020
Stock Options	11,502,417	10,772,582	9,663,677
Unvested restricted stock awards and units	2,516,336	1,519,831	982,058
Total	14,018,753	12,292,413	10,645,735

11. Stock award plan

In May 2013, the Company's Board of Directors and stockholders approved the 2013 Long Term Incentive Plan, which became effective upon the closing of the Company's IPO. The 2013 Long Term Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards. On June 8, 2022 (the "Restatement Effective Date"), the Company's stockholders approved the Amended and Restated 2013 Long-Term Incentive Plan (the "Amended 2013 LTIP"). The Amended 2013 LTIP provides for the grant of incentive stock options, nonstatutory stock options, restricted stock units and other stock-based awards. The number of shares of common stock reserved for issuance under the Amended 2013 LTIP is the sum of (A) the number of shares of the Company's common stock (up to 16,724,212 shares) that is equal to the sum of (1) the number of shares issued under the 2013 Long-Term Incentive Plan prior to the Restatement Effective Date, (2) the number of shares that remain available for issuance under the 2013 Long-Term Incentive Plan immediately prior to the Restatement Effective Date and (3) the number of shares subject to awards granted under the 2013 Long-Term Incentive Plan prior to the Restatement Effective Date that are outstanding as of the Restatement Effective Date, plus (B) from and after the Restatement Effective Date, an additional 8,475,000 shares of Common Stock. As of December 31, 2022, awards for 9,126,463 shares of common stock were available for issuance under the Amended 2013 LTIP.

There are no additional shares of common stock available for issuance under the Company's 1998 Employee, Director and Consultant Stock Option Plan, 2009 Equity and Long Term Incentive Plan or 2013 Stock Incentive Plan.

In January 2020, the Company's Board of Directors approved the 2020 Inducement Stock Incentive Plan. The 2020 Inducement Stock Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards, initially up to an aggregate of 1,000,000 shares of common stock. Any grants made under the 2020 Inducement Stock Incentive Plan must be made pursuant to the Nasdaq Listing Rule 5635(c)(4) inducement grant exception as a material component of the Company's new hires' employment compensation. In December 2020, the Company's Board of Directors approved an additional 1,000,000 shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan. In April 2022, the Company's Board of Directors approved a reduction in the total number of shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan to 1,300,000 shares. In December 2022, the Company's Board of Directors approved an additional 1,700,000 shares of common stock that may be issued under the 2020 Inducement Stock Inventive Plan. As of December 31, 2022, awards for 1,820,565 shares of common stock are available for issuance under the 2020 Inducement Stock Incentive Plan.

The Board of Directors has the authority to select the individuals to whom options are granted and determine the terms of each option, including (i) the number of shares of common stock subject to the option; (ii) the date on which the option becomes exercisable; (iii) the option exercise price, which, in the case of incentive stock options, must be at least 100% (110% in the case of incentive stock options granted to a stockholder owning in excess of 10% of the Company's stock) of the fair market value of the common stock as of the date of grant; and (iv) the duration of the option (which, in the case of incentive stock options, may not exceed ten years). Options typically vest over a four-year period.

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Inducement stock option awards

Pursuant to the Nasdaq inducement grant exception, during the year ended December 31, 2022, the Company issued options to purchase an aggregate of 104,385 shares of common stock to certain new hire employees at a weighted-average exercise price of $42.02 per share under the 2020 Inducement Stock Incentive Plan. Additionally, during the year ended December 31, 2022, the Company issued 43,800 restricted stock units under the 2020 Inducement Stock Incentive Plan. An aggregate of 237,700 of options and 26,336 of restricted stock units previously granted as inducement awards were forfeited during the year ended December 31, 2022 in connection with employee separations from the Company.

Stock option activity—A summary of stock option activity is as follows:

	Number of options	Weighted-average exercise price	Weighted-average remaining contractual term	Aggregate intrinsic value(in thousands)
Outstanding at December 31, 2019	11,043,939	$ 31.67		
Granted	2,777,975	$ 51.06		
Exercised	(3,268,452)	$ 24.25		
Forfeited	(889,785)	$ 42.14		
Outstanding at December 31, 2020	9,663,677	$ 38.72		
Granted	2,487,234	$ 61.36		
Exercised	(635,871)	$ 28.01		
Forfeited	(742,458)	$ 52.04		
Outstanding at December 31, 2021	10,772,582	$ 43.66		
Granted	1,685,435	$ 38.55		
Exercised	(496,863)	$ 29.45		
Forfeited/Cancelled	(458,737)	$ 48.75		
Outstanding at December 31, 2022	11,502,417	$ 43.33	6.38 years	$ 38,330
Vested or Expected to vest at December 31, 2022	3,432,725	$ 47.74	8.20 years	$ 1,694
Exercisable at December 31, 2022	7,774,274	$ 41.23	5.49 years	$ 36,523

The fair values of grants made in the years ended December 31, 2022, 2021 and 2020 were contemporaneously estimated on the date of grant using the following assumptions:

	2022	2021	2020
Risk-free interest rate	1.55% - 4.57%	0.51% - 1.24%	0.34% - 1.45%
Expected volatility	54% - 74%	74% - 89%	87% - 89%
Expected term	5.5 years	5.5 years	5.75 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the years ended December 31, 2022, 2021 and 2020 was $23.54, $43.05, and $36.94 per share, respectively.

Restricted Stock Awards and Restricted Stock Units—Restricted stock awards and Restricted stock units are granted subject to certain restrictions, including in some cases service conditions (restricted stock). The grant-date fair value of

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restricted stock awards, which has been determined based upon the market value of the Company's shares on the grant date, is expensed over the vesting period.

The following table summarizes information on the Company's restricted stock awards and units:

	Restricted Stock Awards and Units	
	Number of Shares	Weighted Average Grant Date Fair Value
Unvested at December 31, 2021	1,519,831	$ 55.43
Granted	1,686,467	38.37
Vested	(504,851)	50.15
Forfeited	(185,111)	46.67
Unvested at December 31, 2022	2,516,336	$ 45.67

Employee Stock Purchase Plan—In June 2016, the Company established an Employee Stock Purchase Plan (as amended, "ESPP" or the "Plan") for certain eligible employees. The Plan is administered by the Company's Board of Directors or a committee appointed by the Board. In June 2021, the Plan was amended to increase the total number of shares available for purchase under the Plan from one million shares to two million shares of the Company's common stock. Employees may participate over a six-month period through payroll withholdings and may purchase, at the end of the six-month period, the Company's common stock at a purchase price of at least 85% of the closing price of a share of the Company's common stock on the first business day of the offering period or the closing price of a share of the Company's common stock on the last business day of the offering period, whichever is lower. No participant will be granted a right to purchase the Company's common stock under the Plan if such participant would own more than 5% of the total combined voting power of the Company or any subsidiary of the Company after such purchase. For the period ending December 31, 2022, the Company recorded $2.4 million in compensation expense related to the ESPP.

The Company recorded share-based compensation expense in the statement of operations related to incentive stock options, nonstatutory stock options, restricted stock awards, restricted stock units and the ESPP as follows:

	Year ended December 31,		
	2022	2021	2020
Research and development	$ 55,869	$ 53,632	$ 38,716
Selling, general and administrative	54,464	49,881	31,609
Total	$ 110,333	$ 103,513	$ 70,325

As of December 31, 2022, there was approximately $176.1 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the Company's Plans. This cost is expected to be recognized as compensation expense over the weighted average remaining service period of approximately 2.19 years.

12. Other comprehensive income (loss) and accumulated other comprehensive items

Other comprehensive income (loss) includes changes in equity that are excluded from net loss, such as unrealized gains and losses on marketable securities.

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The following table summarizes other comprehensive income (loss) and the changes in accumulated other comprehensive items, by component, for the years ended December 31, 2022, 2021, and 2020, respectively.

	Unrealized Gains (Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2019	$ 755	$ (11,339)	$ (10,584)
Other comprehensive income (loss) before reclassifications	479	(51,518)	(51,039)
Amounts reclassified from other comprehensive items	666	—	666
Other comprehensive income (loss)	1,145	(51,518)	(50,373)
Balance at December 31, 2020	$ 1,900	$ (62,857)	$ (60,957)
Other comprehensive (loss) income before reclassifications	(3,279)	39,177	35,898
Amounts reclassified from other comprehensive items	777	—	777
Other comprehensive (loss) income	(2,502)	39,177	36,675
Balance at December 31, 2021	$ (602)	$ (23,680)	$ (24,282)
Other comprehensive income before reclassifications	4,072	28,970	33,042
Amounts reclassified from other comprehensive items	(3,964)	—	(3,964)
Other comprehensive income	108	28,970	29,078
Balance at December 31, 2022	$ (494)	$ 5,290	$ 4,796

Reclassified amounts from other comprehensive items were determined using the actual realized gains and losses from the sales of marketable securities.

13. Revenue recognition

Net product sales

During the years ended December 31, 2022, 2021, and 2020, net product sales in the United States were $218.3 million, $187.3 million, and $139.0 million, respectively, consisting solely of sales of Emflaza, and net product sales outside of the United States were $316.9 million, $241.6 million, and $194.4 million respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net product revenues made up $288.6 million, $236.0 million, and $191.9 million of the net product sales outside the United States for the years ended December 31, 2022, 2021, and 2020, respectively. During the year ended December 31, 2022, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $218.3 million and $59.7 million of the net product sales, respectively. During the years ended December 31, 2021 and 2020, only the United States accounted for at least 10% of the Company's net product sales. For the years ended December 31, 2022, 2021, and 2020, two of the Company's distributors each accounted for over 10% of the Company's net product sales.

As of December 31, 2022, the Company has a contract liabilities balance of $1.4 million relating to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. As of December 31, 2021, the Company did not have a contract liabilities balance. The Company did not have any contract assets for the years ended December 31, 2022 and 2021. For the year ended December 31, 2022, the Company did not recognize any revenue related to the amounts included in the contract liability balance at the beginning of the period. For the year ended December 31, 2021, the Company recognized revenues of $4.0 million related to amounts included in contract liability balance at the

beginning of the period, which related to Translarna net product revenue. The Company has not made significant changes to the judgments made in applying ASC Topic 606 for the years ended December 31, 2022 and 2021.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of December 31, 2022, the aggregate amount of transaction price allocated to remaining performance obligations related to plasmid DNA and AAV vector production for external customers is $1.4 million. The Company expects to recognize revenue over the next one year, as the specific timing for satisfying the performance obligations is contingent upon a number of factors, including customers' needs and schedules. As of December 31, 2021, the Company did not have any remaining performance obligations.

Collaboration revenue and Royalty revenue

In November 2011, the Company and the Spinal Muscular Atrophy Foundation ("SMA Foundation") entered into a licensing and collaboration agreement with F. Hoffman-La Roche Ltd and Hoffman- La Roche Inc. (collectively, "Roche"). Under the terms of the SMA License Agreement, Roche acquired an exclusive worldwide license to the Company's SMA program.

Under the agreement, the Company is eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of certain sales events, and up to double digit royalties on worldwide annual net sales of a commercial product.

For the years ended December 31, 2022, 2021, and 2020, the Company recognized revenue related to the licensing and collaboration agreement with Roche of $50.1 million, $55.0 million, and $42.6 million, respectively. The below summarizes the milestone achievements associated with the Company's SMA program during the years ended December 31, 2022, 2021, and 2020.

The SMA program currently has one approved product, Evrysdi, which was approved in August 2020 by the FDA for the treatment of SMA in adults and children two months and older. The first commercial sale of Evrysdi in the United States was made in August 2020. This event triggered a $20.0 million milestone payment to the Company from Roche. In August 2020, the EMA accepted the MAA filed by Roche for Evrysdi for the treatment of SMA, which triggered a $15.0 million milestone payment to the Company from Roche. In October 2020, Chugai, a subsidiary of Roche, filed an NDA in Japan for Evrysdi for the treatment of SMA, which triggered a $7.5 million milestone payment to the Company from Roche. Under ASC Topic 606, the acceptance of the NDA filing resolved the uncertainty of whether the milestone was probable of being achieved. The Company recorded these three milestone payments as collaboration revenue for the year ended December 31, 2020.

The first commercial sale of Evrysdi in the EU was made in March 2021. This event triggered a $20.0 million milestone payment to the Company from Roche. The first commercial sale in Japan was made in August 2021, which was the final research and development milestone received by the Company. This event triggered a $10.0 million payment to the Company from Roche. In December 2021, the Company recorded its first sales milestone of $25.0 million for the achievement of $500.0 million in worldwide annual net sales from Evrysdi. The Company recorded these three milestone payments as collaboration revenue for the year ended December 31, 2021.

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In September 2022, the Company recognized a sales milestone of $50.0 million for the achievement of $750.0 million in worldwide annual net sales from Evrysdi, which is recorded on the balance sheet within prepaid expenses and other current assets as of December 31, 2022. The remaining potential sales milestones as of December 31, 2022 is $250.0 million upon achievement of certain sales events. As of December 31, 2022, the Company does not have any remaining research and development milestones that can be received.

In addition to research and development and sales milestones, the Company is eligible to receive up to double-digit royalties on worldwide annual net sales of a commercial product under the SMA License Agreement. For the years ended December 31, 2022, 2021, and 2020 the Company has recognized $113.5 million, $54.6 million, and $4.8 million of royalty revenue related to Evrysdi, respectively.

14. Income taxes

The loss from operations before tax benefit (expense) consisted of the following for the years ended December 31, 2022, 2021, and 2020:

	2022	2021	2020
Domestic	$ (591,126)	$ (487,726)	$ (452,475)
Foreign	3,639	(30,614)	49,543
Total	$ (587,487)	$ (518,340)	$ (402,932)

The Income Tax Provision consisted of the following for the years ended December 31, 2022, 2021 and 2020:

	2022	2021	2020
Current:			
U.S. Federal	$ —	$ —	$ —
U.S. State and Local	(4,224)	(3,844)	(24,984)
Foreign	(1,582)	(1,340)	(4,372)
Deferred:			
U.S. Federal	23,689	—	—
U.S. State and Local	10,587	(377)	(5,872)
Foreign	—	—	—
Total tax benefit (expense)	$ 28,470	$ (5,561)	$ (35,228)

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A reconciliation of the U.S. statutory income tax rate to the Company's effective tax rate is as follows:

	December 31,		
	2022	2021	2020
Federal income tax provision at statutory rate	21.00 %	21.00 %	21.00 %
State income tax provision, net of federal benefit	3.07	(0.74)	(3.31)
Permanent differences	(1.83)	(4.06)	(6.66)
Research and development	5.89	4.50	4.93
Change in valuation allowances	(23.36)	(29.03)	(26.40)
Change in deferred tax assets	(0.10)	12.05	2.93
Foreign tax rate differential	(0.17)	0.01	0.72
Tax rate change	0.34	0.01	(1.46)
(Accrual) Release of uncertain tax positions	—	(4.78)	(0.61)
Other	—	(0.03)	0.12
Effective income tax rate	4.84 %	(1.07)%	(8.74)%

Accounting for income taxes under U.S. GAAP requires that individual tax-paying entities of the company offset all deferred tax liabilities and assets within each particular tax jurisdiction and present them as a noncurrent deferred tax liability or asset. Amounts in different tax jurisdictions cannot be offset against each other. The noncurrent deferred income tax asset is recorded within deposits and other assets on the balance sheet. The amount of deferred income taxes are as follows:

	December 31,	
	2022	2021
Assets:		
Noncurrent deferred income taxes	$ —	$ —
Liabilities:		
Noncurrent deferred income taxes	(102,834)	(137,110)
Deferred income taxes - net	$ (102,834)	$ (137,110)

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The significant components of the Company's deferred tax assets and liabilities at December 31, 2022 and 2021 are as follows:

	2022	2021
Deferred tax assets:		
Accrued expense	$ 2,124	$ 8,208
Amortization	52,532	87,998
Federal tax credits	174,802	142,595
State tax credits	9,787	8,054
Federal net operating losses	69,957	76,589
State net operating losses	10,316	9,159
Foreign net operating losses	4,837	3,316
Capitalized research and development costs	110,219	241
Share based compensation and other	27,054	15,273
Liability for sale of future royalties	185,589	148,503
Noncash interest expense	30,160	26,040
Other comprehensive loss	(719)	143
Total gross deferred tax assets	676,658	526,119
Less valuation allowance	(672,172)	(525,570)
Total deferred tax assets, net of valuation allowance	$ 4,486	$ 549
Deferred tax liabilities:		
Depreciation	$ (4,486)	$ (549)
Indefinite lived intangible	(102,834)	(137,110)
Total gross deferred tax liabilities	(107,320)	(137,659)
Net deferred tax assets (liabilities)	$ (102,834)	$ (137,110)

For the year ended December 31, 2022, the Company generated taxable income in the U.S. of $61.6 million. The Company has not recorded any federal income tax provision after considering the federal NOL, section 250 deduction, available general business credits, and foreign tax credits. The Company recorded a state income tax provision of $4.2 million which is primarily attributable to state income taxes paid in the current year.

At December 31, 2022 and 2021, the Company recorded a valuation allowance against its net deferred tax assets of $672.2 million and $525.6 million, respectively. The change in the valuation allowance during the years ended December 31, 2022 and 2021 was $146.6 million and $146.0 million, respectively. A valuation allowance has been recorded since, in the judgment of management, these assets are not more likely than not to be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during periods in which those temporary differences and carryforwards become deductible or are utilized. As of December 31, 2022, the Company had $333.1 million and $164.9 million of federal and state net operating loss carryforwards, respectively.

The Company recorded a deferred tax liability in conjunction with the Agilis Merger of $122.0 million in 2018, related to the tax basis difference in the IPRD indefinite-lived intangibles acquired. The Company's policy is to record a deferred tax liability related to acquired IPR&D which may eventually be realized either upon amortization of the asset when the research is completed, and a product is successfully launched or the write-off of the asset if it is abandoned or unsuccessful. In July 2022, the Company received EMEA approval for a portion of the IPR&D assets, and thus, began the amortization of the intangible.

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As of December 31, 2022, research and development credit carryforward for federal purposes is $29.0 million. In addition, the Orphan Drug Credit Carryover available as of December 31, 2022 is $145.8 million. The Company's federal credit carryforwards could begin to expire in 2023 if not otherwise utilized as projected.

As a result of U.S. tax reform legislation, federal net operating losses generated in 2018 carryforward indefinitely. State net operating loss carryforwards begin to expire in 2037. Sections 382 and 383 of the Internal Revenue Code of 1986 subject the future utilization of net operating losses and certain other tax attributes, such as research and development tax credits, to an annual limitation in the event of certain ownership changes, as defined. The Company has undergone an ownership change and has determined that a "change in ownership" as defined by IRC Section 382 of the Internal Revenue Code of 1986, as amended, and the rules and regulations promulgated thereunder, did occur in June of 2013. Accordingly, about $231.5 million of the Company's NOL carryforwards are limited and the Company can only use $16.7 million for the first five years from the ownership change and $5.7 million per year going forward. Therefore, $169.2 million of the NOL's will be freed up over the next 10 years and $62.3 million are expected to expire unused which are not included in the deferred tax assets listed above. At December 31, 2020, the Company utilized $364.1 million of NOLs of which $97.7 million was the section 382 NOL. The Company did not utilize any NOLs in the year ended December 31, 2021. At December 31, 2022, the Company utilized $49.3 million of NOLs of which $11.4 million is section 382 NOL. At December 31, 2022, there is $333.1 million available for immediate use and an additional $5.7 million will free up in 2023.

The income tax benefit (expense) for the years ended December 31, 2022 and 2021 differed from the amounts computed by applying the U.S. federal income tax rate of 21% to loss before tax expense as a result of the IPR&D assets becoming partially amortizable in 2022, foreign taxes, the impact of temporary difference, including the updated section 174, the impact of permanent differences, including "global intangible low-taxed income" ("GILTI"), tax credits generated, true up of net operating loss carryforwards, and increase in the Company's valuation allowance.

Under the 2017 Tax Cuts and Jobs Act, the ability to currently deduct qualifying research and experimental costs under section 174, as well as software development costs, are eliminated for tax years beginning after December 31, 2021. Under the new rule, these costs must be capitalized and amortized over a five-year or fifteen-year period, depending on whether the research is conducted in the U.S. or abroad, respectively. The rule became effective for the Company during the year, and resulted in an increased current taxable income of the Company by $450.1 million for the tax year ended December 31, 2022.

The Company applies the elements of FASB ASC 740-10 regarding accounting for uncertainty in income taxes. This clarifies the accounting for uncertainty in income taxes recognized in financial statements and required impact of a tax position to be recognized in the financial statements if that position is more likely than not of being sustained by the taxing authority. As of December 31, 2022, the Company recorded unrecognized tax benefits in the amount of $27.2 million including interest and penalties through 2022. The Company's policy is to recognize interest and penalties related to tax matters within the income tax provision. Tax years beginning in 2014 are generally subject to examination by taxing authorities, although net operating losses from all years are subject to examinations and adjustments for at least three years following the year in which the attributes are used. The Company is currently under a wage tax audit in Germany for tax years 2018 through 2021. Although the outcome of tax audits is always uncertain, the company does not expect any adjustment to result for these years as of December 31, 2022.

For all years through December 31, 2016, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's research and

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development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carryforwards and the valuation allowance.

As a result of U.S. tax reform legislation, distributions of profits from non-U.S. subsidiaries are not expected to cause a significant incremental U.S. tax impact in the future. However, distributions may be subject to non-U.S. withholding taxes if profits are distributed from certain jurisdictions. As of December 31, 2022, for purposes of ASC 740-10-25-3, the Company had $65.1 million of undistributed earnings from non-U.S. subsidiaries that it intends to reinvest permanently in its non-U.S. operations. As these ASC 740-10-25-3 earnings are considered permanently reinvested, no tax provision has been accrued. It is not feasible to estimate the amount of tax that might be payable on the eventual remittance of such earnings.

Unrecognized Tax Benefits

A reconciliation of the gross amount of unrecognized tax benefits, excluding accrued interest and penalties, is as follows:

	Unrecognized Tax Benefits
Balance at December 31, 2021	27,217
Additions based on tax positions related to the current year	—
Balance at December 31, 2022	$ 27,217

Uncertain tax positions, for which management's assessment is that there is a more than 50% probability of sustaining the position upon challenge by a taxing authority based upon its technical merits, are subject to certain recognition and measurement criteria. The nature of the uncertain tax positions is often very complex and subject to change, and the amounts at issue can be substantial. The Company develops its cumulative probability assessment of the measurement of uncertain tax positions using internal experience, judgment, and assistance from professional advisors. The Company re-evaluates these uncertain tax positions on a quarterly basis based on a number of factors including, but not limited to, changes in facts or circumstances, changes in tax law, and effectively settled issues under audit and new audit activity. Any change in these factors could result in the recognition of a tax benefit or an additional charge to the tax provision.

For the year ended December 31, 2022, the Company did not record any unrecognized tax benefits. While it is reasonably possible that a further change in the unrecognized tax benefits may occur within the next twelve months, the Company is unable to estimate the amount of any such change.

The Company records penalties and tax-related interest expense on unrecognized tax benefits as a component of the provision for income taxes in the accompanying consolidated statement of operations. The Company has not recorded any interest and penalties related to uncertain tax positions for the year ended December 31, 2022, in the accompanying consolidated balance sheet. Future changes in the Company’s unrecognized tax benefits will affect the Company’s annual effective tax rate.

15. Commitments and contingencies

Under various agreements, the Company will be required to pay royalties and milestone payments upon the successful development and commercialization of products. The Company has entered into funding agreements with The Wellcome Trust Limited (“Wellcome Trust”) for the research and development of small molecule compounds in connection with its

oncology and antibacterial programs. As the Company has discontinued development under its antibacterial program, it no longer expects that milestone and royalty payments from the Company to Wellcome Trust will apply under that agreement, resulting in a change to the total amount of development and regulatory milestone payments the Company may become obligated to pay for this program. Under the oncology platform funding agreement, to the extent that the Company develops and commercializes certain program intellectual property on a for-profit basis itself or in collaboration with a partner (provided the Company retains overall control of worldwide commercialization), the Company may become obligated to pay to Wellcome Trust development and regulatory milestone payments and single-digit royalties on sales of any research program product. The Company's obligation to pay such royalties would continue on a country-by-country basis until the longer of the expiration of the last patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country. The Company made the first development milestone payment of $0.8 million to Wellcome Trust under the oncology platform funding agreement during the second quarter of 2016. During the year ended December 31, 2022, the Company incurred $2.5 million of development milestones in connection with the enrollment of patients in the registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS, which is recorded in other long-term liabilities on the balance sheet and will be payable upon the earlier to occur of the first dose administered to the last patient enrolled in the study or the termination of dosing of all patients in the study. Additional milestone payments of up to an aggregate of $14.5 million may become payable by the Company to Wellcome Trust under this agreement.

The Company has also entered into a collaboration agreement with the SMA Foundation. The Company may become obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that is successfully developed and subsequently commercialized or, with respect to collaboration products the Company outlicenses, including Evrysdi, a specified percentage of certain payments the Company receives from its licensee. The Company is not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. Since inception, the SMA Foundation has earned $28.5 million, $24.5 million which was paid and $4.0 million which was accrued as of December 31, 2022. The Company's obligation to make such payments would end upon its payment to the SMA Foundation of an aggregate of $52.5 million.

Pursuant to the asset purchase agreement ("Asset Purchase Agreement") between the Company and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC) ("Marathon"), Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza up to a specified aggregate maximum amount over the expected commercial life of the asset. In addition, Marathon received a $50.0 million sales-based milestone during the year ended December 31, 2022.

Pursuant to the Agilis Merger Agreement with Agilis, Agilis equityholders were previously entitled to receive contingent consideration payments from the Company based on (i) the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million, (ii) the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher up to an aggregate maximum amount of $535.0 million, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $150.0 million, and (iv) a percentage of annual net sales for Friedreich Ataxia and Angelman Syndrome during specified terms, ranging from 2%-6%. The Company was required to pay $40.0 million of the development milestone payments upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the applicable milestones have been achieved.

Pursuant to the terms of the Rights Exchange Agreement, the Participating Rightholders canceled and forfeited their rights under the Agilis Merger Agreement to receive (i) $174.0 million, in the aggregate, of potential milestone payments based on the achievement of certain regulatory milestones and (ii) $37.6 million, in the aggregate, of $40.0 million in

development milestone payments that would have been due upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the milestones are achieved.

The Rights Exchange Agreement has no effect on the Agilis Merger Agreement other than to provide for the cancellation and forfeiture of the Participating Rightholders' rights to receive $211.6 million, in the aggregate, of the milestone payments described above. As a result, all other rights and obligations under the Agilis Merger Agreement remain in effect pursuant to their terms, including the Company's obligation to pay up to an aggregate maximum amount of $20.0 million upon the achievement of certain development milestones (representing the remaining portion of potential development milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement while excluding the remaining $2.4 million milestone payment that was due and paid upon the passing of the second anniversary of the closing of the Agilis Merger), up to an aggregate maximum amount of $361.0 million upon the achievement of certain regulatory milestones (representing the remaining portion of potential regulatory milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement), up to a maximum aggregate amount of $150.0 million upon the achievement of certain net sales milestones and a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2% to 6%, pursuant to the terms of the Agilis Merger Agreement.

In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. As a result of such approval, the Company paid the former equityholders of Agilis $50.0 million in accordance with the terms of the Agilis Merger Agreement in the year ended December 31, 2022.

On October 25, 2019, the Company completed the acquisition of substantially all of the assets of BioElectron Technology Corporation ("BioElectron"), a Delaware corporation, including certain compounds that the Company has begun to develop as part of its Bio-e platform, pursuant to an asset purchase agreement by and between the Company and BioElectron, dated October 1, 2019 (the "BioElectron Asset Purchase Agreement"). BioElectron was a private company with a pipeline focused on inflammatory and central nervous system (CNS) disorders. The lead program, vatiquinone, is in late stage development for CNS disorders with substantial unmet need and significant commercial opportunity that are complementary to PTC's existing pipeline.

Subject to the terms and conditions of the BioElectron Asset Purchase Agreement, BioElectron may become entitled to receive contingent milestone payments of up to $200.0 million (in cash or in shares of the Company's common stock, as determined by the Company) from the Company based on the achievement of certain regulatory and net sales milestones. Subject to the terms and conditions of the BioElectron Asset Purchase Agreement, BioElectron may also become entitled to receive contingent payments based on a percentage of net sales of certain products.

Subject to the terms and conditions of the Censa Merger Agreement, former Censa securityholders may become entitled to receive contingent payments from the Company based on (i) the achievement of certain development and regulatory milestones up to an aggregate maximum amount of $217.5 million for sepiapterin's two most advanced programs and receipt of a priority review voucher from the FDA as set forth in the Censa Merger Agreement, (ii) $109.0 million in development and regulatory milestones for each additional indication of sepiapterin, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $160.0 million, (iv) a percentage of annual net sales during specified terms, ranging from single to low double digits of the applicable net sales threshold amount, and (v) any sublicense fees paid to the Company in consideration of any sublicense of Censa's intellectual property to commercialize sepiapterin, on a country-by-country basis, which contingent payment will equal to a mid-double digit percentage of any such sublicense fees. Pursuant to the Censa Merger Agreement, the Company has the option to pay the initial $30.0 million development milestone, for the completion of enrollment of a Phase 3 clinical trial for sepiapterin for PKU, if achieved, in cash or shares of the Company's common stock.

PTC Therapeutics, Inc.

Notes to consolidated financial statements (Continued)

December 31, 2022

(In thousands except share and per share amount)

The Company also has the Tegsedi-Waylivra Agreement for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean. Akcea is entitled to receive royalty payments subject to certain terms set forth in the Tegsedi-Waylivra Agreement.

The Company has employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur. Additionally, the Company has royalty payments associated with Translarna, Emflaza, and Upstaza net product revenue, payable quarterly or annually in accordance with the terms of the related agreements.

From time to time in the ordinary course of its business, the Company is subject to claims, legal proceedings and disputes. The Company is not currently aware of any material legal proceedings against it.

16. Geographic information

The Company views its operations and manages its business in one operating segment. The following table presents financial information based on the geographic location of the facilities of the Company as of and for the years ended:

	Year Ended December 31, 2022		
	United States	Non-US	Total
Total assets	$ 1,473,770	$ 231,849	$ 1,705,619
Fixed assets, net	$ 71,754	$ 836	$ 72,590
Revenue	$ 498,567	$ 200,234	$ 698,801

	Year Ended December 31, 2021		
	United States	Non-US	Total
Total assets	$ 1,744,225	$ 193,831	$ 1,938,056
Fixed assets, net	$ 51,626	$ 959	$ 52,585
Revenue	$ 297,005	$ 241,588	$ 538,593

17. 401(k) plan

The Company maintains a 401(k) plan for its employees. Employee contributions are voluntary. The Company may match employee contributions in amounts to be determined at the Company's sole discretion. The Company provided an 100% matching contribution for up to the first 6% of each contributing employee's base salary contributions for the years ended December 31, 2022, 2021 and 2020, respectively. The Company made matching contributions to the 401(k) plan and recorded expense of approximately $8.4 million, $6.6 million, and $5.3 million for the years ended December 31, 2022, 2021 and 2020, respectively.

PTC Therapeutics, Inc.

Notes to consolidated financial statements (Continued)

December 31, 2022

(In thousands except share and per share amount)

18. Intangible assets and goodwill

Definite-lived intangibles

Definite lived intangible assets consisted of the following at December 31, 2022 and 2021:

Definite lived intangibles assets, gross	Ending Balance at December 31, 2021	Additions	Reclass from Indefinite Lived to Definite Lived	Impairment	Foreign currency translation	Ending Balance at December 31, 2022
Emflaza	$ 291,875	$ 128,378	$ —	$ —	$ —	$ 420,253
Waylivra	9,904	—	—	—	(588)	9,316
Tegsedi	4,060	3,240	—	—	(191)	7,109
Upstaza	—	—	89,550	—	—	89,550
Total definite lived intangibles, gross	$ 305,839	$ 131,618	$ 89,550	$ —	$ (779)	$ 526,228

Definite lived intangibles assets, accumulated amortization	Ending Balance at December 31, 2021	Amortization	Foreign currency translation	Ending Balance at December 31, 2022
Emflaza	$ (154,594)	$ (111,429)	$ —	$ (266,023)
Waylivra	(1,821)	(1,020)	90	(2,751)
Tegsedi	(1,083)	(685)	59	(1,709)
Upstaza	—	(3,420)	—	(3,420)
Total definite lived intangibles, accumulated amortization	$ (157,498)	$ (116,554)	$ 149	$ (273,903)
Total definite lived intangibles, net				$ 252,325

Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza beginning in 2018, up to a specified aggregate maximum amount over the expected commercial life of the asset. In accordance with the guidance for an asset acquisition, the Company records the milestone payment when it becomes payable to Marathon and increase the cost basis for the Emflaza rights intangible asset. For the year ended December 31, 2022, milestone payments of $128.4 million were recorded, which included a $50.0 million sales-based milestone. These payments are being amortized over the remaining useful life of the Emflaza rights asset on a straight line basis. As of December 31, 2022, a milestone payable to Marathon of $32.8 million was recorded on the balance sheet within accounts payable and accrued expenses.

Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Tegsedi-Waylivra Agreement related to sales of Waylivra and Tegsedi. In accordance with the guidance for an asset acquisition, the Company records royalty payments when they become payable to Akcea and increase the cost basis for the Waylivra and Tegsedi intangible assets, respectively. For the year ended December 31, 2022, royalty payments of $3.2 million were recorded for Tegsedi. As of December 31, 2022, a royalty payable of $0.5 million for Tegsedi was recorded on the balance sheet within accounts payable and accrued expenses.

PTC Therapeutics, Inc.

Notes to consolidated financial statements (Continued)

December 31, 2022

(In thousands except share and per share amount)

For the years ended December 31, 2022, 2021, and 2020, the Company recognized amortization expense of $116.6 million, $54.8 million, and $36.9 million respectively, related to the Emflaza rights, Waylivra, Tegsedi, and Upstaza intangible assets.

The estimated future amortization of the Emflaza rights, Waylivra, Tegsedi, and Upstaza intangible assets is expected to be as follows:

	As of December 31, 2022
2023	$ 142,324
2024	30,609
2025	9,352
2026	9,352
2027 and thereafter	60,688
Total	$ 252,325

The weighted average remaining amortization period of the definite-lived intangibles as of December 31, 2022 is 4.9 years.

Indefinite-lived intangibles

Indefinite lived intangible assets consisted of the following at December 31, 2022 and 2021:

Indefinite lived intangibles assets	Ending Balance at December 31, 2021	Additions	Reclass from Indefinite Lived to Definite Lived	Impairment	Foreign currency translation	Ending Balance at December 31, 2022
Upstaza	$ 358,700	$ —	$ (89,550)	$ (33,384)	$ —	$ 235,766
PTC-FA	112,500	—	—	—	—	112,500
PTC-AS	105,300	—	—	—	—	105,300
Total indefinite lived intangibles	$ 576,500	$ —	$ (89,550)	$ (33,384)	$ —	$ 453,566
Total intangible assets, net						$ 705,891

In connection with the acquisition of the Company's gene therapy platform from Agilis, the Company acquired rights to Upstaza, for the treatment of AADC deficiency. AADC deficiency is a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. The gene therapy platform also includes PTC-FA, an asset targeting Friedreich ataxia, a rare and life-shortening neurodegenerative disease caused by a single defect in the FXN gene which causes reduced production of the frataxin protein. Additionally, the gene therapy platform includes two other programs targeting CNS disorders, including PTC-AS for Angelman syndrome, a rare, genetic, neurological disorder characterized by severe developmental delays.

In accordance with the acquisition method of accounting, the Company allocated the acquisition cost for the Agilis Merger to the underlying assets acquired and liabilities assumed, based upon the estimated fair values of those assets and liabilities at the date of acquisition. The Company classified the fair value of the acquired IPR&D as indefinite lived

intangible assets until the successful completion or abandonment of the associated research and development efforts. The value allocated to the indefinite lived intangible assets was $576.5 million. With the approval of Upstaza by the European Commission in July 2022, $89.6 million was reclassified from indefinite lived intangible assets to definite lived intangible assets.

The Company performed an annual test for its indefinite-lived intangible assets as of October 1, 2022. In the fourth quarter of 2022, the Company recorded a partial impairment on the Upstaza indefinite lived intangible asset of $33.4 million, which is recorded as intangible asset impairment in the statement of operations. The impairment was related to a decrease in projected cash flows due to refinements in current market assumptions and the timing of patient treatments. To calculate the impairment amount, the Company utilized a discounted cash flow model under the income method, which primarily utilized Level 3 fair value inputs. The discount rate utilized in the discounted cash flow model was 15%, and the weighted average probability of success was 88%. As of December 31, 2022, the remaining balance of the Upstaza indefinite lived intangible asset is $235.8 million. No impairments were identified for the Company's additional gene therapy portfolio pertaining to PTC-FA and PTC-AS.

Goodwill

As a result of the Agilis Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. There have been no changes to the balance of goodwill since the date of the Agilis Merger. Accordingly, the goodwill balance as of December 31, 2022 and 2021 was $82.3 million. The Company performed an annual impairment test for goodwill as of October 1, 2022. The Company's single reporting unit had a negative carrying value and thus the Company determined there was no impairment of goodwill.

19. Subsequent events

In February 2023, the Company completed enrollment of its Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event and in accordance with the Censa Merger Agreement, the Company is obligated to pay a $30.0 million development milestone to the former Censa securityholders, which the Company has the option to pay in cash or shares of its common stock.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Financial Officer (principal financial officer), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2022. The term "disclosure controls and procedures", as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2022, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting for our company. Internal control over financial reporting is defined in Rule 13a-15(f) or 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, the company's principal executive and principal financial officers and effected by the company's board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP and includes those policies and procedures that: (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of our company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our company's assets that could have a material effect on the financial statements.

Internal control over financial reporting is designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements prepared for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Our management, with the participation of our Chief Executive Officer (principal executive officer) and Chief Financial Officer (principal financial officer), assessed the effectiveness of our internal control over financial reporting as of December 31, 2022. In making this assessment, our management used the criteria set forth in the *Internal Control—Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on its assessment, management concluded that our internal control over financial reporting was effective as of December 31, 2022 based on those criteria.

The effectiveness of our internal control over financial reporting as of December 31, 2022, has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in their report which appears herein.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting occurred during the year ended December 31, 2022 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of PTC Therapeutics, Inc.

Opinion on Internal Control over Financial Reporting

We have audited PTC Therapeutics, Inc.'s internal control over financial reporting as of December 31, 2022, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). In our opinion, PTC Therapeutics, Inc. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2022, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the accompanying consolidated balance sheets of the Company as of December 31, 2022 and 2021, the related consolidated statements of operations, comprehensive loss, stockholders' (deficit)/ equity and cash flows for each of the three years in the period ended December 31, 2022, and the related notes and our report dated February 21, 2023 expressed an unqualified opinion thereon.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Ernst & Young LLP
Iselin, New Jersey
February 21, 2023

Item 9B. Other Information.

None.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

None.

PART III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this item as set forth under the captions “Proposal 1—Election of Directors”, “Executive Officers”, “Delinquent Section 16(a) Reports”, “Corporate Governance—Code of Conduct”, “Corporate Governance—Director Nominations”, “Corporate Governance—Board Committees and Audit Committee”, and “Stockholder Proposals and Nominations for Director” in our Proxy Statement for the 2023 Annual Meeting of Shareholders is incorporated in this Annual Report on Form 10-K by reference.

Code of Ethics

We have adopted a written Code of Business Conduct and Ethics, which is a code of ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. We have posted a current copy of the Code of Business Conduct and Ethics on the Corporate Governance page of the Investors section of our website, *www.ptcbio.com*, and it is available in print to any person who requests it. We intend to post on our website all disclosures that are required by applicable law, the rules of the Securities and Exchange Commission or the Nasdaq Global Select Market concerning any amendment to, or waiver from, any provision of the Code of Business Conduct and Ethics.

Item 11. Executive Compensation

The information required by this item (other than the information required by Item 402(v) of Regulation S-K) as set forth in under the captions “Executive Compensation”, “2022 Director Compensation”, “Corporate Governance—Risk Oversight” and “Corporate Governance—Compensation Committee Interlocks and Insider Participation” in our Proxy Statement for the 2023 Annual Meeting of Shareholders is incorporated in this Annual Report on Form 10-K by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item as set forth under the captions “Equity Compensation Plan Information” and “Principal Stockholders” in our Proxy Statement for the 2023 Annual Meeting of Shareholders is incorporated in this Annual Report on Form 10-K by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this item as set forth under the captions “Corporate Governance—Policies and Procedures for Related Person Transactions”, “Corporate Governance—Related Person Transactions”, and “Corporate Governance—Director Independence” in our Proxy Statement for the 2023 Annual Meeting of Shareholders is incorporated in this Annual Report on Form 10-K by reference.

Item 14. Principal Accountant Fees and Services

The information required by this item as set forth under the caption “Proposal 2—Ratification of Election of Independent Registered Public Accounting Firm” in our Proxy Statement for the 2023 Annual Meeting of Shareholders is incorporated in this Annual Report on Form 10-K by reference.

PART IV

Item 15. Exhibits and Financial Statement Schedules

Financial Statements

The following statements and supplementary data are included in Part II, Item 8. of the Annual Report on Form 10-K.

- Reports of independent registered public accounting firm
- Consolidated Balance Sheets as of December 31, 2022 and 2021
- Consolidated Statements of Operations for the years ended December 31, 2022, 2021 and 2020
- Consolidated Statements of Comprehensive Loss for the years ended December 31, 2022, 2021 and 2020
- Consolidated Statements of Stockholders' Equity for the years ended December 31, 2022, 2021 and 2020
- Consolidated Statements of Cash Flows for the years ended December 31, 2022, 2021 and 2020
- Notes to Consolidated Financial Statements

Exhibits

Those exhibits required to be filed by Item 601 of Regulation S-K are listed in the Exhibit Index immediately preceding the exhibits hereto and such listing is incorporated herein by reference.

Exhibit Index

Exhibit Number	Description of Exhibit
2.1††	Asset Purchase Agreement, dated March 15, 2017, between PTC Therapeutics, Inc. and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC) (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on March 16, 2017)
2.2	Amendment to Asset Purchase Agreement, dated April 20, 2017, between PTC Therapeutics, Inc. and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC) (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on April 20, 2017)
2.3†	Agreement and Plan of Merger, dated July 19, 2018, by and among PTC Therapeutics, Inc., Agility Merger Sub, Inc., Agilis Biotherapeutics, Inc. and, solely in its capacity as equityholder representative, Shareholder Representative Services LLC (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on July 19, 2018)
2.4*	Asset Purchase Agreement by and between PTC Therapeutics, Inc. and BioElectron Technology Corporation, dated October 1, 2019 (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on October 30, 2019)
2.5*	Agreement and Plan of Merger, dated May 5, 2020, by and among PTC Therapeutics, Inc., Hydro Merger Sub, Inc., Censa Pharmaceuticals Inc. and, solely in its capacity as securityholder representative, Shareholder Representative Services LLC (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on May 6, 2020)

Exhibit Number	Description of Exhibit
3.1	Restated Certificate of Incorporation of the Registrant, as amended (incorporated by reference to Exhibit 3.1 to the Quarterly Report on Form 10-Q filed by the Registrant on July 29, 2021)
3.2	Amended and Restated Bylaws of the Registrant, effective December 5, 2022 (incorporated by reference to Exhibit 3.1 to the Current Report on Form 8-K filed by the Registrant on December 6, 2022)
4.1	Description of Registered Securities (incorporated by reference to Exhibit 4.1 to the Annual Report on Form 10-K filed by the Registrant on February 22, 2022)
4.2	Specimen Stock Certificate evidencing the shares of common stock (incorporated by reference to Exhibit 4.1 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
4.3	Indenture (including Form of Notes), dated as of September 20, 2019, by and between PTC Therapeutics, Inc. and U.S. Bank National Association, a national banking association, as trustee (incorporated by reference to Exhibit 4.1 to the Current Report on Form 8-K filed by the Registrant on September 20, 2019)
10.1+	2009 Equity and Long Term Incentive Plan, as amended (incorporated by reference to Exhibit 10.4 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.2+	Form of Notice of Award for Incentive Stock Option under 2009 Equity and Long Term Incentive Plan (incorporated by reference to Exhibit 10.5 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.3+	Form of Notice of Award for Nonstatutory Stock Option under 2009 Equity and Long Term Incentive Plan (incorporated by reference to Exhibit 10.6 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.4+	Form of Restricted Stock Agreement under 2009 Equity and Long Term Incentive Plan (incorporated by reference to Exhibit 10.19 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.5+	2013 Stock Incentive Plan (incorporated by reference to Exhibit 10.7 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.6+	Form of Restricted Stock Agreement under 2013 Stock Incentive Plan (incorporated by reference to Exhibit 10.8 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.7+	Form of Nonstatutory Stock Option Agreement under 2013 Stock Incentive Plan (incorporated by reference to Exhibit 10.9 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.8+	PTC Therapeutics, Inc. Amended and Restated 2013 Long-Term Incentive Plan (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on June 9, 2022)
10.9+	Form of Incentive Stock Option Agreement under 2013 Long Term Incentive Plan—2013/2014 (incorporated by reference to Exhibit 10.11 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.10+	Form of Nonstatutory Stock Option Agreement under 2013 Long Term Incentive Plan—2013/2014 (incorporated by reference to Exhibit 10.12 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)

Exhibit Number	Description of Exhibit
10.11+	Form of Nonqualified Stock Option Agreement Inducement Grant Agreement—2014-2022 (incorporated by reference to Exhibit 10.14 to the Annual Report on Form 10-K filed by the Registrant on March 2, 2015)
10.12+	Form of Incentive Stock Option Agreement under 2013 Long Term Incentive Plan—2014-2022 (incorporated by reference to Exhibit 10.15 to the Annual Report on Form 10-K filed by the Registrant on March 2, 2015)
10.13+	Form of Nonstatutory Stock Option Agreement under 2013 Long Term Incentive Plan—2014-2022 (incorporated by reference to Exhibit 10.16 to the Annual Report on Form 10-K filed by the Registrant on March 2, 2015)
10.14+	Form of Nonstatutory Stock Option Agreement under 2013 Long Term Incentive Plan—Non-employee Director (incorporated by reference to Exhibit 10.31 to the Annual Report on Form 10-K filed by the Registrant on February 29, 2016)
10.15+	Form of Restricted Stock Unit Agreement under 2013 Long Term Incentive Plan —2016-2022 (incorporated by reference to Exhibit 10.32 to the Annual Report on Form 10-K filed by the Registrant on February 29, 2016)
10.16+	Form of Restricted Stock Agreement under 2013 Long Term Incentive Plan —2017-2022 (incorporated by reference to Exhibit 10.19 to the Annual Report on Form 10-K filed by the Registrant on March 16, 2017)
10.17+	Form of Nonqualified Restricted Stock Award Agreement Inducement Grant Agreement-2018 (incorporated by reference to Exhibit 99.3 to the Registration Statement on Form S-8 (File No. 333-229126), of the Registrant)
10.18+	Form of Incentive Stock Option Agreement under Amended and Restated 2013 Long Term Incentive Plan**
10.19+	Form of Nonstatutory Stock Option Agreement under Amended and Restated 2013 Long Term Incentive Plan**
10.20+	Form of Nonstatutory Stock Option Agreement under Amended and Restated 2013 Long Term Incentive Plan—Non-employee Director**
10.21+	Form of Restricted Stock Unit Agreement under Amended and Restated 2013 Long Term Incentive Plan**
10.22†	Funding Agreement, dated as of May 26, 2010, by and between the Registrant and The Wellcome Trust Limited (incorporated by reference to Exhibit 10.16 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.23*	Amendments to the Funding Agreement, dated as of May 26, 2010, by and between the Registrant and The Wellcome Trust Limited**
10.24+	Amended and Restated Employment Agreement between the Registrant and Stuart W. Peltz (incorporated by reference to Exhibit 10.20 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.25+	Amended and Restated Employment Agreement between the Registrant and Mark E. Boulding (incorporated by reference to Exhibit 10.22 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)

Exhibit Number	Description of Exhibit
10.26+	Amended and Restated Employment Agreement between the Registrant and Neil Almstead (incorporated by reference to Exhibit 10.24 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)
10.27†	Exclusive License and Supply Agreement, dated as of May 12, 2015, as amended, by and between Faes Farma, S.A. and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC), as assigned by Complete Pharma Holdings, LLC to the Registrant on April 20, 2017 (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on August 9, 2017)
10.28†	Commercial Manufacturing Agreement, dated as of September 18, 2015, as amended, by and between Alcami Corporation (f/k/a/ AAI Pharma Services Corp.) and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC), as assigned by Complete Pharma Holdings, LLC to the Registrant on April 20, 2017 (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by the Registrant on May 3, 2022)
10.29+	Employment Agreement, as amended, between the Registrant and Christine Utter (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by the Registrant on August 6, 2019)
10.30†	License and Technology Transfer Agreement, dated December 23, 2015, by and among National Taiwan University, Professor Wuh-Lian(Paul) Hwu and Agilis Biotherapeutics, Inc. (formerly Agilis Biotherapeutics, LLC) (incorporated by reference to Exhibit 10.3 on Form 10-Q filed by Registrant on November 5, 2018)
10.31*	License and Technology Transfer Agreement Amendment No. 2, dated December 1, 2019, by and among National Taiwan University, Professor Wu-Lian (Paul) Hwu and PTC Therapeutics GT, Inc. (incorporated by reference to Exhibit 10.42 on Form 10-K filed by Registrant on March 2, 2020)
10.32†	Collaboration and License Agreement, dated August 1, 2018, by and between PTC Therapeutics International Limited and Akcea Therapeutics, Inc. (incorporated by reference to Exhibit 10.3 on Form 10-Q filed by Registrant on November 5, 2018)
10.33	Amended and Restated 2016 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on June 9, 2021)
10.34+	Employment Agreement, as amended, between the Registrant and Emily Hill (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on August 6, 2019)
10.35*	Lease Agreement dated as of August 3, 2019, by and between Bristol-Myers Squibb Company and PTC Therapeutics, Inc. (incorporated by reference to Exhibit 10.5 to the Quarterly Report on Form 10-Q filed by the Registrant on October 30, 2019)
10.36	Irrevocable Standby Letter of Credit, dated September 3, 2019, issued by HSBC Bank USA, N.A. in favor of Bristol-Myers Squibb Company for the Account of PTC Therapeutics, Inc., as amended (incorporated by reference to Exhibit 10.6 to the Quarterly Report on Form 10-Q filed by the Registrant on October 30, 2019)
10.37+	2020 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 99.3 to the Registration Statement on Form S-8 (File No. 333-235823), of the Registrant)
10.38+	Form of Inducement Option Agreement under the 2020 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 99.4 to the Registration Statement on Form S-8 (File No. 333-235823), of the Registrant)

Exhibit Number	Description of Exhibit
10.39+	Form of Inducement Restricted Stock Agreement under the 2020 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 99.5 to the Registration Statement on Form S-8 (File No. 333-235823), of the Registrant)
10.40+	Amendment No. 1 to 2020 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 99.3 to the Registration Statement on Form S-8 (File No. 333-251878) of the Registrant)
10.41+	Amendment No. 2 to 2020 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 99.1 to Post-Effective Amendment No. 1 to the Registration Statement on Form S-8 (File No. 333-251878) of the Registrant)
10.42+	Amendment No. 3 to 2020 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 99.4 to the Registration Statement on Form S-8 (File No. 333-268851), of the Registrant)
10.43*	First Amendment to Lease Agreement dated as of October 7, 2019 by and between Bristol-Myers Squibb Company and PTC Therapeutics, Inc. (incorporated by reference to Exhibit 10.51 to the Annual Report on Form 10-K filed by the Registrant on March 2, 2020)
10.44*	Second Amendment to Lease Agreement dated as of March 25, 2020 by and between Bristol-Myers Squibb Company and PTC Therapeutics, Inc. (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on April 30, 2020)
10.45*	License Agreement dated as of February 8, 2016, as amended, by and between Shiratori Pharmaceutical Co. Ltd. and Censa Pharmaceuticals Inc. (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on August 5, 2020)
10.46*	Royalty Purchase Agreement, dated as of July 17, 2020, by and among PTC Therapeutics, Inc., RPI 2019 Intermediate Finance Trust, and, solely for the limited purposes set forth therein, Royalty Pharma PLC (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by the Registrant on August 5, 2020)
10.47+	Employment Agreement, as amended, between the Registrant and Matthew Klein (incorporated by reference to Exhibit 10.44 to the Annual Report on Form 10-K filed by the Registrant on February 22, 2022)
10.48+	Employment Agreement, as amended, between the Registrant and Eric Pauwels (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed by the Registrant on August 5, 2020)
10.49*	Rights Exchange Agreement, by and among PTC Therapeutics, Inc., the Rightholders set forth therein, and, for the limited purposes set forth therein, Shareholder Representatives Services LLC, dated as of April 29, 2020 (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on April 30, 2020)
10.50	At the Market Offering Sales Agreement, dated August 7, 2019, among PTC Therapeutics, Inc., Cantor Fitzgerald & Co. and RBC Capital Markets, LLC (incorporated by reference to Exhibit 1.1 to the Current Report on Form 8-K filed by the Registrant on August 7, 2019)
10.51*	Lease Agreement dated May 24, 2022, between Warren CC Acquisitions, LLC and PTC Therapeutics, Inc. (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by Registrant on August 4, 2022)

Exhibit Number	Description of Exhibit
10.52	Irrevocable Transferable Standby Letter of Credit, dated June 22, 2022, issued by HSBC Bank USA, N.A. in favor of Warren CC Acquisitions LLC c/o Vision Real Estate Partners for the Account of PTC Therapeutics, Inc. (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by Registrant on August 4, 2022)
10.53*	Letter Agreement re: Collaboration and License Agreement, dated July 25, 2022, by and between Akcea Therapeutics, Inc. and PTC Therapeutics International Limited (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by Registrant on October 27, 2022)
10.54*	Letter Agreement re: Collaboration and License Agreement, dated September 14, 2022, by and between Akcea Therapeutics, Inc. and PTC Therapeutics International Limited (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by Registrant on October 27, 2022)
10.55*	Credit Agreement dated as of October 27, 2022, among PTC Therapeutics, Inc., as the Borrower, each subsidiary of the Borrower from time to time party thereto, as Guarantors, the Lenders from time to time party thereto and Wilmington Trust, National Association, as Administrative Agent**
10.56	Stock Purchase Agreement, dated as of October 27, 2022 by and among the investors listed therein and PTC Therapeutics, Inc.**
21.1	Subsidiaries of the Registrant**
23.1	Consent of Independent Registered Public Accounting Firm**
24.1	Power of attorney (included on the signature page to this Form 10-K)
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002**
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002**
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**
32.2	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002**
101.INS	Inline XBRL Instance Document**
101.SCH	Inline XBRL Taxonomy Extension Schema Document**
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document**
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Database**
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document**
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document**
104	Cover Page Interactive Data File (formatted Inline XBRL and contained in Exhibit 101)

†† Confidential treatment has been granted as to certain portions, which portions have been omitted and separately filed with the Securities and Exchange Commission.

† Confidential treatment has been granted for certain portions that are omitted from this exhibit. The omitted information has been filed separately with the U.S. Securities and Exchange Commission (the "SEC") pursuant to the registrant's application for confidential treatment. In addition, schedules have been omitted from this exhibit pursuant to Item 601(b)(2) of Regulation S-K. A copy of any omitted schedule will be furnished supplementally to the SEC upon request; provided, however, that the registrant may request confidential treatment for any document so furnished.

+ Management contract, compensatory plan or arrangement.

* Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

** Submitted electronically herewith.

Stockholders may obtain (without charge) a copy of this Annual Report on Form 10-K (including the financial statements and financial statement schedules) and a copy of any exhibit thereto (upon payment of a fee limited to our reasonable expenses in furnishing such exhibit) by writing to PTC Therapeutics, Inc., 100 Corporate Court, South Plainfield, New Jersey 07080.

Item 16. Form 10-K Summary

None.

SIGNATURES

Pursuant to the requirements to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

PTC THERAPEUTICS, INC.

Date: February 21, 2023

By: /s/ STUART W. PELTZ
Stuart W. Peltz, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

POWER OF ATTORNEY

We, the undersigned officers and directors of PTC Therapeutics, Inc., hereby severally constitute and appoint Stuart W. Peltz and Mark E. Boulding, and each of them singly (with full power to each of them to act alone), our true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution in each of them for him and in his name, place and stead, and in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite or necessary to be done in and about the premises, as full to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents or any of them, or their or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Dated: February 21, 2023

By: /s/ STUART W. PELTZ
Stuart W. Peltz
Chief Executive Officer and Director

Dated: February 21, 2023

By: /s/ EMILY HILL
Emily Hill
Chief Financial Officer
(Principal Financial Officer)

Dated: February 21, 2023

By: /s/ CHRISTINE UTTER
Christine Utter
Chief Accounting Officer
(Principal Accounting Officer)

Dated: February 21, 2023

By: /s/ MICHAEL SCHMERTZLER
Michael Schmertzler
Director

Dated: February 21, 2023

By: /s/ ALLAN JACOBSON
Allan Jacobson
Director

Dated: February 21, 2023

By: /s/ STEPHANIE S. OKEY
Stephanie S. Okey
Director

Dated: February 21, 2023	By:	/s/ EMMA REEVE Emma Reeve *Director*
Dated: February 21, 2023	By:	/s/ MARY SMITH Mary Smith *Director*
Dated: February 21, 2023	By:	/s/ DAVID P. SOUTHWELL David P. Southwell *Director*
Dated: February 21, 2023	By:	/s/ GLENN D. STEELE Glenn D. Steele *Director*
Dated: February 21, 2023	By:	/s/ ALETHIA YOUNG Alethia Young *Director*
Dated: February 21, 2023	By:	/s/ JEROME B. ZELDIS Jerome B. Zeldis *Director*

PTC Therapeutics, Inc.

Incentive Stock Option Agreement
Granted Under Amended and Restated 2013 Long-Term Incentive Plan

1. Grant of Option.

This agreement evidences the grant by PTC Therapeutics, Inc., a Delaware corporation (the "Company"), on [Grant Date] (the "Grant Date") to [Participant Name], an employee of the Company (the "Participant"), of an option to purchase, in whole or in part, on the terms provided herein and in the Company's Amended and Restated 2013 Long-Term Incentive Plan (the "Plan"), a total of [Number of Awards Granted] shares (the "Shares") of common stock, $0.001 par value per share, of the Company ("Common Stock") at $[Grant Price] per Share. Unless earlier terminated, this option shall expire at 5:00 p.m., Eastern time, on [Expiration Date] (the "Final Exercise Date").

It is intended that the option evidenced by this agreement shall be an incentive stock option as defined in Section 422 of the Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the "Code") to the maximum extent permitted by applicable federal tax laws. Except as otherwise indicated by the context, the term "Participant", as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

2. Vesting Schedule.

This option will become exercisable ("vest") in accordance with the vesting schedule set forth in the Appendix hereto.

Please refer to Appendix: Vesting Schedule on the last page of this agreement for details.

To the extent that the Participant's stock option grant has been split and memorialized in two agreements, one agreement for incentive stock options (the "ISO Agreement") and one agreement for nonstatutory stock options (the "NSO Agreement"), then the vesting schedules identified in Section 2 of the ISO Agreement and in Section 2 of the NSO Agreement should be read as one combined vesting schedule, with options from the NSO Agreement only vesting if options under the ISO Agreement have all vested or have vested in a given year up to the maximum allowable under the Code.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Shares for which it is vested until the earlier of the Final Exercise Date or the termination of this option under Section 3 hereof or the Plan.

3 Exercise of Option.

(a) Form of Exercise. Each election to exercise this option shall be in writing, signed by the Participant, and received by the Company at its principal office, accompanied by this

agreement, and payment in full in the manner provided in the Plan. The Participant may purchase less than the number of shares covered hereby, provided that no partial exercise of this option may be for any fractional share or for fewer than ten whole shares.

(b) Continuous Relationship with the Company Required. Except as otherwise provided in this Section 3, this option may not be exercised unless the Participant, at the time he or she exercises this option, is, and has been at all times since the Grant Date, an employee, officer or director of, or consultant or advisor to, the Company or any parent or subsidiary of the Company as defined in Section 424(e) or (f) of the Code (an "Eligible Participant").

(c) Termination of Relationship with the Company. If the Participant ceases to be an Eligible Participant for any reason, then, except as provided in paragraphs (d), (e) and (f) below, the right to exercise this option shall terminate three months after such cessation (but in no event after the Final Exercise Date), provided that this option shall be exercisable only to the extent that the Participant was entitled to exercise this option on the date of such cessation. Notwithstanding the foregoing, if the Participant, prior to the Final Exercise Date, violates the non-competition or confidentiality provisions of any employment contract, confidentiality and nondisclosure agreement or other agreement between the Participant and the Company, the right to exercise this option shall terminate immediately upon written notice to the Participant from the Company describing such violation.

(d) Exercise Period Upon Death or Disability. If the Participant dies or becomes disabled (within the meaning of Section 22(e)(3) of the Code) prior to the Final Exercise Date while he or she is an Eligible Participant and the Company has not terminated such relationship for "cause" as specified in paragraph (e) below, this option shall be exercisable, within the period of one year following the date of death or disability of the Participant, by the Participant (or in the case of death by an authorized transferee), provided that this option shall be exercisable only to the extent that this option was exercisable by the Participant on the date of his or her death or disability, and further provided that this option shall not be exercisable after the Final Exercise Date.

(e) Termination for Cause. If, prior to the Final Exercise Date, the Participant's employment is terminated by the Company for Cause (as defined below), the right to exercise this option shall terminate immediately upon the effective date of such termination of employment. If, prior to the Final Exercise Date, the Participant is given notice by the Company of the termination of his or her employment by the Company for Cause, and the effective date of such employment termination is subsequent to the date of delivery of such notice, the right to exercise this option shall be suspended from the time of the delivery of such notice until the earlier of (i) such time as it is determined or otherwise agreed that the Participant's employment shall not be terminated for Cause as provided in such notice or (ii) the effective date of such termination of employment (in which case the right to exercise this option shall, pursuant to the preceding sentence, terminate upon the effective date of such termination of employment). If the Participant is party to an employment or severance agreement with the Company that contains a definition of "cause" for termination of employment, "Cause" shall have the meaning ascribed to such term in such agreement. Otherwise, "Cause" shall mean willful misconduct by the

Participant or willful failure by the Participant to perform his or her responsibilities to the Company (including, without limitation, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or other similar agreement between the Participant and the Company), as determined by the Company, which determination shall be conclusive. The Participant's employment shall be considered to have been terminated for Cause if the Company determines, within 30 days after the Participant's resignation, that termination for Cause was warranted.

(f) Accelerated Vesting upon Termination in connection with Corporate Change.

(1) In the event that the Participant's employment is terminated by the Company without Cause or by the Participant for Good Reason (as defined in Section 3(h) of this Agreement), the unvested portion of the option award granted hereunder will not be subject to any accelerated vesting except as otherwise provided for in this Section 3(f) or as otherwise set forth in an employment or other agreement between the Participant and the Company.

(2) In the event that the Participant's employment is terminated by the Company without Cause or by the Participant for Good Reason within the period of three (3) months prior to (but only if negotiations relating to the particular Corporate Change, as defined in Section 3(g) of this Agreement, that occurs are ongoing at the date of the notice of termination) or twelve (12) months after a Corporate Change that occurs while the Participant is employed by the Company (such fifteen-month period, the "Protected Period"), one hundred percent (100%) of the unvested portion of the option award granted hereunder shall vest immediately.

(g) Definition of "Corporate Change". For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company); (iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the U.S. Securities Exchange Act of 1934, as amended (excluding, for this purpose, the Company or any Subsidiary, or any employee benefit plan of the Company or any Subsidiary, or any "group" in which all or substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of an initial public offering of the Company's common stock, or as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) the Participant forms part of an executive management team that consists of

substantially the same group of individuals and the Participant is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to Section 409A, the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

(h) Definition of "Good Reason". If the Participant is party to an employment, consulting or severance agreement with the Company that contains a definition of "good reason" for termination of employment or other relationship, "Good Reason" shall have the meaning ascribed to such term in such agreement. Otherwise, "Good Reason" shall mean any of the following, unless (i) the basis for such Good Reason is cured within a reasonable period of time (determined in the light of the cure appropriate to the basis of such Good Reason, but in no event less than thirty (30) nor more than ninety (90) days) after the Company receives written notice (which must be received from the Participant within ninety (90) days of the initial existence of the condition giving rise to such Good Reason) specifying the basis for such Good Reason or (ii) Participant has consented to the condition that would otherwise be a basis for Good Reason:

(1) An change in the principal location at which the Participant provides services to the Company to a location more than fifty (50) miles from the location at which the Participant provided services as of immediately prior to the Corporate Change and/or to a location in New York City (either of), which change the Company has reasonably determined as of the date hereof, would constitute a material change in the geographic location at which the Participant provides services to the Company);

(2) A material adverse change by the Company in the Participant's duties, authority or responsibilities which causes the Participant's position with the Company to become of materially less responsibility or authority than the Participant's position immediately prior to the Corporate Change. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change the Participant's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business;

(3) A material reduction in the Participant's base compensation (including his or her base salary);

(4) A material breach of this Agreement by the Company which has not been cured within thirty (30) days after written notice thereof by the Participant; or

(5) Failure to obtain the assumption (assignment) of this Agreement by any successor to the Company.

4. Agreement in Connection with Public Offering.

The Participant agrees, in connection with any underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (i) not to (a) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any other securities of the Company or (b) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of shares of Common Stock or other securities of the Company, whether any transaction described in clause (a) or (b) is to be settled by delivery of securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days after the date of the final prospectus relating to the offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address Rule 2711(f) of the National Association of Securities Dealers, Inc. or any similar successor provision), and (ii) to execute any agreement reflecting clause (i) above as may be requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of any applicable "lock-up" period(s).

5. Tax Matters.

(a) Withholding. No Shares will be issued pursuant to the exercise of this option unless and until the Participant pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of this option.

(b) Disqualifying Disposition. If the Participant disposes of Shares acquired upon exercise of this option within two years from the Grant Date or one year after such Shares were acquired pursuant to exercise of this option, the Participant shall notify the Company in writing of such disposition.

6. Transfer Restrictions.

(a) This option may not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) The Participant agrees that he or she will not transfer any Shares issued pursuant to the exercise of this option unless the transferee, as a condition to such transfer, delivers to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of Section 4; provided that such a written confirmation shall not be required with respect to Section 4 upon the completion of any applicable "lock-up" period(s) in connection with any underwritten public offering of Common Stock.

(c) Provisions of the Plan.

This option is subject to the provisions of the Plan (including the provisions relating to amendments to the Plan), a copy of which is furnished to the Participant with this option.

The Company has caused this option to be executed by its duly authorized officer.

PTC THERAPEUTICS, INC.

By: _____
Name: _____
Title: _____

PARTICIPANT'S ACCEPTANCE

The undersigned hereby accepts the foregoing option and agrees to the terms and conditions thereof. The undersigned hereby acknowledges receipt of a copy of the Company's Amended and Restated 2013 Long-Term Incentive Plan.

PARTICIPANT

_____
Acceptance Date: _____

PTC Therapeutics, Inc.
Nonstatutory Stock Option Agreement
Granted Under Amended and Restated 2013 Long-Term Incentive Plan

1. Grant of Option.

This agreement evidences the grant by PTC Therapeutics, Inc., a Delaware corporation (the "Company"), on [Grant Date] (the "Grant Date") to [Participant Name], an employee, consultant or advisor of the Company (the "Participant"), of an option to purchase, in whole or in part, on the terms provided herein and in the Company's Amended and Restated 2013 Long-Term Incentive Plan (the "Plan"), a total of [Number of Awards Granted] shares (the "Shares") of common stock, $0.001 par value per share, of the Company ("Common Stock") at $[Grant Price] per Share. Unless earlier terminated, this option shall expire at 5:00 p.m., Eastern time, on [Expiration Date] (the "Final Exercise Date").

It is intended that the option evidenced by this agreement shall not be an incentive stock option as defined in Section 422 of the Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the "Code"). Except as otherwise indicated by the context, the term "Participant", as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

2. Vesting Schedule.

This option will become exercisable ("vest") in accordance with the vesting schedule set forth in the Appendix hereto.

Please refer to Appendix: Vesting Schedule on the last page of this agreement for details.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Shares for which it is vested until the earlier of the Final Exercise Date or the termination of this option under Section 3 hereof or the Plan.

3 Exercise of Option.

(a) Form of Exercise. Each election to exercise this option shall be in writing, signed by the Participant, and received by the Company at its principal office, accompanied by this agreement, and payment in full in the manner provided in the Plan. The Participant may purchase less than the number of shares covered hereby, provided that no partial exercise of this option may be for any fractional share or for fewer than ten whole shares.

(b) Continuous Relationship with the Company Required. Except as otherwise provided in this Section 3, this option may not be exercised unless the Participant, at the time he or she exercises this option, is, and has been at all times since the Grant Date, an employee, officer or a director of, or consultant or advisor to, the Company or any other entity the employees, officers, directors, consultants, or advisors of which are eligible to receive option grants under the Plan (an "Eligible Participant").

(c) Termination of Relationship with the Company. If the Participant ceases to be an Eligible Participant for any reason, then, except as provided in paragraphs (d), (e) and (f) below, the right to exercise this option shall terminate three months after such cessation (but in no event after the Final Exercise Date), provided that this option shall be exercisable only to the extent that the Participant was entitled to exercise this option on the date of such cessation. Notwithstanding the foregoing, if the Participant, prior to the Final Exercise Date, violates the non-competition or confidentiality provisions of any employment contract, confidentiality and nondisclosure agreement or other agreement between the Participant and the Company, the right to exercise this option shall terminate immediately upon written notice to the Participant from the Company describing such violation.

(d) Exercise Period Upon Death or Disability. If the Participant dies or becomes disabled (within the meaning of Section 22(e)(3) of the Code) prior to the Final Exercise Date while he or she is an Eligible Participant and the Company has not terminated such relationship for "cause" as specified in paragraph (e) below, this option shall be exercisable, within the period of one year following the date of death or disability of the Participant, by the Participant (or in the case of death by an authorized transferee), provided that this option shall be exercisable only to the extent that this option was exercisable by the Participant on the date of his or her death or disability, and further provided that this option shall not be exercisable after the Final Exercise Date.

(e) Termination for Cause. If, prior to the Final Exercise Date, the Participant's employment or other relationship with the Company is terminated by the Company for Cause (as defined below), the right to exercise this option shall terminate immediately upon the effective date of such termination of employment or other relationship. If, prior to the Final Exercise Date, the Participant is given notice by the Company of the termination of his or her employment or other relationship by the Company for Cause, and the effective date of such employment or other termination is subsequent to the date of the delivery of such notice, the right to exercise this option shall be suspended from the time of the delivery of such notice until the earlier of (i) such time as it is determined or otherwise agreed that the Participant's employment or other relationship shall not be terminated for Cause as provided in such notice or (ii) the effective date of such termination of employment or other relationship (in which case the right to exercise this option shall, pursuant to the preceding sentence, terminate immediately upon the effective date of such termination of employment or other relationship). If the Participant is party to an employment, consulting or severance agreement with the Company that contains a definition of "cause" for termination of employment or other relationship, "Cause" shall have the meaning ascribed to such term in such agreement. Otherwise, "Cause" shall mean willful misconduct by the Participant or willful failure by the Participant to perform his or her responsibilities to the Company (including, without limitation, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or other similar agreement between the Participant and the Company), as determined by the Company, which determination shall be conclusive. The Participant's employment or other relationship shall be considered to have been terminated for Cause if the Company determines, within 30 days after the Participant's resignation, that termination for Cause was warranted.

(f) Accelerated Vesting upon Termination in connection with Corporate Change.

(1) In the event that the Participant's employment is terminated by the Company without Cause or by the Participant for Good Reason (as defined in Section 3(h) of this Agreement), the unvested portion of the option award granted hereunder will not be subject to any accelerated vesting except as otherwise provided for in this Section 3(f) or as otherwise set forth in an employment or other agreement between the Participant and the Company.

(2) In the event that the Participant's employment is terminated by the Company without Cause or by the Participant for Good Reason within the period of three (3) months prior to (but only if negotiations relating to the particular Corporate Change, as defined in Section 3(g) of this Agreement, that occurs are ongoing at the date of the notice of termination) or twelve (12) months after a Corporate Change that occurs while the Participant is employed by the Company (such fifteen-month period, the "Protected Period"), one hundred percent (100%) of the unvested portion of the option award granted hereunder shall vest immediately.

(g) Definition of "Corporate Change". For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company); (iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the U.S. Securities Exchange Act of 1934, as amended (excluding, for this purpose, the Company or any Subsidiary, or any employee benefit plan of the Company or any Subsidiary, or any "group" in which all or substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of an initial public offering of the Company's common stock, or as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) the Participant forms part of an executive management team that consists of substantially the same group of individuals and the Participant is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to Section 409A, the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

(h) Definition of "Good Reason". If the Participant is party to an employment, consulting or severance agreement with the Company that contains a definition of "good reason" for termination of employment or other relationship, "Good Reason" shall have the meaning ascribed to such term in such agreement. Otherwise, "Good Reason" shall mean any of the

following, unless (i) the basis for such Good Reason is cured within a reasonable period of time (determined in the light of the cure appropriate to the basis of such Good Reason, but in no event less than thirty (30) nor more than ninety (90) days) after the Company receives written notice (which must be received from the Participant within ninety (90) days of the initial existence of the condition giving rise to such Good Reason) specifying the basis for such Good Reason or (ii) Participant has consented to the condition that would otherwise be a basis for Good Reason:

(1) A change in the principal location at which the Participant provides services to the Company to a location more than fifty (50) miles from the location at which the Participant provided services as of immediately prior to the Corporate Change and/or to a location in New York City (either of), which change the Company has reasonably determined as of the date hereof, would constitute a material change in the geographic location at which the Participant provides services to the Company);

(2) A material adverse change by the Company in the Participant's duties, authority or responsibilities which causes the Participant's position with the Company to become of materially less responsibility or authority than the Participant's position immediately prior to the Corporate Change. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change the Participant's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business;

(3) A material reduction in the Participant's base compensation (including his or her base salary);

(4) A material breach of this Agreement by the Company which has not been cured within thirty (30) days after written notice thereof by the Participant; or

(5) Failure to obtain the assumption (assignment) of this Agreement by any successor to the Company.

4. Agreement in Connection with Public Offering.

The Participant agrees, in connection with any underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (i) not to (a) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any other securities of the Company or (b) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of shares of Common Stock or other securities of the Company, whether any transaction described in clause (a) or (b) is to be settled by delivery of securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days after the date of the final prospectus relating to the offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address Rule 2711(f) of the National Association of Securities Dealers, Inc. or any

similar successor provision), and (ii) to execute any agreement reflecting clause (i) above as may be requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of any applicable "lock-up" period(s).

5. Tax Matters.

(a) Withholding. No Shares will be issued pursuant to the exercise of this option unless and until the Participant pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of this option.

6. Transfer Restrictions.

(a) This option may not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) The Participant agrees that he or she will not transfer any Shares issued pursuant to the exercise of this option unless the transferee, as a condition to such transfer, delivers to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of Section 4; provided that such a written confirmation shall not be required with respect to Section 4 upon the completion of any applicable "lock-up" period(s) in connection with any underwritten public offering of Common Stock.

(c) Provisions of the Plan.

This option is subject to the provisions of the Plan (including the provisions relating to amendments to the Plan), a copy of which is furnished to the Participant with this option.

The Company has caused this option to be executed by its duly authorized officer.

PTC THERAPEUTICS, INC.

By: _____
Name: _____
Title: _____

PARTICIPANT'S ACCEPTANCE

The undersigned hereby accepts the foregoing option and agrees to the terms and conditions thereof. The undersigned hereby acknowledges receipt of a copy of the Company's Amended and Restated 2013 Long-Term Incentive Plan.

PARTICIPANT

_____

Acceptance Date: _____

PTC Therapeutics, Inc.
Nonstatutory Stock Option Agreement
Granted Under Amended and Restated
2013 Long-Term Incentive Plan

1. Grant of Option.

This Agreement evidences the grant by PTC Therapeutics, Inc., a Delaware corporation (the "Company"), on [Grant Date] (the "Grant Date") to [Participant Name] a director of the Company (the "Participant"), of an option to purchase, in whole or in part, on the terms provided herein and in the Company's Amended and Restated 2013 Long-Term Incentive Plan (the "Plan"), a total of [Number of Awards Granted] shares (the "Shares") of common stock, $0.001 par value per share, of the Company ("Common Stock") at [Grant Price] per Share. Unless earlier terminated, this option shall expire at 5:00 p.m., Eastern time, on [Expiration Date](the "Final Exercise Date").

It is intended that the option evidenced by this Agreement shall not be an incentive stock option as defined in Section 422 of the Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the "Code"). Except as otherwise indicated by the context, the term "Participant", as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms. The Participant is participating in the Plan voluntarily.

2. Vesting Schedule.

This option will become exercisable ("vest") in accordance with the vesting schedule set forth in the Appendix hereto.

Please refer to Appendix: Vesting Schedule on the last page of this agreement for details.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Shares for which it is vested until the earlier of the Final Exercise Date or the termination of this option under Section 3 hereof or the Plan.

3 Exercise of Option.

(a) Form of Exercise. Each election to exercise this option shall be in writing, signed by the Participant, and received by the Company at its principal office, accompanied by this Agreement, and payment of the exercise price in full in the manner provided under Sections 5(f)(1), (2) or (6) of the Plan plus required Tax Withholding (as defined in Section 5 of this Agreement). The Participant may purchase less than the number of shares covered hereby, provided that no partial exercise of this option may be for any fractional share or for fewer than ten whole shares.

(b) Continuous Relationship with the Company Required. Except as otherwise

provided in this Section 3, this option may not be exercised unless the Participant, at the time heor she exercises this option, is, and has been at all times since the Grant Date, an employee, officer or a director of, or consultant or advisor to, the Company or any other entity the employees, officers, directors, consultants, or advisors of which are eligible to receive option grants under the Plan (an "Eligible Participant").

(c) Termination of Relationship with the Company. If the Participant ceases to be an Eligible Participant for any reason, then, except as provided in Section 3(e) of this Agreement, the Participant's right to exercise this option shall terminate three years after such cessation (butin no event after the Final Exercise Date), provided that this option shall be exercisable only to the extent that the Participant was entitled to exercise this option on the date of such cessation. Notwithstanding the foregoing or any other provision of this Agreement, if the Participant, priorto the Final Exercise Date, violates the non-competition or confidentiality provisions of any employment contract, confidentiality and nondisclosure agreement or other agreement between the Participant and the Company, the right to exercise this option shall terminate immediately upon written notice to the Participant from the Company describing such violation.

(d) Accelerated Vesting and Exercise Period Upon Death or Disability. If the Participant dies or becomes disabled (within the meaning of Section 22(e)(3) of the Code) prior to the Final Exercise Date while he or she is an Eligible Participant and the Company has not terminated such relationship for "Cause" as specified in Section 3(e) of this Agreement, one hundred percent (100%) of the unvested portion of the option award granted hereunder shall vest immediately on the date of death or disability of the Participant and this option shall be exercisable, within the period of three years following the date of the death or disability of the Participant, by the Participant (or in the case of death by an authorized transferee), provided that this option shall not be exercisable after the Final Exercise Date.

(e) Termination for Cause. If, prior to the Final Exercise Date, the Participant's employment or other relationship with the Company is terminated by the Company for Cause (as defined below), the right to exercise this option shall terminate immediately upon the effective date of such termination of employment or other relationship. If, prior to the Final Exercise Date, the Participant is given notice by the Company of the termination of his or her employmentor other relationship by the Company for Cause, and the effective date of such employment or other termination is subsequent to the date of the delivery of such notice, the right to exercise thisoption shall be suspended from the time of the delivery of such notice until the earlier of (i) such time as it is determined or otherwise agreed that the Participant's employment or other relationship shall not be terminated for Cause as provided in such notice or (ii) the effective date of such termination of employment or other relationship (in which case the right to exercise this option shall, pursuant to the preceding sentence, terminate immediately upon the effective date of such termination of employment or other relationship). If the Participant is party to an employment, consulting or severance agreement with the Company that contains a definition of "cause" for termination of employment or other relationship, "Cause" shall have the meaning ascribed to such term in such agreement. Otherwise, "Cause" shall mean willful misconduct by the Participant or willful failure by the Participant to perform his or her responsibilities to the Company (including, without limitation, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or other similar agreement between the Participant and the Company), as determined by the Company, which determination

shall be conclusive. The Participant's employment or other relationship shall be considered to have been terminated for Cause if the Company determines, within 30 days after the Participant's resignation, that termination for Cause was warranted.

(f) Accelerated Vesting and Exercise Period Upon a Corporate Change. Upon a Corporate Change, as defined in Section 3(g) of this Agreement, one hundred percent (100%) of the unvested portion of the option award granted hereunder shall vest immediately and this option shall be exercisable, within the period of three years following the date of the Corporate Change, by the Participant, provided that this option shall not be exercisable after the Final Exercise Date.

(g) Definition of "Corporate Change". For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company); (iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the U.S. Securities Exchange Act of 1934, as amended (excluding, for this purpose, the Company or any Subsidiary,or any employee benefit plan of the Company or any Subsidiary, or any "group" in which all or substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of an initial public offering of the Company's common stock, or as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) the Participant forms part of an executive management team that consists of substantially the same group of individuals and the Participant is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to Section 409A, the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

4. Agreement in Connection with Public Offering.

The Participant agrees, in connection with any underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (i) not to (a) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any other securities of the Company or (b) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of shares of Common Stock or

other securities of the Company, whether any transaction described in clause (a) or (b) is to be settled by delivery of securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days after the date of the final prospectus relating to the offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address Rule 2711(f) of the National Association of Securities Dealers, Inc. or any similar successor provision), and (ii) to execute any agreement reflecting clause (i) above as maybe requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of any applicable "lock-up" period(s).

5. Tax Matters.

(a) The Company will determine any taxes and other required source deductions ("Tax Withholding") which it is required by law or regulation of any governmental authority whatsoever to remit in connection with this Agreement, the exercise or surrender of this option, or any issuance of Shares of Common Stock. If there is no cash payment being made by the Company to the Participant from which such Tax Withholding can be withheld or deducted, the Company may take all reasonable steps as it considers necessary and appropriate to collect the amount of any such Tax Withholding from the Participant. The delivery of Shares of Common Stock to Participant may be made conditional upon the Participant (or other person) reimbursingor compensating the Company or making arrangements satisfactory to the Company for the payment in a timely manner of all such Tax Withholdings required to be remitted by the Company for the account of the Participant. Without limiting the foregoing, the Company maydeduct and withhold those amounts the Company is required to remit from any fees, salary or wages or other amounts payable to the Participant, whether or not such amounts are related to this option or this Agreement. If the Company elects not to or cannot withhold from other compensation, the Company will notify the Participant of the amount of Tax Withholdings that the Participant must pay to the Company, or arrange to have a broker tender to the Company on the Participant's behalf. On the exercise of all or a portion of this option, a payment by the Participant to the Company of Tax Withholding pursuant to this Section 5(a) is due at the same time as payment of the exercise price, unless the Company determines otherwise.

6. Transfer Restrictions.

(a) This option may not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) The Participant agrees that he or she will not transfer any Shares issued pursuant to the exercise of this option unless the transferee, as a condition to such transfer, delivers to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of Section 4; provided that such a written confirmation shall not be required with respect to Section 4 upon the completion of any applicable "lock-up" period(s) in connection

with any underwritten public offering of Common Stock.

(c) Provisions of the Plan.

This option is subject to the provisions of the Plan (including the provisions relating to amendments to the Plan), a copy of which is furnished to the Participant with this option.

The Company has caused this option to be executed by its duly authorized officer.

PTC THERAPEUTICS, INC.

By: _____

Name:

_____

Title: _____

PARTICIPANT'S ACCEPTANCE

The undersigned hereby accepts the foregoing option and agrees to the terms and conditions thereof. The undersigned hereby acknowledges receipt of a copy of the Company's Amended and Restated 2013 Long-Term Incentive Plan.

PARTICIPANT

Name_____

ACCEPTED _____

Exhibit 10.21

PTC THERAPEUTICS, INC.
RESTRICTED STOCK UNIT AGREEMENT

PTC Therapeutics, Inc. (the "Company") hereby grants the following restricted stock units pursuant to its Amended and Restated 2013 Long-Term Incentive Plan. The terms and conditions attached hereto are also a part hereof.

Notice of Grant

Name of recipient (the "Participant"):	[Participant Name]
Grant Date:	[Grant Date]
Number of Restricted Stock Units ("RSUs") granted:	[Number of Awards Granted]
Vesting Start Date:	[Vest from Hire Date]

Vesting Schedule:

Each RSU Award granted shall vest in accordance with the vesting schedule set forth in the Appendix hereto. **Please refer to Appendix: Vesting Schedule on the last page of this agreement for details.**
All vesting is dependent on the Participant continuing to perform services for the Company, as provided herein. Vesting may be accelerated in accordance with the terms of the attached terms and conditions.

This grant of RSUs satisfies in full all commitments that the Company has to the Participant with respect to the issuance of stock, stock options or other equity securities.

PTC THERAPEUTICS, INC.

[Signature of Participant]

By:
Title:

PTC THERAPEUTICS, INC.

Restricted Stock Unit Agreement
Incorporated Terms and Conditions

For valuable consideration, receipt of which is acknowledged, the parties hereto agree as follows:

1. Award of Restricted Stock Units.

In consideration of services rendered and to be rendered to the Company, by the Participant, the Company has granted to the Participant, subject to the terms and conditions set forth in this Restricted Stock Unit Agreement (this "Agreement") and in the Company's Amended and Restated 2013 Long-Term Incentive Plan (the "Plan"), an award with respect to the number of restricted shares units (the "RSUs") set forth in the Notice of Grant that forms part of this Agreement (the "Notice of Grant"). Each RSU represents the right to receive one share of common stock, $0.001 par value per share, of the Company (the "Common Stock") upon vesting of the RSU, subject to the terms and conditions set forth herein.

2. Vesting.

The RSUs shall vest in accordance with the Vesting Schedule set forth in the Notice of Grant (the "Vesting Schedule") and, if applicable, Section 4 hereof. Upon the vesting of the RSU, the Company will deliver to the Participant, for each RSU that becomes vested, one share of Common Stock, subject to the payment of any taxes pursuant to Section 8. The Common Stock will be delivered to the Participant as soon as practicable following each vesting date, but in any event within 30 days of such date.

3. Forfeiture of Unvested RSUs Upon Cessation of Service.

Subject to the provisions of Section 4 hereof, in the event that the Participant ceases to perform services to the Company for any reason or no reason, with or without Cause (as defined in Section 4(c) hereof), all of the RSUs that are unvested as of the time of such cessation shall be forfeited immediately and automatically to the Company, without the payment of any consideration to the Participant, effective as of such cessation. The Participant shall have no further rights with respect to the unvested RSUs or any Common Stock that may have been issuable with respect thereto. If the Participant provides services to a subsidiary of the Company, any references in this Agreement to provision of services to the Company shall instead be deemed to refer to service with such subsidiary.

4. Accelerated Vesting upon Cessation of Services in connection with Corporate Change.

(a) In the event that the Participant's provision of services to the Company is terminated by the Company without Cause or by the Participant for Good Reason (as defined in Section 4(d) hereof) within the period of three (3) months prior to (but only if negotiations relating to the particular Corporate Change (as defined in Section 4(b) hereof) that occurs are ongoing at the date of the notice of termination) or twelve (12) months after a Corporate Change

that occurs while the Participant is employed by the Company (such fifteen-month period, the "Protected Period"), all of the RSUs that are unvested as of the time of such cessation shall vest immediately prior to the consummation of such Corporate Change if the cessation occurs during the first three (3) months of the Protected Period or immediately upon such cessation, if the cessation occurs during the last twelve (12) months of the Protected Period.

(b) For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company); (iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the U.S. Securities Exchange Act of 1934, as amended (excluding, for this purpose, the Company or any subsidiary, or any employee benefit plan of the Company or any subsidiary, or any "group" in which all or substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) the Participant forms part of an executive management team that consists of substantially the same group of individuals and the Participant is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to of Section 409A of the Internal Revenue Code and the Treasury Regulations issued thereunder ("Section 409A"), the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

(c) If the Participant is party to an employment, consulting or severance agreement with the Company that contains a definition of "cause" for termination of employment or other relationship, "Cause" shall have the meaning ascribed to such term in such agreement. Otherwise, "Cause" shall mean willful misconduct by the Participant or willful failure by the Participant to perform his or her responsibilities to the Company (including, without limitation, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or other similar agreement between the Participant and the Company), as determined by the Company, which determination shall be conclusive. The Participant's employment or other relationship shall be considered to have been terminated for Cause if the Company determines, within 30 days after the Participant's resignation, that termination for Cause was warranted.

(d) If the Participant is party to an employment, consulting or severance agreement with the Company that contains a definition of "good reason" for termination of employment or other relationship, "Good Reason" shall have the meaning ascribed to such term in such agreement. Otherwise, "Good Reason" shall mean any of the following, unless (i) the basis for such Good Reason is cured within a reasonable period of time (determined in the light of the cure appropriate to the basis of such Good Reason, but in no event less than thirty (30) nor more than ninety (90) days) after the Company receives written notice (which must be received from the Participant within ninety (90) days of the initial existence of the condition giving rise to such Good Reason) specifying the basis for such Good Reason or (ii) Participant has consented to the condition that would otherwise be a basis for Good Reason:

(i) An change in the principal location at which the Participant provides services to the Company to a location more than fifty (50) miles from the location at which the Participant provided services as of immediately prior to the Corporate Change and/or to a location in New York City (either of), which change the Company has reasonably determined as of the date hereof, would constitute a material change in the geographic location at which the Participant provides services to the Company);

(ii) A material adverse change by the Company in the Participant's duties, authority or responsibilities which causes the Participant's position with the Company to become of materially less responsibility or authority than the Participant's position immediately prior to the Corporate Change. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change the Participant's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business;

(iii) A material reduction in the Participant's base compensation (including his or her base salary);

(iv) A material breach of this Agreement by the Company which has not been cured within thirty (30) days after written notice thereof by the Participant; or

(v) Failure to obtain the assumption (assignment) of this Agreement by any successor to the Company.

5. Restrictions on Transfer.

The Participant shall not sell, assign, transfer, pledge, hypothecate or otherwise dispose of, by operation of law or otherwise (collectively "transfer") any RSUs, or any interest therein. The Company shall not be required to treat as the owner of any RSUs or issue any Common Stock to any transferee to whom such RSUs have been transferred in violation of any of the provisions of this Agreement.

6. Rights as a Shareholder.

The Participant shall have no rights as a stockholder of the Company with respect to any shares of Common Stock that may be issuable with respect to the RSUs until the issuance of the shares of Common Stock to the Participant following the vesting of the RSUs.

7. Provisions of the Plan.

This Agreement is subject to the provisions of the Plan, a copy of which is furnished to the Participant with this Agreement.

8. Tax Matters.

(a) Acknowledgments; No Section 83(b) Election. The Participant acknowledges that he or she is responsible for obtaining the advice of the Participant's own tax advisors with respect to the award of RSUs and the Participant is relying solely on such advisors and not on any statements or representations of the Company or any of its agents with respect to the tax consequences relating to the RSUs. The Participant understands that the Participant (and not the Company) shall be responsible for the Participant's tax liability that may arise in connection with the acquisition, vesting and/or disposition of the RSUs. The Participant acknowledges that no election under Section 83(b) of the Internal Revenue Code, as amended, is available with respect to RSUs.

(b) Withholding. The Participant acknowledges and agrees that the Company has the right to deduct from payments of any kind otherwise due to the Participant any federal, state, local or other taxes of any kind required by law to be withheld with respect to the vesting of the RSUs. At such time as the Participant is not aware of any material nonpublic information about the Company or the Common Stock, the Participant shall execute the instructions set forth in Schedule A attached hereto (the "Automatic Sale Instructions") as the means of satisfying such tax obligation. If the Participant does not execute the Automatic Sale Instructions prior to an applicable vesting date, then the Participant agrees that if under applicable law the Participant will owe taxes at such vesting date on the portion of the Award then vested the Company shall be entitled to immediate payment from the Participant of the amount of any tax required to be withheld by the Company. The Company shall not deliver any shares of Common Stock to the Participant until it is satisfied that all required withholdings have been made.

9. Miscellaneous.

(a) Authority of Compensation Committee. In making any decisions or taking any actions with respect to the matters covered by this Agreement, the Compensation Committee shall have all of the authority and discretion, and shall be subject to all of the protections, provided for in the Plan. All decisions and actions by the Compensation Committee with respect to this Agreement shall be made in the Compensation Committee's discretion and shall be final and binding on the Participant.

(b) No Right to Continued Service. The Participant acknowledges and agrees that, notwithstanding the fact that the vesting of the RSUs is contingent upon his or her continued service to the Company, this Agreement does not constitute an express or implied promise of continued service relationship with the Participant or confer upon the Participant any rights with respect to a continued service relationship with the Company.

(c) Section 409A. The RSUs awarded pursuant to this Agreement are intended to be exempt from or comply with the requirements of Section 409A. The delivery of shares of Common Stock on the vesting of the RSUs may not be accelerated or deferred unless permitted or required by Section 409A.

(d) Data Privacy. The Participant hereby explicitly and unambiguously consents to the collection, use and transfer, in electronic or other form, of his or her personal data as described in this Agreement by and among, as applicable, his or her employer or contracting party and the Company for the exclusive purpose of implementing, administering and managing his or her participation in the Plan.

The Participant understands that the Company holds certain personal information about him or her, including, but not limited to, his or her name, home address and telephone number, work location and phone number, date of birth, hire date, details of all RSUs awarded, cancelled, vested, unvested or outstanding in the Participant's favor, for the purpose of implementing, administering and managing the Plan ("Personal Data"). The Participant understands that Personal Data may be transferred to any third parties assisting in the implementation, administration and management of the Plan, that these recipients may be located in the Participant's country or elsewhere, and that the recipient's country may have different data privacy laws and protections than the Participant's country. The Participant understands that he or she may request a list with the names and addresses of any potential recipients of the Personal Data by contacting his or her local human resources representative. The Participant authorizes the recipients to receive, possess, use, retain and transfer the Personal Data, in electronic or other form, for the purposes of implementing, administering and managing his or her participation in the Plan, including any requisite transfer of such Personal Data as may be required to a broker or other third party with whom the Participant may elect to deposit any Common Stock acquired upon vesting of the RSUs or in connection with the Participant's execution of the Automatic Sale Instructions and the sale of the Participant's Common Stock pursuant to Schedule A. The Participant understands that Personal Data will be held only as long as is necessary to implement, administer and manage his or her participation in the Plan. The Participant understands that he or she may, at any time, view Personal Data, request additional information about the storage and processing of Personal Data, require any necessary amendments to Personal Data or refuse or withdraw the consents herein, in any case without cost, by contacting in writing his or her local human resources representative. The Participant understands, however, that refusing or withdrawing his or her consent may affect his or her ability to participate in the Plan. For more information on the consequences of Participant's refusal to consent or withdrawal of consent, the Participant understands that he or she may contact his or her local human resources representative. For purposes of this Section 9(d), if the Participant provides services to a subsidiary of the Company, any references in this Section 9(d) to the Company shall be deemed to also refer to such subsidiary.

(e) Participant's Acknowledgements. The Participant acknowledges that he or she: (i) has read this Agreement; (ii) has been represented in the preparation, negotiation and execution of this Agreement by legal counsel of the Participant's own choice or has voluntarily declined to seek such counsel; (iii) understands the terms and consequences of this Agreement; and (iv) is fully aware of the legal and binding effect of this Agreement.

(f) Governing Law. This Agreement shall be construed, interpreted and enforced in accordance with the internal laws of the State of Delaware without regard to any applicable conflicts of laws provisions.

I hereby acknowledge that I have read this Agreement, have received and read the Plan, and understand and agree to comply with the terms and conditions of this Agreement and the Plan.

_____

PARTICIPANT ACCEPTANCE

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

Exhibit 10.23

wellcome trust

Private and Confidential

TEL: +44 (0)20 7611 8888 Direct: 8241
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E-MAIL: s.molton@wellcome.ac.uk

Dr Thomas Davis
PTC Therapeutics Inc.
100 Corporate Court
South Plainfield
NJ 07079
USA

Our Ref: 092687
08 May 2012

Dear Dr Davis

No Cost Milestone Extension to the Seeding Drug Discovery Initiative Funding Agreement (the "Extension Letter")

We refer to the Funding Agreement between (1) The Wellcome Trust Limited as trustee of the Wellcome Trust (the "Trust") and (2) PTC Therapeutics Inc. dated 26 May 2010 (the **"Agreement"**), together with the Award Letter from the Trust to the Principal Investigator dated 26 May 2010.

Unless the context otherwise requires, all capitalised terms used in this Extension Letter shall have the meanings given to them in the Agreement.

The Agreement was to enable the Trust to grant PTC Therapeutics Inc. ("PTC") up to a maximum amount of $5,397,000 (five million and three hundred thousand and ninety-seven thousand United States dollars) payable in milestone dependent instalments for conducting a research programme aimed at identifying small molecules that selectively decrease the production of Bmi-1 expression in tumour stem cells. The Parties acknowledge and agree that to this date $3,099,000 (three million and ninety-nine thousand United States dollars) has been drawn-down by PTC pursuant to the Agreement.

The Trust is now willing to grant a no cost Milestone extension to PTC for the achievement of Milestone 2 set out in the Award Letter. In consideration for the sum of one pound (£1), the receipt of which is hereby acknowledged by the Trust, the Parties agree to amend the Award Letter as follows:

The Milestone table set out in the Award Letter shall be deleted and replaced with the following:

Milestone	**Description of Milestone**	**Milestone Date**
1	Milestone 1: Months 1-6 **Identification of molecules that have in vivo efficacy and that cross the blood-brain barrier.** ● Identify compounds for which the ratios of both the trough plasma and brain tissue concentrations relative to the *in vitro* EC_{50} are greater than or equal to 2 (Cp6h / ECSO > 2; Cbrain6hr / EC_{50} > 2)	6 months after the Commencement Date

	following dosing. ● Identify compounds with good PK properties and an EC_{50}< 500 nM (ELISA of tumour cell extract) ● Demonstrate selectivity to Bmi-1 versus other targets to rule out a general cytotoxic effect. Establish a selectivity profile *in vitro* and *in vivo:* EC_{50} Bmi-1<10X EC_{50} protein X (eg. X= p27, actin, tubulin, histone 2A, EZH2). ● Demonstrate activity of compounds in animal models of tumour growth to establish proof of concept and a PK/PD relationship. Primary Goal: >50% reduction in intra-tumour Bmi-1 levels; Secondary Goal: >50% reduction in tumour growth. ● Plans for determining Mechanism of Action approved by the RSG	
2	Milestone 2 Time: Months 7 - 23 **Identify a single compound as the Development Candidate (DC).** ● Identify a compound with PK trough plasma exposure upon dosing, at 6 hours (C6h (usually a 10 mg/kg dose) that is at least > 2 times the concentration of the *in vitro* EC_{50} and > 0.5 µg/ml. ● Improve compound potency. Goal: EC50< 100 nM. ● Establish the gene expression selectivity and safety profile of the compound both in vitro and in vivo. Goals will include: establishing the specificity of the compound to the production of Bmi-1 versus other proteins (EC_{50} Bmi-1<100X EC_{50} proteinX (X= other protein such as p27, actin, tubulin, or histone 2A); establishing a >3-fold selectivity versus other UTR driven reporters; establishing the cytotoxicity profile with CC_{50}/EC_{50} Bmi-1 = >100 fold. ● Identifying compounds with: 1) <20% hERG inhibition at 5 µM; 2) no off-target effects at 10x therapeutic exposures; negative in exploratory Ames tests, acceptable in vitro off-target assay panel. ● Demonstrate anti-tumour activity as mono- and combination therapy in orthotopic models of glioblastoma. Goal: >50% reduction in intra tumour Bmi-1 levels: >50% reduction in tumour growth and demonstration of additivity or synergy with standard of care (e.g. temozolomide). ● Determine the activity and selectivity against the tumour stem cell fraction versus other tumour cell populations. Stem cell toxicity window: CC50 stem cell/EC_{50} >10-fold. ● Perform non-GLP pharmacokinetic and safety toxicology studies in rats, dogs and/or monkeys to enable the submission of an IND application. These studies will include: dose-escalating studies and 2- week safety studies to monitor safety and	23 months after the Commencement Date

	toxicology. Compound effects to be monitored by histopathology, clinical chemistry and haematology. Goal: a 10X safety window (exposure of efficacious dose vs. NOAEL, dose with no adverse effects); adequate compound exposure and safety in two species (rodent and non-rodent) to support further pre-clinical and clinical development.	
3	Milestone 3: Months 24 - 35. **Data package sufficient for an IND application (Months 24 - 35).** Complete chemical manufacture and control (CMC) and non-clinical development to support an IND application. A comprehensive program of safety pharmacology and toxicology studies will be performed. These studies will be conducted in compliance with FDA Good Laboratory Practices (21 CFR Part 58), and will include analysis of dose formulations for confirmation of content, homogeneity and stability using a validated method. The same batch of material will be used for the safety pharmacology and toxicology studies. These activities will include: ● Development of a chemical synthesis process suitable for kg-scale manufacture of DC. ● Synthesis of sufficient quantities of GMP compound for toxicology testing. ● Identification and testing of an oral formulation suitable for toxicology studies. ● Conductance of IND-enabling safety pharmacology and toxicology studies. Studies will include: 1) assessment of neurological function in rodents treated with compound (Irwin or FOB); 2) assessment of cardiovascular function in dogs treated with compound; 3) assessment of respiratory function in rodents. Goal: Identification of a compound with an adequate safety profile in rats and dogs (or monkeys) following dosing for one month.	35 months after the Commencement Date

The amendments set out above shall have effect as from the date of the last signature of this Extension Letter. Except as set out in this Extension Letter the provisions of the Agreement and the Award Letter shall remain in full force and effect. Should there be any inconsistencies between this Extension Letter, the Agreement and the Award Letter, then this Extension Letter shall prevail.

This Extension Letter (and any dispute, controversy, proceedings or claim of whatever nature arising out of this Extension Letter or its formation) shall be governed by and construed in accordance with the laws of England. The Parties irrevocably submit to the exclusive jurisdiction of the Courts of England.

Please would you arrange for two copies of this Extension Letter to be signed on behalf of PTC and return one to us for our records.

Signed for and on behalf of
THE WELLCOME TRUST LIMITED
as trustee of the Wellcome Trust:

Signature:	/s/ Dr Richard Seabrook	Signature:	/s/ Dr Bina Rawal
Name:	Dr Richard Seabrook Head of Business Development Technology Transfer	Name:	Dr Bina Rawal Head of Clinical Development Technology Transfer
Date:	8 May 2012	Date:	9 May 2012

Accepted and signed for and on behalf of
PTC THERAPEUTICS INC:

Signature:	/s/ Mark E. Boulding
Name:	Mark E. Boulding Executive VP, Chief Legal Officer
Date:	5/15/12

wellcome trust

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E-MAIL: s.molton@wellcome.ac.uk

FAO: Legal Department
PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield
NJ 07079
USA

Our Ref: 092687

24 May 2013

Dear Sirs

Second No Cost Milestone Extension to the Seeding Drug Discovery Initiative Funding Agreement (the "Second Extension Letter")

We write with reference to:

1. the funding agreement between The Wellcome Trust Limited as trustee of the Wellcome Trust (the "**Trust**") and PTC Therapeutics, Inc. ("**PTC**") dated 26 May 2010; and
2. the no cost milestone extension letter to the funding agreement dated 08 May 2012 (the "**Extension Letter**"),

together, the "**Agreement**"; and the Award Letter from the Trust to the Principal Investigator dated 26 May 2010.

The Agreement was to enable the Trust to grant PTC up to a maximum amount of US$5,397,000 (five million three hundred thousand and ninety-seven thousand United States dollars) payable in milestone dependent instalments for conducting a research programme aimed at identifying small molecules that selectively decrease the production of Bmi-1 expression in tumour stem cells.

Pursuant to Clause 2.4 of the Agreement, the Trust is now willing to grant a no cost Milestone Extension to PTC for the achievement of Milestone 3. In consideration of the sum of one pound (£1), the receipt of which is hereby acknowledged by the Trust, the Parties agree to amend the Award Letter as follows:

1. No cost milestone extension

The wording "Milestone 3: Months 24-35 Data package sufficient for an IND application (Months 24-35)" set out in the Award Letter for Milestone 3 under the heading 'Description of Milestone' shall be deleted and replaced with the wording "Milestone 3: Months 24-45 Data package sufficient for an IND application (Months 24-45)."

The wording "35 months after the Commencement Date" set *out in the Award Letter for Milestone 3 under the heading 'Milestone Date' shall be deleted and replaced with the wording "45 months after the Commencement Date."*

The amendment set out above shall have effect as from the date this Second Extension Letter is countersigned by PTC. Except as set out in this Second Extension Letter, all other provisions of the Agreement and the Award Letter shall remain in full force and effect. All capitalised terms in this Second Extension Letter shall have the same meaning as those in the Agreement and the Award Letter. This Second Extension Letter shall be governed by and interpreted in accordance with English law.

Please would you arrange for two copies of this Second Extension Letter to be signed on behalf of PTC and return one to us for our records.

Signed for and on behalf of
THE WELLCOME TRUST LIMITED
as trustee of the Wellcome Trust:

Signature:	/s/ Dr Richard Seabrook	Signature:
Name:	Dr Richard Seabrook Head of Business Development Technology Transfer	Name:
Date:	5th June 2013	Date:

Accepted and signed for and on behalf of
PTC THERAPEUTICS, INC:

Signature:	/s/ Mark E. Boulding	Signature:
Name:	Mark E. Boulding Executive Vice President Chief Legal Officer	Name:
Date:	24 May 2013	Date:

wellcome trust

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FAO: Legal Department
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100 Corporate Court
South Plainfield
NJ 07079
USA

Our Ref: 092687

3 March 2014

Dear Sirs

Third No Cost Milestone Extension to the Seeding Drug Discovery Initiative Funding Agreement (the "Third Extension Letter")

We write with reference to:

1. the funding agreement between The Wellcome Trust Limited as trustee of the Wellcome Trust (the "**Trust**") and PTC Therapeutics Inc. ("**PTC**") dated 26 May 2010;
2. the no cost milestone extension letter to the funding agreement dated 08 May 2012 (the "**Extension Letter**"); and
3. the second no cost milestone extension letter to the funding agreement dated 24 May 2013 (the "**Second Extension Letter**"),

together, the "**Agreement**"; and the Award Letter from the Trust to the Principal Investigator dated 26 May 2010.

The Agreement was to enable the Trust to grant PTC up to a maximum amount of US$5,397,000 (five million three hundred thousand and ninety-seven thousand United States dollars) payable in milestone dependent instalments for conducting a research programme aimed at identifying small molecules that selectively decrease the production of Bmi-1 expression in tumour stem cells.

Pursuant to Clause 2.4 of the Agreement, the Trust is now willing to grant a no cost Milestone Extension to PTC for the achievement of Milestone 3. In consideration of the sum of one pound (£1), the receipt of which is hereby acknowledged by the Trust, the Parties agree to amend the Award Letter as follows:

1. No cost milestone extension

The wording "Milestone 3: Months 24-45 Data package sufficient for an IND application (Months 24-45) set out in the Award Letter for Milestone 3 under the heading 'Description of Milestone' *shall be deleted and replaced with the wording "Milestone 3: Months 24-54 Data package sufficient for an IND application (Months 24-54)."*

The wording "45 months after the Commencement Date" set out in the Award Letter for Milestone 3 under the heading 'Milestone Date' *shall be deleted and replaced with the wording "54 months after the Commencement Date"*

The amendments set out above shall have effect as from the date this Third Extension Letter is countersigned by PTC. Except as set out in this Third Extension Letter, all other provisions of the Agreement and the Award Letter shall remain in full force and effect. All capitalised terms in this Third Extension Letter shall have the same meaning as those in the Agreement and the Award Letter. This Third Extension Letter shall be governed by and interpreted in accordance with English law.

Please would you arrange for two copies of this Third Extension Letter to be signed on behalf of PTC and return one to us for our records.

Signed for and on behalf of
THE WELLCOME TRUST LIMITED
as trustee of the Wellcome Trust:

Signature: /s/ Dr Richard Seabrook
Name: Dr Richard Seabrook
Head of Business Development
Technology Transfer

Date: 3/3/14

Accepted and signed for and on behalf of
PTC THERAPEUTICS, INC:

Signature: /s/ Mark E. Boulding
Name: Mark E. Boulding
Executive Vice President
Chief Legal Officer

Date: 3/11/14

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100 Corporate Court
South Plainfield
NJ 07079
USA

Our Ref: 092687

27 March 2015

Dear Sirs

Phase 1 Milestone Deferral Amendment to the Seeding Drug Discovery Initiative Funding Agreement (the "Milestone Deferral Letter")

We write with reference to:

1. the agreement for the provision of funding between The Wellcome Trust Limited as trustee of the Wellcome Trust (the "**Trust**") and PTC Therapeutics Inc. ("**PTC**") dated 26 May 2010 (the "**Funding Agreement**");
2. the award letter from the Trust to the Principal Investigator dated 26 May 2010 (the "**Award Letter**");
3. the no cost milestone extension letter to the Funding Agreement dated 08 May 2012 (the "**Extension Letter**");
4. the second no cost milestone extension letter to the Funding Agreement dated 24 May 2013 (the "**Second Extension Letter**"),
5. the third no cost milestone extension letter to the Funding Agreement dated 3 March 2014 (effective 11 March 2014) (the "**Third Extension Letter**").

Together, the Original Agreement, the Award Letter, the Extension Letter, the Second Extension Letter and the Third Extension Letter are herein referred to as the "**Agreement**".

The Agreement was to enable the Trust to grant PTC up to a maximum amount of US$5,397,000 (five million three hundred and ninety-seven thousand United States dollars) payable in milestone dependent instalments for conducting a research programme (led by Dr Thomas Davis) aimed at identifying small molecules that selectively decrease the production of Bmi-1 expression in tumour stem cells.

Pursuant to discussions with PTC about deferring payment of the first milestone, the Trust is now willing to grant an extension of time to pay the milestone due from PTC in connection with the first enrolment of a subject in a Phase 1 Clinical Trial of a Product. In consideration of the sum of one pound (£1), the receipt of which is hereby acknowledged by the Trust, the Parties agree to amend Sections 2(a)(i)(1) and 2(a)(iv)(1) of Schedule 6 to the Funding Agreement as follows:

1. The wording:

"(1) "First enrolment of a *subject in* a *Phase 1 Clinical Trial of* a *Product: 0.1x the Trust Contribution."*

being the entirety of Section 2(a)(i)(1) of Schedule 6 of the Funding Agreement shall be deleted and replaced with the following wording:

"(1) "First anniversary of the enrolment of a subject in a Phase 1 Clinical Trial of a Product: 0.15x the Trust Contribution."

2. The wording:

"(1) Phase 1-Phase 3 milestones shall be payable in equal quarterly instalments over expected term of study, with the first installment payment due within [**] Business Days of the milestone triggering event. PTC may by written notice to the Trust elect to defer payment of the Phase 3 milestone until after completion of the first Phase 3 study to be completed in either the USA or the EAA (whichever is the sooner) required for Regulatory Approval (the "Trigger Phase 3"), in which case PTC shall pay such Phase 3 milestone (including additional 1x due for the deferral option) within [**] of completion of the Trigger Phase 3 study. For clarity, if deferred, the payment of Phase 3 milestone is due regardless of outcome of Phase 3 trial(s); provided, that the Trust agrees to accept alternative consideration, such as equity, in the event a cash payment after a Phase 3 trial failure would place PTC in financial distress."

being the entirety of Section 2(a)(iv)(1) of Schedule 6 of the Funding Agreement shall be deleted and replaced with the following wording:

"(1) The Phase 1 milestone shall be payable as a lump sum on the first anniversary of the enrolment of a subject in a Phase 1 Clinical Trial of a Product.

The Phase 2-Phase 3 milestones shall be payable in equal quarterly instalments over expected term of study, with the first instalment payment due within [**] Business Days of the milestone triggering event.

PTC may by written notice to the Trust elect to defer payment of the Phase 3 milestone until after completion of the first Phase 3 study to be completed in either the USA or the EAA (whichever is the sooner) required for Regulatory Approval (the "Trigger Phase 3"), in which case PTC shall pay such Phase 3 milestone (including additional 1x due for the deferral option) within [**] of completion of the Trigger Phase 3 study. For clarity, if deferred, the payment of Phase 3 milestone is due regardless of outcome of Phase 3 trial(s); provided, that the Trust agrees to accept alternative consideration, such as equity, in the event a cash payment after a Phase 3 trial failure would place PTC in financial distress."

The amendments set out above shall have effect as from the date this Milestone Deferral Letter is countersigned by PTC. Except as set out in this Milestone Deferral Letter, all other provisions of the Agreement shall remain in full force and effect. All capitalised terms in this Milestone Deferral Letter shall have the same meaning as those in the Agreement. This Milestone Deferral Letter shall be governed by and interpreted in accordance with English law.

Please would you arrange for two copies of this Milestone Deferral Letter to be signed on behalf of PTC and return one to us for our records.

Signed for and on behalf of
THE WELLCOME TRUST LIMITED
as trustee of the Wellcome Trust:

Signature:	/s/ Dr Richard Seabrook	Signature:	/s/ Sari Watson
Name:	Dr Richard Seabrook Head of Business Development Innovations	Name:	Sari Watson Innovations
Date:	27/3/15	Date:	27/3/15

Accepted and signed for and on behalf of
PTC THERAPEUTICS, INC:

Signature:	/s/ Mark E. Boulding	Signature:	
Name:	Mark E. Boulding Executive Vice President Chief Legal Officer	Name:	
Date:	27 March 2015	Date:	

Private and Confidential

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E-MAIL: t.jinks@wellcome.ac.uk

Our Ref: 092687

19 January 2017

Dear Sirs

Amendment to the Seeding Drug Discovery Initiative Funding Agreement (the "Fifth Amendment")

We write with reference to:

1. the funding agreement between The Wellcome Trust Limited as trustee of the Wellcome Trust (the "**Trust**") and PTC Therapeutics, Inc. ("**PTC**") dated 26 May 2010;
2. the no cost milestone extension letter to the funding agreement dated 08 May 2012 (fully executed 15 May 2012);
3. the Second Amendment dated 31 May 2013 (fully executed 05 June 2013);
4. the Third Extension Letter dated 03 March 2014 (fully executed 11 March 2014); and
5. the Phase 1 Milestone Deferral Amendment dated 27 March 2015,

together, the "**Agreement**"; and the Award Letter from the Trust to the Principal Investigator dated 26 May 2010.

The Agreement was to enable the Trust to grant PTC up to a maximum amount of US$5,397,000 (five million three hundred thousand and ninety-seven thousand United States dollars) payable in milestone dependent instalments for conducting a research programme aimed at identifying small molecules that selectively decrease the production of Bmi-1 expression in tumour stem cells.

Pursuant to Clause 23.2 of the Agreement and in consideration of the sum of one pound (£1) from PTC, the receipt of which is hereby acknowledged by the Trust, the Parties agree to amend the Agreement as follows:

> *SCHEDULE 6 (REVENUE SHARING TERMS) is deleted in its entirety and replaced with SCHEDULE 6V2 (REVENUE SHARING TERMS) attached to this Fifth Amendment.*

The amendment set out above shall have effect as from the date this Fifth Amendment is countersigned by PTC. Except as set out in this Fifth Amendment, all other provisions of the Agreement and the Award Letter shall remain in full force and effect. All capitalised terms in this Fifth Amendment shall have the same meaning as those in the Agreement and the Award Letter. This Fifth Amendment shall be governed by and interpreted in accordance with English law.

Please would you arrange for two copies of this Fifth Amendment to be signed on behalf of PTC and return one to us for our records.

Signed for and on behalf of
THE WELLCOME TRUST LIMITED
as trustee of the Wellcome Trust:

Signature: /s/ Sari Watson
Name: Sari Watson
Innovations

Date: 19/1/2017

Accepted and signed for and on behalf of
PTC THERAPEUTICS, INC:

Signature: /s/ Mark E. Boulding
Name: Mark E. Boulding
Executive Vice President
Chief Legal Officer

Date: 25 Jan 2017

SCHEDULE 6V2

REVENUE SHARING TERMS

1) Introduction

a) This Schedule 6 sets out the revenue sharing terms ("Revenue Sharing Terms") agreed between the parties.

b) Each scenario below shall apply based on the description of the scenario.

2) **Scenario 1**: PTC exploits the Programme Intellectual Property on a For-Profit Basis alone (or in collaboration with a Distributor or marketing/sales agent under which PTC retains overall control of worldwide commercialization).

a) PTC shall pay the following stage-based milestones based on multiples of the total Trust Contribution through Regulatory Approval:

i) Milestone triggering events and amounts:

(1) First enrolment of a subject in a Phase 1 Clinical Trial of a Product: 0.15x the Trust Contribution.

(2) First enrolment of a subject in a Phase 2 Clinical Trial of a Product: $2.5 million.

(3) First enrolment of a subject in a Phase 3 Clinical Trial of a Product (with option to delay to as provided below for an additional 1x payment): $4.5 million.

(4) Regulatory Approval of a Product: $10 million.

(5) Provided, that the third and fourth milestones above (for the Phase 3 trial and Regulatory Approval) shall be payable only in the event the Trust Contribution represents at least 80 % of the proposed $5.4 million US funding amount.

ii) Worked Example: assumes Trust funds $5.4 million US and PTC does not elect to defer payment of the Phase 3 milestone:

(1) Phase 1 milestone amount = $810,000

(2) Phase 2 milestone amount = $2.5 million

(3) Phase 3 milestone amount = $4.5 million

(4) Regulatory Approval milestone amount = $10 million

(5) Total of all milestone amounts= $17,810,000

iii) Worked Example: assumes Trust funds $5.4 million and PTC defers at Phase 3:

(1) Phase 1 milestone amount = $810,000

(2) Phase 2 milestone amount = $2.5 million

(3) Phase 3 milestone amount = $9.9 million (deferred as provided below)

(4) Regulatory Approval milestone amount =$10 million

(5) Total of all milestone amounts = $23,210,000

iv) Payment of milestones

(1) The Phase 1 milestone shall be payable as a lump sum on the first anniversary of the enrolment of a subject in a Phase 1 Clinical Trial of a Product.

The Phase 2 milestone shall be payable as a lump sum due within [**] Business Days of the earlier of the completion of enrolment (last patient, first dose) or termination of dosing of all patients in the first Phase 2 study.

The Phase 3 milestone shall be payable in equal quarterly installments over expected term of study, with the first installment payment due within [**] Business Days of the milestone triggering event.

PTC may by written notice to the Trust elect to defer payment of the Phase 3 milestone until after completion of the first Phase 3 study to be completed in either the USA or the EAA (whichever is the sooner) required for Regulatory Approval (the "Trigger Phase 3"), in which case PTC shall pay such Phase 3 milestone (including additional 1x due for the deferral option) within [**] of completion of the Trigger Phase 3 study. For clarity, if deferred, the payment of Phase 3 milestone is due regardless of outcome of Phase 3 trial(s); provided, that the Trust agrees to accept alternative consideration, such as equity, in the event a cash payment after a Phase 3 trial failure would place PTC in financial distress.

(2) For clarity, milestones are payable only for the first Product to reach the applicable milestone.

(3) The Regulatory Approval milestone shall be payable on the [**]; provided, however, that the Trust will consider in good faith payment of the Regulatory Approval milestone in installments if PTC revenue from all products at the time of Regulatory Approval is less than $[**].

b) In addition to any milestones payable in accordance with the preceding section, PTC shall also pay royalties on Net Sales of Products, on a Product-by-Product basis; provided, that such royalties shall only be payable in the event the Trust Contribution represents at least [**]% of the proposed $5.4 million US funding amount, and shall be scaled proportionately in the event the Trust Contribution is greater than [**]% but less than 100% of the proposed $5.4 million US funding amount:

i) Royalty based on Net Sales of Product: [**]%

ii) Royalties payable shall be payable on a country-by-country basis until the longer of (a) the expiration last Valid Claim of a patent in the applicable country or region covering the Product, or (ii) the expiration of marketing exclusivity of a Product in the applicable country or region based on applicable law.

3) **Scenario 2**: PTC exploits the Programme Intellectual Property on a For-Profit Basis through outlicensing of a Product to a Third Party on a worldwide, exclusive basis prior to Regulatory Approval.

a) The parties shall hold an economic stake ("Base Shares") in the Product calculated as of outlicensing effective date based on their respective economic contributions.

i) On the Commencement Date, PTC begins with $5.4 million Base Shares, and the Trust with zero.

ii) As the Trust pays the proposed the proposed $5.4 million US funding amount over the Programme Term, the Trust's Base Share shall increase proportionately. By way of example, [**]

iii) Following the Programme Term, PTC's ownership of Base Shares shall increase proportionately based on PTC's continuing economic contribution. By way of example, [**]

b) All consideration attributable to outlicensing to a Third Party (other than debt at arm's length interest rates or bona fide research funding) shall be divided between PTC and the Trust according to relative Base Share ownership at the time of such outlicensing, provided that, PTC will guarantee total payments to the Trust (including all prior payments per Scenario 1 and Scenario 2 of this Schedule) of at least 1x the Trust Contribution (i.e., $5.4 million). By way of example, [**].

c) For clarity, once outlicensing under this scenario has occurred, then the milestones provided for in scenario 1 shall no longer apply following the effective date of the outlicense; provided, that if a milestone trigger event occurred prior to the outlicense but installment payments are ongoing, PTC must complete such milestone payments.

d) For clarity, neither PTC nor the Third Party gaining the outlicense shall make any royalty payments to the Trust under this scenario.

e) License or access payments to Third Parties for enabling technologies required, in the good faith judgment of PTC, to develop and commercialize a Product shall be counted in the calculation of Base Shares under this scenario; provided, however, that such payments shall not include license or access payments made with respect to the composition of matter or method of use of those active ingredient(s) in the Product that incorporate, comprise or are derived from the Programme Intellectual Property.

4) **Scenario 3**: PTC exploits the Programme Intellectual Property on a For-Profit Basis by retaining development/commercialization rights to Product in some regions of the World or with respect to some uses of the Product (either alone or in a collaboration with a Distributor or marketing/sales agent under which PTC retains overall control of commercialization)), and outlicenses the Product on an exclusive basis in other regions of the World or with respect to other uses of the Product.

a) In this scenario, any consideration from outlicensing (other than debt at arm's length interest rates or bona fide research funding) shall be divided between the parties according to Base Shares as of effective date of the outlicense.

b) In addition, following such outlicense, PTC shall pay milestones and royalties based on scenario 1 for those regions of the World or uses of the Product for which it retains rights, subject to the following adjustments:

i) PTC will prepare a written proposal for adjustment to milestones and royalties based on its modeling of the relative values of market share outlicensed vs. market share retained by PTC.

(1) The Trust shall consider PTC's proposal in good faith, and prepare a written counterproposal if it wishes;

(2) The parties shall negotiate in good faith for reasonable allocation of relative value of markets based on their proposals;

(3) If the parties cannot agree within [**] days, then the matter shall be referred for final determination via arbitration pursuant to Clause 19.3(a).

(4) Once the relative value of the markets outlicensed versus the markets retained by PTC is determined, PTC's obligation to make continuing milestone and royalty payments pursuant to Scenario 1 shall be reduced according to relative value of markets outlicensed versus the markets retained. By way of example, if PTC outlicensed [**] of the market value of a Product, then a milestone payment of $[**] owed under scenario 1 would be reduced to a milestone payment of $[**] under this scenario 3, and a [**]% Net Sales royalty under scenario 1 would become a [**]% Net Sales royalty under this scenario 3.

5) **Other Scenarios:**

a) If a situation arises that is not covered by any of the foregoing three scenarios, the parties will negotiate in good faith for an appropriate economic arrangement based on Base Shares.

i) If the parties cannot agree, each party shall prepare a written proposal and accompanying rationale for an appropriate economic arrangement.

ii) The parties shall then negotiate in good faith based on their respective proposals.

iii) If parties cannot agree within [**] days, then the matter shall be referred for final determination via arbitration pursuant to Clause 19.3(a).

Exhibit 10.55

Execution Version

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

CREDIT AGREEMENT

dated as of October 27, 2022

among

PTC THERAPEUTICS, INC.,
as the Borrower,

EACH SUBSIDIARY OF THE BORROWER FROM TIME TO TIME PARTY HERETO,
as Guarantors,

THE LENDERS FROM TIME TO TIME PARTY HERETO

and

WILMINGTON TRUST, NATIONAL ASSOCIATION,
as Administrative Agent

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SCHEDULES

EXHIBITS

CREDIT AGREEMENT

This **CREDIT AGREEMENT** is entered into as of October 27, 2022, among PTC THERAPEUTICS, INC., a Delaware corporation (the "*Borrower*"), the Guarantors (as defined herein) from time to time party hereto, the Lenders (as defined herein) from time to time party hereto and Wilmington Trust, National Association, as Administrative Agent (as defined herein).

PRELIMINARY STATEMENTS:

WHEREAS, the Borrower has requested that the Lenders extend term loan facilities comprised of (a) an initial term loan facility in the aggregate principal amount of $300,000,000, which will be funded on the Closing Date (as defined herein), subject to the conditions provided herein, and (b) a delayed draw term loan facility in an aggregate principal amount of up to $150,000,000, subject to the conditions provided herein;

WHEREAS, the Lenders have agreed to make such term loan facilities available to the Borrower on the terms and subject to the conditions set forth herein; and

WHEREAS, the Borrower may further request, and, subject to Required Lender consent in their sole discretion, one or more of the Lenders may consider providing incremental investments in a form and on terms to be determined, in connection with future development opportunities, in an aggregate amount of up to $500,000,000, it being understood that no commitment exists or is being created hereunder for any Lender to provide, or to consider providing, any such incremental investments.

NOW THEREFORE, in consideration of the mutual covenants and agreements herein contained, the parties hereto covenant and agree as follows:

ARTICLE I

DEFINITIONS AND ACCOUNTING TERMS

1.01 Defined Terms.

As used in this Agreement, the following terms shall have the meanings set forth below:

"*Acquisition*" means the purchase, inbound license or other acquisition, or option to purchase, license or otherwise acquire, whether through a single transaction or a series of related transactions, of (a) a majority of the Equity Interests, whether by purchase of such Equity Interests or upon the exercise of an option or warrant for, or conversion of securities into, such Equity Interests, of another Person, (b) assets of another Person which constitute all or substantially all of the assets of such Person or of a division, line of business or other business unit of such Person or (c) assets including IP Rights, royalty rights or similar assets of such Person.

"*Action*" means any claim, action, cause of action or suit, litigation, assessment, arbitration, mediation, investigation, audit, hearing, charge, complaint, demand, notice or proceeding (in each case, whether in contract, tort or otherwise, whether at law or in equity, and whether civil or criminal) to, from, by or before any Governmental Authority.

"*Adjusted Term SOFR*" means, for purposes of any calculation, the rate per annum equal to (a) Term SOFR for such calculation plus (b) the Term SOFR Adjustment.

"*Administrative Agent*" means Wilmington Trust, National Association, solely in its capacity as administrative agent under any of the Loan Documents, or any successor administrative agent permitted by the terms hereof.

"*Administrative Agent's Account*" means the Administrative Agent's account as the Administrative Agent may from time to time notify the Borrower and the Lenders in accordance with Section 11.02.

"*Administrative Questionnaire*" means an Administrative Questionnaire in substantially the form of Exhibit A or any other form approved by the Administrative Agent.

"*Affected Financial Institution*" means (a) any EEA Financial Institution or (b) any UK Financial Institution.

"*Affiliate*" means, with respect to a specified Person, another Person that directly, or indirectly through one or more intermediaries, Controls or is Controlled by or is under common Control with the Person specified.

"*Agency Fee Letter*" means that certain letter agreement, dated as of the date hereof, between the Borrower and the Administrative Agent.

"*Aggregate Commitments*" means, at any time, the sum of the Initial Commitments and the Delayed Draw Commitments, as reduced, respectively, by the Initial Loans and any Delayed Draw Loans.

"*Agreement*" means this Credit Agreement, including all schedules, exhibits and annexes hereto.

"*Applicable Law*" means, as to any Person, all applicable Laws binding upon such Person or to which such a Person is subject.

"*Applicable Percentage*" means, with respect to any Lender at any time, (i) with respect to all payments, computations and other matters relating to the Initial Loans and/or Initial Commitments, the percentage obtained by dividing (a) the unused Initial Commitment and Outstanding Amount of Initial Loans of such Lender at such time by (b) the aggregate unused Initial Commitments and aggregate Outstanding Amount of Initial Loans of all of the Lenders at such time; and (ii) with respect to all payments, computations and other matters relating to the Delayed Draw Loans and/or Delayed Draw Commitments, the percentage obtained by dividing (a) the unused Delayed Draw Commitment and Outstanding Amount of Delayed Draw Loans of such Lender at such time by (b) the aggregate unused Delayed Draw Commitments and aggregate Outstanding Amount of Delayed Draw Loans of all of the Lenders at such time.

"*Applicable Premium*" means, with respect to any repayment or prepayment of principal made, or required to be made and upon any acceleration of the Maturity Date that, in each case, is effected or occurs (the date of such repayment, prepayment or acceleration, a "*Calculation Date*") of any Loans: (a) on or prior to the third anniversary of the funding of the applicable Loans, the sum of (i) 3.00% of the principal amount of Loans being repaid or subject to such acceleration plus (ii) an amount, determined by the Required Lenders in good faith computed using a discount rate equal to the Treasury Rate as of such Calculation Date plus 50 basis points, equal to all required remaining scheduled interest payments which would have been due on the Loans being repaid or subject to such acceleration if such Loans had remained outstanding and unaccelerated, in each case, through the third anniversary of such funding date (excluding accrued and unpaid interest to, but excluding, the Calculation Date) (provided that any interest that would otherwise have accrued from the period commencing after the Interest Payment Date occurring immediately prior to such third anniversary and ending on and including the third anniversary of such funding date shall

be deemed for purposes of this definition to be a required remaining scheduled interest payment that would have otherwise been due on the third anniversary of the applicable funding date) and provided that for purposes of this calculation, the interest rate shall be deemed to be the Applicable Rate in effect on the Calculation Date, (b) after the third anniversary of the funding of the applicable Loans and on or prior to the fourth anniversary of such funding, an amount equal to 3.00% of the principal amount of the Loan being repaid or subject to such acceleration, (c) after the fourth anniversary of the funding of the applicable Loans and on or prior to the fifth anniversary of such funding, an amount equal to 2.00% of the principal amount of the Loan being repaid or subject to such acceleration, (d) after the fifth anniversary of the funding of the applicable Loans and on or prior to the sixth anniversary of such funding, an amount equal to 1.00% of the principal amount of the Loan being repaid or subject to such acceleration and (e) after the sixth anniversary of such funding, 0.00%. The Borrower and the Guarantors acknowledge that the Lenders shall suffer damages on account of the payment of the Loans prior to the sixth anniversary of the funding date thereof or the acceleration of the Maturity Date and that the Applicable Premium is a reasonable calculation of the lost profits of the Lenders holding the Loans in view of the difficulties and impracticality of determining actual damages resulting from such repayment, prepayment or acceleration.

"*Applicable Rate*" means (a) with respect to any SOFR Loan, Adjusted Term SOFR plus 7.25% per annum, and (b) with respect to any Base Rate Loan, the Base Rate plus 6.25% per annum.

"*Approved Fund*" means any Person (other than a natural Person) that is (a) a Blackstone Investor or Blackstone Entity or (b) a Fund that is administered, managed or advised by (i) a Lender, (ii) an Affiliate of a Lender or (iii) an entity or an Affiliate of an entity that administers, manages or advises a Lender.

"*Assignment and Assumption*" means an assignment and assumption entered into by a Lender and an Eligible Assignee (with the written consent of any party whose consent is required by Section 11.06(b)), and accepted by the Administrative Agent, in substantially the form of Exhibit B or any other form (including an electronic documentation form generated by use of an electronic platform) approved by the Administrative Agent.

"*Attributable Indebtedness*" means, on any date, (a) in respect of any Finance Lease of any Person, the capitalized amount thereof that would appear on a balance sheet of such Person prepared as of such date in accordance with GAAP, and (b) in respect of any Synthetic Lease Obligation, the capitalized amount of the remaining lease or similar payments under the relevant lease or other applicable agreement or instrument that would appear on a balance sheet of such Person prepared as of such date in accordance with GAAP if such lease or other agreement or instrument were accounted for as a Finance Lease.

"*Audited Financial Statements*" means the audited Consolidated balance sheet of the Borrower and its Subsidiaries for the fiscal year ended December 31, 2021, and the related Consolidated statements of operations and comprehensive loss, shareholders' equity and cash flows for such fiscal year of the Borrower and its Subsidiaries, including the notes thereto.

"*Available Tenor*" means, as of any date of determination and with respect to the then-current Benchmark, as applicable, (a) if such Benchmark is a term rate, any tenor for such Benchmark (or component thereof) that is or may be used for determining the length of an interest period pursuant to this Agreement or (b) otherwise, any payment period for interest calculated with reference to such Benchmark (or component thereof) that is or may be used for determining any frequency of making payments of interest calculated with reference to such Benchmark pursuant to this Agreement, in each case, as of such date and not including, for the avoidance of doubt, any tenor for such Benchmark that is then-removed from the definition of "Interest Period" pursuant to Section 3.03(d).

"*Bail-In Action*" means the exercise of any Write-Down and Conversion Powers by the applicable EEA Resolution Authority in respect of any liability of an Affected Financial Institution.

"*Bail-In Legislation*" means, (a) with respect to any EEA Member Country implementing Article 55 of Directive 2014/59/EU of the European Parliament and of the Council of the European Union, the implementing law, regulation, rule or requirement for such EEA Member Country from time to time which is described in the EU Bail-In Legislation Schedule and (b) with respect to the United Kingdom, Part I of the United Kingdom Banking Act 2009 (as amended from time to time) and any other law, regulation or rule applicable in the United Kingdom relating to the resolution of unsound or failing banks, investment firms or other financial institutions or their affiliates (other than through liquidation, administration or other insolvency proceedings).

"*Base Rate*" means for any day a fluctuating rate of interest per annum equal to the highest of (a) the Federal Funds Rate *plus* 0.50%, (b) the Prime Rate, (c) Adjusted Term SOFR (or if such day is not a Business Day, the immediately preceding Business Day) based on an interest period of one (1) month, subject to the interest rate floors set forth therein, *plus* 1.00% and (d) 2.00%. Any announced change in the Prime Rate shall take effect at the opening of business on the day specified in the public announcement of such change. If the Administrative Agent shall have determined (which determination shall be conclusive absent manifest error) that it is unable to ascertain the Federal Funds Rate or Term SOFR for any reason, the Base Rate shall be determined without regard to clause (a) or (c) above, as applicable, until the circumstances giving rise to such inability no longer exist. If the Base Rate is being used as an alternate rate of interest pursuant to Section 3.03 hereof, then the Base Rate shall be the greater of clauses (a), (b) and (d) above and shall be determined without reference to clause (c) above.

"*Base Rate Loan*" means a Loan that bears interest based on the Base Rate.

"*Benchmark*" means, initially, the Term SOFR Reference Rate; provided that if a Benchmark Transition Event has occurred with respect to the Term SOFR Reference Rate or the then-current Benchmark, then "Benchmark" means the applicable Benchmark Replacement to the extent that such Benchmark Replacement has replaced such prior benchmark rate pursuant to Section 3.03.

"*Benchmark Replacement*" means, with respect to any Benchmark Transition Event, the sum of: (a) the alternate benchmark rate that has been selected by Administrative Agent (acting at the direction of the Required Lenders) and Borrower giving due consideration to (i) any selection or recommendation of a replacement benchmark rate or the mechanism for determining such a rate by the Relevant Governmental Body or (ii) any evolving or then-prevailing market convention for determining a benchmark rate as a replacement for the then-current Benchmark for Dollar-denominated syndicated credit facilities and (b) the related Benchmark Replacement Adjustment; provided that if such Benchmark Replacement as so determined would be less than 1.00% per annum, such Benchmark Replacement shall be deemed to be 1.00% per annum for the purposes of this Agreement and the other Loan Documents.

"*Benchmark Replacement Adjustment*" means, with respect to any replacement of the then-current Benchmark with an Unadjusted Benchmark Replacement for any applicable Available Tenor (if applicable), the spread adjustment, or method for calculating or determining such spread adjustment, (which may be a positive or negative value or zero) that has been selected by Administrative Agent (acting at the direction of the Required Lenders) and Borrower giving due consideration to (a) any selection or recommendation of a spread adjustment, or method for calculating or determining such spread adjustment, for the replacement of such Benchmark with the applicable Unadjusted Benchmark Replacement by the Relevant Governmental Body or (b) any evolving or then-prevailing market convention for determining a spread adjustment, or method for calculating or determining such spread adjustment, for the replacement

of such Benchmark with the applicable Unadjusted Benchmark Replacement for Dollar-denominated syndicated credit facilities at such time.

"*Benchmark Replacement Date*" means the earliest to occur of the following events with respect to the then-current Benchmark:

(a) in the case of clause (a) or (b) of the definition of "Benchmark Transition Event," the later of (i) the date of the public statement or publication of information referenced therein and (ii) the date on which the administrator of such Benchmark (or the published component used in the calculation thereof) permanently or indefinitely ceases to provide all Available Tenors (if applicable) of such Benchmark (or such component thereof) or

(b) in the case of clause (c) of the definition of "Benchmark Transition Event", the first date on which such Benchmark (or the published component used in the calculation thereof) has been determined and announced by the regulatory supervisor for the administrator of such Benchmark (or such component thereof) to be non-representative; provided, that such non-representativeness will be determined by reference to the most recent statement or publication referenced in such clause (c) and even if any Available Tenor (if applicable) of such Benchmark (or such component thereof) continues to be provided on such date.

For the avoidance of doubt, if the then-current Benchmark has any Available Tenors, the "Benchmark Replacement Date" will be deemed to have occurred in the case of clause (a) or (b) with respect to any Benchmark upon the occurrence of the applicable event or events set forth therein with respect to all then-current Available Tenors of such Benchmark (or the published component used in the calculation thereof).

"*Benchmark Transition Event*" means the occurrence of one or more of the following events with respect to the then-current Benchmark:

(a) a public statement or publication of information by or on behalf of the administrator of such Benchmark (or the published component used in the calculation thereof) announcing that such administrator has ceased or will cease to provide all Available Tenors (if applicable) of such Benchmark (or such component thereof), permanently or indefinitely, provided that, at the time of such statement or publication, there is no successor administrator that will continue to provide any Available Tenor (if applicable) of such Benchmark (or such component thereof);

(b) a public statement or publication of information by the regulatory supervisor for the administrator of such Benchmark (or the published component used in the calculation thereof), the Board of Governors, the Federal Reserve Bank of New York, an insolvency official with jurisdiction over the administrator for such Benchmark (or such component), a resolution authority with jurisdiction over the administrator for such Benchmark (or such component) or a court or an entity with similar insolvency or resolution authority over the administrator for such Benchmark (or such component), which states that the administrator of such Benchmark (or such component) has ceased or will cease to provide all Available Tenors (if applicable) of such Benchmark (or such component thereof) permanently or indefinitely, provided that, at the time of such statement or publication, there is no successor administrator that will continue to provide any Available Tenor (if applicable) of such Benchmark (or such component thereof); or

(c) a public statement or publication of information by the regulatory supervisor for the administrator of such Benchmark (or the published component used in the calculation thereof)

announcing that all Available Tenors of such Benchmark (or such component thereof) are not, or as of a specified future date will not be, representative.

For the avoidance of doubt, if the then-current Benchmark has any Available Tenors, a "Benchmark Transition Event" will be deemed to have occurred with respect to any Benchmark if a public statement or publication of information set forth above has occurred with respect to each then-current Available Tenor of such Benchmark (or the published component used in the calculation thereof).

"*Benchmark Transition Start Date*" means, in the case of a Benchmark Transition Event, the earlier of (a) the applicable Benchmark Replacement Date and (b) if such Benchmark Transition Event is a public statement or publication of information of a prospective event, the 90th day prior to the expected date of such event as of such public statement or publication of information (or if the expected date of such prospective event is fewer than 90 days after such statement or publication, the date of such statement or publication).

"*Benchmark Unavailability Period*" means the period (if any) (x) beginning at the time that a Benchmark Replacement Date has occurred if, at such time, no Benchmark Replacement has replaced the then-current Benchmark for all purposes hereunder and under any Loan Document in accordance with Section 3.03 and (y) ending at the time that a Benchmark Replacement has replaced the then-current Benchmark for all purposes hereunder and under any Loan Document in accordance with Section 3.03.

"*Beneficial Ownership Certification*" means a certification regarding beneficial ownership required by the Beneficial Ownership Regulation.

"*Beneficial Ownership Regulation*" means 31 C.F.R. § 1010.230.

"*Bermudan Loan Party*" means PTC Therapeutics Holdings (Bermuda) Corp. Limited, an exempted company incorporated in Bermuda.

"*Blackstone Affiliated Entities*" shall mean, collectively, the Blackstone Entities, any Blackstone Investor and any Controlled Investment Affiliates of a Blackstone Entity.

"*Blackstone Credit*" shall mean Blackstone Alternative Credit Advisors LP.

"*Blackstone Life Sciences*" shall mean Blackstone Life Sciences Advisors L.L.C.

"*Blackstone Investor*" means any investor (or any Controlled Investment Affiliate of such investor) of a fund managed or advised by a Blackstone Credit or Blackstone Life Sciences to which such investor (or any Controlled Investment Affiliate of such investor) Blackstone Credit or Blackstone Life Sciences, as applicable, is providing certain administrative and other services and controls investment decisions with respect to any Commitments and Loans held by such investor (or Controlled Investment Affiliate of such investor).

"*Blackstone Entities*" shall mean Blackstone Life Sciences, Blackstone Credit, Blackstone Holdings Finance Co. L.L.C and any other Person that directly, or indirectly through one or more intermediaries, controls or is controlled by or is under common control with any of the foregoing entities, and funds and accounts administered, managed, agented or advised by any of them, and any warehouse entity. For purposes of this definition "control" of a Person is either (i) the beneficial ownership of, or power to vote, more than 50% of the Voting Stock of such Person, or (ii) such Person constituting, or having the power to designate, the managing member or general partner of such Person or to appoint a majority of the members of the board of directors or board of managers or other governing body of such Person, or (iii)

the right to direct or cause the direction of the management and policies of such other Person (whether by contract or otherwise).

"*Bona Fide Debt Fund*" means any debt fund that is an Affiliate of any Competitor or Competitor Controller that is primarily engaged in, or advises funds that are primarily engaged in, making, purchasing, or holding commercial loans, notes and similar extensions of credit or securities in the ordinary course of its business, but only to the extent that no personnel involved therewith (A) makes (or has the right to make or participate with others in making) investment decisions on behalf of, or otherwise cause the direction of the investment policies of, such Competitor or Competitor Controller or (B) has access to any information (other than information that is publicly available) relating to the Borrower or its Subsidiaries and/or any entity that forms part of any of their respective businesses (including any of their respective subsidiaries).

"*Borrower*" has the meaning specified in the introductory paragraph hereto.

"*Borrower Investment Policy*" means the investment policy of the Borrower and its Subsidiaries as in effect on the Closing Date and any amendments, modifications or supplements thereto following the Closing Date that are (1) approved and duly adopted by the board of directors (or other governing body) of the Borrower and (2) agreed to by the Required Lenders in their reasonable discretion.

"*Borrower Materials*" has the meaning specified in Section 6.02.

"*Borrowing*" means a borrowing consisting of simultaneous Loans of the same Type and, in the case of SOFR Loans, having the same Interest Period made by each of the Lenders pursuant to Section 2.01.

"*Brazilian Loan Party*" means PTC Farmacêutica do Brasil Ltda., a Sociedade de responsabilidade limitada organized under the laws of Brazil.

"*Business Day*" means any day other than a Saturday, Sunday or other day on which commercial banks are authorized to close under the Laws of, or are in fact closed in, the state of New York; provided, however, that when used in connection with a SOFR Loan, the term "Business Day" shall also exclude any day on which the Securities Industry and Financial Markets Association recommends that the fixed income departments of its members be closed for the entire day for purposes of trading in United States government securities.

"*Cash Collateralize*" means to pledge and deposit with or deliver to the Administrative Agent, for the benefit of the Secured Parties, as collateral for the Obligations, cash, Cash Equivalents or deposit account balances or, if the Required Lenders shall agree in their sole discretion, other credit support, in each case, pursuant to documentation in form and substance reasonably satisfactory to the Required Lenders. "Cash Collateral" shall have a meaning correlative to the foregoing and shall include the proceeds of such cash collateral and other credit support.

"*Cash Equivalents*" means any of the following types of Investments, to the extent owned by the Borrower or any of its Subsidiaries free and clear of all Liens (other than Permitted Liens):

(a) (i) readily marketable obligations issued or directly and fully guaranteed or insured by the United States or any agency or instrumentality thereof having maturities of not more than one (1) year from the date of acquisition thereof; *provided* that the full faith and credit of the United States is pledged in support thereof and (ii) readily marketable direct obligations issued by any state, commonwealth or territory of the United States of America or political subdivision or taxing

authority thereof that is rated AAA by S&P and Aaa by Moody's maturing within one (1) year from the date of acquisition thereof;

(b) time deposits with, or insured certificates of deposit or bankers' acceptances of, any commercial bank that (i) (A) is a Lender or (B) is organized under the laws of the United States, any state thereof or the District of Columbia or is the principal banking subsidiary of a bank holding company organized under the laws of the United States, any state thereof or the District of Columbia, and is a member of the Federal Reserve System, (ii) issues (or the parent of which issues) commercial paper rated as described in clause (c) of this definition and (iii) has combined capital and surplus of at least $[**], in each case with maturities of not more than one (1) year from the date of acquisition thereof;

(c) commercial paper issued by a corporation or other Person rated at least "A-2" or "P-2" or the equivalent thereof by Moody's or S&P or Fitch (or, if at any time neither Moody's nor S&P shall be rating such obligations, an equivalent rating from another nationally recognized statistical rating agency selected by the Required Lenders) and in each case maturing within one (1) year from the date of acquisition thereof;

(d) marketable short-term money market and similar highly liquid securities having a rating of at least P-2 or A-2 from either Moody's or S&P, respectively (or, if at any time neither Moody's nor S&P shall be rating such obligations, an equivalent rating from another nationally recognized statistical rating agency selected by the Required Lenders) and in each case maturing within one (1) year from the date of acquisition thereof;

(e) solely with respect to Foreign Subsidiaries, investments of the type and maturities described in clauses (a) through (d) above, issued where relevant, by any commercial bank of recognized international standing chartered in the country where such Foreign Subsidiary is domiciled having unimpaired capital and surplus of at least $[**], *provided* such country is a member of the Organization for Economic Cooperation and Development, and such bank maintains a short-term commercial paper rating of at least P-1 or A-1 from either Moody's or S&P, respectively (or, if at any time neither Moody's nor S&P shall be rating such obligations, an equivalent rating from another nationally recognized statistical rating agency selected by the Required Lenders);

(f) (i) Dollars, Euros, Pounds Sterling, Swiss Francs, Canadian dollars or any national currency of any member state of the European Union or (ii) any other foreign currency held by the Borrower or any of its Subsidiaries in the ordinary course of business;

(g) Investments, classified in accordance with GAAP as current assets of the Borrower or any of its Subsidiaries, in money market investment programs registered under the Investment Company Act of 1940, which are administered by financial institutions that have the highest rating obtainable from either Moody's or S&P, and the portfolios of which are limited solely to Investments of the character, quality and maturity described in clauses (a) through (f) of this definition; and

(h) Investments made pursuant to the Borrower Investment Policy.

"*CERCLA*" means the Comprehensive Environmental Response, Compensation and Liability Act of 1980.

"*Change in Law*" means the occurrence, after the Closing Date, of any of the following: (a) the adoption or taking effect of any law, rule, regulation or treaty, (b) any change in any law, rule, regulation or treaty or in the administration, interpretation, implementation or application thereof by any Governmental Authority or (c) the making or issuance of any request, rule, guideline or directive (whether or not having the force of law) by any Governmental Authority; *provided* that notwithstanding anything herein to the contrary, (i) the Dodd-Frank Wall Street Reform and Consumer Protection Act and all requests, rules, guidelines or directives thereunder or issued in connection therewith or in the implementation thereof and (ii) all requests, rules, guidelines or directives promulgated by the Bank for International Settlements, the Basel Committee on Banking Supervision (or any successor or similar authority) or the United States or foreign regulatory authorities, in each case pursuant to Basel III, shall in each case be deemed to be a "Change in Law", regardless of the date enacted, adopted, issued or implemented.

"*Change of Control*" means and shall be deemed to have occurred if any "person" or "group" (within the meaning of Rule 13d-5 of the Exchange Act) shall own, directly or indirectly, beneficially or of record, determined on a fully diluted basis, more than 35% of the Voting Stock of the Borrower.

"*Class*", when used in reference to any Loan or Borrowing, refers to whether such Loan, or the Loans comprising such Borrowing, are Initial Loans or Delayed Draw Loans and when used in reference to any Commitment, refers to whether such Commitment is an Initial Commitment or a Delayed Draw Commitment.

"*Closing Date*" means the date on which the conditions to the funding of the Initial Loans have been satisfied or waived by the Lenders, and the Initial Loans have funded, which date is October 27, 2022.

"*CMS*" means the U.S. Centers for Medicare & Medicaid Services.

"*Code*" means the Internal Revenue Code of 1986.

"*Collateral*" means all of the "Collateral" and "Mortgaged Property" referred to in the Collateral Documents and all of the other property that is or is intended under the terms of the Collateral Documents to be subject to Liens in favor of the Administrative Agent for the benefit of the Secured Parties in order to secure the Obligations. Notwithstanding anything to the contrary, the Collateral shall not include any Excluded Property. For the avoidance of doubt, the Collateral shall include any assets related to the production, distribution, commercialization or exploitation of any IP Collateral or Specified Product and all proceeds thereof.

"*Collateral Documents*" means, collectively, the U.S. Security Agreement, the Irish Security Documents, the Intercompany Note, any Mortgages, any related Mortgaged Property Support Documents, the Qualifying Control Agreements, each Joinder Agreement, each of the collateral assignments, security agreements, pledge agreements, account control agreements or other similar agreements delivered to the Administrative Agent pursuant to Section 6.14, and each of the other agreements, instruments or documents that creates or purports to create a Lien in favor of the Administrative Agent for the benefit of the Secured Parties.

"*Commitment*" means, with respect to a Lender, such Lender's Initial Commitment or Delayed Draw Commitment, as the context may require.

"*Commodity Exchange Act*" means the Commodity Exchange Act (7 U.S.C. § 1 *et seq*.), as amended from time to time, and any successor statute.

"*Companies Act (Ireland)*" means the Companies Act of Ireland 2014, as amended.

"*Competitor*" means any Person that competes with the business of the Borrower and its Subsidiaries from time to time.

"*Competitor Controller*" means any Person (excluding any Bona Fide Debt Fund) that is a direct or indirect holding company of a Competitor or an Affiliate of a Competitor that is controlled by such Competitor.

"*Compliance Certificate*" means a certificate substantially in the form of Exhibit C.

"*Conforming Changes*" means, with respect to either the use or administration of Term SOFR or the use, administration, adoption or implementation of any Benchmark Replacement, any technical, administrative or operational changes (including changes to the definition of "Base Rate", the definition of "Business Day," the definition of "U.S. Government Securities Business Day," the definition of "Interest Period" or any similar or analogous definition (or the addition of a concept of "interest period"), timing and frequency of determining rates and making payments of interest, timing of borrowing requests or prepayment, conversion or continuation notices, the applicability and length of lookback periods, the applicability of Section 3.03 and other technical, administrative or operational matters) that the Administrative Agent (acting in consultation with the Required Lenders) decides may be appropriate to reflect the adoption and implementation of any such rate or to permit the use and administration thereof by the Administrative Agent in a manner substantially consistent with market practice (or, if the Administrative Agent decides that adoption of any portion of such market practice is not administratively feasible or if the Administrative Agent determines that no market practice for the administration of any such rate exists, in such other manner of administration as the Administrative Agent (acting in consultation with the Required Lenders) decides is reasonably necessary in connection with the administration of this Agreement and the other Loan Documents).

"*Connection Income Taxes*" means Other Connection Taxes that are imposed on or measured by net income (however denominated) or that are franchise Taxes or branch profits Taxes.

"*Consolidated*" means, when used with reference to financial statements or financial statement items of the Borrower and its Subsidiaries or any other Person, such statements or items on a consolidated basis in accordance with the consolidation principles of GAAP.

"*Consolidated Total Assets*" means, at any date, total assets of the Loan Parties calculated in accordance with GAAP on a Consolidated basis as of such date, determined on a Pro Forma Basis.

"*Contractual Obligation*" means, as to any Person, any provision of any security issued by such Person or of any enforceable agreement, instrument or other undertaking to which such Person is a party or by which it or any of its property is bound.

"*Control*" means (i) the power of such Person, directly or indirectly, (x) to vote 10% or more of the Voting Stock (determined on a fully diluted basis) of another Person, or (y) to direct or cause the direction of the management and policies of such other Person (whether by contract or otherwise), and/or (ii) the ownership by such Person of 10% or more of the Equity Interests of another Person. "*Controlling*" and "*Controlled*" have meanings correlative thereto.

"*Controlled Investment Affiliate*" shall mean, with respect to any Person, any other Person that (a) directly or indirectly, is in control of, is controlled by, or is under common control with, such Person, and (b) is organized primarily for the purpose of making equity or debt investments in one or more companies.

For purposes of this definition, "control" means the possession, directly or indirectly through one or more intermediaries, of the power to direct the management and policies of a Person, whether through the ownership of Equity Interests, by contract, or otherwise.

"*Convertible Debt*" means Indebtedness issued by the Borrower having a feature which entitles the holder thereof to convert or exchange all or a portion of such Indebtedness into Equity Interests of the Borrower.

"*Copyrights*" means all copyrights, whether statutory or common law, and all exclusive licenses from third parties or rights to use copyrights owned by such third parties, along with any and all (i) renewals, revisions, extensions, derivative works, enhancements, modifications, updates and new releases thereof, (ii) income, royalties, damages, claims and payments now and hereafter due and/or payable with respect thereto, including, without limitation, damages and payments for past, present or future infringements thereof, (iii) rights to sue for past, present and future infringements thereof, and (iv) foreign copyrights and any other rights corresponding thereto throughout the world.

"*Covered Entity*" means any of the following: (a) a "covered entity" as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 252.82(b); (b) a "covered bank" as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 47.3(b); or (c) a "covered FSI" as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 382.2(b).

"*Debtor Relief Laws*" means the Bankruptcy Code of the United States, and all other liquidation, conservatorship, bankruptcy, assignment for the benefit of creditors, moratorium, rearrangement, receivership, insolvency, reorganization, examinership, rescue process or similar debtor relief Laws of the United States or other applicable jurisdictions from time to time in effect.

"*Default*" means any event or condition that constitutes an Event of Default or that, with the giving of any notice, the passage of time, or both, would be an Event of Default.

"*Default Rate*" means (a) with respect to any Obligation for which a rate is specified, a rate per annum equal to two percent (2%) per annum in excess of the rate otherwise applicable thereto and (b) with respect to any Obligation for which a rate is not specified or available, a rate per annum equal to the Base Rate *plus* the Applicable Rate for Base Rate Loans *plus* two percent (2%), in each case, to the fullest extent permitted by Applicable Law.

"*Defaulting Lender*" means, subject to Section 2.12(b), any Lender that (a) has failed to (i) fund all or any portion of its Loans within two (2) Business Days of the date such Loans were required to be funded hereunder unless such Lender notifies the Administrative Agent and the Borrower in writing that such failure is the result of such Lender's determination that one or more conditions precedent to funding (each of which conditions precedent, together with any applicable default, shall be specifically identified in such writing) has not been satisfied, or (ii) pay to the Administrative Agent or any other Lender any other amount required to be paid by it hereunder within two (2) Business Days of the date when due, (b) has notified the Borrower or the Administrative Agent in writing that it does not intend to comply with its funding obligations hereunder, or has made a public statement to that effect (unless such writing or public statement relates to such Lender's obligation to fund a Loan hereunder and states that such position is based on such Lender's determination that a condition precedent to funding (which condition precedent, together with any applicable default, shall be specifically identified in such writing or public statement) cannot be satisfied), (c) has failed, within three (3) Business Days after written request by the Administrative Agent (acting at the direction of the Required Lenders), the Required Lenders or the Borrower, to confirm in writing to the Administrative Agent, the Required Lenders and the Borrower that it will comply with its prospective funding obligations hereunder (*provided* that such Lender shall cease to be a Defaulting Lender pursuant to

this clause (c) upon receipt of such written confirmation by the Administrative Agent, the Required Lenders and the Borrower), or (d) has, or has a direct or indirect parent company that has, (i) become the subject of a proceeding under any Debtor Relief Law, (ii) had appointed for it a receiver, custodian, conservator, trustee, administrator, assignee for the benefit of creditors or similar Person charged with reorganization or liquidation of its business or assets, including the Federal Deposit Insurance Corporation or any other state or federal regulatory authority acting in such a capacity or (iii) become the subject of a Bail-In Action; *provided* that a Lender shall not be a Defaulting Lender solely by virtue of the ownership or acquisition of any Equity Interest in that Lender or any direct or indirect parent company thereof by a Governmental Authority so long as such ownership interest does not result in or provide such Lender with immunity from the jurisdiction of courts within the United States or from the enforcement of judgments or writs of attachment on its assets or permit such Lender (or such Governmental Authority) to reject, repudiate, disavow or disaffirm any contracts or agreements made with such Lender. Any determination by the Administrative Agent (acting at the direction of the Required Lenders) or the Required Lenders that a Lender is a Defaulting Lender under any one or more of clauses (a) through (d) above, and the effective date of such status, shall be conclusive and binding absent manifest error, and such Lender shall be deemed to be a Defaulting Lender (subject to Section 2.12(b)) as of the date established therefor by the Administrative Agent or the Required Lenders in a written notice of such determination, which shall be delivered by the Administrative Agent, the Required Lenders to the Borrower and each other Lender promptly following such determination.

"*Deferred Acquisition Consideration*" means any purchase price adjustments, royalty, earn-out, milestone payments, contingent or other deferred payment payments of a similar nature (including any non-compete payments and consulting payments) made in connection with any Permitted Acquisition or other Acquisition or Investment.

"*Delayed Draw Availability Period*" means the period from and including the Closing Date to the earliest of (i) the Outside Date, (ii) the date of termination of the Delayed Draw Commitments pursuant to Section 2.04(a) or Section 2.04(b), and (iii) the date of termination of the Aggregate Commitments of each Lender pursuant to Section 8.02.

"*Delayed Draw Commitment*" means, as to each Lender, its obligation to make Delayed Draw Loans to the Borrower pursuant to Section 2.01(b) in an aggregate principal amount not to exceed the amount set forth opposite such Lender's name on Schedule 1.01(b) under the caption "Delayed Draw Commitment" or opposite such caption in the Assignment and Assumption pursuant to which such Lender becomes a party hereto, as applicable, as such amount may be adjusted from time to time in accordance with this Agreement. The aggregate Delayed Draw Commitments of all of the Lenders on the Closing Date shall be $150,000,000.

"*Delayed Draw Funding Date*" means the date or dates on which the conditions specified in Section 4.02 are satisfied (or waived in accordance with Section 11.01) and the Delayed Draw Loans are funded in accordance with the terms hereof.

"*Delayed Draw Loans*" has the meaning specified in Section 2.01(b).

"*Designated Jurisdiction*" means any country or territory to the extent that such country or territory is itself the subject of any Sanction.

"*Designated Non-Cash Consideration*" means the fair market value (as determined by the Borrower in good faith) of non-cash consideration received by the Loan Parties and their Subsidiaries in connection with a Disposition pursuant to Section 7.05(f) that is designated as Designated Non-Cash Consideration pursuant to a certificate of a Responsible Officer, setting forth the basis of such valuation

(which amount will be reduced by the amount of cash or Cash Equivalents received in connection with a subsequent sale or conversion of such Designated Non-Cash Consideration to cash or Cash Equivalents).

"*Disposition*" or "*Dispose*" means the sale, transfer, license, lease or other disposition (including any Sale/Leaseback Transaction and any Permitted License) of any property by any Loan Party that constitutes Collateral, including any sale, assignment, transfer or other disposal, with or without recourse, of any notes, accounts receivable, royalties, milestones, other payments or any rights and claims associated therewith.

"*Disputes*" has the meaning specified in Section 5.23(d).

"*Disqualified Equity Interests*" means any Equity Interest that, by its terms (or by the terms of any security or other Equity Interest(s) into which it is convertible or for which it is exchangeable), or upon the happening of any event or condition, (a) matures or is mandatorily redeemable (other than solely for (x) Qualified Equity Interests and (y) cash in lieu of fractional shares), pursuant to a sinking fund obligation or otherwise (except (i) as a result of a change of control, fundamental change, asset sale or (ii) upon the prior repayment of the Loans and all other Obligations that are accrued and payable and the termination of the Aggregate Commitments), (b) is or becomes redeemable at the option of the holder thereof (other than solely for (x) Qualified Equity Interests and (y) cash in lieu of fractional shares), in whole or in part (except (i) as a result of a change of control, fundamental change, asset sale or (ii) upon the prior repayment of the Loans and all other Obligations that are accrued and payable and the termination of the Aggregate Commitments), (c) is or becomes convertible into or exchangeable for (i) Indebtedness or (ii) any other Equity Interests that would constitute Disqualified Equity Interests or (d) provides for the scheduled payments of dividends in cash, in each case of clauses (a) through (d), in each case, prior to the date that is 91 days after the Maturity Date; *provided* that, if such Equity Interests are issued pursuant to any plan for the benefit of any employee, director, manager or consultant of the Borrower or its Subsidiaries or by any such plan to such employee, director, manager or consultant, such Equity Interests shall not constitute Disqualified Equity Interests because it may be required to be repurchased by the Borrower or its Subsidiaries (x) to the extent permitted by Section 7.06(e), in order to satisfy applicable statutory or regulatory obligations or (y) to the extent permitted by Section 7.06(j), as a result of the termination, death or disability of such employee, director, manager or consultant.

"*Disqualified Institution*" means (a) those banks, financial institutions and other persons that have been specified to the Administrative Agent and the Required Lenders by the Borrower in writing at any time prior to the Closing Date, which list described in this clause (a) may be updated from time to time with the prior written consent of the Required Lenders after the Closing Date (such list, the "Disqualified Lender List"), (b) any Competitor or Competitor Controller that has been identified to the Administrative Agent and the Required Lenders by the Borrower in writing at any time prior to the Closing Date (which list may be provided to the Lenders upon their request), which list described in this clause (b) may be updated from time to time upon written notification thereof to the Administrative Agent and the Required Lenders by the Borrower after the Closing Date (such list, the "Competitors List" and together with the Disqualified Lender List, the "Disqualified Institutions List"); *provided* that no addition to the Disqualified Institutions List shall apply retroactively to disqualify any party (i) that has previously acquired an assignment or participation interest or (ii) is party to a permitted pending trade as of the date of identification, and which in any event, such addition shall not become effective until two Business Days after such date identified by name in writing by the Borrower to the Administrative Agent and the Required Lenders and (c) any Person that is an Affiliate of the Persons described in clauses (a) and (b) (excluding Bona Fide Debt Fund) that is (i) identified in writing by the Borrower to the Administrative Agent and the Required Lenders from time to time (which addition shall not apply retroactively to disqualify any party (1) that has previously acquired an assignment or participation interest or (2) is party to a permitted pending trade as of the date of such identification, and which in any event, such addition shall not become effective until two Business Days

after such date identified by name in writing by the Borrower to the Administrative Agent and the Required Lenders) or (ii) readily identifiable as an Affiliate of such Persons solely on the basis of such Person's name. For the avoidance of doubt, no Affiliate of the Blackstone Entities shall be considered a Disqualified Institution.

"*Dollar*" and "*$*" mean lawful money of the United States.

"*Domestic Subsidiary*" means any Subsidiary that is organized under the laws of the United States, any state thereof or the District of Columbia.

"*EEA Financial Institution*" means (a) any credit institution or investment firm established in any EEA Member Country which is subject to the supervision of an EEA Resolution Authority, (b) any entity established in an EEA Member Country which is a parent of an institution described in clause (a) of this definition, or (c) any financial institution established in an EEA Member Country which is a Subsidiary of an institution described in clauses (a) or (b) of this definition and is subject to consolidated supervision with its parent.

"*EEA Member Country*" means any of the member states of the European Union, Iceland, Liechtenstein, and Norway.

"*EEA Resolution Authority*" means any public administrative authority or any Person entrusted with public administrative authority of any EEA Member Country (including any delegee) having responsibility for the resolution of any EEA Financial Institution.

"*Eligible Assignee*" means any Person that meets the requirements to be an assignee under Section 11.06 (subject to such consents, if any, as may be required under Section 11.06(b)(iii)).

"*Emflaza*" means that certain pharmaceutical product of the Borrower known by the non-proprietary name deflazacort, which, as of the Closing Date, is being marketed by Borrower under the trade name Emflaza for the treatment of Duchenne muscular dystrophy.

"*Environment*" means ambient air, indoor air, vapor, surface water, groundwater, drinking water, soil, surface and subsurface strata, and natural resources such as wetland, flora and fauna.

"*Environmental Claim*" means any written notice, claim, demand, litigation, request for information, complaint, citation, summons, investigation, notice of non-compliance or violation, cause of action, consent order, consent decree, or other proceeding by any Governmental Authority or any other Person, arising out of, based on or pursuant to any Environmental Law or related in any way to any actual, alleged or threatened Environmental Liability.

"*Environmental Laws*" means any and all federal, state, local, and foreign statutes, laws (including common law), regulations, standards, ordinances, rules, judgments, orders, decrees, permits, agreements or governmental restrictions relating to pollution or the protection of the Environment or human health (to the extent related to exposure to hazardous materials), including those relating to the handling, use, manufacture, registration, distribution, formulation, packaging, labeling, generation transport, storage, disposal, treatment, Release or threat of Release of or exposure to any Hazardous Materials.

"*Environmental Liability*" means any liability, contingent or otherwise (including any liability for damages, costs of environmental remediation, fines, penalties or indemnities), obligation, responsibility or cost whether based in contract, tort, implied or express warranty, strict liability, criminal or civil statute or common law relating to (a) any violation of, or liability under, Environmental Law, (b) the presence,

generation, use, handling, transportation, storage, treatment, packaging, labelling or disposal of any Hazardous Materials, (c) exposure to any Hazardous Materials, (d) Release of any Hazardous Materials, (e) natural resource damage, or (f) any contract, agreement or other consensual arrangement pursuant to which liability is assumed or imposed with respect to any of the foregoing.

"*Environmental Permit*" means any permit, certification, registration, approval, identification number, license or other authorization required under any Environmental Law.

"*Equity Interests*" means, with respect to any Person, shares of capital stock of (or other ownership or profit interests in) such Person, warrants, options or other rights for the purchase or acquisition from such Person of shares of capital stock of (or other ownership or profit interests in) such Person, securities convertible into or exchangeable for shares of capital stock of (or other ownership or profit interests in) such Person or warrants, rights or options for the purchase or acquisition from such Person of such shares (or such other interests), and other ownership or profit interests in such Person (including partnership, member or trust interests therein), whether voting or nonvoting, and whether or not such shares, warrants, options, rights or other interests are outstanding on any date of determination; *provided* that Convertible Debt that is either the Existing Convertible Debt or that constitutes Permitted Subordinated Debt shall not be considered an "Equity Interest" prior to the conversion of such.

"*Equity Issuance*" means the issuance on the Closing Date to the Lenders or their designees of shares of common stock of the Borrower pursuant to a stock purchase agreement satisfactory to the Borrower and the Lenders.

"*ERISA*" means the Employee Retirement Income Security Act of 1974, as amended, and the rules and regulations promulgated thereunder.

"*ERISA Affiliate*" means any trade or business (whether or not incorporated) under common control with the Borrower within the meaning of Sections 414(b) or (c) of the Code (and Sections 414(m) and (o) of the Code for purposes of provisions relating to Section 412 of the Code).

"*ERISA Event*" means (a) a Reportable Event with respect to a Pension Plan; (b) the withdrawal of the Borrower or any ERISA Affiliate from a Pension Plan subject to Section 4063 of ERISA during a plan year in which such entity was a "substantial employer" as defined in Section 4001(a)(2) of ERISA or a cessation of operations that is treated as such a withdrawal under Section 4062(e) of ERISA; (c) a complete or partial withdrawal by the Borrower or any ERISA Affiliate from a Multiemployer Plan; (d) the filing of a notice of intent to terminate, the treatment of a Pension Plan amendment as a termination under Section 4041 or 4041A of ERISA; (e) the institution by the PBGC of proceedings to terminate a Pension Plan; (f) any event or condition which constitutes grounds under Section 4042 of ERISA for the termination of, or the appointment of a trustee to administer, any Pension Plan; (g) the determination that any Pension Plan is considered an at-risk plan or a plan in endangered or critical status within the meaning of Sections 430, 431 and 432 of the Code or Sections 303, 304 and 305 of ERISA; (h) the imposition of any liability under Title IV of ERISA, other than for PBGC premiums due but not delinquent under Section 4007 of ERISA, upon the Borrower or any ERISA Affiliate or (i) a failure by the Borrower or any ERISA Affiliate to meet all applicable requirements under the Pension Funding Rules in respect of a Pension Plan, whether or not waived, or the failure by the Borrower or any ERISA Affiliate to make any required contribution to a Multiemployer Plan.

"*Erroneous Payment*" has the meaning specified in Section 9.11(a).

"*Erroneous Payment Subrogation Rights*" has the meaning specified in Section 9.11(d).

"*EU Bail-In Legislation Schedule*" means the EU Bail-In Legislation Schedule published by the Loan Market Association (or any successor person), as in effect from time to time.

"*Event of Default*" has the meaning specified in Section 8.01.

"*Excluded Account*" means any of the following: (a) accounts exclusively used for payroll, payroll Taxes and other employee wage and benefit payments to or for the benefit of any Loan Party's employees, which shall in no event hold in the aggregate more than the amount reasonably expected to meet such payroll expenses for the following calendar month, including bonuses and other payments to be paid within the following calendar month, (b) zero balance accounts, (c) accounts (including trust accounts) used exclusively for third party escrow, customs, insurance, deposits or fiduciary purposes, (d) merchant accounts for which the obligations were incurred in the ordinary course of business, (e) accounts used exclusively for compliance with any (i) Applicable Law to the extent such Applicable Law prohibits the granting of a Lien thereon or (ii) Contractual Obligation to the extent such Contractual Obligation prohibits the granting of a Lien thereon (other than Liens in favor of a counterparty to such Contractual Obligation), (f) accounts which are exclusively used to hold cash or Cash Equivalents that serves as collateral in respect of a Permitted Lien, (g) accounts exclusively used for the receipt of receivables solely funded by Medicare or Medicaid and whose total cash balances shall be automatically swept to an account that is not an Excluded Account, (h) other accounts, the cash balance of which such accounts, in the case of this clause (h), does not exceed $[**] in the aggregate at any time, and (i) accounts which hold exclusively the proceeds of Excluded IP Rights and Excluded IP Related Assets.

"*Excluded IP Related Assets*" means any assets related exclusively to the production, distribution, commercialization or exploitation of any Excluded IP Rights and all, to the extent held in an Excluded Account, the proceeds thereof, including, without limitation, all of the following to the extent solely related to Excluded IP Rights (and not related to Specified Product IP) (each of the following capitalized terms is as defined in the Security Agreement): Accounts, Chattel Paper, Commercial Tort Claims, Documents, Equipment, Fixtures, General Intangibles, Goods, Instruments, Inventory, Investment Property, Letter of Credit Rights and Letters of Credit, Supporting Obligations, and all books, records, writings, databases and information related thereto. For the avoidance of doubt, "Excluded IP Related Assets" shall not include any assets that include, or are commingled with or related to, any Specified IP Rights or Specified Product or any product, technology or Intellectual Property licensed or otherwise obtained by a Loan Party in connection with an Acquisition.

"*Excluded IP Rights*" means all IP Rights of the Loan Parties and their Subsidiaries (i) as of the Closing Date, other than the Specified Product IP and (ii) those IP Rights internally developed by a Loan Party or its Subsidiaries following the Closing Date that do not constitute Specified Product IP or derive from or relate to, in whole or in part, any Specified Product, any Specified Product IP or any product, technology, asset or Intellectual Property licensed or otherwise obtained by Borrower or any of its Subsidiaries in connection with an Acquisition (including any Permitted Acquisition).

"*Excluded Property*" means, with respect to any Loan Party (a) any leased or subleased real property, (b) any owned real property which is not Material Real Property, (c) Excluded IP Rights and Excluded IP Related Assets, (d) motor vehicles and other assets subject to certificates of title, (e) assets for which a pledge thereof or a security interest therein is prohibited by Applicable Laws (including any Sanctions) after giving effect to the applicable anti-assignment provisions of the UCC and other Applicable law (including any requirement to obtain the consent of any Governmental Authority or third person, unless such consent has been obtained) other than to the extent that any such prohibition would be rendered ineffective pursuant to the UCC or any other Applicable Law (in each case, other than with regard to Sanctions), (f) any lease, license or other agreements, or any goods or other property subject to a purchase money security interest, Finance Lease or similar arrangements, in each case to the extent permitted under

the Loan Documents, to the extent that a pledge thereof or a security interest therein would violate or invalidate such lease, license or agreement, purchase money, Finance Lease or similar arrangement, or create a right of termination in favor of any other party thereto (other than the Borrower and its Subsidiaries) after giving effect to the applicable anti-assignment clauses of the UCC, Applicable Laws or principles of equity, other than the proceeds thereof the assignment of which is expressly deemed effective under Applicable Laws notwithstanding such prohibition, (g) any intent-to-use United States trademark application for which neither (i) an amendment to allege use to bring the application into conformity with 15 U.S.C. §1051(c) has been filed with and accepted by the USPTO nor (ii) a verified statement of use under 15 U.S.C. §1051(d) has been filed with and accepted by the USPTO, (h) letter of credit rights, (i) the [**] and any Patents licensed to [**] thereunder and the [**], unless and until a security interest in the [**] is granted by the Borrower to the Administrative Agent and the Lenders pursuant to Section 6.20, (j) assets for which a pledge thereof or a security interest therein is prohibited by any contract binding on such assets, or would result in material adverse financial, tax, regulatory or accounting consequences, as reasonably determined in good faith by the Borrower; *provided*, that (i) any such limitation described in this clause (j) shall only apply to the extent that any such prohibition could not be rendered ineffective pursuant to the UCC or any other Applicable Law or principles of equity and shall not apply to any proceeds or receivables thereof, the assignment of which is expressly deemed effective under the UCC notwithstanding such prohibition and (ii) in the event of the termination or elimination of any such prohibition contained in the UCC or any Applicable Law, a security interest in such assets shall be automatically and simultaneously granted under the applicable Collateral Documents and shall be included as Collateral, (k) any governmental licenses (but not the proceeds thereof) or state or local franchises, charters and authorizations, to the extent security interests in favor of the Administrative Agent in such licenses, franchises, charters or authorizations are prohibited or restricted thereby; *provided* that (i) any such limitation described in this clause (k) on the security interests granted shall only apply to the extent that any such prohibition or restriction could not be rendered ineffective pursuant to the UCC of any applicable jurisdiction or any other Applicable Law or principles of equity and shall not apply to any proceeds or receivables thereof, the assignment of which is expressly deemed effective under the UCC notwithstanding such prohibition and (ii) in the event of the termination or elimination of any such prohibition or restriction contained in any applicable license, franchise, charter or authorization, a security interest in such licenses, franchises, charters or authorizations shall be automatically and simultaneously granted under the applicable Collateral Documents and such licenses, franchises, charters or authorizations shall be included as Collateral, (l) Equity Interests in any Person (other than the Borrower and its wholly owned Subsidiaries) to the extent and for so long as the pledge thereof in favor of the Administrative Agent is not permitted by the terms of such Person's joint venture agreement or other applicable Organization Documents; *provided*, that such prohibition exists on the Closing Date or at the time such Equity Interests are acquired (so long as such prohibition did not arise in contemplation of the Closing Date or such acquisition), (m) margin stock within the meaning of Section 5.13, (n) Excluded Accounts, (o) those assets as to which the Required Lenders and the Borrower reasonably agree that the cost of obtaining such a security interest or perfection thereof are excessive in relation to the benefit to the Lenders of the security to be afforded thereby, (p) Equity Interests owned by any Loan Party in Agilis GTR Japan Inc., in Immaterial Subsidiaries (to the extent that the Liens securing the Obligations cannot be perfected through the filing of a Uniform Commercial Code financing statement, it being understood that no other perfection requirements shall be applicable for Immaterial Subsidiaries), or in any Subsidiary that exclusively owns Excluded IP Rights and Excluded IP Related Assets, and (q) the Equity Interests of any Foreign Subsidiary or Foreign Subsidiary Holdco of any Loan Party to the extent a pledge of such Equity Interests would cause actual or anticipated material adverse tax consequences to any Loan Party (in the Borrower's reasonable determination from time to time); *provided* that the Collateral shall at all times include (and "Excluded Property" shall not include) (i) all proceeds arising from the sale or transfer of any of the foregoing Excluded Property to the extent such proceeds do not otherwise constitute Excluded Property except that the proceeds of any Excluded IP or Excluded IP Related Assets shall, to the extent held in an Excluded Account pursuant to clause (i) of the definition

thereof, remain "Excluded Property", and (ii) except with respect to clause (c), (e) and (i) above, any of the following items solely to the extent such items pertain to, arise from, are collected on, are distributed on account of or are given in exchange for or in settlement of any Collateral: (1) Accounts, (2) Chattel Paper (including Electronic Chattel Paper and Tangible Chattel Paper), (3) Commercial Tort Claims, (4) Documents, (5) General Intangibles, (6) Instruments, (7) Letter-of-Credit Rights and (8) insurance and insurance claims (it being understood that the foregoing items (1) through (7) shall have the meanings set forth in the UCC).

"*Excluded Subsidiary*" means any of the following, (a) each Immaterial Subsidiary, (b) any (i) non-wholly owned Subsidiary or (ii) Foreign Subsidiary or Foreign Subsidiary Holdco (to the extent the Guarantee of the Obligations by such Foreign Subsidiary or Foreign Subsidiary Holdco would cause actual or anticipated material adverse tax consequences to any Loan Party (in the Borrower's reasonable determination from time to time)), in each case, that is formed, capitalized or acquired solely through an Investment permitted pursuant to Sections 7.03(h), (u)(i) or (v), and (c) any other Subsidiary acquired or formed after the Closing Date with respect to which the Required Lenders and the Borrower reasonably agree that the cost or other consequences (including adverse tax consequences and adverse regulatory consequences) of providing a Guarantee of or granting Liens to secure the Obligations are likely to be excessive in relation to the value to be afforded thereby. Notwithstanding the foregoing, no Subsidiary which holds any IP Collateral or which holds any Equity Interests or other Investment (other than intercompany loans) in any Subsidiary of the Borrower which holds IP Collateral shall be an Excluded Subsidiary.

"*Excluded Taxes*" means any of the following Taxes imposed on or with respect to any Recipient or required to be withheld or deducted from a payment to a Recipient, (a) Taxes imposed on or measured by net income (however denominated), franchise Taxes, and branch profits Taxes, in each case, (i) imposed as a result of such Recipient being organized or incorporated under the laws of, or having its principal office or, in the case of any Lender, its Lending Office located in, the jurisdiction imposing such Tax (or any political subdivision thereof) or (ii) that are Other Connection Taxes, (b) in the case of a Lender, U.S. federal withholding Taxes imposed on amounts payable to or for the account of such Lender with respect to an applicable interest in a Loan or Commitment pursuant to a law in effect on the date on which (i) such Lender acquires such interest in the Loan or Commitment (other than pursuant to an assignment request by the Borrower under Section 11.13) or (ii) such Lender changes its Lending Office, except in each case to the extent that, pursuant to Section 3.01, amounts with respect to such Taxes were payable either to such Lender's assignor immediately before such Lender became a party hereto or to such Lender immediately before it changed its Lending Office, (c) Taxes attributable to such Recipient's failure to comply with Section 3.01(f) and (d) any U.S. federal withholding Taxes imposed pursuant to FATCA.

"*Existing Convertible Debt*" means the convertible senior notes of the Borrower due 2026 in principal amount of $287,500,000, as issued and outstanding as of the Closing Date.

"*Facility Termination Date*" means the date as of which all of the following shall have occurred: (a) the Aggregate Commitments have terminated and (b) all Obligations have been paid in full (other than contingent indemnification obligations).

"*Faes License Agreement"* means the Exclusive License and Supply Agreement dated May 12, 2015 by and between Faes Farma S.A. and Marathon Pharmaceuticals, LLC, including any and all amendments thereto.

"*FASB ASC*" means the Accounting Standards Codification of the Financial Accounting Standards Board.

"*FATCA*" means Sections 1471 through 1474 of the Code, as of the date of this Agreement (or any amended or successor version that is substantively comparable and not materially more onerous to comply with), any current or future regulations or official interpretations thereof and any agreements entered into pursuant to Section 1471(b)(1) of the Code and any fiscal or regulatory legislation, rules or practices adopted pursuant to any intergovernmental agreement, treaty or convention among Governmental Authorities and implementing such Sections of the Code.

"*FDA*" means the U.S. Food and Drug Administration, or any successor agency thereto having substantially the same functions and jurisdiction.

"*Federal Funds Rate*" means, for any day, the rate per annum calculated by the Federal Reserve Bank of New York based on such day's federal funds transactions by depository institutions (as determined in such manner as the Federal Reserve Bank of New York shall set forth on its public website from time to time) and published on the next succeeding Business Day by the Federal Reserve Bank of New York as the federal funds effective rate; *provided* that (a) if such day is not a Business Day, the Federal Funds Rate for such day shall be such rate on such transactions on the next preceding Business Day as so published on the next succeeding Business Day and (b) if no such rate is so published on such next succeeding Business Day, the Federal Funds Rate for such day shall be the average of the quotations (rounded upward, if necessary, to a whole multiple of 1/100 of 1%) for the day of such transactions received by the Administrative Agent from three federal funds brokers of recognized standing selected by it; *provided, further,* that if the Federal Funds Rate as so determined would be less than zero, such rate shall be deemed to be zero for the purposes of this Agreement.

"*Fee Letter*" means that certain Fee Letter, dated as of the date hereof, between the Borrower, the Lenders and the other parties thereto.

"*Finance Lease*" means any lease that has been or is required to be, in accordance with GAAP, recorded, classified and accounted for as a financing lease. For the avoidance of doubt, an operating lease shall not be considered a Finance Lease.

"*Foreign Collateral*" means (x) Collateral located outside the United States and (y) Collateral in respect of which documentation or action under the Laws of a Foreign Jurisdiction is required or reasonably desirable (in the sole discretion of the Administrative Agent, acting at the direction of the Required Lenders) for the attachment and perfection a valid first-priority Lien in such Collateral. For the avoidance of doubt, all (x) Equity Interests issued by any Foreign Subsidiary and (y) all Accounts arising under the Laws of any Foreign Jurisdiction, in each case, other than to the extent constituting Excluded Property, shall constitute "Foreign Collateral" hereunder, except as agreed in writing by the Administrative Agent (acting at the direction of the Required Lenders) in its sole discretion.

"*Foreign Collateral Requirements*" means, in respect of Foreign Collateral:

(i) the Borrower shall have delivered to the Administrative Agent (a) a duly executed security and/or pledge agreement (as applicable) governed by the Laws of jurisdiction in which such Collateral is located or under the Laws of which such Collateral arises or is governed;

(ii) the Borrower shall have delivered to the Administrative Agent evidence of the filing with all necessary Governmental Authorities of financing statements and other registrations of pledge or Lien which may be requested by the Administrative Agent (acting at the direction of the Required Lenders) in its sole discretion;

(iii) if requested by the Administrative Agent (acting at the direction of the Required Lenders), the Borrower shall have delivered to the Administrative Agent and the Lenders an opinion or opinions of reputable counsel to the Borrower (or, if the Administrative Agent shall agree, counsel to the Administrative Agent) licensed to practice in the jurisdiction in which such Collateral is located (or under the Laws of which such Collateral arises or is governed) opining as to the enforceability of the applicable security agreement and other documentation (in the Administrative Agent's discretion, acting at the direction of the Required Lenders), attachment, perfection and priority of the related security interest and any other matters reasonably requested by the Administrative Agent (acting at the direction of the Required Lenders);

(iv) the Borrower shall have delivered to the Administrative Agent (x) the results of lien, litigation, tax, lawsuit and judgment searches with respect to each applicable jurisdiction and such other searches as may be requested by the Administrative Agent in its sole discretion (acting at the direction of the Required Lenders), in form and substance satisfactory to the Administrative Agent and the Required Lenders in their sole discretion; (y) to the extent requested by the Administrative Agent, termination statements (which shall be in a form satisfactory to the Administrative Agent (acting at the direction of the Required Lenders), and, in any event, shall be in a legally effective form suitable for filing in each applicable filing office), with respect to Liens or other encumbrances (other than Permitted Liens); (z) evidence of the filing with all necessary Governmental Authorities of financing statements and other registrations of pledge or Lien which may be requested by the Administrative Agent in its sole discretion (acting at the direction of the Required Lenders);

(v) if requested by the Administrative Agent (acting at the direction of the Required Lenders), (x) with respect to Collateral in the possession of any warehouseman, bailee, or other third party (any such Person, a "*Foreign Collateral Bailee*"), written evidence that the such Foreign Collateral Bailee shall have been notified of and shall have acknowledged in writing the Administrative Agent's first priority Lien in the Collateral held by such Foreign Collateral Bailee, (y)an agreement duly executed by the applicable Foreign Collateral Bailee requiring, among other things, such Foreign Collateral Bailee to comply with directions of the Administrative Agent upon notice from the Administrative Agent (acting at the direction of the Required Lenders) and/or (z) other documentation requested by the Administrative Agent in its sole discretion as may be necessary or advisable to provide a first priority perfected Lien (or the equivalent under local law) in the relevant Collateral in the possession of such Foreign Collateral Bailee;

(vi) the Borrower shall have delivered such other documents, filings or agreements (in form and substance satisfactory to the Administrative Agent, acting at the direction of the Required Lenders) and taken such other actions as reasonably desirable or necessary (in the determination of the Administrative Agent, acting at the direction of the Required Lenders) to grant to the Administrative Agent a valid and enforceable first priority Lien.

"*Foreign Jurisdiction*" means any municipality, state, province, country, other political subdivision or similar designation other than the United States.

"*Foreign Lender*" means a Lender that is not a U.S. Person.

"*Foreign Subsidiary*" means any Subsidiary that is not a Domestic Subsidiary.

"*Foreign Subsidiary Holdco*" means any Domestic Subsidiary that has no material assets other than Equity Interests or Equity Interests and Indebtedness of one or more Foreign Subsidiaries.

"*FRB*" means the Board of Governors of the Federal Reserve System of the United States.

"*Fund*" means any Person (other than a natural Person) that is (or will be) engaged in making, purchasing, holding or otherwise investing in commercial loans and similar extensions of credit in the ordinary course of its activities.

"*GAAP*" means generally accepted accounting principles in the United States set forth from time to time in the opinions and pronouncements of the Accounting Principles Board and the American Institute of Certified Public Accountants and statements and pronouncements of the Financial Accounting Standards Board (or agencies with similar functions of comparable stature and authority within the accounting profession) including, without limitation, the FASB Accounting Standards Codification, that are applicable to the circumstances as of the date of determination, consistently applied and subject to Section 1.03.

"*Governmental Authority*" means (a) the government of the United States or (b) any other nation or jurisdiction, or of any political subdivision thereof, whether state or local, and any agency, authority, instrumentality, regulatory body, court, central bank or other entity exercising executive, legislative, judicial, taxing, regulatory or administrative powers or functions of or pertaining to government (including, without limitation, any supra-national bodies such as the European Union or the European Central Bank).

"*Government Reimbursement Program*" means (a) Medicare, (b) Medicaid, (c) the Federal Employees Health Benefit Program under 5 U.S.C. §§ 8902 et seq., (d) TRICARE, (e) CHAMPVA, (f) any "federal health care program" as defined in 42 U.S.C. §1320a-7b(f), and (g) if applicable within the context of this Agreement, any agent, administrator, administrative contractor, intermediary or carrier for any of the foregoing.

"*Guarantee*" means, as to any Person, (a) any obligation, contingent or otherwise, of such Person guaranteeing or having the economic effect of guaranteeing any Indebtedness of the kind described in clauses (a) through (g) of the definition thereof or other obligation payable or performable by another Person (the "*primary obligor*") in any manner, whether directly or indirectly, and including any obligation of such Person, direct or indirect, (i) to purchase or pay (or advance or supply funds for the purchase or payment of) such Indebtedness or other obligation, (ii) to purchase or lease property, securities or services for the purpose of assuring the obligee in respect of such Indebtedness or other obligation of the payment or performance of such Indebtedness or other obligation, (iii) to maintain working capital, equity capital or any other financial statement condition or liquidity or level of income or cash flow of the primary obligor so as to enable the primary obligor to pay such Indebtedness or other obligation, or (iv) entered into for the purpose of assuring in any other manner the obligee in respect of such Indebtedness or other obligation of the payment or performance thereof or to protect such obligee against loss in respect thereof (in whole or in part), or (b) any Lien on any assets of such Person securing any Indebtedness of the kind described in clauses (a) through (g) of the definition thereof or other obligation of any other Person, whether or not such Indebtedness or other obligation is assumed or expressly undertaken by such Person (or any right, contingent or otherwise, of any holder of such Indebtedness to obtain any such Lien); *provided* that the term "Guarantee" shall not include endorsements for collection or deposit, in either case, in the ordinary course of business, or customary or reasonable indemnity obligations in effect on the Closing Date, or entered into in connection with any acquisition or Disposition of assets permitted under this Agreement (other than such obligations with respect to Indebtedness). The amount of any Guarantee shall be deemed to be an amount equal to the stated or determinable amount of the related primary obligation, or portion thereof, in respect of which such Guarantee is made or, if not stated or determinable, the maximum reasonably anticipated liability in respect thereof as determined by the guaranteeing Person in good faith. The term "*Guarantee*" as a verb has a corresponding meaning.

"*Guaranteed Obligations*" has the meaning set forth in Section 10.01.

"*Guarantors*" means, collectively, the Subsidiaries of the Borrower that are not Excluded Subsidiaries as are or may from time to time become parties to this Agreement pursuant to Section 6.13.

"*Guaranty*" means, collectively, the Guarantee made by the Guarantors under Article X in favor of the Secured Parties, together with each other guaranty delivered pursuant to Section 6.13.

"*GTRI License*" means the Manufacturing and Collaboration Agreement between Gene Therapy Research Institution and Agilis Biotherapeutics, LLC (now PTC Therapeutics GT, Inc.) dated as of July 2017, including any and all amendments thereto ([**]).

"*Hazardous Materials*" means all explosive or radioactive substances or wastes and all hazardous or toxic substances, wastes or other pollutants, including petroleum or petroleum distillates, natural gas, natural gas liquids, asbestos or asbestos-containing materials, polychlorinated biphenyls, per- and polyfluoroalkyl substances, radon gas, toxic mold, infectious or medical wastes and all other substances, wastes, chemicals, pollutants, contaminants or compounds of any nature in any form regulated pursuant to any Environmental Law.

"*Health Care Activities*" means activities involving or related to research, development, manufacture, packaging, labeling, storage, testing, commercialization, import, export, distribution, promotion, marketing, product and patient support, funding by Borrower or its Subsidiaries of third party activities to advance science or promote access to health care, pricing and price reporting, sale, coverage and reimbursement of a pharmaceutical or biological product, including the Specified Products, by Government Reimbursement Programs or Private Programs or related services.

"*Health Care Laws*" means all Laws related to Health Care Activities applicable to the Borrower and each Subsidiary or by which any of their respective properties, operations, products or other assets is bound or affected, including: (a) the Federal Food, Drug, and Cosmetic Act (21 U.S.C. §§ 301 et seq.), (b) the Public Health Service Act (42 U.S.C. §§ 262 et. seq), (c) fraud and abuse Laws such as the federal Anti-kickback Statute (42 U.S.C. § 1320a-7b(b)), the civil False Claims Act (31 U.S.C. §§ 3729 et. seq.), the Civil Monetary Penalties Law, 42 U.S.C. § 1320a-7a, the administrative False Claims Law (42 U.S.C. § 1320a-7b(a)), the exclusion Laws (42 U.S.C. § 1320a-7), the Program Fraud Civil Remedies Act, 31 U.S.C. §§ 3801-3812, the Anti-Kickback Act of 1986, 41 U.S.C. §§ 51-58, and the health care fraud statute, 18 U.S.C. § 1347, the so-called federal "sunshine" law or Open Payments program (42 U.S.C. § 1320a-7h) and analogous state Laws requiring reporting of transfers of value between pharmaceutical manufacturers and members of the health care industry, state Laws regulating interactions between pharmaceutical manufacturers and members of the health care industry, (d) Laws regulating the purchase of any health care product or service by or on behalf of any Governmental Authority or other Person and the pricing, price reporting, discounting or rebating of such health care product or service such as the Medicaid Drug Rebate Program (42 U.S.C. § 1396r-8) and any state supplemental rebate program, the 340B Drug Pricing Program of the Public Health Services Act (42 U.S.C. § 256b), Medicare average sales price reporting (42 U.S.C. § 1395w-3a), , the VA Federal Supply Schedule (38 U.S.C. § 8126) or any applicable state pharmaceutical assistance program agreement or U.S. Department of Veterans Affairs agreement and any successor government programs, (e) Laws related any Government Reimbursement Program, including the Medicare statute (Title XVIII of the Social Security Act), including the Medicare Part D program and the Medicare Advantage program, the Medicaid statute (Title XIX of the Social Security Act, 42 U.S.C. § 1396 et seq.), and the federal TRICARE statute (10 U.S.C. § 1071 et seq.), (f) Laws applicable to the manufacture or distribution of prescription drugs, including state Laws requiring licensure of pharmaceutical manufacturers and distributors; (g) coding, coverage, payment, or reimbursement of a health care product or service by a Government Reimbursement Program or private health care benefit program, (h) HIPAA and other federal, state or local Laws governing the privacy and security of health information or breach of same, (i) state consumer protections Laws, and (j), any and all other federal, state, local or foreign health care Laws

applicable to the Company or any Company Subsidiary or affecting their respective businesses, including for each of the foregoing as amended, modified or supplemented from time to time, and any successor statutes thereto, together with any rules, regulations, policies and guidance promulgated or issued from time to time thereunder.

"*HIPAA*" means the Health Insurance Portability and Accountability Act of 1996 as amended by the Health Information Technology for Economic and Clinical Health Act (Title XIII of the American Recovery and Reinvestment Act of 2009), as the same may be amended, modified, or supplemented from time to time, and any successor statute thereto, and any and all rules or regulations promulgated from time to time thereunder.

"*Immaterial Subsidiary*" means, on any date of determination, any Subsidiary of the Borrower that, together with its Subsidiaries, after eliminating intercompany obligations solely as between such Subsidiary and its Subsidiaries, as of the last day of the most recently ended Measurement Period, generated less than 5.00% of Consolidated revenues and holds less than 5.00% of Consolidated Total Assets of the Borrower and its Subsidiaries; *provided*, *however*, that if at any time there are Subsidiaries which are classified as "Immaterial Subsidiaries" but which collectively, as of the last day of the most recently ended Measurement Period, generated more than 5.00% of Consolidated revenues or held more than 5.00% of Consolidated Total Assets of the Borrower and its Subsidiaries, after eliminating intercompany obligations, then the Borrower shall promptly re-designate one or more of such Subsidiaries as not Immaterial Subsidiaries such that, after such designation hereunder, the Subsidiaries that are designated as Immaterial Subsidiaries did not, as of the last day of the most recently ended Measurement Period, generate more than 5.00% of Consolidated revenues and hold more than 5.00% of Consolidated Total Assets of the Borrower and its Subsidiaries. Notwithstanding the foregoing, no Subsidiary which holds any Specified Product IP or which holds any Equity Interests or other Investment in any Subsidiary of the Borrower which holds Specified Product IP shall be an Immaterial Subsidiary.

"*Indebtedness*" means, as to any Person at a particular time, without duplication, all of the following:

(a) all obligations of such Person for borrowed money and all obligations of such Person evidenced by bonds, debentures, notes, loan agreements or other similar instruments;

(b) all reimbursement obligations of such Person arising under letters of credit (including standby and commercial), bankers' acceptances, bank guaranties, surety bonds and similar instruments (the amount of such obligations being equal at any time to the aggregate then undrawn and unexpired amount of such letters of credit or other instruments plus the aggregate amount of drawings thereunder that have not been reimbursed);

(c) net obligations of such Person under any Swap Contract;

(d) all obligations (including, without limitation, earnout obligations) of such Person to pay the deferred purchase price of property or services (other than (i) trade accounts payable or similar obligations, including accrued expenses owed to a trade creditor incurred in the ordinary course of business, (ii) accruals for payroll and other similar employee liabilities accrued in the ordinary course of business and (iii) royalty or milestone payments not yet due and payable), in each case, to the extent such obligations have become due and payable;

(e) all indebtedness (excluding prepaid interest thereon) secured by a Lien on property owned or being purchased by such Person (including indebtedness arising under conditional sales or other title retention agreements), whether or not such indebtedness shall have been assumed by

such Person or is limited in recourse, but limited to the lesser of the fair market value of such property (as determined in good faith by the Borrower at the date of determination) and the principal amount of such Indebtedness if recourse is solely to such property;

(f) all Attributable Indebtedness in respect of Finance Leases and Synthetic Lease Obligations of such Person and all Synthetic Debt of such Person;

(g) all obligations of such Person in respect of Disqualified Equity Interests or Convertible Debt; and

(h) all Guarantees of such Person in respect of any of the foregoing.

For all purposes hereof, the Indebtedness of any Person shall include the Indebtedness of any partnership or joint venture (other than a joint venture that is itself a corporation or limited liability company) in which such Person is a general partner or a joint venturer, unless such Indebtedness is expressly made non-recourse to such Person. The amount of any net obligation under any Swap Contract on any date shall be deemed to be the Swap Termination Value thereof as of such date.

Notwithstanding anything herein to the contrary, Indebtedness shall not include (i) prepaid or deferred revenue arising in the ordinary course of business, (ii) endorsements of checks or drafts arising in the ordinary course of business, (iii) Equity Interests of the Borrower to the extent not constituting Disqualified Equity Interest, (iv) any obligations in respect of any Permitted Bond Hedge Transaction and any Permitted Warrant Transaction, (v) purchase price adjustments, earn out, contingent or other deferred payment payments of a similar nature (including any non-compete payments and consulting payments) made in connection with any Investment or other acquisitions, in each case, to the extent such obligations have not become due and payable, (vi) non-compete or consulting obligations incurred in connection with Investments or other acquisitions until such obligations become a liability on the balance sheet of such Person in accordance with GAAP, (vii) deferred compensation and severance, pension, health and welfare retirement and equivalent benefits or any deferred obligations incurred under ERISA until such obligations become a liability on the balance sheet of such Person in accordance with GAAP, (viii) deemed Indebtedness pursuant to Accounting Standards Codification 825 or 480 (formerly SFAS Nos. 133 or 150, respectively), (ix) installment payments or the deferred purchase price of property or services to the extent payable solely in Equity Interests (other than Disqualified Equity Interests) of the Borrower, (x) purchase price holdbacks arising in the ordinary course of business in respect of a portion of the purchase price of an asset to satisfy unperformed obligations of the seller of such asset, (xi) notes or loans if (and so long as) proceeds are held in escrow pursuant to customary escrow arrangements pending the release thereof to repay, defease, redeem or satisfy and discharge such notes or loans, and (xii) notes that have been discharged or legally defeased under any indenture or similar agreement pursuant to customary discharge or defeasance provisions and such notes would not be a liability on the balance sheet of such Person in accordance with GAAP.

The amount of Indebtedness of any Person at any date shall be the outstanding balance at such date of all obligations as described above; *provided* that in the case of Indebtedness issued with original issue discount, the amount of such Indebtedness at any time will be the accreted value thereof at such time.

"*Indemnified Taxes*" means all (a) Taxes, other than Excluded Taxes, imposed on or with respect to any payment made by or on account of any obligation of any Loan Party under any Loan Document and (b) to the extent not otherwise described in clause (a), Other Taxes.

"*Indemnitee*" has the meaning specified in Section 11.04(b).

"*Information*" has the meaning specified in Section 11.07(a).

"*Initial Commitment*" means, as to each Lender, its obligation to make Initial Loans to the Borrower pursuant to Section 2.01(a) in an aggregate principal amount not to exceed the amount set forth opposite such Lender's name on Schedule 1.01(b) under the caption "Initial Commitment" or opposite such caption in the Assignment and Assumption pursuant to which such Lender becomes a party hereto, as applicable, as such amount may be adjusted from time to time in accordance with this Agreement. The aggregate Initial Commitments of all of the Lenders on the Closing Date shall be $300,000,000, which shall be funded on the Closing Date.

"*Initial Loans*" has the meaning specified in Section 2.01(a).

"*Intellectual Property*" means all (i) Patents and all patent applications of any type, registrations and renewals, reissues, reexaminations and patent rights in any lawful form thereof; (ii) Trademarks and all applications, registrations and renewals thereof; (iii) Copyrights and other works of authorship (registered or unregistered), and all applications, registrations and renewals therefor; (iv) computer software, databases, data and documentation; (v) trade secrets and confidential business information, whether patentable or unpatentable and whether or not reduced to practice, know-how, inventions, manufacturing processes and techniques, research and development information, data and other information, including any such information included in or supporting Key Permits; (vi) proprietary financial, marketing and business data, pricing and cost information, business, finance and marketing plans, customer and prospective customer lists and information, and supplier and prospective supplier lists and information; (vii) other intellectual property or similar proprietary rights; (viii) copies and tangible embodiments of any of the foregoing (in whatever form or medium); and (ix) any and all improvements, developments, refinements, additions or subtractions to any of the foregoing.

"*Intercompany Debt*" has the meaning specified in Section 7.02(d).

"*Intercompany Note*" shall mean that certain Intercompany Note, dated as of the Closing Date, by and among the Borrower and each Subsidiary of the Borrower.

"*Interest Payment Date*" means, (a) as to any SOFR Loan, the last day of each Interest Period applicable to such Loan and the Maturity Date; *provided*, *however*, that if any Interest Period for a SOFR Loan exceeds three (3) months, the respective dates that fall every three (3) months after the beginning of such Interest Period shall also be Interest Payment Dates; and (b) as to any Base Rate Loan, the last Business Day of each March, June, September and December and the Maturity Date.

"*Interest Period*" means, with respect to each SOFR Loan, a period commencing on the date of the making of such Term SOFR Loan (or the continuation of a SOFR Loan or the conversion of a Reference Rate Loan to a SOFR Loan) and ending, one month, three months or six months thereafter; provided, however, that (a) if any Interest Period would end on a day that is not a Business Day, such Interest Period shall be extended (subject to clauses (c)-(e) below) to the next succeeding Business Day, (b) interest shall accrue at the applicable rate based upon the Term SOFR Reference Rate from and including the first day of each Interest Period to, but excluding, the day on which any Interest Period expires, (c) any Interest Period that would end on a day that is not a Business Day shall be extended to the next succeeding Business Day unless such Business Day falls in another calendar month, in which case such Interest Period shall end on the next preceding Business Day, (d) with respect to an Interest Period that begins on the last Business Day of a calendar month (or on a day for which there is no numerically corresponding day in the calendar month at the end of such Interest Period), the Interest Period shall end on the last Business Day of the calendar month that is one, three or six months after the date on which the Interest Period began, as applicable, and (e) the Borrower may not elect an Interest Period which will end after the Maturity Date.

"*Investment*" means, as to any Person, any direct or indirect acquisition or investment by such Person, whether by means of (a) the purchase or other acquisition of Equity Interests of another Person, (b) a loan, advance or capital contribution to, Guarantee or assumption of debt of, or purchase or other acquisition of any other debt or interest in, another Person (including any partnership or joint venture interest in such other Person and any arrangement pursuant to which the investor guaranties Indebtedness of such other Person); *provided*, *however* that endorsements of negotiable instruments and documents in the ordinary course of business or consistent with past practice will not be deemed to be an Investment, or (c) the purchase or other acquisition (in one transaction or a series of transactions) of assets of another Person which constitute all or substantially all of the assets of such Person or of a division, line of business or other business unit of such Person. For purposes of covenant compliance, the amount of any Investment shall be (i) the amount actually invested, without adjustment for subsequent increases or decreases in the value of such Investment, minus (ii) (x) the amount of dividends or distributions actually received in connection with such Investment and any return of capital and any payment of principal received in respect of such Investment that in each case is received in cash or Cash Equivalents (not in excess of the amount of Investments originally made) and (y) any cancellation of any Investment in the form of a Guarantee (not in excess of the amount of Investment originally made). If the Borrower or any Subsidiary issues, sells or otherwise Disposes of Equity Interests of a Person that is a Subsidiary, such that after giving effect thereto such Person is no longer a Subsidiary, any Investment by the Borrower or any Subsidiary in such Person remaining after giving effect thereto will be deemed to be a new Investment at such time.

"*Involuntary Disposition*" means any loss of, damage to or destruction of, or any condemnation or other taking for public use of, any property of any Loan Party that constitutes Collateral.

"*IP Collateral*" means all IP Rights (including, for the avoidance of doubt, the Specified Product IP) other than Excluded IP Rights.

"*IP Rights*" means, collectively, any Owned Intellectual Property and/or any Licensed Intellectual Property.

"*Irish Loan Party*" means PTC Therapeutics International Limited (company no. 545830) and any other Loan Party from time to time incorporated under the laws of Ireland.

"*Irish Security Documents*" means any Collateral Document which is governed by the laws of Ireland entered into at any time on or after the Closing Date by any Loan Party in favor of the Administrative Agent for the benefit of the Secured Parties.

"*IRS*" means the United States Internal Revenue Service.

"*Joinder Agreement*" means a joinder agreement substantially in the form of Exhibit D executed and delivered in accordance with the provisions of Section 6.13.

"*Joint Ventures*" means (a) any Person which would constitute an "equity method investee" of the Borrower or any of the Subsidiaries and (b) any Person in whom the Borrower or any of the Subsidiaries beneficially owns any Equity Interest that is not a Subsidiary.

"*Key Contracts*" means (a) the [**], (b) the RPI Royalty Purchase Agreement, (c) the Shiratori License Agreement, (d) the Faes License Agreement, (e) the NTU License, (f) the GTRI License, (g) any contracts, agreements or licenses (including any contracts with a Governmental Authority) involving payments in excess of $[**] in any fiscal year, (h) any contract with a Governmental Authority to the extent the termination or breach thereof would reasonably be expected to materially and adversely affect the ability of a Loan Party to operate, commercialize or distribute in any jurisdiction that is material to the business of

the Borrower and its Subsidiaries, taken as a whole, and (i) all other contracts, agreements or licenses, that the early termination, cancellation, loss, abandonment or other disposition of which, individually or in the aggregate, would reasonably be expected to result in a Material Adverse Effect, in each case, as amended, supplemented or otherwise modified from time to time.

"*Key Permits*" means all Permits (a) relating to the Specified Products, and (b) necessary or required under the Health Care Laws, including without limitation, any Investigational New Drug applications, New Drugs Applications, drug establishment registrations and listings, orphan drug designations or approvals, and other designations, submissions, applications, or approvals.

"*Knowledge*" or "*knowledge*" shall mean and refer to (i) the actual knowledge of a Responsible Officer of any Loan Party or (ii) the knowledge that such Responsible Officer would have obtained if such officer had engaged in good faith and diligent performance of such officer's duties. For the avoidance of doubt, "know", "known" and "knew," words, or phrases of similar import, relating to the knowledge or the awareness of any Loan Party used in this Agreement or any other Loan Document, shall have the respective correlative meaning thereto.

"*Laws*" means, collectively, all supranational, foreign, federal, state, local, provincial, municipal or other constitution, treaties, rules, legally binding regulatory policy statement, statute, law (including common law), ordinance, regulation, rule, code or similar requirement of law enacted, adopted, issued or promulgated by any Governmental Authority charged with the enforcement, interpretation or administration thereof, and all applicable administrative orders, directed duties, requests, licenses, authorizations and permits of, and agreements with, any Governmental Authority, in each case whether or not having the force of law.

"*Lender*" means each of the Persons identified as a "Lender" on the signature pages hereto, each other Person that becomes a "Lender" in accordance with this Agreement and, their permitted successors and assigns in accordance with this Agreement.

"*Lender Notice*" has the meaning specified in Section 11.21.

"*Lending Office*" means, as to the Administrative Agent or any Lender, the office or offices of such Person described as such in such Person's Administrative Questionnaire, or such other office or offices as such Person may from time to time notify the Borrower and the Administrative Agent; which office may include any Affiliate of such Person or any domestic or foreign branch of such Person or such Affiliate.

"*Licensed Intellectual Property*" means all Intellectual Property that is not Owned Intellectual Property, which is licensed to, or otherwise used or held for use, by the Borrower or any Subsidiary.

"*Lien*" means any mortgage, pledge, hypothecation, assignment, deposit arrangement, encumbrance, lien (statutory or otherwise), charge, or preference, priority or other security interest or preferential arrangement in the nature of a security interest of any kind or nature whatsoever (including any conditional sale or other title retention agreement, any easement, right of way or other encumbrance on title to real property and any financing lease having substantially the same economic effect as any of the foregoing).

"*Limited Condition Transaction*" means any Acquisition or similar Investment of or in any assets, business or Person permitted by this Agreement that the Borrower or one or more of its Subsidiaries is contractually committed to consummate (it being understood that such commitment may be subject to conditions precedent, which conditions precedent may be amended, satisfied or waived in accordance with

the terms of the applicable agreement) and the consummation of which is not conditioned on the availability of, or on obtaining, third party financing.

"*Liquidity*" means, as of any date of determination, the aggregate amount of unrestricted cash and Cash Equivalents of the Loan Parties as of such date maintained in accounts (other than the Reserve Account) in the United States that are not subject to any Liens other than Liens securing the Obligations, Liens permitted by Section 7.01(c) and bankers' liens.

"*Liquidity Increase Date*" shall mean [**].

"*Loan*" means an extension of credit by a Lender to the Borrower under Article II.

"*Loan Documents*" means, collectively, (a) this Agreement, (b) the Notes, (c) the Collateral Documents, (d) each Joinder Agreement, (e) the Fee Letter, (f) the Agency Fee Letter and (g) all other certificates, agreements, documents and instruments executed and delivered, in each case, by or on behalf of any Loan Party pursuant to the foregoing and any amendments, modifications or supplements thereto or to any other Loan Document or waivers hereof or to any other Loan Document; *provided*, *however*, that for purposes of Section 11.01, "Loan Documents" shall mean this Agreement and the Collateral Documents.

"*Loan Notice*" means a notice of (a) a Borrowing, (b) a conversion of Loans from one Type to the other, or (c) a continuation of SOFR Loans, pursuant to Section 2.02(a), which, in each case, shall be substantially in the form of Exhibit E or such other form as may be approved by the Administrative Agent (including any form on an electronic platform or electronic transmission system as shall be approved by the Administrative Agent), appropriately completed and signed by a Responsible Officer of the Borrower.

"*Loan Parties*" means, collectively, the Borrower and each Guarantor.

"*Mandatory Prepayment*" has the meaning set forth in Section 2.03(b)(i).

"*Master Agreement*" has the meaning set forth in the definition of "Swap Contract."

"*Material Adverse Effect*" means a material adverse effect on (a) the operations, business, assets, or financial condition of the Borrower and its Subsidiaries taken as a whole; or (b (i) the ability of the Loan Parties, taken as a whole, to perform their payment Obligations under the Loan Documents to which they are a party, (ii) the legality, validity, binding effect or enforceability against the Loan Parties of this Agreement or of the other Loan Documents to which they are a party, taken as whole or (iii) the rights and remedies of the Administrative Agent and the Lenders under any Loan Documents (other than due to the action or inaction of the Administrative Agent, the Lenders or any other Secured Party).

"*Material Impact*" means any of the following: (a) a Material Adverse Effect, or (b) a material adverse effect on the value of any Specified Product, the Collateral, or the Specified Product IP, in each case, taken as a whole; *provided*, that solely for purposes of Section 8.01(m), (1) a material adverse effect under the foregoing clause (b) shall arise [**].

"*Material Real Property*" means any fee-owned real property that is owned by any Loan Party with a fair market value in excess of $[**], as reasonably estimated by the Borrower in good faith.

"*Maturity Date*" means the date that is seven years from the Closing Date; *provided*, *however*, that if such date is not a Business Day, the Maturity Date shall be the next preceding Business Day.

"*Measurement Period*" means, at any date of determination, the most recently completed four (4) fiscal quarters of the Borrower for which financial statements have been delivered, or were required to have been delivered, pursuant Section 6.01.

"*Moody's*" means Moody's Investors Service, Inc. and any successor thereto.

"*Mortgage*" or "*Mortgages*" means, individually and collectively, as the context requires, each of the fee mortgages, deeds of trust and deeds executed by a Loan Party that purport to grant a Lien to the Administrative Agent (or a trustee for the benefit of the Administrative Agent) for the benefit of the Secured Parties in any Mortgaged Properties, in form and substance reasonably satisfactory to the Administrative Agent and the Required Lenders.

"*Mortgaged Property*" means any Material Real Property of a Loan Party listed on Schedule 1.01(c) and, thereafter, shall include each other Material Real Property with respect to which a Mortgage is granted pursuant to Section 6.14(b).

"*Mortgaged Property Support Documents*" means, with respect to any real property subject to a Mortgage, the deliveries and documents described in Section 6.14(b) and Section 6.18(b).

"*Multiemployer Plan*" means any employee benefit plan of the type described in Section 4001(a)(3) of ERISA, to which the Borrower or any ERISA Affiliate makes or is obligated to make contributions, or during the preceding five (5) plan years, has made or been obligated to make contributions.

"*Multiple Employer Plan*" means a Plan which has two or more contributing sponsors (including the Borrower or any ERISA Affiliate) at least two of whom are not under common control, as such a plan is described in Section 4064 of ERISA.

"*Net Cash Proceeds*" means the aggregate cash or Cash Equivalents proceeds received by the Borrower or any Subsidiary in respect of any Disposition, Debt Issuance or Involuntary Disposition (excluding, for the avoidance of doubt, any such amounts held in escrow), net of (a) fees, costs and expenses incurred in connection therewith (including, without limitation, legal, accounting, recording, consultant, and investment banking fees and sales commissions), (b) taxes paid or reasonably estimated to be payable in connection therewith and any repatriation costs associated with receipt or distribution by the applicable taxpayer of such proceeds, (c) in the case of any Disposition or any Involuntary Disposition, the amount necessary to retire any Indebtedness secured by a Lien on the assets subject to such Disposition or Involuntary Disposition other than any Indebtedness with a Lien ranking *pari passu* with or junior to the Lien securing the Obligations, together with any applicable premiums, penalties, interest or breakage costs, (d) any reserve for adjustment in respect of (x) the sale price of the property that is the subject of such Disposition established in accordance with GAAP and (y) any liabilities associated with such property and retained by any Loan Party after such Disposition, including pension and other post-employment benefit liabilities and liabilities related to environmental matters or against any indemnification obligations associated with such transaction and (e) in the case of any Disposition or Involuntary Disposition by a Subsidiary that is a joint venture or other Subsidiary that is not a wholly owned Subsidiary, the pro rata portion of the Net Cash Proceeds thereof (calculated without regard to this clause (e)) attributable to the minority interests and not available for distribution to or for the account of the Borrower or a wholly owned Subsidiary as a result thereof, and it being understood that "Net Cash Proceeds" shall include, without limitation, (i) any cash or Cash Equivalents received upon the Disposition of any non-cash consideration received by the Borrower or any Subsidiary in any such Disposition or Involuntary Disposition and (ii) upon the reversal (without the satisfaction of any applicable liabilities in cash in a corresponding amount) of any reserve described in clause (d).

"*Non-Consenting Lender*" means any Lender that does not approve any consent, waiver or amendment that (a) requires the approval of all Lenders or all affected Lenders in accordance with the terms of Section 11.01 and (b) has been approved by the Required Lenders.

"*Non-Defaulting Lender*" means, at any time, each Lender that is not a Defaulting Lender at such time.

"*Note*" means a promissory note made by the Borrower in favor of a Lender evidencing Loans made by such Lender, substantially in the form of Exhibit G.

"*Notice of Loan Prepayment*" means a notice of prepayment with respect to a Loan, which shall be substantially in the form of Exhibit J or such other form as may be approved by the Administrative Agent (including any form on an electronic platform or electronic transmission system as shall be approved by the Administrative Agent), appropriately completed and signed by a Responsible Officer.

"*NPL*" means the National Priorities List under CERCLA.

"*NTU License*" means the License and Technology Transfer Agreement dated December 23, 2015, by and between National Taiwan University, Professor Wuh-Liang (Paul) Hwu, and Agilis Biotherapeutics, LLC, including any and all amendments thereto.

"*Obligations*" means (a) all advances to, and debts, liabilities, obligations, covenants and duties of, any Loan Party arising under any Loan Document and (b) all reasonable and documented out-of-pocket costs and expenses incurred in connection with enforcement and collection of the foregoing, including the fees, charges and disbursements of counsel, in each case whether direct or indirect (including those acquired by assumption), absolute or contingent, due or to become due, now existing or hereafter arising and including interest, expenses and fees that accrue after the commencement by or against any Loan Party or any Affiliate thereof pursuant to any proceeding under any Debtor Relief Laws naming such Person as the debtor in such proceeding, regardless of whether such interest, expenses and fees are allowed claims in such proceeding. Without limiting the foregoing, the Obligations include Erroneous Payment Subrogation Rights.

"*OFAC*" means the Office of Foreign Assets Control of the United States Department of the Treasury.

"*Offer Notice*" has the meaning specified in Section 11.21.

"*Officer's Certificate*" means a certificate substantially the form of Exhibit H or any other form approved by the Required Lenders.

"*Organization Documents*" means, (a) with respect to any corporation, the charter or certificate or articles of incorporation and the bylaws (or equivalent or comparable constitutive documents with respect to any non-U.S. jurisdiction); (b) with respect to any limited liability company, the certificate or articles of formation or organization and operating agreement or limited liability company agreement (or equivalent or comparable documents with respect to any non-U.S. jurisdiction); (c) with respect to any partnership, joint venture, trust or other form of business entity, the partnership, joint venture or other applicable agreement of formation or organization (or equivalent or comparable documents with respect to any non-U.S. jurisdiction) and (d) with respect to all entities, any agreement, instrument, filing or notice with respect thereto filed in connection with its formation or organization with the applicable Governmental Authority in the jurisdiction of its formation or organization (or equivalent or comparable documents with respect to any non-U.S. jurisdiction).

"*Other Administrative Proceeding*" means any administrative proceeding relating to a dispute involving a patent office or other relevant intellectual property registry which relates to validity, opposition, revocation, ownership or enforceability of the relevant Intellectual Property.

"*Other Connection Taxes*" means, with respect to any Recipient, Taxes imposed as a result of a present or former connection between such Recipient and the jurisdiction imposing such Tax (other than connections arising from such Recipient having executed, delivered, become a party to, performed its obligations under, received payments under, received or perfected a security interest under, engaged in any other transaction pursuant to or enforced any Loan Document, or sold or assigned an interest in any Loan or Loan Document).

"*Other Taxes*" means all present or future stamp, court or documentary, intangible, recording, filing or similar Taxes that arise from any payment made under, from the execution, delivery, performance, enforcement or registration of, from the receipt or perfection of a security interest under, or otherwise with respect to, any Loan Document, except any such Taxes that are Other Connection Taxes imposed with respect to an assignment (other than an assignment made pursuant to Section 3.06).

"*Outside Date*" means April 27, 2024.

"*Outstanding Amount*" means, on any date, the aggregate outstanding principal amount of Loans after giving effect to any Borrowings and prepayments or repayments of Loans occurring on such date.

"*Owned Intellectual Property*" means all Intellectual Property that is owned or purported to be owned (solely or jointly with others) by the Borrower or any Subsidiary.

"*Participant*" has the meaning specified in Section 11.06(d).

"*Participant Register*" has the meaning specified in Section 11.06(d).

"*Patent*" means any patent, any type of patent application or invention disclosure, including all divisions, continuations, continuations in-part, provisionals, continued prosecution applications, substitutions, reissues, reexaminations, inter partes review, post-grant review by any Governmental Authority, renewals, extensions, adjustments, restorations, supplemental protection certificates and patent rights in any form and other additions in connection therewith, whether in or related to the United States or any foreign country or other jurisdiction.

"*Patriot Act*" has the meaning specified in Section 11.19.

"*PBGC*" means the Pension Benefit Guaranty Corporation.

"*Pension Funding Rules*" means the rules of the Code and ERISA regarding minimum funding standards with respect to Pension Plans and set forth in Sections 412, 430, 431, 432 and 436 of the Code and Sections 302, 303, 304 and 305 of ERISA.

"*Pension Plan*" means any employee pension benefit plan (including a Multiple Employer Plan or a Multiemployer Plan) that is maintained or is contributed to by the Borrower and any ERISA Affiliate or with respect to which the Borrower or any ERISA Affiliate has any liability and is either covered by Title IV of ERISA or is subject to the minimum funding standards under Section 412 of the Code.

"*Permits*" means any permit, approval, clearance, authorization, license, certificate, certification, concession, grant, franchise, variance, submission, notification, registration, amendment, supplement,

exemption or permission obtained from or submitted to any Governmental Authority, including any Key Permit or Environmental Permit.

"*Permitted Acquisition*" means an Acquisition as to which the following conditions have been satisfied:

(a) immediately before and after giving effect to such Acquisition, no Event of Default shall then exist or would exist after giving effect thereto; provided, that, in the case of an Acquisition that constitutes a Limited Condition Transaction, such condition shall be deemed satisfied so long as (i) no Event of Default is continuing as of the date the applicable definitive agreement in respect of such Limited Condition Transaction is entered into and (ii) no Event of Default is continuing as of the date such Limited Condition Transaction is consummated;

(b) before and after giving effect to the Acquisition on a Pro Forma Basis, the Loan Parties are in compliance with the Liquidity covenant set forth in Section 7.11; and

(c) (i) the assets acquired shall be acquired by a Loan Party (except for any R&D License by a non-Loan Party), (ii) if an entity or entities are acquired, such entities shall become Loan Parties in accordance with the timing and documentation requirements of Section 6.13 and shall, unless constituting Excluded Property (subject to clause (iii) below), be pledged as Collateral pursuant to documentation, and with accompanying legal opinions or other deliverables or process, which confers a first ranking Lien on such assets, subject to Permitted Liens, in form and scope customary for the applicable jurisdiction and type of assets, and (iii) no Acquisition of assets constituting property described in clauses (c), (i), (l), (p) or (q) of the definition of Excluded Property shall be permitted pursuant to this definition.

"*Permitted Bond Hedge Transaction*" means any call or capped call option (or substantively equivalent derivative transaction) on the Borrower's Equity Interests purchased by the Borrower in connection with the issuance of any Permitted Subordinated Debt that is Convertible Debt; *provided* that the premium paid by the Loan Parties for such Permitted Bond Hedge Transaction, less the proceeds received by the Loan Parties from the sale of any related Permitted Warrant Transaction, does not exceed the net proceeds received by the Borrower from the sale of such Permitted Subordinated Debt that is Convertible Debt issued in connection with the Permitted Bond Hedge Transaction.

"*Permitted License*" means (a) any license or sublicense (whether non-exclusive or exclusive) or covenant not to sue that is in existence on the Closing Date and listed on Schedule 1.01(d); (b) as to Intellectual Property other than the Specified Product IP, any license or sublicense (whether non-exclusive or exclusive) or covenant not to sue; or (c) as to Specified Product IP, any license or sublicense (whether non-exclusive or exclusive) or covenant not to sue: (i) entered into in the ordinary course of business, solely to facilitate the development, manufacture, production, commercialization (including commercial sales to end users), marketing, promotion, co-promotion, sales or distribution (the "*Permitted Activities*") for any Specified Products on behalf of any Loan Party, or (ii) entered into for Permitted Activities and (A) solely between Loan Parties or between a Loan Party and a Permitted Licensee; (B) to the extent that the Specified Product IP includes PTC923, the license, sublicense, or covenant not to sue relates solely to the Permitted Activities for PTC923 outside of the PTC923 Major Markets; or (iii) to the extent that the Specified Product IP does not include PTC923, the license, sublicense, or covenant not to sue relates solely to Permitted Activities for Specified Product outside of the United States.

"*Permitted Licensee*" means any internationally recognized pharmaceutical or life sciences company with (i) market capitalization at the time any Permitted License is entered into in excess of $[**],

and (ii) significant experience in the development and commercialization of treatment for rare diseases in the jurisdiction of the Permitted License.

"*Permitted Liens*" has the meaning set forth in Section 7.01.

"*Permitted Subordinated Debt*" means Indebtedness issued by the Borrower (which may include, for the avoidance of doubt, Convertible Debt) that: (i) is unsecured and contractually subordinated to the Loans pursuant to documentation satisfactory to the Required Lenders, (ii) is not guaranteed by any Subsidiary of the Borrower, (iii) does not include any financial maintenance covenants and only includes covenants and defaults that are customary for public market convertible indebtedness (pursuant to a public offering or an offering under Rule 144A or Regulation S of the Securities Act), as determined by the Borrower in its good faith judgment, and (iv) does not require cash payments prior to, or have a scheduled maturity date earlier than, October 27, 2030, other than cash interest payments in an aggregate amount per fiscal year not to exceed $[**] less any payments made on Existing Convertible Debt in such fiscal year; provided, that at the time of the issuance of such Indebtedness (x) no Event of Default shall have occurred and be continuing or would result therefrom, and (y) the Borrower shall have delivered to the Administrative Agent a certificate of a Responsible Officer of the Borrower certifying to the satisfaction of all of the foregoing requirements.

"*Permitted Warrant Transaction*" means any call option, warrant or contractual right to purchase (or substantively equivalent derivative transaction) Borrower's Equity Interests sold by the Borrower substantially concurrently with any purchase by the Borrower of a related Permitted Bond Hedge Transaction.

"*Person*" means any natural person, corporation, limited liability company, trust, joint venture, association, company, partnership, fund, account, Governmental Authority or other entity.

"*Phase 3 Positive Readout*" means with respect to the Phase 3 trial for PTC923 (NCT05099640), the achievement of the primary endpoint in a statistically significant manner using the proposed primary analysis as defined in the final statistical analysis plan wherein the data from such trial demonstrates the safety and efficacy of PTC923 using the 60mg/kg dosing regimen and is reasonably likely to support PKU Regulatory Approval.

"*PKU*" means Phenylketonuria.

"*PKU Regulatory Approval*" means approval (whether accelerated or otherwise) from the FDA of a new drug application for PTC923 for the treatment of PKU on or prior to January 1, 2026.

"*Plan*" means any employee benefit plan within the meaning of Section 3(3) of ERISA (including a Pension Plan), maintained for employees of the Borrower or any ERISA Affiliate or any such Plan to which the Borrower or any ERISA Affiliate is required to contribute on behalf of any of its employees.

"*Platform*" has the meaning specified in Section 6.02.

"*Pledged Equity*" has the meaning specified in the U.S. Security Agreement and any other Equity Interests, certificates, options or rights of any nature whatsoever in respect of the equity interests of any Person that may be issued or granted to, or held by, any Loan Party and pledged pursuant to any Collateral Document.

"*Prime Rate*" means, for any day, a rate per annum equal to the rate last quoted by The Wall Street Journal as the "base rate on corporate loans posted by at least 70% of the nation's largest banks" in the

United States or, if The Wall Street Journal ceases to quote such rate, the highest per annum interest rate published by the Federal Reserve Board in Federal Reserve Statistical Release H.15 (519) (Selected Interest Rates) as the "bank prime loan" rate or, if such rate is no longer quoted therein, any similar rate quoted therein (as reasonably determined by the Administrative Agent (which determination shall be conclusive absent manifest error)) or any similar release by the Federal Reserve Board (as reasonably determined by the Administrative Agent (which determination shall be conclusive absent manifest error)).

"*Private Programs*" means all third party payors, including, without limitation, managed care organizations, insurance companies, self-insured health plans, health maintenance organizations and preferred provider organizations, excluding Government Reimbursement Programs.

"*Pro Forma Basis*" means, with respect to the calculation of Consolidated Total Assets or Liquidity, that such calculation shall give pro forma effect to all payments (or receipts, excluding the proceeds of any Delayed Draw Term Loan) from a subject transaction or other proposed action; provided, that such payments with respect to any Permitted Acquisitions shall exclude payments which are calculated as a percentage of net sales or a comparable metric.

"*PTC923*" means that certain pharmaceutical product of the Borrower known by the non-proprietary name sepiapterin, which, as of the Closing Date, is being developed by Borrower for the treatment of phenylketonuria.

"*PTC923 Major Markets*" means [**].

"*Public Lender*" has the meaning specified in Section 6.02.

"*Qualifying Control Agreement*" means an agreement among a Loan Party, a depository institution or securities intermediary and the Administrative Agent, which agreement is in form and substance reasonably acceptable to the Administrative Agent and the Required Lenders and which provides the Administrative Agent with "control" (as such term is used in Article 9 of the UCC) over the deposit account(s) or securities account(s) described therein; provided, that with regard to the Reserve Account, such agreement shall be a "fully blocked account" that provides the Administrative Agent with sole control.

"*R&D License*" means any inbound license entered into in the ordinary course of business for research and development (but not, for the avoidance of doubt, for commercialization) purposes with an acquisition cost of $[**] or less and which is not part of a larger transaction or series of transactions.

"*Recipient*" means the Administrative Agent, any Lender or any other recipient of any payment to be made by or on account of any obligation of any Loan Party hereunder.

"*Register*" has the meaning specified in Section 11.06(c).

"*Regulation U*" means Regulation U of the FRB, as in effect from time to time and all official rulings and interpretations thereunder or thereof.

"*Reinvestment Period*" has the meaning set forth in Section 2.03(b)(i).

"*Related Parties*" means, with respect to any Person, such Person's Affiliates and the partners, directors, officers, employees, investment committee members, accountants, agents, trustees, administrators, managers, advisors, consultants, attorneys, current or prospective investors, advisors, financing or funding sources, service providers and representatives of such Person and of such Person's

Affiliates; provided that "Related Parties" shall not include prospective investors for purposes of Sections 11.04(b), 11.04(c), 11.06(a), or 11.07(a) of this Agreement.

"*Release*" means any actual or threatened release, spill, emission, discharge, deposit, dispersal, disposal, leaking, pumping, pouring, dumping, emptying, placing, injection or leaching or migration into or through the Environment.

"*Reportable Event*" means any of the events set forth in Section 4043(c) of ERISA, other than events for which the thirty (30) day notice period has been waived.

"*Required Lenders*" means, at any time, Lenders having Total Credit Exposures representing more than 50% of the Total Credit Exposures of all Lenders. The Total Credit Exposure of any Defaulting Lender shall be disregarded in determining Required Lenders at any time.

"*Reserve Account*" has the meaning set forth in subpart (a) of Schedule 6.20.

"*Resignation Effective Date*" has the meaning set forth in Section 9.06(a).

"*Resolution Authority*" means an EEA Resolution Authority or, with respect to any UK Financial Institution, a UK Resolution Authority.

"*Responsible Officer*" means the chief executive officer, chief financial officer, chief compliance officer, chief strategy officer, general counsel or controller of a Loan Party (and in the case of any Irish Loan Party, a director or secretary of such Irish Loan Party), and, solely for purposes of the delivery of incumbency certificates pursuant to Section 4.01(b), the secretary or any assistant secretary of a Loan Party and, solely for purposes of notices given pursuant to Article II, any other officer or employee of the applicable Loan Party so designated by any of the foregoing officers in a notice to the Administrative Agent or any other officer or employee of the applicable Loan Party designated in or pursuant to an agreement between the applicable Loan Party, the Administrative Agent and the Required Lenders. Any document delivered hereunder that is signed by a Responsible Officer of a Loan Party shall be conclusively presumed to have been authorized by all necessary corporate, partnership and/or other action on the part of such Loan Party and such Responsible Officer shall be conclusively presumed to have acted on behalf of such Loan Party. To the extent requested by the Administrative Agent or the Required Lenders, each Responsible Officer will provide an incumbency certificate and to the extent requested by the Administrative Agent, appropriate authorization documentation, in form and substance reasonably satisfactory to the Required Lenders.

"*Restricted Payment*" means (a) any dividend or other distribution, direct or indirect, on account of any shares (or equivalent) of any class of Equity Interests of the Borrower or any of its Subsidiaries, now or hereafter outstanding, (b) any redemption, retirement, sinking fund or similar payment, purchase or other acquisition for value, direct or indirect, of any shares (or equivalent) of any class of Equity Interests of the Borrower or any of its Subsidiaries, now or hereafter outstanding, and (c) any payment made to retire, or to obtain the surrender of, any outstanding warrants, options or other rights (in each case, other than Permitted Warrant Transactions) to acquire shares of any class of Equity Interests of any Loan Party or any of its Subsidiaries, now or hereafter outstanding; *provided*, that the issuance of, entry into (including any payments of premiums in connection therewith), performance of obligations under, and conversion, exercise, repurchase, redemption, settlement or early termination or cancellation of (whether in whole or in part and including by netting or set-off) (in each case, whether in cash, common stock of the Borrower or, following a merger event or other change of the common stock of Borrower, other securities or property) any Permitted Subordinated Debt (subject to Section 7.14), or the satisfaction of any condition that would permit or require any of the foregoing.

"*[**]*" means, [**].

"*[**]*" means [**].

"*[**]*" means, [**].

"*[**]*" shall mean [**].

"*[**]*" shall mean [**].

"*[**]*" means [**].

"*[**]*" shall mean [**].

"*[**]*" means [**].

"*RPI Royalty Purchase Agreement*" means that certain Royalty Purchase Agreement, dated as of July 17, 2020, by and among the Borrower, RPI 2019 Intermediate Finance Trust and Royalty Pharma plc.

"*S&P*" means Standard & Poor's Financial Services LLC, a subsidiary of S&P Global Inc., and any successor thereto.

"*Sale/Leaseback Transaction*" means an arrangement relating to property now owned or hereafter acquired by any Loan Party or any Subsidiary whereby any Loan Party or any Subsidiary transfers such property to a Person and any Loan Party or any Subsidiary leases it from such Person, other than leases between Loan Parties and their Subsidiaries.

"*Sanction*(s)" means any sanction administered or enforced by the United States Government (including, without limitation, OFAC), the United Nations Security Council, the European Union, Her Majesty's Treasury ("*HMT*") or other relevant sanctions authority.

"*SEC*" means the Securities and Exchange Commission, or any Governmental Authority succeeding to any of its principal functions.

"*Secured Parties*" means, collectively, the Administrative Agent, the Lenders, the Indemnitees and each co-agent or sub-agent appointed by the Administrative Agent from time to time pursuant to Section 9.05.

"*Securities Act*" means the Securities Act of 1933, including all amendments thereto and regulations promulgated thereunder.

"*SEMS*" means Superfund Enterprise Management System maintained by the U.S. Environmental Protection Agency.

"*Shiratori License Agreement*" means the License Agreement dated February 8, 2015 by and between Shiratori Pharmaceutical Co., Ltd. And Censa Pharmaceuticals Inc., including any and all amendments thereto.

"*SOFR*" means a rate per annum equal to the secured overnight financing rate for such Business Day published by the Federal Reserve Bank of New York (or a successor administrator of the secured overnight financing rate) on the website of the Federal Reserve Bank of New York, currently at

http://www.newyorkfed.org (or any successor source for the secured overnight financing rate identified as such by the administrator of the secured overnight financing rate from time to time).

"*SOFR Loan*" means a Loan that bears interest at a rate based on Adjusted Term SOFR (other than pursuant to clause (c) of the definition of "Base Rate").

"*Solvency Certificate*" means a solvency certificate in substantially in the form of Exhibit F.

"*Solvent*" and "*Solvency*" mean, with respect to any Person on any date of determination, that on such date (a) the aggregate fair value of the property of such Person is greater than the total amount of liabilities, including contingent liabilities, of such Person, (b) the aggregate present fair saleable value of the assets of such Person is not less than the amount that will be required to pay the probable liability of such Person on its debts as they become absolute and matured, (c) such Person does not intend to, and does not believe that it will, incur debts or liabilities beyond such Person's ability to pay such debts and liabilities as they mature, (d) such Person is not engaged in business or a transaction, and is not about to engage in business or a transaction, for which such Person's property would constitute an unreasonably small capital, and (e) such Person is able to pay its debts and liabilities, contingent obligations and other commitments as they mature in the ordinary course of business. The amount of contingent liabilities at any time shall be computed as the amount that, in the light of all the facts and circumstances existing at such time, represents the amount that can reasonably be expected to become an actual or matured liability.

"Specified Key Contracts" means those agreements listed in clauses (a) through (f) of the definition of "Key Contracts", in each case, as amended, supplemented or otherwise modified from time to time.

"*Specified Product IP*" means any and all Patents, Copyrights, Trademarks, trade secrets and other Intellectual Property recognized under applicable Law reasonably related to any Specified Products, in each case, such as that are necessary for, or reasonably useful to, the research, development, manufacture, commercialization, or other exploitation or defense of any Specified Products, in each case, during the term of this Agreement.

"*Specified Products*" means Translarna, Emflaza, Upstaza, PTC923 and, until the Vatiquinone Release Conditions are met, Vatiquinone, in each case, together with any other forms, formulations, or methods of delivery of any such products, and regardless of trade or brand name.

"*Specified Products Business*" has the meaning specified in Section 5.23(a).

"*Stock Purchase Agreement*" means that certain Stock Purchase Agreement, dated as of the Closing Date, by and among the investors listed on Exhibit A thereto and the Borrower.

"*Subsidiary*" of a Person means a corporation, partnership, joint venture, limited liability company or other business entity of which a majority of the shares of Voting Stock is at the time beneficially owned, or the management of which is otherwise controlled, directly, or indirectly through one or more intermediaries, or both, by such Person. Unless otherwise specified, all references herein to a "Subsidiary" or to "Subsidiaries" shall refer to a Subsidiary or Subsidiaries of the Borrower.

"*Swap Contract*" means (a) any and all rate swap transactions, basis swaps, credit derivative transactions, forward rate transactions, commodity swaps, commodity options, forward commodity contracts, equity or equity index swaps or options, bond or bond price or bond index swaps or options or forward bond or forward bond price or forward bond index transactions, interest rate options, forward foreign exchange transactions, cap transactions, floor transactions, collar transactions, currency swap transactions, cross-currency rate swap transactions, currency options, spot contracts, or any other similar

transactions or any combination of any of the foregoing (including any options to enter into any of the foregoing), whether or not any such transaction is governed by or subject to any master agreement, and (b) any and all transactions of any kind, and the related confirmations, which are subject to the terms and conditions of, or governed by, any form of master agreement published by the International Swaps and Derivatives Association, Inc., any International Foreign Exchange Master Agreement, or any other master agreement (any such master agreement, together with any related schedules, a "*Master Agreement*"), including any such obligations or liabilities under any Master Agreement.

"*Swap Obligations*" means any obligation to pay or perform under any agreement, contract or transaction that constitutes a "swap" within the meaning of Section 1a(47) of the Commodity Exchange Act.

"*Swap Termination Value*" means, in respect of any one or more Swap Contracts, after taking into account the effect of any legally enforceable netting agreement relating to such Swap Contracts, (a) for any date on or after the date such Swap Contracts have been closed out and termination value(s) determined in accordance therewith, such termination value(s), and (b) for any date prior to the date referenced in clause (a), the amount(s) determined as the mark-to-market value(s) for such Swap Contracts, as determined based upon one or more mid-market or other readily available quotations provided by any recognized dealer in such Swap Contracts (which may include a Lender or any Affiliate of a Lender).

"*Synthetic Debt*" means, with respect to any Person as of any date of determination thereof, all obligations of such Person in respect of transactions entered into by such Person that are intended to function primarily as a borrowing of funds (including any minority interest transactions that function primarily as a borrowing) but are not otherwise included in the definition of "Indebtedness" or as a liability on the Consolidated balance sheet of such Person and its Subsidiaries in accordance with GAAP.

"*Synthetic Lease Obligation*" means the monetary obligation of a Person under (a) a so-called synthetic, off-balance sheet or tax retention lease, or (b) an agreement for the use or possession of property (including Sale/Leaseback Transactions), in each case, creating obligations that do not appear on the balance sheet of such Person but which, upon the application of any Debtor Relief Laws to such Person, would be characterized as the indebtedness of such Person (without regard to accounting treatment).

"*Taxes*" means all present or future taxes, levies, imposts, duties, deductions, withholdings (including backup withholding), assessments, fees or other charges imposed by any Governmental Authority, including any interest, additions to tax or penalties applicable thereto.

"*Term SOFR*" means the greater of (a) 1.00% per annum or (b) the Term SOFR Reference Rate for a one-month, three-month or six-month tenor, as applicable, on the day (such day, the "*Periodic Term SOFR Determination Day*") that is two (2) Business Days prior to the first day of such Interest Period, as such rate is published by the Term SOFR Administrator; provided, however, that if as of 5:00 p.m. (New York City time) on any Periodic Term SOFR Determination Day the Term SOFR Reference Rate for the applicable tenor has not been published by the Term SOFR Administrator and a Benchmark Replacement Date with respect to the Term SOFR Reference Rate has not occurred, then Term SOFR will be the Term SOFR Reference Rate for such tenor as published by the Term SOFR Administrator on the first preceding Business Day for which such Term SOFR Reference Rate for such tenor was published by the Term SOFR Administrator so long as such first preceding Business Day is not more than three (3) Business Days prior to such Periodic Term SOFR Determination Day.

"*Term SOFR Reference Rate*" means the forward-looking term rate based on SOFR.

"*Term SOFR Adjustment*" means a percentage per annum equal to 0.10% for any Interest Period.

"*Third Party*" means any Person other than the Borrower or any of its Subsidiaries.

"*Threshold Amount*" means $[**].

"*Total Credit Exposure*" means, as to any Lender at any time, the unused Commitments and Outstanding Amount of all Loans of such Lender at such time.

"*Trade Date*" has the meaning specified in Section 11.06(g)(i).

"*Trademark*" means any trademark, service mark, trade name, logo, symbol, trade dress, domain name, corporate name or other indicator of source or origin, and all applications and registrations therefor, together with all of the goodwill associated therewith.

"*Transactions*" means, collectively, the execution and delivery of this Agreement and the other Loan Documents, and the funding of the Initial Loans and the Equity Issuance and the payment of fees and expenses incurred in connection therewith.

"*Translarna*" means that certain pharmaceutical product of the Borrower known by the non-proprietary name ataluren, which, as of the Closing Date, is being marketed by Borrower under the trade name Translarna for the treatment of Duchenne muscular dystrophy due to a nonsense mutation.

"*Treasury Rate*" means, as of any Calculation Date, the yield to maturity as of such Calculation Date of United States Treasury securities with a constant maturity (as compiled and published in the most recent Federal Reserve Statistical Release H.15 (519) that has become publicly available at least two (2) Business Days prior to the prepayment date (or, if such Statistical Release is no longer published, any publicly available source of similar market data)) most nearly equal to the period from the Calculation Date to the applicable Make-Whole Terminal Date.

"*TTM PTC 923 Revenue*" means, with respect to a specified calculation date or test, the aggregate revenues, in accordance with GAAP, of the Borrower and its Subsidiaries from sales of PTC923 for the specified trailing twelve-month period.

"*TTM Special Products Revenue*" means, with respect to a specified calculation date or test, the aggregate revenues, in accordance with GAAP, of the Borrower and its Subsidiaries from sales of Specified Products for the specified trailing twelve-month period.

"*Type*" means, with respect to a Loan, its character as a Base Rate Loan or a SOFR Loan.

"*UCC*" means the Uniform Commercial Code as in effect in the State of New York; *provided* that, if perfection or the effect of perfection or non-perfection or the priority of any security interest in any Collateral is governed by the Uniform Commercial Code as in effect in a jurisdiction other than the State of New York, "*UCC*" means the Uniform Commercial Code as in effect from time to time in such other jurisdiction for purposes of the provisions hereof relating to such perfection, effect of perfection or non-perfection or priority.

"*UK Financial Institution*" means any BRRD Undertaking (as such term is defined under the PRA Rulebook (as amended from time to time) promulgated by the United Kingdom Prudential Regulation Authority) or any person falling within IFPRU 11.6 of the FCA Handbook (as amended from time to time) promulgated by the United Kingdom Financial Conduct Authority, which includes certain credit institutions and investment firms, and certain affiliates of such credit institutions or investment firms.

"*UK Resolution Authority*" means the Bank of England or any other public administrative authority having responsibility for the resolution of any UK Financial Institution.

"*United States*" and "*U.S.*" mean the United States of America.

"*Upstaza*" means that certain gene therapy product of the Borrower known by the non-proprietary name eladocagene exuparvovec, which, as of the Closing Date, is being marketed by Borrower under the trade name Upstaza for the treatment of aromatic L-amino acid decarboxylase deficiency.

"*U.S. Person*" means any Person that is a "United States Person" as defined in Section 7701(a)(30) of the Code.

"*U.S. Security Agreement*" means the pledge and security agreement, dated as of the Closing Date, executed in favor of the Administrative Agent by each of the Loan Parties, as amended, modified, extended, restated, replaced or supplemented from time to time.

"*U.S. Tax Compliance Certificate*" has the meaning specified in Section 3.01(f)(ii)(B)(3).

"*USPTO*" means the United States Patent and Trademark Office.

"*Vatiquinone*" means that certain pharmaceutical product of the Borrower known by the non-proprietary name vatiquinone, which, as of the Closing Date, is being developed by Borrower under the product candidate name PTC-743 for the treatment of Friedreich ataxia and mitochondrial disease associated with seizures.

"*Vatiquinone Regulatory Approval*" means full approval (whether accelerated or otherwise) from the FDA of a new drug application for Vatiquinone for the treatment of either Friedreich ataxia or mitochondrial epilepsy.

"*Vatiquinone Release Conditions*" means the occurrence of both of the following (x) receipt of PKU Regulatory Approval and (y) TTM 923 Revenue with respect to PTC923 for any trailing twelve-month period ending on or before December 31, 2026 is greater than $[**].

"*Voting Stock*" means, with respect to any Person, Equity Interests issued by such Person the holders of which are ordinarily, in the absence of contingencies, entitled to vote for the election of directors (or persons performing similar functions) of such Person, even though the right to so vote has been suspended by the happening of such contingency.

"*Withholding Agent*" means the Borrower and the Administrative Agent.

"*Write-Down and Conversion Powers*" means, (a) with respect to any EEA Resolution Authority, the write-down and conversion powers of such EEA Resolution Authority from time to time under the Bail-In Legislation for the applicable EEA Member Country, which write-down and conversion powers are described in the EU Bail-In Legislation Schedule and (b) with respect to the United Kingdom, any powers of the applicable Resolution Authority under the Bail-In Legislation to cancel, reduce, modify or change the form of a liability of any UK Financial Institution or any contract or instrument under which that liability arises, to convert all or part of that liability into shares, securities or obligations of that person or any other person, to provide that any such contract or instrument is to have effect as if a right had been exercised under it or to suspend any obligation in respect of that liability or any of the powers under that Bail-In Legislation that are related to or ancillary to any of those powers.

1.02 Other Interpretive Provisions.

With reference to this Agreement and each other Loan Document, unless otherwise specified herein or in such other Loan Document:

(a) The definitions of terms herein shall apply equally to the singular and plural forms of the terms defined. Whenever the context may require, any pronoun shall include the corresponding masculine, feminine and neuter forms. The words "include," "includes" and "including" shall be deemed to be followed by the phrase "without limitation." The word "will" shall be construed to have the same meaning and effect as the word "shall." Unless the context requires otherwise, (i) any definition of or reference to any agreement, instrument or other document (including the Loan Documents and any Organization Document) shall be construed as referring to such agreement, instrument or other document as from time to time amended, amended and restated, modified, extended, restated, replaced or supplemented from time to time (subject to any restrictions on such amendments, supplements or modifications set forth herein or in any other Loan Document), (ii) any reference herein to any Person shall be construed to include such Person's successors and assigns, (iii) the words "hereto," "herein," "hereof" and "hereunder," and words of similar import when used in any Loan Document, shall be construed to refer to such Loan Document in its entirety and not to any particular provision thereof, (iv) all references in a Loan Document to Articles, Sections, Preliminary Statements, Exhibits and Schedules shall be construed to refer to Articles and Sections of, and Preliminary Statements, Exhibits and Schedules to, the Loan Document in which such references appear, (v) any reference to any law shall include all statutory and regulatory rules, regulations, orders and provisions consolidating, amending, replacing or interpreting such law and any reference to any law, rule or regulation shall, unless otherwise specified, refer to such law, rule or regulation as amended, modified, extended, restated, replaced or supplemented from time to time, and (vi) the words "asset" and "property" shall be construed to have the same meaning and effect and to refer to any and all tangible and intangible assets and properties, including cash, securities, accounts and contract rights.

(b) In the computation of periods of time from a specified date to a later specified date, the word "from" means "from and including;" the words "to" and "until" each mean "to but excluding;" and the word "through" means "to and including."

(c) Section headings herein and in the other Loan Documents are included for convenience of reference only and shall not affect the interpretation of this Agreement or any other Loan Document.

(d) Any reference herein to a merger, transfer, consolidation, amalgamation, assignment, sale, disposition or transfer, or similar term, shall be deemed to apply to a division of or by a limited liability company, or an allocation of assets to a series of a limited liability company (or the unwinding of such a division or allocation), as if it were a merger, transfer, consolidation, amalgamation, assignment, sale, disposition or transfer, or similar term, as applicable, to, of or with a separate Person. Any division of a limited liability company shall constitute a separate Person hereunder (and each division of any limited liability company that is a Subsidiary, joint venture or any other like term shall also constitute such a Person or entity).

1.03 Accounting Terms.

(a) Generally. All accounting terms not specifically or completely defined herein shall be construed in conformity with, and all financial data (including financial ratios and other financial calculations) required to be submitted pursuant to this Agreement shall be prepared in conformity

with, GAAP applied on a consistent basis, as in effect from time to time, except as otherwise specifically prescribed herein. Notwithstanding the foregoing, for purposes of determining compliance with any covenant contained herein, (i) Indebtedness of the Borrower and its Subsidiaries shall be deemed to be carried at 100% of the outstanding principal amount thereof, and the effects of FASB ASC 825 and FASB ASC 470–20 on financial liabilities shall be disregarded, (ii) obligations of a Person that are or would have been treated as operating leases or capital leases for purposes of GAAP prior to the issuance by the Financial Accounting Standards Board on February 25, 2016 of an Accounting Standards Update (Topic 842) (the "ASU") shall continue to be accounted for as operating leases or capital leases (whether or not such operating lease obligations or capital lease obligations, as applicable, were in effect on such date) notwithstanding the fact that such obligations are required in accordance with the ASU (on a prospective or retroactive basis or otherwise) to be treated as capitalized lease obligations in accordance with GAAP, and (iii) all terms of an accounting or financial nature used herein shall be construed, and all computations of amounts and ratios referred to herein shall be made, without giving effect to any election under FASB ASC Topic 825 "Financial Instruments" (or any other financial accounting standard having a similar result or effect) to value any Indebtedness of the Borrower or any Subsidiary at "fair value", as defined therein. For purposes of determining the amount of any outstanding Indebtedness, no effect shall be given to any election by the Borrower to measure an item of Indebtedness using fair value (as permitted by Financial Accounting Standards Board Accounting Standards Codification 825–10–25 (formerly known as FASB 159) or any similar accounting standard).

(b) Changes in GAAP. If at any time any change in GAAP would affect the computation of any financial ratio or requirement set forth in any Loan Document, and either the Borrower, the Required Lenders or the Required Lenders shall so request, the Required Lenders and the Borrower shall negotiate in good faith to amend such ratio or requirement to preserve the original intent thereof in light of such change in GAAP (subject to the approval of the Required Lenders); *provided* that, until so amended, (i) such ratio or requirement shall continue to be computed in accordance with GAAP prior to such change therein and (ii) the Borrower shall provide to the Administrative Agent (for distribution to the Lenders) financial statements and other documents required under this Agreement or as reasonably requested hereunder setting forth a reconciliation between calculations of such ratio or requirement made before and after giving effect to such change in GAAP.

(c) Consolidation of Variable Interest Entities. All references herein to Consolidated financial statements of the Borrower and its Subsidiaries or to the determination of any amount for the Borrower and its Subsidiaries on a Consolidated basis or any similar reference shall, in each case, be deemed to include each variable interest entity that the Borrower is required to consolidate pursuant to FASB ASC 810 as if such variable interest entity were a Subsidiary as defined herein.

1.04 Rounding.

Any financial ratios required to be maintained by the Borrower pursuant to this Agreement shall be calculated by dividing the appropriate component by the other component, carrying the result to one place more than the number of places by which such ratio is expressed herein and rounding the result up or down to the nearest number (with a rounding-up if there is no nearest number).

1.05 Times of Day.

Unless otherwise specified, all references herein to times of day shall be references to Eastern time (daylight or standard, as applicable).

1.06 UCC Terms.

Terms defined in the UCC in effect on the Closing Date and not otherwise defined herein shall, unless the context otherwise indicates, have the meanings provided by those definitions. Subject to the foregoing, the term "UCC" refers, as of any date of determination, to the UCC then in effect.

1.07 Rates.

The Administrative Agent shall not be under any obligation (i) to monitor, determine or verify the unavailability or cessation of the Term SOFR Reference Rate (or any other applicable benchmark), or whether or when there has occurred, or to give notice to any other party to this Agreement of the occurrence of (except as directed by the Required Lenders), any termination date relating to the Term SOFR Reference Rate, (ii) to select, determine or designate any alternative rate, or other successor or replacement benchmark index, or whether any conditions to the designation of such a rate have been satisfied, (iii) to select, determine or designate any other modifier to any alternative rate or (iv) to determine whether or what alternative rate changes are necessary or advisable, if any, in connection with any of the foregoing. The Administrative Agent shall not be liable for any inability, failure or delay on its part to perform any of its duties set forth in this Agreement as a result of the unavailability of the Term SOFR Reference Rate (or any other applicable benchmark) and absence of a designated replacement benchmark, including as a result of any inability, delay, error or inaccuracy on the part of the Required Lenders in providing any direction, instruction, notice or information with respect thereto required or contemplated by the terms of this Agreement and reasonably required for the performance of such duties by the Administrative Agent. The Administrative Agent does not warrant or accept any responsibility for, and shall not have any liability with respect to, the administration, submission or any other matter related to any alternate replacement index to the Term SOFR Reference Rate, including without limitation, whether the composition or characteristics of any such alternate replacement index to the Term SOFR Reference Rate will be similar to, or produce the same value or economic equivalence of, the Term SOFR Reference Rate or have the same volume or liquidity as did the Term SOFR Reference Rate prior to its discontinuance or unavailability, except for performance or non-performance of its duties and obligations with respect thereto that are expressly set forth herein.

1.08 Limited Condition Transactions.

For purposes of (i) determining compliance with any provision of this Agreement or the other Loan Documents which requires the calculation of any ratio, (ii) determining the accuracy of any representations or warranties or determining whether any Default or Event of Default has occurred or (iii) testing availability under baskets set forth in this Agreement or the other Loan Documents, in each case, in connection with a Limited Condition Transaction, at the option of the Borrower (the Borrower's election to exercise such option in connection with any Limited Condition Transaction, an "LCT Election"), the date of determination of whether any such action is permitted hereunder, shall be deemed to be the date the definitive agreement for such Limited Condition Transaction is entered into (such date, the "LCT Test Date"), and if, on a Pro Forma Basis after giving effect to the Limited Condition Transaction and the other transactions to be entered into in connection therewith (including any incurrence of Indebtedness and the use of proceeds thereof) as if they had occurred at the beginning of the most recent Measurement Period, as applicable, ending prior to the LCT Test Date, the Borrower could have taken such action on the relevant LCT Test Date in compliance with such ratio or basket, such ratio or basket shall be deemed to have been complied with. If the Borrower has made an LCT Election for any Limited Condition Transaction, then in connection with any subsequent calculation of any ratio or basket on or following the relevant LCT Test Date and prior to the earlier of (i) the date on which such Limited Condition Transaction is consummated, and (ii) the date that the definitive agreement for such Limited Condition Transaction is terminated or expires without consummation of such Limited Condition Transaction, any such ratio or basket shall be

calculated and tested on a Pro Forma Basis assuming such Limited Condition Transaction and other transactions in connection therewith have been consummated until such time as the applicable Limited Condition Transaction has actually closed or the definitive agreement for such Limited Condition Transaction has been terminated. For the avoidance of doubt, if any of such ratios or amounts are exceeded as a result of changes in such ratio or amount, at or prior to the consummation of the relevant transaction or action, such ratios will not be deemed to have been exceeded as a result of such changes solely for purposes of determining whether the relevant transaction or action is permitted to be consummated or taken.

1.09 **Irish Terms**.

(a) Dissolution of an Irish Loan Party includes such entity being struck off the Register of Companies in Ireland.

(b) Enforcing (or any derivation) the Collateral includes the appointment of an administrator, examiner or process advisor (or any analogous officer in any jurisdiction) of an Irish Loan Party by the Administrative Agent.

(c) An examiner means an examiner (including any interim examiner) appointed under section 509 of the Companies Act (Ireland) and examinership shall be construed accordingly.

(d) Process advisor means a person appointed or acting as a process advisor within the meaning of section 558A(1) of the Companies Act (Ireland).

(e) A rescue process means the rescue process for small and micro companies contemplated by Part 10A of the Companies Act (Ireland).

(f) A person being unable to pay its debts includes that person being unable to pay its debts within the meaning of Sections 509(3) and 570 of the Companies Act (Ireland).

ARTICLE II

COMMITMENTS AND BORROWINGS

2.01 Loans.

(a) Initial Loans. Subject to the terms and conditions set forth herein, each Lender severally agrees to make on the Closing Date Loans to the Borrower, in Dollars, in an amount equal to its Initial Commitment (the "*Initial Loans*"). The funding of the Initial Loans shall consist of Loans made simultaneously by the Lenders in accordance with their respective Applicable Percentage of the Initial Commitments. The Initial Commitments shall be reduced by the funding of the Initial Loans, and amounts borrowed under this Section 2.01(a) and repaid or prepaid may not be reborrowed.

(b) Delayed Draw Borrowings. Subject to the terms and conditions set forth herein, each Lender severally agrees to make one or more Loans to the Borrower, in Dollars, on any Business Day during the Delayed Draw Availability Period in an amount not to exceed its remaining Delayed Draw Commitment as of the applicable Delayed Draw Funding Date ("*Delayed Draw Loans*"); provided, that there shall be no more than 3 separate Delayed Draw Funding Dates, and each funding of Delayed Draw Loans shall be in an aggregate amount of not less than $50,000,000 or the then-remaining amount of the Delayed Draw Commitments. The Delayed Draw Loans shall consist of Loans made simultaneously by the Lenders in accordance with their

respective Applicable Percentage of the Delayed Draw Commitments as of the applicable Delayed Draw Funding Date. The Delayed Draw Commitments shall be reduced by each funding of Delayed Draw Loans, and amounts borrowed under this Section 2.01(b) and repaid or prepaid may not be reborrowed.

2.02 Borrowings, Conversions and Continuations of Loans.

(a) Notice of Borrowing. Each Borrowing, each conversion of Loans from one Type to the other, and each continuation of SOFR Loans shall be made upon the Borrower's irrevocable notice to the Administrative Agent, which notice shall be given by a Loan Notice. Each such Loan Notice must be received by the Administrative Agent not later than 11:00 a.m. (x) ten (10) Business Days prior to the requested date of any Borrowing and (y) three (3) Business Days prior to the requested date of any conversion to or continuation of SOFR Loans or of any conversion of SOFR Loans to Base Rate Loans (in each case, or such shorter period as may be agreed by the Administrative Agent and the Required Lenders). Each Borrowing of, conversion to or continuation of SOFR Loans shall be in a principal amount of $5,000,000 or a whole multiple of $1,000,000 in excess thereof (or, in connection with any conversion or continuation of a Loan, if less, the entire principal thereof then outstanding). Each Borrowing of or conversion to Base Rate Loans shall be in a principal amount of $500,000 or a whole multiple of $100,000 in excess thereof (or, in connection with any conversion or continuation of a Loan, if less, the entire principal thereof then outstanding). Each Loan Notice shall specify (I) whether the Borrower is requesting a Borrowing, a conversion of Loans from one Type to the other, or a continuation of Loans, as the case may be (and the Class of Loans to which such Borrowing, conversion or continuation applies), (II) the requested date of the Borrowing, conversion or continuation, as the case may be (which shall be a Business Day), (III) the principal amount of Loans to be borrowed, converted or continued, (IV) the Type of Loans to be borrowed or to which existing Loans are to be converted or the SOFR Loans to be continued, (V) if applicable, the duration of the Interest Period with respect thereto, and (VI) in the case of a Borrowing, the wiring information of the Borrower's account to which funds are to be disbursed. If the Borrower fails to specify a Type of Loan in a Loan Notice or if the Borrower fails to give a timely notice requesting a conversion or continuation, then the applicable Loans shall be made as, or converted to, Base Rate Loans. Any such automatic conversion to Base Rate Loans shall be effective as of the last day of the Interest Period then in effect with respect to the applicable SOFR Loans. If the Borrower requests a Borrowing of, conversion to, or continuation of SOFR Loans in any such Loan Notice, but fails to specify an Interest Period, it will be deemed to have specified an Interest Period of one (1) month.

(b) Advances. Following receipt of a Loan Notice for a Borrowing, the Administrative Agent shall promptly notify each Lender of the amount of its Applicable Percentage of the applicable Loans, and if no timely notice of a conversion or continuation is provided by the Borrower, the Administrative Agent shall notify each Lender of the details of any automatic conversion to Base Rate Loans described in Section 2.02(a). In the case of a Borrowing, each Lender shall make the amount of its Loan available to the Administrative Agent in immediately available funds to the Administrative Agent's Account not later than 12:00 p.m. on the Business Day specified in the applicable Loan Notice. Upon satisfaction of the applicable conditions set forth in (x) with respect to the Initial Loans, Section 4.01, and (y) with respect to the Delayed Draw Loans, Section 4.02, the Administrative Agent shall make all funds so received available to the Borrower in like funds as received by the Administrative Agent by wire transfer of such funds to the account of the Borrower specified in the applicable Loan Notice.

(c) SOFR Loans. Except as otherwise provided herein, a SOFR Loan may be continued or converted only on the last day of an Interest Period for such SOFR Loan. During the

existence of an Event of Default, no Loans may be requested as, converted to or continued as SOFR Loans without the consent of the Required Lenders, and the Required Lenders may demand that any or all of the outstanding SOFR Loans be converted immediately to Base Rate Loans.

(d) Interest Rates. Each determination of an interest rate by the Administrative Agent pursuant to any provision of this Agreement shall be conclusive and binding on the Borrower and the Lenders in the absence of manifest error.

(e) Interest Periods. After giving effect to all Borrowings, all conversions of Loans from one Type to the other, and all continuations of Loans as the same Type, there shall not be more than five Interest Periods in effect.

(f) Cashless Settlement Mechanism. Notwithstanding anything to the contrary in this Agreement, any Lender may exchange, continue or rollover all or the portion of its Loans in connection with any refinancing, extension, loan modification or similar transaction permitted by the terms of this Agreement, pursuant to a cashless settlement mechanism approved by the Borrower, the Administrative Agent and such Lender.

2.03 Prepayments.

(a) Optional.

(i) The Borrower may, by delivery to the Administrative Agent of a Notice of Loan Prepayment, at any time or from time to time voluntarily prepay Loans in whole or in part subject to payment of the Applicable Premium, and to Section 3.05, but otherwise without any other premium or penalty; *provided* that, unless otherwise agreed by the Administrative Agent, (A) such notice must be received by the Administrative Agent not later than 11:00 a.m. three (3) Business Days prior to any date of prepayment of any Loans; and (B) any prepayment of Loans shall be in a principal amount of $5,000,000 or a whole multiple of $1,000,000 in excess thereof or, in each case, if less, the entire principal amount thereof then outstanding. Each such notice shall specify the date and amount of such prepayment and the Type(s) and Class of Loans to be prepaid and, if SOFR Loans are to be prepaid, the Interest Period(s) of such Loans. The Administrative Agent will promptly notify each Lender of its receipt of each such notice, and of the amount of such Lender's ratable portion of such prepayment. If such notice is given by the Borrower, the Borrower shall make such prepayment and the payment amount specified in such notice shall be due and payable on the date specified therein; *provided* that the Notice of Loan Prepayment may state that such notice is conditioned upon the effectiveness of other credit facilities, indentures or similar agreements or other transactions, in which case such notice may be revoked or postponed by the Borrower (by written notice to the Administrative Agent on or prior to the specified effective date) if such condition is not satisfied.

(b) Mandatory.

(i) Dispositions and Involuntary Dispositions. The Borrower shall apply 100% of the Net Cash Proceeds received by the Loan Parties from Dispositions of Collateral pursuant to Section 7.05(f), (g) and (h) and Involuntary Dispositions of Collateral to the prepayment of the Loans, within ten (10) Business Days of the receipt of such Net Cash Proceeds; provided, that the first $[**] in the aggregate of such Net Cash Proceeds shall be excluded from this prepayment requirement.

(ii) Debt Issuance. Within one (1) Business Day of the receipt by the Borrower or any Subsidiary of Net Cash Proceeds of any issuance of Indebtedness not permitted by Section 7.02, the Borrower shall prepay the Loans in an aggregate amount equal to 100% of such Net Cash Proceeds.

(c) Prepayments. Each prepayment of Loans pursuant to the provisions of this Section 2.03 shall be applied first to the Initial Loans and then to Delayed Draw Loans in direct order of the funding date of such Delayed Draw Loans. All prepayments under this Section 2.03 shall be accompanied by the Applicable Premium and any amounts required under Section 3.05, and shall be accompanied by accrued and unpaid interest on the principal amount prepaid. The Applicable Premium shall be fully earned, and due and payable, on the date of the applicable prepayment, or, if earlier, on the date such prepayment is required to be made, and shall be non-refundable. The Loan Parties acknowledge and agree that the Applicable Premium is not intended to act as a penalty or to punish the Loan Parties for any such prepayment or amendment.

(d) Option to Decline. Any mandatory prepayment may be declined in whole or in part by any Lender without prejudice to such Lender's rights hereunder to accept or decline any future payments in respect of any Mandatory Prepayments, by providing notice to Administrative Agent no later than 11:00 a.m. (New York, NY time) one (1) Business Day (or such other date acceptable to Administrative Agent) prior to the date of such prepayment. Each such notice from a Lender shall specify the principal amount of the Mandatory Prepayment to be rejected by such Lender. If a Lender fails to deliver such notice to Administrative Agent within the time frame specified above or such notice fails to specify the principal amount of the Mandatory Prepayment to be rejected, any such failure will be deemed an acceptance of the total amount of such Mandatory Prepayment. If a Lender chooses not to accept payment in respect of a Mandatory Prepayment in whole or in part on or before the date otherwise due hereunder, such declined proceeds shall be retained by the Loan Parties.

(e) Notice of Prepayments. The Borrower shall notify the Administrative Agent in writing of any prepayment pursuant to Section 2.03 in the form of Notice of Loan Prepayment not later than 11:00 a.m. three (3) Business Days before the date of such prepayment. Each such notice shall specify the prepayment date, the amount of the prepayment and the Loans being prepaid and shall contain a reasonably detailed calculation of the amount of such prepayment. Promptly following receipt of any such notice, the Administrative Agent shall advise the Lenders of the contents thereof.

Notwithstanding anything to the contrary in this Agreement or any other Loan Document, it is understood and agreed that if the Obligations are accelerated as a result of the occurrence of any Event of Default (including by operation of law or otherwise), the Applicable Premium will also be due and payable and shall constitute part of the Obligations for all purposes herein. The Applicable Premium shall also be payable in the event the Obligations (and/or this Agreement) are satisfied or released by foreclosure (whether by power of judicial proceeding), deed in lieu of foreclosure or by any other means. THE LOAN PARTIES EXPRESSLY WAIVE THE PROVISIONS OF ANY PRESENT OR FUTURE STATUTE OR LAW THAT PROHIBITS OR MAY PROHIBIT, THE ACCRUAL OR COLLECTION OF THE APPLICABLE PREMIUM. The Loan Parties expressly agree that (i) the Applicable Premium is reasonable and is the product of an arm's length transaction between sophisticated business people, ably represented by counsel, (ii) the Applicable Premium shall be payable notwithstanding the then prevailing market interest rates at the time payment is made, (iii) there has been a course of conduct between the Lenders and the Loan Parties giving specific consideration in this transaction for such agreement to pay the Applicable Premium, (iv) the Loan Parties shall be estopped hereafter from claiming differently than as agreed to in this paragraph, (v) the Loan Parties' agreement to pay the Applicable Premium is a material inducement to

the Lenders to make the Loans, and (vi) the Applicable Premium represents a good faith, reasonable estimate and calculation of the lost profits or damages of the Lenders, not a penalty, and that it would be impractical and extremely difficult to ascertain the actual amount of damages to the Lenders or profits lost by the Lenders.

2.04 Termination or Reduction of Commitments.

(a) Optional. On any date prior to the expiration of the Delayed Draw Availability Period, the Borrower may elect to permanently reduce all or a portion of the undrawn Delayed Draw Commitments upon notice to the Administrative Agent and the Required Lenders; *provided*, that any such notice shall be received by the Administrative Agent and the Required Lenders no later than 11:00 a.m. three (3) Business Days prior to the date of reduction; *provided further* that any such notice may state that such notice is conditioned upon the effectiveness of other credit facilities, indentures or similar agreements or other transactions, in which case such notice may be revoked or postponed by the Borrower (by written notice to the Administrative Agent and the Required Lenders on or prior to the specified effective date) if such condition is not satisfied.

(b) Mandatory.

(i) The aggregate Initial Commitments shall be automatically and permanently reduced to zero on the Closing Date following the funding of the Initial Loans.

(ii) The aggregate Delayed Draw Commitments shall be automatically and permanently reduced by each funding of Delayed Draw Loans.

(c) Outside Date. If the conditions in Section 4.02 have not been satisfied as of the Outside Date (unless extended by the Required Lenders and the Borrower in their sole discretion and the Administrative Agent has been notified in writing by the Borrower or the Required Lenders of such extension), any portion of the Delayed Draw Commitments that remain outstanding shall be automatically terminated.

2.05 Maturity.

On the Maturity Date, the Borrower shall pay to the Administrative Agent, for the ratable account of the Lenders, the outstanding principal amount of all Loans together with all interest accrued and unpaid thereon, and all other amounts owing or payable hereunder, including the Applicable Premium, if any, with respect to the Delayed Draw Loans.

2.06 Interest and Default Rate.

(a) Interest. Subject to the provisions of Section 2.06(b), the unpaid principal amount of e Loan shall bear interest at a rate per annum equal to the Applicable Rate, in each case from the date of the Borrowing thereof until maturity (whether by acceleration or otherwise).

(b) Default Rate. While any Event of Default exists, unless waived by the Required Lenders, all Obligations shall accrue at a rate per annum equal to the Default Rate. Accrued and unpaid interest on past due amounts (including interest on past due interest) shall be due and payable on each subsequent Interest Payment Date or otherwise promptly upon demand.

(c) Interest Payments. Interest on each Loan shall be due and payable in arrears on each Interest Payment Date applicable thereto and at such other times as may be specified herein.

Interest hereunder shall be due and payable in accordance with the terms hereof before and after judgment, and before and after the commencement of any proceeding under any Debtor Relief Law.

2.07 Fees.

(a) Agency Fee Letter. The Borrower shall pay to the Administrative Agent, for its own account, fees in the amounts and at the times specified in the Agency Fee Letter.

(b) Fee Letter. The Borrower shall pay to the Lenders, for their own account, fees in the amounts and at the times specified in the Fee Letter.

2.08 Computation of Interest and Fees.

All computations of interest for Base Rate Loans (including Base Rate Loans determined by reference to Adjusted Term SOFR) shall be made on the basis of a year of 365 or 366 days, as the case may be, and actual days elapsed. All other computations of fees and interest shall be made on the basis of a three hundred sixty (360) day year and actual days elapsed (which results in more fees or interest, as applicable, being paid than if computed on the basis of a 365 day year). Interest shall accrue on each Loan for the day on which the Loan is made, and shall not accrue on a Loan, or any portion thereof, for the day on which the Loan or such portion is paid, *provided* that any Loan that is repaid on the same day on which it is made shall, subject to Section 2.10(a), bear interest for one (1) day. Each determination by the Administrative Agent of an interest rate or fee hereunder shall be conclusive and binding for all purposes, absent manifest error. The Administrative Agent shall, at the request of the Borrower, deliver to the Borrower a statement showing the methodology used by the Administrative Agent in determining any interest rate hereunder.

2.09 Evidence of Debt.

The Loans made by each Lender shall be evidenced by one or more accounts or records maintained by such Lender in the ordinary course of business. The Administrative Agent shall maintain the Register in accordance with Section 11.06(c). The accounts or records maintained by each Lender shall be conclusive absent manifest error of the amount of the Loans made by the Lenders to the Borrower and the interest and payments thereon. Any failure to so record or any error in doing so shall not, however, limit or otherwise affect the obligation of the Borrower hereunder to pay any amount owing with respect to the Obligations. In the event of any conflict between the accounts and records maintained by any Lender and the Register, the Register shall control in the absence of manifest error. Upon the request of any Lender to the Borrower, the Borrower shall execute and deliver to such Lender a Note, which shall evidence such Lender's Loans in addition to such accounts or records. Each Lender may attach schedules to its Note and endorse thereon the date, Type (if applicable), amount and maturity of its Loans and payments with respect thereto.

2.10 Payments Generally; Administrative Agent's Clawback.

(a) General. All payments to be made by the Borrower shall be made free and clear of and without condition or deduction for any counterclaim, defense, recoupment or setoff. Except as otherwise expressly provided herein, all payments by the Borrower hereunder shall be made to the Administrative Agent, for the account of the respective Lenders to which such payment is owed, to the Administrative Agent's Account in Dollars and in immediately available funds not later than 12:00 p.m. on the date specified herein. The Administrative Agent will promptly distribute to each Lender its Applicable Percentage (or other applicable share as provided herein) of such payment in like funds as received by wire transfer to such Lender's Lending Office. All payments received by the Administrative Agent after 12:00 p.m. shall, in the discretion of the Administrative Agent, be deemed received on the next succeeding Business Day and any applicable interest or fee shall

continue to accrue. Except as otherwise specifically provided for in this Agreement, if any payment to be made by the Borrower shall come due on a day other than a Business Day, payment shall be made on the next following Business Day, and such extension of time shall be reflected in computing interest or fees, as the case may be; provided that, if any payment on a SOFR Loan becomes due and payable on a day other than a Business Day, such payment shall be due and payable on the next following Business Day unless the result of such extension would be to extend such payment into another calendar month, in which case, such payment shall be made on the immediately preceding Business Day.

(b) Funding by Lenders; Presumption by Administrative Agent.

(i) Unless the Administrative Agent shall have received notice from a Lender prior to the proposed date of any Borrowing of Loans that such Lender will not make available to the Administrative Agent such Lender's share of such Borrowing, the Administrative Agent may assume that such Lender has made such share available on such date in accordance with Section 2.02 (or, in the case of a Borrowing of Base Rate Loans, that such Lender has made such share available in accordance with and at the time required by Section 2.02) and may, in reliance upon such assumption, but shall have no obligation to do so, make available to the Borrower a corresponding amount. In such event, if a Lender has not in fact made its share of the applicable Borrowing available to the Administrative Agent, then the applicable Lender and the Borrower severally agree to pay to the Administrative Agent forthwith on demand such corresponding amount in immediately available funds with interest thereon, for each day from and including the date such amount is made available to the Borrower to but excluding the date of payment to the Administrative Agent, at (A) in the case of a payment to be made by such Lender, the greater of the Federal Funds Rate and a rate determined by the Administrative Agent in accordance with banking industry rules on interbank compensation, *plus* any administrative, processing or similar fees customarily charged by the Administrative Agent in connection with the foregoing, and (B) in the case of a payment to be made by the Borrower, the interest rate applicable to Base Rate Loans. If the Borrower and such Lender shall pay such interest to the Administrative Agent for the same or an overlapping period, the Administrative Agent shall promptly remit to the Borrower the amount of such interest paid by the Borrower for such period. If such Lender pays its share of the applicable Borrowing to the Administrative Agent, then the amount so paid shall constitute such Lender's Loan included in such Borrowing. Any payment by the Borrower shall be without prejudice to any claim the Borrower may have against a Lender that shall have failed to make such payment to the Administrative Agent.

(ii) Payments by Borrower; Presumptions by Administrative Agent. Unless the Administrative Agent shall have received notice from the Borrower prior to the date on which any payment is due to the Administrative Agent for the account of the Lenders hereunder that the Borrower will not make such payment, the Administrative Agent may assume that the Borrower has made such payment on such date in accordance herewith and may, in reliance upon such assumption, but shall have no obligation to do so, distribute to the Lenders the amount due. In such event, if the Borrower has not in fact made such payment, then each of the Lenders severally agrees to repay to the Administrative Agent forthwith on demand the amount so distributed to such Lender, in immediately available funds with interest thereon, for each day from and including the date such amount is distributed to it to but excluding the date of payment to the Administrative Agent, at the greater of the Federal Funds Rate and a rate determined by the Administrative Agent in accordance with banking industry rules on interbank compensation.

A notice of the Administrative Agent to any Lender or the Borrower with respect to any amount owing under this clause (b) shall be conclusive, absent manifest error.

(c) Failure to Satisfy Conditions Precedent. If any Lender makes available to the Administrative Agent funds for any Loan to be made by such Lender as provided in the foregoing provisions of this Article II, and such funds are not made available to the Borrower by the Administrative Agent because the conditions to the applicable Borrowing set forth in Article IV are not satisfied or waived in accordance with the terms hereof, the Administrative Agent shall return such funds (in like funds as received from such Lender) to such Lender, without interest.

(d) Obligations of Lenders Several. The obligations of the Lenders hereunder to make Loans and to make payments pursuant to Section 11.04(c) are several and not joint. The failure of any Lender to make any Loan, to fund any such participation or to make any payment under Section 11.04(c) on any date required hereunder shall not relieve any other Lender of its corresponding obligation to do so on such date, and no Lender shall be responsible for the failure of any other Lender to so make its Loan, to purchase its participation or to make its payment under Section 11.04(c).

(e) Funding Source. Nothing herein shall be deemed to obligate any Lender to obtain the funds for any Loan in any particular place or manner or to constitute a representation by any Lender that it has obtained or will obtain the funds for any Loan in any particular place or manner.

(f) Pro Rata Treatment. Except to the extent otherwise provided herein: (i) each Borrowing shall be made from the Lenders, each payment of fees under Section 2.07 shall be made for account of the Lenders, and each termination or reduction of the amount of the Commitments shall be applied to the respective Commitments of the Lenders, *pro rata* according to the amounts of their respective Commitments; (ii) each Borrowing shall be allocated pro rata among the Lenders according to the amounts of their respective Commitments or their respective Loans that are to be included in such Borrowing (in the case of conversions and continuations of Loans); (iii) each payment or prepayment of principal of Loans by the Borrower shall be made for account of the Lenders pro rata in accordance with the respective unpaid principal amounts of the Loans held by them; and (iv) each payment of interest on Loans by the Borrower shall be made for account of the applicable Lenders pro rata in accordance with the amounts of interest on such Loans then due and payable to the respective Lenders.

2.11 Sharing of Payments by Lenders.

If any Lender shall, by exercising any right of setoff or counterclaim or otherwise, obtain payment in respect of (a) Obligations due and payable to such Lender hereunder and under the other Loan Documents at such time in excess of its ratable share (according to the proportion of (i) the amount of such Obligations due and payable to such Lender at such time to (ii) the aggregate amount of the Obligations due and payable to all Lenders hereunder and under the other Loan Documents at such time) of payments on account of the Obligations due and payable to all Lenders hereunder and under the other Loan Documents at such time obtained by all the Lenders at such time or (b) Obligations owing (but not due and payable) to such Lender hereunder and under the other Loan Documents at such time in excess of its ratable share (according to the proportion of (i) the amount of such Obligations owing (but not due and payable) to such Lender at such time to (ii) the aggregate amount of the Obligations owing (but not due and payable) to all Lenders hereunder and under the other Loan Documents at such time) of payments on account of the Obligations owing (but not due and payable) to all Lenders hereunder and under the other Loan Documents at such time obtained by all of the Lenders at such time, then, in each case under clauses (a) and (b) above, the Lender receiving such greater proportion shall (A) notify the Administrative Agent and the Required Lenders of

such fact, and (B) purchase (for cash at face value) participations in the Loans of the other Lenders, or make such other adjustments as shall be equitable, so that the benefit of all such payments shall be shared by the Lenders ratably in accordance with the aggregate amount of Obligations then due and payable to the Lenders or owing (but not due and payable) to the Lenders, as the case may be, *provided* that:

(i) if any such participations or sub-participations are purchased and all or any portion of the payment giving rise thereto is recovered, such participations or sub-participations shall be rescinded and the purchase price restored to the extent of such recovery, without interest; and

(ii) the provisions of this Section 2.11 shall not be construed to apply to (A) any payment made by or on behalf of the Borrower pursuant to and in accordance with the express terms of this Agreement (including the application of funds arising from the existence of a Defaulting Lender) or (B) any payment obtained by a Lender as consideration for the assignment of or sale of a participation in any of its Loans to any assignee or participant, other than an assignment to any Loan Party or any Affiliate thereof (as to which the provisions of this Section 2.11 shall apply).

Each Loan Party consents to the foregoing and agrees, to the extent it may effectively do so under Applicable Law, that any Lender acquiring a participation pursuant to the foregoing arrangements may exercise against such Loan Party rights of setoff and counterclaim with respect to such participation as fully as if such Lender were a direct creditor of such Loan Party in the amount of such participation.

2.12 Defaulting Lenders.

(a) Adjustments. Notwithstanding anything to the contrary contained in this Agreement, if any Lender becomes a Defaulting Lender, then, until such time as that Lender is no longer a Defaulting Lender, to the extent permitted by Applicable Law:

(i) Waivers and Amendments. Such Defaulting Lender's right to approve or disapprove any amendment, waiver or consent with respect to this Agreement shall be restricted as set forth in the definition of "Required Lenders" and Section 11.01.

(ii) Defaulting Lender Waterfall. Any payment of principal, interest, fees or other amounts received by the Administrative Agent for the account of such Defaulting Lender (whether voluntary or mandatory, at maturity, pursuant to Article VIII or otherwise) or received by the Administrative Agent from a Defaulting Lender pursuant to Section 11.08 shall be applied at such time or times as may be determined by the Required Lenders as follows: *first*, to the payment of any amounts owing by such Defaulting Lender to the Administrative Agent hereunder and under the other Loan Documents; *second*, as the Borrower may request (so long as no Default or Event of Default exists), to the funding of any Loan in respect of which such Defaulting Lender has failed to fund its portion thereof as required by this Agreement, as determined by the Required Lenders; *third*, if so determined by the Required Lenders and the Borrower, to be held in a deposit account and released *pro rata* in order to satisfy such Defaulting Lender's potential future funding obligations with respect to Loans under this Agreement; *fourth*, to the payment of any amounts owing to the Lenders as a result of any judgment of a court of competent jurisdiction obtained by any Lender, against such Defaulting Lender as a result of such Defaulting Lender's breach of its obligations under this Agreement; *fifth*, so long as no Default or Event of Default exists, to the payment of any amounts owing to the Borrower as a result of any judgment of a court of competent jurisdiction obtained by the Borrower

against such Defaulting Lender as a result of such Defaulting Lender's breach of its obligations under this Agreement; and *sixth*, to such Defaulting Lender or as otherwise as may be required under the Loan Documents in connection with any Lien conferred thereunder or directed by a court of competent jurisdiction; *provided* that if (x) such payment is a payment of the principal amount of any Loans in respect of which such Defaulting Lender has not fully funded its appropriate share, and (y) such Loans were made at a time when the conditions set forth in Section 4.01 or Section 4.02, as applicable, were satisfied or waived, such payment shall be applied solely to pay the Loans of all Non-Defaulting Lenders on a *pro rata* basis prior to being applied to the payment of any Loans of such Defaulting Lender until such time as all Loans are held by the Lenders *pro rata* in accordance with the Commitments hereunder. Any payments, prepayments or other amounts paid or payable to a Defaulting Lender that are applied (or held) to pay amounts owed by a Defaulting Lender shall be deemed paid to and redirected by such Defaulting Lender, and each Lender irrevocably consents hereto.

(b) Defaulting Lender Cure. If the Borrower, the Administrative Agent and the Required Lenders agree in writing that a Lender is no longer a Defaulting Lender, the Administrative Agent will so notify the parties hereto, whereupon as of the effective date specified in such notice and subject to any conditions set forth therein, that Lender will, to the extent applicable, purchase at par that portion of outstanding Loans of the other Lenders or take such other actions as the Administrative Agent may determine to be necessary to cause the Loans to be held pro rata by the Lenders in accordance with their Commitments, whereupon such Lender will cease to be a Defaulting Lender; *provided* that no adjustments will be made retroactively with respect to fees accrued or payments made by or on behalf of the Borrower while that Lender was a Defaulting Lender; and *provided*, *further*, that except to the extent otherwise expressly agreed by the affected parties, no change hereunder from Defaulting Lender to Lender will constitute a waiver or release of any claim of any party hereunder arising from that Lender's having been a Defaulting Lender.

2.13 **Discretionary Incremental Facility**.

By written notice to the Administrative Agent and the Required Lenders, the Borrower may request from the Lenders, on one or more occasions, additional funding in an aggregate amount of up to $500,000,000 (if such request is approved as provided below, "Incremental Commitments"); provided that (i) the terms and conditions, including the use of proceeds, and form of any Incremental Commitments shall be subject to the consent of the Required Lenders, in its sole discretion; (ii) no Lender will have an obligation to provide any portion of the Incremental Commitments, and (iii) the Incremental Commitments shall be made on terms and conditions, and in a form, as agreed between the Borrower and the Lenders providing such Incremental Commitments. In the event that any such Incremental Commitments are approved, and to the extent the form is an increase in the Commitments hereunder, this Agreement shall be amended to reflect the terms thereof, with such amendment being subject to the consent of the Required Lenders, the Borrower and the Administrative Agent.

ARTICLE III

TAXES, YIELD PROTECTION AND ILLEGALITY

3.01 Taxes.

(a) Defined Terms. For purposes of this Section 3.01, the term "Applicable Law" includes FATCA.

(b) Payments Free of Taxes; Obligation to Withhold; Payments on Account of Taxes. Any and all payments by or on account of any obligation of any Loan Party under any Loan Document shall be made without deduction or withholding for any Taxes, except as required by Applicable Laws. If any Applicable Laws (as determined in the good faith discretion of an applicable Withholding Agent) require the deduction or withholding of any Tax from any such payment by a Withholding Agent, then the applicable Withholding Agent shall be entitled to make such deduction or withholding and shall timely pay the full amount deducted or withheld to the relevant Governmental Authority in accordance with Applicable Law and, if such Tax is an Indemnified Tax, then the sum payable by the applicable Loan Party shall be increased as necessary so that after any such withholdings or deductions (including such withholdings or deductions applicable to additional sums payable under this Section 3.01) the applicable Recipient receives an amount equal to the sum it would have received had no such withholding or deduction been made.

(c) Payment of Other Taxes by the Borrower. The Borrower shall timely pay to the relevant Governmental Authority in accordance with Applicable Law, or at the option of the Administrative Agent or the Required Lenders timely reimburse it for the payment of, any Other Taxes.

(d) Tax Indemnifications.

(i) Each of the Loan Parties shall, and does hereby, jointly and severally indemnify each Recipient, and shall make payment in respect thereof within ten (10) days after demand therefor, for the full amount of any Indemnified Taxes (including Indemnified Taxes imposed or asserted on or attributable to amounts payable under this Section 3.01) payable or paid by such Recipient or required to be withheld or deducted from a payment to such Recipient, and any penalties, interest and reasonable expenses arising therefrom or with respect thereto, whether or not such Indemnified Taxes were correctly or legally imposed or asserted by the relevant Governmental Authority. A certificate as to the amount of such payment or liability delivered to the Borrower by a Lender (with a copy to the Administrative Agent), or by the Administrative Agent on its own behalf or on behalf of a Lender, shall be conclusive absent manifest error.

(ii) Each Lender shall, and does hereby, severally indemnify and shall make payment in respect thereof within ten (10) days after demand therefor, (A) the Administrative Agent against any Indemnified Taxes attributable to such Lender (but only to the extent that any Loan Party has not already indemnified the Administrative Agent for such Indemnified Taxes and without limiting the obligation of the Loan Parties to do so), (B) the Administrative Agent against any Taxes attributable to such Lender's failure to comply with the provisions of Section 11.06(d) relating to the maintenance of a Participant Register and (C) the Administrative Agent against any Excluded Taxes attributable to such Lender, in each case, that are payable or paid by the Administrative Agent in connection with any Loan Document, and any reasonable expenses arising therefrom or with respect thereto, whether or not such Taxes were correctly or legally imposed or asserted by the relevant Governmental Authority. A certificate as to the amount of such payment or liability delivered to any Lender by the Administrative Agent shall be conclusive absent manifest error. Each Lender hereby authorizes the Administrative Agent to set off and apply any and all amounts at any time owing to such Lender under any Loan Document or otherwise payable by the Administrative Agent to the Lender from any other source against any amount due to the Administrative Agent under this clause (d)(ii).

(e) Evidence of Payments. As soon as practicable after any payment of Taxes by any Loan Party to a Governmental Authority, as provided in this Section 3.01, the Borrower shall deliver to the Administrative Agent the original or a certified copy of a receipt issued by such Governmental Authority evidencing such payment, a copy of any return reporting such payment or other evidence of such payment reasonably satisfactory to the Required Lenders.

(f) Status of Lenders; Tax Documentation.

(i) Any Lender that is entitled to an exemption from or reduction of withholding Tax with respect to payments made under any Loan Document shall deliver to the Borrower and the Administrative Agent, at the time or times reasonably requested by the Borrower or the Administrative Agent, such properly completed and executed documentation reasonably requested by the Borrower or the Administrative Agent as will permit such payments to be made without withholding or at a reduced rate of withholding. In addition, any Lender, if reasonably requested by the Borrower or the Administrative Agent, shall deliver such other documentation prescribed by Applicable Law or reasonably requested by the Borrower or the Administrative Agent as will enable the Borrower or the Administrative Agent to determine whether or not such Lender is subject to backup withholding or information reporting requirements. Notwithstanding anything to the contrary in the preceding two sentences, the completion, execution and submission of such documentation (other than such documentation set forth in Section 3.01(f)(ii)(A), (ii)(B) and (ii)(D) below) shall not be required if in the Lender's reasonable judgment such completion, execution or submission would subject such Lender to any material unreimbursed cost or expense or would materially prejudice the legal or commercial position of such Lender.

(ii) Without limiting the generality of the foregoing, in the event that the Borrower is a U.S. Person,

(A) any Lender that is a U.S. Person shall deliver to the Borrower and the Administrative Agent on or prior to the date on which such Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of the Borrower or the Administrative Agent), executed copies of IRS Form W–9 certifying that such Lender is exempt from U.S. federal backup withholding tax;

(B) any Foreign Lender shall, to the extent it is legally entitled to do so, deliver to the Borrower and the Administrative Agent (in such number of copies as shall be requested by the recipient) on or prior to the date on which such Foreign Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of the Borrower or the Administrative Agent), whichever of the following is applicable:

(1) in the case of a Foreign Lender claiming the benefits of an income tax treaty to which the United States is a party (x) with respect to payments of interest under any Loan Document, executed copies of IRS Form W–8BEN–E (or W–8BEN, as applicable) establishing an exemption from, or reduction of, U.S. federal withholding Tax pursuant to the "interest" article of such tax treaty and (y) with respect to any other applicable payments under any Loan Document, IRS Form W–8BEN–E (or W–8BEN, as applicable) establishing an exemption from, or reduction

of, U.S. federal withholding Tax pursuant to the "business profits" or "other income" article of such tax treaty;

(2) executed copies of IRS Form W–8ECI;

(3) in the case of a Foreign Lender claiming the benefits of the exemption for portfolio interest under Section 881(c) of the Code, (x) a certificate substantially in the form of Exhibit I–1 to the effect that such Foreign Lender is not a "bank" within the meaning of Section 881(c)(3)(A) of the Code, a "10 percent shareholder" of the Borrower within the meaning of Section 881(c)(3)(B) of the Code, or a "controlled foreign corporation" described in Section 881(c)(3)(C) of the Code (a "*U.S. Tax Compliance Certificate*") and (y) executed copies of IRS Form W–8BEN–E (or W–8BEN, as applicable); or

(4) to the extent a Foreign Lender is not the beneficial owner, executed copies of IRS Form W–8IMY, accompanied by IRS Form W–8ECI, IRS Form W–8BEN–E (or W–8BEN, as applicable), a U.S. Tax Compliance Certificate substantially in the form of Exhibit I–2 or Exhibit I–3, IRS Form W–9, and/or other certification documents from each beneficial owner, as applicable; *provided* that if the Foreign Lender is a partnership and one or more direct or indirect partners of such Foreign Lender are claiming the portfolio interest exemption, such Foreign Lender may provide a U.S. Tax Compliance Certificate substantially in the form of Exhibit I–4 on behalf of each such direct and indirect partner;

(C) any Foreign Lender shall, to the extent it is legally entitled to do so, deliver to the Borrower and the Administrative Agent (in such number of copies as shall be requested by the recipient) on or prior to the date on which such Foreign Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of the Borrower or the Administrative Agent), executed copies (or originals, as required) of any other form prescribed by Applicable Law as a basis for claiming exemption from or a reduction in U.S. federal withholding Tax, duly completed, together with such supplementary documentation as may be prescribed by Applicable Law to permit the Borrower or the Administrative Agent to determine the withholding or deduction required to be made; and

(D) if a payment made to a Lender under any Loan Document would be subject to U.S. federal withholding Tax imposed by FATCA if such Lender were to fail to comply with the applicable reporting requirements of FATCA (including those contained in Section 1471(b) or 1472(b) of the Code, as applicable), such Lender shall deliver to the Borrower and the Administrative Agent at the time or times prescribed by law and at such time or times reasonably requested by the Borrower or the Administrative Agent such documentation prescribed by Applicable Law (including as prescribed by Section 1471(b)(3)(C)(i) of the Code) and such additional documentation reasonably requested by the Borrower or the Administrative Agent as may be necessary for the Borrower and the Administrative Agent to comply with their obligations under FATCA and to determine that such Lender has complied with such Lender's obligations under FATCA or to determine the amount to deduct and withhold from

such payment. Solely for the purposes of this clause (f)(ii)(D), "FATCA" shall include any amendments made to FATCA after the date of this Agreement.

(iii) Each Lender agrees that if any form or certification it previously delivered pursuant to this Section 3.01 expires or becomes obsolete or inaccurate in any respect, it shall update such form or certification or promptly notify the Borrower and the Administrative Agent in writing of its legal inability to do so.

(g) Treatment of Certain Refunds. If any party determines, in its sole discretion exercised in good faith, that it has received a refund of any Taxes as to which it has been indemnified pursuant to this Section 3.01, including any payments of additional amounts pursuant to this Section 3.01, it shall pay to the indemnifying party an amount equal to such refund (but only to the extent of indemnity payments made under this Section 3.01 with respect to the Taxes giving rise to such refund), net of all out-of-pocket expenses (including Taxes) of such indemnified party and without interest (other than any interest paid by the relevant Governmental Authority with respect to such refund). Such indemnifying party, upon the request of such indemnified party, shall repay to such indemnified party the amount paid over pursuant to this clause (g) (plus any penalties, interest or other charges imposed by the relevant Governmental Authority) in the event that such indemnified party is required to repay such refund to such Governmental Authority. Notwithstanding anything to the contrary in this clause (g), in no event will the indemnified party be required to pay any amount to an indemnifying party pursuant to this clause (g) the payment of which would place the indemnified party in a less favorable net after-Tax position than the indemnified party would have been in if the Tax subject to indemnification and giving rise to such refund had not been deducted, withheld or otherwise imposed and the indemnification payments or additional amounts with respect to such Tax had never been paid. This clause (g) shall not be construed to require any indemnified party to make available its Tax returns (or any other information relating to its Taxes that it deems confidential) to the indemnifying party or any other Person.

(h) Survival. Each party's obligations under this Section 3.01 shall survive the resignation or replacement of the Administrative Agent or any assignment of rights by, or the replacement of, a Lender, the termination of the Commitments and the repayment, satisfaction or discharge of all obligations under any Loan Document.

3.02 Changes in Law; Impracticability or Illegality.

(a) In the event that any change in market conditions or any law, regulation, treaty, or directive, or any change therein or in the interpretation of application thereof, shall at any time after the date hereof, in the reasonable opinion of any Lender, make it unlawful or impractical for such Lender to fund or maintain SOFR Loans or to continue such funding or maintaining, or to determine or charge interest rates at Adjusted Term SOFR, such Lender shall give notice of such changed circumstances to the Borrower and the Administrative Agent, and the Administrative Agent promptly shall transmit the notice to each other Lender and (i) in the case of any SOFR Loans of such Lender that are outstanding, the date specified in such Lender's notice shall be deemed to be the last day of the Interest Period of such SOFR Loans, and interest upon the SOFR Loans of such Lender thereafter shall accrue interest at the rate then applicable to Base Rate Loans of the same type hereunder, and (ii) the Borrower shall not be entitled to elect the SOFR Option (including in any borrowing, conversion or continuation then being requested) until such Lender determines that it would no longer be unlawful or impractical to do so.

(b) The obligations of the Loan Parties under this Section 3.02 shall survive the termination of this Agreement and the payment of the Loans and all other amounts payable hereunder.

3.03 Benchmark Replacement.

(a) Notwithstanding anything to the contrary herein or in any other Loan Document, upon the occurrence of a Benchmark Transition Event, the Administrative Agent and the Required Lenders may amend this Agreement to replace the then-current Benchmark with a Benchmark Replacement. The parties shall use commercially reasonable efforts to satisfy any applicable IRS guidance, including Treasury Regulation Section 1.1001-6 and any future guidance, to the effect that the implementation of a Benchmark Replacement will not result in a deemed exchange for U.S. federal income tax purposes of any Loan under this Agreement for U.S. federal income tax purposes.

(b) In connection with the use, administration, adoption or implementation of a Benchmark Replacement, the Administrative Agent will have the right to make Conforming Changes from time to time and, notwithstanding anything to the contrary herein or in any other Loan Document, any amendments implementing such Conforming Changes will become effective without any further action or consent of any other party to this Agreement (and the Lenders hereby (i) authorize and direct the Administrative Agent to make any Conforming Changes (and to enter into any modifications to this Agreement or the other Loan Documents implementing such Conforming Changes) that have been consented or agreed to by the Required Lenders, or in respect of which the Administrative Agent has received a direction from the Required Lenders to implement, and (ii) acknowledge and agree that the Administrative Agent shall be entitled to all of the exculpations, protections and indemnifications provided for in this Agreement in favor of the Administrative Agent in implementing any Conforming Changes (or in entering into any modifications to this Agreement or the other Loan Documents implementing the same) that have been consented or agreed to by the Required Lenders, or in respect of which the Administrative Agent has received a direction from the Required Lenders to implement).

(c) Any determination, decision or election that may be made by the Administrative Agent and the Required Lenders pursuant to this Section 3.03, including any determination with respect to a tenor, rate or adjustment or of the occurrence or non-occurrence of an event, circumstance or date and any decision to take or refrain from taking any action or any selection, will be conclusive and binding absent manifest error and may be made in its or their sole discretion and without consent from any other party to this Agreement or any other Loan Document.

(d) Notwithstanding anything to the contrary herein or in any other Loan Document, at any time (including in connection with the implementation of a Benchmark Replacement), (i) if the then-current Benchmark is a term rate (including the Term SOFR Reference Rate) and either (A) any tenor for such Benchmark is not displayed on a screen or other information service that publishes such rate from time to time as selected by the Administrative Agent in its reasonable discretion or (B) the regulatory supervisor for the administrator of such Benchmark has provided a public statement or publication of information announcing that any tenor for such Benchmark is not or will not be representative, then the Administrative Agent may modify the definition of "Interest Period" (or any similar or analogous definition) for any Benchmark settings at or after such time to remove such unavailable or non-representative tenor and (ii) if a tenor that was removed pursuant to clause (i) above either (A) is subsequently displayed on a screen or information service for a Benchmark (including a Benchmark Replacement) or (B) is not, or is no longer, subject to an announcement that it is not or will not be representative for a Benchmark

(including a Benchmark Replacement), then the Administrative Agent may modify the definition of "Interest Period" (or any similar or analogous definition) for all Benchmark settings at or after such time to reinstate such previously removed tenor.

(e) Upon the Borrower's receipt of notice of the commencement of a Benchmark Unavailability Period, the Borrower may revoke any pending request for a borrowing of, conversion to or continuation of SOFR Loans to be made, converted or continued during any Benchmark Unavailability Period and, failing that, the Borrower will be deemed to have converted any such request into a request for a Borrowing of or conversion to Base Rate Loans.

3.04 Increased Costs.

(a) Increased Costs Generally. If any Change in Law shall:

(i) impose, modify or deem applicable any reserve, special deposit, compulsory loan, insurance charge or similar requirement against assets of, deposits with or for the account of, or credit extended or participated in by, any Lender (except any reserve requirement contemplated by Section 3.04(e));

(ii) subject any Recipient to any Taxes (other than (A) Indemnified Taxes, (B) Taxes described in clauses (b) through (d) of the definition of Excluded Taxes and (C) Connection Income Taxes) on its loans, loan principal, letters of credit, commitments, or other obligations, or its deposits, reserves, other liabilities or capital attributable thereto; or

(iii) impose on any Lender any other condition, cost or expense (other than Taxes) affecting this Agreement or SOFR Loans made by such Lender;

and the result of any of the foregoing shall be to increase the cost to such Lender of making, converting to, continuing or maintaining any Loan (or of maintaining its obligation to make any such Loan) or to reduce the amount of any sum received or receivable by such Lender hereunder (whether of principal, interest or any other amount) then, upon request of such Lender, the Borrower will pay to such Lender such additional amount or amounts as will compensate such Lender for such additional costs incurred or reduction suffered. Notwithstanding anything to the contrary in this Section 3.04(a), it shall be a condition to any Lender's exercise of its rights, if any, under this Section 3.04(a) that such Lender shall generally be exercising similar rights with respect to borrowers under similar agreements.

(b) Capital Requirements. If any Lender determines that any Change in Law affecting such Lender or any Lending Office of such Lender or such Lender's holding company, if any, regarding capital or liquidity requirements has or would have the effect of reducing the rate of return on such Lender's capital or on the capital of such Lender's holding company, if any, as a consequence of this Agreement, the Commitments of such Lender or the Loans made by such Lender, to a level below that which such Lender or such Lender's holding company could have achieved but for such Change in Law (taking into consideration such Lender's policies and the policies of such Lender's holding company with respect to capital adequacy), then from time to time the Borrower will pay to such Lender such additional amount or amounts as will compensate such Lender or such Lender's or holding company for any such reduction suffered. Notwithstanding anything to the contrary in this Section 3.04(b), it shall be a condition to any Lender's exercise of its rights, if any, under this Section 3.04(b) that such Lender shall generally be exercising similar rights with respect to borrowers under similar agreements.

(c) Certificates for Reimbursement. A certificate of a Lender setting forth the amount or amounts necessary to compensate such Lender or its holding company, as the case may be, as specified in clause (a) or (b) of this Section 3.04 and delivered to the Borrower (with a copy to the Administrative Agent) shall be conclusive absent manifest error. The Borrower shall pay such Lender the amount shown as due on any such certificate within ten (10) days after receipt thereof.

(d) [Reserved].

(e) Delay in Requests. Failure or delay on the part of any Lender to demand compensation pursuant to the foregoing provisions of this Section 3.04 shall not constitute a waiver of such Lender's right to demand such compensation, *provided* that the Borrower shall not be required to compensate a Lender pursuant to the foregoing provisions of this Section 3.04 for any increased costs incurred or reductions suffered more than nine (9) months prior to the date that such Lender notifies the Borrower of the Change in Law giving rise to such increased costs or reductions and of such Lender's intention to claim compensation therefor (except that, if the Change in Law giving rise to such increased costs or reductions is retroactive, then the nine (9) month period referred to above shall be extended to include the period of retroactive effect thereof).

3.05 Compensation for Losses.

Upon demand of any Lender (with a copy to the Administrative Agent and the Required Lenders) from time to time, the Borrower shall promptly compensate such Lender for and hold such Lender harmless from any loss, cost or expense (other than lost profits) incurred by it as a result of:

(a) any continuation, conversion, payment or prepayment of any Loan other than a Base Rate Loan on a day other than the last day of the Interest Period for such Loan (whether voluntary, mandatory, automatic, by reason of acceleration, or otherwise);

(b) any failure by the Borrower (for a reason other than the failure of such Lender to make a Loan) to prepay, borrow, continue or convert any Loan other than a Base Rate Loan on the date or in the amount notified by the Borrower; or

(c) any assignment of a SOFR Loan on a day other than the last day of the Interest Period therefor as a result of a request by the Borrower pursuant to Section 11.13;

excluding any loss of anticipated profits but including any loss or expense arising from the liquidation or reemployment of funds obtained by it to maintain such Loan or from fees payable to terminate the deposits from which such funds were obtained.

3.06 Mitigation Obligations; Replacement of Lenders.

(a) Designation of a Different Lending Office. If any Lender requests compensation under Section 3.04, or requires the Borrower to pay any Indemnified Taxes or additional amounts to any Lender, or any Governmental Authority for the account of any Lender pursuant to Section 3.01, or if any Lender gives a notice pursuant to Section 3.02, then at the request of the Borrower, such Lender shall use reasonable efforts to designate a different Lending Office for funding or booking its Loans hereunder or to assign its rights and obligations hereunder to another of its offices, branches or affiliates, if, in the judgment of such Lender such designation or assignment (i) would eliminate or reduce amounts payable pursuant to Section 3.01 or 3.04, as the case may be, in the future, or eliminate the need for the notice pursuant to Section 3.02, as applicable, and (ii) in each case, would not subject such Lender or to any unreimbursed cost or expense and would

not otherwise be disadvantageous to such Lender. The Borrower hereby agrees to pay all reasonable costs and expenses incurred by any Lender in connection with any such designation or assignment.

(b) Replacement of Lenders. If any Lender requests compensation under Section 3.04, gives a notice pursuant to Section 3.02 or if the Borrower is required to pay any Indemnified Taxes or additional amounts to any Lender or any Governmental Authority for the account of any Lender pursuant to Section 3.01 and, in each case, such Lender has declined or is unable to designate a different lending office in accordance with Section 3.06(a), the Borrower may replace such Lender (and, in the case of a Lender that gives a notice pursuant to Section 3.02, with the consent of the Required Lenders) in accordance with Section 11.13.

3.07 Survival.

All of the Borrower's obligations under this Article III shall survive termination of the Aggregate Commitments, repayment of all other Obligations hereunder, resignation of the Administrative Agent and the Required Lenders and the Facility Termination Date.

ARTICLE IV

CONDITIONS PRECEDENT

4.01 Conditions of Effectiveness and the Borrowing of Initial Loans.

The effectiveness of this Agreement and the obligation of each Lender to make its portion of the Initial Loan hereunder on the Closing Date is subject to satisfaction or waiver of the following conditions precedent:

(a) Execution of Credit Agreement; Loan Documents. The Administrative Agent and the Lenders shall have received (i) counterparts of this Agreement, executed by a Responsible Officer of each Loan Party and a duly authorized officer of each Lender, (ii) for the account of each Lender that has requested a Note prior to the Closing Date, a Note executed by the Borrower, (iii) counterparts of the U.S. Security Agreement executed by a Responsible Officer of the applicable Loan Parties and a duly authorized officer of each other Person party thereto, as applicable and (iv) counterparts of the Agency Fee Letter (which shall only be provided to the Administrative Agent, and not the Lenders), the Fee Letter, and any other Loan Documents to be entered into on the Closing Date.

(b) Officer's Certificate. The Administrative Agent and the Lenders shall have received an Officer's Certificate dated the Closing Date, certifying as to the Organization Documents of each Loan Party (which, to the extent filed with a Governmental Authority, shall be certified as of a recent date by such Governmental Authority), the resolutions of the governing body of each Loan Party, the good standing (to the extent such concept is applicable in the corresponding jurisdiction), existence or its equivalent of each Loan Party in its jurisdiction of formation or incorporation and of the incumbency (including specimen signatures) of the Responsible Officers of each Loan Party, and certifying as to the satisfaction of the conditions set forth in this Section 4.01 (other than those constituting the satisfaction of the Administrative Agent or any Lender) to the funding of Initial Loans on the Closing Date. In respect of any Irish Loan Party, the Officer's Certificate shall further certify that it and each other Loan Party constitute a group of companies for the purposes of section 239 of the Companies Act (Ireland) and that it has done all that is necessary to comply with section 82 of the Companies Act (Ireland) in order to enable such Loan Party to enter into the Loan Documents and perform its obligations under the Loan Documents.

(c) Legal Opinions of Counsel. The Administrative Agent and the Lenders shall have received a customary opinion or opinions of counsel for the Loan Parties, dated the Closing Date and addressed to the Administrative Agent and the Lenders party to this Agreement on the Closing Date.

(d) Personal Property Collateral. The Administrative Agent and the Lenders shall have received:

(i) (A) searches of UCC filings (or equivalent) in the jurisdiction of incorporation or formation, as applicable, of each Loan Party and each jurisdiction where a filing would need to be made in order to perfect the Administrative Agent's security interest in the Collateral and copies of the financing statements on file in such jurisdictions and (B) tax lien, judgment, bankruptcy and insolvency searches;

(ii) searches of ownership of Intellectual Property in the appropriate governmental offices and such patent/trademark/copyright filings as requested by the Administrative Agent or the Required Lenders prior to the Closing Date in order to perfect the Administrative Agent's security interest in the Intellectual Property; and

(iii) forms of UCC financing statements for each appropriate jurisdiction as is necessary, in the Required Lenders's sole discretion, to perfect the Administrative Agent's security interest in the Collateral.

(e) Insurance. The Administrative Agent and the Lenders shall have received copies of insurance certificates evidencing insurance meeting the requirements set forth herein or in the Collateral Documents.

(f) Solvency Certificate. The Administrative Agent and the Lenders shall have received a Solvency Certificate signed by a Responsible Officer of the Borrower as to the Solvency of the Borrower and its Subsidiaries, on a Consolidated basis, after giving effect to the transactions contemplated hereby.

(g) Anti-Money-Laundering; Beneficial Ownership. Upon the reasonable request of the Administrative Agent, the Required Lenders or any other Lender made at least five (5) Business Days prior to the Closing Date, the Borrower shall have provided to the Administrative Agent, the Required Lenders or such other Lender or the Required Lenders the documentation and other information so requested in connection with applicable "know your customer" and anti-money-laundering rules and regulations, including, without limitation, the Patriot Act, and any Loan Party that qualifies as a "legal entity customer" under the Beneficial Ownership Regulation shall have delivered to each Lender that so requests, a Beneficial Ownership Certification in relation to such Loan Party.

(h) Consents. The Administrative Agent and the Lenders shall have received evidence that all members, boards of directors, governmental, shareholder and material third party consents and approvals necessary in connection with the entering into of this Agreement have been obtained.

(i) Fees and Expenses. The Administrative Agent and the Lenders shall have received all fees and expenses owing hereunder or under the Agency Fee Letter and under the Fee Letter on the Closing Date, but, in the case of expenses, only to the extent invoiced prior to the Closing Date.

(j) Representations and Warranties. The representations and warranties of the Borrower and each other Loan Party contained in Article II, Article V or any other Loan Document, shall (i) with respect to representations and warranties that contain a materiality qualification, be true and correct on and as of the date of such Borrowing, except to the extent such representations and warranties specifically relate to an earlier date, in which case such representations and warranties shall have been true and correct on and as of such earlier date and (ii) with respect to representations and warranties that do not contain a materiality qualification, be true and correct in all material respects on and as of the date of such Borrowing, except to the extent such representations and warranties specifically relate to an earlier date, in which case such representations and warranties shall have been true and correct in all material respects on and as of such earlier date, and except that for purposes of this Section 4.01, the representations and warranties contained in Sections 5.05(a) shall be deemed to refer to the most recent statements furnished pursuant to Sections 6.01(a).

(k) Default. No Default shall exist, or would immediately result from such proposed Borrowing or from the application of the proceeds thereof.

(l) Loan Notice. The Administrative Agent shall have received a Loan Notice in accordance with the requirements hereof.

(m) Equity Issuance. The Equity Issuance shall have been consummated, or shall be consummated simultaneously with the funding of the Initial Loans pursuant to the terms of the Stock Purchase Agreement.

Without limiting the generality of the provisions of Section 9.03(c), for purposes of determining compliance with the conditions specified in this Section 4.01 and each Lender that has signed this Agreement shall be deemed to have consented to, approved or accepted or to be satisfied with, each document or other matter required thereunder to be consented to or approved by or acceptable or satisfactory to the Required Lenders or any Lender unless the Administrative Agent shall have received notice from the Required Lenders or such Lender prior to the proposed Closing Date specifying its objection thereto.

4.02 Conditions to Borrowing of Delayed Draw Loans.

The obligation of each Lender to honor a Loan Notice with respect to each Borrowing of Delayed Draw Loans is subject to the following conditions precedent:

(a) Representations and Warranties. The representations and warranties of the Borrower and each other Loan Party contained in Article II, Article V or any other Loan Document, shall (i) with respect to representations and warranties that contain a materiality qualification, be true and correct on and as of the date of such Borrowing, except to the extent such representations and warranties specifically relate to an earlier date, in which case such representations and warranties shall have been true and correct on and as of such earlier date and (ii) with respect to representations and warranties that do not contain a materiality qualification, be true and correct in all material respects on and as of the date of such Borrowing, except to the extent such representations and warranties specifically relate to an earlier date, in which case such representations and warranties shall have been true and correct in all material respects on and as of such earlier date, and except that for purposes of this Section 4.02, the representations and warranties contained in Sections 5.05(a) shall be deemed to refer to the most recent statements furnished pursuant to Sections 6.01(a).

(b) Default. No Default shall exist, or would immediately result from such proposed Borrowing or from the application of the proceeds thereof.

(c) Fees and Expenses. The Administrative Agent and the Lenders shall have received all fees and expenses owing hereunder or under the Fee Letter with respect to such funding of Delayed Draw Loans, but, in the case of expenses, only to the extent invoiced prior to the Closing Date.

(d) Loan Notice. The Administrative Agent shall have received a Loan Notice in accordance with the requirements hereof.

(e) Liquidity. Liquidity, both before, and immediately after giving effect to such funding and the proposed use of proceeds, shall not be less than (i) prior to the Liquidity Increase Date, $100,000,000 and (ii) from and after the Liquidity Increase Date, $200,000,000; provided that, for the avoidance of doubt, the proceeds to be received in respect of a Borrowing of any Delayed Draw Loans shall not be included in the calculation of Liquidity under this Section 4.02(e) for purposes of meeting the condition precedent to the making of such Delayed Draw Loans.

(f) TTM Revenue. With respect to any proposed funding of Delayed Draw Loans after March 31, 2023, TTM Special Products Revenue for the twelve-month period ending with the most recent month ended prior to the proposed funding date shall be at least $[**].

(g) Phase 3 Positive Readout. Either (i) the Phase 3 Positive Readout shall have been received or (ii) $[**] shall have been deposited in the Reserve Account in accordance with Schedule 6.20 (for the avoidance of doubt, prior to any funding of the Delayed Draw Loans).

(h) Officer's Certificate. The Administrative Agent and the Lenders shall have received an Officer's Certificate dated the proposed funding date, certifying as to the satisfaction of the conditions to such funding, with supporting calculations and documentation in form and substance reasonably satisfactory to the Required Lenders.

Each Loan Notice (other than a Loan Notice requesting only a conversion of Loans to the other Type or a continuation of SOFR Loans) submitted by the Borrower shall be deemed to be a representation and warranty that the conditions specified in Sections 4.02(a) and (b) have been satisfied on and as of the date of the applicable Borrowing.

ARTICLE V

REPRESENTATIONS AND WARRANTIES

Each Loan Party represents and warrants to the Administrative Agent and the Lenders, as of the date made or deemed made, that:

5.01 Existence, Qualification and Power.

Each Loan Party and each of its Subsidiaries (a) is duly organized or formed, validly existing and, as applicable, in good standing under the Laws of the jurisdiction of its incorporation or organization, (b) has all requisite power and authority and all requisite governmental licenses, authorizations, consents and approvals to (i) own or lease Collateral it owns and carry on its business and (ii) execute, deliver and perform its obligations under the Loan Documents to which it is a party, and (c) is duly qualified and is licensed and, as applicable, in good standing under the Laws of each jurisdiction where its ownership, lease

or operation of properties or the conduct of its business requires such qualification or license; except in each case referred to in clause (b)(i) or (c), to the extent that failure to do so could not reasonably be expected to have a Material Adverse Effect.

5.02 Authorization; No Contravention.

The execution, delivery and performance by each Loan Party of each Loan Document to which such Person is or is to be a party have been duly authorized by all necessary corporate or other organizational action, and do not and will not (a) contravene the terms of any of such Person's Organization Documents; (b) conflict with or result in any breach or contravention of, or the creation of (or the requirement to create) any Lien under, or require any payment to be made under (i) any material Contractual Obligation to which such Person is a party or affecting such Person or the properties constituting Collateral of such Person or any of its Subsidiaries or (ii) any order, injunction, writ or decree of any Governmental Authority or any arbitral award to which such Person or its property constituting Collateral is subject; or (c) violate any Applicable Law, except in the case of this Section 5.02(c), with respect to any conflict, breach, violation, or payment, to the extent that such conflict, breach, violation, or payment would not reasonably be expected to have a Material Adverse Effect.

5.03 Governmental Authorization; Other Consents.

No approval, consent, exemption, authorization, or other action by, or notice to, or filing with, any Governmental Authority or any other Person is necessary or required in connection with (a) the execution, delivery or performance by, or enforcement against, any Loan Party of this Agreement or any other Loan Document, (b) the grant by any Loan Party of the Liens granted by it pursuant to the Collateral Documents, (c) the perfection or maintenance of the Liens created under the Collateral Documents (including the first priority nature thereof) or (d) the exercise by the Administrative Agent or any Lender of its rights under the Loan Documents or the remedies in respect of the Collateral pursuant to the Collateral Documents, other than (i) authorizations, approvals, actions, notices and filings which have been duly obtained, (ii) filings to perfect the Liens created by the Collateral Documents (including, in respect of Irish Security Documents, compliance with the procedure under section 409(3) or 409(4) of the Companies Act (Ireland) and section 1001(3A) of the Taxes Consolidation Act 1997 of Ireland), (iii) filings by the Borrower required under applicable securities laws and (iv) those approvals, consents, exemptions, authorizations or other actions, notices or filings, the failure of which to obtain or make could not reasonably be expected to have a Material Adverse Effect.

5.04 Binding Effect.

This Agreement has been, and each other Loan Document, when delivered hereunder, will have been, duly executed and delivered by each Loan Party that is party thereto. This Agreement constitutes, and each other Loan Document when so delivered will constitute, a legal, valid and binding obligation of such Loan Party, enforceable against each Loan Party that is party thereto in accordance with its terms, subject to applicable bankruptcy, insolvency, reorganization, moratorium, examinership, rescue process or other laws affecting creditors' rights generally and subject to general principals of equity.

5.05 Financial Statements; No Material Adverse Effect.

(a) Audited Financial Statements. The Audited Financial Statements (i) were prepared in accordance with GAAP consistently applied throughout the period covered thereby, except as otherwise expressly noted therein and (ii) fairly present in all material respects the financial condition of the Borrower and its Subsidiaries as of the date thereof and their results of operations and comprehensive loss, cash flows and changes in shareholders' equity for the period covered

thereby in accordance with GAAP consistently applied throughout the period covered thereby, except as otherwise expressly noted therein.

(b) Material Adverse Effect. Since December 31, 2021, there has been no event or circumstance, either individually or in the aggregate, that has had or would reasonably be expected to have a Material Adverse Effect.

5.06 Litigation.

There are no actions, suits, proceedings, claims or disputes pending or, to the knowledge of the Loan Parties, threatened in writing, at law, in equity, in arbitration or before any Governmental Authority, by or against any Loan Party or any Subsidiary that (a) purport to affect or pertain to this Agreement or any other Loan Document or any of the transactions contemplated hereby, or (b) either individually or in the aggregate would reasonably be expected to have a Material Adverse Effect.

5.07 [Reserved].

5.08 Ownership of Property.

Each Loan Party and each of its Subsidiaries has good record and marketable title in fee simple to, or valid leasehold interests in, all real property necessary or used in the ordinary conduct of its business, subject to Permitted Liens and except for such defects in title as would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

5.09 Environmental Matters.

(a) (A) except as could not reasonably be expected to result in material liability to any Loan Party or any of its Subsidiaries, none of the properties currently or formerly owned, leased or operated by any Loan Party or any of its Subsidiaries is listed or formally proposed for listing on the NPL or on the SEMS list; (B) except as would not have a Material Adverse Effect, as of the Closing Date, to the knowledge of the Loan Parties and their Subsidiaries, there is no asbestos or asbestos-containing material on, at or in any property currently owned, leased or operated by any Loan Party or any of its Subsidiaries; (C) except as would not have a Material Adverse Effect, there has not been a Release of Hazardous Materials on, at, under or from any property currently, or to the knowledge of the Loan Parties and their Subsidiaries, formerly owned, leased or operated by any Loan Party or any of its Subsidiaries, or otherwise arising from the operations of any Loan Party or any of its Subsidiaries; and (D) except as would not have a Material Adverse Effect, no Environmental Claim is pending or, to the knowledge of the Loan Parties and their Subsidiaries, threatened, with respect to or in connection with any Loan Party or any of its Subsidiaries or any real properties currently or formerly owned, leased or operated by any Loan Party or any of its Subsidiaries;

(b) (A) except as would not have a Material Adverse Effect, neither any Loan Party nor any of its Subsidiaries is undertaking, either individually or together with other potentially responsible parties, any investigation or assessment or remedial or response action relating to any Release of Hazardous Materials at, on, under, or from any site, location or operation, either voluntarily or pursuant to the order of any Governmental Authority or the requirements of any Environmental Law; and (B) except as would not have a Material Adverse Effect, all Hazardous Materials generated, used, treated, handled, stored, or transported by or on behalf of any Loan Party or any of its Subsidiaries have been disposed of in a manner which could not reasonably expected to result in liability to any Loan Party or any of its Subsidiaries; and

(c) except as would not have a Material Adverse Effect, each of the Loan Parties and their respective Subsidiaries is, and within the period of all applicable statutes of limitation has been, in compliance with all Environmental Laws (which compliance includes, but is not limited to, the possession of all Environmental Permits and compliance with the terms and conditions thereof).

5.10 Insurance.

The properties of each Loan Party are insured with financially sound and reputable insurance companies not Affiliates of the Borrower, in such amounts, with such deductibles and covering such risks as are customarily carried by companies engaged in similar businesses and owning similar properties in localities where the applicable Loan Party or the applicable Subsidiary operates. The general liability, casualty, property, terrorism and business interruption insurance coverage of the Loan Parties as in effect on the Closing Date is outlined as to carrier, policy number, expiration date, type, amount and deductibles on Schedule 5.10 and such insurance coverage complies with the requirements set forth in this Agreement and the other Loan Documents.

5.11 Taxes.

Each Loan Party and its Subsidiaries have filed all federal, state and other material tax returns and reports required to be filed, and have paid all federal, state and other material taxes, assessments, fees and other governmental charges levied or imposed upon them or their properties, income or assets otherwise due and payable, except those which are being contested in good faith by appropriate proceedings diligently conducted and for which adequate reserves have been provided in accordance with GAAP or where the failure to do so would not reasonably be expected to result in a Material Adverse Effect. There is no proposed tax assessment against any Loan Party or any Subsidiary that would, if made, have a Material Adverse Effect, nor is there any tax sharing agreement applicable to the Borrower or any Subsidiary (other than any tax sharing agreement solely between Borrower and one or more of its Subsidiaries).

5.12 ERISA Compliance.

(a) Each Plan is in compliance in all material respects with the applicable provisions of ERISA, the Code and other federal or state laws except as would not have a Material Adverse Effect. Each Pension Plan that is intended to be a qualified plan under Section 401(a) of the Code has received a favorable determination letter or is subject to a favorable opinion letter from the IRS to the effect that the form of such Plan is qualified under Section 401(a) of the Code and the trust related thereto has been determined by the IRS to be exempt from federal income tax under Section 501(a) of the Code, or an application for such a letter is currently being processed by the IRS except as would not have a Material Adverse Effect. To the knowledge of the Loan Parties, nothing has occurred that would prevent or cause the loss of such tax-qualified status except as would not have a Material Adverse Effect.

(b) There are no pending or, to the knowledge of the Loan Parties, threatened claims, actions or lawsuits, or action by any Governmental Authority, with respect to any Plan that would reasonably be expected to have a Material Adverse Effect. There has been no prohibited transaction or violation of the fiduciary responsibility rules with respect to any Plan that has resulted or could reasonably be expected to result in a Material Adverse Effect.

(c) (i) No ERISA Event has occurred, and no Loan Party nor any ERISA Affiliate is aware of any fact, event or circumstance that could reasonably be expected to constitute or result in an ERISA Event with respect to any Pension Plan or Multiemployer Plan except as would not

have a Material Adverse Effect; (ii) as of the most recent valuation date for any Pension Plan, the funding target attainment percentage (as defined in Section 430(d)(2) of the Code) is 60% or higher and no Loan Party nor any ERISA Affiliate knows of any facts or circumstances that could reasonably be expected to cause the funding target attainment percentage for any such plan to drop below 60% as of the most recent valuation date except as would not have a Material Adverse Effect; (iii) no Loan Party nor any ERISA Affiliate has incurred any liability to the PBGC other than for the payment of premiums, and there are no premium payments which have become due that are unpaid except as would not have a Material Adverse Effect; (iv) neither the Borrower nor any ERISA Affiliate has engaged in a transaction that could be subject to Section 4069 or Section 4212(c) of ERISA except as would not have a Material Adverse Effect; and (v) no Pension Plan has been terminated by the plan administrator thereof nor by the PBGC, and no event or circumstance has occurred or exists that could reasonably be expected to cause the PBGC to institute proceedings under Title IV of ERISA to terminate any Pension Plan except as would not have a Material Adverse Effect.

(d) Neither the Borrower nor any ERISA Affiliate maintains or contributes to, or has any unsatisfied obligation to contribute to, or liability under, any active or terminated Pension Plan other than (i) on the Closing Date, those listed on Schedule 5.12 hereto and (ii) thereafter, Pension Plans not otherwise prohibited by this Agreement.

(e) All pension schemes operated by any Irish Loan Party are operated or provided on a defined contribution basis.

5.13 Margin Regulations; Investment Company Act.

(a) Margin Regulations. Neither the Borrower nor any of its Subsidiaries is engaged or will engage, principally or as one of its important activities, in the business of purchasing or carrying margin stock (within the meaning of Regulation U), or extending credit for the purpose of purchasing or carrying margin stock. Following the application of the proceeds of each Borrowing, not more than twenty-five percent (25%) of the value of the assets (either of the Borrower only or of the Borrower and its Subsidiaries on a Consolidated basis) subject to the provisions of Section 7.01 or Section 7.05 or subject to any restriction contained in any agreement or instrument between the Borrower or any of its Subsidiaries and any Lender or any Affiliate of any Lender relating to Indebtedness and within the scope of Section 8.01(e) will be margin stock.

(b) Investment Company Act. No Loan Party is required to be registered as an "investment company" under the Investment Company Act of 1940, as amended.

5.14 Disclosure.

(a) No report, financial statement, certificate or other written information with respect to the Borrower or its Subsidiaries furnished by or on behalf of any Loan Party to the Administrative Agent, the Required Lenders or any Lender in connection with the transactions contemplated hereby and the negotiation of this Agreement or delivered hereunder or under any other Loan Document (in each case as modified or supplemented by other information so furnished and other than projected financial information, pro forma information and information of a general economic or industry nature), when taken as a whole and when furnished, contains any material misstatement of fact or omits to state any material fact necessary to make the statements therein, in the light of the circumstances under which they were made, not materially misleading; *provided* that, with respect to projected financial information, pro forma information each Loan Party represents only that such information was prepared in good faith based upon assumptions believed to be reasonable

at the time of preparation and delivery; it being understood that actual results may vary from such forecasts and that such variances may be material.

(b) As of the Closing Date, the Immaterial Subsidiaries of the Borrower are listed on Schedule 5.14(b)(i) hereto. The Borrower does not own or hold, directly or indirectly, any Equity Interests of any Person other than the Investments listed on Schedule 5.14(b)(ii).

5.15 Compliance with Laws.

The Borrower and each Subsidiary is in compliance with all Applicable Laws, except in such instances in which (i) such requirement of Applicable Law or order, writ, injunction or decree is being contested in good faith by appropriate proceedings diligently conducted or (ii) the failure to comply therewith could not reasonably be expected to have a Material Adverse Effect.

5.16 Solvency.

The Borrower and its Subsidiaries are Solvent on a Consolidated basis.

5.17 Reserved.

5.18 Sanctions Concerns and Anti-Corruption Laws.

(a) Sanctions Concerns. No Loan Party, nor any Subsidiary, nor, to the knowledge of the Loan Parties and their Subsidiaries, any director, officer, employee, agent, affiliate or representative thereof, is an individual or entity that is, or is owned or controlled by one or more individuals or entities that are (i) currently the subject or target of any Sanctions, (ii) included on OFAC's List of Specially Designated Nationals or HMT's Consolidated List of Financial Sanctions Targets, or any similar list enforced by any other relevant sanctions authority or (iii) located (except for sporadic personal travel by individuals, not engaged in business for the Borrower or its Subsidiaries), organized, incorporated or resident in a Designated Jurisdiction. The Borrower and its Subsidiaries have conducted their businesses in compliance with all applicable Sanctions and have instituted and maintained policies and procedures designed to promote and achieve compliance with such Sanctions.

(b) Anti-Corruption Laws. The Loan Parties and their Subsidiaries have conducted their business in compliance in all material respects with the United States Foreign Corrupt Practices Act of 1977, the UK Bribery Act 2010, the Criminal Justice (Corruption Offences) Act 2018 of Ireland, as amended and the Criminal Justice (Money Laundering and Terrorist Financing) Act 2010 to 2021 of Ireland and other applicable anti-corruption legislation in other jurisdictions, and have instituted and maintained policies and procedures designed to promote and achieve compliance with such laws.

5.19 EEA Financial Institutions.

No Loan Party is an EEA Financial Institution.

5.20 Covered Entities.

No Loan Party is a Covered Entity.

5.21 Beneficial Ownership Certification.

The information included in the Beneficial Ownership Certification, if applicable, is true and correct in all material respects as of the date when furnished.

5.22 Reserved.

5.23 Intellectual Property; Licenses, Etc.

(a) Each Loan Party owns, or possesses the right to use, all of the IP Collateral that are used in or reasonably necessary for the development, manufacture, and commercialization of the Specified Products ("*Specified Products Business*"), and no IP Collateral is owned or in-licensed by any Affiliate of the Borrower that is not a Loan Party. To Borrower's knowledge, the Loan's Party's rights in and to the IP Collateral do not conflict with the rights of any other Person.

(b) Schedule 5.23(b) sets forth a true, correct and complete listing, under separate headings, of all written Contractual Obligations under which any Loan Party or any of its Subsidiaries (i) has received a license or other right to practice or use any IP Collateral that any other Person owns, or (ii) owes any royalties or other payments to any Person for the use of any IP Collateral, or (iii) has granted any Person any right or interest in any IP Collateral in return for royalties or other payments, excluding, in the case of each of clause (i)-(iii), Contractual Obligations that are (x) not material or (y) entered into in the ordinary course of business and which do not include any right to commercialize any Specified Product (which, for the avoidance of doubt, includes investigator-initiated study agreements and material transfer agreements related to research, in both cases entered into in the ordinary course of business); *provided, however,* that, the Borrower may update Schedule 5.23(b) as of the date of borrowing of any Delayed Draw Loans.

(c) Schedule 5.23(c) sets forth a complete and accurate list of all of the IP Collateral that are U.S. (federal or state) and foreign (i) issued Patents and any Patent applications, (ii) registered and material unregistered Trademarks (including domain names) and any pending registrations for Trademarks, and (iii) any other registered Owned Intellectual Property or Licensed Intellectual Property. For each item of Intellectual Property listed on Schedule 5.23(c), the Borrower has, where relevant, indicated (A) the countries in each case in which such item is registered, (B) the application numbers, (C) the registration or patent numbers, (D) with respect to Patents for which an extension of term has been filed for or granted, the expected expiration date of such Patents and (E) the owner of such item of Intellectual Property and, if licensed to any Loan Party, the applicable agreement pursuant to which the Intellectual Property is so licensed. Except as identified in Schedule 5.23(c), (1) with respect to the IP Collateral, the owner listed on Schedule 5.23(c) is the exclusive owner of such registration or application; (2) to the Borrower's knowledge, the registrations of the IP Collateral that is Owned Intellectual Property are valid, subsisting and enforceable; (3) with respect to the IP Collateral that is Owned Intellectual Property, none of those registrations or applications have lapsed or been abandoned, cancelled or expired; (4) solely with respect to such registrations or applications within the IP Collateral that is Owned Intellectual Property, such Loan Party has taken commercially reasonable steps to maintain such registrations or applications, including by paying filing fees and submitting responses prior to final deadlines; and (5) solely with respect to such registrations or applications within the IP Collateral that is Owned Intellectual Property, each individual (including, to the Borrower's knowledge, individuals not employed by a Loan Party) associated with the filing and prosecution of such registrations or applications, including the named inventors, has complied in all material respects with all applicable duties of candor and good faith in dealing with any patent office, including the USPTO, in those jurisdictions where such duties exist. The Borrower shall update Schedule 5.23(c) as of the

date of borrowing of any Delayed Draw Loans, and shall make the foregoing representations with respect to, all IP Collateral.

(d) Other than those listed in Schedule 5.23(d), there is no opposition, interference, reexamination, inter partes review, post-grant review, derivation or other post-grant proceeding, injunction, claim, suit, action, subpoena, hearing, inquiry, investigation (by the International Trade Commission or otherwise), complaint, arbitration, mediation, demand, decree or other dispute, proceeding or claim (collectively, "Disputes") to which a Loan Party or a Subsidiary is a party or in which any of the Licensed Intellectual Property or Owned Intellectual Property is involved (or, to the Borrower's knowledge, to which a Loan Party or a Subsidiary is not a party) that is pending or, to Borrower's knowledge, currently threatened, that challenges the legality, scope, validity, enforceability, infringement, ownership, inventorship or other rights with respect to any of the IP Collateral, except, in each case, as may arise in the ordinary, day-to-day course of prosecution of intellectual property applications and registrations. As of the Closing Date, no Loan Party or Subsidiary has received any written notice that there is any, and to the Borrower's knowledge there is no, Person who is or claims to be an inventor under any Patent included in the IP Collateral who is not a named inventor thereof. The Borrower shall update Schedule 5.23(d) as of the date of borrowing of any Delayed Draw Loans.

(e) To the knowledge of the Borrower, neither the Specified Products Business of the Loan Parties as currently conducted, nor the discovery, development, manufacture, use, import, export or commercialization of any Specified Product or any other product, or related service, process, method, substance, part or other material now used by any Loan Party or any of its Subsidiaries in the Specified Products Business or otherwise infringes, misappropriates or otherwise violates any IP Rights held by any other Person in any material respect,. There is no pending or, to the knowledge of the Borrower, threatened, and, to the knowledge of the Borrower, no event has occurred or circumstance exists that (with or without notice or lapse of time, or both) would reasonably be expected to give rise to or serve as a basis for any reasonable action, suit, or proceeding, or any investigation or claim by any Person that claims or alleges that the discovery, development, manufacture, use, import, export or commercialization of any Specified Product anywhere in the world infringes on any Patent or other IP Rights of any other Person or constitutes misappropriation of any other Person's trade secrets or other Intellectual Property rights in any material respect. To the knowledge of the Borrower, no slogan or other advertising device, product, process, method, substance, part or other material now employed by any Loan Party or any Subsidiary in connection with the Specified Products Business infringes upon any rights held by any other Person in any material respect. Except as would not reasonably be expected to have a Material Impact, to the Borrower's knowledge, no third party is infringing, misappropriating or otherwise violating any IP Collateral owned or used by any Loan Party, or any of their respective licensees.

(f) Except as disclosed in Schedule 5.23(f), no Loan Party or any of its Subsidiaries has entered into any Contractual Obligation (other than this Agreement and the other Loan Documents) (i) creating a lien, charge, security interest or other encumbrance on, the IP Collateral (other than Permitted Licenses entered into after the Closing Date) or any royalties on, or proceeds from, sales of any Specified Product, (ii) pursuant to which a Loan Party or any of its Subsidiaries has sold, transferred, assigned or pledged to any Person royalties on, or proceeds from, sales of any Specified Product or (iii) except in connection with a Permitted Acquisition, providing for milestone payments or similar development, commercialization or intellectual property-related payments by a Loan Party to any Person applicable (or that with further development and commercialization may become applicable) to any Specified Product. To the knowledge of the

Borrower, no Third Party has created a lien, charge, security interest or other encumbrance on, the IP Collateral (other than Permitted Licenses entered into after the Closing Date).

(g) The Borrower owns, has a valid license or rights in any other form to all rights associated with the Owned Intellectual Property and Licensed Intellectual Property (as applicable), and the Borrower owns the Owned Intellectual Property and holds, to the knowledge of the Borrower, its rights under the Licensed Intellectual Property, in each case of Licensed Intellectual Property, that constitutes IP Collateral, in each case, free and clear of any and all Liens (other than Permitted Licenses entered into after the Closing Date) and, in the case, of the Owned Intellectual Property, any ownership interest of any other person. No Owned Intellectual Property or Licensed Intellectual Property that constitutes IP Collateral has been forfeited, cancelled, or abandoned due to any act or omission of a Borrower or any of its Subsidiaries, other than immaterial IP Collateral abandoned in the ordinary course of business consistent with past practice and the practices of similarly-sized companies in the same industry as Borrower and its Subsidiaries.

(h) With respect to each license agreement listed on Schedule 5.23(b), such license agreement (i) is in full force and effect and is binding upon and enforceable against the Borrower and the Subsidiaries party thereto and, to the knowledge of the Borrower, all other parties thereto in accordance with its terms, (ii) has not been amended or otherwise modified, except as set forth on Schedule 5.23(b), and (iii) has not, to the knowledge of the Borrower, suffered a default or breach thereunder. To the knowledge of the Borrower, none of the Borrower or any of the Subsidiaries has taken or omitted to take any action that would permit any other Person party to any such license agreement to have, and no such Person otherwise has, any defenses, counterclaims, termination rights or rights of setoff thereunder.

(i) None of the Borrower or any of its Subsidiaries has received written notice from any Third Party alleging that the conduct of its business (including the development, manufacture, use, sale or other commercialization of any Specified Product) infringes, violates misappropriate or conflicts with (or may infringe, misappropriate, violate or conflict with) any Intellectual Property of that Third Party, in each case, (x) as of the Closing Date, in any respect and (y) as of the date of borrowing of any Delayed Draw Loans, in any material respect with respect to the conduct of its business related to any Specified Product.

5.24 Labor Matters.

As of the Closing Date, there are no collective bargaining agreements or Multiemployer Plans covering the employees of the Borrower or any of its Domestic Subsidiaries as of the Closing Date and neither the Borrower nor any Subsidiary has suffered any strikes, walkouts, work stoppages or other material labor difficulty within the last five (5) years preceding the Closing Date.

5.25 Regulatory Matters.

(a) Each of the Borrower and the Subsidiaries has all Permits from the FDA or other Governmental Authority necessary or required to conduct its business as currently conducted (including for the manufacture, distribution, and sale of the Specified Products), and each such Permit is valid and subsisting in full force and effect, and there are no breaches, violations, or defaults thereunder, in each case, except where the failure to do so would not reasonably be expected to be material to the Borrower and its Subsidiaries, taken as a whole. Neither the Borrower nor the Subsidiaries has received written notice that the FDA or any other Governmental Authority is considering, limiting, suspending, or revoking such Permits, alleging any deficiencies, breaches, violations, or defaults under such Permits, or changing the marketing classification or labeling of

any Specified Products under such Permits that, in each case, would reasonably be expected to be material to the Borrower and its Subsidiary, taken as a whole. To the knowledge of the Borrower and the Subsidiaries,, (i) there is no fact, situation, circumstance, condition or other basis exists which, with notice or lapse of time or both, would constitute a breach, violation or default under such Permit or give any Governmental Authority grounds to suspend, revoke or terminate any such Permit, other than expiration of any such Permit, (ii) there is no false or materially misleading information or significant omission in any Specified Product application or other required notification, submission or report to the FDA or other Governmental Authority that was not corrected by subsequent submission, (iv) all fees and charges presently due with respect to such Permits have been paid in full, and each of the Borrower and the Subsidiaries has timely submitted all renewal applications, reports, registration and other documents required to be filed, paid all taxes, fees and other assessments, and taken such other steps reasonably necessary to maintain all such Permits in full force and effect, and (v) there are no open surveys with respect to any of the Permits for which the appropriate Governmental Authority or other Person has not accepted a plan of correction from the Borrower or any Subsidiary, as applicable, except, in each case of the foregoing, as would not reasonably be expected to be material to the Borrower and its Subsidiaries, taken as a whole. Neither Borrower nor the Subsidiaries has failed to fulfill and perform its obligations which are due under each such Permit, and no event has occurred which would constitute a breach or default by a Borrower or the Subsidiaries under any such Permit, in each case that would reasonably be expected to be material to Borrower and its Subsidiaries, taken as a whole. To the knowledge of the Borrower and the Subsidiaries, any Third Party that is engaged by the Borrower or any Subsidiary in Health Care Activities related to the Specified Products is in compliance in all material respects with all applicable Health Care Laws and Permits insofar as they pertain to the Specified Products except as would not reasonably be expected to be material to Borrower and its Subsidiaries, taken as a whole. The transactions contemplated by the Loan Documents (or contemplated by the conditions to effectiveness of any Loan Document) will not impair the Borrower's or any Subsidiary's ownership of or rights under (or the license or other right to use, as the case may be) any Permits relating to the Specified Products.

(b) All Health Care Activities related to the Specified Products have been and are in material compliance in all respects with the Health Care Laws. Except as would not reasonably be expected to result in a Material Impact, the Borrower and its Subsidiaries have filed or maintained with the applicable Governmental Authorities all notices, documents, listings, supplemental applications or notifications, reports, submissions, and other filings required under the Health Care Laws, including annual reports, adverse event reports, advertising and promotional material submissions, and clinicaltrials.gov registrations and reports, and each such filing was true, complete and correct as of the date of submission, and the Borrower and its Subsidiaries have submitted any necessary or required updates, changes, corrections, amendments, supplements or modifications to such filings. All manufacturing facilities owned or operated by the Borrower or any of the Subsidiaries, or used in the production of any Specified Product are and have been operated in material compliance with Health Care Laws, including without limitation 21 C.F.R. Parts 210 and 211, except as would not reasonably be expected to have a Material Adverse Effect.

(c) Except as set forth on Schedule 5.25(c), as of the Closing Date, there have been no recalls, withdrawals, removals, field alerts, "dear doctor" letters, investigator notices, safety alerts, or other notices of action relating to an alleged lack of safety, efficacy, or regulatory compliance of, the Specified Products. To the knowledge of the Borrower and the Subsidiaries, no Specified Products have been adulterated or misbranded and there are no defects in the formulation of any approved Specified Products that are reasonably expected to prevent the safe and effective performance of any such Specified Product for its intended use (other than such limitations specified in the applicable package insert), in each case, except as would not reasonably be expected

to have a Material Impact. None of the Specified Products has been the subject of any products liability or warranty action against the Borrower nor any Subsidiary, in each case, except as would not reasonably be expected to have a Material Impact.

(d) The preclinical and clinical studies conducted by or on behalf of the Borrower and the Subsidiaries with respect to the Specified Products, were, and, if still pending, are being, conducted in all material respects in accordance with the Health Care Laws and applicable standards for products or product candidates comparable to those being developed by the Borrower and the Subsidiaries, including without limitation 21 C.F.R. Parts 11, 50, 54, 56, 58, and 312, except as would not reasonably be expected to have a Material Impact. Except as set forth on Schedule 5.25(d), neither the Borrower nor any Subsidiary has received any written notices from the FDA or other Governmental Authority or from any institutional review board or comparable authority requesting or requiring the termination, suspension, material modification, or clinical hold of, or alleging material noncompliance with any Health Care Law related to, any clinical studies with respect to the Specified Products.

(e) Except as set forth on Schedule 5.25(e), as of the Closing Date, neither the Borrower nor any Subsidiary, nor, to the knowledge of the Borrower, their respective suppliers, has received any written notice or communication from the FDA or other Governmental Authority relating to any Specified Products alleging material noncompliance with any Health Care Law, including without limitation any Form FDA 483, notice of inspectional observation, notice of adverse finding, notice of violation, warning letters, untitled letters or other notices. Except as would not reasonably be expected to result in a Material Impact, no Specified Product has been seized, withdrawn, detained, or subject to a suspension of research, manufacturing, distribution or commercialization activity imposed by a Governmental Authority.

(f) As of the Closing Date, neither the Borrower nor any Subsidiary, nor any of their respective officers, directors, nor, to the knowledge of the Borrower and the Subsidiaries, any of their employees, or agents, is or has, within the past five (5) years, been (i) debarred, suspended or excluded, or charged with or convicted of any crime or engaged in any conduct that would (A) result in, or would reasonably be expected to result in, a debarment, suspension or exclusion by FDA or from any Government Reimbursement Program or (B) relate to the delivery of an item or service under any Government Reimbursement Program, (ii) the recipient of a civil monetary penalty under 42 U.S.C. §1320a-7a or any judgment, stipulation, order or decree of, or criminal or civil fine or penalty imposed by, any Governmental Authority related to fraud, theft, embezzlement, breach of fiduciary responsibility, financial misconduct, or obstruction of an investigation; (iii) listed on the list of parties excluded from federal procurement programs and non-procurement programs as maintained in the Government Services Administration's System for Award Management; (iv) the subject of or the target of, any existing, or, to the knowledge of the Borrower or any Subsidiary, potential investigation relating to (A) any Government Reimbursement Program-related offense or (B) any violation of Laws related to fraud, theft, embezzlement, breach of fiduciary responsibility, financial misconduct, obstruction of an investigation; or (v) has engaged in any activity that is in violation of, or is cause for civil penalties or mandatory or permissive exclusion under federal or state Law. Consistent with the requirements and limitations of Applicable Law or industry best practices, the Borrower and each Subsidiary regularly screens officers, employees, contractors and agents for such convictions, exclusions, suspensions, debarments or restrictions.

(g) As of the Closing Date, and for the past five (5) years, neither the Borrower nor any Subsidiary, nor any of their respective directors or officers, nor to the knowledge of the Borrower or any Subsidiary, any employee or contractor, is or has been a party or bound by to nor

has any ongoing reporting obligations pursuant to any individual integrity agreement, corporate integrity agreement, corporate compliance agreement, deferred prosecution agreement, monitoring agreement, consent decree, settlement order, plan of correction or similar agreement imposed by any Governmental Authority concerning compliance with Health Care Laws, any Government Reimbursement Program or the requirements of any Permit and to the knowledge of the Borrower and the Subsidiaries, no such agreement is currently contemplated, proposed or pending.

(h) There is no arrangement with, or, to the knowledge of the Borrower and the Subsidiaries, relating to, the Borrower or the Subsidiaries providing for any rebates, kickbacks or other forms of compensation that are unlawful to be paid to any Person in return for the referral of business or for the arranging or recommending of such referrals. None of the Borrower and the Subsidiaries has received any written notice from the United States Department of Justice, any U.S. Attorney, any State Attorney General, or other Governmental Authority alleging any violation of any Health Care Laws, the Foreign Corrupt Practices Act, any applicable federal Laws, or similar state or foreign Laws.

(i) During the last five (5) years, no right of the Borrower or any of the Subsidiaries to sell products for which reimbursement is available by any Government Reimbursement Program or Private Program has ever been terminated or otherwise adversely affected as a result of any investigation or enforcement action, whether by any Governmental Authority or other Third Party, and none of the Borrower or any Subsidiary has to its knowledge during such period been the subject of any inspection, investigation, or audit, by any Governmental Authority for the purpose of any alleged improper activity or is aware of any facts which could give rise to such action.

(j) Except as would not reasonably be expected to result in a Material Impact, the Borrower and its Subsidiaries are in compliance with all applicable Health Care Laws relating to Product pricing, price reporting, discounts and rebates. Each of the Borrower and its Subsidiaries: (i) holds, and has held for all periods in which required, valid Medicaid Drug Rebate Program National Drug Rebate Agreements, 340B Pharmaceutical Pricing Agreements, and U.S. Department of Veterans Affairs and Federal Supply Schedule Master Agreements and Pharmaceutical Pricing Agreements, and (ii) is in material compliance with all such agreements, including, without limitation, provisions in such agreements pertaining to the timely and accurate submission of pricing data to Governmental Authorities. Except as would not reasonably be expected to result in a Material Impact, the Borrower and its Subsidiaries have maintained and timely submitted, filed, or furnished, all required reports, documents, records, claims, notices, registrations, pricing and other product-related submissions, or other filings pertaining to government pricing and price reporting, including any updates, changes, corrections, amendments, restatements, supplements or modifications to such filings, required by Governmental Authorities or required pursuant to a Health Care Law ("Price Reporting Submissions"). All such Price Reporting Submissions were true and complete in all material respects on the date submitted, as applicable. None of the Borrower or any of its Subsidiaries has received any inquiries from any Government Authority or representatives of the same related to its Product pricing.

(k) There are no material Actions, or, to the knowledge of the Borrower or Subsidiary as applicable, any material Actions threatened in writing, against or affecting Borrower or any Subsidiary, relating to or arising under any Health Care Laws. None of the Borrower or any Subsidiary has received notice from any third party, including employees, third-party vendors, former employees or competitors alleging that any operation or activity of the Borrower or any Subsidiary or with respect to a third-party vendor, any Health Care Activities provided by such vendor on behalf of the Borrower or the Subsidiaries, is in violation of any Health Care Laws or other Applicable Law. Without limiting the generality of the foregoing, to the knowledge of the

Borrower or Subsidiary as applicable, no Person has filed against the Borrower or any of its Subsidiaries any legal action under any federal or state whistleblower statute, including under the civil False Claims Act (31 U.S.C. § 3729 et seq.), or threatened to file any such action to the extent related to any actual material non-compliance or violation of Applicable Law by the Borrower or its Subsidiaries.

(l) None of the Borrower or its Subsidiaries is a "covered entity" or a "business associate" pursuant to HIPAA (as those terms are defined in 45 C.F.R. §160.103) or has entered into a business associate agreement (BAA) under HIPAA, except the BAAs entered into from time to time at the request of a counterparty in the ordinary course of business, which are listed on Schedule 5.25(l). To the extent that the Borrower or any Subsidiary has entered into such BAAs, the Company operates in strict compliance with the same.

(m) Each of the Borrower and each Subsidiary that is an operating entity has an operational healthcare compliance program that (i) governs all employees and contractors, including sales representatives; (ii) is consistent in all material respects with the current U.S. Federal Sentencing Guidelines standards for effective compliance programs; (iii) materially complies with the Pharmaceutical Research and Manufacturers of America Code on Interactions with Healthcare Professionals and any similar codes or standards promulgated by similar organizations outside the U.S., including but not limited to European Federation of Pharmaceutical Industries and the International Federation of Pharmaceutical Manufacturers and Associations; and (iv) reasonably addresses compliance with all applicable material Health Care Laws. Each of the Borrower and the Subsidiaries further operates in material compliance with such healthcare compliance program. The Borrower regularly seeks advice of outside legal counsel knowledgeable of Health Care Laws on its Health Care Activities and undertakes such activities materially consistent with applicable Health Care Laws, its compliance program, and such advice.

(n) Except as would not reasonably be expected to result in a Material Impact, each of the Borrower and its Subsidiaries has developed and implemented patient or product support activities, such as (but not limited to) free genetic testing and counseling, co-pay assistance, access and insurance reimbursement support, compassionate use provision of medicinal products without cost, care coordination services, and physician specialist locator services, with the advice of outside legal counsel knowledgeable of Health Care Laws and undertakes such activities consistent with that advice. Each of the Borrower and its Subsidiaries represents and warrants that: (i) in providing any donations or other financial support to a third party that provides financial assistance to patients in meeting the costs of clinical care, including copayment and deductible costs for drugs, it has complied in all material respects with all applicable Health Care Laws, and guidance of Governmental Authorities for such support, (ii) it has implemented effective compliance policies and procedures to ensure that such financial interactions are compliant in all material respects with applicable Health Care Laws and current guidance of Governmental Authorities; (iii) it has operated materially consistent with such policies and procedures and (iv) it has not received any communication from any third party, including any Governmental Authority, that has raised potential compliance concerns about such financial interactions or indicated that the financial interactions are under investigation.

(o) The Borrower and the Subsidiaries has delivered or made available to the Lenders (i) all material Permits from the FDA or other Governmental Authority for the Specified Products, including all supplements and amendments thereto, and (ii) all material correspondence submitted to or received from FDA or other Governmental Authority concerning such Permits and the Specified Products.

5.26 Key Contracts.

As of the Closing Date, set forth on Schedule 5.26 is a list, and reasonably detailed description of, all Key Contracts of the Loan Parties. As of the date of the borrowing of any Delayed Draw Loans, set forth on Schedule 5.26 (as updated from time to time) is a list, and reasonably detailed description of, all Key Contracts of the Loan Parties entered into following the Closing Date. Each Key Contract (a) is in full force and effect and is binding upon and enforceable against the applicable Loan Party and, to such Loan Party's knowledge, each other Person that is a party thereto in accordance with its terms, (b) has not been otherwise amended or modified (other than amendments or modifications permitted by Section 7.18), and (c) no notice of material default or termination has been provided to the applicable Loan Party, or to such Loan Party's knowledge, each other Person party to such has been delivered such a notice, and no material default or other material circumstance giving rise to a right to terminate (or to non-renew) by a party to any such Key Contract exists. The Borrower has provided to the Lenders full, complete and correct copies of the Key Contracts (including all exhibits and schedules thereto). With respect to Key Contracts, (v) Borrower is in compliance with its obligations under the Key Contracts in all material respects, including timely payments thereunder, (w) no audit has been conducted and no claim for indemnification made except, following the Closing Date, routine audits and claims not giving rise to or evidencing, any material default thereunder, (x) no party thereto has given notice of an intention to terminate or not renew the applicable agreement (except to the extent the replacement thereof would not result in an Event of Default pursuant to Section 8.01(q)) and (y) there currently are no material disputes or threatened material disputes.

5.27 **Loan Parties**.

As of the Closing Date, no Excluded Subsidiary holds any assets or property that, if held by a Loan Party, would constitute Collateral.

5.28 **Irish Companies Act and Other Legislation**.

(a) No Loan Party which is a party to an Irish Security Document or has otherwise created a Lien over any asset situate in Ireland pursuant to the Security Documents is a relevant external company, as that term is defined in section 1301 of the Companies Act (Ireland).

(b) Each Irish Loan Party is a member of the same group of companies consisting of a holding company and its subsidiaries (each within the meaning of section 8 of the Companies Act (Ireland) for the purposes of section 239 of the Companies Act (Ireland).

5.29 **Centre of Main Interests**.

For the purposes of the EU Insolvency Regulation, the centre of main interest (as that term is used in Article 3(1) of the EU Insolvency Regulation) of each Loan Party which is organized or incorporated under the laws of a member state of the European Union is situated in its jurisdiction of incorporation and, as of the Closing Date, it has no "establishment" (as that term is used in Article 2(10) of the EU Insolvency Regulation) in any other jurisdiction.

ARTICLE VI

AFFIRMATIVE COVENANTS

Each of the Loan Parties hereby covenants and agrees that on the Closing Date and thereafter until the Facility Termination Date, such Loan Party shall, and shall cause each of its Subsidiaries to:

6.01 Financial Statements.

Deliver to the Administrative Agent (for distribution to each Lender):

(a) Audited Financial Statements. As soon as available, but in any event within ninety (90) days after the end of each fiscal year of the Borrower, a Consolidated balance sheet of the Borrower and its Subsidiaries as at the end of such fiscal year, and the related Consolidated statements of operations and comprehensive income (loss), changes in shareholders' equity and cash flows for such fiscal year, setting forth in each case in comparative form the figures for the previous fiscal year, all in reasonable detail and prepared in accordance with GAAP, audited and accompanied by a report and opinion of Ernst & Young LLP or any other independent certified public accountant selected by the Borrower of nationally recognized standing or that is otherwise reasonably acceptable to the Required Lenders, which report and opinion shall be prepared in accordance with generally accepted auditing standards and shall not be subject to any "going concern" or like qualification or exception or any qualification or exception as to the scope of such audit other than solely with respect to, or resulting from, the maturity date of the Loans occurring within one (1) year from the time such opinion is delivered.

(b) Quarterly Financial Statements. As soon as available, but in any event within forty-five (45) days after the end of each fiscal quarter of the Borrower, (i) a Consolidated balance sheet of the Borrower and its Subsidiaries as at the end of such fiscal quarter, and the related Consolidated statements of operations and comprehensive loss, changes in shareholders' equity and cash flows for such fiscal quarter and for the portion of the Borrower's fiscal year then ended, setting forth in each case in comparative form the figures for the corresponding fiscal quarter of the previous fiscal year and the corresponding portion of the previous fiscal year, all in reasonable detail and prepared in accordance with GAAP, in each case, such Consolidated statements to be certified by the chief executive officer, chief financial officer, treasurer or controller who is a Responsible Officer of the Borrower as fairly presenting, in all material respects, the financial condition, results of operations and comprehensive loss, shareholders' equity and cash flows of the Borrower and its Subsidiaries, subject only to normal year-end audit adjustments and the absence of footnotes, and (ii) any quarterly plans and updated forecasts, solely to the extent internally prepared.

6.02 Certificates; Other Information.

Deliver to the Administrative Agent (for distribution to each Lender):

(a) Compliance Certificate. Concurrently with the delivery of the financial statements referred to in Sections 6.01(a) and (b), a duly completed Compliance Certificate signed by a Responsible Officer of the Borrower.

(b) SEC Notices. Promptly (but in any event within ten (10) Business Days after receipt thereof by any Loan Party or any Subsidiary thereof), copies of each notice or other correspondence received from the SEC concerning any investigation or possible investigation by

such agency regarding financial or other operational results of any Loan Party or any Subsidiary thereof that would reasonably be expected to result in a Material Adverse Effect.

(c) Environmental Notice. Promptly (but in any event within ten (10) Business Days after receipt thereof by any Loan Party or any Subsidiary thereof) after any Responsible Officer of a Loan Party becomes aware of the assertion or occurrence thereof, (i) any Release required to be reported by any Loan Party or any of its Subsidiaries to any Governmental Authority under any applicable Environmental Laws, and any remedial actions related thereto; and (ii) any notice of any Environmental Claim received by any Loan Party or any of its Subsidiaries of any noncompliance by any Loan Party or any of its Subsidiaries with any applicable Environmental Law or Environmental Permit, or that causes any property described in the Mortgages to be subject to any material restrictions on ownership, occupancy, use or transferability under any Environmental Law, in each case, that could reasonably be expected to result in a Material Adverse Effect.

(d) Anti-Money-Laundering; Beneficial Ownership Regulation. Promptly following any request therefor, information and documentation reasonably requested by the Administrative Agent, the Required Lenders or any Lender for purposes of compliance with applicable "know your customer" and anti-money-laundering rules and regulations, including, without limitation, the Patriot Act.

(e) Beneficial Ownership. To the extent any Loan Party qualifies as a "legal entity customer" under the Beneficial Ownership Regulation, an updated Beneficial Ownership Certification promptly (but in any event no later than the later of ten (10) Business Days and the next date a Compliance Certificate is required to be delivered pursuant to Section 6.02(a)) following any change in the information provided in the Beneficial Ownership Certification delivered to any Lender in relation to such Loan Party that would result in a change to the list of beneficial owners identified in such certification.

(f) FDA Notifications. Promptly (but in any event no later than the later of ten (10) Business Days and the next date a Compliance Certificate is required to be delivered pursuant to Section 6.02(a)) after the receipt thereof by any Responsible Officer of the Borrower, details with respect to any (i) "warning letter" or material untitled letter, or (ii) notification of a mandated or requested recall affecting the Specified Products, in each case, from the FDA or other Governmental Authority.

(g) Health Care Laws Notifications. Promptly after any Responsible Officer of the Borrower becomes aware of the assertion or occurrence thereof, any notice from a Governmental Authority alleging non-compliance with any Health Care Law that could reasonably be expected to be materially adverse to the business of the Borrower and its Subsidiaries taken as a whole.

(h) Key Contracts. Notice (i) promptly (but in any event within ten (10) Business Days after receipt thereof by any Loan Party or any Subsidiary thereof) after a Loan Party receives a written notice of default or event of default under any Key Contract (other than immaterial or easily curable defaults or events of defaults which are promptly cured), (ii) promptly (but in any event within ten (10) Business Days after receipt thereof by any Loan Party or any Subsidiary thereof) after a Loan Party receives any written termination notice (or any notice communicating an intent not to renew) under any Key Contract or (iii) promptly (but in any event no later than the later of ten (10) Business Days and the next date a Compliance Certificate is required to be delivered pursuant to Section 6.02(b)), of any new Key Contract is entered into by the Loan Parties, in each case, together with a copy of such notice or new agreement.

(i) Specified Product Rights. Promptly (but in any event within ten (10) Business Days after receipt thereof by any Loan Party or any Subsidiary thereof) after the date any Responsible Officer of the Borrower becomes aware of the assertion or occurrence thereof, written notice of any material Dispute involving any Loan Party and any Specified Product IP or any Collateral.

(j) Additional Information. Promptly, such additional information regarding the business, financial, legal or corporate affairs of any Loan Party or any Subsidiary thereof, or compliance with the terms of the Loan Documents, as the Administrative Agent, the Required Lenders or any Lender may from time to time reasonably request in writing to the extent such information is reasonably available to such Loan Party or any Subsidiary.

Notwithstanding anything to the contrary in this Section 6.02 or any other provision of the Loan Documents, neither the Borrower nor any of its Subsidiaries will be required to disclose or permit the inspection or discussion of, any document, information or other matter (i) in respect of which disclosure (or their respective representatives or contractors) is prohibited by Law or any binding agreement, (ii) that constitutes non-financial trade secrets or non-financial proprietary information, or (iii) that is subject to attorney client or similar privilege or constitutes attorney work product.

Documents required to be delivered pursuant to Section 6.01(a) or (b) (to the extent any such documents are included in materials otherwise filed with the SEC) may be delivered electronically and if so delivered, shall be deemed to have been delivered on the date (i) on which the Borrower posts such documents, or provides a link thereto on the Borrower's website on the Internet at the website address listed on Schedule 1.01(a); or (ii) on which such documents are posted on the Borrower's behalf on an Internet or intranet website, if any, to which each Lender and the Administrative Agent have access (whether a commercial, third-party website or whether sponsored by the Administrative Agent). The Administrative Agent shall have no obligation to request the delivery of or to maintain paper copies of the documents referred to above, and in any event shall have no responsibility to monitor compliance by the Borrower with any such request by a Lender for delivery, and each Lender shall be solely responsible for requesting delivery to it or maintaining its copies of such documents.

The Borrower hereby acknowledges that (i) the Borrower, Administrative Agent and/or an Affiliate thereof may, but shall not be obligated to, make available to the Lenders materials and/or information provided by or on behalf of the Borrower hereunder (collectively, "Borrower Materials") by providing Borrower Materials directly to the Lenders or posting the Borrower Materials on IntraLinks, Syndtrak, DebtDomain, ClearPar or a substantially similar electronic transmission system (the "Platform") and (ii) certain of the Lenders (each, a "Public Lender") may have personnel who do not wish to receive material non-public information with respect to the Borrower or its Affiliates, or the respective securities of any of the foregoing, and who may be engaged in investment and other market-related activities with respect to such Persons' securities. The Borrower hereby agrees, unless otherwise directed by the Required Lenders (but, notwithstanding any such direction from the Required Lenders, without limiting the obligation of the Borrower to notify the Administrative Agent as to whether Borrower Materials delivered to the Administrative Agent can be posted to the portion of the Platform designated "Public Side Information"), that it will use commercially reasonable efforts to identify that portion of the Borrower Materials that may be distributed to the Public Lenders and that (A) all such Borrower Materials shall be clearly and conspicuously marked "PUBLIC" which, at a minimum, shall mean that the word "PUBLIC" shall appear prominently on the first page thereof; (B) by marking Borrower Materials "PUBLIC," the Borrower shall be deemed to have authorized the

Administrative Agent, any Affiliate thereof and the Lenders to treat such Borrower Materials as not containing any material non-public information (although it may be sensitive and proprietary) with respect to the Borrower or its securities for purposes of United States federal and state securities laws; (C) all Borrower Materials marked "PUBLIC" are permitted to be made available by the Borrower directly to the Lenders or by the Administrative Agent through a portion of the Platform designated "Public Side Information;" (D) the Borrower and any Affiliate thereof shall be entitled to deliver any Borrower Materials that are not marked "PUBLIC" as being suitable only for delivery to the designated representative of Lender entitled to receive such non-PUBLIC information, and (E) the Administrative Agent and any Affiliate thereof shall be entitled to treat any Borrower Materials that are not marked "PUBLIC" as being suitable only for posting on a portion of the Platform not designated "Public Side Information" and shall only post such Borrower Materials on a portion of the Platform designated "PRIVATE." Each Lender agrees to designate in writing to Borrower and to Administrative Agent the names and contact information (including email addresses) of one or more representatives entitled to receive Public Side Information and one or more representatives to receive non-PUBLIC information.

6.03 Notices.

Promptly, but in any event within five (5) Business Days, notify the Administrative Agent (for distribution to each Lender) upon obtaining knowledge of:

(a) the occurrence of any Default;

(b) any matter, including, without limitation, (i) breach or non-performance of, or any default under, a Contractual Obligation of the Borrower or any Subsidiary; (ii) any action, suit, dispute, litigation, investigation, proceeding or suspension involving the Borrower or any Subsidiary or any of their respective properties and any Governmental Authority; or (iii) the commencement of, or any material development in, any litigation or proceeding affecting the Borrower or any Subsidiary, including pursuant to any applicable Environmental Laws, in each case of this clause (b), that has resulted or would reasonably be expected to result in a Material Adverse Effect;

(c) the occurrence of any ERISA Event that would reasonably be expected to result in a Material Adverse Effect;

(d) any occurrence of any Disposition of property or assets for which the Borrower is required to make a Mandatory Prepayment pursuant to Section 2.03(b)(i) or Section 2.03(b)(ii);

(e) (i) any written notice received by the Borrower from any Governmental Authority alleging any potential or actual violations of any Health Care Law by the Borrower, (ii) any written notice that the FDA or other Governmental Authority is limiting, suspending or revoking any Key Permit, (iii) any written notice that the Borrower has become subject to any administrative or regulatory enforcement action, proceeding or investigation issued by the FDA or other Governmental Authority, (iv) notice of the exclusion or debarment from any governmental healthcare program or debarment or disqualification by FDA of the Borrower, (v) any written notice that FDA or other Governmental Authority is changing the market classification or labeling under any such Key Permit, or (vi) the receipt of notice, or occurrence of any decision, to conduct a voluntary or mandatory recall, withdrawal, removal, suspension of manufacturing or marketing, or discontinuation of any Specified Product, in each case of clauses (i) through (vi), to the extent such

notice would reasonably be expected to result in material and adverse consequences to the Borrower and its Subsidiaries, taken as a whole; and

(f) At any time prior to the date that the Reserve Account is fully funded under Sections 6.20(a) through (e) or, if funded, fully released pursuant to subpart (f) of Schedule 6.20: (A)(i) promptly (but in any event within ten (10) Business Days) after the Borrower or any other Loan Party having knowledge of any Third Party having created a lien, charge, security interest or other encumbrance on, any of the Intellectual Property which is licensed under the [**] (other than in connection with any event described in the following clause (B), which are subject to the notice provisions thereof) and (B) promptly after any [**], and in any event within three (3) Business Days after the occurrence of such transaction, notice of the occurrence of such transaction; *provided* that, to the extent that any [**] will, upon its occurrence, create an obligation to fund the Reserve Account under subpart (e) of Schedule 6.20, at least one (1) Business Day before the occurrence of such transaction, notice that such transaction will occur and (ii) at all times thereafter, promptly after any [**], and in any event within three (3) Business Days after the occurrence of such transaction, notice of the occurrence of such transaction.

Each notice pursuant to this Section 6.03 (other than Section 6.03(d) or (e)) shall be accompanied by a statement of a Responsible Officer of the Borrower setting forth details of the occurrence referred to therein and to the extent applicable, stating what action the Borrower has taken and proposes to take with respect thereto.

6.04 Payment of Obligations.

Pay and discharge as the same shall become due and payable, all its obligations and liabilities, including (a) all tax liabilities, assessments and governmental charges or levies upon it or its properties or assets, unless the same are being contested in good faith by appropriate proceedings diligently conducted and adequate reserves in accordance with GAAP are being maintained by the Borrower or such Subsidiary; (b) all lawful claims which, if unpaid, would by law become a Lien upon its property (other than Permitted Liens); and (c) all Indebtedness, as and when due and payable, but subject to any subordination provisions contained in any instrument or agreement evidencing such Indebtedness, in each case, except where the failure to do so would not reasonably be expected to have a Material Adverse Effect.

6.05 Preservation of Existence, Etc.

(a) Preserve, renew and maintain in full force and effect its legal existence and good standing (to the extent such concept is applicable in the corresponding jurisdiction) under the Laws of the jurisdiction of its organization except in a transaction permitted by Section 7.04 or 7.05;

(b) take all reasonable action to maintain all rights, privileges, permits, licenses and franchises necessary or desirable in the normal conduct of its business, except to the extent that failure to do so could not reasonably be expected to have a Material Adverse Effect; and

(c) use commercially reasonable efforts to preserve or renew all of its registered patents, trademarks, trade names and service marks related to the IP Collateral, the non-preservation of which would reasonably be expected to have a Material Adverse Effect.

6.06 Maintenance of Properties.

(a) Except if the failure to do so would not reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect, maintain, preserve and protect all of its

material properties and equipment necessary in the operation of its business in good working order and condition, ordinary wear and tear and casualty and condemnation events excepted; and

(b) make all necessary repairs thereto and renewals and replacements thereof except where the failure to do so could not reasonably be expected to have a Material Adverse Effect.

6.07 Maintenance of Insurance.

(a) Maintenance of Insurance. Maintain with financially sound and reputable (as determined by the Borrower in good faith) insurance companies not Affiliates of the Borrower, insurance with respect to its properties and business against loss or damage of the kinds customarily insured against by Persons engaged in the same or similar business, of such types and in such amounts as are customarily carried under similar circumstances by such other Persons.

(b) Evidence of Insurance. Subject to Section 6.18(b), cause the Administrative Agent to be named as lenders' loss payable, loss payee or mortgagee, as its interest may appear, with respect to property polices and/or additional insured with respect of any such insurance providing general liability coverage, product liability coverage or coverage in respect of any Collateral (other than cyber, directors and officers, professional liability, employment practice liability and workers compensation policies), and cause, unless otherwise agreed to by the Required Lenders, each provider of any such insurance to agree, by endorsement upon the policy or policies issued by it or by independent instruments furnished to the Administrative Agent that it will give the Administrative Agent thirty (30) days prior written notice before any such policy or policies shall be altered or cancelled (or ten (10) days prior notice in the case of cancellation due to the nonpayment of premiums). No more than [**], the Loan Parties shall provide, or cause to be provided, to the Administrative Agent, evidence of insurance, to the extent such evidence is reasonably requested by the Administrative Agent (acting at the written direction of the Required Lenders) or the Required Lenders. Notwithstanding the foregoing, so long as no Event of Default exists, except as required under Section 2.03(b)(i), the Borrower and its Subsidiaries may retain all or any portion of the proceeds of any insurance of Borrower and its Subsidiaries (and Administrative Agent shall promptly remit to Borrower or the applicable Subsidiary any proceeds with respect to such insurance received by Administrative Agent).

6.08 Compliance with Laws.

Comply with the requirements of all Applicable Laws and Privacy Requirements (including applicable Health Care Laws) and all orders, writs, injunctions and decrees applicable to it or to its business or property, except in such instances in which (a) such requirement of Law or order, writ, injunction or decree is being contested in good faith by appropriate proceedings diligently conducted; or (b) the failure to comply therewith would not reasonably be expected to have a Material Adverse Effect.

6.09 Books and Records.

(a) Maintain proper books of record and account, in which full, true and correct entries in all material respects in conformity with GAAP consistently applied shall be made of all financial transactions and matters involving the assets and business of such Loan Party or such Subsidiary, as the case may be; and

(b) maintain such books of record and account in material conformity with all applicable requirements of any Governmental Authority having regulatory jurisdiction over such Loan Party or such Subsidiary, as the case may be.

6.10 Inspection and Access Rights.

(a) Permit representatives of the Administrative Agent or the Required Lenders to visit and inspect any of its properties, to examine its corporate, financial and operating records, and make copies thereof or abstracts therefrom, and to discuss its affairs, finances and accounts with its directors, officers, and independent public accountants (subject to such accountants' customary policies and procedures and after giving the Borrower an opportunity to participate in any such discussions with such accountants), not more than [**] at reasonable times during normal business hours and upon reasonable advance notice to the Borrower; *provided*, *however*, that when an Event of Default exists the Administrative Agent, the Required Lenders or any Lender (or any of their respective representatives or independent contractors) may do any of the foregoing at the expense of the Borrower. Notwithstanding anything to the contrary in this Section 6.10, none of the Borrower and its Subsidiaries will be required to disclose or permit the inspection or discussion of, any document, information or other matter (i) that constitutes non-financial trade secret or non-financial proprietary information, (ii) in respect of which disclosure (or their respective representatives or contractors) is prohibited by Law or any binding agreement or (iii) that is subject to attorney client or similar privilege or constitutes attorney work product.

(b) Not less and not more than [**], at a time mutually agreed with the Required Lenders, after the date on which financial statements were delivered (or required to be delivered) pursuant to Section 6.01(b)), participate in a conference call between senior management of the Borrower and the Required Lenders to discuss the financial condition and results of operations of the Borrower and its Subsidiaries for the most recently-ended fiscal period.

6.11 Use of Proceeds.

Use the proceeds of any Borrowing to fund (i) general corporate purposes and working capital not in contravention of any Loan Document (but not to fund the Reserve Account) and (ii) transaction fees and expenses in connection with this Agreement and such funding.

6.12 Reserved.

6.13 Covenant to Guarantee Obligations.

The Loan Parties will cause each of their Subsidiaries (other than any Excluded Subsidiary) whether newly formed, after acquired or otherwise existing (including any Subsidiary ceasing to be an Excluded Subsidiary) to promptly (and in any event within forty-five (45) days after such Subsidiary is formed or acquired or ceases to be an Excluded Subsidiary (or such longer period of time as agreed to by the Required Lenders in its reasonable discretion)) become a Guarantor hereunder by way of execution of a Joinder Agreement. In connection with the foregoing, the Loan Parties shall deliver to the Administrative Agent and the Required Lenders, with respect to each new Guarantor to the extent applicable, substantially the same documentation required pursuant to Sections 4.01(b) – (d), and 6.14 and such other documents or agreements as the Administrative Agent or the Required Lenders may reasonably request, including without limitation, updated Schedules 5.10, 5.12 and 5.26.

6.14 Covenant to Give Security.

Except with respect to Excluded Property:

(a) Equity Interests and Personal Property. Each Loan Party will cause the Pledged Equity and all of its tangible and intangible personal property that constitutes Collateral now owned or hereafter acquired by it to be subject at all times to a first priority, perfected Lien (subject to Permitted Liens) in favor of the Administrative Agent for the benefit of the Secured Parties to secure the Obligations pursuant to, and as and when required by, the terms and conditions of the Collateral Documents. Each Loan Party shall provide opinions of counsel to the extent reasonably requested by the Required Lenders and any filings and deliveries reasonably necessary in connection therewith to perfect the security interests therein, all in form and substance reasonably satisfactory to the Required Lenders.

(b) Real Property. If any Loan Party acquires a fee ownership interest in any Material Real Property, it shall provide to the Administrative Agent within one hundred eighty (180) days (or such extended period of time as reasonably agreed to by the Required Lenders) a Mortgage and such other support documents as the Administrative Agent (acting at the written direction of the Required Lenders) or the Required Lenders may reasonably request to cause such Material Real Property to be subject to a first priority, perfected Lien (subject in each case to Permitted Liens) in favor of the Administrative Agent for the benefit of the Secured Parties to secure the Obligations pursuant to the terms and conditions of the Collateral Documents.

(c) Account Control Agreements. Subject to Section 6.18, each of the Loan Parties shall not open, maintain or otherwise have any deposit or other accounts (including securities accounts) at any bank or other financial institution, or any other account where money or securities are or may be deposited or maintained with any Person, other than (i) deposit accounts that are maintained at all times with depositary institutions as to which the Administrative Agent shall have received a Qualifying Control Agreement within 30 days after the opening or acquisition thereof (or such extended period of time as reasonably agreed to by the Required Lenders), (ii) securities accounts that are maintained at all times with financial institutions as to which the Administrative Agent shall have received a Qualifying Control Agreement thirty (30) days after the opening or acquisition thereof (or such extended period of time as reasonably agreed to by the Required Lenders), and (iii) Excluded Accounts.

(d) Further Assurances. At any time upon the reasonably written request of the Administrative Agent (acting at the written direction of the Required Lenders) or the Required Lenders, promptly execute and deliver any and all further instruments and documents and take all such other action as the Administrative Agent or the Required Lenders may reasonably deem necessary or desirable to maintain in favor of the Administrative Agent, for the benefit of the Secured Parties, Liens on the Collateral that are duly perfected in accordance with the requirements of, or the obligations of the Loan Parties under, the Loan Documents and all Applicable Laws.

(e) Notwithstanding anything to the contrary in this Agreement or any other Loan Document, in no event shall the Loan Parties be required, nor shall the Administrative Agent be authorized, to take any of the following actions (i) execute, deliver or otherwise obtain any agreement, instrument or documents governed by foreign law, (ii) perfect any pledge, security interest or Mortgage by any means other than by (A) filings pursuant to the UCC in the office of the secretary of state (or similar central filing office) of the relevant states and jurisdictions, (B) filings in the applicable real estate records with respect to any Material Real Property with respect to which a Mortgage has been granted or any fixtures relating to any Material Real Property with

respect to which a Mortgage has been granted to the extent provided in clause (b) above, (C) filings with the USPTO, as applicable, with respect to Intellectual Property, (D) to the extent certificated, delivery of stock certificates and other certificated securities and the applicable transfer powers endorsed in blank and (E) entering into Control Agreements pursuant to clause (c) above or Section 6.18(a), or (iii) to take any actions with respect to any assets not located in the United States (including any Intellectual Property registered or applied for in any jurisdiction outside the United States) or enter into any security document governed by the laws of a jurisdiction other than a jurisdiction within the United States; *provided* that this clause (iii) shall not apply to the Collateral described in clause (ii)(D) above.

6.15 Environmental Matters.

Except as could reasonably be expected to result in liabilities or expenditures of the Loan Parties in excess of the Threshold Amount, (a) comply in all material respects and take commercially reasonable steps to cause all lessees and other Persons operating or occupying its properties utilized in the Specified Products Business to comply in all material respects, with all applicable Environmental Laws and Environmental Permits; (b) obtain and renew all Environmental Permits necessary for its operations and properties utilized in the Specified Products Business; (c) conduct any investigation, study, sampling and testing, cleanup, removal, remedial or other action to remove and clean up all Hazardous Materials from any of its properties utilized in the Specified Products Business to the extent required of any Loan Party under, and in accordance with, all applicable Environmental Laws; and (d) make an appropriate response to any Environmental Claim against such Loan Party.

6.16 Anti-Corruption Laws; Sanctions.

Conduct its business in compliance with the United States Foreign Corrupt Practices Act of 1977, the UK Bribery Act 2010, the Criminal Justice (Corruption Offences) Act 2018 of Ireland, as amended and the Criminal Justice (Money Laundering and Terrorist Financing) Act 2010 to 2021 of Ireland and other applicable anti-corruption legislation in other jurisdictions and with all applicable Sanctions, and maintain policies and procedures designed to promote and achieve compliance with such laws and Sanctions.

6.17 Further Assurances.

(a) Promptly upon the reasonable written request by the Administrative Agent (acting at the written direction of the Required Lenders), the Required Lenders, or any Lender through the Administrative Agent, (a) correct any material defect or error that may be discovered in any Loan Document or in the execution, acknowledgment, filing or recordation thereof, and (b) do, execute, acknowledge, deliver, record, re-record, file, re-file, register and re-register any and all such further acts, deeds, certificates, assurances and other instruments as the Administrative Agent, the Required Lenders, or any Lender through the Administrative Agent, may reasonably require from time to time in order to (i) carry out more effectively the purposes of the Loan Documents, (ii) to the fullest extent permitted by Applicable Law, subject any Loan Party's or any of its Subsidiaries' properties, assets, rights or interests to the Liens now or hereafter intended to be covered by any of the Collateral Documents, (iii) perfect and maintain the validity, effectiveness and priority of any of the Collateral Documents and any of the Liens intended to be created thereunder to the extent provided in Section 6.14 and (iv) assure, convey, grant, assign, transfer, preserve, protect and confirm more effectively unto the Secured Parties the rights granted or now or hereafter intended to be granted to the Secured Parties under any Loan Document or under any other instrument executed in connection with any Loan Document to which any Loan Party is or is to be a party, and cause each of its Subsidiaries to do so.

(b) With respect to any Loan Party organized under the Laws of a Foreign Jurisdiction, the Loan Parties shall deliver to the Administrative Agent, upon the request of the Administrative Agent (acting at the direction of the Required Lenders) each document, agreement, filing and evidence of each action that, in each case, is required or reasonably desirable (in the sole determination of the Administrative Agent, acting at the direction of the Required Lenders) to legally bind such entity as "Loan Party" hereunder and under each applicable Loan Document, and to evidence and enforce the obligations of such Loan Party hereunder and under each Loan Document. In addition, if requested by the Administrative Agent (acting at the direction of the Required Lenders), the Borrower shall deliver to the Administrative Agent and the Lenders each opinion or opinions of reputable counsel to the Borrower (or, if the Administrative Agent shall agree, counsel to the Administrative Agent) licensed to practice in the jurisdiction in which such Loan Party is formed, opining as to the enforceability of any or all of the documentation described in this paragraph.

(c) With respect to any and all Collateral that constitutes Foreign Collateral, the Loan Parties shall (x) promptly notify the Administrative Agent of the existence of such Collateral and (y) to the extent requested by the Administrative Agent (acting at the direction of the Required Lenders) promptly comply with the Foreign Collateral Requirements in relation to such Collateral.

6.18 Post-Closing Covenants.

(a) Qualifying Control Agreements. By the date that is [**] days after the Closing Date, or such later date as agreed to by the Required Lenders, deliver to the Administrative Agent, each Qualifying Control Agreement (other than with respect to the Reserve Account) required to be delivered pursuant to Section 6.14 and Section 6.20 (with respect to the Reserve Account).

(b) Insurance. By the date that is [**] days after the Closing Date, or such later date as reasonably agreed to by the Required Lenders, deliver to the Administrative Agent all endorsements with respect to the insurance certificates delivered to the Administrative Agent on the Closing Date pursuant to Section 4.01(e), in each case meeting the requirements set forth in Section 6.07(b).

(c) Additional Guaranty and Collateral Documentation. By the date that is no later than the date that is [**] days after the Closing Date: (i) deliver to the Administrative Agent each of the documents set forth on Schedule 6.18(c) hereto, in each case, in form and substance satisfactory to the Administrative Agent and the Required Lenders); (ii) deliver to the Administrative Agent solely with respect to each of the Irish Loan Party, the Bermudan Loan Party and the Brazilian Loan Party, each of the documents (other than a Joinder Agreement) required to be delivered, and evidence of each action required to be taken, pursuant to Section 6.13 hereof, as if such entity were an incoming Guarantor, and Section 6.18 hereof; (iii) deliver to the Administrative Agent each signature page to the Global Intercompany Note of each Subsidiary of the Borrower not delivered prior to the Closing Date; and (iv) comply with the Foreign Collateral Requirements with respect to all Collateral constituting Foreign Collateral as of the Closing Date.

6.19 Maintenance, Defense and Enforcement of IP Rights.

Each Loan Party shall take those steps that are commercially reasonable under the circumstances to file, prosecute, maintain, defend and enforce the IP Collateral that is owned or controlled by a Loan Party.

6.20 Reserve Account.

The Borrower shall take the actions (or, as applicable, refrain from the actions) set forth on Schedule 6.20.

6.21 Right of First Negotiation.

(a) Prior to the Borrower or its Subsidiaries engaging with, soliciting proposals from, negotiating with or consummating, any product financing transaction ([**]) (whether in the form of a financing, royalty sale, co-development agreement or otherwise) (any such potential product financing, a "Product Financing"), the Borrower shall first provide written notice to the Required Lenders of its interest in completing such Product Financing (a "Product Financing Notice"). The Product Financing Notice shall include a general description of the assets or products which are the subject of such Product Financing, the amount (or range) of financing which the Borrower is seeking to generate and the proposed timing of the transaction (the "Target Completion Date" of such Product Financing). The Borrower shall not engage with any third party financing source with respect to a given Product Financing transaction, until the earlier of [**] days following delivery of the Product Financing Notice and such earlier date, if any, on which the Required Lenders advises the Borrower that it is not interested in exploring such Product Financing (the "Exclusivity Period"), and, to the extent the Required Lenders advise the Borrower that they wish to explore such Product Financing, will negotiate with them in good faith during the Exclusivity Period in an effort to reach agreement on terms. For the sake of clarity, the Borrower shall have no obligation to enter into any Product Financing with the Required Lenders or its Affiliates, but only to provide a [**]-day review period prior to engaging with a third party on such transaction.

(b) Each new product financing transaction under consideration by the Borrower or its Subsidiaries shall require a Product Financing Notice, regardless whether a prior Product Financing Transaction was completed with the Required Lenders or its Affiliates; provided, that a Product Financing that was the subject of a prior Product Financing Notice which is not completed by the applicable Target Completion Date shall be treated as a new product financing, and a new Product Financing Notice and a new 30 day review period shall be required.

6.22 Financial Assistance.

Each Irish Loan Party shall comply in all respects with section 82 of the Companies Act (Ireland), including in relation to the execution of the Loan Documents to which it is a party and performance of its obligations under such Loan Documents.

6.23 Centre of Main Interests.

No Irish Loan Party shall, without prior written consent of Administrative Agent (acting at the direction of the Required Lenders): (i) deliberately cause or allow its centre of main interests (as such term is used in Article 3(1) of the the EU Insolvency Regulation) to change or (ii) to have an "establishment" (as that term in used in Article 2(10) of the EU Insolvency Regulation) in any other jurisdiction, to the extent such matters would materially adversely affect the Lenders.

ARTICLE VII
NEGATIVE COVENANTS

Each of the Loan Parties hereby covenants and agrees that on the Closing Date and thereafter until the Facility Termination Date, no Loan Party shall, nor shall it permit any Subsidiary to, directly or indirectly:

7.01 Liens.

Create, incur, assume or suffer to exist any Lien upon any of its property or assets, whether now owned or hereafter acquired, except for the following (the "*Permitted Liens*"):

(a) Liens pursuant to any Loan Document;

(b) Liens existing on the Closing Date and listed on Schedule 7.01 and any renewals or extensions thereof, *provided* that (i) the property covered thereby is not changed, (ii) the amount secured or benefited thereby is not increased except as contemplated by Section 7.02(b) and (iii) any renewal, refinancings, replacements or extension of the obligations secured or benefited thereby is permitted by Section 7.02(b);

(c) Liens for Taxes, assessments or other governmental charges or levies not yet due and not yet overdue for 30 days or which are being contested in good faith and by appropriate proceedings diligently conducted, if adequate reserves with respect thereto are maintained on the books of the applicable Person in accordance with GAAP for property Taxes on property such Person or one of its Subsidiaries has determined to abandon if the sole recourse for such Tax, assessment, charge, levy or claim is to such property;

(d) Statutory Liens such as carriers', warehousemen's, landlords', mechanics', materialmen's, repairmen's, construction contractors', airports', navigation authority's or other like Liens arising in the ordinary course of business which are not overdue for a period of more than sixty (60) days or which are being contested in good faith and by appropriate proceedings diligently conducted (or which, if due and payable, are being contested in good faith by appropriate proceedings and for which adequate reserves are being maintained, to the extent required by GAAP); *provided* that adequate reserves with respect thereto are maintained on the books of the applicable Person;

(e) pledges or deposits in the ordinary course of business in connection with workers' compensation, unemployment insurance and other social security legislation, other than any Lien imposed by ERISA;

(f) deposits to secure the performance of bids, tenders, trade contracts and leases (other than Indebtedness), statutory obligations, surety, stay, customs and appeal bonds, performance bonds, or as security for contested taxes or import duties or for the payment of rent, and other obligations of a like nature incurred in the ordinary course of business;

(g) Liens in favor of the issuers of performance and surety bonds, bid, indemnity, warranty, release, appeal or similar bonds or with respect to regulatory requirements or letters of credit or bankers' acceptances issued and completion of guarantees provided for, in each case, pursuant to the request of and for the account of the Borrower or any Subsidiary in the ordinary course of its business;

(h) survey exceptions, non-monetary encumbrances, easements or reservations of, or rights of others for, licenses, rights-of-way, servitudes, sewers, electric lines, drains, telegraph and telephone and cable television lines, gas and oil pipelines and other similar purposes, reservations of rights or zoning, building codes or other restrictions (including, without limitation, minor defects or irregularities in title and similar encumbrances) affecting real property or incidental to the conduct of business of the applicable Person or to the ownership of its properties, which, in the aggregate, do not in any case materially interfere with the ordinary conduct of the business of the Borrower or its Subsidiaries and do not materially detract from the value thereof;

(i) Liens securing judgments for the payment of money (or appeal or other surety bonds relating to such judgments) not constituting an Event of Default under Section 8.01(h);

(j) Liens securing Indebtedness permitted under Section 7.02(c) or (k); *provided* that (i) such Liens do not at any time encumber any property other than the property financed by such Indebtedness and (ii) the Indebtedness secured thereby does not exceed the cost or fair market value, whichever is lower, of the property being acquired on the date of acquisition;

(k) bankers' Liens, rights of setoff and other similar Liens existing solely with respect to cash and Cash Equivalents on deposit in one or more accounts maintained by the Borrower or any of its Subsidiaries with any Lender, in each case in the ordinary course of business in favor of the bank or banks with which such accounts are maintained, securing solely the customary amounts owing to such bank with respect to cash management and operating account arrangements; *provided*, that in no case shall any such Liens secure (either directly or indirectly) the repayment of any Indebtedness;

(l) Liens arising out of judgments or awards not resulting in an Event of Default; *provided* the applicable Loan Party or Subsidiary shall in good faith be prosecuting an appeal or proceedings for review;

(m) Any interest or title of a lessor, licensor or sublessor under any lease, license, sublease, sublicense, occupancy agreement or assignment of or in respect of real or personal property and covering only those assets so leased, subleased, licensed or sublicensed;

(n) Liens of a collection bank arising under Section 4–210 of the UCC on items in the course of collection;

(o) Liens on property of a Person existing at the time such Person acquired the property or the Person is merged into or consolidated with the Borrower or any Subsidiary of the Borrower or becomes a Subsidiary of the Borrower; *provided* that such Liens were not created in contemplation of such merger, consolidation or Investment and do not extend to any assets other than those of the Person merged into or consolidated with the Borrower or such Subsidiary or acquired by the Borrower or such Subsidiary, and the applicable Indebtedness secured by such Lien is permitted under Section 7.02(t);

(p) Liens securing Indebtedness permitted under Section 7.02(f) or (j);

(q) any zoning, building or similar laws or rights reserved to or vested in any Governmental Authority;

(r) other Liens securing Indebtedness in an aggregate secured principal amount not to exceed $[**] at any time outstanding; *provided* that any such Liens on the Collateral will be

subordinated to the Liens securing the Obligations pursuant to an intercreditor agreement satisfactory to Required Lenders providing for such subordination and containing other customary terms;

(s) Liens on specific items of inventory or other goods and proceeds of the Borrower or a Subsidiary securing such Person's obligations in respect of bankers' acceptances or letters of credit entered into in the ordinary course of business issued or created for the account of such Person to facilitate the purchase, shipment or storage of such inventory or other goods;

(t) Liens arising from, or from UCC financing statement filings regarding, operating leases or consignments entered into by the Borrower or its Subsidiaries in the ordinary course of business;

(u) deposits made or other security provided in the ordinary course of business to secure liability to insurance carriers or under self-insurance arrangements in respect of such obligations;

(v) Liens arising out of conditional sale, title retention, consignment or similar arrangements for the sale of goods entered into in the ordinary course of business;

(w) Liens in favor of customs and revenue authorities arising as a matter of law to secure payment of customs duties in connection with the importation and exportation of goods in the ordinary course of business;

(x) Liens (i) of a collection bank arising under Section 4-210 of the UCC, or any comparable or successor provision, on items in the course of collection; (ii) attaching to pooling, commodity trading accounts or other commodity brokerage accounts incurred in the ordinary course of business; and (iii) in favor of banking or other financial institutions or entities, or electronic payment service providers, arising as a matter of law encumbering deposits (including the right of set-off) and which are within the general parameters customary in the banking or finance industry;

(y) any Liens with respect to Equity Interests of any joint venture, co-promotion agreement or similar arrangement pursuant to any joint venture, co-promotion or similar agreement;

(z) Liens on insurance policies and the proceeds thereof securing the financing of the premiums with respect thereto;

(aa) (x) Liens solely on any cash earnest money deposits made by the Borrower or its Subsidiaries in connection with any letter of intent or other agreement in respect of any permitted Investment, (y) Liens on advances of cash or Cash Equivalents in favor of the seller of any property to be acquired in a permitted Investment to be applied against the purchase price for such Investment and (z) on the escrowed cash portion of any earnest moneys paid or the purchase price received in connection with any Investment, acquisition or Disposition permitted by this Agreement or any other Loan Document to secure guarantees, indemnities, or obligations thereunder;

(bb) Liens on any asset that constitutes Excluded Property;

(cc) Liens listed as exceptions on any mortgage insurance policy;

(dd) in the case of any account described in clause (e)(ii) of the definition of "Excluded Accounts," Liens on such account in favor of any counterparty to the applicable Contractual Obligation; and

(ee) any Permitted License.

7.02 Indebtedness.

Create, incur, assume or suffer to exist any Indebtedness, except:

(a) Indebtedness under the Loan Documents;

(b) Indebtedness outstanding on the date hereof and listed on Schedule 7.02(b) and any refinancings, refundings, renewals or extensions thereof; *provided* that (i) (A) the amount of such Indebtedness is not increased at the time of such refinancing, refunding, renewal or extension except by an amount equal to a reasonable premium or other reasonable amount paid, and fees and expenses reasonably incurred, in connection with such refinancing and by an amount equal to any existing commitments unutilized thereunder, (B) the direct or any contingent obligor with respect thereto is not changed and the Indebtedness that is refinanced, refunded, renewed or extended is not recourse to any Loan Party or any of its Subsidiaries that is liable on account of the obligations other than those Persons which were originally obligated, as a result of or in connection with such refinancing, refunding, renewal or extension and (C) the terms relating to principal amount, amortization, maturity, scope of collateral (if any), priority and subordination, standstill and related terms (if any), and other material terms taken as a whole, of any such refinancing, refunding, renewing or extending Indebtedness, and of any agreement entered into and of any instrument issued in connection therewith, are no less favorable, when taken as a whole, in any material respect to the Loan Parties and their Subsidiaries, on the one hand, or the Lenders, on the other hand, than the terms of any agreement or instrument governing the Indebtedness being refinanced, refunded, renewed or extended and (ii) the interest rate applicable to any such refinancing, refunding, renewing or extending Indebtedness does not exceed the then applicable market interest rate;

(c) Indebtedness in respect of Finance Leases (including such that constitute automobile leases), Synthetic Lease Obligations and purchase money obligations (within the limitations set forth in Section 7.01(j)) solely with respect to the financing of the acquisition or construction of new assets after the Closing Date, in each case, including, for the avoidance of doubt, any such Finance Leases, Synthetic Lease Obligations or purchase money obligations outstanding as of the Closing Date (each of which is set forth on Schedule 7.02(c)); *provided*, *however*, that the aggregate amount of all such Indebtedness (to the extent constituting Indebtedness under GAAP) at any one time outstanding (including, for the avoidance of doubt, such outstanding as of the Closing Date) shall not exceed [**];

(d) Unsecured Indebtedness of the Borrower or any Subsidiary of the Borrower owed to the Borrower or another Subsidiary of the Borrower, which Indebtedness is permitted under the provisions of Section 7.03 (other than Section 7.03(k)) ("*Intercompany Debt*"), so long as such Intercompany Debt is subject at all times to the Intercompany Note;

(e) Guarantees of the Borrower or any Subsidiary in respect of Indebtedness otherwise expressly permitted hereunder of the Borrower or any Subsidiary; *provided* that if the Indebtedness being guaranteed is subordinated to the Obligations, such Guarantee shall be subordinated to the Obligations on terms at least as favorable to the Lenders;

(f) Swap Obligations (contingent or otherwise) existing or arising under any Swap Contract, including any payments in connection with the termination of any Swap Obligations, *provided* that such Swap Obligations are (or were) entered into by such Person for the purpose of directly mitigating risks associated with fluctuations in interest rates or foreign exchange rates and not for speculative purposes;

(g) Indebtedness owed to any financial institution in respect of overdrafts, netting services, purchasing, debit card programs or credit card programs and related liabilities arising from ordinary course treasury, depository or cash management services or in connection with any automated clearing house transfers of funds, including any payments in connection with the termination thereof;

(h) Indebtedness in respect of (A) letters of credit issued for the account of the Borrower or a Subsidiary in an aggregate principal amount (including, for the avoidance of doubt, any such letters of credit outstanding as of the Closing Date, each of which is set forth on Schedule 7.02(h)) not to exceed $[**], and (B) bankers' acceptances, bank guaranties, surety bonds and similar instruments, in each case, in the ordinary course of business;

(i) Indebtedness consisting of obligations of the Borrower or any of its Subsidiaries in respect of deferred compensation, indemnification, earn-outs, milestone payments, adjustment of purchase or other similar arrangements or Deferred Acquisition Consideration incurred, in each case, by such Person in connection with Permitted Acquisitions;

(j) Indebtedness of the Borrower and its Subsidiaries consisting of (x) the financing of insurance premiums or (y) take-or-pay obligations contained in supply arrangements, in each case, in the ordinary course of business;

(k) Indebtedness owed to future, current or former officers, directors, managers, employees, consultants and independent contractors thereof or any direct or indirect parent thereof, their respective estates, heirs, family members, spouses or former spouses, in each case to finance the purchase or redemption of Equity Interests of the Borrower, in an aggregate principal amount not to exceed $[**];

(l) customer deposits and advance payments received in the ordinary course of business from customers for goods purchased in the ordinary course of business;

(m) guarantees incurred in the ordinary course of business in respect of obligations to suppliers, customers, franchisees, lessors, licensees, sub-licensees and distribution partners;

(n) unfunded pension fund and other employee benefit plan obligations and liabilities to the extent that they are permitted to remain unfunded under Applicable Law;

(o) Indebtedness of the Borrower or any Subsidiary consisting of (i) deferred compensation or equity based compensation to current or former officers, directors, consultants, advisors or employees thereof, in each case in the ordinary course of business or (ii) Taxes, assessments or governmental charges to the extent such Taxes are being contested in good faith;

(p) Other Indebtedness not to exceed $[**] in the aggregate principal amount at any time outstanding;

(q) Indebtedness of Subsidiaries that are not Loan Parties not to exceed $[**] in an aggregate principal amount at any time outstanding;

(r) Indebtedness assumed pursuant to any Permitted Acquisition not to exceed $[**] in the aggregate principal amount at any time outstanding, so long as no such Indebtedness was created or incurred in connection with, or in contemplation of, such Permitted Acquisition, and any refinancings, refundings, renewals or extensions thereof; *provided* that (i) (A) the amount of such Indebtedness is not increased at the time of such refinancing, refunding, renewal or extension except by an amount equal to a reasonable premium or other reasonable amount paid, and fees and expenses reasonably incurred, in connection with such refinancing and by an amount equal to any existing commitments unutilized thereunder, (B) the direct or any contingent obligor with respect thereto is not changed and the Indebtedness that is refinanced, refunded, renewed or extended is not recourse to any Loan Party or any of its Subsidiaries that is liable on account of the obligations other than those Persons which were originally obligated, as a result of or in connection with such refinancing, refunding, renewal or extension and (C) the terms relating to principal amount, amortization, maturity, scope of collateral (if any), priority and subordination, standstill and related terms (if any), and other material terms taken as a whole, of any such refinancing, refunding, renewing or extending Indebtedness, and of any agreement entered into and of any instrument issued in connection therewith, are no less favorable, when taken as a whole, in any material respect to the Loan Parties and their Subsidiaries, on the one hand, or the Lenders, on the other hand, than the terms of any agreement or instrument governing the Indebtedness being refinanced, refunded, renewed or extended and (ii) the interest rate applicable to any such refinancing, refunding, renewing or extending Indebtedness does not exceed the then applicable market interest rate;

(s) Permitted Subordinated Debt;

(t) Indebtedness in respect of performance, stay, customs, surety or appeal bonds provided in the ordinary course of business;

(u) customary indemnification obligations in favor of purchasers in connection with Dispositions permitted hereunder; and

(v) Indebtedness incurred by the Borrower and its Subsidiaries in connection with bankers' acceptances, discounted bills of exchange, warehouse receipts or similar facilities or the discounting or factoring of receivables for credit management purposes, in each case incurred or undertaken in the ordinary course of business.

7.03 Investments.

Make or hold any Investments, except:

(a) Loans and advances to, or guarantees of Indebtedness of, officers, directors, employees, managers, consultants or independent contractors of the Borrower and its Subsidiaries (i) for payroll payments, travel, entertainment, relocation and analogous ordinary business purposes and (ii) solely in the case of loans and advances, in connection with such Person's acquisition of Equity Interests of the Borrower, to the extent permitted pursuant to Section 7.06(j);

(b) Investments consisting of extensions of credit in the nature of accounts receivable or notes receivable arising from the grant of trade credit in the ordinary course of business, and Investments received in satisfaction or partial satisfaction thereof from financially troubled account debtors to the extent reasonably necessary in order to prevent or limit loss;

(c) Guarantees permitted by Section 7.02;

(d) Investments existing on the date hereof (and set forth on Schedule 7.03 and Investments consisting of an extension, modification, replacement or renewal of any Investment existing on, or made pursuant to a binding commitment existing on, the Closing Date; *provided* that the amount of any such Investment may be increased (a) as required by the terms of such Investment as in existence on the Closing Date or (b) as otherwise permitted under this Agreement;

(e) Permitted Acquisitions and other Permitted Licenses;

(f) Investments (including debt obligations) received in connection with the bankruptcy or reorganization of suppliers and customers and in settlement of delinquent obligations of, and other disputes with, customers and suppliers arising in the ordinary course of business;

(g) other Investments in an aggregate amount not to exceed $[**] at the time such Investment is made; provided that no such Investments shall be made with any assets that constitute IP Collateral;

(h) Swap Contracts permitted under Sections 7.02(f);

(i) Investments consisting of purchases or acquisitions of inventory, supplies, materials and equipment or purchases, licenses, sublicenses or leases or subleases of rights or assets (other than Intellectual Property), in each case in the ordinary course of business;

(j) Investments consisting of (v) Liens permitted under Section 7.01, (w) Indebtedness (including guarantees) permitted under Section 7.02, (x) mergers, amalgamations, consolidations and transfers of all or substantially all assets permitted under Section 7.04, (y) Dispositions permitted under Section 7.05, or (z) Restricted Payments permitted under Section 7.06;

(k) Investments consisting of purchases and acquisitions of assets or services (other than Intellectual Property) useful in the business of the Borrower or any Subsidiary in the ordinary course of business;

(l) any Investment of the non-cash consideration received from a Disposition that was made pursuant to and in compliance with Section 7.05;

(m) Investments consisting of earnest money deposits made by the Borrower or its Subsidiaries in connection with any letter of intent or other agreement in respect of any Investment permitted by this Section 7.03;

(n) acquisitions of obligations of one or more officers or other employees of any direct or indirect parent of the Borrower, the Borrower or any Subsidiary of the Borrower in connection with such officer's or employee's acquisition of Equity Interests of any direct or indirect parent of the Borrower, so long as no cash is actually advanced by the Borrower or any Subsidiary to such officers or employees in connection with the acquisition of any such obligations;

(o) guarantees of operating leases (for the avoidance of doubt, excluding obligations in respect of Finance Leases) or of other obligations, in each case, that do not constitute Indebtedness and are entered into by the Borrower or any Subsidiary in the ordinary course of business;

(p) Investments consisting of the redemption, purchase, repurchase or retirement of any Equity Interests permitted by Section 7.06;

(q) non-cash Investments made in connection with bona fide tax planning and reorganization activities as determined in good faith by the Borrower;

(r) Investments made in the ordinary course of business in connection with obtaining, maintaining or renewing client and customer contracts and loans or advances made to, and guarantees with respect to obligations of, distributors, suppliers, licensors and licensees in the ordinary course of business;

(s) Investments of a Subsidiary acquired after the Closing Date or of an entity merged into or amalgamated or consolidated with a Subsidiary in a transaction that is not prohibited by Section 7.03 after the Closing Date to the extent that such Investments were not made in contemplation of such acquisition, merger, amalgamation or consolidation and were in existence on the date of such acquisition, merger, amalgamation or consolidation;

(t) (i) Investments by a Subsidiary that is not a Loan Party in another Subsidiary that is not a Loan Party and (ii) Investments by a Loan Party in another Loan Party;

(u) any Investments in connection with a Permitted Bond Hedge Transaction; and

(v) (i) cash and Cash Equivalents and (ii) Investments consisting of deposit accounts and securities accounts maintained in accordance with the terms of this Agreement and the other Loan Documents containing cash and such Cash Equivalents.

Notwithstanding the foregoing, nothing in this Section 7.03 shall permit the Borrower or any of its Subsidiaries to make an Acquisition that does not constitute a Permitted Acquisition, or to transfer Collateral (other than Cash and Cash Equivalents to fund operations in the ordinary course of business to the extent required) from the Borrower or any Domestic Subsidiary to any Foreign Subsidiary; *provided* that Collateral may be transferred to any Foreign Subsidiary that is a Loan Party with the consent of the Required Lenders (such consent not to be unreasonably withheld, conditioned or delayed); *provided further* that it shall be reasonable to withhold, condition or delay consent if any such transfer would adversely affect (x) the enforceability and priority of the Liens in favor of Administrative Agent and Lenders or (y) the availability of any remedies of the Administrative Agent or the Lenders under this Agreement or any Collateral Document.

7.04 Fundamental Changes.

Merge, dissolve, liquidate, consolidate with or into another Person, or Dispose of (whether in one transaction or in a series of transactions) all or substantially all of its assets (whether now owned or hereafter acquired) to or in favor of any Person, except that, so long as no Default exists or would result therefrom:

(a) any Subsidiary of the Borrower may merge with (i) the Borrower; *provided* that the Borrower shall be the continuing or surviving Person or (ii) any one or more other Subsidiaries, *provided* that when any Loan Party is merging with another Subsidiary (that is not also a Loan Party), such Loan Party shall be the continuing or surviving Person;

(b) any Loan Party may Dispose of all or substantially all of its assets (upon voluntary liquidation or otherwise) to any Subsidiary of the Borrower that is a Loan Party;

(c) any Subsidiary that is not a Loan Party may Dispose of all or substantially all its assets (including any Disposition that is in the nature of a liquidation) to (i) another wholly owned Subsidiary of the Borrower that is not a Loan Party or (ii) to any wholly-owned Subsidiary of the Borrower that is a Loan Party;

(d) any Subsidiary of the Borrower may merge into or consolidate with any other Person or permit any other Person to merge into or consolidate with it; *provided* that (i) the Person surviving such merger shall be a wholly-owned Subsidiary of the Borrower and (ii) in the case of any such merger to which any Loan Party (other than the Borrower) is a party, a Loan Party is the surviving Person;

(e) each of the Borrower and any of its Subsidiaries may merge into or consolidate with any other Person or permit any other Person to merge into or consolidate with it; *provided*, *however*, that in each case, immediately after giving effect thereto (i) in the case of any such merger to which the Borrower is a party, the Borrower is the surviving Person and (ii) in the case of any such merger to which any Loan Party (other than the Borrower) is a party, a Loan Party is the surviving Person; and

(f) any Subsidiary of the Borrower may dissolve, liquidate or wind up its affairs at any time, provided, that, such dissolution, liquidation or winding up could not reasonably be expected to have a Material Adverse Effect and all of its assets and business are transferred to a Loan Party or solely in the case of a Subsidiary that is not a Loan Party, another Subsidiary that is not a Loan Party prior to or concurrently with such dissolution, liquidation or winding up.

7.05 Dispositions.

Make any Disposition or enter into any agreement to make any Disposition, except for the following Dispositions:

(a) Dispositions of obsolete or worn out property, whether now owned or hereafter acquired, in the ordinary course of business;

(b) Dispositions of equipment to the extent that (i) such property is exchanged for credit against the purchase price of similar replacement property or (ii) the proceeds of such Disposition are reasonably promptly applied to the purchase price of such replacement property;

(c) Dispositions permitted by Section 7.04;

(d) the settlement or early termination or cancellation of any Permitted Bond Hedge Transaction or any related Permitted Warrant Transaction;

(e) Dispositions consisting of Permitted Liens;

(f) other Dispositions of assets for not less than the fair market value (as determined by the Borrower in good faith) thereof so long as (1) at least 75.0% of the consideration paid in connection therewith shall be cash or Cash Equivalents paid substantially concurrently with consummation of the transaction, (2) immediately after giving effect to such Disposition and after giving effect to the application of the proceeds thereof as required hereunder, the Borrower would be in compliance with the Liquidity covenant set forth in Section 7.11, (3) the Net Cash Proceeds received in respect of a Disposition made in reliance on this Section 7.05(f) shall be applied in accordance with Section 2.03(b), (4) the aggregate value of assets Disposed of pursuant to this

Section 7.05(f) shall not exceed $[**] per fiscal year of the Borrower and (5) none of the assets Disposed of pursuant to this Section 7.05(f) shall include any Specified Product IP; *provided* that for the purposes of this Section 7.05(f), the following shall be deemed to be cash (x) any securities received by the Loan Parties or any Subsidiary from such transferee that are converted by such Person into cash or Cash Equivalents upon the closing of the applicable Disposition, (y) any purchase price adjustment, milestone payment, royalty, earnout, contingent payment, back-end or other deferred payment of a similar nature and (z) any Designated Non-Cash Consideration received in respect of such Disposition having an aggregate fair market value, taken together with all other Designated Non-Cash Consideration received pursuant to this clause (z) that is at that time outstanding, not to exceed $[**], determined at the time of such Disposition;

(g) other Dispositions in an amount not to exceed $[**] in any fiscal year of the Borrower;

(h) Permitted Licenses;

(i) Dispositions to landlords of improvements made to leased real property pursuant to customary terms of leases entered into in the ordinary course of business;

(j) Dispositions of inventory, including to end users (through wholesalers or other typical sales channels) or to distributors, in the ordinary course of business;

(k) Dispositions of property to the Borrower or any Subsidiary; provided, that if the transferor of such property is a Loan Party then the transferee thereof must be a Loan Party;

(l) Dispositions of receivables in connection with the collection, settlement or compromise thereof, and the forgiveness, release or compromise of any amount owed to any Loan Party or Subsidiary in the ordinary course of business;

(m) other than with respect to Intellectual Property, licenses, sublicenses, leases or subleases granted to others not interfering in any material respect with the business of the Borrower and its Subsidiaries;

(n) dispositions of cash and Cash Equivalents in the ordinary course of business or otherwise in transactions permitted hereunder;

(o) Involuntary Dispositions;

(p) the unwinding of any Swap Contract permitted by Section 7.02(f) pursuant to its terms;

(q) in connection with any transaction permitted under Section 7.01, 7.03 (other than Section 7.03(k)) or 7.06;

(r) dispositions in the ordinary course of business consisting of the abandonment of Intellectual Property (other than Specified Product IP) which, in the reasonable good faith determination of Borrower, are not material to the conduct of the business of Borrower or any of its Subsidiaries;

(s) the sale, transfer, issuance or other disposition of a *de minimis* number of shares of the Equity Interests of a Foreign Subsidiary in order to qualify members of the governing body of such Foreign Subsidiary if required by Applicable Law; and

(t) the exercise by the Borrower or any Subsidiary of termination rights under any lease, sublease, license, sublicense, concession or other agreements.

For the avoidance of doubt, and notwithstanding the foregoing, (i) no Disposition of Specified Product IP shall be permitted under this Section 7.05 or otherwise, other than Dispositions constituting a Permitted License, (ii) no Disposition or issuance of the Equity Interests of any Loan Party (other than the Borrower) shall be permitted under this Section 7.05 or otherwise other than any Disposition or issuance to another Loan Party, (iii) nothing herein shall limit dispositions of assets that are not Collateral, and (iv) all cash proceeds from any Disposition of Collateral shall be kept in accounts that are not Excluded Accounts.

7.06 Restricted Payments.

Declare or make, directly or indirectly, any Restricted Payment, or incur any obligation (contingent or otherwise) to do so, except that:

(a) Each Subsidiary may make Restricted Payments to any Loan Party that owns Equity Interests in such Subsidiary, and each non Loan Party Subsidiary may make Restricted Payments to its equityholders ratably according to their respective holdings of the type of Equity Interest in respect of which such Restricted Payment is being made;

(b) The Borrower may declare and pay dividends with respect to its capital stock and Equity Interests payable solely in additional shares of its common stock and its Equity Interests (other than Disqualified Equity Interests);

(c) The Borrower may make repurchases of its common stock deemed to occur upon the cash-less or net exercise of stock options, warrants or other convertible or exchangeable securities;

(d) The Borrower may make repurchases of Equity Interests deemed to occur upon the withholding of a portion of the Equity Interests granted or awarded to a current or former officer, director, employee or consultant to pay for the taxes payable by such Person upon such grant or award (or upon vesting or exercise thereof);

(e) any required payment with respect to, or required early unwind or settlement of, any Permitted Bond Hedge Transaction or Permitted Warrant Transaction, in each case, in accordance with the terms of the agreement governing such Permitted Bond Hedge Transaction or Permitted Warrant Transaction; *provided* that, in the case of this clause (e), to the extent cash is required to be paid under a Permitted Warrant Transaction as a result of the election of "cash settlement" (or substantially equivalent term) as the "settlement method" (or substantially equivalent term) thereunder by the Borrower (including in connection with the exercise and/or early unwind or settlement thereof), the payment of such cash shall not be permitted by this clause (e) other than to the extent such payment is offset against any payments received by the Borrower pursuant to the exercise, settlement, termination or unwind of any related Permitted Bond Hedge Transaction substantially concurrently with, or a commercially reasonable period of time before or after, the unwind or settlement of the relevant Permitted Warrant Transaction;

(f) The Borrower may make payments of cash in lieu of the issuance of fractional shares of Equity Interests in connection with any merger, consolidation, amalgamation or other business combination, or in connection with any dividend, distribution or split of or upon exercise, conversion or exchange of Equity Interests, warrants, options or other securities exercisable or convertible into, Equity Interests of the Borrower;

(g) The Borrower may declare or make, or agree to pay or make Restricted Payments which are contingent upon either (i) the prior consent of the Required Lenders or (ii) the repayment in full of the Obligations (other than contingent indemnification and expense reimbursement obligations for which no claims have been made) and the termination of the Commitments;

(h) So long as (i) no Event of Default shall have occurred or be continuing and (ii) before and after giving effect to such Restricted Payment on a Pro Forma Basis, the Loan Parties are in compliance with the Liquidity covenant set forth in Section 7.11, the Borrower may make other Restricted Payments not to exceed $[**] in any fiscal year of the Borrower;

(i) The Borrower may make any dividend or distribution or consummation of any redemption within 60 days after the date of declaration thereof or the giving of a redemption notice related thereto, if at the date of declaration or notice such payment would have complied with the provisions of this Agreement;

(j) So long as, after giving effect thereto, the Borrower is in compliance with Section 7.11, the Borrower may make Restricted Payments for the purpose of redeeming from former directors, officers, employees, members of management, managers or consultants of the Borrower or any Subsidiary (or their respective family members, former spouses or estate) Equity Interest of the Borrower (and/or making payments on Indebtedness issued by the Borrower or its Subsidiaries pursuant to Section 7.02) and any tax payments related thereto, in an aggregate amount not to exceed $[**] in any fiscal year and $[**] in the aggregate (it being agreed that, to the extent constituting an Investment permitted by Section 7.03, the amount of any Indebtedness of such Persons owing to the Borrower or any Subsidiary forgiven in connection with such Restricted Payment shall be excluded from any determination pursuant to this clause (j));

(k) The Borrower and its Subsidiaries may make (i) payments made or expected to be made by any Loan Party in respect of withholding or similar taxes payable or expected to be payable by any future, present or former director, officer, employee, manager, consultant or independent contractor of any Loan Party (or their respective Affiliates, estates or immediate family members) in connection with the exercise of stock options or the grant, vesting or delivery of Equity Interests of the Borrower in an aggregate amount not to exceed $[**] in any fiscal year; provided that such $[**] limit shall not apply to any such payments where the exercising party remits in exchange for such payment Equity Interests of the Borrower with a value that corresponds to the amount of such payment, and instead the limit on such payments shall be an aggregate amount not to exceed $[**] in any fiscal year; and (ii) loans or advances to officers, directors, employees, managers, consultants and independent contractors of the Borrower or any Subsidiary of the Borrower in connection with such Person's purchase of Equity Interests of the Borrower; *provided* that (1) no cash is actually advanced pursuant to this clause (k)(ii) other than to pay taxes due in connection with such purchase, unless immediately repaid and (2) such loan or advance is permitted under Section 7.03; and

(l) The Borrower may make Restricted Payments with or from the Net Cash Proceeds received by the Borrower from the sale of Equity Interests (other than Disqualified Equity Interests) of the Borrower so long as (i) any such Restricted Payments are made within 90 days after the

receipt of such proceeds, (ii) no Event of Default has occurred and is continuing or would result from such Restricted Payments and (iii) immediately prior to, and after giving effect to such Restricted Payments, the Borrower would be in compliance with the Liquidity covenant set forth in Section 7.11; *provided* that Restricted Payments made in reliance on this Section 7.06(k) shall not exceed $[**] during the term of this Agreement.

7.07 Change in Nature of Business.

Engage in any material line of business substantially different from those lines of business conducted by the Borrower and its Subsidiaries on the date hereof or any business reasonably related, complementary, incidental, ancillary thereto or any reasonable extensions thereto.

7.08 Transactions with Affiliates.

Enter into or permit to exist any transaction or series of transactions with any officer, director or Affiliate of such Person (including for the purchase, lease or exchange of property or the rendering of services) (other than the Borrower or any Subsidiary) with a value in excess of $[**], other than (a) compensation, employee benefits, reimbursement of expenses and indemnification of officers, directors and employees, and customary payment of insurance premiums on behalf of officers and directors, in each case, (b) transactions that are on terms that are not less favorable to the Borrower or a Subsidiary in any material respect than would be obtainable by the Borrower or such Subsidiary at such time in a comparable arm's-length transaction with a Person other than an Affiliate (as determined in good faith by the senior management or the board of directors of the Borrower), (c) Restricted Payments permitted by Section 7.06, (d) any (i) employment, consulting, service or termination agreement, or customary indemnification arrangements, entered into by the Loan Parties with current, former or future officers, directors, employees, managers, consultants and independent contractors, (ii) subscription agreement or similar agreement pertaining to the repurchase of Equity Interests pursuant to put/call rights or similar rights with current, former or future officers, directors, employees, managers, consultants and independent contractors of the Borrower or its Subsidiaries and (iii) payment of compensation or other employee compensation, benefit plan or arrangement, any health, disability or similar insurance plan which covers officers, directors, employees, managers, consultants and independent contractors of the Borrower or its Subsidiaries (including amounts paid pursuant to any management equity plan or any other management or employee benefit plan or agreement or any stock subscription or shareholder agreement, stock option or similar plans and any successor plan thereto and any supplemental executive retirement benefit plans or arrangements), in each case in the ordinary course of business or as otherwise approved in good faith by the board of directors of the Borrower or another Loan Party, (e) transactions between Loan Parties or transfers of cash and assets to any Loan Party, (f) intercompany transactions expressly permitted by this Agreement and (g) transactions existing on the Closing Date and described on Schedule 7.08 (and any amendment thereto or replacement thereof to the extent such an amendment or replacement is not materially adverse to the Lenders).

7.09 Burdensome Agreements.

Enter into, or permit to exist, any Contractual Obligation (except for this Agreement and the other Loan Documents) that encumbers or restricts the ability of any such Person to (i) make Restricted Payments to any Loan Party, (ii) pay any Obligations owed to any Loan Party, (iii) make loans or advances to any Loan Party, or (iv) create any Lien upon any of their properties or assets, whether now owned or hereafter acquired that constitute Collateral, except the preceding restrictions will not apply to encumbrances or restrictions existing under or by reason of:

(a) contractual encumbrances or restrictions of the Borrower or any Subsidiary in effect on the Closing Date, including pursuant to this Agreement and the other Loan Documents, related Swap Contracts and Indebtedness permitted pursuant to Section 7.02(b);

(b) Applicable Law or any applicable rule, regulation or order;

(c) any agreement or other instrument of a Person acquired by or merged, amalgamated or consolidated with or into Loan Party that was in existence at the time of such acquisition (or at the time it merges with or into any Loan Party in connection with the acquisition of assets from such Person (but, in each case, not created in contemplation thereof)), which encumbrance or restriction is not applicable to any Person, or the properties or assets of any Person, other than the Person, or the property or assets of the Person, so acquired or designated; *provided* that in connection with a merger, amalgamation or consolidation under this clause (c), if a Person other than any Loan Party is the successor company with respect to such merger, amalgamation or consolidation, any agreement or instrument of such Person or any Subsidiary of such Person, shall be deemed acquired or assumed, as the case may be, by any Loan Party, as the case may be, at the time of such merger, amalgamation or consolidation;

(d) customary encumbrances or restrictions contained in contracts or agreements for the sale of assets applicable to such assets pending consummation of such sale, including customary restrictions with respect to a Subsidiary imposed pursuant to an agreement entered into for the sale or disposition of capital stock or assets of such Subsidiary;

(e) restrictions on cash or other deposits or net worth imposed by customers under contracts entered into in the ordinary course of business;

(f) customary provisions in operating or other similar agreements, asset sale agreements and stock sale agreements entered into in connection with the entering into of such transaction, which limitation is applicable only to the assets that are the subject of those agreements;

(g) purchase money obligations for property acquired and obligations in respect of Finance Leases, to the extent such obligations impose restrictions on the property so acquired, solely as permitted by, the terms of this Agreement;

(h) customary provisions contained in leases, sub-leases, licenses, sublicenses, contracts and other similar agreements entered into in the ordinary course of business restricting the assignment thereof or restricting the assignment, pledge, transfer or sublease or sublicense of the property leased, licensed or otherwise the subject thereof;

(i) any encumbrance or restriction contained in other Indebtedness of the Borrower or any Subsidiary that is incurred subsequent to the Closing Date pursuant to Section 7.02, *provided* that (i) such encumbrances and restrictions contained in any agreement or instrument will not materially affect the Borrower's ability to make anticipated principal or interest payments under this Agreement (as determined by the Borrower in good faith) and (ii) such encumbrances and restrictions contained in any agreement or instrument taken as a whole are not materially more restrictive on the Borrower and its Subsidiaries than the encumbrances and restrictions contained in this Agreement (as determined by the Borrower in good faith);

(j) any encumbrance or restriction arising or agreed to in the ordinary course of business, not relating to any Indebtedness, and that do not, individually or in the aggregate, (x) detract from the value of the property or assets of the Borrower or any Subsidiary in any manner

material to the Borrower or any Subsidiary or (y) materially affect the Borrower's ability to make future principal or interest payments under this Agreement, in each case, as determined by the Borrower in good faith;

(k) any encumbrance or restriction contained in secured Indebtedness otherwise permitted to be incurred pursuant to Sections 7.01 and 7.02 to the extent limiting the right of the debtor to dispose of the assets securing such Indebtedness;

(l) customary provisions in joint venture agreements or arrangements and other similar agreements or arrangements relating solely to the applicable joint venture;

(m) any encumbrances or restrictions of the type referred to in the immediately preceding clauses (a) through (m) above imposed by any amendments, modifications, restatements, renewals, increases, supplements, refundings, replacements or refinancings of the contracts, instruments or obligations referred to such immediately preceding clauses (a) through (m) above; *provided* that such encumbrances and restrictions contained in any such amendment, modification, restatement, renewal, increase, supplement, refunding, replacement or refinancing are, in the good faith judgment of the Borrower, not materially more restrictive, taken as a whole, than the encumbrances and restrictions prior to such amendment, modification, restatement, renewal, increase, supplement, refunding, replacement or refinancing;

(n) any encumbrance or restriction contained in any agreements governing any Permitted License;

(o) restrictions or encumbrances in any agreement evidencing Permitted Subordinated Debt that restricts the merger or consolidation of, or the sale of all or substantially all of the assets of, the Borrower or taken as a whole, are not more restrictive on the Borrower and its Subsidiaries in any material respect than the comparable restrictions and encumbrances in the Loan Documents, taken as a whole (as reasonably determined by a Responsible Officer of Borrower in good faith);

(p) prohibitions, restrictions and conditions contained in any agreement or document relating to the consummation of a transaction which is conditioned upon (i) the amendment, restatement, modification or replacement of this Agreement which would have the effect of consenting to such prohibition, restriction or condition or (ii) the repayment in full (other than contingent indemnification and expense reimbursement obligations for which no claim has been made) of Obligations owing under this Agreement and the termination of the Commitments; and

(q) limitations associated with Permitted Liens pursuant to any document or instrument governing any Permitted Lien restricting the assignment, pledge or transfer of the property the subject thereof.

7.10 Use of Proceeds.

Use the proceeds of any Borrowing, whether directly or indirectly, and whether immediately, incidentally or ultimately, to purchase or carry margin stock (within the meaning of Regulation U) or to extend credit to others for the purpose of purchasing or carrying margin stock or to refund indebtedness originally incurred for such purpose.

7.11 Liquidity Covenant.

Permit Liquidity as of the last day of any fiscal quarter of the Borrower to be less than (i) prior to the Liquidity Increase Date, $100,000,000 and (ii) from and after the Liquidity Increase Date, $200,000,000.

7.12 Fiscal Year; Legal Name, State of Formation; Form of Entity and Accounting Changes.

(a) Amend any of its Organization Documents in any manner materially adverse to the interests of the Lenders in their capacities as such (as reasonably determined by the Borrower);

(b) change its fiscal year without delivery of prior written notice to the Administrative Agent;

(c) without providing ten (10) days prior written notice to the Administrative Agent and the Required Lenders (or such extended period of time as agreed to by the Required Lenders), change its name, state or other jurisdiction of formation or incorporation, form of organization or incorporation or principal place of business; or

(d) make any material change in accounting policies or reporting practices without delivery of prior written notice to the Administrative Agent, except as required by GAAP.

7.13 Sale/Leaseback Transactions.

Enter into any Sale/Leaseback Transaction unless (a) the Disposition of the property thereunder is permitted by Section 7.05 and (b) any Liens arising in connection therewith (including Liens deemed to arise in connection with any such Capital Lease Obligations and Synthetic Lease Obligations) are permitted by Section 7.01.

7.14 **Prepayments, Etc. of Certain Debt; Payments of Deferred Acquisition Consideration**.

(a) Pay, prepay, redeem, purchase, defease or otherwise satisfy or make any payment of any Permitted Subordinated Debt or prepay, redeem, repurchase, defease or otherwise satisfy or make any payment of any other Indebtedness, except (a) any payment of the Loans in accordance with the terms of this Agreement, (b) regularly scheduled or required repayments or redemptions of Indebtedness under the Indebtedness set forth in Schedule 7.02(b), Schedule 7.02(c) or Schedule 7.02(h) and refinancings and refundings of such Indebtedness in compliance with Section 7.02(b), (c) payments at stated maturity of Indebtedness (other than Permitted Subordinated Debt) and payments of secured Indebtedness that becomes due as a result of the voluntary sale or transfer of the property or assets securing such Indebtedness (provided that such Indebtedness and such sale or transfer is permitted hereunder), (d) regularly scheduled or required repayments or redemptions of the Existing Convertible Debt and refinancings and refundings of such Existing Convertible Debt in compliance with Section 7.02(b), (e) in the case of Permitted Subordinated Debt, (i) payments solely made or settled in Equity Interests and cash in lieu of fractional shares, (ii) scheduled payments of interest paid-in-kind on such Indebtedness and (iii) up to an aggregate of $[**] in regularly scheduled cash interest payments in any fiscal year less any payments made on Existing Convertible Debt in such fiscal year, (e) the redemption, purchase, exchange, early termination or cancellation of Permitted Subordinated Debt in an aggregate principal amount not to exceed the Net Cash Proceeds received by the Borrower from the issuance of additional Permitted Subordinated Debt or Equity Interests (other than Disqualified Equity Interests) in

connection with a refinancing of the Permitted Subordinated Debt being redeemed, purchased, exchanged, terminated or cancelled, and (f) payments of the initial purchase price for each Swap Contract and Permitted Bond Hedge Transaction.

(b) Pay any Deferred Acquisition Consideration (other than Deferred Acquisition Consideration which is calculated as a percentage of net sales or a comparable metric) with respect to any Acquisition effected following the Closing Date unless, before and after giving effect to such payment on a Pro Forma Basis, the Loan Parties are in compliance with the Liquidity covenant set forth in Section 7.11 and no Event of Default is then continuing.

7.15 **Amendment, Etc. of Debt Documents**.

Amend, modify or change in any manner any term or condition of any Permitted Subordinated Debt that would result in such Permitted Subordinated Debt no longer constituting Permitted Subordinated Debt.

7.16 Sanctions.

Directly or indirectly, use any Borrowing or the proceeds of any Borrowing, or lend, contribute or otherwise make available such Borrowing or the proceeds of any Borrowing to any Person, to unlawfully fund any activities of or business with any Person, that, at the time of such funding, is the subject of Sanctions, or in any other manner that will result in a violation by any Person (including any Person participating in the transaction, whether as Lender, Administrative Agent, or otherwise) of Sanctions.

7.17 Anti-Corruption Laws.

Directly or indirectly, use any Borrowing or the proceeds of any Borrowing for any purpose which would breach the United States Foreign Corrupt Practices Act of 1977, the UK Bribery Act 2010, the Criminal Justice (Corruption Offences) Act 2018 of Ireland, as amended and the Criminal Justice (Money Laundering and Terrorist Financing) Act 2010 to 2021 of Ireland and other anti-corruption legislation in other jurisdictions.

7.18 Key Contracts.

Without the Required Lenders' prior written consent:

(a) No Loan Party or any of its Subsidiaries shall agree to any material set-off, counter-claim or other deduction under or with respect to any Key Contract, or amend, or permit the amendment of any material provision of any Key Contract, or waive any of its respective rights under any such Key Contract, other than any such set-off, counter-claim, other deduction, amendment, waiver that, taken individually and in the aggregate for all Key Contracts and all such agreements, amendments or waivers since the Closing, have or are reasonably likely to have a Material Adverse Effect; and

(b) No Loan Party or any of its Subsidiaries shall agree to any material set-off, counter-claim or other deduction under or with respect to any Specified Key Contract, or amend, or permit the amendment of any material provision of any Specified Key Contract, or waive any of its respective rights under any such Specified Key Contract, or consent to the early termination of such Specified Key Contract, in each case, other than any such agreement, waiver or consent to a modification which is not adverse in any material respect to the Borrower and its Subsidiaries.

ARTICLE VIII

EVENTS OF DEFAULT AND REMEDIES

8.01 Events of Default.

Any of the following shall constitute an event of default (each, an "*Event of Default*"):

(a) Non-Payment. The Borrower or any other Loan Party fails to pay (i) when and as required to be paid herein, any amount of principal of any Loan or any Applicable Premium, or (ii) within five (5) Business Days after the same becomes due, any interest on any Loan or any fee due hereunder, or (iii) within seven (7) days after the same becomes due, any other amount payable hereunder or under any other Loan Document; or

(b) Specific Covenants. Any Loan Party fails to perform or observe any term, covenant or agreement contained in any of Section 6.03(a), Section 6.05 (solely with respect to the Borrower), 6.10, 6.11, or Article VII; or

(c) Other Defaults. Any Loan Party fails to perform or observe any other covenant or agreement (not specified in Section 8.01(a) or (b) above) contained in any Loan Document on its part to be performed or observed and such failure continues for thirty (30) days after written notice thereof by the Administrative Agent (given at the written direction of the Required Lenders) to the Borrower; or

(d) Representations and Warranties. Any representation, warranty, certification or statement of fact made or deemed made by or on behalf of the Borrower or any other Loan Party herein, in any other Loan Document, or in any document required to be delivered in connection herewith or therewith shall be incorrect or misleading in any material respect when made or deemed made; or

(e) Cross-Default. (i) Any Loan Party or any Subsidiary thereof (A) fails to make any payment beyond the applicable grace period with respect thereto, if any (whether by scheduled maturity, required prepayment, acceleration, demand, or otherwise) in respect of any Indebtedness or Guarantee (other than Indebtedness hereunder and Indebtedness under Swap Contracts) for borrowed money having an aggregate outstanding principal amount of more than the Threshold Amount, or (B) fails to observe or perform any other agreement or condition relating to any such Indebtedness or Guarantee or contained in any instrument or agreement evidencing, securing or relating thereto, or any other event occurs, the effect of which default or other event is to cause, or to permit the holder or holders of such Indebtedness or the beneficiary or beneficiaries of such Guarantee (or a trustee or agent on behalf of such holder or holders or beneficiary or beneficiaries) after the expiration of any applicable grace or cure period therefor to cause, with the giving of notice if required, such Indebtedness for borrowed money to be demanded or to become due or to be repurchased, prepaid, defeased or redeemed (automatically or otherwise), or an offer to repurchase, prepay, defease or redeem such Indebtedness to be made, prior to its stated maturity, or such Guarantee to become payable or cash collateral in respect thereof to be demanded; *provided* that it is understood that (x) a conversion (or the occurrence of any customary "conversion triggers") of Existing Convertible Debt or any Permitted Subordinated Debt and (y) secured Indebtedness that becomes due as a result of the voluntary sale or transfer of the property or assets securing such Indebtedness (provided that such Indebtedness and such sale or transfer is permitted hereunder), in each case, shall not constitute an Event of Default under this Section 8.01(e)(i); or (ii) there occurs under any Swap Contract an Early Termination Date (as defined in such Swap

Contract) resulting from (A) any event of default under such Swap Contract as to which a Loan Party or any Subsidiary thereof is the Defaulting Party (as defined in such Swap Contract) or (B) any Termination Event (as so defined) under such Swap Contract as to which a Loan Party or any Subsidiary thereof is an Affected Party (as so defined) and, in either event, the Swap Termination Value owed by such Loan Party or such Subsidiary as a result thereof is greater than the Threshold Amount; *provided* that this clause (e)(ii) shall not apply to any early payment requirement or unwinding or termination with respect to any Permitted Bond Hedge Transaction or Permitted Warrant Transaction, or satisfaction of any condition giving rise to or permitting the foregoing, in accordance with the terms thereof, so long as, in any such case, Borrower is not the "defaulting party" (or substantially equivalent term) under the terms of such Permitted Bond Hedge Transaction or Permitted Warrant Transaction, as applicable; or

(f) Insolvency Proceedings, Etc. Any Loan Party or any of its Subsidiaries institutes or consents to the institution of any proceeding under any Debtor Relief Law, or makes an assignment for the benefit of creditors; or applies for or consents to the appointment of any receiver, trustee, custodian, conservator, liquidator, rehabilitator, examiner, process advisor or similar officer for it or for all or any material part of its property; or any receiver, trustee, custodian, conservator, liquidator, rehabilitator, examiner, process advisor or similar officer is appointed without the application or consent of such Person and the appointment continues undischarged or unstayed for sixty (60) calendar days; or any proceeding under any Debtor Relief Law relating to any such Person or to all or any material part of its property is instituted without the consent of such Person and continues undismissed or unstayed for sixty (60) calendar days, or an order for relief is entered in any such proceeding; or

(g) Inability to Pay Debts; Attachment. (i) Any Loan Party or any of its Subsidiaries becomes unable or admits in writing its inability or fails generally to pay its debts as they become due, or (ii) any writ or warrant of attachment or execution or similar process is issued or levied against all or any material part of the property of any such Person and is not released, vacated or fully bonded within sixty (60) days after its issue or levy; or

(h) Judgments. There is entered against any Loan Party or any Subsidiary thereof (i) one or more final judgments or orders for the payment of money in an aggregate amount (as to all such judgments and orders) exceeding the Threshold Amount (to the extent not covered (other than to the extent of customary deductibles) by independent third-party insurance), or (ii) any one or more non-monetary final judgments that have, or would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect and, in either case, (A) enforcement proceedings are commenced by any creditor upon such judgment or order, or (B) there is a period of sixty (60) consecutive days during which a stay of enforcement of such judgment, by reason of a pending appeal or otherwise, is not in effect; or

(i) ERISA. (i) An ERISA Event occurs with respect to a Pension Plan or Multiemployer Plan which has resulted or could reasonably be expected to result in liability of any Loan Party under Title IV of ERISA to the Pension Plan, Multiemployer Plan or the PBGC in an aggregate amount in excess of the Threshold Amount, or (ii) the Borrower or any ERISA Affiliate fails to pay when due, after the expiration of any applicable grace period, any installment payment with respect to its withdrawal liability under Section 4201 of ERISA under a Multiemployer Plan in an aggregate amount in excess of the Threshold Amount; or

(j) Invalidity of Loan Documents. In each case, any material provision of any Loan Document, at any time after its execution and delivery and for any reason other than as expressly permitted hereunder or thereunder or satisfaction in full of all Obligations arising under the Loan

Documents, ceases to be in full force and effect; or any Loan Party or any other Person contests in writing the validity or enforceability of any provision of any Loan Document; or any Loan Party denies in writing that it has any or further liability or obligation under any provision of any Loan Document, or purports in writing to revoke or rescind any provision of any Loan Document; or it is or becomes unlawful for a Loan Party to perform any of its obligations under the Loan Documents; or

(k) Collateral Documents. Subject in all respects to any applicable post-closing periods and certain other time periods and exceptions under the Loan Documents for any Loan Party or Subsidiary to take perfection actions, any Collateral Document after delivery thereof pursuant to the terms of the Loan Documents shall for any reason cease to create a valid and perfected first priority Lien (subject to Permitted Liens) on any material portion of the Collateral purported to be covered thereby, or any Loan Party shall assert the invalidity of such Liens; or

(l) Change of Control. There occurs any Change of Control; or

(m) Regulatory Matters. If any of the following occurs: (i) (A) any Key Permit or any of the Borrower's or any of Subsidiary's material rights or interests thereunder is terminated or amended in any manner adverse to the Borrower and its Subsidiaries in any material respect and such termination or amendment is not otherwise revoked within [**] days (or, if a resolution to such revocation is being pursued in good faith by appropriate proceedings diligently conducted, and solely if the applicable event or circumstance has not actually resulted in a reduction of revenues that is in excess of the threshold described in the definition of Material Impact, an additional [**] days thereafter) after the occurrence thereof; (B) the FDA, CMS, EMA or any other Governmental Authority (x) terminates, or delivers a letter or other written communication to the Borrower or its Subsidiaries asserting that any Specified Product lacks, a required Key Permit, which termination or assertion is not withdrawn or otherwise resolved within [**] days (or, if a resolution to such withdrawal is being pursued in good faith through appropriate proceedings diligently conducted, and solely if the applicable event or circumstance has not actually resulted in a reduction of revenues that is in excess of the threshold described in the definition of Material Impact, an additional [**] days thereafter) after such Person's receipt of such termination, letter or other written communication or (y) initiates enforcement action against or issues a warning letter with respect to the Borrower or any of the Subsidiaries, or any of their Specified Products or the Health Care Activities therefor, that causes the Borrower or such Subsidiary to discontinue or suspend the sale of, or withdraw from the market, any of its Specified Products, which discontinuance, suspension or withdrawal would reasonably be expected to last for more than [**] days (or, if a resolution to such discontinuance, suspension or withdrawal is being pursued in good faith through appropriate proceedings diligently conducted, and solely if the applicable event or circumstance has not actually resulted in a reduction of revenues that is in excess of the threshold described in the definition of Material Impact, an additional [**] days thereafter) and, in the case of each of the foregoing clauses (A) and (B), such event or circumstance has had, or would reasonably be expected to have, a Material Impact; (ii) a recall that has resulted in, or would reasonably be expected to result in, a Material Impact; or (iii) the Borrower or any of the Subsidiaries enters into one or more settlement agreements with the FDA, CMS, EMA or any other Governmental Authority that results in aggregate liability for all such settlement agreements entered into since the Closing Date in excess of [**] as of the applicable settlement; or

(n) Specified Key Contracts. (i) Any material default or material breach by the Borrower or any of the Subsidiaries or termination event (other than as a result of any expiration of such Specified Key Contract in accordance with its terms) occurs and is continuing under any of the Specified Key Contracts, which material default or material breach is not cured or waived within

any express grace period therein provided and would permit any Person (other than the Borrower or any Subsidiary) party to any Specified Key Contract to have any termination right thereunder; provided that any Default or Event of Default under this Section 8.01(n) caused by such material breach shall be automatically waived and cured under this Agreement and the other Loan Documents without any action on the part of any Secured Party immediately following the waiver and/or cure of the breach or default under the applicable Specified Key Contract or (ii) any of the Specified Key Contracts is terminated for any reason, other than as a result of any expiration of such Specified Key Contract in accordance with its own terms.

Without limiting the provisions of Article IX, if a Default shall have occurred under the Loan Documents, then such Default will continue to exist until it either is cured (to the extent specifically permitted) in accordance with the Loan Documents or is otherwise expressly waived by the Required Lenders as determined in accordance with Section 11.01; and once an Event of Default occurs under the Loan Documents, then such Event of Default will continue to exist until it is expressly waived by the Required Lenders or by the Administrative Agent with the consent of the Required Lenders, as required hereunder in Section 11.01. The Administrative Agent and the Lenders agree that in connection with any foreclosure or other exercise of rights under this Agreement or any other Loan Document with respect to Specified Product IP, the rights of the licensees under Permitted Licenses will not be terminated, limited or otherwise adversely affected so long as no default exists under the Permitted License that would permit the licensor to terminate such Permitted License (commonly known as a non-disturbance).

8.02 Remedies upon Event of Default.

If any Event of Default occurs and is continuing, the Administrative Agent shall, at the request of, or may, with the consent of, the Required Lenders, take any or all of the following actions:

(a) declare the Commitment of each Lender to make Loans to be terminated, whereupon such commitments and obligation shall be terminated;

(b) declare the unpaid principal amount of all outstanding Loans, all interest accrued and unpaid thereon, and all other amounts owing or payable hereunder (including the Applicable Premium) or under any other Loan Document to be immediately due and payable, without presentment, demand, protest or other notice of any kind, all of which are hereby expressly waived by the Borrower; and

(c) exercise on behalf of itself, the Lenders all rights and remedies available to it and the Lenders under the Loan Documents or Applicable Law or equity;

provided, *however*, that upon the occurrence of an event described in Section 8.01(f) with respect to the Borrower, the Commitment of each Lender to make Loans shall automatically terminate, the unpaid principal amount of all outstanding Loans and all interest and other amounts (including the Applicable Premium) as aforesaid shall automatically become due and payable, in each case without further act of the Required Lenders, the Administrative Agent or any Lender.

8.03 Application of Funds.

After the exercise of remedies provided for in Section 8.02 (or after the Loans have automatically become immediately due and payable as set forth in the proviso to Section 8.02) or if at any time insufficient funds are received by and available to the Administrative Agent to pay fully all Obligations then due hereunder, any amounts received on account of the Obligations shall, subject to the provisions of Section 2.12, be applied by the Administrative Agent in the following order:

First, to payment of that portion of the Obligations constituting fees, indemnities, expenses and other amounts (including fees, charges and disbursements of counsel to the Administrative Agent and amounts payable under Article III) payable to the Administrative Agent in its capacity as such;

Second, to payment of that portion of the Obligations constituting fees, indemnities and other amounts (other than principal and interest), including the Applicable Premium, payable to the Lenders (including fees, charges and disbursements of counsel to the respective Lenders (including fees and time charges for attorneys who may be employees of any Lender)) arising under the Loan Documents and amounts payable under Article III, ratably among them in proportion to the respective amounts described in this *Second* clause payable to them;

Third, to payment of that portion of the Obligations constituting accrued and unpaid interest on the Loans and other Obligations arising under the Loan Documents, ratably among the Lenders in proportion to the respective amounts described in this *Third* clause payable to them;

Fourth, to payment of that portion of the Obligations constituting unpaid principal of the Loans; and

Last, the balance, if any, after all of the Obligations have been indefeasibly paid in full, to the Borrower or as otherwise required by Law.

ARTICLE IX

ADMINISTRATIVE AGENT

9.01 Appointment and Authority.

(a) Appointment. Each of the Lenders hereby irrevocably appoints, designates and authorizes Wilmington Trust, National Association to act on its behalf as the Administrative Agent hereunder and under the other Loan Documents and authorizes the Administrative Agent to take such actions on its behalf and to exercise such powers as are delegated to the Administrative Agent by the terms hereof or thereof, together with such actions and powers as are reasonably incidental thereto. The provisions of this Article IX (other than Sections 9.06 and 9.10) are solely for the benefit of the Administrative Agent and the Lenders, and neither the Borrower nor any other Loan Party shall have rights as a third party beneficiary of any of such provisions. It is understood and agreed that the use of the term "agent" herein or in any other Loan Documents (or any other similar term) with reference to the Administrative Agent is not intended to connote any fiduciary or other implied (or express) obligations arising under agency doctrine of any Applicable Law. Instead such term is used as a matter of market custom, and is intended to create or reflect only an administrative relationship between contracting parties.

(b) Collateral Agent. The Administrative Agent shall also act as the "collateral agent" under the Loan Documents, and each of the Lenders hereby irrevocably appoints and authorizes the Administrative Agent to act as the agent of such Lender for purposes of acquiring, holding and enforcing any and all Liens on Collateral granted by any of the Loan Parties to secure any of the Obligations, together with such powers and discretion as are reasonably incidental thereto. In this connection, the Administrative Agent, as "collateral agent" and any co-agents, sub-agents and attorneys-in-fact appointed by the Administrative Agent pursuant to Section 9.05 for purposes of holding or enforcing any Lien on the Collateral (or any portion thereof) granted under the Collateral Documents, or for exercising any rights and remedies thereunder at the direction of the

Administrative Agent, shall be entitled to the benefits of all provisions of this Article IX and Article XI (including Section 11.04(c), as though such co-agents, sub-agents and attorneys-in-fact were the "collateral agent" under the Loan Documents) as if set forth in full herein with respect thereto, and all references to Administrative Agent in this Article IX and Article XI (including Section 11.04(c)) shall, where applicable, be read as including a reference to the Administrative Agent acting as the "collateral agent". Without limiting the generality of the foregoing, the Lenders hereby expressly authorize the Administrative Agent to (i) execute any and all documents (including releases) with respect to the Collateral (including any intercreditor agreement and any amendment, supplement, modification or joinder with respect thereto) and the rights of the Secured Parties with respect thereto, as contemplated by and in accordance with the provisions of this Agreement and the Collateral Documents and acknowledge and agree that any such action by the Administrative Agent shall bind the Lenders and (ii) negotiate, enforce or settle any claim, action or proceeding affecting the Lenders in their capacity as such, at the direction of the Required Lenders, which negotiation, enforcement or settlement will be binding upon each Lender.

(c) Any corporation or association into which the Administrative Agent may be converted or merged, or with which it may be consolidated, or to which it may sell or transfer all or substantially all of its corporate trust business and assets as a whole or substantially as a whole, or any corporation or association resulting from any such conversion, sale, merger, consolidation or transfer to which the Administrative Agent is a party, will be and become the successor Administrative Agent, as applicable, under this Agreement and will have and succeed to the rights, powers, duties, immunities and privileges as its predecessor, without the execution or filing of any instrument or paper or the performance of any further act.

9.02 Rights as a Lender.

If a Lender is serving as the Administrative Agent hereunder, such Person shall have the same rights and powers in its capacity as a Lender as any other Lender and may exercise the same as though it were not the Administrative Agent and the term "Lender" or "Lenders" shall, unless otherwise expressly indicated or unless the context otherwise requires, include, if applicable, the Person serving as the Administrative Agent hereunder in its individual capacity. Such Person and its Affiliates may accept deposits from, lend money to, own securities of, act as the financial advisor or in any other advisory capacity for and generally engage in any kind of banking, trust, financial, advisory, underwriting or other business with any Loan Party or any Subsidiary or other Affiliate thereof as if such Person were not the Administrative Agent hereunder and without any duty to account therefor to the Lenders or to provide notice to or consent of the Lenders with respect thereto.

9.03 Exculpatory Provisions.

(a) Neither the Administrative Agent nor any of its officers, partners, directors, employees or agents shall have any duties or obligations except those expressly set forth herein and in the other Loan Documents, and its duties hereunder shall be administrative in nature. Without limiting the generality of the foregoing, the Administrative Agent and each of its officers, partners, directors, employees or agents:

(i) shall not be subject to any fiduciary or other implied duties, regardless of whether a Default or Event of Default has occurred and is continuing and without limiting the generality of the foregoing, the use of the term "agent" herein and in other Loan Documents with reference to the Administrative Agent is not intended to connote any fiduciary or other implied (or express) obligations arising under any agency doctrine of any Applicable Law and instead, such term is used merely as a matter of market custom, and is

intended to create or reflect only an administrative relationship between independent contracting parties;

(ii) shall not have any duty to take any discretionary action or exercise any discretionary powers, except discretionary rights and powers expressly contemplated hereby or by the other Loan Documents that the Administrative Agent is required to exercise as directed in writing by the Required Lenders (or such other number or percentage of the Lenders as shall be expressly provided for herein or in the other Loan Documents); *provided* that the Administrative Agent shall not be required to take any action that, in its opinion or the opinion of its counsel, may expose the Administrative Agent to liability or that is contrary to any Loan Document or Applicable Law, including for the avoidance of doubt refraining from any action that, in its respective opinion or the opinion of its respective counsel, may be a violation of the automatic stay under any Debtor Relief Law or that may effect a forfeiture, modification or termination of property of a Defaulting Lender in violation of any Debtor Relief Law;

(iii) shall not have any duty or responsibility to disclose, and shall not be liable for the failure to disclose, to any Lender any credit or other information concerning the business, prospects, operations, property, financial and other condition or creditworthiness of any of the Loan Parties or any of their Affiliates that is communicated to, or in the possession of, the Administrative Agent or any of its Related Parties in any capacity, except for notices, reports and other documents expressly required to be furnished to the Lenders by the Administrative Agent herein.

(iv) shall not be liable for any action taken or not taken by it (i) with the consent or at the request of the Required Lenders (or such other number or percentage of the Lenders as shall be necessary, or as the Administrative Agent shall believe in good faith shall be necessary, under the circumstances as provided in Sections 8.02 and 11.01) or (ii) in the absence of its own gross negligence or willful misconduct as determined by the final and non-appealable judgment of a court of competent jurisdiction. The Administrative Agent shall not be deemed to have knowledge of any Default or Event of Default unless and until written notice describing such Default or Event of Default is given to the Administrative Agent by the Borrower or a Lender;

(v) shall not be responsible for or have any duty to ascertain or inquire into (A) any statement, warranty or representation made in or in connection with this Agreement or any other Loan Document, (B) the contents of any certificate, report or other document delivered hereunder or thereunder or in connection herewith or therewith, (C) the performance or observance of any of the covenants, agreements or other terms or conditions set forth herein or therein or the occurrence of any Default or Event of Default, (D) the validity, enforceability, effectiveness or genuineness of this Agreement, any other Loan Document or any other agreement, instrument or document, or the creation, perfection or priority of any Lien purported to be created by the Collateral Documents, (E) the value or the sufficiency of any Collateral, (F) compliance with the provisions hereof relating to the calculation of the Applicable Premium or (G) the satisfaction of any condition set forth in Article IV or elsewhere herein, other than to confirm receipt of items expressly required to be delivered to the Administrative Agent;

(vi) shall not be responsible for (A) perfecting, maintaining, monitoring, preserving or protecting the security interest or Lien granted under this Agreement, the Collateral Documents, any other Loan Document or any agreement or instrument

contemplated hereby or thereby, (B) the filing, re-filing, recording, re-recording or continuing or any document, financing statement, mortgage, assignment, notice, instrument of further assurance or other instrument in any public office at any time or times or (C) providing, maintaining, monitoring or preserving insurance on or the payment of taxes with respect to any of the Collateral. The actions described in items (A) through (C) shall be the sole responsibility of the Borrower;

(vii) shall not be liable for any failure or delay in the performance of its obligations under this Agreement or any related documents because of circumstances beyond the Administrative Agent's control, including, but not limited to, a failure, termination, or suspension of a clearing house, securities depositary, settlement system or central payment system in any applicable part of the world or acts of God, flood, war (whether declared or undeclared), civil or military disturbances or hostilities, nuclear or natural catastrophes, political unrest, explosion, severe weather or accident, earthquake, terrorism, fire, riot, labor disturbances, strikes or work stoppages for any reason, embargo, government action, including any laws, ordinances, regulations or the like (whether domestic, federal, state, county or municipal or foreign) which delay, restrict or prohibit the providing of the services contemplated by this Agreement or any related documents, or the unavailability of communications or computer facilities, the failure of equipment or interruption of communications or computer facilities, or the unavailability of the Federal Reserve Bank wire or telex or other wire or communication facility, or any other causes beyond the Administrative Agent's control whether or not of the same class or kind as specified above;

(viii) shall not be (A) required to qualify in any jurisdiction in which it is not presently qualified to perform its obligations as such Administrative Agent or (B) required to take any enforcement action against a Loan Party or any other obligor outside of the United States;

(ix) shall not be responsible or have any liability for, or have any duty to ascertain, inquire into, monitor the list or identities of, or enforce, compliance with the provisions hereof relating to Disqualified Institutions. Without limiting the generality of the foregoing, the Administrative Agent shall not (A) be obligated to ascertain, monitor or inquire as to whether any Lender or Participant or prospective Lender or Participant is a Disqualified Institution or (B) have any liability with respect to or arising out of any assignment or participation of Loans, or disclosure of confidential information, to any Disqualified Institution;

(x) shall not be responsible for the negligence, misconduct or other action or inaction of any sub-agent that it selects as provided in Section 9.05 absent gross negligence or willful misconduct by the Administrative Agent (as determined in a final and non-appealable judgment by a court of competent jurisdictions) in the selection of such sub-agents;

(xi) shall neither be responsible for, nor chargeable with, knowledge of the terms and conditions of any other agreement, instrument, or document other than this Agreement and any other Loan Document to which Administrative Agent is a party, whether or not an original or a copy of such agreement has been provided to the Administrative Agent; and

(xii) shall not be responsible for nor have any duty to monitor the performance or any action of the Loan Parties, the Lenders, or any of their directors, members, officers, agents, affiliates or employee, nor shall they have any liability in connection with the malfeasance or nonfeasance by such party; the Administrative Agent may assume performance by all such Persons of their respective obligations.

(b) Each Lender acknowledges and agrees that neither such Lender, nor any of its Affiliates, participants or assignees, may rely on the Administrative Agent to carry out such Lender's, Affiliate's, participant's or assignee's customer identification program, or other obligations required or imposed under or pursuant to any anti-terrorism Law, including any programs involving any of the following items relating to or in connection with the Loan Parties or their respective Subsidiaries, any of their respective Affiliates or agents, the Loan Documents or the transactions hereunder: (i) any identity verification procedures, (ii) any record keeping, (iii) any comparisons with government lists, (iv) any customer notices or (v) any other procedures required under any anti-terrorism Law.

(c) The delivery by the Borrower or any other Loan Party of any reports, information and documents to the Administrative Agent is for informational purposes only and the Administrative Agent's receipt of such shall not constitute constructive notice of any information contained therein or determinable from information contained therein. Each party to this Agreement acknowledges and agrees that the Administrative Agent may, but shall not be obligated to, from time to time use one or more outside service providers for the tracking of all UCC financing statements (and/or other collateral related filings and registrations from time to time) required to be filed or recorded pursuant to the Loan Documents and the notification to the Administrative Agent, of, among other things, the upcoming lapse or expiration thereof, and that each of such service providers will be deemed to be acting at the request and on behalf of Borrower and the other Loan Parties. The Administrative Agent shall not be liable for any action taken or not taken by any such service provider. Neither the Administrative Agent nor any of its officers, partners, directors, employees or agents shall be liable to the Lenders for any action taken or omitted by the Administrative Agent under or in connection with any of the Loan Documents.

(d) Nothing in this Agreement or any other Loan Document shall require the Administrative Agent to expend or risk its own funds or otherwise incur any financial liability in the performance of any of its duties or in the exercise of any of its rights or powers hereunder.

9.04 Reliance by Administrative Agent.

The Administrative Agent shall be entitled to rely upon, and shall be fully protected in relying and shall not incur any liability for relying upon, any notice, request, certificate, communication, consent, statement, instrument, document, legal order, judgment, decree or other writing (including any electronic message, Internet or intranet website posting or other distribution) believed by it to be genuine and to have been signed, sent or otherwise authenticated by the proper Person. The Administrative Agent also may rely upon any statement made to it orally or by telephone and believed by it to have been made by the proper Person, and shall be fully protected in relying and shall not incur any liability for relying thereon. In determining compliance with any condition hereunder to the making of a Loan that by its terms must be fulfilled to the satisfaction of a Lender, the Administrative Agent may presume that such condition is satisfactory to such Lender unless the Administrative Agent shall have received notice to the contrary from such Lender prior to the making of such Loan. The Administrative Agent may consult with legal counsel (who may be counsel for the Loan Parties), independent accountants and other experts selected by it, and shall not be liable for any action taken or not taken by it in accordance with the advice of any such counsel, accountants or experts. For purposes of determining compliance with the conditions specified in Section

4.01, each Lender that has signed this Agreement shall be deemed to have consented to, approved or accepted or to be satisfied with, each document or other matter required thereunder to be consented to or approved by or acceptable or satisfactory to a Lender unless the Administrative Agent shall have received written notice from such Lender prior to the proposed Closing Date specifying its objections.

9.05 Delegation of Duties.

The Administrative Agent may perform any and all of its duties and exercise its rights and powers hereunder or under any other Loan Document by or through any one or more sub-agents appointed by the Administrative Agent. The Administrative Agent and any such sub-agent may perform any and all of its duties and exercise its rights and powers by or through their respective Related Parties. The exculpatory provisions of this Article IX shall apply to any such sub-agent and to the Related Parties of the Administrative Agent and any such sub-agent, and shall apply to their respective activities in connection with the syndication of the credit facilities provided for herein as well as activities as Administrative Agent. The Administrative Agent shall not be responsible for the negligence or misconduct of any sub-agents except to the extent that a court of competent jurisdiction determines in a final and non-appealable judgment that the Administrative Agent acted with gross negligence or willful misconduct in the selection of such sub-agents.

9.06 Resignation.

(a) Notice. The Administrative Agent may at any time give notice of its resignation to the Lender and the Borrower. Upon receipt of any such notice of resignation, the Required Lenders shall have the right to appoint a successor with the prior written consent of the Borrower (unless an Event of Default has occurred and is continuing), which shall be a financial institution with an office in the United States, or an Affiliate of any such financial institution with an office in the United States. If no such successor shall have been so appointed by the Required Lenders and shall have accepted such appointment within thirty (30) days after the retiring Administrative Agent gives notice of its resignation (or such earlier day as shall be agreed by the Required Lenders) (the "*Resignation Effective Date*"), then the retiring Administrative Agent may (but shall not be obligated to) on behalf of the Required Lenders appoint a successor Administrative Agent meeting the qualifications set forth above; *provided* that in no event shall any successor Administrative Agent be a Defaulting Lender. Whether or not a successor has been appointed, such resignation shall become effective in accordance with such notice on the Resignation Effective Date. In addition, upon not less than ten (10) days prior written notice (the "*Removal Effective Date*"), the Administrative Agent may be removed (with or without cause) by the Required Lenders at any time in its sole discretion, but with the prior written consent of the Borrower (such consent not to be unreasonably withheld, conditioned or delayed).

(b) Effect of Resignation or Removal. With effect from the Resignation Effective Date or the Removal Effective Date, as applicable (i) the retiring or removed Administrative Agent shall be discharged from its duties and obligations hereunder and under the other Loan Documents and (ii) except for any indemnity payments or other amounts then owed to the retiring or removed Administrative Agent, all payments, communications and determinations provided to be made by, to or through the Administrative Agent shall instead be made by or to each Lender directly, until such time, if any, as the Required Lenders appoints a successor Administrative Agent with the consent of the Borrower as provided for above. Upon the acceptance of a successor's appointment as Administrative Agent hereunder, such successor shall succeed to and become vested with all of the rights, powers, privileges and duties of the retiring Administrative Agent (other than as provided in Section 3.01(g) and other than any rights to indemnity payments or other amounts owed to the retiring or removed Administrative Agent as of the Resignation Effective Date or the Removal

Effective Date, as applicable), and the retiring or removed Administrative Agent shall be discharged from all of its duties and obligations hereunder or under the other Loan Documents (if not already discharged therefrom as provided above in this Section 9.06). The fees payable by the Borrower to a successor Administrative Agent shall be the same as those payable to its predecessor unless otherwise agreed between the Borrower and such successor. After the retiring or removed Administrative Agent's resignation or removal hereunder and under the other Loan Documents, the provisions of this Article XI and Section 11.04 shall continue in effect for the benefit of such retiring or removed Administrative Agent, its sub-agents and their respective Related Parties in respect of any actions taken or omitted to be taken by any of them (A) while the retiring Administrative Agent was acting as Administrative Agent and (B) after such resignation for as long as any of them continues to act in any capacity hereunder or under the other Loan Documents, including, without limitation, (1) acting as collateral agent or otherwise holding any collateral security on behalf of any of the Secured Parties and (2) in respect of any actions taken in connection with transferring the agency to any successor Administrative Agent.

9.07 Non-Reliance on Administrative Agent and the Lenders.

Each Lender expressly acknowledges that the Administrative Agent has not made any representation or warranty to it, and that no act by the Administrative Agent hereafter taken, including any consent to, and acceptance of any assignment or review of the affairs of any Loan Party or any Affiliate thereof, shall be deemed to constitute any representation or warranty by the Administrative Agent to any Lender as to any matter, including whether the Administrative Agent have disclosed material information in their (or their Related Parties') possession. Each Lender represents to the Administrative Agent that it has, independently and without reliance upon the Administrative Agent, any other Lender or any of their Related Parties and based on such documents and information as it has deemed appropriate, made its own credit analysis of, appraisal of, and investigation into, the business, prospects, operations, property, financial and other condition and creditworthiness of the Loan Parties and their Subsidiaries, and all applicable bank or other regulatory Laws relating to the transactions contemplated hereby, and made its own decision to enter into this Agreement and to extend credit to the Borrower hereunder. Each Lender also acknowledges that it will, independently and without reliance upon the Administrative Agent, any other Lender or any of their Related Parties and based on such documents and information as it shall from time to time deem appropriate, continue to make its own credit analysis, appraisals and decisions in taking or not taking action under or based upon this Agreement, any other Loan Document or any related agreement or any document furnished hereunder or thereunder, and to make such investigations as it deems necessary to inform itself as to the business, prospects, operations, property, financial and other condition and creditworthiness of the Loan Parties. Each Lender represents and warrants that (i) the Loan Documents set forth the terms of a commercial lending facility and (ii) it is engaged in making, acquiring or holding commercial loans in the ordinary course and is entering into this Agreement as a Lender for the purpose of making, acquiring or holding commercial loans and providing other facilities set forth herein as may be applicable to such Lender, and not for the purpose of purchasing, acquiring or holding any other type of financial instrument, and each Lender agrees not to assert a claim in contravention of the foregoing. Each Lender represents and warrants that it is sophisticated with respect to decisions to make, acquire and/or hold commercial loans and to provide other facilities set forth herein, as may be applicable to such Lender, and either it, or the Person exercising discretion in making its decision to make, acquire and/or hold such commercial loans or to provide such other facilities, is experienced in making, acquiring or holding such commercial loans or providing such other facilities.

9.08 No Other Duties, Etc.

The Administrative Agent may at any time request instructions from the Required Lenders or the Lenders with respect to any actions or approvals which by the terms of this Agreement or of any of the

Loan Documents the Administrative Agent is permitted or desires to take or to grant, and if such instructions are promptly requested, the Administrative Agent shall be absolutely entitled to refrain from taking any action or to withhold any approval and shall not be under any liability whatsoever to any Person for refraining from any action or withholding any approval under any of the Loan Documents until it shall have received such instructions from the Required Lenders or all or such other portion of the Lenders as shall be prescribed by this Agreement. Without limiting the foregoing, no Person shall have any right of action whatsoever against the Administrative Agent as a result of the Administrative Agent acting or refraining from acting under this Agreement or any of the other Loan Documents in accordance with the instructions of the Required Lenders (or all or such other portion of the Lenders as shall be prescribed by this Agreement) and, notwithstanding the instructions of the Required Lenders (or such other applicable portion of the Lenders), the Administrative Agent shall have no obligation to take any action if it believes, in good faith, that such action would violate applicable law or exposes the Administrative Agent to any liability for which it is not entitled to satisfactory reimbursement and indemnification in accordance with the provisions of Section 11.04. Anything herein to the contrary notwithstanding, none of the titles listed on the cover page hereof shall have any powers, duties or responsibilities under this Agreement or any of the other Loan Documents, except in its capacity, as applicable, as the Administrative Agent or a Lender hereunder.

9.09 Administrative Agent May File Proofs of Claim; Credit Bidding.

(a) In case of the pendency of any proceeding under any Debtor Relief Law or any other judicial proceeding relative to any Loan Party, the Administrative Agent (irrespective of whether the principal of any Loan shall then be due and payable as herein expressed or by declaration or otherwise and irrespective of whether the Administrative Agent shall have made any demand on the Borrower) shall be entitled and empowered, by intervention in such proceeding or otherwise:

(i) to file and prove a claim for the whole amount of the principal and interest owing and unpaid in respect of the Loans and all other Obligations that are owing and unpaid and to file such other documents as may be necessary or advisable in order to have the claims of the Lenders and the Administrative Agent (including any claim for the reasonable compensation, expenses, disbursements and advances of the Lenders and the Administrative Agent and their respective agents and counsel to the extent provided for herein and all other amounts due the Lenders and the Administrative Agent under Sections 2.07 and 11.04) allowed in such judicial proceeding; and

(ii) to collect and receive any monies or other property payable or deliverable on any such claims and to distribute the same;

and any custodian, receiver, assignee, trustee, liquidator, sequestrator, examiner, process advisor or other similar official in any such judicial proceeding is hereby authorized by each Lender to make such payments to the Administrative Agent and, in the event that the Administrative Agent shall consent to the making of such payments directly to the Lenders, to pay to the Administrative Agent any amount due for the reasonable compensation, expenses, disbursements and advances of the Administrative Agent and its agents and counsel, and any other amounts due the Administrative Agent under Sections 2.07 and 11.04.

(b) Nothing contained herein shall be deemed to authorize the Administrative Agent to authorize or consent to or accept or adopt on behalf of any Lender any plan of reorganization, arrangement, adjustment or composition affecting the Obligations or the rights of any to authorize the Administrative Agent to vote in respect of the claim of any Lender or in any such proceeding.

(c) The Secured Parties hereby irrevocably authorize the Administrative Agent, at the direction of the Required Lenders, to credit bid all or any portion of the Obligations (including accepting some or all of the Collateral in satisfaction of some or all of the Obligations pursuant to a deed in lieu of foreclosure or otherwise) and in such manner purchase (either directly or through one or more acquisition vehicles) all or any portion of the Collateral (i) at any sale thereof conducted under the provisions of the Bankruptcy Code of the United States, including under Sections 363, 1123 or 1129 of the Bankruptcy Code of the United States, or any similar Laws in any other jurisdictions to which a Loan Party is subject, (ii) at any other sale or foreclosure or acceptance of collateral in lieu of debt conducted by (or with the consent or at the direction of) the Administrative Agent (whether by judicial action or otherwise) in accordance with any Applicable Law. In connection with any such credit bid and purchase, the Obligations owed to the Secured Parties shall be entitled to be, and shall be, credit bid on a ratable basis (with Obligations with respect to contingent or unliquidated claims receiving contingent interests in the acquired assets on a ratable basis that would vest upon the liquidation of such claims in an amount proportional to the liquidated portion of the contingent claim amount used in allocating the contingent interests) in the asset or assets so purchased (or in the Equity Interests or debt instruments of the acquisition vehicle or vehicles that are used to consummate such purchase). In connection with any such bid (A) the Administrative Agent shall be authorized to form one or more acquisition vehicles to make a bid, (B) to adopt documents providing for the governance of the acquisition vehicle or vehicles (*provided* that any actions by the Administrative Agent with respect to such acquisition vehicle or vehicles, including any disposition of the assets or Equity Interests thereof shall be governed, directly or indirectly, by the vote of the Required Lenders, irrespective of the termination of this Agreement), and (C) to the extent that Obligations that are assigned to an acquisition vehicle are not used to acquire Collateral for any reason (as a result of another bid being higher or better, because the amount of Obligations assigned to the acquisition vehicle exceeds the amount of debt credit bid by the acquisition vehicle or otherwise), such Obligations shall automatically be reassigned to the Lenders *pro rata* and the Equity Interests and/or debt instruments issued by any acquisition vehicle on account of the Obligations that had been assigned to the acquisition vehicle shall automatically be cancelled, without the need for any Secured Party or any acquisition vehicle to take any further action.

9.10 Collateral and Guaranty Matters.

(a) Each of the Lenders irrevocably consents to the release (or subordination, as applicable, in the case of Section 9.10(ii)), and hereby directs the Administrative Agent, and the Administrative Agent hereby agrees, to automatically release (or subordinate, in the case of Section 9.10(ii)),

(i) any Lien on any property granted to or held by the Administrative Agent under any Loan Document (A) upon the Facility Termination Date, (B) that is sold or otherwise Disposed of or to be sold or otherwise Disposed of as part of or in connection with any sale or other Disposition permitted hereunder or under any other Loan Document, including, for the avoidance of doubt, in connection with any Permitted License (it being understood and agreed with respect to release of Liens under this subsection that the Administrative Agent may conclusively rely without further inquiry on a certificate of a Responsible Officer as to the release of Liens being made in full compliance with the provisions of the Loan Documents), (C) any assets becoming Excluded Property (it being understood and agreed with respect to release of Liens under this subsection that the Administrative Agent may conclusively rely without further inquiry on a certificate of a Responsible Officer as to the release of Liens being made in full compliance with the

provisions of the Loan Documents), or (D) if approved, authorized or ratified in writing by the Required Lenders in accordance with Section 11.01;

(ii) any Lien on any property granted to or held by the Administrative Agent under any Loan Document to the holder of any Lien on such property that is permitted by Section 7.01(j) or (o); and

(iii) any Guarantor from its obligations under the Guaranty, the Collateral Documents and all other Loan Documents to which it is a party if such Person ceases to be a Loan Party or otherwise becomes an Excluded Subsidiary (other than pursuant to clause (b)(i) of the definition thereof) as a result of a transaction permitted under the Loan Documents (it being understood and agreed with respect to release of Liens under this subsection that the Administrative Agent may conclusively rely without further inquiry on a certificate of a Responsible Officer as to the release of Liens being made in full compliance with the provisions of the Loan Documents).

(b) Upon request by the Administrative Agent at any time, the Required Lenders will confirm in writing the Administrative Agent's authority to release or subordinate its interest in particular types or items of property, or to release any Guarantor from its obligations under the Guaranty pursuant to this Section 9.10. In each case as specified in this Section 9.10, the Administrative Agent will, at the Borrower's expense, execute and deliver to the applicable Loan Party such documents as such Loan Party may reasonably request to evidence the release of such item of Collateral from the assignment and security interest granted under the Collateral Documents and deliver to the Borrower, at the expense of the Borrower, any portion of such Collateral so released that is in possession of the Administrative Agent or to subordinate its interest in such item, or to release such Guarantor from its obligations under the Guaranty, in each case in accordance with the terms of the Loan Documents and this Section 9.10.

(c) The Administrative Agent shall not be responsible for or have a duty to ascertain or inquire into any representation or warranty regarding the existence, value or collectability of the Collateral, the existence, priority or perfection of the Administrative Agent's Lien thereon, or any certificate prepared by any Loan Party in connection therewith, nor shall the Administrative Agent be responsible or liable to the Lenders for any failure to monitor or maintain any portion of the Collateral.

(d) In addition, in connection with any Permitted Licenses, each Lender hereby authorizes Administrative Agent to, and at the request of the Borrower, the Administrative Agent shall enter into a non-disturbance agreement and other similar agreements in form and substance reasonably satisfactory to Administrative Agent and the Required Lenders.

(e) In connection with the performance of its obligations under Section 9.10(a) or (b), the Administrative Agent shall be entitled to request from the Borrower, and the Borrower shall deliver to the Administrative Agent prior to the Administrative Agent taking any actions under either Section, a certificate of a Responsible Officer certifying that the transaction giving rise to the requested release is permitted under the Loan Documents (and the Lenders authorize the Administrative Agent to rely on such certificate in performing its obligations under such Sections).

9.11 **Erroneous Payments**.

(a) If the Administrative Agent (x) notifies a Lender or any Person who has received funds on behalf of a Lender (any such Lender or other recipient (and each of their respective

successors and assigns), a "Payment Recipient") that the Administrative Agent has determined in its sole discretion (whether or not after receipt of any notice under immediately succeeding clause (b)) that any funds (as set forth in such notice from the Administrative Agent) received by such Payment Recipient from the Administrative Agent or any of its Affiliates were erroneously or mistakenly transmitted to, or otherwise erroneously or mistakenly received by, such Payment Recipient (whether or not known to such Lender or other Payment Recipient on its behalf) (any such funds, whether transmitted or received as a payment, prepayment or repayment of principal, interest, fees, distribution or otherwise, individually and collectively, an "Erroneous Payment") and (y) demands in writing the return of such Erroneous Payment (or a portion thereof), such Erroneous Payment shall at all times remain the property of the Administrative Agent pending its return or repayment as contemplated below in this Section 9.11 and held in trust for the benefit of the Administrative Agent, and such Lender shall (or, with respect to any Payment Recipient who received such funds on its behalf, shall cause such Payment Recipient to) promptly, but in no event later than ten (10) Business Days after the receipt of the demand in clause (y) above (or such later date as the Administrative Agent may, in its sole discretion, specify in writing), return to the Administrative Agent the amount of any such Erroneous Payment (or portion thereof) as to which such a demand was made, in same day funds (in the currency so received), together with interest thereon (except to the extent waived in writing by the Administrative Agent) in respect of each day from and including the date such Erroneous Payment (or portion thereof) was received by such Payment Recipient to the date such amount is repaid to the Administrative Agent in same day funds at the greater of the Federal Funds Rate and a rate determined by the Administrative Agent in accordance with banking industry rules on interbank compensation from time to time in effect. A notice of the Administrative Agent to any Payment Recipient under this clause (a) shall be conclusive, absent manifest error.

(b) Without limiting immediately preceding clause (a), each Lender or any Person who has received funds on behalf of a Lender (and each of their respective successors and assigns), agrees that if it receives a payment, prepayment or repayment (whether received as a payment, prepayment or repayment of principal, interest, fees, distribution or otherwise) from the Administrative Agent (or any of its Affiliates) (x) that is in a different amount than, or on a different date from, that specified in this Agreement or in a notice of payment, prepayment or repayment sent by the Administrative Agent (or any of its Affiliates) with respect to such payment, prepayment or repayment, (y) that was not preceded or accompanied by a notice of payment, prepayment or repayment sent by the Administrative Agent (or any of its Affiliates), or (z) that such Lender or other such recipient, otherwise becomes aware was transmitted, or received, in error or by mistake (in whole or in part), then in each such case:

(i) it acknowledges and agrees that (A) in the case of immediately preceding clauses (x) or (y), an error and mistake shall be presumed to have been made (absent written confirmation from the Administrative Agent to the contrary) or (B) an error and mistake has been made (in the case of immediately preceding clause (z)), in each case, with respect to such payment, prepayment or repayment; and

(ii) such Lender shall (and shall cause any other recipient that receives funds on its respective behalf to) promptly (and, in all events, within one Business Day of its knowledge of the occurrence of any of the circumstances described in immediately preceding clauses (x), (y) and (z)) notify the Administrative Agent of its receipt of such payment, prepayment or repayment, the details thereof (in reasonable detail) and that it is so notifying the Administrative Agent pursuant to this Section 9.11(b).

For the avoidance of doubt, the failure to deliver a notice to the Administrative Agent pursuant to this Section 9.11(b) shall not have any effect on a Payment Recipient's obligations pursuant to Section 9.11(a) or on whether or not an Erroneous Payment has been made.

(c) Each Lender hereby authorizes the Administrative Agent to set off, net and apply any and all amounts at any time owing to such Lender under any Loan Document, or otherwise payable or distributable by the Administrative Agent to such Lender under any Loan Document with respect to any payment of principal, interest, fees or other amounts, against any amount that the Administrative Agent has demanded to be returned under immediately preceding clause (a).

(d) The parties hereto agree that (x) irrespective of whether the Administrative Agent may be equitably subrogated, in the event that an Erroneous Payment (or portion thereof) is not recovered from any Payment Recipient that has received such Erroneous Payment (or portion thereof) for any reason, the Administrative Agent shall be subrogated to all the rights and interests of such Payment Recipient (and, in the case of any Payment Recipient who has received funds on behalf of a Lender to the rights and interests of such Lender, as the case may be) under the Loan Documents with respect to such amount (the "Erroneous Payment Subrogation Rights") and (y) an Erroneous Payment shall not pay, prepay, repay, discharge or otherwise satisfy any Obligations owed by the Borrower; provided that this Section 9.11 shall not be interpreted to increase (or accelerate the due date for), or have the effect of increasing (or accelerating the due date for), the Obligations of the Borrower relative to the amount (or timing for payment) of the Obligations that would have been payable had such Erroneous Payment not been made by the Administrative Agent; provided, further, that for the avoidance of doubt, immediately preceding clauses (x) and (y) shall not apply to the extent any such Erroneous Payment is, and solely with respect to the amount of such Erroneous Payment that is, comprised of funds received by the Administrative Agent from, or on behalf of (including through the exercise of remedies under any Loan Document), the Borrower for the purpose of a payment on the Obligations.

(e) To the extent permitted by Applicable Law, no Payment Recipient shall assert any right or claim to an Erroneous Payment, and hereby waives, and is deemed to waive, any claim, counterclaim, defense or right of set-off or recoupment with respect to any demand, claim or counterclaim by the Administrative Agent for the return of any Erroneous Payment received, including, without limitation, any defense based on "discharge for value" or any similar doctrine.

Each party's obligations, agreements and waivers under this Section 9.11 shall survive the resignation or replacement of the Administrative Agent, any transfer of rights or obligations by, or the replacement of, a Lender, the termination of the applicable Commitments or the repayment, satisfaction or discharge of all Obligations (or any portion thereof) under any Loan Document.

9.12 Survival.

This Article IX shall survive the termination of this Agreement, the repayment, satisfaction or discharge of all Obligations and the resignation, removal or replacement of the Administrative Agent.

9.13 **Certain Irish Matters**.

(a) The Administrative Agent shall be party to any Irish Security Document in its capacity as collateral agent and security trustee for and on behalf of itself and the Secured Parties pursuant to the terms and conditions of this Agreement and the relevant Irish Security Document. It will exercise its powers and authority under the Irish Security Documents in the manner provided for in the this Agreement and, in so acting, the Administrative Agent shall have the protections,

immunities, rights, powers, authorisations, indemnities and benefits conferred on it under and by this Agreement and the other Loan Documents.

(b) The Administrative Agent shall not owe any fiduciary duties to any party to the Irish Security Documents or any of their directors, employees, agents or affiliates.

(c) On the terms set out in the Loan Documents, the Administrative Agent declares itself trustee of the security created pursuant to the Irish Security Documents and other rights (including but not limited to the benefit of the covenants contained therein), titles and interests constituted by the Irish Security Documents and of all monies, property and assets paid to the Administrative Agent or to its order or held by the Administrative Agent or its nominee or received or recovered by the Administrative Agent or its nominee pursuant to or in connection with any Irish Security Documents with effect from the date of the relevant Irish Security Document to hold the same on trust for itself and each of the Secured Parties absolutely in accordance with their entitlements under the Loan Documents (save as may otherwise be agreed between the Administrative Agent and the other Secured Parties from time to time).

(d) All moneys received by the Administrative Agent shall be held by it upon trust for itself and the Secured Parties according to their respective interests to apply the same in accordance with the Loan Documents.

(e) The rights, powers and discretions conferred on the Administrative Agent by the Irish Security Documents shall be supplemental to the Trustee Acts of Ireland 1888 to 1989 and in addition to any other rights, powers and discretions which may be vested in the Administrative Agent by the Loan Documents, law or otherwise.

(f) Where there are any inconsistencies between the Trustee Acts of Ireland 1888 to 1989 and the provisions of the Loan Documents, the provisions of the Loan Documents shall, to the extent allowed by law, prevail.

(g) The trusts set out in this section in respect of the Irish Security Documents shall be wound up when the Liens created thereunder are released in accordance with Sections 9.10 or11.03. At that time the Administrative Agent shall, at the request of and at the sole cost of the relevant Loan Party, release, without recourse or warranty, all of the security then held by it pursuant to the relevant Irish Security Document and the Administrative Agent shall be released from its obligations thereunder (save for those which arose prior to such winding-up).

ARTICLE X

CONTINUING GUARANTY

10.01 Guaranty.

Each Guarantor hereby absolutely and unconditionally, jointly and severally guarantees, as primary obligor and as a guaranty of payment and performance and not merely as a guaranty of collection, prompt payment when due, whether at stated maturity, by required prepayment, upon acceleration, demand or otherwise, and at all times thereafter, of any and all Obligations, (for each Guarantor, subject to the proviso in this sentence, its "*Guaranteed Obligations*"); *provided* that the liability of each Guarantor individually with respect to this Guaranty shall be limited to an aggregate amount equal to the largest amount that would

not render its obligations hereunder subject to avoidance under Section 548 of the Bankruptcy Code of the United States or any comparable provisions of any applicable state law. Without limiting the generality of the foregoing, the Guaranteed Obligations shall include any such indebtedness, obligations, and liabilities, or portion thereof, which may be or hereafter become unenforceable or compromised or shall be an allowed or disallowed claim under any proceeding or case commenced by or against any debtor under any Debtor Relief Laws. The Administrative Agent's books and records showing the amount of the Obligations shall be admissible in evidence in any action or proceeding, and shall be binding upon each Guarantor, and conclusive for the purpose of establishing the amount of the Obligations absent manifest error. This Guaranty shall not be affected by the genuineness, validity, regularity or enforceability of the Obligations or any instrument or agreement evidencing any Obligations, or by the existence, validity, enforceability, perfection, non-perfection or extent of any collateral therefor, or by any fact or circumstance relating to the Obligations which might otherwise constitute a defense to the obligations of the Guarantors, or any of them, under this Guaranty, and each Guarantor hereby irrevocably waives any defenses it may now have or hereafter acquire in any way relating to any or all of the foregoing (other than that the Obligations have been in paid in full).

10.02 Rights of Lenders.

Each Guarantor consents and agrees that the Secured Parties may, at any time and from time to time, without notice or demand, and without affecting the enforceability or continuing effectiveness hereof: (a) amend, extend, renew, compromise, discharge, accelerate or otherwise change the time for payment or the terms of the Obligations or any part thereof; (b) take, hold, exchange, enforce, waive, release, fail to perfect, sell, or otherwise dispose of any security for the payment of this Guaranty or any Obligations; (c) apply such security and direct the order or manner of sale thereof as the Required Lenders in their sole discretion may determine; and (d) release or substitute one or more of any endorsers or other guarantors of any of the Obligations. Without limiting the generality of the foregoing, each Guarantor consents to the taking of, or failure to take, any action which might in any manner or to any extent vary the risks of such Guarantor under this Guaranty or which, but for this provision, might operate as a discharge of such Guarantor.

10.03 Certain Waivers.

Each Guarantor waives (a) any defense arising by reason of any disability or other defense of the Borrower or any other guarantor, or the cessation from any cause whatsoever (including any act or omission of any Secured Party) of the liability of the Borrower or any other Loan Party; (b) any defense based on any claim that such Guarantor's obligations exceed or are more burdensome than those of the Borrower or any other Loan Party; (c) the benefit of any statute of limitations affecting any Guarantor's liability hereunder; (d) any right to proceed against the Borrower or any other Loan Party, proceed against or exhaust any security for the Obligations, or pursue any other remedy in the power of any Secured Party whatsoever; (e) any benefit of and any right to participate in any security now or hereafter held by any Secured Party; and (f) to the fullest extent permitted by Applicable Law, any and all other defenses or benefits that may be derived from or afforded by Applicable Law limiting the liability of or exonerating guarantors or sureties. Each Guarantor expressly waives all setoffs and counterclaims and all presentments, demands for payment or performance, notices of nonpayment or nonperformance, protests, notices of protest, notices of dishonor and all other notices or demands of any kind or nature whatsoever with respect to the Obligations, and all notices of acceptance of this Guaranty or of the existence, creation or incurrence of new or additional Obligations.

10.04 Obligations Independent.

The obligations of each Guarantor hereunder are those of primary obligor, and not merely as surety, and are independent of the Obligations and the obligations of any other guarantor, and a separate action may be brought against each Guarantor to enforce this Guaranty whether or not the Borrower or any other person or entity is joined as a party.

10.05 Subrogation.

No Guarantor shall exercise any right of subrogation, contribution, indemnity, reimbursement or similar rights with respect to any payments it makes under this Guaranty until the Facility Termination Date. If any amounts are paid to a Guarantor in violation of the foregoing limitation, then such amounts shall be held in trust for the benefit of the Secured Parties and shall forthwith be paid to the Secured Parties to reduce the amount of the Obligations, whether matured or unmatured.

10.06 Termination; Reinstatement.

This Guaranty is a continuing and irrevocable guaranty of all Obligations now or hereafter existing and shall remain in full force and effect until the Facility Termination Date. Notwithstanding the foregoing, this Guaranty shall continue in full force and effect or be revived, as the case may be, if any payment by or on behalf of the Borrower or a Guarantor is made, or any of the Secured Parties exercises its right of setoff, in respect of the Obligations and such payment or the proceeds of such setoff or any part thereof is subsequently invalidated, declared to be fraudulent or preferential, set aside or required (including pursuant to any settlement entered into by any of the Secured Parties in their discretion) to be repaid to a trustee, receiver or any other party, in connection with any proceeding under any Debtor Relief Laws or otherwise, all as if such payment had not been made or such setoff had not occurred and whether or not the Secured Parties are in possession of or have released this Guaranty and regardless of any prior revocation, rescission, termination or reduction. The obligations of each Guarantor under this Section 10.06 shall survive termination of this Guaranty.

10.07 Stay of Acceleration.

If acceleration of the time for payment of any of the Obligations is stayed, in connection with any case commenced by or against a Guarantor or the Borrower under any Debtor Relief Laws, or otherwise, all such amounts shall nonetheless be payable by each Guarantor, jointly and severally, immediately upon demand by the Secured Parties.

10.08 Condition of Borrower.

Each Guarantor acknowledges and agrees that it has the sole responsibility for, and has adequate means of, obtaining from the Borrower and any other guarantor such information concerning the financial condition, business and operations of the Borrower and any such other guarantor as such Guarantor requires, and that none of the Secured Parties has any duty, and such Guarantor is not relying on the Secured Parties at any time, to disclose to it any information relating to the business, operations or financial condition of the Borrower or any other guarantor (each Guarantor waiving any duty on the part of the Secured Parties to disclose such information and any defense relating to the failure to provide the same).

10.09 Appointment of Borrower.

Subject to Applicable Law, each of the Loan Parties hereby appoints the Borrower to act as its agent for all purposes of this Agreement, the other Loan Documents and all other documents and electronic

platforms entered into in connection herewith and agrees that (a) the Borrower may execute such documents and provide such authorizations on behalf of such Loan Parties as the Borrower deems appropriate in its sole discretion and each Loan Party shall be obligated by all of the terms of any such document and/or authorization executed on its behalf, (b) any notice or communication delivered by the Administrative Agent or a Lender to the Borrower shall be deemed delivered to each Loan Party and (c) the Administrative Agent or the Lenders may accept, and be permitted to rely on, any document, authorization, instrument or agreement executed by the Borrower on behalf of each of the Loan Parties.

10.10 Right of Contribution.

The Guarantors agree among themselves that, in connection with payments made hereunder, each Guarantor shall have contribution rights against the other Guarantors as permitted under Applicable Law.

10.11 **Guarantee Limitations**

Notwithstanding any provision to the contrary contained herein or in any other of the Loan Document, the liabilities of each Guarantor under any Loan Documents shall not apply to any liability to the extent that it would result in such guaranty constituting unlawful financial assistance under section 82 of the Companies Act (Ireland) or result in a breach of section 239 of the Companies Act (Ireland).

ARTICLE XI. MISCELLANEOUS

11.01 Amendments, Etc.

(a) Subject to Section 3.03(c) and Section 11.01(d), no amendment or waiver of any provision of this Agreement or any other Loan Document, and no consent to any departure by the Borrower or any other Loan Party therefrom, shall be effective unless in writing signed by the Required Lenders (or by the Administrative Agent with the consent of the Required Lenders) and the Borrower or the applicable Loan Party, as the case may be, and acknowledged by the Administrative Agent, and each such waiver or consent shall be effective only in the specific instance and for the specific purpose for which given; *provided*, *however*, that no such amendment, waiver or consent shall:

(i) extend or increase the Commitment of any Lender (or reinstate any Commitment terminated pursuant to Section 8.02) without the written consent of such Lender (it being understood and agreed that a waiver of any condition precedent in Section 4.02 or of any Default or Event of Default or a mandatory reduction in Commitments is not considered an extension or increase in Commitments of any Lender);

(ii) postpone any date fixed by this Agreement or any other Loan Document for any payment (excluding Mandatory Prepayments) of principal, interest, fees or other amounts due to the Lenders (or any of them) hereunder or under such other Loan Document without the written consent of each Lender entitled to such payment;

(iii) reduce the principal of, or the rate of interest specified herein on, any Loan, or any fees or other amounts payable hereunder or under any other Loan Document without the written consent of each Lender entitled to such amount; *provided*, *however*, that only the consent of the Required Lenders shall be necessary to amend the definition of "Default Rate" or to waive any obligation of the Borrower to pay interest at the Default Rate;

(iv) change (i) Section 8.03, Section 2.10 or Section 2.12 in a manner that would alter the pro rata sharing of payments required thereby without the written consent of each Lender or (ii) Section 2.10(f) in a manner that would alter the *pro rata* application required thereby without the written consent of each Lender directly affected thereby;

(v) change any provision of this Section 11.01 or the definition of "Required Lenders" or any other provision of any Loan Document specifying the number or percentage of Lenders required to amend, waive or otherwise modify any rights hereunder or thereunder or make any determination or grant any consent hereunder, without the written consent of each Lender;

(vi) release all or substantially all of the Collateral in any transaction or series of related transactions, without the written consent of each Lender, except upon the occurrence of the Facility Termination Date;

(vii) subordinate the Loans in right of payment to any other Indebtedness, without the written consent of each Lender directly affected thereby;

(viii) release all or substantially all of the value of the Guarantees, without the written consent of each Lender, except to the extent the release of any Subsidiary from the Guaranty is permitted pursuant to Section 9.10; or

(ix) release the Borrower or permit the Borrower to assign or transfer any of its rights or obligations under this Agreement or the other Loan Documents without the consent of each Lender;

and *provided*, *further*, that (A) no amendment, waiver or consent shall, unless in writing and signed by the Administrative Agent in addition to the Lenders required above, affect the rights or duties of, or any fees or other amounts payable to, the Administrative Agent under this Agreement or any other Loan Document and (B) the Agency Fee Letter and the Fee Letter may be amended, or rights or privileges thereunder waived, in a writing executed only by the parties thereto.

(b) Notwithstanding anything to the contrary herein, (i) no Defaulting Lender shall have any right to approve or disapprove any amendment, waiver or consent hereunder (and any amendment, waiver or consent which by its terms requires the consent of all Lenders or each affected Lender, or all Lenders, may be effected with the consent of the applicable Lenders other than Defaulting Lenders), except that (A) the Commitment of any Defaulting Lender may not be increased or extended without the consent of such Lender and (B) any waiver, amendment or modification requiring the consent of all Lenders or each affected Lender that by its terms affects any Defaulting Lender disproportionately adversely relative to other affected Lenders shall require the consent of such Defaulting Lender; (ii) each Lender is entitled to vote as such Lender sees fit on any bankruptcy reorganization plan that affects the Loans, and each Lender acknowledges that the provisions of Section 1126(c) of the Bankruptcy Code of the United States supersedes the unanimous consent provisions set forth herein and (iii) the Required Lenders shall determine whether or not to allow a Loan Party to use cash collateral in the context of a bankruptcy or insolvency proceeding and such determination shall be binding on all of the Lenders.

(c) Notwithstanding anything to the contrary herein, this Agreement may be amended and restated without the consent of any Lender (but with the consent of the Borrower, the Required Lenders and the Administrative Agent) if, upon giving effect to such amendment and restatement, such Lender shall no longer be a party to this Agreement (as so amended and restated), the

Commitments of such Lender shall have terminated, such Lender shall have no other commitment or other obligation hereunder and shall have been paid in full all principal, interest and other amounts owing to it or accrued for its account under this Agreement.

(d) Notwithstanding any provision herein to the contrary, if the Administrative Agent, the Required Lenders and the Borrower acting together identify any ambiguity, omission, mistake, typographical error or other defect in any provision of this Agreement or any other Loan Document (including the schedules and exhibits thereto), then the Administrative Agent, the Required Lenders and the Borrower shall be permitted to amend, modify or supplement such provision to cure such ambiguity, omission, mistake, typographical error or other defect, and such amendment shall become effective without any further action or consent of any other party to this Agreement.

11.02 Notices; Effectiveness; Electronic Communications.

(a) Notices Generally. Except in the case of notices and other communications expressly permitted to be given by telephone (and except as provided in clause (b) below), all notices and other communications provided for herein shall be in writing and shall be delivered by hand or overnight courier service, mailed by certified or registered mail or sent by fax transmission or e-mail transmission as follows, and all notices and other communications expressly permitted hereunder to be given by telephone shall be made to the applicable telephone number, as follows:

(i) if to the Borrower or any other Loan Party, the Administrative Agent, or the Required Lenders, to the address, fax number, e-mail address or telephone number specified for such Person on Schedule 1.01(a); and

(ii) if to any other Lender, to the address, fax number, e-mail address or telephone number specified in its Administrative Questionnaire (including, as appropriate, notices delivered solely to the Person designated by a Lender on its Administrative Questionnaire then in effect for the delivery of notices that may contain material non-public information relating to the Borrower).

Notices and other communications sent by hand or overnight courier service, or mailed by certified or registered mail, shall be deemed to have been given when received; notices and other communications sent by fax transmission shall be deemed to have been given when sent (except that, if not given during normal business hours for the recipient, shall be deemed to have been given at the opening of business on the next Business Day for the recipient). Notices and other communications delivered through electronic communications to the extent provided in clause (b) below shall be effective as provided in such clause (b).

(b) Electronic Communications.

(i) Notices and other communications to the Administrative Agent, the Lenders and the Required Lenders hereunder may be delivered or furnished by electronic communication (including e-mail, FPML messaging, and Internet or intranet websites) pursuant to an electronic communications agreement (or such other procedures approved by the Administrative Agent in its sole discretion); *provided* that the foregoing shall not apply to notices to any Lender pursuant to Article II if such Lender has notified the Administrative Agent that it is incapable of receiving notices under such Article II by electronic communication. The Administrative Agent, the Required Lenders or the Borrower may each, in its discretion, agree to accept notices and other communications to

it hereunder by electronic communications pursuant to procedures approved by it, *provided* that approval of such procedures may be limited to particular notices or communications.

(ii) Unless the Administrative Agent otherwise prescribes, (A) notices and other communications sent to an e-mail address shall be deemed received upon the sender's receipt of an acknowledgment from the intended recipient (such as by the "return receipt requested" function, as available, return e-mail or other written acknowledgement) and (B) notices and other communications posted to an Internet or intranet website shall be deemed received by the intended recipient upon the sender's receipt of an acknowledgement from the intended recipient (such as by the "return receipt requested" function, as available, return e-mail address or other written acknowledgement) indicating that such notice or communication is available and identifying the website address therefor; *provided* that for both clauses (A) and (B), if such notice or other communication is not sent during the normal business hours of the recipient, such notice, email or communication shall be deemed to have been sent at the opening of business on the next Business Day for the recipient.

(c) The Platform. THE PLATFORM IS PROVIDED "AS IS" AND "AS AVAILABLE." THE AGENT PARTIES (AS DEFINED BELOW) DO NOT WARRANT THE ACCURACY OR COMPLETENESS OF THE BORROWER MATERIALS OR THE ADEQUACY OF THE PLATFORM, AND EXPRESSLY DISCLAIM LIABILITY FOR ERRORS IN OR OMISSIONS FROM THE BORROWER MATERIALS. NO WARRANTY OF ANY KIND, EXPRESS, IMPLIED OR STATUTORY, INCLUDING ANY WARRANTY OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, NON-INFRINGEMENT OF THIRD PARTY RIGHTS OR FREEDOM FROM VIRUSES OR OTHER CODE DEFECTS, IS MADE BY ANY AGENT PARTY IN CONNECTION WITH THE BORROWER MATERIALS OR THE PLATFORM. In no event shall the Administrative Agent or any of their Related Parties (collectively, the "*Agent Parties*") have any liability to the Borrower, any Lender or any other Person for losses, claims, damages, liabilities or expenses of any kind (whether in tort, contract or otherwise) arising out of the Borrower's, any Loan Party's or the Administrative Agent's transmission of Borrower Materials or notices through the Platform, any other electronic platform or electronic messaging service, or through the Internet, except to the extent that such losses, claims, damages, liabilities or expenses are determined by a court of competent jurisdiction by a final and non-appealable judgment to have resulted from the gross negligence or willful misconduct of such Agent Party.

(d) Change of Address, Etc. Each of the Borrower, the Administrative Agent, and the Required Lenders may change its address, fax number or telephone number or e-mail address for notices and other communications hereunder by notice to the other parties hereto. Each other Lender may change its address, fax number or telephone number or e-mail address for notices and other communications hereunder by notice to the Borrower, the Administrative Agent and the Required Lenders. In addition, each Lender agrees to notify the Administrative Agent from time to time to ensure that the Administrative Agent has on record (i) an effective address, contact name, telephone number, fax number and e-mail address to which notices and other communications may be sent and (ii) accurate wire instructions for such Lender. Furthermore, each Public Lender agrees to cause at least one (1) individual at or on behalf of such Public Lender to at all times have selected the "Private Side Information" or similar designation on the content declaration screen of the Platform in order to enable such Public Lender or its delegate, in accordance with such Public Lender's compliance procedures and Applicable Law, including United States federal and state securities Laws, to make reference to Borrower Materials that are not made available through the "Public Side Information" portion of the Platform and that may contain material non-public

information with respect to the Borrower or its securities for purposes of United States federal or state securities laws.

(e) Reliance by Administrative Agent and Lenders. The Administrative Agent and the Lenders shall be entitled to rely and act upon any notices (including, without limitation, telephonic or electronic notices, Loan Notices and Notice of Loan Prepayment) purportedly given by or on behalf of any Loan Party even if (i) such notices were not made in a manner specified herein, were incomplete or were not preceded or followed by any other form of notice specified herein, or (ii) the terms thereof, as understood by the recipient, varied from any confirmation thereof. The Loan Parties shall indemnify the Administrative Agent, the Required Lenders, each Lender and the Related Parties of each of them from all losses, costs, expenses and liabilities resulting from the reliance by such Person on each notice purportedly given by or on behalf of a Loan Party. All telephonic notices to and other telephonic communications with the Administrative Agent may be recorded by the Administrative Agent, and each of the parties hereto hereby consents to such recording.

11.03 No Waiver; Cumulative Remedies; Enforcement.

(a) No failure by any Lender, the Required Lenders or the Administrative Agent to exercise, and no delay by any such Person in exercising, any right, remedy, power or privilege hereunder or under any other Loan Document shall operate as a waiver thereof; nor shall any single or partial exercise of any right, remedy, power or privilege hereunder or under any other Loan Document preclude any other or further exercise thereof or the exercise of any other right, remedy, power or privilege. The rights, remedies, powers and privileges herein provided, and provided under each other Loan Document, are cumulative and not exclusive of any rights, remedies, powers and privileges provided by law.

(b) Notwithstanding anything to the contrary contained herein or in any other Loan Document, the authority to enforce rights and remedies hereunder and under the other Loan Documents against the Loan Parties or any of them shall be vested exclusively in, and all actions and proceedings at law in connection with such enforcement shall be instituted and maintained exclusively by, the Administrative Agent in accordance with Section 8.02 for the benefit of all the Lenders; *provided*, *however*, that the foregoing shall not prohibit (a) the Administrative Agent from exercising on its own behalf the rights and remedies that inure to its benefit (solely in its capacity as Administrative Agent) hereunder and under the other Loan Documents, (b) any Lender from exercising setoff rights in accordance with Section 11.08 (subject to the terms of Section 2.12), or (c) any Lender from filing proofs of claim or appearing and filing pleadings on its own behalf during the pendency of a proceeding relative to any Loan Party under any Debtor Relief Law; and *provided*, *further*, that if at any time there is no Person acting as Administrative Agent hereunder and under the other Loan Documents, then (i) the Required Lenders shall have the rights otherwise ascribed to the Administrative Agent pursuant to Section 8.02 and (ii) in addition to the matters set forth in clauses (b) and (c) of the preceding proviso, any Lender may, with the consent of the Required Lenders, enforce any rights and remedies available to it and as authorized by the Required Lenders.

11.04 Expenses; Indemnity; Damage Waiver.

(a) Costs and Expenses. The Loan Parties shall pay (i) all reasonable and documented out-of-pocket expenses incurred by the Administrative Agent and the Lenders (limited, in the case of legal counsel, to the reasonable and documented fees, charges and disbursements of one legal counsel to the Lenders and one legal counsel to the Administrative Agent and, if reasonably

necessary, of one local counsel to the Administrative Agent and of one local counsel to the Lenders (which may include a single special counsel acting in multiple jurisdictions), in each case, in each relevant jurisdiction material to the interests of the Administrative Agent and the Lenders, in connection with the preparation, negotiation, execution, delivery and administration of this Agreement and the other Loan Documents or any amendments, modifications or waivers of the provisions hereof or thereof (whether or not the transactions contemplated hereby or thereby shall be consummated), subject (A) with respect to amounts owing to the Lenders, to the terms of the Fee Letter, and ((B) with respect to amounts owing to the Administrative Agent, to the terms of the Agency Fee Letter, and (ii) all reasonable, documented out-of-pocket expenses incurred by the Administrative Agent, or any Lender (limited to the reasonable fees, documented out-of-pocket disbursements and other charges of one legal counsel to the Administrative Agent and one legal counsel to the Lenders (taken as a whole), and, if reasonably necessary, of one local counsel to the Administrative Agent and of one local counsel to the Lenders (taken as a whole) (which may include a single special counsel acting in multiple jurisdictions), in each case, in each relevant jurisdiction material to the interests of the Administrative Agent and the Lenders, in connection with the enforcement or protection of its rights (A) in connection with this Agreement and the other Loan Documents, including its rights under this Section 11.04, or (B) in connection with Loans made hereunder, including all such out-of-pocket expenses incurred during any workout, restructuring or negotiations in respect of such Loans.

(b) Indemnification by the Loan Parties. The Loan Parties shall indemnify the Administrative Agent (and any sub-agent thereof), each Lender and the Required Lenders, and each Related Party of any of the foregoing Persons (each such Person being called an "*Indemnitee*") against, and hold each Indemnitee harmless from, any and all losses, claims, damages, liabilities and related expenses (limited, in the case of legal counsel, to the reasonable fees, documented out-of-pocket disbursements and other charges of one legal counsel to the Administrative Agent and its Related Parties and one legal counsel to the other Indemnitees (taken as a whole), and, if reasonably necessary, of one local counsel to the Administrative Agent and its Related Parties and one local counsel to the other Indemnitees (taken as a whole), in each case, in each relevant material jurisdiction (which may include a single special counsel acting in multiple jurisdictions, in each case, in relevant jurisdictions material to the interests of the Lenders)) incurred by any Indemnitee or asserted against any Indemnitee by any Person (including the Borrower or any other Loan Party) (provided that, in the case of any conflict (perceived or actual), one additional primary and local counsel in each relevant material jurisdiction for similarly situated Indemnitees shall also be covered) arising out of, in connection with, or as a result of (i) the execution or delivery of this Agreement, any other Loan Document or any agreement or instrument contemplated hereby or thereby, the performance by the parties hereto of their respective obligations hereunder or thereunder or the consummation of the transactions contemplated hereby or thereby, or, in the case of the Administrative Agent (and any sub-agent thereof) and its Related Parties only, the administration of this Agreement and the other Loan Documents (including in respect of any matters addressed in Section 3.01), (ii) any Loan or the use or proposed use of the proceeds therefrom, (iii) any actual or alleged presence or Release of Hazardous Materials on or from any property owned, leased or operated by a Loan Party or any of its Subsidiaries, or any Environmental Liability related in any way to a Loan Party or any of its Subsidiaries, or (iv) any actual or prospective claim, litigation, investigation or proceeding relating to any of the foregoing, whether based on contract, tort or any other theory, whether brought by a third party or by the Borrower or any other Loan Party, and regardless of whether any Indemnitee is a party thereto; *provided* that such indemnity shall not, as to any Indemnitee, be available to the extent that such losses, claims, damages, liabilities or related expenses (x) are determined by a court of competent jurisdiction by final and non-appealable judgment to have resulted from the gross negligence or willful misconduct of such Indemnitee, (y) other than in the case of the Administrative Agent and its Related Parties,

result from a claim brought by the Borrower or any other Loan Party against an Indemnitee for a material breach of such Indemnitee's obligations hereunder or under any other Loan Document, if the Borrower or such Loan Party has obtained a final and non-appealable judgment in its favor on such claim as determined by a court of competent jurisdiction or (z) result from a claim not involving an act or omission of the Borrower and that is brought by an Indemnitee against another Indemnitee (other than against the Administrative Agent, any sub-agents and their Related Parties, in their capacities as such). Each applicable Indemnitee shall use commercially reasonable efforts to consult with the Borrower prior to entering into a settlement of any proceeding that would result in any liability to any Loan Party. Without limiting the provisions of Section 3.01(c), this Section 11.04(b) shall not apply with respect to Taxes other than any Taxes that represent losses, claims, damages, etc. arising from any non-Tax claim.

(c) Reimbursement by Lenders. To the extent that the Loan Parties for any reason fail to indefeasibly pay any amount required under clause (a) or (b) of this Section 11.04 to be paid by them to the Administrative Agent (or any sub-agent thereof) or any Related Party of any of the foregoing, each Lender severally agrees to pay to the Administrative Agent (or any such sub-agent) or such Related Party such Lender's pro rata share (determined as of the time that the applicable unreimbursed expense or indemnity payment is sought (or, in the event at such time all the Commitments shall have terminated and all the Loans shall have been repaid in full, as of the time most recently prior thereto when any Loans or Commitments remained outstanding)) of such unpaid amount; *provided*, that the unreimbursed expense or indemnified loss, claim, damage, liability or related expense, as the case may be, was incurred by or asserted against the Administrative Agent (or any such sub-agent) in its capacity as such, or against its Related Party acting for the Administrative Agent (or any such sub-agent) in connection with such capacity. For purposes hereof, "pro rata share" shall mean, with respect to any Lender at any time, a fraction (expressed as a percentage, carried out to the ninth decimal place), the numerator of which is the amount of the unused Commitments and the Outstanding Amount of the Loans of such Lender at such time and the denominator of which is the aggregate amount of the unused Commitments and the aggregate Outstanding Amount of the Loans at the time. Each Lender hereby agrees that, notwithstanding any exclusions from the Loan Parties' indemnification obligations under Section 11.04(b) for gross negligence or willful misconduct of the applicable Indemnitee, no action taken (or not taken) by the Administrative Agent (or any sub-agent thereof) or any Related Party of any of the foregoing in accordance with the directions of the Required Lenders or the Required Lenders (or such other number or percentage of the Lenders as shall be provided by the Loan Documents) shall be deemed to constitute gross negligence or willful misconduct for the purposes of the Lenders' payment and indemnification obligations under this clause (c). Each Lender hereby authorizes the Administrative Agent to set off and apply any and all amounts at any time owing to such Lender under any Loan Document or otherwise payable by the Administrative Agent to such Lender from any source against any amount due to the Administrative Agent under this clause (c). The obligations of the Lenders under this clause (c) are subject to the provisions of Section 2.10(d).

(d) Waiver of Consequential Damages, Etc. To the fullest extent permitted by Applicable Law, no Loan Party shall assert, and each Loan Party hereby waives, and acknowledges that no other Person shall have, any claim against any Indemnitee, on any theory of liability, for special, indirect, consequential or punitive damages (as opposed to direct or actual damages) arising out of, in connection with, or as a result of, this Agreement, any other Loan Document or any agreement or instrument contemplated hereby, the transactions contemplated hereby or thereby, any Loan or the use of the proceeds thereof. No Indemnitee referred to in clause (b) above shall be liable for any damages arising from the use by unintended recipients of any information or other materials distributed to such unintended recipients by such Indemnitee through

telecommunications, electronic or other information transmission systems in connection with this Agreement or the other Loan Documents or the transactions contemplated hereby or thereby.

(e) Payments. All amounts due under this Section 11.04 shall be payable not later than fifteen (15) Business Days after demand therefor together with a reasonably detailed invoice with respect thereto.

(f) Survival. The agreements in this Section 11.04 and the indemnity provisions of Section 11.02(e) shall survive the resignation or removal of the Administrative Agent or the Required Lenders, the replacement of any Lender, the termination of the Aggregate Commitments and the repayment, satisfaction or discharge of all the other Obligations.

11.05 Payments Set Aside.

To the extent that any payment by or on behalf of the Borrower is made to the Administrative Agent or any Lender, or the Administrative Agent or any Lender exercises its right of setoff, and such payment or the proceeds of such setoff or any part thereof is subsequently invalidated, declared to be fraudulent or preferential, set aside or required (including pursuant to any settlement entered into by the Administrative Agent or such Lender in its discretion) to be repaid to a trustee, receiver or any other party, in connection with any proceeding under any Debtor Relief Law or otherwise, then (a) to the extent of such recovery, the obligation or part thereof originally intended to be satisfied shall be revived and continued in full force and effect as if such payment had not been made or such setoff had not occurred, and (b) each Lender severally agrees to pay to the Administrative Agent upon demand its applicable share (without duplication) of any amount so recovered from or repaid by the Administrative Agent, plus interest thereon from the date of such demand to the date such payment is made at a rate per annum equal to the Federal Funds Rate from time to time in effect. The obligations of the Lenders under clause (b) of the preceding sentence shall survive the payment in full of the Obligations and the termination of this Agreement.

11.06 Successors and Assigns.

(a) Successors and Assigns Generally. The provisions of this Agreement and the other Loan Documents shall be binding upon and inure to the benefit of the parties hereto and thereto and their respective successors and assigns permitted hereby, except neither the Borrower nor any other Loan Party may assign or otherwise transfer any of its rights or obligations hereunder or under any other of the other Loan Documents without the prior written consent of the Administrative Agent and each Lender and no Lender may assign or otherwise transfer any of its rights or obligations hereunder or under any of the other Loan Documents except (i) to an assignee in accordance with the provisions of Section 11.06(b), (ii) by way of participation in accordance with the provisions of Section 11.06(d), or (iii) by way of pledge or assignment of a security interest subject to the restrictions of Section 11.06(e) (and any other attempted assignment or transfer by any party hereto shall be null and void). Nothing in this Agreement, expressed or implied, shall be construed to confer upon any Person (other than the parties hereto, their respective successors and assigns permitted hereby, Participants to the extent provided in Section 11.06(d) and, to the extent expressly contemplated hereby, the Related Parties of each of the Administrative Agent and the Lenders) any legal or equitable right, remedy or claim under or by reason of this Agreement.

(b) Assignments by Lenders. Any Lender may at any time assign to one or more assignees all or a portion of its rights and obligations under this Agreement and the other Loan Documents (including all or a portion of its Commitment and the Loans at the time owing to it); *provided* that any such assignment shall be subject to the following conditions:

(i) Minimum Amounts.

(A) in the case of an assignment of the entire remaining amount of the assigning Lender's Commitment and/or the Loans at the time owing to it or contemporaneous assignments to related Approved Funds (determined after giving effect to such assignments) that equal at least the amount specified in clause (b)(i)(B) of this Section 11.06 in the aggregate or in the case of an assignment to a Lender, an Affiliate of a Lender or an Approved Fund, no minimum amount need be assigned; and

(B) in any case not described in clause (b)(i)(A) of this Section 11.06, the aggregate amount of the Commitment (which for this purpose includes Loans outstanding thereunder) or, if the Commitment is not then in effect, the principal outstanding balance of the Loans of the assigning Lender subject to each such assignment, determined as of the date the Assignment and Assumption with respect to such assignment is delivered to the Administrative Agent or, if "Trade Date" is specified in the Assignment and Assumption, as of the Trade Date, shall not be less than $5,000,000, unless each of the Administrative Agent and, so long as no Event of Default has occurred and is continuing, the Borrower otherwise consents (each such consent not to be unreasonably withheld or delayed).

(ii) Proportionate Amounts. Each partial assignment shall be made as an assignment of a proportionate part of all the assigning Lender's rights and obligations under this Agreement and the other Loan Documents with respect to the Loans and/or the Commitment assigned.

(iii) Required Consents. No consent shall be required for any assignment except to the extent required by clause (b)(i)(B) of this Section 11.06 and, in addition, the consent of the Borrower (such consent not to be unreasonably withheld or delayed, except that such consent may be withheld in the Borrower's sole discretion if such assignment would result in Blackstone Affiliated Entities no longer constituting Required Lenders) shall be required unless (1) an Event of Default has occurred and is continuing at the time of such assignment or (2) such assignment is to a Lender, an Affiliate of a Lender or an Approved Fund (provided that in the case of any such assignment by a Blackstone Affiliated Entity the assignee must be a Blackstone Affiliated Entity and must remain a Blackstone Affiliated Entity for so long as such assignee is a holder of Loans); provided that the Borrower shall be deemed to have consented to any such assignment unless it shall object thereto by written notice to the Administrative Agent within five (5) Business Days after having received notice thereof.

(iv) Assignment and Assumption. The parties to each assignment shall execute and deliver to the Administrative Agent an Assignment and Assumption, together with a processing and recordation fee in the amount of $3,500; *provided*, *however*, that the Administrative Agent may, in its sole discretion, elect to waive such processing and recordation fee in the case of any assignment. The assignee, if it is not a Lender, shall deliver to the Administrative Agent (x) an Administrative Questionnaire and its applicable tax form required under Section 3.01(f) and (y) if requested by the Administrative Agent, information reasonably requested by the Administrative Agent reasonably required by regulatory authorities under "know your customer" and anti-money laundering rules and regulations, including the PATRIOT Act.

(v) No Assignment to Certain Persons. No such assignment shall be made (A) to the Borrower or any of the Borrower's Affiliates or Subsidiaries, (B) to any Defaulting Lender or any of its Subsidiaries, or any Person who, upon becoming a Lender hereunder, would constitute any of the foregoing Persons described in this clause (B), (C) to a natural Person (or a holding company, investment vehicle or trust for, or owned and operated by or for the primary benefit of one or more natural Persons) or (D) except during an Event of Default pursuant to Section 8.01(f) or following an acceleration of the Loans, any Disqualified Institution.

(vi) Certain Additional Payments. In connection with any assignment of rights and obligations of any Defaulting Lender hereunder, no such assignment shall be effective unless and until, in addition to the other conditions thereto set forth herein, the parties to the assignment shall make such additional payments to the Administrative Agent in an aggregate amount sufficient, upon distribution thereof as appropriate (which may be outright payment, purchases by the assignee of participations or sub-participations, or other compensating actions, including funding, with the consent of the Borrower and the Administrative Agent, the applicable pro rata share of Loans previously requested but not funded by the Defaulting Lender, to each of which the applicable assignee and assignor hereby irrevocably consent), to (A) pay and satisfy in full all payment liabilities then owed by such Defaulting Lender to the Administrative Agent or any Lender hereunder (and interest accrued thereon) and (B) acquire (and fund as appropriate) its full pro rata share of all Loans in accordance with its Applicable Percentage. Notwithstanding the foregoing, in the event that any assignment of rights and obligations of any Defaulting Lender hereunder shall become effective under Applicable Law without compliance with the provisions of this clause (b)(vi), then the assignee of such interest shall be deemed to be a Defaulting Lender for all purposes of this Agreement until such compliance occurs.

(vii) Effect of Assignment. Subject to acceptance and recording thereof by the Administrative Agent pursuant to Section 11.06(c), from and after the effective date specified in each Assignment and Assumption, the assignee thereunder shall be a party to this Agreement and, to the extent of the interest assigned by such Assignment and Assumption, have the rights and obligations of a Lender under this Agreement, and the assigning Lender thereunder shall, to the extent of the interest assigned by such Assignment and Assumption, be released from its obligations under this Agreement (and, in the case of an Assignment and Assumption covering all of the assigning Lender's rights and obligations under this Agreement, such Lender shall cease to be a party hereto but shall continue to be entitled to the benefits of Sections 3.01, 3.04, 3.05 and 11.04 with respect to facts and circumstances occurring prior to the effective date of such assignment); *provided*, that except to the extent otherwise expressly agreed by the affected parties, no assignment by a Defaulting Lender will constitute a waiver or release of any claim of any party hereunder arising from that Lender's having been a Defaulting Lender. Upon request, the Borrower (at its expense) shall execute and deliver a Note to the assignee Lender. Any assignment or transfer by a Lender of rights or obligations under this Agreement that does not comply with this clause (b) shall be treated for purposes of this Agreement as a sale by such Lender of a participation in such rights and obligations in accordance with Section 11.06(d).

(c) Register. The Administrative Agent, acting solely for this purpose as a non-fiduciary agent of the Borrower (and such agency being solely for Tax purposes), shall maintain at one of its offices in the United States a copy of each Assignment and Assumption delivered to it (or the equivalent thereof in electronic form) and a register for the recordation of the names and

addresses of the Lenders, and the Commitments of, and principal amounts (and interest amounts) of the Loans owing to, each Lender pursuant to the terms hereof from time to time (the "***Register***"). The entries in the Register shall be conclusive, absent manifest error, and the Borrower, the Administrative Agent and the Lenders shall treat each Person whose name is recorded in the Register pursuant to the terms hereof as a Lender hereunder for all purposes of this Agreement. The Register shall be available for inspection by the Borrower and any Lender (with respect to such Lender's interest only), at any reasonable time and from time to time upon reasonable prior notice.

(d) Participations.

(i) Any Lender may at any time, without the consent of, or notice to, the Borrower or the Administrative Agent, sell participations to any Person (other than a natural Person, or a holding company, investment vehicle or trust for, or owned and operated for the primary benefit of one or more natural Persons, a Defaulting Lender, a Disqualified Institution or the Borrower or any of the Borrower's Affiliates or Subsidiaries) (each, a "***Participant***") in all or a portion of such Lender's rights and/or obligations under this Agreement (including all or a portion of its Commitment and/or the Loans owing to it); *provided* that (i) such Lender's obligations under this Agreement shall remain unchanged, (ii) such Lender shall remain solely responsible to the other parties hereto for the performance of such obligations and (iii) the Borrower, the Administrative Agent and the Lenders shall continue to deal solely and directly with such Lender in connection with such Lender's rights and obligations under this Agreement. For the avoidance of doubt, each Lender shall be responsible for the indemnity under Section 11.04(c) without regard to the existence of any participations.

(ii) Any agreement or instrument pursuant to which a Lender sells such a participation shall provide that such Lender shall retain the sole right to enforce this Agreement and to approve any amendment, modification or waiver of any provision of this Agreement; *provided* that such agreement or instrument may provide that such Lender will not, without the consent of the Participant, agree to any amendment, waiver or other modification described in the first proviso to Section 11.01 that affects such Participant. The Borrower agrees that each Participant shall be entitled to the benefits of Sections 3.01, 3.04 and 3.05 (subject to the requirements and limitations therein, including the requirements under Section 3.01(f) (it being understood that the documentation required under Section 3.01(e) shall be delivered to the Lender who sells the participation)) to the same extent as if it were a Lender and had acquired its interest by assignment pursuant to clause (b) of this Section 11.06; *provided* that such Participant (A) shall be subject to the provisions of Sections 3.06 and 11.13 as if it were an assignee under clause (b) of this Section 11.06 and (B) shall not be entitled to receive any greater payment under Sections 3.01 or 3.04, with respect to any participation, than the Lender from whom it acquired the applicable participation would have been entitled to receive, except to the extent such entitlement to receive a greater payment results from a Change in Law that occurs after the Participant acquired the applicable participation. Each Lender that sells a participation agrees, at the Borrower's request and expense, to use reasonable efforts to cooperate with the Borrower to effectuate the provisions of Section 3.06 with respect to any Participant. To the extent permitted by Applicable Law, each Participant also shall be entitled to the benefits of Section 11.08 as though it were a Lender; *provided* that such Participant agrees to be subject to Section 2.12 as though it were a Lender. Each Lender that sells a participation shall, acting solely for this purpose as a non-fiduciary agent of the Borrower, maintain a register on which it enters the name and address of each Participant and the principal amounts (and interest amounts) of each Participant's interest in the Loans or other

obligations under the Loan Documents (the "*Participant Register*"); *provided* that no Lender shall have any obligation to disclose all or any portion of the Participant Register (including the identity of any Participant or any information relating to a Participant's interest in any commitments, loans, letters of credit or its other obligations under any Loan Document) to any Person except to the extent that such disclosure is necessary to establish that such commitment, loan, letter of credit or other obligation is in registered form under Section 5f.103–1(c) of the United States Treasury Regulations. The entries in the Participant Register shall be conclusive absent manifest error, and such Lender shall treat each Person whose name is recorded in the Participant Register as the owner of such participation for all purposes of this Agreement notwithstanding any notice to the contrary. For the avoidance of doubt, the Administrative Agent (in its capacity as Administrative Agent) shall have no responsibility for maintaining a Participant Register.

(e) Certain Pledges. Any Lender may at any time pledge or assign a security interest in all or any portion of its rights under this Agreement (including under its Note or Notes, if any) to secure obligations of such Lender, including any pledge or assignment to secure obligations to a Federal Reserve Bank; *provided* that no such pledge or assignment shall release such Lender from any of its obligations hereunder or substitute any such pledgee or assignee for such Lender as a party hereto.

11.07 Treatment of Certain Information; Confidentiality.

(a) Treatment of Certain Information. Each of the Administrative Agent and the Lenders agrees to maintain the confidentiality of, and not to disclose to any Person, the Information (as defined below), except that Information may be disclosed (i) to its respective Affiliates, its auditors and its and their respective Related Parties (and, if such Person is a member of the Blackstone Entities, then it may make disclosures to any other member of the Blackstone Entities) (it being understood that the Persons to whom such disclosure is made will be informed of the confidential nature of such Information and instructed to keep such Information confidential), (ii) to the extent required or requested by any regulatory authority purporting to have jurisdiction over such Person or its Related Parties (including any self-regulatory authority, such as the National Association of Insurance Commissioners) (in which case the disclosing Administrative Agent or Lender, as applicable, agrees (except with respect to any audit or examination conducted by bank accountants or any governmental regulatory authority or self-regulatory authorities exercising examination or regulatory authority), to the extent not prohibited by Applicable Law, to promptly notify the Borrower of such disclosure, (iii) to the extent required by Applicable Laws or regulations or by any subpoena or similar legal process, in each case based upon the reasonable advice of the disclosing Administrative Agent's or Lender's legal counsel (in which case the disclosing Administrative Agent or Lender, as applicable, agrees (except with respect to any audit or examination conducted by bank accountants or any governmental bank regulatory authority or self-regulatory authorities exercising examination or regulatory authority), to the extent not prohibited by Applicable Law, to promptly notify the Borrower of such disclosure, (iv) to any other party hereto, (v) in connection with the exercise of any remedies hereunder or under any other Loan Document or any action or proceeding relating to this Agreement or any other Loan Document or the enforcement of rights hereunder or thereunder, (vi) subject to an agreement containing provisions substantially the same (or at least as restrictive) as those of this Section 11.07 (or as may otherwise be reasonably acceptable to the Borrower), to (A) any assignee of or Participant in, or any prospective assignee of or Participant in, any of its rights and obligations under this Agreement; *provided* that no such disclosure shall be made by such Lender or any of its respective Affiliates to any such Person that is a Disqualified Institution or that does not agree to be bound by the provisions of this Section 11.07(a); or (B) any actual or prospective party (or its Related Parties) to any swap,

derivative or other transaction under which payments are to be made by reference to the Borrower and its obligations, this Agreement or payments hereunder, (vii) on a confidential basis to (A) any rating agency in connection with rating the Borrower or its Subsidiaries or the credit facilities provided hereunder or (B) the provider of any Platform or other electronic delivery service used by the Administrative Agent to deliver Borrower Materials or notices to the Lenders, (viii) with the consent of the Borrower or (ix) to the extent such Information (A) becomes publicly available other than as a result of a breach of this Section 11.07, (B) becomes available to the Administrative Agent, any Lender or any of their respective Affiliates on a nonconfidential basis from a source other than the Borrower or on the Borrower's behalf and not in violation of any confidentiality agreement or obligation owed to the Borrower or (C) is already in the possession of or is independently discovered or developed by a party hereto without utilizing any Information received from the Borrower or violating the terms of this Section 11.07. For purposes of this Section 11.07, "*Information*" means all information received from, or on behalf of, the Borrower or any Subsidiary relating to the Borrower or any Subsidiary or any of their respective businesses, whether before or after the date of this Agreement, and any analyses, compilations, forecasts, and/or other documents prepared by the Lender or its Affiliates containing or based in whole or in part on any such furnished non-public information, other than any such information that is available to the Administrative Agent or any Lender on a nonconfidential basis prior to disclosure by the Borrower or any Subsidiary. Any Person required to maintain the confidentiality of Information as provided in this Section 11.07 shall be considered to have complied with its obligation to do so if such Person has exercised the same degree of care to maintain the confidentiality of such Information as such Person would accord to its own confidential information. In addition, the Administrative Agent and the Lenders may disclose the existence of this Agreement and information about this Agreement to market data collectors, similar service providers to the lending industry and service providers to the Administrative Agent and the Lenders in connection with the administration of this Agreement, the other Loan Documents and the Commitments; *provided* that such Person is advised of their obligation to keep information of this type confidential. This Section 11.07 shall survive the termination of this Agreement and the repayment, satisfaction or discharge of all Obligations but shall terminate on the later of (x) with respect to Information specifically identified by the Borrower as a trade secret, until such Information no longer constitutes a trade secret under applicable law, and (y) with respect to Information specifically identified by the Borrower related to third-party agreements subject to specific restrictions concerning disclosure of such information to sources of capital and their advisers, until the applicable term of such restrictions specified in the applicable agreement have lapsed.

(b) Non-Public Information. Each of the Administrative Agent and the Lenders acknowledges that (i) the Information may include material non-public information concerning a Loan Party or a Subsidiary, as the case may be, (ii) it has developed compliance procedures regarding the use of material non-public information and (iii) it will handle such material non-public information in accordance with Applicable Law, including United States federal and state securities Laws. Notwithstanding anything to the contrary contained in this Agreement, before the Borrower or any of its Subsidiaries discloses to the Administrative Agent or any Lender any information that could reasonably be determined to be material non-public information, such Person shall, prior to any such disclosure, first confirm with the applicable recipient whether it consents to the receipt of material non-public information; provided that no Default or Event of Default hereunder shall arise as a result of a recipient's refusal to consent to receive information required to be delivered hereunder, and Borrower and its Subsidiaries shall not be obligated to indemnify or otherwise reimburse or be liable to any Person hereunder for providing information that it reasonably determines in its good faith judgement does not contain material non-public information at the time provided.

(c) Press Releases. The Loan Parties and their Affiliates agree that they will not in the future issue any press releases or other public disclosure (other than filings required by applicable securities laws) using the name of the Administrative Agent (other than in any press release announcing the consummation of the Transactions, so long as such press release has been approved in writing by the Required Lenders), the Required Lenders or any Lender or their respective Affiliates or referring to this Agreement or any of the Loan Documents without the prior written consent of the Administrative Agent and the Required Lenders, unless (and only to the extent that) the Loan Parties or such Affiliate reasonably determine that it is necessary or desirable for them to do so under Applicable Law and then, in any event the Loan Parties or such Affiliate will consult with such Person before issuing such press release or other public disclosure.

11.08 Right of Setoff.

If an Event of Default shall have occurred and be continuing, each Lender and each of its respective Affiliates is hereby authorized at any time and from time to time, after obtaining the prior written consent of the Required Lenders, to the fullest extent permitted by Applicable Law to set off and apply any and all deposits (general or special, time or demand, provisional or final, in whatever currency) at any time held and other obligations (in whatever currency) at any time owing by such Lender or any such Affiliate to or for the credit or the account of the Borrower against any and all of the obligations of the Borrower now or hereafter existing under this Agreement or any other Loan Document to such Lender or such Affiliates, irrespective of whether or not such Lender or Affiliate shall have made any demand under this Agreement or any other Loan Document and although such obligations of the Borrower may be contingent or unmatured, secured or unsecured, or are owed to a branch, office or Affiliate of such Lender different from the branch, office or Affiliate holding such deposit or obligated on such indebtedness; *provided* that in the event that any Defaulting Lender shall exercise any such right of setoff, (a) all amounts so set off shall be paid over immediately to the Administrative Agent for further application in accordance with the provisions of Section 2.12 and, pending such payment, shall be segregated by such Defaulting Lender from its other funds and deemed held in trust for the benefit of the Administrative Agent and the Lenders, and (b) the Defaulting Lender shall provide promptly to the Administrative Agent a statement describing in reasonable detail the Obligations owing to such Defaulting Lender as to which it exercised such right of setoff. The rights of each Lender and its respective Affiliates under this Section 11.08 are in addition to other rights and remedies (including other rights of setoff) that such Lender or its respective Affiliates may have under Applicable Law. Each Lender agrees to notify the Borrower and the Administrative Agent promptly after any such setoff and application, *provided* that the failure to give such notice shall not affect the validity of such setoff and application.

11.09 Interest Rate Limitation.

Notwithstanding anything to the contrary contained in any Loan Document, the interest paid or agreed to be paid under the Loan Documents shall not exceed the maximum rate of non-usurious interest permitted by Applicable Law (the "*Maximum Rate*"). If the Administrative Agent or any Lender shall receive interest in an amount that exceeds the Maximum Rate, the excess interest shall be applied to the principal of the Loans or, if it exceeds such unpaid principal, refunded to the Borrower. In determining whether the interest contracted for, charged, or received by the Administrative Agent or a Lender exceeds the Maximum Rate, such Person may, to the extent permitted by Applicable Law, (a) characterize any payment that is not principal as an expense, fee, or premium rather than interest, (b) exclude voluntary prepayments and the effects thereof, and (c) amortize, prorate, allocate, and spread in equal or unequal parts the total amount of interest throughout the contemplated term of the Obligations hereunder.

11.10 Counterparts; Integration; Effectiveness.

This Agreement and each of the other Loan Documents may be executed in counterparts (and by different parties hereto in different counterparts), each of which shall constitute an original, but all of which when taken together shall constitute a single contract. This Agreement, the other Loan Documents, and any separate letter agreements with respect to fees payable to the Administrative Agent constitute the entire contract among the parties relating to the subject matter hereof and supersede any and all previous agreements and understandings, oral or written, relating to the subject matter hereof. Except as provided in Section 4.01, this Agreement shall become effective when it shall have been executed by the Administrative Agent and when the Administrative Agent shall have received counterparts hereof that, when taken together, bear the signatures of each of the other parties hereto. Delivery of an executed counterpart of a signature page of this Agreement or any other Loan Document, or any certificate delivered thereunder, by fax transmission or e-mail transmission (*e.g.*, "pdf" or "tif") shall be effective as delivery of a manually executed counterpart of this Agreement or such other Loan Document or certificate. Without limiting the foregoing, to the extent a manually executed counterpart is not specifically required to be delivered under the terms of any Loan Document, upon the request of any party, such fax transmission or e-mail transmission shall be promptly followed by such manually executed counterpart.

11.11 Survival of Representations and Warranties.

All representations and warranties made hereunder and in any other Loan Document or other document delivered pursuant hereto or thereto or in connection herewith or therewith shall survive the execution and delivery hereof and thereof. Such representations and warranties have been or will be relied upon by the Administrative Agent and each Lender, regardless of any investigation made by the Administrative Agent or any Lender or on their behalf and notwithstanding that the Administrative Agent or any Lender may have had notice or knowledge of any Default at the time of any Borrowing, and shall continue in full force and effect as long as any Loan or any other Obligation hereunder shall remain unpaid or unsatisfied.

11.12 Severability.

If any provision of this Agreement or the other Loan Documents is held to be illegal, invalid or unenforceable, (a) the legality, validity and enforceability of the remaining provisions of this Agreement and the other Loan Documents shall not be affected or impaired thereby and (b) the parties shall endeavor in good faith negotiations to replace the illegal, invalid or unenforceable provisions with valid provisions the economic effect of which comes as close as possible to that of the illegal, invalid or unenforceable provisions. The invalidity of a provision in a particular jurisdiction shall not invalidate or render unenforceable such provision in any other jurisdiction. Without limiting the foregoing provisions of this Section 11.12, if and to the extent that the enforceability of any provisions in this Agreement relating to Defaulting Lenders shall be limited by Debtor Relief Laws, as determined in good faith by the Administrative Agent, then such provisions shall be deemed to be in effect only to the extent not so limited.

11.13 Replacement of Lenders.

(a) If the Borrower is entitled to replace a Lender pursuant to the provisions of Section 3.06, or if any Lender is a Defaulting Lender or a Non-Consenting Lender, then the Borrower may, at its sole expense and effort, upon written notice to such Lender and the Administrative Agent, require such Lender to assign and delegate, without recourse (in accordance with and subject to the restrictions contained in, and consents required by, Section 11.06), all of its interests, rights (other than its existing rights to payments pursuant to Sections 3.01 and 3.04) and obligations under this Agreement and the related Loan Documents to an Eligible Assignee that

shall assume such obligations (which assignee may be another Lender, if a Lender accepts such assignment), *provided* that:

(i) the Borrower shall have paid to the Administrative Agent the processing and recordation fee (if any) specified in Section 11.06(b);

(ii) such Lender shall have received payment of an amount equal to 100% of the outstanding principal of its Loans, accrued interest thereon, accrued fees and all other amounts payable to it hereunder and under the other Loan Documents (including any amounts under Section 3.05) from the assignee (to the extent of such outstanding principal and accrued interest and fees) or the Borrower (in the case of all other amounts);

(iii) in the case of any such assignment resulting from a claim for compensation under Section 3.04 or payments required to be made pursuant to Section 3.01, such assignment will result in a reduction in such compensation or payments thereafter;

(iv) such assignment does not conflict with Applicable Laws; and

(v) in the case of an assignment resulting from a Lender becoming a Non-Consenting Lender, the applicable assignee shall have consented to the applicable amendment, waiver or consent.

(b) A Lender shall not be required to make any such assignment or delegation if, prior thereto, as a result of a waiver by such Lender or otherwise, the circumstances entitling the Borrower to require such assignment and delegation cease to apply.

(c) Each party hereto agrees that (i) an assignment required pursuant to this Section 11.13 may be effected pursuant to an Assignment and Assumption executed by the Borrower, the Administrative Agent and the assignee and (ii) the Lender required to make such assignment need not be a party thereto in order for such assignment to be effective and shall be deemed to have consented to an be bound by the terms thereof; *provided*, that, following the effectiveness of any such assignment, the other parties to such assignment agree to execute and deliver such documents necessary to evidence such assignment as reasonably requested by the applicable Lender, *provided further* that any such documents shall be without recourse to or warranty by the parties thereto.

11.14 Governing Law; Jurisdiction; Etc.

(a) GOVERNING LAW. THIS AGREEMENT AND THE OTHER LOAN DOCUMENTS (EXCEPT, AS TO ANY OTHER LOAN DOCUMENT, AS EXPRESSLY SET FORTH THEREIN) AND ANY CLAIMS, CONTROVERSY, DISPUTE OR CAUSE OF ACTION (WHETHER IN CONTRACT OR TORT OR OTHERWISE) BASED UPON, ARISING OUT OF OR RELATING TO THIS AGREEMENT OR ANY OTHER LOAN DOCUMENT (EXCEPT, AS TO ANY OTHER LOAN DOCUMENT, AS EXPRESSLY SET FORTH THEREIN) AND THE TRANSACTIONS CONTEMPLATED HEREBY AND THEREBY SHALL BE GOVERNED BY, AND CONSTRUED IN ACCORDANCE WITH, THE LAW OF THE STATE OF NEW YORK, WITHOUT REGARD TO PRINCIPLES OF CONFLICTS OF LAWS THAT WOULD RESULT IN THE APPLICATION OF THE LAWS OF ANY OTHER JURISDICTION; PROVIDED THAT SECTION 5-1401 OF THE NEW YORK GENERAL OBLIGATIONS LAW SHALL APPLY.

(b) SUBMISSION TO JURISDICTION. THE BORROWER AND EACH OTHER LOAN PARTY IRREVOCABLY AND UNCONDITIONALLY AGREES THAT IT WILL NOT COMMENCE ANY ACTION, LITIGATION OR PROCEEDING OF ANY KIND OR DESCRIPTION, WHETHER IN LAW OR EQUITY, WHETHER IN CONTRACT OR IN TORT OR OTHERWISE, AGAINST THE ADMINISTRATIVE AGENT, THE REQUIRED LENDERS, ANY LENDER, OR ANY RELATED PARTY OF THE FOREGOING IN ANY WAY RELATING TO THIS AGREEMENT OR ANY OTHER LOAN DOCUMENT OR THE TRANSACTIONS RELATING HERETO OR THERETO, IN ANY FORUM OTHER THAN THE COURTS OF THE STATE OF NEW YORK SITTING IN NEW YORK COUNTY AND OF THE UNITED STATES DISTRICT COURT OF THE SOUTHERN DISTRICT OF NEW YORK, AND ANY APPELLATE COURT FROM ANY THEREOF, AND EACH OF THE PARTIES HERETO IRREVOCABLY AND UNCONDITIONALLY SUBMITS TO THE JURISDICTION OF SUCH COURTS AND AGREES THAT ALL CLAIMS IN RESPECT OF ANY SUCH ACTION, LITIGATION OR PROCEEDING MAY BE HEARD AND DETERMINED IN SUCH NEW YORK STATE COURT OR, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, IN SUCH FEDERAL COURT. EACH OF THE PARTIES HERETO AGREES THAT A FINAL JUDGMENT IN ANY SUCH ACTION, LITIGATION OR PROCEEDING SHALL BE CONCLUSIVE AND MAY BE ENFORCED IN OTHER JURISDICTIONS BY SUIT ON THE JUDGMENT OR IN ANY OTHER MANNER PROVIDED BY LAW. NOTHING IN THIS AGREEMENT OR IN ANY OTHER LOAN DOCUMENT SHALL AFFECT ANY RIGHT THAT THE ADMINISTRATIVE AGENT, THE REQUIRED LENDERS OR ANY LENDER MAY OTHERWISE HAVE TO BRING ANY ACTION OR PROCEEDING RELATING TO THIS AGREEMENT OR ANY OTHER LOAN DOCUMENT AGAINST THE BORROWER OR ANY OTHER LOAN PARTY OR ITS PROPERTIES IN THE COURTS OF ANY JURISDICTION.

(c) WAIVER OF VENUE. THE BORROWER AND EACH OTHER LOAN PARTY IRREVOCABLY AND UNCONDITIONALLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, ANY OBJECTION THAT IT MAY NOW OR HEREAFTER HAVE TO THE LAYING OF VENUE OF ANY ACTION OR PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT OR ANY OTHER LOAN DOCUMENT IN ANY COURT REFERRED TO IN CLAUSE (b) OF THIS SECTION 11.14. THE BORROWER AND EACH OTHER LOAN PARTY IRREVOCABLY AND UNCONDITIONALLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, THE DEFENSE OF AN INCONVENIENT FORUM TO THE MAINTENANCE OF SUCH ACTION OR PROCEEDING IN ANY SUCH COURT.

(d) SERVICE OF PROCESS. EACH PARTY HERETO IRREVOCABLY CONSENTS TO SERVICE OF PROCESS IN THE MANNER PROVIDED FOR NOTICES IN SECTION 11.02. NOTHING IN THIS AGREEMENT WILL AFFECT THE RIGHT OF ANY PARTY HERETO TO SERVE PROCESS IN ANY OTHER MANNER PERMITTED BY APPLICABLE LAW.

11.15 Waiver of Jury Trial.

EACH PARTY HERETO HEREBY IRREVOCABLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, ANY RIGHT IT MAY HAVE TO A TRIAL BY JURY IN ANY LEGAL PROCEEDING DIRECTLY OR INDIRECTLY ARISING OUT OF OR RELATING TO THIS AGREEMENT OR ANY OTHER LOAN DOCUMENT OR THE TRANSACTIONS CONTEMPLATED HEREBY OR THEREBY (WHETHER BASED ON CONTRACT, TORT OR ANY OTHER THEORY). EACH PARTY HERETO (a) CERTIFIES THAT NO REPRESENTATIVE, AGENT OR ATTORNEY

OF ANY OTHER PERSON HAS REPRESENTED, EXPRESSLY OR OTHERWISE, THAT SUCH OTHER PERSON WOULD NOT, IN THE EVENT OF LITIGATION, SEEK TO ENFORCE THE FOREGOING WAIVER AND (b) ACKNOWLEDGES THAT IT AND THE OTHER PARTIES HERETO HAVE BEEN INDUCED TO ENTER INTO THIS AGREEMENT AND THE OTHER LOAN DOCUMENTS BY, AMONG OTHER THINGS, THE MUTUAL WAIVERS AND CERTIFICATIONS IN THIS SECTION 11.15.

11.16 Subordination.

Each Loan Party (a "*Subordinating Loan Party*") hereby subordinates the payment of all obligations and indebtedness of any other Loan Party owing to it, whether now existing or hereafter arising, including but not limited to any obligation of any such other Loan Party to the Subordinating Loan Party as subrogee of the Secured Parties or resulting from such Subordinating Loan Party's performance under this Guaranty, to the indefeasible payment in full in cash of all Obligations. If the Secured Parties so request, any such obligation or indebtedness of any such other Loan Party to the Subordinating Loan Party shall be enforced and performance received by the Subordinating Loan Party as trustee for the Secured Parties and the proceeds thereof shall be paid over to the Secured Parties on account of the Obligations, but without reducing or affecting in any manner the liability of the Subordinating Loan Party under this Agreement. Without limitation of the foregoing, so long as no Event of Default has occurred and is continuing and, except in the case of the acceleration of the Obligations or upon the occurrence and during the continuation of an Event of Default pursuant to Section 8.01(f), the Borrower has not received written notice from the Required Lenders, the Loan Parties may make and receive payments with respect to Intercompany Debt; *provided*, that in the event that any Loan Party receives any payment of any Intercompany Debt at a time when such payment is prohibited by this Section 11.16, such payment shall be held by such Loan Party, in trust for the benefit of, and shall be paid forthwith over and delivered, upon written request, to the Administrative Agent.

11.17 No Advisory or Fiduciary Responsibility.

(a) In connection with all aspects of each transaction contemplated hereby (including in connection with any amendment, waiver or other modification hereof or of any other Loan Document), the Borrower and each other Loan Party acknowledges and agrees, and acknowledges its Affiliates' understanding, that: (a) (i) the arranging and other services regarding this Agreement provided by the Administrative Agent and the Lenders and their respective Affiliates are arm's-length commercial transactions between the Borrower each other Loan Party and their respective Affiliates, on the one hand, and the Administrative Agent and the Lenders and their respective Affiliates, on the other hand, (ii) each of the Borrower and the other Loan Parties has consulted its own legal, accounting, regulatory and tax advisors to the extent it has deemed appropriate, and (iii) the Borrower and each other Loan Party is capable of evaluating, and understands and accepts, the terms, risks and conditions of the transactions contemplated hereby and by the other Loan Documents; (b) (i) the Administrative Agent and each Lender and each of their respective Affiliates each is and has been acting solely as a principal and, except as expressly agreed in writing by the relevant parties, has not been, is not, and will not be acting as an advisor, agent or fiduciary, for the Borrower any other Loan Party or any of their respective Affiliates, or any other Person and (ii) neither the Administrative Agent, the Required Lenders, nor any Lender nor any of their respective Affiliates has any obligation to the Borrower any other Loan Party or any of their respective Affiliates with respect to the transactions contemplated hereby except those obligations expressly set forth herein and in the other Loan Documents; and (c) the Administrative Agent and the Lenders and their respective Affiliates may be engaged in a broad range of transactions that involve interests that differ from those of the Borrower the other Loan Parties and their respective Affiliates, and neither the Administrative Agent, the Required Lenders, nor any Lender nor any of

their respective Affiliates has any obligation to disclose any of such interests to the Borrower any other Loan Party or any of their respective Affiliates. To the fullest extent permitted by Applicable Law, each of the Borrower and each other Loan Party hereby waives and releases any claims that it may have against the Administrative Agent, the Required Lenders, the Lenders and their respective Affiliates with respect to any breach or alleged breach of agency or fiduciary duty in connection with any aspect of any transactions contemplated hereby.

(b) The Borrower further acknowledges that the Required Lenders, the Lenders and their respective Affiliates may acquire, hold or sell, for their own accounts and the accounts of investors, the equity, debt and other securities and financial instruments (including, common equity, bank loans and other obligations) of, the Borrower and other companies with which the Borrower may have commercial or other relationships. With respect to any securities and/or financial instruments so held by the Required Lenders, the Lenders or their respective Affiliates or any of their respective investors, all rights in respect of such securities and financial instruments, including any voting rights, will be exercised by the holder of the rights, in its sole discretion.

11.18 Electronic Execution; Electronic Records.

(a) The words "delivery," "execute," "execution," "signed," "signature," and words of like import in any Loan Document or any other document executed in connection herewith shall be deemed to include electronic signatures, the electronic matching of assignment terms and contract formations on electronic platforms approved by the Administrative Agent, or the keeping of records in electronic form, each of which shall be of the same legal effect, validity or enforceability as a manually executed signature, physical delivery thereof or the use of a paper-based recordkeeping system, as the case may be, to the extent and as provided for in any Applicable Law, including the Federal Electronic Signatures in Global and National Commerce Act, the New York State Electronic Signatures and Records Act, or any other similar state laws based on the Uniform Electronic Transactions Act; *provided* that notwithstanding anything contained herein to the contrary, the Administrative Agent is under no obligation to agree to accept electronic signatures in any form or in any format unless expressly agreed to by the Administrative Agent pursuant to procedures approved by it; *provided*, *further*, without limiting the foregoing, upon the reasonable request of the Administrative Agent, any electronic signature shall be promptly followed by such manually executed counterpart. For the avoidance of doubt, the authorization under this paragraph may include, without limitation, use or acceptance by the Administrative Agent and each of the Lenders of a manually signed paper document, amendment, approval, consent, information, notice, certificate, request, statement, disclosure or authorization related to this Agreement (each a "Communication") which has been converted into electronic form (such as scanned into PDF format), or an electronically signed Communication converted into another format, for transmission, delivery and/or retention.

(b) The Borrower hereby acknowledges the receipt of a copy of this Agreement and all other Loan Documents. The Administrative Agent and each Lender may, on behalf of the Borrower, create a microfilm or optical disk or other electronic image of this Agreement and any or all of the other Loan Documents. The Administrative Agent and each Lender may store the electronic image of this Agreement and the other Loan Documents in its electronic form and then destroy the paper original as part of the Administrative Agent's, the Required Lenders's and each Lender's normal business practices, with the electronic image deemed to be an original and of the same legal effect, validity and enforceability as the paper originals.

11.19 USA Patriot Act Notice.

Each Lender that is subject to the Patriot Act and the Administrative Agent (for itself and not on behalf of any Lender) hereby notifies the Borrower and the other Loan Parties that pursuant to the requirements of the USA PATRIOT Act (Title III of Pub. L. 107–56 (signed into law October 26, 2001)) (the "*Patriot Act*"), it is required to obtain, verify and record information that identifies the Borrower and each other Loan Party, which information includes the name and address of the Borrower and each other Loan Party and other information that will allow such Lender or the Administrative Agent, as applicable, to identify the Borrower and each other Loan Party in accordance with the Patriot Act. The Borrower and each other Loan Party shall, promptly following a request by the Administrative Agent or any Lender, provide all such other documentation and information that the Administrative Agent or such Lender requests in order to comply with its ongoing obligations under applicable "know your customer" and anti-money laundering rules and regulations, including the Patriot Act.

11.20 Acknowledgement and Consent to Bail-In of EEA and UK Financial Institutions.

Notwithstanding anything to the contrary in any Loan Document or in any other agreement, arrangement or understanding among any such parties, each party hereto acknowledges that any liability of any Lender that is an Affected Financial Institution arising under any Loan Document, to the extent such liability is unsecured, may be subject to the write-down and conversion powers of the applicable Resolution Authority and agrees and consents to, and acknowledges and agrees to be bound by (a) the application of any Write-Down and Conversion Powers by the applicable Resolution Authority to any such liabilities arising hereunder which may be payable to it by any party hereto that is an Affected Financial Institution; and (b) the effects of any Bail-In Action on any such liability, including, if applicable: (i) a reduction in full or in part or cancellation of any such liability; (ii) a conversion of all, or a portion of, such liability into shares or other instruments of ownership in such Affected Financial Institution, its parent undertaking, or a bridge institution that may be issued to it or otherwise conferred on it, and that such shares or other instruments of ownership will be accepted by it in lieu of any rights with respect to any such liability under this Agreement or any other Loan Document; or (iii) the variation of the terms of such liability in connection with the exercise of the write-down and conversion powers of the applicable Resolution Authority.

[*Signature Pages Follow*]

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be duly executed as of the date first above written.

BORROWER: **PTC THERAPEUTICS, INC.**

By: /s/ Stuart W. Peltz
Name: Stuart W. Peltz, Ph.D.
Title: Chief Executive Officer and President

GUARANTORS:

PTC THERAPEUTICS HOLDINGS (BERMUDA) CORP. LIMITED

By: /s/ Sherman Taylor
Name: Sherman Taylor
Title: Director

PTC THERAPEUTICS INTERNATIONAL LIMITED

By: /s/ Adrian Haigh
Name: Adrian Haigh
Title: Director

PTC FARMACÊUTICA DO BRASIL LTDA.

By: /s/ Rogerio Ribeiro da Silva
Name: Rogerio Ribeiro da Silva
Title: Director

PTC THERAPEUTICS HOLDINGS, INC.

By: /s/ Sherman Ta/s/ Stuart W. Peltz
Name: Stuart W. Peltz, Ph.D.
Title: President

PTC THERAPEUTICS MP, INC.

By: /s/ Stuart W. Peltz
Name: Stuart W. Peltz, Ph.D.
Title: President

PTC THERAPEUTICS GT, INC.

By: /s/ Stuart W. Peltz
Name: Stuart W. Peltz, Ph.D.
Title: President

WILMINGTON TRUST, NATIONAL ASSOCIATION,
as Administrative Agent

By: /s/ Matthew Garvis
Name: Matthew Garvis
Title: Vice President

[Signature Page to Credit Agreement]

LENDERS:

BLACKSTONE COF IV HOLDCO LP,
as a Lender

By: GSO Capital Opportunities Associates IV LP, its general partner
By: GSO Capital Opportunities Associates IV (Delaware) LLC, its general partner

By: /s/ Marisa Beeney
Name: Marisa Beeney
Title: Authorized Signatory

BXLS YIELD – TRANSLATE L.P.,
as a Lender

By: Blackstone Life Sciences Advisors L.L.C. on behalf of BXLS Yield – Translate L.P.

By: /s/ Robert Liptak
Name: Robert Liptak
Title: Chief Operating Officer

Exhibit 10.56

Execution Version

STOCK PURCHASE AGREEMENT

BY AND AMONG

THE INVESTORS LISTED HEREIN

AND

PTC THERAPEUTICS, INC.

DATED AS OF OCTOBER 27, 2022

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STOCK PURCHASE AGREEMENT

THIS STOCK PURCHASE AGREEMENT (this "**Agreement**"), dated as of October 27, 2022 (the "**Effective Date**"), by and among the investors listed in Exhibit A attached hereto (the "**Investors**") and PTC Therapeutics, Inc. (the "**Company**").

WHEREAS, pursuant to the terms and subject to the conditions set forth in this Agreement, the Company desires to issue and sell to the Investors, and the Investors desire to subscribe for and purchase from the Company, certain shares of common stock, par value $0.001 per share, of the Company (the "**Common Stock**").

NOW, THEREFORE, in consideration of the following mutual promises and obligations, and for good and valuable consideration, the adequacy and sufficiency of which are hereby acknowledged, the Investors and the Company agree as follows:

1. Definitions.

1.1 Defined Terms. When used in this Agreement, the following terms shall have the respective meanings specified therefor below:

"**Affiliate**" shall mean, with respect to any designated Person, any other Person that, directly or indirectly, controls, is controlled by or is under common control with such designated Person. For purposes of this definition, "control" of a Person means the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by contract or otherwise, and the terms "controlled" and "controlling" have meanings correlative to the foregoing. For the purposes of this Agreement, in no event shall any Investor or any of its Affiliates be deemed Affiliates of the Company or any of its Affiliates, nor shall the Company or any of its Affiliates be deemed Affiliates of any Investor or any of its Affiliates.

"**Agreement**" shall have the meaning set forth in the Preamble, including all Exhibits attached hereto.

"**Approved Fund**" means any Person (other than a natural Person) that is (a) a Blackstone Investor or Blackstone Entity or (b) a Fund that is administered, managed or advised by (i) an Investor, (ii) an Affiliate of an Investor or (iii) an entity or an Affiliate of an entity that administers, manages or advises an Investor.

"**Blackstone Affiliated Entities**" shall mean, collectively, the Blackstone Entities, and their Affiliates and Approved Funds.

"**Blackstone Investor**" means any investor (or an Affiliate of such investor) of a fund managed or advised by Blackstone Credit or Blackstone Life Sciences to which such investor (or an Affiliate of such investor), Blackstone Credit or Blackstone Life Sciences, as applicable, is providing certain administrative and other services.

"**Blackstone Entities**" shall mean Blackstone Life Sciences, Blackstone Credit, Blackstone Holdings Finance Co. L.L.C and any of their respective Affiliates and funds and accounts administered, managed, agented or advised by any of them, and any warehouse entity.

"**Business Day**" shall mean any day that is not a Saturday, Sunday or other day on which commercial banks in New York City are authorized or required by applicable Law to remain closed. For the avoidance of doubt, with respect to any notice or other communication required to be given hereunder, limitations on the operations of commercial banks due to the outbreak of a contagious disease, epidemic or pandemic (including COVID-19), or any quarantine, shelter-in-place or similar or related directive, shall not prevent a day that would otherwise be a Business Day hereunder from so being a Business Day.

"**Change of Control**" means and shall be deemed to have occurred if any "person" or "group" (within the meaning of Rule 13d-5 of the Exchange Act) acquires, directly or indirectly, beneficially or of record, (x) determined on a fully diluted basis, more than 50% of the Voting Stock of the Company or (y) all or substantially all of the Company's consolidated assets. Notwithstanding anything to the contrary in this definition or any provision of Section 13d-3 of the Securities Exchange Act of 1934, any person or group shall not be deemed to beneficially own Equity Interests to be acquired by such person or group pursuant to a stock or asset purchase agreement, merger agreement, option agreement, warrant agreement or similar agreement (or voting or option or similar agreement related thereto) until the consummation of the acquisition of the Equity Interests or assets in connection with the transactions contemplated by such agreement.

"**Credit Agreement**" shall mean the credit agreement dated October 27, 2022 between the Company, the Investors and such other parties thereto.

"**Disposition**" or "**Dispose of**" shall mean any (i) offer, pledge, sale, contract to sell, sale of any option or contract to purchase, purchase of any option or contract to sell, grant of any option, right or warrant for the sale of, or other disposition of or transfer of any Lock-Up Securities, including, without limitation, any "short sale" or similar arrangement, or (ii) swap or any other agreement or any transaction that transfers, in whole or in part, directly or indirectly, the economic consequence of ownership of Lock-Up Securities, whether any such swap or transaction is to be settled by delivery of securities, in cash or otherwise.

"**Effect**" shall have the meaning set forth in the definition of "Material Adverse Effect."

"**Equity Interests**" means, with respect to any Person, shares of capital stock of (or other ownership or profit interests in) such Person, warrants, options or other rights for the purchase or acquisition from such Person of shares of capital stock of (or other ownership or profit interests in) such Person, securities convertible into or exchangeable for shares of capital stock of (or other ownership or profit interests in) such Person or warrants, rights or options for the purchase or acquisition from such Person of such shares (or such other interests), and other ownership or profit interests in such Person (including partnership, member or trust interests therein), whether voting or nonvoting, and whether or not such shares, warrants, options, rights or other interests are outstanding on any date of determination; *provided* that convertible debt that constitutes

Permitted Subordinated Debt (as defined in the Credit Agreement) shall not be considered an "Equity Interest".

"**Facility Termination Date**" shall have the meaning set forth in the Credit Agreement.

"**Governmental Authority**" shall mean any court, agency, authority, department, regulatory authority or other instrumentality of any government or country or of any national, federal, state, provincial, regional, county, city or other political subdivision of any such government or country or any supranational organization of which any such country is a member.

"**Healthcare Laws**" shall mean the Federal Food, Drug and Cosmetic Act of 1938, as amended, the Public Health Service Act of 1944, as amended, Medicare and Medicaid, the federal Anti-Kickback Statute, the Civil Monetary Penalty Law, the civil False Claims Act, the administrative False Claims Law, the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), the Health Information Technology for Economic and Clinical Health Act ("HITECH") and the exclusion laws, the Prescription Drug Marketing Act of 1987, the Sunshine/Open Payments Law, all regulations or guidance promulgated pursuant to such Laws, and any other federal, state or foreign Law that regulates the design, development, testing, studying, manufacturing, processing, storing, importing or exporting, licensing, labeling or packaging, advertising, distributing, selling, pricing, or marketing of pharmaceutical products, or that is related to remuneration (including ownership) to or by physicians or other health care providers (including kickbacks).

"**Intellectual Property**" shall mean trademarks, trade names, trade dress, service marks, copyrights, and similar rights (including registrations and applications to register or renew the registration of any of the foregoing), patents and patent applications, trade secrets, and any other similar intellectual property rights.

"**Intellectual Property License**" shall mean any license, permit, authorization, approval, contract or consent granted, issued by or with any Person relating to the use of Intellectual Property.

"**Law**" or "**Laws**" shall mean, with respect to any Person, all laws, statutes, rules, regulations, orders, judgments, injunctions and/or ordinances of any Governmental Authority applicable to such Person or any of its properties or assets.

"**Material Adverse Effect**" shall mean any change, event or occurrence (each, an "**Effect**") that, individually or when taken together with all other Effects, has had, or is reasonably expected to have, (i) a material adverse effect on the business, financial condition, assets or results of operations of the Company and its Subsidiaries, taken as a whole, or (ii) a material adverse effect on the Company's ability to perform its obligations, or consummate the Transaction, in accordance with the terms of this Agreement, except in the case of (i) or (ii) to the extent that any such Effect results from or arises out of: (A) changes in conditions in the United States or global economy or capital or financial markets generally, including any disruption thereof and any decline in the price of any security or any market index, (B) changes in general legal, regulatory, political, economic or business conditions or changes in generally

accepted accounting principles in the United States or interpretations thereof that, in each case, generally affect the biotechnology or biopharmaceutical industries, (C) any change in the trading prices or trading volume of the Common Stock (it being understood that the facts giving rise to or contributing to any such change may be deemed to constitute, or be taken into account when determining whether there has been or will be, a Material Adverse Effect, except to the extent any of such facts is an Effect referred to in clauses (A), (B) or (D) through (G) of this definition), (D) acts of war, sabotage or terrorism, or any escalation or worsening of any such acts of war, sabotage or terrorism, (E) earthquakes, hurricanes, floods or other natural disasters, (F) the outbreak of contagious disease, epidemic or pandemic (including COVID-19), any quarantine, shelter-in-place or similar or related directive, policy or guidance or other action by any Governmental Authority, or (G) any action taken by the Company required by this Agreement; provided that, with respect to clauses (A), (B), (D), (E) and (F), such Effect does not have a material disproportionate and adverse impact on the Company relative to other companies in the biotechnology or biopharmaceutical industries.

"**Organizational Documents**" shall mean (i) the Restated Certificate of Incorporation of the Company dated as of June 25, 2013, as amended as of June 9, 2021 and (ii) the Amended and Restated Bylaws of the Company effective as of April 21, 2017.

"**Permitted Transferee**" shall mean the Blackstone Affiliated Entities; provided, however, that no such Blackstone Affiliated Entity shall be deemed a Permitted Transferee for any purpose under this Agreement unless (i) the Permitted Transferee, prior to or simultaneously with such transfer or assignment, shall have agreed in writing with the Company to be subject to and bound by all restrictions and obligations applicable to such Investor set forth in this Agreement, and (ii) such Investor shall, within five (5) days prior to such transfer, furnish to the Company written notice of the name and address of such Permitted Transferee, details of its status as a Permitted Transferee and details of the Registrable Securities with respect to which such registration rights are being assigned. Following such transfer, the Permitted Transferee shall be deemed to be an Investor for all purposes under this Agreement.

"**Person**" shall mean any individual, partnership, limited liability company, firm, corporation, trust, unincorporated organization, government or any department or agency thereof or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

"**Registrable Securities**" shall mean (i) the Shares, together with any shares of Common Stock issued in respect thereof as a result of any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization and (ii) any Common Stock issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the shares of Common Stock described in clause (i) of this definition, excluding in all cases, however, (A) any Registrable Securities sold to or through a broker or dealer or underwriter in a public distribution or a public securities transaction or (B) Registrable Securities eligible for resale pursuant to Rule 144(b)(1)(i) under the Securities Act without the requirement for the Company to be in compliance with the current public information required under Rule 144(c)(1) under the Securities Act as to such Shares.

"**Registration Statement**" shall mean any registration statement of the Company under the Securities Act that covers any of the Registrable Securities pursuant to the provisions of this Agreement, including the related prospectus, all amendments and supplements to such registration statement (including post-effective amendments), and all exhibits and all materials incorporated by reference or explicitly deemed to be incorporated by reference in such registration statement.

"**Third Party**" shall mean any Person (other than a Governmental Authority) other than any Investor, the Company or any Affiliate of an Investor or the Company.

"**Transaction**" shall mean the issuance and sale of the Shares by the Company, and the purchase of the Shares by the Investors, in accordance with the terms hereof.

"**Underwritten Offering**" shall mean a registration in which shares of Common Stock are sold to an underwriter for reoffering to the public.

"**Voting Stock**" means, with respect to any Person, Equity Interests issued by such Person the holders of which are ordinarily, in the absence of contingencies, entitled to vote for the election of directors (or persons performing similar functions) of such Person, even though the right to so vote has been suspended by the happening of such contingency.

1.2 Additional Defined Terms. In addition to the terms defined in Section 1.1, the following terms shall have the respective meanings assigned thereto in the sections indicated below:

Defined Term	Section
Closing	Section 3.1
Common Stock	Recitals
Company	Preamble
Company SEC Documents	Section 4.11(a)
Exchange Act	Section 4.11(a)
Investor	Preamble
Lock-Up Securities	Section 9.1
Lock-Up Term	Section 9.1
Modified Clause	Section 10.7
Permits	Section 4.10
Registration Notice	Section 9.2(b)

Defined Term	Section
Required Period	Section 9.2(e)
Required Registration	Section 9.2(a)
SEC	Section 4.7
Securities Act	Section 4.11(a)
Shares	Section 2.1
Subsidiaries	Section 4.3
Violation	Section 9.2(j)(i)

2. Purchase and Sale of Common Stock.

2.1 Purchase and Sale. Subject to the terms and conditions of this Agreement, at the Closing, the Company shall issue and sell to each Investor, free and clear of all liens, other than any liens arising as a result of any action by any Investor, and each Investor shall purchase from the Company, the number of shares of Common Stock set forth opposite such Investor's name on Exhibit A for a purchase price of $45.65 per share. The shares of Common Stock issued to the Investors pursuant to this Agreement shall be referred to in this Agreement as the "**Shares**."

2.2 Tax Treatment. For U.S. federal income and other applicable tax purposes, the Investors and the Company agree to treat the Transaction as separate and independent from any transactions entered into by the Company and any of the Investors or their Affiliates, other than those contemplated by this Agreement, and to report the transactions contemplated by this Agreement on U.S. federal income tax and other applicable tax returns in accordance with this Section 2.2 unless otherwise required by applicable Law.

3. Closing; Deliveries.

3.1 Closing. Subject to the satisfaction or waiver of all the conditions to the Closing set forth in Sections 6, 7 and 8 hereof, the closing of the purchase and sale of the Shares hereunder (the "**Closing**") shall be held at 10:00 a.m. New York City time on the date hereof, remotely via the electronic exchange of executed documents and other closing deliverables, or at such other time, date and location as the parties may agree in writing.

3.2 Deliveries.

(a) Deliveries by the Company. At the Closing, the Company shall instruct its transfer agent to register the Shares in book-entry in the name of each Investor and in the amounts set forth on Exhibit A. The Company shall also deliver at the Closing: (i) a certificate in form and substance reasonably satisfactory to each Investor and duly executed on behalf of the Company by an authorized executive officer of the Company, certifying that the

conditions to Closing set forth in Sections 6 and 8.3(b) of this Agreement have been fulfilled; (ii) a certificate of the secretary of the Company dated as of the date hereof certifying (A) that attached thereto is a true and complete copy of the Amended and Restated Bylaws of the Company as in effect on the date hereof; (B) that attached thereto is a true and complete copy of all resolutions adopted by the Board of Directors of the Company authorizing the execution, delivery and performance of this Agreement and the Transaction and that all such resolutions are in full force and effect, have not been amended and are all the resolutions adopted in connection with the transactions contemplated hereby as of the date hereof; and (C) that attached thereto is a true and complete copy of the Company's Restated Certificate of Incorporation as in effect on the date hereof; (iii) a legal opinion of Wilmer Cutler Pickering Hale and Dorr LLP, counsel to the Company, in form and substance reasonably acceptable to the Investors; and (iv) the Credit Agreement executed by the Company.

(b) Deliveries by the Investors. At the Closing, each Investor shall deliver to the Company the aggregate purchase price set forth opposite such Investor's name on Exhibit A by wire transfer of immediately available United States funds to an account designated by the Company. Each Investor shall also deliver, or cause to be delivered, at the Closing, (i) a certificate in form and substance reasonably satisfactory to the Company duly executed by an authorized signatory of such Investor certifying that the conditions to Closing set forth in Section 7 of this Agreement have been fulfilled and (ii) the Credit Agreement executed by such Investor.

4. Representations and Warranties of the Company. The Company hereby represents and warrants to each Investor that:

4.1 Organization, Good Standing and Qualification.

(a) Each of the Company and the Subsidiaries (as defined below) is a corporation duly organized, validly existing and in good standing under the laws of the jurisdiction of its organization, with the requisite power and authority to own and use its properties and assets and to carry on its business as currently conducted. Each of the Company and the Subsidiaries has all requisite corporate power and corporate authority to own, lease and operate its properties and assets, to carry on its business as now conducted, and as proposed to be conducted as described in the Company SEC Documents, and the Company has all requisite corporate power to enter into this Agreement to issue and sell the Shares and to perform its obligations under and to carry out the other transactions contemplated by this Agreement.

(b) Each of the Company and its Subsidiaries is qualified to transact business and is in good standing in each jurisdiction in which the character of the properties owned, leased or operated by the Company or Subsidiary, as applicable, or the nature of the business conducted by the Company or Subsidiary, as applicable, makes such qualification necessary, except where the failure to be so qualified would not have a Material Adverse Effect.

4.2 Capitalization and Voting Rights.

(a) The authorized capital of the Company as of the date hereof consists of: (i) 250,000,000 shares of Common Stock of which, as of September 30, 2022, 71,854,892 shares were issued and outstanding and (ii) 5,000,000 shares of preferred stock, par

value $0.001 per share, none of which are issued and outstanding as of the date of this Agreement. All of the issued and outstanding shares of Common Stock (A) have been duly authorized and validly issued, (B) are fully paid and non-assessable, and (C) were issued in compliance with all applicable federal and state securities Laws.

(b) All of the authorized shares of Common Stock are entitled to one (1) vote per share.

(c) Except as described or referred to in Section 4.2(a) above, and as set forth in the Company SEC Documents (except for the grant of stock options under the Company's Amended and Restated 2013 Long-Term Incentive Plan or 2020 Inducement Stock Incentive Plan or equity securities issued upon the vesting or exercise of equity awards outstanding under such plans as of September 30, 2022), as of the date hereof, there are not: (i) any outstanding equity securities, options, warrants, rights (including conversion or preemptive rights) or other agreements pursuant to which the Company is or may become obligated to issue, sell or repurchase any shares of its capital stock or any other securities of the Company or (ii) any restrictions on the transfer of capital stock of the Company other than pursuant to state and federal securities Laws.

(d) Except as set forth in the Company SEC Documents, the Company is not a party to or subject to any agreement or understanding relating to the voting of shares of capital stock of the Company or the giving of written consents by a stockholder or director of the Company.

4.3 Subsidiaries. Set forth on Schedule 1 hereto is a list of each of the Company's "significant subsidiaries" (as defined in Rule 1-02(w) of Regulation S-X) as of the date hereof (the "**Subsidiaries**"). The Company owns, directly or indirectly, all of the capital stock or other equity interests of each Subsidiary free and clear of any liens, and all of the issued and outstanding shares of capital stock of each Subsidiary are validly issued and are fully paid, non-assessable and free of preemptive and similar rights to subscribe for or purchase securities.

4.4 Authorization.

(a) All requisite corporate action on the part of the Company, its directors and stockholders required by applicable Law for the authorization, execution and delivery by the Company of this Agreement and the performance of all obligations of the Company hereunder, including the authorization, issuance and delivery of the Shares, has been taken.

(b) This Agreement has been duly executed and delivered by the Company, and upon the due execution and delivery of this Agreement by the Investors, this Agreement will constitute a valid and legally binding obligation of the Company, enforceable against the Company in accordance with its terms (except as such enforceability may be limited by (i) applicable bankruptcy, insolvency, reorganization, moratorium or other Laws of general application relating to or affecting enforcement of creditors' rights and (ii) rules of Law governing specific performance, injunctive relief or other equitable remedies and limitations of public policy).

(c) No stop order or suspension of trading of the Common Stock has been implemented by The Nasdaq Stock Market LLC, the SEC or any other Governmental Authority and remains in effect.

4.5 No Defaults. The Company is not in default under or in violation of (a) the Organizational Documents, (b) any provision of applicable Law or any ruling, writ, injunction, order, Permit, judgment or decree of any Governmental Authority or (c) any agreement, arrangement or instrument, whether written or oral, by which the Company or any of its assets are bound, except, in the case of subsections (b) and (c), as would not have a Material Adverse Effect. There exists no condition, event or act which after notice, lapse of time, or both, would constitute a default or violation by the Company under any of the foregoing, except, in the case of subsections (b) and (c), as would not have a Material Adverse Effect.

4.6 No Conflicts. The execution, delivery and performance of this Agreement and compliance with the provisions hereof by the Company do not and shall not: (a) violate any provision of applicable Law or any ruling, writ, injunction, order, permit, judgment or decree of any Governmental Authority, (b) constitute a breach of, or default under (or an event which, with notice or lapse of time or both, would become a default under) or conflict with, or give rise to any right of termination, cancellation or acceleration of, any agreement, arrangement or instrument, whether written or oral, by which the Company or any of its assets are bound, (c) violate or conflict with any of the provisions of the Organizational Documents or (d) result in any encumbrance upon any of the Shares, other than restrictions pursuant to this Agreement or securities Laws, or any of the properties or assets of the Company or any Subsidiary, except, in the case of subsections (a) and (b), as would not have a Material Adverse Effect.

4.7 No Governmental Authority or Third-Party Consents. No consent, approval, authorization or other order of, or filing with, or notice to, any Governmental Authority or other Third Party is required to be obtained or made by the Company in connection with the authorization, execution and delivery by the Company of this Agreement or with the authorization, issuance and sale by the Company of the Shares, except such filings as may be required to be made with the Securities and Exchange Commission (the "**SEC**") and with any state blue sky or securities regulatory authority, which filings shall be made in a timely manner in accordance with all applicable Laws.

4.8 Valid Issuance of Shares. When issued, sold and delivered at the Closing in accordance with the terms hereof, the Shares shall be duly authorized, validly issued, fully paid and nonassessable, free from any liens, encumbrances or restrictions on transfer, including preemptive rights, rights of first refusal or other similar rights, other than as arising pursuant to this Agreement, as a result of any action by any Investor or under federal or state securities Laws.

4.9 Litigation. Except as set forth in the Company SEC Documents filed prior to the date of this Agreement, there is no action, suit, proceeding or investigation pending (of which the Company has received notice or otherwise has knowledge) or, to the Company's knowledge, threatened, against the Company or which the Company intends to initiate which has had or is reasonably likely to have a Material Adverse Effect.

4.10 Licenses and Other Rights; Compliance with Laws. The Company has all franchises, permits, licenses and other rights and privileges ("**Permits**") necessary to permit it to own its properties and to conduct its business as presently conducted and is in compliance thereunder, except where the failure to be in compliance does not and would not have a Material Adverse Effect. The Company has not taken any action that would interfere with the Company's ability to renew all such Permit(s), except where the failure to renew such Permit(s) would not have a Material Adverse Effect. The Company is and has been in compliance with all Laws applicable to its business, properties and assets, and to the products and services sold by it, except where the failure to be in compliance does not and would not have a Material Adverse Effect.

4.11 Company SEC Documents; Financial Statements; Nasdaq Stock Market.

(a) Since December 31, 2021, the Company has timely filed all reports, schedules, forms, statements and other documents (including exhibits and all other information incorporated therein) required to be filed by it under the Securities Act of 1933, as amended (the "**Securities Act**"), or the Securities Exchange Act of 1934, as amended (the "**Exchange Act**"), and any required amendments to any of the foregoing, with the SEC (the "**Company SEC Documents**"). As of their respective filing dates, each of the Company SEC Documents complied in all material respects with the requirements of the Securities Act, the Exchange Act and the rules and regulations of the SEC promulgated thereunder applicable to such Company SEC Documents, and no Company SEC Documents when filed, declared effective or mailed, as applicable, contained any untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading.

(b) The financial statements of the Company included in its Annual Report on Form 10-K for the fiscal year ended December 31, 2021 comply in all material respects with applicable accounting requirements and the published rules and regulations of the SEC with respect thereto, have been prepared in accordance with United States generally accepted accounting principles applied on a consistent basis during the periods involved (except as may be indicated in the notes thereto) and fairly present in all material respects the financial position of the Company as of the dates thereof and the results of its operations and cash flows for the periods then ended. Except (i) as set forth in the Company SEC Documents or (ii) for liabilities incurred in the ordinary course of business subsequent to the date of the most recent balance sheet contained in the Company SEC Documents, the Company has no liabilities, whether absolute or accrued, contingent or otherwise, other than those that would not, individually or in the aggregate, have a Material Adverse Effect.

(c) As of the date of this Agreement, the Common Stock is listed on The Nasdaq Global Select Market, and the Company has taken no action designed to, or which is likely to have the effect of, terminating the registration of the Common Stock under the Exchange Act or delisting the Common Stock from The Nasdaq Global Select Market. As of the date of this Agreement, the Company has not received any notification that, and has no knowledge that the SEC or The Nasdaq Stock Market LLC is contemplating terminating such listing or registration.

(d) Other than as has been disclosed to the Investors, there are no outstanding or unresolved comments in comment letters received from the SEC or its staff.

4.12 Absence of Certain Changes. Except as disclosed in the Company SEC Documents, since December 31, 2021, there has not occurred any event that has caused or would reasonably be expected to cause a Material Adverse Effect.

4.13 Internal Controls; Disclosure Controls and Procedures. The Company maintains internal control over financial reporting as defined in Rule 13a-15(f) under the Exchange Act. The Company has implemented the "disclosure controls and procedures" (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) required in order for the Principal Executive Officer and Principal Financial Officer of the Company to engage in the review and evaluation process mandated by the Exchange Act and is in compliance with such disclosure controls and procedures in all material respects. Each of the Principal Executive Officer and the Principal Financial Officer of the Company (or each former Principal Executive Officer of the Company and each former Principal Financial Officer of the Company, as applicable) has made all certifications required by Sections 302 and 906 of the Sarbanes-Oxley Act of 2002 with respect to all reports, schedules, forms, statements and other documents required to be filed by the Company with the SEC.

4.14 Intellectual Property. The Intellectual Property that is owned by the Company is owned free from any liens or restrictions, and all of the Company's material Intellectual Property Licenses are in full force and effect in accordance with their terms and are free of any liens or restrictions except (a) where the failure to be free from such liens or restrictions would not have a Material Adverse Effect or (b) as set forth in any such Intellectual Property License. Except as set forth in the Company SEC Documents, there is no legal claim or demand of any Person pertaining to, or any proceeding which is pending (of which the Company has received notice or otherwise has knowledge) or, to the knowledge of the Company, threatened, (i) challenging the right of the Company in respect of any Company Intellectual Property, or (ii) that claims that any default exists under any Intellectual Property License, except, in the case of (i) and (ii) above, where any such claim, demand or proceeding would not have a Material Adverse Effect.

4.15 Offering. Subject to the accuracy of the Investors' representations set forth in Sections 5.5, 5.6, 5.7, 5.8 and 5.9, the offer, sale and issuance of the Shares to be issued in conformity with the terms of this Agreement constitute transactions which are exempt from the registration requirements of the Securities Act and from all applicable state registration or qualification requirements. Neither the Company nor any Person acting on its behalf will take any action that would cause the loss of such exemption.

4.16 No Integration. The Company has not, directly or through any agent, sold, offered for sale, solicited offers to buy or otherwise negotiated in respect of, any security (as defined in the Securities Act) which is or will be integrated with the Shares sold pursuant to this Agreement in a manner that would require the registration of the Shares under the Securities Act.

4.17 Brokers' or Finders' Fees. No broker, finder, investment banker or other Person is entitled to any brokerage, finder's or other fee or commission from the Company in connection with the transactions contemplated by this Agreement.

4.18 Not Investment Company. The Company is not, and immediately after receipt of the aggregate purchase price for the Shares will not be, an "investment company" as defined in the Investment Company Act of 1940, as amended.

4.19 Regulatory Matters.

(a) The Company is and has been in material compliance with all applicable Healthcare Laws. There has been no material civil, criminal, administrative, or other proceeding, notice or demand pending, received by or filed, or to the knowledge of the Company, threatened in writing, against the Company alleging any violation by the Company of any applicable Healthcare Laws.

(b) The Company is and has been in compliance in all material respects with HIPAA, as amended by HITECH, as well as applicable requirements of corollary international Laws, including the EU General Data Protection Regulation (2016/679), EU Data Protection Directive 95/46/EC, and national implementations thereof. The Company has operated its business in compliance in all material respects with all Laws, clinical trial protocols, and contractual or other requirements relating to personal information, medical records and medical or personal information privacy that regulate or limit the maintenance, use, disclosure or transmission of medical records, clinical trial data, patient information or other personal information made available to or collected by the Company in connection with the operation of its business. The Company is and has been, in compliance with all other federal and state privacy, data security, data protection, data localization, and data breach notification Laws in all material respects. To the knowledge of the Company, after due inquiry neither the Company, nor any of its service providers (in the course of handling data for or on behalf of the Company), have suffered any material privacy or data security breach (including a cyber-attack) resulting in the unauthorized access, acquisition, use, or disclosure of any data or that triggered any reporting requirement under any breach notification Laws.

4.20 No General Solicitation. Neither the Company nor any person acting on behalf of the Company has offered or sold any of the Shares by any form of general solicitation or general advertising.

4.21 Foreign Corrupt Practices. Neither the Company, nor to the knowledge of the Company, any agent or other person acting on behalf of the Company, has: (i) directly or indirectly, used any funds for unlawful contributions, gifts, entertainment or other unlawful expenses related to foreign or domestic political activity, (ii) made any unlawful payment to foreign or domestic government officials or employees or to any foreign or domestic political parties or campaigns from corporate funds, (iii) failed to disclose fully any contribution made by the Company (or made by any person acting on its behalf of which the Company is aware) which is in violation of Law or (iv) violated in any material respect any provision of the Foreign Corrupt Practices Act of 1977, as amended, or any applicable non-U.S. anti-bribery Law.

4.22 Regulation M Compliance. The Company has not taken, directly or indirectly, any action designed to cause or to result in the stabilization or manipulation of the price of any security of the Company to facilitate the sale or resale of any of the Shares.

4.23 Office of Foreign Assets Control. Neither the Company nor, to the Company's knowledge, any director, officer, agent, employee or Affiliate of the Company is currently subject to any U.S. sanctions administered by the Office of Foreign Assets Control of the U.S. Treasury Department.

4.24 U.S. Real Property Holding Corporation. The Company is not and has never been a U.S. real property holding corporation within the meaning of Section 897 of the Internal Revenue Code of 1986, as amended, and the Company shall so certify upon an Investor's request.

5. Representations and Warranties of the Investors. Each Investor hereby represents and warrants to the Company, on behalf of itself, that:

5.1 Organization; Good Standing. The Investor is duly organized or formed, validly existing and in good standing under the laws of the jurisdiction of its organization or formation. The Investor has all requisite power and authority to enter into this Agreement, to purchase the number of shares of Common Stock set forth opposite the Investor's name on Exhibit A and to perform its obligations under and to carry out the other transactions contemplated by this Agreement.

5.2 Authorization. All requisite action on the part of the Investor and its general and limited partners, required by applicable Law for the authorization, execution and delivery by the Investor of this Agreement and the performance of all of its obligations hereunder, including the subscription for and purchase of the number of shares of Common Stock set forth opposite the Investor's name on Exhibit A, has been taken. This Agreement has been, duly executed and delivered by the Investor and upon the due execution and delivery hereof by the Company and each other Investor, will constitute valid and legally binding obligations of the Investor, enforceable against the Investor in accordance with its terms (except as such enforceability may be limited by (a) applicable bankruptcy, insolvency, reorganization, moratorium or other Laws of general application relating to or affecting enforcement of creditors' rights and (b) rules of Law governing specific performance, injunctive relief or other equitable remedies and limitations of public policy).

5.3 No Conflicts. The execution, delivery and performance of this Agreement and compliance with the provisions hereof by the Investor do not and shall not: (a) violate any provision of applicable Law or any ruling, writ, injunction, order, permit, judgment or decree of any Governmental Authority, (b) constitute a breach of, or default under (or an event which, with notice or lapse of time or both, would become a default under) or conflict with, or give rise to any right of termination, cancellation or acceleration of, any agreement, arrangement or instrument, whether written or oral, by which the Investor or any of its assets, are bound, or (c) violate or conflict with any of the provisions of the Investor's organizational documents (including any articles or memoranda of organization or association, charter, bylaws or similar documents), except, in the case of subsections (a) or (b), as would not have a material adverse

effect on the ability of the Investor to consummate the Transactions and perform its obligations under this Agreement.

5.4 No Governmental Authority or Third-Party Consents. No consent, approval, authorization or other order of any Governmental Authority or other Third Party is required to be obtained by the Investor in connection with the authorization, execution and delivery of this Agreement or with the subscription for and purchase of the Shares.

5.5 Purchase Entirely for Own Account. The shares of Common Stock purchased by the Investor hereunder shall be acquired for investment for the Investor's own account, not as a nominee or agent, and not with a view to the resale or distribution of any part thereof, and the Investor has no present intention of selling, granting any participation or otherwise distributing such shares. The Investor does not have and will not have as of the Closing any contract, undertaking, agreement or arrangement with any Person to sell, transfer or grant participation to a Person any of the Shares.

5.6 Disclosure of Information. The Investor has received all the information from the Company and its management that the Investor considers necessary for deciding whether to purchase any Shares hereunder. The Investor further represents that it has had an opportunity to ask questions and receive answers from the Company regarding the Company, its financial condition, results of operations and prospects and the terms and conditions of the offering of the Shares sufficient to enable it to evaluate its investment.

5.7 Investment Experience and Accredited Investor Status. The Investor is (i) a qualified institutional buyer (as defined in Rule 144A of the Securities Act) or (ii) an institutional "accredited investor" (as described in Rule 501(a)(1), (2), (3) or (7) under the Securities Act). The Investor has such knowledge and experience in financial or business matters that it is capable of evaluating the merits and risks of the investment in the Shares to be purchased by the Investor hereunder.

5.8 Restricted Securities. The Investor understands that the shares of Common Stock to be purchased by the Investor hereunder, when issued, shall be "restricted securities" under the federal securities Laws inasmuch as they are being acquired from the Company in a transaction not involving a public offering and that under such Laws such shares may be resold without registration under the Securities Act only in certain limited circumstances. The Investor represents that it is familiar with Rule 144 of the Securities Act, as presently in effect.

5.9 Legends. The Investor understands that the shares of Common Stock to be purchased hereunder shall be in book-entry form and subject to the following legend:

"These securities have not been registered under the Securities Act of 1933. They may not be sold, offered for sale, pledged or hypothecated in the absence of a registration statement in effect with respect to the securities under the Securities Act or an opinion of counsel (which counsel shall be reasonably satisfactory to PTC Therapeutics, Inc.) that such registration is not required or unless sold pursuant to Rule 144 of the Securities Act."

5.10 Financial Assurances. The Investor has access to cash in an amount sufficient to pay to the Company the aggregate purchase price for the number of shares of Common Stock set forth opposite the Investor's name on Exhibit A.

6. Investors' Conditions to Closing. Each Investor's obligation to purchase the shares of Common Stock set forth opposite such Investor's name on Exhibit A at the Closing is subject to the fulfillment as of the Closing of the following conditions (unless waived in writing by such Investor):

6.1 Representations and Warranties. The representations and warranties made by the Company in Section 4 hereof shall be true and correct as of the date of this Agreement, except to the extent such representations and warranties were specifically made as of a particular date, in which case such representations and warranties shall have been true and correct as of such date.

6.2 Covenants. All covenants and agreements contained in this Agreement to be performed or complied with by the Company at or prior to the Closing shall have been performed or complied with in all material respects.

7. Company's Conditions to Closing. The Company's obligation to issue and sell the Shares at the Closing is subject to the fulfillment as of the Closing of the following conditions (unless waived in writing by the Company):

7.1 Representations and Warranties. The representations and warranties made by each Investor in Section 5 hereof shall be true and correct as of the date of this Agreement, except to the extent such representations and warranties were specifically made as of a particular date, in which case such representations and warranties shall have been true and correct as of such date.

7.2 Covenants. All covenants and agreements contained in this Agreement to be performed or complied with by each Investor at or prior to the Closing shall have been performed or complied with in all material respects.

8. Mutual Conditions to Closing. The obligations of the Investors and the Company to consummate the Closing are subject to the fulfillment as of the Closing of the following conditions:

8.1 Injunctions. There shall be no Law or ruling enacted, promulgated, issued, entered, amended or enforced by any Governmental Authority in effect enjoining, restraining, preventing or prohibiting the consummation of the transactions contemplated by this Agreement or making the consummation of the transactions contemplated by this Agreement illegal.

8.2 Absence of Litigation. There shall be no action, suit, proceeding or investigation by a Governmental Authority pending or currently threatened in writing against the Company or the Investors that questions the validity of this Agreement, the right of the Company or the Investors to enter into this Agreement or to consummate the transactions contemplated hereby or thereby or which, if determined adversely, would impose substantial

monetary damages on the Company or the Investors as a result of the consummation of the transactions contemplated by this Agreement.

8.3 No Prohibition; Market Listing. (a) No provision of any applicable Law and no decree that prohibits, makes illegal or enjoins the consummation of the Transaction shall be in effect; and (b) the Shares shall be eligible for listing on The Nasdaq Global Select Market.

8.4 Credit Agreement. The Credit Agreement shall be in full force and effect.

9. Additional Covenants and Agreements.

9.1 Lock-Up. From and after the Closing and until the date that is ninety (90) days after the date of the Closing (the "**Lock-Up Term**"), without the prior written approval of the Company, no Investor shall Dispose of (x) any of the Shares or any shares of capital stock issued in respect thereof as a result of any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization, or (y) any Common Stock issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the Shares or shares of capital stock described in clause (x) of this sentence (collectively, the "**Lock-Up Securities**"); provided, however, that the foregoing shall not prohibit an Investor from transferring any of the Lock-Up Securities to a Permitted Transferee.

9.2 Registration Rights.

(a) Required Registration. On or prior to the date that is sixty (60) days after the date of the Closing, the Company shall file a Registration Statement under the Securities Act (the "**Required Registration**") to cover the resale of the Registrable Securities and shall use its commercially reasonable efforts to, as soon as practicable thereafter, effect the registration of the Registrable Securities to permit or facilitate the sale and distribution of the Registrable Securities, subject however, to the conditions and limitations set forth herein.

(b) Company Registration. Effective from the expiration of the Lock-Up Term until the end of the Required Period, the Company shall notify each Investor that holds Registrable Securities in writing at least ten (10) days prior to the filing of any Registration Statement related to an Underwritten Offering including shares of Common Stock by the Company or one or more selling stockholders (other than the Investors) ("**Registration Notice**") and will afford each Investor an opportunity, subject to the terms and conditions of this Agreement, to include in such Registration Statement the number of Registrable Securities then held by such Investor that such Investor wishes to include in such Registration Statement. Each Investor desiring to include in any such Registration Statement all or any part of the Registrable Securities held by such Investor shall, within five (5) days after receipt of the Registration Notice, so notify the Company in writing, and in such notification, inform the Company of the number of Registrable Securities such Investor wishes to include in such Registration Statement. If an Investor decides not to include Registrable Securities in any Registration Statement thereafter filed by the Company, such Investor shall nevertheless continue to have the right to include Registrable Securities in any subsequent Registration Statement or Registration Statements as may be filed by the Company with respect to offerings of its securities, all upon

the terms and conditions set forth herein. Each Investor shall keep confidential and not disclose to any Third Party (i) its receipt of any Registration Notice and (ii) any information regarding the proposed offering as to which such notice is delivered, except as required by law, regulation or as compelled by subpoena. The right of any such Investor to include Registrable Securities in a registration statement pursuant to this Section 9.2(b) shall be conditioned upon such Investor's participation in such underwriting and the inclusion of such Investor's Registrable Securities in the underwriting to the extent provided herein. The Investors proposing to distribute their Registrable Securities through such underwriting shall enter into an underwriting agreement in customary form with the managing underwriter or underwriters selected for such underwriting. Notwithstanding any other provision of this Section 9.2(b), if the managing underwriter for the Underwritten Offering determines in good faith that marketing factors require a limitation of the number of shares of Registrable Securities to be included in such Underwritten Offering and advises the Investors of such determination in writing, then the managing underwriter may exclude shares (including up to 100% of the Registrable Securities) from the registration and the underwriting, with the number of Registrable Securities, if any, included in the registration and the underwriting being allocated to each of Investors requesting inclusion of their Registrable Securities in such Registration Statement and all other stockholders selling shares of Common Stock pursuant to such Registration Statement on a pro rata basis based on the total number of shares of Common Stock then held by each such Investor or other stockholder. Notwithstanding the foregoing, the Company shall have the right to terminate or withdraw any registration initiated by it under this Section 9.2(b) prior to the effectiveness of such registration whether or not any Investor has elected to include securities in such registration.

(c) Primary Shares in Required Registration. With respect to the Required Registration, the Company may also propose to sell shares of Common Stock on its own behalf.

(d) Revocation of Required Registration. With respect to the Required Registration, each Investor may, at any time prior to the effective date of such Registration Statement, waive the requirement to have all of any of the Registrable Securities owned by such Investor included therein by providing a written notice to the Company, in which case such Registrable Securities will not be included in such Registration Statement.

(e) Continuous Effectiveness of Registration Statement. The Company will use its commercially reasonable efforts to cause the Registration Statement filed pursuant to this Section 9 to be declared effective by the SEC or to become effective under the Securities Act as promptly as practicable and to keep each such Registration Statement that has been declared or becomes effective continuously effective until the earlier of (i) such time as any securities registered pursuant to this Section 9 shall cease to become Registrable Securities and (ii) the date six (6) months following the date on which the Shares are issued and sold pursuant to this Agreement (the "**Required Period**"), subject, however, to the conditions and limitations set forth herein.

(f) Obligations of the Company. The Company shall, as expeditiously as reasonably possible following the Closing, subject, however, to the conditions and limitations set forth in this Section 9:

(i) prepare and file with the SEC a Registration Statement with respect to the Registrable Securities; provided that at least five (5) Business Days prior to filing the Registration Statement or any prospectus or any amendments or supplements thereto (other than any document filed under the Exchange Act that is incorporated by reference into the Registration Statement), the Company shall furnish to the Investors and their counsel copies of all such documents proposed to be filed, and the Investors shall have the opportunity to comment on any information that is contained therein and the Company shall consider all such comments in good faith and shall make the corrections reasonably requested by the Investors with respect to any information pertaining solely to the Investors and the plan of distribution prior to filing the Registration Statement or other documents;

(ii) prepare and file with the SEC such amendments and post-effective amendments to the Registration Statement and any prospectus used in connection therewith as may be necessary to keep the Registration Statement effective for the Required Period, and cause the prospectus to be supplemented by any required prospectus supplement, and as so supplemented to be filed pursuant to Rule 424 under the Securities Act, to comply with the provisions of the Securities Act with respect to the disposition of all Registrable Securities covered by such Registration Statement for the Required Period; provided that at least five (5) Business Days prior to filing any such amendments and post effective amendments or supplements thereto (other than any document filed under the Exchange Act that is incorporated by reference into the Registration Statement), the Company shall furnish to the Investors and their counsel copies of all such documents proposed to be filed, and the Investors have the opportunity to comment on any information that is contained therein and the Company shall consider all such comments in good faith and make the corrections reasonably requested by the Investors with respect to any information pertaining solely to the Investors and the plan of distribution prior to filing any such documents;

(iii) furnish to the Investors such numbers of copies of the Registration Statement, each amendment and supplement thereto, the prospectus included in such Registration Statement (including each preliminary prospectus or free writing prospectus) in conformity with the requirements of the Securities Act, and such other

documents as they may reasonably request in order to facilitate the disposition of Registrable Securities owned by them;

(iv) notify the Investors, promptly after the Company shall receive notice thereof, of the time when the Registration Statement becomes or is declared effective or when any amendment or supplement or any prospectus forming a part of such Registration Statement has been filed (other than any document filed under the Exchange Act that is incorporated by reference into the Registration Statement);

(v) notify the Investors promptly of any request by the SEC for the amending or supplementing of the Registration Statement or prospectus or for additional information and promptly deliver to the Investors copies of any comments received from the SEC;

(vi) notify the Investors promptly of any stop order suspending the effectiveness of the Registration Statement or Prospectus or the initiation of any proceedings for that purpose, and use all reasonable efforts to obtain the withdrawal of any such order or the termination of such proceedings;

(vii) use all reasonable efforts to register and qualify the Registrable Securities covered by the Registration Statement under such other securities or blue sky Laws of such jurisdictions as shall be reasonably requested by the Investors, use all reasonable efforts to keep each such registration or qualification effective, including through new filings, or amendments or renewals, during the Required Period, and notify the Investors of the receipt of any written notification with respect to any suspension of any such qualification; provided, however, that the Company shall not be required in connection therewith or as a condition thereto to qualify to do business or to file a general consent to service of process in any such states or jurisdictions, except as may be required by the Securities Act;

(viii) promptly notify the Investors at any time when a prospectus relating thereto is required to be delivered under the Securities Act of the happening of any event as a result of which the prospectus included in the Registration Statement or any offering memorandum or other offering document includes an untrue statement of a material fact or omits to state any material fact required to be stated therein or necessary to make the statements therein not misleading in the light of the circumstances then existing, and, subject to Section 9.4(g), promptly prepare a supplement or amendment to such prospectus or file any other required document so that, as thereafter delivered to the purchasers

of such Registrable Securities, such prospectus will not contain an untrue statement of material fact or omit to state any fact necessary to make the statements therein not misleading;

(ix) use commercially reasonable efforts to comply with all applicable rules and regulations of the SEC relating to such registration and make generally available to its security holders earning statements satisfying the provisions of Section 11(a) of the Securities Act, provided that the Company will be deemed to have complied with this Section 9.2(f)(ix) with respect to such earning statements if it has satisfied the provisions of Rule 158 promulgated under the Securities Act;

(x) if requested by the Investors, promptly incorporate in a prospectus supplement or post-effective amendment such information as the Investors reasonably request to be included therein, with respect to the Registrable Securities being sold by the Investors, and promptly make all required filings of such prospectus supplement or post-effective amendment; and

(xi) cause the Registrable Securities covered by such Registration Statement to be listed on each securities exchange, if any, on which equity securities issued by the Company are then listed.

The obligations of the Company set forth in this Section 9.2(f) shall terminate upon the end of the Required Period.

(g) Suspension of Registration Statement. Notwithstanding anything to the contrary in this Section 9, the Company may, upon notice to the Investors, postpone the filing or the effectiveness of a Registration Statement or of a supplement or amendment thereto or suspend the use of an effective Registration Statement if the Company is engaged in any matter that the Company determines in good faith, upon advice of counsel, that the public disclosure requirements imposed on the Company under the Securities Act in connection with the Registration Statement would require disclosure of such matter before a party makes any further offer or sale pursuant to such Registration Statement; provided, however, that the Company may not invoke this right for an aggregate period of more than 10 Business Days during the Required Period; Each Investor agrees to keep and hold confidential the fact of, and any information contained or referenced in, any communications with respect to the postponement or suspension of a Registration Statement described in this Section 9.2(g).

(h) Information; Investor Covenants. It shall be a condition precedent to the obligations of the Company to take any action pursuant to this Section 9 with respect to the Registrable Securities that each Investor furnish to the Company such information regarding itself, and the Registrable Securities held by it as is required by Regulation S-K Item 507, as shall be necessary to effect the registration of the Investor's Registrable Securities or as the Company may reasonably request in writing in connection with the Registration Statement. Each Investor agrees that, upon receipt of any notice from the Company of the happening of an

event pursuant to Section 9.2(f)(viii) hereof or suspension of a Registration Statement pursuant to Section 9.4(f), such Investor will immediately discontinue disposition of Registrable Securities pursuant to any Registration Statement covering such Registrable Securities, until the Investors are advised by the Company that such dispositions may again be made. Each Investor covenants and agrees that it will comply with the prospectus delivery requirements of the Securities Act as applicable to it or an exemption therefrom in connection with sales of Registrable Securities pursuant to any Registration Statement.

(i) Expenses. The Company will pay all expenses associated with the preparation and filing of a Registration Statement, including, without limitation, filing fees, the Company's counsel and accounting fees and expenses, costs associated with clearing the Registrable Securities for sale under applicable state securities laws and listing fees. In no event shall the Company be responsible for any discounts, commissions, fees of underwriters, selling brokers, dealer managers or similar securities industry professionals with respect to the Registrable Securities being sold.

(j) Indemnification. In the event any Registrable Securities are included in a Registration Statement under this Agreement:

(i) The Company shall indemnify and hold harmless each Investor including Registrable Securities in any such Registration Statement, any underwriter (as defined in the Securities Act) for such Investor and each Person, if any, who controls such Investor or underwriter within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act and the officers, directors, owners, agents and employees of such controlling Persons, against any and all losses, claims, damages or liabilities (joint or several) to which they may become subject under any securities Laws including, without limitation, the Securities Act, the Exchange Act, or any other statute or common law of the United States or any other country or political subdivision thereof, or otherwise, including the amount paid in settlement of any litigation commenced or threatened (including any amounts paid pursuant to or in settlement of claims made under the indemnification or contribution provisions of any underwriting or similar agreement entered into by such Investor in connection with any offering or sale of securities covered by this Agreement, provided, however, that, if available, the Investor shall seek indemnification or contribution under such underwriting or similar agreement before seeking indemnification from the Company pursuant to this Section 9.2(j)(i)), and shall promptly reimburse them, as and when incurred, for any legal or other expenses incurred by them in connection with investigating any claims and defending any actions, insofar as any such losses, claims, damages or liabilities (or actions in respect thereof) arise out of or are based upon any of the following statements, omissions or violations (each, a "**Violation**"): (i) any untrue statement or alleged untrue statement

of a material fact contained in or incorporated by reference into such Registration Statement, including any preliminary prospectus or final prospectus contained therein or any free writing prospectus or any amendments or supplements thereto, or in any offering memorandum or other offering document relating to the offering and sale of such securities, (ii) the omission or alleged omission to state therein a material fact required to be stated therein, or necessary to make the statements therein not misleading or (iii) any violation or alleged violation by the Company (or any of its agents or Affiliates) of the Securities Act, the Exchange Act, any state securities Law, or any rule or regulation promulgated under any state securities Law, in each case arising from such Registration Statement; provided, however, the Company shall not be liable in any such case for any such loss, claim, damage, liability or action to the extent that it (A) arises out of or is based upon a Violation which occurs solely in reliance upon and in conformity with written information furnished expressly for use in connection with such registration by such Investor; or (B) is caused by such Investor's disposition of Registrable Securities after notice from the Company pursuant to Section 9.2(f)(vi) or 9.2(g) during any period during which such Investor is obligated to discontinue any disposition of Registrable Securities as a result of any stop order suspending the effectiveness of any Registration Statement or prospectus with respect to Registrable Securities or a suspension of the Registration Statement. The Company shall pay, as incurred, any legal or other expenses reasonably incurred by any Person intended to be indemnified pursuant to this Section 9.2(j)(i), in connection with investigating or defending any such loss, claim, damage, liability or action; provided, however, that the indemnity agreement contained in this Section 9.2(j)(i) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability or action if such settlement is effected without consent of the Company, which consent shall not be unreasonably withheld, conditioned or delayed.

(ii) Each Investor including Registrable Securities in a Registration Statement shall indemnify and hold harmless the Company, each of its directors, each of its officers who has signed the Registration Statement, each Person, if any, who controls the Company within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act and the officers, directors, owners, agents and employees of such controlling Persons, any underwriter, any other Investor selling securities in such Registration Statement and any controlling Person of any such underwriter or other Investor, against any losses, claims, damages or liabilities (joint or several) to which any of the foregoing Persons may become subject, under liabilities (or actions in respect thereto) which arise out of or are

based upon any Violation, in each case to the extent (and only to the extent) that such Violation: (i) arises out of or is based upon a Violation which occurs solely in reliance upon and in conformity with written information furnished expressly for use in connection with such registration by such Investor; or (ii) is caused by such Investor's disposition of Registrable Securities after notice from the Company pursuant to Section 9.2(f)(vi) or 9.2(g) during any period during which such Investor is obligated to discontinue any disposition of Registrable Securities as a result of any stop order suspending the effectiveness of any Registration Statement or prospectus with respect to Registrable Securities or a suspension of the Registration Statement. Each such Investor shall pay, as incurred, any legal or other expenses reasonably incurred by any Person intended to be indemnified pursuant to this Section 9.2(j)(ii), in connection with investigating or defending any such loss, claim, damage, liability or action; provided, however, that the indemnity agreement contained in this Section 9.2(j)(ii) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability or action if such settlement is effected without consent of each such Investor, which consent shall not be unreasonably withheld.

(iii) Promptly after receipt by an indemnified party under this Section 9.2(j) of notice of the commencement of any action (including any action by a Governmental Authority), such indemnified party shall, if a claim in respect thereof is to be made against any indemnifying party under this Section 9.2(j), deliver to the indemnifying party a written notice of the commencement thereof and the indemnifying party shall have the right to participate in, and, to the extent the indemnifying party so desires, jointly with any other indemnifying party similarly noticed, to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party shall have the right to retain its own counsel, with the reasonable fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such proceeding. The failure to deliver written notice to the indemnifying party within a reasonable time of the commencement of any such action, if prejudicial in a material respect to its ability to defend such action, shall relieve such indemnifying party of any liability to the indemnified party under this Section 9.2(j), but the omission so to deliver written notice to the indemnifying party shall not relieve it of any liability that it may have to any indemnified party otherwise than under this Section 9.2(j).

(iv) In order to provide for just and equitable contribution to joint liability in any case in which a claim for indemnification is made pursuant to this Section 9.2(j) but it is judicially determined (by the entry of a final judgment or decree by a court of competent jurisdiction and the expiration of time to appeal or the denial of the last right of appeal) that such indemnification may not be enforced in such case notwithstanding the fact that this Section 9.2(j) provided for indemnification in such case, the Company and each Investor including Registrable Securities in a Registration Statement shall contribute to the aggregate losses, claims, damages or liabilities to which they may be subject (after contribution from others) in proportion to the relative fault of the Company, on the one hand, and such Investor, severally, on the other hand; provided, however, that in any such case, no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any Person who was not guilty of such fraudulent misrepresentation; provided further, however, that in no event shall any contribution under this Section 9.2(j)(iv) on the part of any Investor exceed the net proceeds received by such Investor from the sale of Registrable Securities giving rise to such contribution obligation, except in the case of willful misconduct or fraud by such Investor.

(v) The obligations of the Company and the Investors under this Section 9.2(j) shall survive the completion of any offering of Registrable Securities in a Registration Statement under this Agreement and otherwise.

(k) SEC Reports. With a view to making available to the Investors the benefits of Rule 144 under the Securities Act and any other rule or regulation of the SEC that may at any time permit the Investors to sell Registrable Securities of the Company to the public without registration, the Company agrees to, for a period of one (1) year following the date on which the Shares are issued and sold pursuant to this Agreement, provided that the Company remains a reporting company under Section 13 or 15(d) of the Exchange Act during such period:

(i) file with the SEC in a timely manner all reports and other documents required of the Company under the Exchange Act; and

(ii) furnish to each Investor, so long as such Investor owns any Registrable Securities, forthwith upon request (i) a written statement by the Company that it has complied with the reporting requirements of the Exchange Act, (ii) a copy of the most recent annual or quarterly report of the Company and such other reports and documents so filed by the Company, and (iii) such other information as may be reasonably requested in availing the Investor of any rule or regulation of the SEC (exclusive of Rule

144A) which permits the selling of any Registrable Securities without registration.

(l) Assignment of Registration Rights. The rights to cause the Company to register any Registrable Securities pursuant to this Agreement shall automatically be assigned in whole or in part (but only with all restrictions and obligations set forth in this Agreement) by an Investor to a Permitted Transferee which acquires Registrable Securities from such Investor.

9.3 Legend Removal. After the expiration of the Lock-Up Term, the Company shall use its commercially reasonable efforts to cause the legends set forth in Section 5.10 to be removed from the shares of Common Stock held by an Investor, no later than two (2) Business Days from receipt of a written request from such Investor pursuant to this Section 9.3, if (i) the Investor certifies such shares will be, or have been, resold under an effective Registration Statement, (ii) such shares have been or will be transferred in compliance with Rule 144 under the Securities Act, (iii) such shares are eligible for resale pursuant to Rule 144(b)(1)(i) under the Securities Act without the requirement for the Company to be in compliance with the current public information required under Rule 144(c)(1) under the Securities Act as to such Shares and without volume or manner-of-sale restrictions or (iv) such Investor shall have provided the Company with an opinion of counsel, reasonably satisfactory to the Company, stating that such securities may lawfully be transferred without registration under the Securities Act.

9.4 Standstill. During the period commencing on the Effective Date and ending on the earliest of (i) the date on which any Third Party unaffiliated with the Investors commences a tender offer or exchange offer for more than 50% of the Company's outstanding Common Stock (provided that if any transaction contemplated by such offer is terminated or abandoned, then the Standstill Period and the provisions of this Section 9.4 shall again become effective 24 hours after delivery of written notice of such termination or abandonment to the Standstill Parties), (ii) the date the Company publicly announces its intent, pursuant to a definitive written agreement to consummate a Change of Control, (iii) the date on which the Company publicly announces or enters into or becomes subject to a voluntary or involuntary bankruptcy, insolvency, reorganization, or moratorium event and (iv) the Facility Termination Date (the "**Standstill Period**"), none of Blackstone Alternative Credit Advisors LP ("**Blackstone Credit Advisor**"), Blackstone Life Sciences Advisors L.L.C. ("**Blackstone Life Sciences Advisor**") and any funds and accounts managed or advised by either the Blackstone Credit Advisor or the Blackstone Life Sciences Advisor (collectively, the "**Standstill Parties**") will, in any manner, directly or indirectly:

(a) make, effect, propose to make or effect, initiate or cause (i) any acquisition of beneficial ownership, directly or indirectly, of Equity Interests of the Company that would require a filing under Rule 13d-1 under the Exchange Act, (ii) any acquisition of all or substantially all of the assets of the Company, (iii) a Change of Control of the Company or other merger or other business combination involving the Company, or (iv) any "solicitation" of "proxies" (as those terms are used in the proxy rules of the SEC) or consents with respect to any securities of the Company; provided that any investment in third-party mutual funds or other

similar passive investment vehicles that hold interests in securities of the Company or any of its subsidiaries shall not be taken into account for the purpose of this subparagraph;

(b) form, join or participate in a "group" (as defined in the Exchange Act and the rules promulgated thereunder) with any third-party other than another Standstill Party or Permitted Transferee with respect to the beneficial ownership of any securities of the Company;

(c) act, alone or in concert with others, to seek to control or influence the management, the Board of Directors or policies of the Company, other than in accordance with the terms of the Credit Agreement;

(d) take any action that might require the Company to make a public announcement regarding any of the types of matters set forth in clause "(a)" of this Section 9.4;

(e) agree or offer to take, or encourage or propose (publicly or otherwise) the taking of, any action referred to in clause "(a)", "(b)", "(c)" of this Section 9.4;

(f) assist, induce or encourage any other Person to take any action of the type referred to in clause "(a)", "(b)", "(c)", or "(d)" of this Section 9.4; or

(g) enter into any discussions, negotiations, arrangement or agreement with any other Person relating to any of the foregoing.

The Standstill Parties also agree during the Standstill Period not to request the Company (or its directors, officers, employees or representatives), directly or indirectly, to amend or waive any provision of this Section 9.4 (including this sentence). Notwithstanding anything to the contrary in this Section 9.4, it is understood and agreed that the Standstill Parties shall not be prohibited from entering into an agreement and having confidential discussions with legal, accounting or financial advisors for the limited purposes of evaluating any of the transactions contemplated by this Section 9.4. For the avoidance of doubt, none of the provisions of this Section 9.4 shall in any way limit the activities of Blackstone Inc. or its affiliates in their business distinct from the activities and business of the Standstill Parties. Nothing under this Section 9.4 will impact or limit any rights or obligations of the parties under the Credit Agreement including, without limitation, the rights and obligations under Section 6.21 of the Credit Agreement.

10. Miscellaneous.

10.1 Governing Law; Submission to Jurisdiction.

(a) Governing Law. THIS AGREEMENT SHALL BE GOVERNED BY AND CONSTRUED IN ACCORDANCE WITH THE INTERNAL SUBSTANTIVE LAWS OF THE STATE OF NEW YORK WITHOUT REFERENCE TO THE RULES THEREOF RELATING TO CONFLICTS OF LAW OTHER THAN SECTION 5-1401 OF THE GENERAL OBLIGATIONS LAW OF THE STATE OF NEW YORK, AND THE OBLIGATIONS, RIGHTS AND REMEDIES OF THE PARTIES HEREUNDER SHALL BE DETERMINED IN ACCORDANCE WITH SUCH LAWS.

(b) Jurisdiction. Each of the parties hereto hereby irrevocably and unconditionally submits, for itself and its property, to the non-exclusive jurisdiction of the Supreme Court of the State of New York sitting in New York County and of the United States District Court of the Southern District of New York, and any appellate court from any thereof, in any action or proceeding arising out of, relating to or in connection with this Agreement, or for recognition or enforcement of any judgment, and each of the parties hereto hereby irrevocably and unconditionally agrees that all claims in respect of any such action or proceeding may be heard and determined in such New York State court or, to the extent permitted by applicable Law, in such federal court. Each of the parties hereto agrees that a final judgment in any such action or proceeding shall be conclusive and may be enforced in other jurisdictions by suit on the judgment or in any other manner provided by applicable Law. Each of the parties hereto hereby irrevocably and unconditionally waives, to the fullest extent it may legally and effectively do so, any objection that it may now or hereafter have to the laying of venue of any suit, action or proceeding arising out of or relating to this Agreement in any court referred to in this Section 10.1(b). Each of the parties hereto hereby irrevocably waives, to the fullest extent permitted by applicable Law, the defense of an inconvenient forum to the maintenance of such action or proceeding in any such court.

(c) Service of Process. Each of the parties hereto irrevocably consents to service of process in the manner provided for notices in Section 10.3. Nothing in this Agreement will affect the right of any party hereto to serve process in any other manner permitted by applicable Law. Each of the parties hereto waives personal service of any summons, complaint or other process, which may be made by any other means permitted by New York law.

(d) Waiver of Jury Trial. EACH PARTY HERETO HEREBY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, ANY RIGHT IT MAY HAVE TO A TRIAL BY JURY IN ANY LEGAL PROCEEDING DIRECTLY OR INDIRECTLY ARISING OUT OF OR RELATING TO THIS PURCHASE AND SALE AGREEMENT, OR THE TRANSACTIONS CONTEMPLATED HEREBY (WHETHER BASED ON CONTRACT, TORT OR ANY OTHER THEORY). EACH PARTY HERETO (I) CERTIFIES THAT NO REPRESENTATIVE, AGENT OR ATTORNEY OF THE OTHER PARTY HERETO HAS REPRESENTED, EXPRESSLY OR OTHERWISE, THAT THE OTHER PARTY HERETO WOULD NOT, IN THE EVENT OF LITIGATION, SEEK TO ENFORCE THE FOREGOING WAIVER AND (II) ACKNOWLEDGES THAT IT AND THE OTHER PARTY HERETO HAVE BEEN INDUCED TO ENTER INTO THIS PURCHASE AND SALE AGREEMENT BY, AMONG OTHER THINGS, THE MUTUAL WAIVERS AND CERTIFICATIONS IN THIS SECTION 10.1(d).

10.2 Waiver. Waiver by a party of a breach hereunder by any other party shall not be construed as a waiver of any subsequent breach of the same or any other provision. No delay or omission by a party in exercising or availing itself of any right, power or privilege hereunder shall preclude the later exercise of any such right, power or privilege by such party. No waiver shall be effective unless made in writing with specific reference to the relevant provision(s) of this Agreement and signed by a duly authorized representative of the party granting the waiver.

10.3 Notices. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address of the relevant party set forth on Exhibit B attached hereto and be effective (a) upon receipt when sent through the mails, registered or certified mail, return receipt requested, postage prepaid, with such receipt to be effective the date of delivery indicated on the return receipt, (b) upon receipt when sent by an overnight courier, (c) on the date personally delivered to an authorized officer of the party to which sent or (d) on the date transmitted by electronic mail with a confirmation of receipt, in all cases, with a copy emailed to the recipient at the applicable address. Any party may change its address by giving notice to the other parties in the manner provided above.

10.4 Entire Agreement. This Agreement contains the entire agreement among the parties with respect to the subject matter hereof and supersedes all prior and contemporaneous arrangements or understandings, whether written or oral, with respect hereto and thereto.

10.5 Amendments. No provision in this Agreement shall be supplemented, deleted or amended except in a writing executed by an authorized representative of each of the Investors and the Company.

10.6 Headings; Nouns and Pronouns; Section References. Headings in this Agreement are for convenience of reference only and shall not be considered in construing this Agreement. Whenever the context may require, any pronouns used herein shall include the corresponding masculine, feminine or neuter forms, and the singular form of names and pronouns shall include the plural and vice-versa. References in this Agreement to a section or subsection shall be deemed to refer to a section or subsection of this Agreement unless otherwise expressly stated.

10.7 Severability. If, under applicable Laws, any provision hereof is invalid or unenforceable, or otherwise directly or indirectly affects the validity of any other material provision(s) of this Agreement in any jurisdiction ("**Modified Clause**"), then, it is mutually agreed that this Agreement shall endure and that the Modified Clause shall be enforced in such jurisdiction to the maximum extent permitted under applicable Laws in such jurisdiction; provided that the parties shall consult and use all reasonable efforts to agree upon, and hereby consent to, any valid and enforceable modification of this Agreement as may be necessary to avoid any unjust enrichment of another party and to match the intent of this Agreement as closely as possible, including the economic benefits and rights contemplated herein.

10.8 Assignment. Neither this Agreement nor any of the rights or obligations hereunder may be assigned by either the Investors or the Company without (a) the prior written consent of the Company in the case of any assignment by an Investor, except as provided in Section 9.2(l) with respect to an Investor's assignment to a Permitted Transferee, or (b) the prior written consent of the Investors in the case of an assignment by the Company.

10.9 Successors and Assigns. This Agreement shall be binding upon and inure to the benefit of the parties hereto and their respective successors and permitted assigns.

10.10 Counterparts. This Agreement may be executed in counterparts, each of which shall be deemed an original but which together shall constitute one and the same instrument.

10.11 Third Party Beneficiaries. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, including any creditor of any party hereto, except with respect to a Permitted Transferee. No Third Party (other than a Permitted Transferee) shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against any party hereto.

10.12 No Strict Construction. This Agreement has been prepared jointly and will not be construed against either party.

10.13 Survival of Warranties. The representations and warranties of the Company and the Investors contained in this Agreement shall survive the Closing for eighteen (18) months, except for the representations and warranties set forth in Sections 4.1, 4.2, 4.4, 4.5(a), 4.6(c), 4.8, 4.15, 4.16, 4.17, 5.1, 5.2, 5.5, 5.7, 5.8 and 5.9, which shall survive the Closing. The parties hereby acknowledge and agree that the rights of the parties hereunder are special, unique and of extraordinary character, and that if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, such refusal or failure would result in irreparable injury to the Company or the Investors as the case may be, the exact amount of which would be difficult to ascertain or estimate and the remedies at law for which would not be reasonable or adequate compensation. Accordingly, if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, then, in addition to any other remedy which may be available to any damaged party at law or in equity, such damaged party will be entitled to seek specific performance and injunctive relief, without posting bond or other security, and without the necessity of proving actual or threatened damages, which remedy such damaged party will be entitled to seek in any court of competent jurisdiction.

10.14 Remedies. The rights, powers and remedies of the parties under this Agreement are cumulative and not exclusive of any other right, power or remedy which such parties may have under any other agreement or Law. No single or partial assertion or exercise of any right, power or remedy of a party hereunder shall preclude any other or further assertion or exercise thereof.

(*Signature Page Follows*)

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

PTC THERAPEUTICS, INC.

By: /s/ Stuart W. Peltz, Ph.D.
Name: Stuart W. Peltz, Ph.D.
Title: Chief Executive Officer and President

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

BLACKSTONE COF IV HOLDCO LP

By: GSO Capital Opportunities Associates IV LP, its general partner

By: GSO Capital Opportunities Associates IV(Delaware) LLC, its general partner

By: /s/ Marisa Beeney
Name: Marisa Beeney
Title: Authorized Signatory

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

BXLS YIELD – TRANSLATE L.P.

By: Blackstone Life Sciences Advisors L.L.C. on behalf of BXLS Yield – Translate L.P

By: /s/ Robert Liptak
Name: Robert Liptak
Title: Chief Operating Officer

EXHIBIT A

SCHEDULE OF INVESTORS

Investor	Number of Shares of Common Stock	Aggregate Purchase Price
BXLS Yield – Translate L.P.	547,645	$24,999,994.25
Blackstone COF IV Holdco LP	547,645	$24,999,994.25

Exhibit 21.1

Significant Subsidiaries of PTC Therapeutics, Inc.

PTC Therapeutics International Limited

PTC Therapeutics GT, Inc.

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in the following Registration Statements:

(1) Registration Statement (Form S-8 No. 333-194323) pertaining to the 2013 Long Term Incentive Plan and the Inducement Stock Option Award,
(2) Registration Statement (Form S-8 No. 333-189962) pertaining to the 2013 Long Term Incentive Plan, the 2013 Stock Incentive Plan, the 2009 Equity and Long Term Incentive Plan, as amended, and the 1998 Employee, Director and Consultant Stock Option Plan, as amended,
(3) Registration Statement (Form S-8 No. 333-203485) pertaining to the Inducement Stock Option Awards (April 2014 - January 2015),
(4) Registration Statement (Form S-8 No. 333-208830) pertaining to the 2013 Long Term Incentive Plan and Inducement Stock Option Awards (February 2015 – October 2015),
(5) Registration Statement (Form S-8 No. 333-211997) pertaining to the 2016 Employee Stock Purchase Plan and the Inducement Stock Option Awards (December 2015 – April 2016),
(6) Registration Statement (Form S-8 No. 333-215407) pertaining to the 2013 Long Term Incentive Plan and the Inducement Stock Option Awards (September 2016 – December 2016),
(7) Registration Statement (Form S-8 No. 333-222391) pertaining to the 2013 Long Term Incentive Plan and the Inducement Stock Option Awards (January 2017 – December 2017),
(8) Registration Statement (Form S-8 No. 333-229126) pertaining to the 2013 Long Term Incentive Plan and the Inducement Grant Awards (January 2018 – December 2018),
(9) Registration Statement (Form S-8 No. 333-235823) pertaining to the 2013 Long Term Incentive Plan, the Inducement Grant Awards (January 2019 – December 2019) and the 2020 Inducement Stock Incentive Plan,
(10) Registration Statement (Form S-3 No. 333-243712) of PTC Therapeutics, Inc.,
(11) Registration Statement (Form S-8 No. 333-251878) pertaining to the 2013 Long Term Incentive Plan and the 2020 Inducement Stock Incentive Plan, as amended,
(12) Registration Statement (Form S-8 No. 333-262018) pertaining to the 2013 Long Term Incentive Plan and the Amended and Restated 2016 Employee Stock Purchase Plan,
(13) Registration Statement (Form S-8 No. 333-265803) pertaining to the Amended and Restated 2013 Long Term Incentive Plan,
(14) Registration Statement (Form S-3 No. 333-268849) of PTC Therapeutics Inc. and
(15) Registration Statement (Form S-8 No. 333-268851) pertaining to the 2020 Inducement Stock Incentive Plan.

of our reports dated February 21, 2023, with respect to the consolidated financial statements of PTC Therapeutics, Inc. and the effectiveness of internal control over financial reporting of PTC Therapeutics, Inc. included in this Annual Report (Form 10-K) of PTC Therapeutics, Inc. for the year ended December 31, 2022.

/s/ Ernst & Young LLP

Iselin, New Jersey
February 21, 2023

Exhibit 31.1

CERTIFICATIONS

I, Stuart W. Peltz, certify that:

1. I have reviewed this Annual Report on Form 10-K of PTC Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 21, 2023

By: /s/ STUART W. PELTZ
Stuart W. Peltz
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Emily Hill, certify that:

1. I have reviewed this Annual Report on Form 10-K of PTC Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 21, 2023

By: /s/ EMILY HILL
Emily Hill
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of PTC Therapeutics, Inc. (the "Company") for the period ended December 31, 2022 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Stuart W. Peltz, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: February 21, 2023

By: /s/ STUART W. PELTZ
Stuart W. Peltz
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of PTC Therapeutics, Inc. (the "Company") for the period ended December 31, 2022 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Emily Hill, Principal Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to her knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: February 21, 2023

By: /s/ EMILY HILL
Emily Hill
Chief Financial Officer
(Principal Financial Officer)