UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **August 23, 2018**

PTC THERAPEUTICS, INC.

(Exact Name of Company as Specified in Charter)

Delaware	**001-35969**	**04-3416587**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)

100 Corporate Court South Plainfield, NJ	**07080**
(Address of Principal Executive Offices)	(Zip Code)

Company's telephone number, including area code: **(908) 222-7000**

Not applicable

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (*see* General Instruction A.2. below):

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company o

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. o

Introductory Note.

On August 23, 2018, PTC Therapeutics, Inc. (the “Company”) completed the previously announced acquisition of Agilis Biotherapeutics, Inc. (“Agilis”), a Delaware corporation (the “Merger”). The Merger was effected pursuant to an agreement and plan of merger, dated as of July 19, 2018 (the “Merger Agreement”), by and among the Company, Agility Merger Sub, Inc., a Delaware corporation and a wholly owned, indirect subsidiary of the Company (“Transitory Subsidiary”), and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, a Colorado limited liability company.

Item 1.01. Entry into a Material Definitive Agreement.

As a result of the Merger, the following Agilis agreements and arrangements effectively became agreements and arrangements of the Company.

Agreements with National Taiwan University

Agilis entered into two agreements with National Taiwan University (“NTU”) relating to its lead product candidate, referred to as "GT-AADC," for the treatment of Aromatic L-Amino Acid Decarboxylase ("AADC") deficiency ("AADC deficiency"): a collaborative research agreement, between Agilis and NTU, dated September 30, 2015, as amended (the “Collaboration Agreement”), and a license and technology transfer agreement, between Agilis, NTU and Professor Wuh-Liang (Paul) Hwu, dated December 23, 2015 (the “Licensing Agreement”).

Collaboration Agreement

The Collaboration Agreement governs the collaboration of Agilis and NTU with respect to the research and clinical trials for AADC deficiency gene therapy (the “Research”). Pursuant to the Collaboration Agreement, NTU is responsible for performing the research and clinical trials and Agilis is responsible for providing related funding. In accordance with such obligations, NTU completed a Phase 1/2 trial, AADC-010, in Taiwan of GT-AADC for the treatment of AADC deficiency and is conducting an ongoing Phase 2b trial, AADC-011, in Taiwan of GT-AADC for the treatment of AADC deficiency, in each case as discussed below under “*Description of Agilis Business*”, and is collaborating on certain other ongoing activities with third parties. Agilis’s funding obligations under the Collaboration Agreement consist of funding payments for NTU’s research paid upon the achievement of certain milestones. As of the closing of the Merger, an aggregate amount of $524,481 in funding payments has been paid to NTU and an additional $289,890 is expected to become due and payable between now and December 31, 2020. Agilis is responsible for any regulatory submissions for GT-AADC for the treatment of AADC deficiency.

Pursuant to the Collaboration Agreement, all intellectual property developed or obtained by NTU relating to the Research shall be owned by NTU. The Collaboration Agreement provides Agilis a right of first refusal (the “ROFR”) for an exclusive, worldwide, royalty bearing license for the results of the Research, which Agilis exercised in 2015 in connection with entering into the Licensing Agreement.

The Collaboration Agreement expires on September 30, 2020, with automatic annual extensions subject to Agilis’s written approval. The Collaboration Agreement can be terminated for certain specified breaches by either party upon 30 or 60 days’ notice, depending on the breach and following a specified cure period. Upon termination at Agilis’s election, NTU is obligated to return to Agilis any unused funding payments made from Agilis to NTU that have not yet been utilized, and Agilis is obligated to pay any non-cancellable expenses incurred by NTU, as of the date of termination.

Licensing Agreement

Pursuant to the Licensing Agreement, NTU granted to Agilis 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 ("CMC") records, documents, confidential information, materials and know-how pertaining to the Research, including GT-AADC for the treatment of AADC deficiency, under the Collaboration Agreement (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 (the “Licensed Products”). Subject to any regulatory delays or issues, Agilis is obligated to research, use and develop the Technology to manufacture Licensed Products by December 23, 2025. Additionally, the Licensing Agreement provides for Agilis to obtain marketing approval of GT-AADC for the treatment of AADC deficiency, either by the U.S. Food and Drug Administration (the “FDA”) or by the European Medicines Agency (the “EMA”), by December 31, 2024. The agreement also stipulates milestones in relation to a Phase 3 trial with respect to GT-AADC for the treatment of AADC deficiency, which such Phase 3 trial the Company does not deem necessary and does not plan to conduct.

Agilis paid to NTU a lump sum of $100,000 upon execution of the Licensing Agreement. Additionally, the Licensing Agreement provides that NTU will be entitled to receive contingent payments from Agilis based on (i) the achievement of certain clinical and regulatory milestones up to an aggregate maximum amount of $2.0 million, (ii) annual license maintenance

fees, (iii) a low double-digit percentage royalty of annual net sales of Licensed Products, and (iv) 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.

Under the Licensing Agreement, all intellectual property relating to the manufacture, production, assembly, use or sale of Technology and any Licensed Products derived thereof are owned by NTU.

The Licensing Agreement expires on December 23, 2035. Upon expiration, Agilis will have a fully paid-up, perpetual, royalty-free exclusive license to the Technology. Agilis may terminate the 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 GT-AADC 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 a MAA with the EMA, or equivalent biologics approval application in another territory with respect to GT-AADC for the treatment of AADC. In such termination event, Agilis must pay $100,000 to NTU within 30 days of termination and NTU would retain all rights to the Technology. Agilis may terminate the Licensing Agreement for material breach by another party following a 30-day cure period. NTU may terminate the Licensing Agreement for Agilis's failure to pay any undisputed license fees or net sales or sublicensing royalty fees within the applicable deadline following a 30-day cure period.

The foregoing descriptions of the Collaboration Agreement and the Licensing Agreement are summaries only and are qualified in their entirety by reference to the terms of the Collaboration Agreement and the Licensing Agreement, copies of which will be filed with the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2018.

Item 2.01. Completion of Acquisition or Disposition of Assets.

Agilis Acquisition

On August 23, 2018, the Company completed the previously announced acquisition of Agilis pursuant to the Merger Agreement. The Merger Agreement provided for the acquisition of Agilis by the Company through the merger of Transitory Subsidiary into Agilis, with Agilis surviving as a wholly owned, indirect subsidiary of the Company.

Upon the closing of the Merger, the Company paid to Agilis equityholders total upfront consideration of approximately $200.0 million. The total upfront consideration was composed of (i) approximately $50.0 million, funded with cash on hand less Agilis transaction expenses and all amounts outstanding under the Bridge Loan Agreement (defined below) as of the closing and subject to certain other pre- and post-closing adjustments, and (ii) 3,500,907 shares of the Company's common stock (the "Closing Stock Consideration"). The Closing Stock Consideration was determined by dividing $150.0 million by the volume-weighted average price per share of the Company's common stock on the Nasdaq Global Select Market for the ten consecutive trading day period ending on the second trading day immediately preceding the closing of the Merger. As previously disclosed, and subject to the terms and conditions of the Merger Agreement, Agilis equityholders may become entitled to receive contingent payments from the Company 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. Under the Merger Agreement, the Company is required to pay $40.0 million of the development milestone payments no later than the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved.

The above description of the Merger Agreement is a summary only and is qualified in its entirety by reference to the terms of the Merger Agreement. A copy of the Merger Agreement was previously filed as Exhibit 2.1 to the Company's Current Report on Form 8-K filed on July 19, 2018.

The representations, warranties and covenants contained in the Merger Agreement were made only for the purposes of the Merger Agreement, were made as of specific dates, were made solely for the benefit of the parties to the Merger Agreement and may not have been intended to be statements of fact but, rather, as a method of allocating risk and governing the contractual rights and relationships among the parties thereto.

Bridge Loan Agreement

As previously disclosed, in connection with its entry into the Merger Agreement, the Company entered into a Bridge Loan and Security Agreement (the "Bridge Loan Agreement") on July 19, 2018 with Agilis and certain of Agilis's domestic subsidiaries, as guarantors. Under the Bridge Loan Agreement, the Company made a term loan advance to Agilis on July 23, 2018 in an original principal amount of $10.0 million. In connection with the closing of the Merger, the original principal amount of $10.0 million plus all accrued and unpaid interest thereon was credited against the cash portion of the upfront consideration paid by the Company pursuant to the terms of the Merger Agreement in satisfaction of Agilis's outstanding payment obligations under the Bridge Loan Agreement, and the Company will have no further obligation to extend any further loan amounts under the Bridge Loan Agreement.

Description of Agilis' Business

Agilis is a biotechnology company advancing a gene therapy platform focused on the development of innovative therapies for rare, debilitating diseases of the central nervous system ("CNS"). Agilis's lead product candidate is GT-AADC 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. 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 and is required to be present 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 (global muscular hypotonia/dystonia), 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 approximately 1 in 40,000 worldwide. While several diagnostic tests for AADC deficiency are available, the condition remains largely misdiagnosed or undiagnosed.

GT-AADC is a large molecule, adeno-associated virus (AAV) gene therapy, which has been assessed in two completed clinical trials, and one trial in which enrollment and dosing is ongoing. The two completed trials include a total of 18 children with severe AADC deficiency who were treated with a one-time total dose of 1.8×10^{11} vg of GT-AADC during a single procedure in which the gene therapy was administered directly to the region of the brain where dopamine is made, called the putamen. 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 secretion 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 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 endpoints of these trials were to assess the safety and efficacy of GT-AADC administered via bilateral putaminal- infusions in patients with severe AADC deficiency at a total one-time dose of 1.8×10^{11} vg. Study enrollment required a diagnosis of AADC deficiency, defined as decreased homovanilic acid ("HVA") and *5*-hydroxyindoleacetic acid ("5-HIAA") and elevated L-Dopa cerebrospinal fluid ("CSF") levels, presence of more than one DDC gene mutation, and presence of clinical symptoms of AADC deficiency (including developmental delay, hypotonia, dystonia, and oculogyric crisis), and 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 ("PDMS-2"), and Alberta Infant Motor Scale ("AIMS")) through the first year after treatment with GT-AADC 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 FDOPA 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 GT-AADC 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 GT-AADC, the combined group of patients showed significant changes from baseline capabilities at one-year post-treatment in functional motor skills assessed with the PDMS-2 total score,

as well as locomotion, grasping, visual-motor integration and stationary subscales. Significant changes from baseline at one-year post-treatment were also observed for the combined group of patients on the AIMS total score and 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-years 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 GT-AADC 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 GT-AADC was dyskinesia and these events completely resolved over time. No serious adverse events have been attributed to GT-AADC.

The ongoing clinical trial, AADC-011, is a single-center, open-label trial to assess the efficacy and safety of GT-AADC 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 homovanillic acid (HVA) or 5-hydroxyindoleacetic acid (HIAA) in the cerebrospinal fluid. A total of 10 patients are planned for recruitment, of which 8 have been enrolled and treated to date.

An end-of-phase 2 meeting was held with the FDA in July 2017, and the clinical, non-clinical and manufacturing data available to date from the two completed clinical trials was reviewed. The FDA provided feedback indicating that the clinical and non-clinical data available to date was sufficient to support the submission of a biologics license application (“BLA”) without undertaking additional trials or studies at this time. Additionally, we have requested a CMC Type C meeting with the FDA to discuss the manufacturing data relating to GT-AADC. Based on the FDA input, we are preparing a BLA for GT-AADC for the treatment of AADC deficiency in the United States, which we anticipate submitting to the FDA in 2019. GT-AADC for the treatment of AADC deficiency has orphan drug designation in the United States and European Union, and rare pediatric disease designation in the United States, and upon BLA approval the FDA may grant us a priority review voucher.

In April 2018, Agilis held a protocol assistance meeting with the Scientific Advice Working Party of the EMA in anticipation of the expected submission of a MAA in the European Union and received feedback indicating the clinical and non-clinical data available to date was sufficient to support the submission of an MAA without undertaking additional trials or studies at this time. We expect to prepare and submit to the EMA an MAA for the treatment of AADC deficiency with GT-AADC in the European Union during 2019.

There is no guarantee that we will be able to make the BLA or MAA submissions within our expected timelines or that following such submissions, the FDA or EMA would not have additional comments or requirements with respect to the respective submissions that we would be required to address before obtaining regulatory approval, or that the FDA, the EMA or any other regulatory authority will approve GT-AADC for treatment of AADC deficiency at all.

If GT-AADC for the treatment of AADC deficiency receives FDA approval, we expect that GT-AADC 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 (“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 of 1983 (the "Orphan Drug Act"). We are pursuing patent protection for GT-AADC, and, in the meantime, we expect to rely on the twelve-year BPCIA regulatory exclusivity and concurrent seven-year Orphan Drug Act exclusivity to commercialize GT-AADC in the United States, if it is approved.

The Agilis pipeline also includes a gene therapy 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. An investigational new drug ("IND") submission with the FDA for this program is expected in 2019. Additionally, the Agilis pipeline includes two other gene therapy programs targeting CNS disorders, including Angelman syndrome, a rare, genetic, neurological disorder characterized by severe developmental delays.

Intellectual Property

As part of our acquisition of Agilis, we are acquiring a patent portfolio consisting of U.S. patents and patent applications, including original filings, continuations and divisional applications, as well as numerous foreign counterparts to many of these patents and patent applications. We exclusively in-license these patents and patent applications with claims directed to composition of matter, formulation and methods of use, including for the target disease AADC. For a further discussion of the material agreements relating to our in-licensing of GT-AADC for the treatment of AADC deficiency, see Item 1.01 of this Current Report on Form 8-K.

Manufacturing

Agilis presently contracts with third parties for the manufacturing of program materials for our gene therapy product candidates. The use of contracted manufacturing and reliance on collaboration partners is relatively cost efficient and has eliminated the need for our direct investment in manufacturing facilities and additional staff early in development of our gene therapy product candidates. Although we rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers. We plan on relying on third-party manufacturers to be capable of providing sufficient quantities of our program materials to meet anticipated clinical trial and commercial scale demands.

Competition

Currently, no treatment options are available for the underlying cause of AADC deficiency, and care is limited to palliative options with significant burden on caregivers. Additionally, we are not aware of any late-stage development product candidates for AADC deficiency. However, 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.

Government Regulation of Gene Therapy

In the United States, the FDA regulates 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 biologic products. Applications to the FDA are required before conducting human clinical testing of biologic products. Additionally, each clinical trial protocol for a gene therapy product candidate is reviewed by the FDA.

Until recently, the National Institutes of Health, or the NIH, through its Recombinant DNA Advisory Committee, or RAC, also reviewed certain proposed gene therapy trials; however, the FDA and the NIH recently proposed to change this practice so that the RAC will no longer review individual human gene transfer protocols. The NIH has stated that it will finalize this change after taking public comments. 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.

U.S. New Drug and Biological Product Development Process

The development process for new biologic products under the FDA is substantially similar to the FDA’s development process for new pharmaceutical drug products. However, while a New Drug Application ("NDA"), the vehicle through which the FDA approves a new pharmaceutical drug product for sale and marketing in the United States, is filed for drug products with the FDA, a Biologics License Application ("BLA") is filed for biologic products with the FDA instead. Following submission of a BLA with the FDA, the FDA approval process of a biologic product is the same as the approval process for regular pharmaceutical drug products. For a further discussion of the FDA's approval process that applies to biologic products and pharmaceutical drug products, see “Item 1. Business-Government Regulation-The new drug approval process” in our Annual Report on Form 10-K for the year ended December 31, 2017.

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 various guidance documents regarding 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 assessment of gene therapies; 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 measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene therapy trials and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these trials.

Compliance with the FDA's current Good Manufacturing Practices ("cGMP") requirements

Manufacturers of biologics must comply with applicable cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. 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 foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process.

Establishments may be subject to periodic, unannounced inspections by government authorities to ensure compliance with cGMP requirements and other laws. Discovery of problems may result in a government entity placing restrictions on a product, manufacturer or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market. The FDA will not approve an application unless it determines that the manufacturing processes and facilities comply with cGMP requirements and adequate to assure consistent production of the product within required specification.

Concurrent with clinical trials, companies usually complete additional preclinical studies and must also develop additional information about the physical characteristics of the biologic product candidate as well as finalize a process for manufacturing the product candidate in commercial quantities in accordance with cGMP requirements. 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 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 stability studies must be conducted to demonstrate that the biologic product candidate does not undergo unacceptable deterioration over its shelf life.

BPCIA Exclusivity

We are currently pursuing patent protection for GT-AADC for the treatment of AADC deficiency, and, in the meantime, we expect to rely on the twelve-year Biologics Price Competition and Innovation Act of 2009 ("BPCIA") regulatory exclusivity to commercialize GT-AADC in the United States, if it is approved.

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. As of January 1, 2018, the FDA has approved nine biosimilar products for use in the United States. No interchangeable biosimilars have been approved. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars. We expect the FDA to finalize additional guidance in the near term.

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 are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. 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. 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 preclinical 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. At present, it is unclear whether products deemed “interchangeable” by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

Government regulation of gene therapy outside of the United States

In addition to regulations in the United States, sponsors are subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of biologic products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries. For a further discussion of government regulation outside the United States that is applicable to biologic products as well, see “Item 1. Business-Government Regulation-Regulation outside the United States” in our Annual Report on Form 10-K for the year ended December 31, 2017.

European Union regulation and exclusivity

To obtain regulatory approval for gene therapy products under the European Union regulatory framework, applicants must submit an MAA to the EMA under the ‘centralized procedure’ pursuant to Regulation 726/2004. This procedure allows the marketing-authorization holder to market the medicine throughout the European Union on the basis of a single marketing authorization. The grant of marketing authorization in the European Union for products containing viable human tissues or cells such as gene therapy medicinal products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal

products. Regulation 1394/2007/EC sets out specific rules concerning the authorization, supervision and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products. Marketing authorization applicants for biological medicinal products, including advanced therapy medicinal products, must demonstrate the quality, safety and efficacy of their product candidates to EMA which provides an opinion regarding the application for marketing authorization. The European Commission grants an MAA on the basis of the EMA opinion (or rejects the opinion of the EMA).

For a further discussion of government regulation in the European Union that is applicable to biologic products as well, see “Item 1. Business-Government Regulation-Regulation in the European Union” in our Annual Report on Form 10-K for the year ended December 31, 2017.

Orphan drug designation

Agilis has received orphan drug designation for GT-AADC for the treatment of AADC deficiency in both the United States and European Union. For a discussion of the general parameters concerning orphan drug designation in the United States and European Union that also is applicable to biologic products, see “Item 1. Business-Government Regulation-U.S. government regulation-Orphan drug designation” and “Item 1. Business-Government Regulation-Regulation in the European Union”, respectively in our Annual Report on Form 10-K for the year ended December 31, 2017.

Item 3.02. Unregistered Sales of Equity Securities.

The description of the Closing Stock Consideration under the terms of the Merger Agreement set forth in Item 2.01 is incorporated herein by reference. In connection with the closing of the Merger, the Company issued to the Agilis equityholders the Closing Stock Consideration pursuant to an exemption from registration afforded by Section 4(a)(2) of the Securities Act of 1933, as amended (the “Securities Act”), and/or Regulation D promulgated thereunder.

Item 7.01. Regulation FD Disclosure.

On August 23, 2018, the Company issued a press release in which it announced the closing of the Merger. A copy of the press release is attached to this Current Report on Form 8-K (this "Report") as Exhibit 99.1 and is incorporated by reference into this Item 7.01.

The information set forth in or incorporated by reference into this Item 7.01, including Exhibit 99.1, shall not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Item 8.01. Other Events.

The Company is filing herewith as Exhibit 99.2 certain risk factors related to the Agilis business that are relevant to the Company, giving effect to the Merger.

Item 9.01. Financial Statements and Exhibits.

(a) Financial Statements of Business Acquired

(i) The audited financial statements of Agilis as of and for the years ended December 31, 2017 and 2016 and the independent auditors’ report thereon are filed as Exhibit 99.3 hereto and are incorporated into this Item 9.01(a) by reference.

(ii) The unaudited financial statements of Agilis as of and for the six months ended June 30, 2018 are filed as Exhibit 99.3 hereto and are incorporated into this Item 9.01(a) by reference.

(b) Pro Forma Financial Information

The unaudited pro forma combined financial statements of the Company are filed as Exhibit 99.4 hereto and are incorporated into this Item 9.01(b) by reference.

(d) Exhibits

Exhibit No.	Description
23.1	Consent of BDO USA, LLP
99.1	Press Release, dated August 23, 2018 issued by PTC Therapeutics, Inc.
99.2	Risk Factors of Agilis's Business
99.3	Audited financial statements of Agilis Biotherapeutics, Inc. as of and for the years ended December 31, 2017 and 2016 and the independent auditors' report thereon and unaudited financial statements of Agilis Biotherapeutics, Inc. as of and for the six months ended June 30, 2018
99.4	Unaudited pro forma combined statements of operations for the year ended December 31, 2017 and for the six months ended June 30, 2018 and unaudited pro forma combined balance sheet as of June 30, 2018

Cautionary Statement Concerning Forward Looking Statements

This Report contains forward-looking statements addressing the Merger and the other transactions contemplated in the Merger Agreement and any other statements about future expectations, prospects, estimates and other matters that are dependent upon future events or developments. All statements, other than those of historical fact, contained in this Report are forward-looking statements, including statements related to the Company's expectations with respect to the potential financial impact and benefits to the Company of the Merger, including with respect to the business of Agilis and the Company's expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the Agilis equityholders with respect thereto; the future expectations, plans and prospects for the Company; the Company's strategy, future operations, future financial position, future revenues or projected costs; the integration of Agilis's operations and employees; and the objectives of management. Other forward-looking statements may be identified by the words "look forward", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions. The Company's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the Company's ability to realize the anticipated benefits of the Merger, including the possibility that the expected benefits from the Merger will not be realized or will not be realized within the expected time period; significant transaction costs; unknown liabilities; the risk of litigation and/or regulatory actions related to the Merger; other business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of Translarna™ (ataluren), Emflaza®, Tegsedi™ (inotersen), Waylivra™ (volanesorsen) or any other product candidate; the sufficiency of the Company's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; the integration of Agilis's operations and employees; and the factors discussed in the "Risk Factors" section of the Company's most recent Quarterly Report on Form 10-Q or Annual Report on Form 10-K as well as any updates to these risk factors filed from time to time in the Company's other filings with the SEC. As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product candidate will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna, Emflaza, Tegsedi, Waylivra, any product candidates acquired in the Merger, including GT-AADC, or any other product candidate. The forward-looking statements contained herein represent the Company's views only as of the date of this Report and the Company does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this Report except as required by law. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

Signature

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Report to be signed on its behalf by the undersigned hereunto duly authorized.

PTC Therapeutics, Inc.

Date: August 24, 2018

By: /s/ Christine Utter

Name: Christine Utter

Title: Principal Financial Officer

Exhibit 23.1

CONSENT OF INDEPENDENT AUDITORS

PTC Therapeutics, Inc.
South Plainfield, New Jersey

We hereby consent to the incorporation by reference in the Registration Statements on Form S3 (No. 333-220151) and Form S-8 (No. 333-194323, 333-189962, 333-203485, 333-208830, 333-211997, 333-215407, and 333-222391) of PTC Therapeutics, Inc. of our report dated August 21, 2018, relating to the consolidated financial statements, which appears in this Current Report on Form 8-K dated August 24, 2018.

/s/ BDO USA, LLP
Boston, Massachusetts

August 24, 2018

Exhibit 99.1

PTC Therapeutics Successfully Completes Acquisition of Agilis Biotherapeutics

SOUTH PLAINFIELD, N.J., August 23, 2018 – PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that it has successfully completed the acquisition of Agilis Biotherapeutics, Inc., a private biotechnology company focused on the advancement of innovative gene therapy programs for rare genetic disorders that affect the central nervous system (CNS).

"We are excited to complete the acquisition and welcome the Agilis employees to PTC," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We have added a team with exceptional gene therapy experience, a targeted micro-delivery gene therapy platform that has demonstrated durable clinical benefit, as well as gene therapy manufacturing and R&D capabilities. This acquisition deepens our pipeline and, as a combined organization, we will be able to optimally treat more rare disorders and accelerate the process of bringing important new therapies to patients worldwide."

With the acquisition, PTC adds GT-AADC, an adeno-associated virus (AAV) gene therapy that treats Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency. AADC Deficiency is a rare CNS disorder that is caused by mutations in the dopa decarboxylase (DDC) gene. There are no treatments that target the underlying cause of this disorder. The results from prospective clinical studies, indicated that treated subjects were found to exhibit substantial gains on motor function and cognitive scales over multiple years following the single gene therapy treatment.

The acquisition also includes gene therapy programs in development, GT-FA, GT-AS, and GT-RLN, for Friedreich Ataxia, Angelman Syndrome and Cognitive Disorders associated with several neurodevelopmental and neurodegenerative disorders, respectively.

About PTC Therapeutics
PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder products and has a global commercial footprint. This success is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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Forward Looking Statements:
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements, other than those of historical fact, contained in this release are forward-looking statements including statements addressing PTC's acquisition of Agilis; PTC's expectations with respect to the potential financial impact and benefits from the acquisition to PTC, including with respect to the business of Agilis and PTC's expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the Agilis equityholders with respect thereto; the future expectations, plans and prospects for PTC; PTC's strategy, future operations, future financial position, future revenues or projected costs; the integration of Agilis's operations and employees; and the objectives of management. Other forward-looking statements may be identified by the words "look forward", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: PTC's ability to realize the anticipated benefits of the acquisition, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; significant transaction costs; unknown liabilities; the risk of litigation and/or regulatory actions related to the acquisition; other business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of Translarna™ (ataluren), Emflaza®, Tegsedi™ (inotersen), Waylivra™ (volanesorsen) or any other product candidate; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; the integration of Agilis's operations and employees; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q or Annual Report on Form 10-K as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product candidate will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna, Emflaza, Tegsedi, Waylivra, any product candidates acquired in the acquisition or any other product candidate.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

Exhibit 99.2

RISK FACTORS

We have set forth in Item 1A to our Annual Report on Form 10-K for the year ended December 31, 2017, risk factors relating to our business, our industry, our structure and our common stock. Please refer to such Item 1A for a more complete understanding of risks concerning us. There have been no material changes in our risk factors since those published in such Form 10-K for the year ended December 31, 2017, other than as reported in Item Part II Item 1A on our Form 10-Q for the period ended March 31, 2018 and for the period ended June 30, 2018, and as reported below.

Risks Related to Our Recent Acquisition of Agilis Biotherapeutics, Inc., or Agilis

We may fail to realize the anticipated benefits of our acquisition of Agilis, those benefits may take longer to realize than expected, and we may encounter significant integration difficulties.

On August 23, 2018, we completed the previously announced acquisition of Agilis pursuant to an agreement and plan of merger, dated July 19, 2018, or the Merger Agreement.

Our ability to realize the anticipated benefits of our acquisition of Agilis will depend, to a large extent, on our ability to integrate Agilis's operations and employees into our business and realize anticipated growth opportunities and synergies. Prior to our acquisition of Agilis, we had no substantial experience developing or manufacturing large molecules including gene therapy. We will be required to devote significant management attention and resources to integrating Agilis's operations and employees into our business and any product candidates acquired from Agilis into our development and commercialization efforts and business strategy. The process may be disruptive to our business and the expected benefits may not be achieved within the anticipated time frame, or at all. The failure to meet the challenges involved and to realize the anticipated benefits of the transaction could cause an interruption of, or a loss of momentum in, our development and commercialization efforts and could adversely affect our business, financial condition and results of operations.

Our ability to realize the anticipated benefits of the transaction is expected to entail numerous material potential difficulties, including, among others:

- the diversion of management attention to integration matters;
- difficulties in achieving anticipated business opportunities and growth prospects from our acquisition of Agilis;
- challenges related to public and market perception of our acquisition of Agilis and gene therapy and increased regulatory scrutiny of gene therapy;
- difficulties in managing the expanded operations of a larger and more complex company following our acquisition of Agilis;
- difficulties in assimilating employees and in attracting and retaining key personnel; and
- potential unknown liabilities, adverse consequences, unforeseen increased expenses or other unanticipated problems associated with the transaction.

Many of these factors are outside of our control, and any one of them could result in increased costs, decreased expected revenues and further diversion of management time and energy, which could materially impact our business, financial condition and results of operations.

All of these factors could decrease or delay the expected accretive effect of the transaction and negatively impact our stock price. As a result, it cannot be assured that our acquisition of Agilis will result in the full realization of the benefits anticipated from the transaction within the anticipated timeframes or at all.

Upfront consideration for our acquisition of Agilis was comprised of approximately $50.0 million in cash, subject to certain post-closing adjustments, and 3,500,907 shares of our common stock, or the Closing Stock Consideration. In addition, pursuant to the Merger Agreement, Agilis equityholders will be entitled to receive contingent payments from us 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 of Friedreich ataxia and Angelman Syndrome products during specified terms, ranging from 2-6%. Under the Merger Agreement, we are required to pay $40.0 million of the development milestone payments no later than August 23, 2020, regardless of whether the applicable milestones have been achieved. There is no guarantee that we will be able to make these milestone payments through cash on hand and expected cash flows and we may be required to raise additional capital in order to fund these payments.

Following completion of the acquisition, we became responsible for Agilis's liabilities and obligations, including with respect to certain agreements, financial, regulatory and compliance matters, in addition to the expenses we expect to incur based on our current commercial, regulatory, research and development plans for GT-AADC and the other assets acquired from Agilis. These expenses and obligations will result in additional cost and investment by us and, if we have underestimated the amount of these costs and investments or if we fail to satisfy any such obligations, we may not realize the anticipated benefits of the transaction. Further, it is possible that there may be undisclosed, contingent or other liabilities or problems that may arise in the future, the existence and/or magnitude of which we were previously unaware. Any such liabilities or problems could have an adverse effect on our business, financial condition or results of operations.

The issuance of our common stock to complete this transaction was dilutive to our existing stockholders and because we have limited financial resources, by investing in this transaction, we may forego or delay pursuit of other opportunities that may have proven to have greater commercial potential.

In addition, the Merger Agreement obligates us to register for resale the Closing Stock Consideration, and the sale or resale of these shares in the public market, or the market's expectation of such sales, may result in a decline in our stock price. Such a decline would adversely affect our stockholders 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.

We may fail to obtain regulatory approval for GT-AADC for the treatment of AADC deficiency within our expected timeline or at all.

In July 2017, Agilis held an end-of-phase 2 meeting with the United States Food and Drug Administration, or FDA, and the clinical, non-clinical and manufacturing data available to date from two completed GT-AADC clinical trials was reviewed. The FDA provided feedback indicating that the clinical and non-clinical data available to date was sufficient to support a submission for a biologics license application, or BLA, without undertaking additional trials at this time. Additionally, we have requested a CMC Type C meeting to discuss GT-AADC manufacturing data with the FDA. Based on the FDA input, we are preparing a BLA for GT-AADC for the treatment of AADC deficiency in the United States, which we anticipate submitting to the FDA in 2019. In April 2018, Agilis held a protocol assistance meeting with the Scientific Advice Working Party of the European Medicines Agency, or EMA, in anticipation of the expected submission of a Marketing Authorization Application, or MAA, in the European Union and received feedback indicating the clinical and non-clinical data available to date was sufficient to support a submission for an MAA without undertaking additional trials or studies at this time. We expect to prepare and submit an MAA for GT-AADC for the treatment of AADC deficiency in the European Union with the EMA during 2019. There is no guarantee that we will be able to make our BLA or MAA submissions within our expected timelines or that upon our making the submissions, the FDA or the EMA would not have additional comments or requirements with respect to the respective submissions that we would be required to address before obtaining regulatory approval, or that the FDA or the EMA will approve GT-AADC for the treatment of AADC deficiency at all. Any delays in obtaining regulatory approval from either the FDA and/or the EMA, or if we never obtain regulatory approval from either the FDA and/or the EMA, could have a material adverse effect on our business, financial condition and results of operations.

Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our gene therapy product candidates or otherwise harm our business.

The manufacture of gene therapy products, such as GT-AADC and the other potential gene therapy product candidates we acquired from Agilis, is technically complex and necessitates substantial expertise and capital

investment. Production difficulties caused by unforeseen events may delay the availability of material for clinical studies and commercial product for any of our gene therapy product candidates that may receive regulatory approval in the future. We presently contract with third parties for the manufacturing of program materials for our gene therapy product candidates. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities for our gene therapy product candidates. The use of contracted manufacturing and reliance on collaboration partners is relatively cost-efficient and has eliminated the need for our direct investment in manufacturing facilities and additional staff early in development of our gene therapy product candidates. Although we rely on contract manufacturers, we have personnel with manufacturing and quality experience to oversee our contract manufacturers.

We plan on relying on third-party manufacturers to be capable of providing sufficient quantities of our program materials to meet anticipated clinical trial scale demands. To meet our projected needs for commercial manufacturing, the third party from whom we currently obtain our clinical supply of GT-AADC may need to increase its scale of production and confirm with the applicable regulatory authorities that the commercial material is comparable to the material used in clinical trials in addition to satisfying other regulatory obligations, or we will need to secure alternate suppliers. In general, 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 and facilities for large-scale production. While we believe that there are alternate 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 alternate suppliers would be able to supply our potential commercial needs. Any switch from our current manufacturer would result in a significant delay and cause material additional costs.

The manufacturers of pharmaceutical products must comply with strictly enforced cGMP requirements, state and federal regulations, as well as foreign requirements when applicable. Any failure by us or our contract manufacturing organizations to adhere to or document compliance to such regulatory requirements could lead to a delay or interruption in the availability of our program materials for clinical studies or commercial use. If we or our manufacturers fail to comply with the FDA, EMA, or other regulatory authorities, it could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates. Our dependence upon others for the manufacture of our product candidates may also adversely affect our business, results of operations, financial condition and prospects, and our ability to commercialize any product candidates that receive regulatory approval on a timely and competitive basis.

The process for administering GT-AADC is complex and includes specific specialized requirements that could delay or prevent the regulatory approval of GT-AADC for the treatment of AADC deficiency, limit its commercial potential or result in significant negative consequences following any potential marketing approval.

GT-AADC is administered directly to the putamen in the brain using sterotactic surgery, a brain surgery requiring significant skill and training. There is little experience with such surgeries being used to deliver drugs and virtually no experience for such surgeries being performed on children. If we are unable to engage with and train sufficient brain surgeons to perform the procedure properly, the availability of GT-AADC for the treatment of AADC deficiency could be substantially diminished.

Any contamination in our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules and adversely affect our ability to meet our supply obligations.

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.

Some of the raw materials and other components required in our manufacturing process are derived from diverse biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A

material shortage, 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 of clinical material, which could materially and adversely affect our development and commercialization timelines, including with respect to GT-AADC for the treatment of AADC deficiency, and our business, financial condition and results of operations.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. Such requirements may lengthen the regulatory review process, require us to perform additional studies, and increase our development costs, or may force us to delay, limit, or terminate certain of our programs.

We may experience development problems related to our gene therapy program that cause significant delays or unanticipated costs, or that cannot be solved. Although numerous companies are currently advancing gene therapy product candidates through clinical trials and the FDA has approved several cell-based gene therapy treatments to date, the FDA has only approved one vector-based gene therapy product to date. In addition, there are only two gene therapy products for genetic diseases approved to date in the European Union**.** As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for GT-AADC for the treatment of AADC deficiency or our other potential gene therapy product candidates in any jurisdiction, if at all. Regulatory requirements governing gene and cell therapy products are still evolving and may continue to change in the future. The FDA may continue to issue new guidance and replace existing guidance. Similarly, the European Commission may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. Regulatory review agencies and the new 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. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring our product candidates to market could have a material adverse effect on our business, results of operations, financial condition and prospects. Even if we do obtain regulatory approval, ethical, social and legal concerns about gene therapy arising in the future could result in additional regulations restricting or prohibiting sale of our products.

In addition, the clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied product candidates.

The FDA has established the Office of Tissues and Advanced Therapies within the Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise the CBER in its review; other international regulatory agencies have also dedicated personnel and/or offices to review gene therapy programs and products.

These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional 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 limitations or restrictions. As we advance our gene therapy product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of certain of our product candidates. These additional requirements may result in a review and approval process that is longer than we otherwise would have expected. Delays as a result of increased or lengthier regulatory approval process and further restrictions on development of our gene therapy product candidates can be costly and could negatively impact our or our collaborators' ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all, any of which could have a material adverse effect on our business, results of operations, financial condition and prospects.

Our gene therapy product candidates and the process for administering such product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

The goal of gene therapy is to be able to correct an inborn genetic defect through one-time administration of therapeutic genetic material containing non-defective gene copies. The gene copies are designed to reside permanently in a patient, allowing the patient to produce an essential protein or ribonucleic acid, or RNA, molecule that a healthy person would normally produce. There is a risk, however, that the new gene copies will produce too much or too little of the desired protein or RNA. There is also a risk that production of the desired protein or RNA will increase or decrease over time. Because the treatment is irreversible, there may be challenges in managing side effects, particularly those caused by overproduction. 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, because the new gene copies are designed to reside permanently in a patient, there is a risk that they will disrupt other normal biological molecules and processes, including other healthy genes, and we may not learn the nature and magnitude of these side effects until long after clinical trials have been completed.

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. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which, while not necessarily adverse to the patient's health, could substantially limit the effectiveness of the treatment.

In addition to any potential side effects caused by any gene therapy product candidate, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur, our clinical trials could be suspended or terminated or we may be required to cease commercial sales of any product candidates that may receive regulatory approval. If in the future we are unable to demonstrate that such adverse events were caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may have a material adverse effect on our business, results of operations, financial conditions and prospects.

Furthermore, if we or others later identify undesirable side effects caused by any of our gene therapy product candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of any product candidate that may receive regulatory approval, thereby preventing or delaying its commercialization;
- regulatory authorities may require additional warnings or limitations of use in product labeling;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused by our products to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our gene therapy assets for which we receive marketing approval and could materially harm our business, financial condition, results of operations and prospects.

Our gene therapy approach utilizes vectors derived from viruses, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of GT-AADC for the treatment of AADC deficiency or our other potential gene therapy product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for GT-AADC or our other potential gene therapy product candidates.

Because gene therapy remains a novel technology, we face uncertainty as to whether gene therapy will gain the acceptance of the public or the medical community. Even if we obtain regulatory approval for our product candidates, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and healthcare payers of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, if approved, prescribing treatments that involve the use of our product candidates, if approved, in lieu of, or in addition to, existing treatments, if any, with which they are familiar and for which greater clinical data may be available. Even if a product candidate displays a favorable efficacy and safety profile in clinical trials and is ultimately approved, market acceptance of the product candidate will not be fully known until after it is commercialized. More restrictive government regulations or negative public opinion would have an adverse effect on our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any product candidates that receive regulatory approval. 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 other vectors. A significant negative development in any other gene therapy program or our failure to satisfy any post-marketing regulatory commitments and requirements to which we may become subject may adversely impact the commercial results and potential of our product candidates. 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 result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any gene therapy products for which we obtain marketing approval. Any of the foregoing could have a material adverse effect on our business, results of operations, financial condition and prospects.

The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our products candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

We expect the cost of a single administration of gene therapy products, including GT-AADC for the treatment of AADC deficiency, to be substantial. We expect that coverage and reimbursement by government and private payers will be essential for most patients to be able to afford these treatments. Accordingly, sales of any product candidates, if approved, will depend substantially, both domestically and abroad, on the extent to which the prices of such product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payers. Coverage and reimbursement by a third-party payer may depend upon several factors, including the third-party payer's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;

- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement for a product from third-party payers is a time-consuming and costly process that could require us to provide to the payer supporting scientific, clinical and cost-effectiveness data. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products, including potential one-time gene therapies, such as GT-AADC for the treatment of AADC deficiency. 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. The Medicare and Medicaid programs increasingly are used as models for how private payers and government payers develop their coverage and reimbursement policies. Currently, there is limited experience with Centers for Medicare and Medicaid Services, or CMS, coverage of gene therapy product. We cannot be assured that Medicare or Medicaid will cover our product candidates that may be approved or provide reimbursement at adequate levels to realize a sufficient return on our investment. Moreover, reimbursement agencies in the European Union 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, in some European countries, the authorities conduct a Health Technology Appraisal, or HTA, to assess the cost-effectiveness of the product (in the UK a HTA assessment is conducted by NICE) which may significantly affect the effective access to the market.

We may face competition from biosimilars approved through an abbreviated regulatory pathway or from separate full applications for approval.

Our gene therapy product candidates are regulated by the FDA as biologics under the Federal Food, Drug and Cosmetics Act, or FDCA, and the Public Health Service Act, or PHSA. Biologics require the submission of a BLA and approval by the FDA prior to being marketed in the United States. Historically, a biologic product approved under a BLA was not subject to the generic drug review and approval provisions of the FDCA. However, the Biologics Price Competition and Innovation Act of 2009, or BPCIA, created a regulatory pathway under the PHSA for the abbreviated approval of biological products that are demonstrated to be “biosimilar” or “interchangeable” with an FDA approved biological product. In order to meet the standard of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. Such biosimilars would reference biological products approved in the United States. The BPCIA establishes a period of 12 years of exclusivity for reference products. Any of our product candidates that may be approved under BLAs in the future could be reference products for biosimilar marketing applications. As a result, any of our product candidates that may receive regulatory approval may face competition from other biological products that receive regulatory approval pursuant to an abbreviated pathway, which may have a material adverse effect on our results of operations, business, financial condition or prospects.

In addition, another company could market a competing version of a biological product if the FDA approval of a full BLA for such product containing the sponsor’s own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency. In the European Union, another company could gain approval for a competing product based on a MAA with a complete independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Our rights to develop and commercialize GT-AADC and our other potential 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 GT-AADC for the treatment of AADC deficiency and our other potential gene therapy product candidates. In particular, we have in-licensed certain intellectual property rights and know-how from the National Taiwan University, or NTU, relevant to GT-AADC 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 GT-AADC for the treatment of AADC deficiency and our other potential 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. In addition, absent an amendment or additional agreement, we may not have the right to use intellectual property in-licensed for one of our programs for use in another program.

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 GT-AADC for the treatment of AADC deficiency, or the License Agreement, 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 GT-AADC 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 to GT-AADC 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.

The success of our gene therapy product candidates depends on our ability to attract, retain and motivate qualified personnel.

Because the field of gene therapies is new, we might face a shortage of skilled individuals with substantial gene therapy experience. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms, if at all, given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. 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. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives for our gene therapy product candidates and could harm our business, financial condition, results of operations and prospects.

Our ability to realize all of the anticipated benefits from our acquisition of Agilis is subject to risk.

Our ability to realize the anticipated benefits of our acquisition of Agilis is subject to general business risk, including risks related to:

- the rate and degree of market acceptance and clinical utility of GT-AADC for the treatment of AADC deficiency or our other potential gene therapy product candidates;
- our sales, marketing and distribution capabilities;
- doing business internationally;
- competition to GT-AADC for the treatment of AADC deficiency or our other potential gene therapy product candidates;
- pricing regulations and reimbursement practices in various jurisdictions;
- potential product liability lawsuits;
- our ability to comply with environmental, health and safety laws and regulations;
- our limited resources and opportunity costs;

- our ability to secure additional funds on favorable terms or at all;
- our ability to comply with obligations in our intellectual property licenses and funding arrangements with third parties;
- our intellectual property;
- our dependence on third-parties; and
- legislative and regulatory changes in the pharmaceutical industry or healthcare systems in various jurisdictions.

In addition, our ability to realize the anticipated benefits of our acquisition of Agilis is subject to additional risks and potential difficulties similar to the risks with respect to Translarna, Emflaza and our other product candidates and other risks relating to our business as set forth in our Form 10-K for the year ended December 31, 2017, our Form 10-Q for the period ended March 31, 2018 and our Form 10-Q for the period ended June 30, 2018.

Exhibit 99.3

Agilis Biotherapeutics, Inc.

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Independent Auditor's Report

Board of Directors
Agilis Biotherapeutics, Inc.
Lynnfield, Massachusetts

We have audited the accompanying consolidated financial statements of Agilis Biotherapeutics, Inc., which comprise the consolidated balance sheets as of December 31, 2017 and 2016, and the related consolidated statements of operations and comprehensive loss, changes in stockholders' deficit, and cash flows for the years then ended, and the related notes to the consolidated financial statements.

Management's Responsibility for the Financial Statements

Management is responsible for the preparation and fair presentation of these consolidated financial statements in accordance with accounting principles generally accepted in the United States of America; this includes the design, implementation, and maintenance of internal control relevant to the preparation and fair presentation of consolidated financial statements that are free from material misstatement, whether due to fraud or error.

Auditor's Responsibility

Our responsibility is to express an opinion on these consolidated financial statements based on our audits. We conducted our audits in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the consolidated financial statements. The procedures selected depend on the auditor's judgment, including the assessment of the risks of material misstatement of the consolidated financial statements, whether due to fraud or error. In making those risk assessments, the auditor considers internal control relevant to the entity's preparation and fair presentation of the consolidated financial statements in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the entity's internal control. Accordingly, we express no such opinion. An audit also includes evaluating the appropriateness of accounting policies used and the reasonableness of significant accounting estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements.

We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our audit opinion.

Opinion

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of Agilis Biotherapeutics, Inc. as of December 31, 2017 and 2016, and the results of its operations and its cash flows for the years then ended in accordance with accounting principles generally accepted in the United States of America.

Emphasis of Matter Regarding Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As described in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations and has an accumulated deficit that raise substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Our opinion is not modified with respect to this matter.

/s/ BDO USA, LLP

Boston, Massachusetts
August 21, 2018

Agilis Biotherapeutics, Inc.
Consolidated Balance Sheets
(in thousands, except share, unit, and per share amounts)

	December 31,		June 30,
	2016	2017	2018
			(unaudited)
Assets			
Current assets:			
Cash and cash equivalents	$ 2,076	$ 12,197	$ 11,829
Prepaid expenses and other current assets	128	86	133
Total current assets	2,204	12,283	11,962
Property and equipment, net	6	74	159
Other assets	7	29	38
Total assets	2,217	12,386	12,159
Liabilities, Redeemable Convertible Preferred Units and Stock and Stockholders' Deficit			
Current liabilities:			
Accounts payable	$ 198	$ 310	$ 739
Accrued expenses	602	955	3,074
Deferred sponsored research	479	155	461
Convertible notes, including accrued interest and conversion discount	6,627	—	—
Total current liabilities	7,906	1,420	4,274
Warrants for the purchase of Series B Preferred shares subject to redemption	—	11,276	7
Total liabilities	7,906	12,696	4,281
Commitments and contingencies (Note 16)			
Redeemable convertible preferred units and stock:			
Series A preferred units, 99,875 units issued and outstanding at December 31, 2016	7,994	—	—
Redeemable convertible preferred stock (Series A and B), $0.001 par value; 23,844,412 shares authorized, 19,579,024 and 23,602,275 shares issued and outstanding as of December 31, 2017 and June 30, 2018, respectively, aggregate liquidation preference of $32,619,000 and $40,666,000 as of December 31, 2017 and June 30, 2018, respectively	—	24,569	41,874
Total redeemable convertible preferred units and stock	7,994	24,569	41,874
Stockholders' deficit:			
Common units, no par value: 38,209 units issued and outstanding as of December 31, 2016	—	—	—
Common stock, $0.001 par value; 36,542,218 shares authorized; 2,661,803 shares issued and outstanding as of December 31, 2017 and June 30, 2018, respectively	—	3	3
Additional paid-in capital	313	3,448	3,779
Accumulated deficit	(13,996)	(28,330)	(37,778)
Total stockholders' deficit	(13,683)	(24,879)	(33,996)
Total liabilities, redeemable convertible preferred units and stock and stockholders' deficit	$ 2,217	$ 12,386	$ 12,159

See accompanying notes.

Agilis Biotherapeutics, Inc.
Consolidated Statements of Operations and Comprehensive Loss
(in thousands)

	Year Ended December 31,		Six Months Ended June 30,	
	2016	2017	2017	2018
			(unaudited)	
Operating expenses:				
Research and development	$ 3,538	$ 4,906	$ 1,836	$ 6,782
General and administrative	1,303	2,445	803	4,745
Total operating expenses	4,841	7,351	2,639	11,527
Loss from operations	(4,841)	(7,351)	(2,639)	(11,527)
Other income (expense):				
Interest income	1	41	1	67
Interest expense	(302)	(34)	(34)	—
Change in fair value of warrants	—	(6,519)	23	2,012
Change in fair value of convertible notes	(1,325)	(8)	(8)	—
Other, net	(28)	—	—	—
Total other income (expense), net	(1,654)	(6,520)	(18)	2,079
Loss before equity losses in unconsolidated entities	(6,495)	(13,871)	(2,657)	(9,448)
Share of loss in joint venture	—	(463)	—	—
Net loss and comprehensive loss	$ (6,495)	$ (14,334)	$ (2,657)	$ (9,448)

See accompanying notes.

Agilis Biotherapeutics, Inc.
Consolidated Statements of Redeemable Convertible Preferred Units and Stock and Stockholders' Deficit
(in thousands, except share and unit amounts)

	Series A Redeemable Convertible Preferred Units		Redeemable Convertible Stock		Common Units		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount			
Balances as of December 31, 2015	99,875	$ 7,994	—	$ —	38,053	$ —	—	$ —	$ 228	$ (7,501)	$ (7,273)
Issuance of restricted profit units	—	—	—	—	1,724	—	—	—	—	—	—
Cancellation of unvested units	—	—	—	—	(1,568)	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	85	—	85
Net loss	—	—	—	—	—	—	—	—	—	(6,495)	(6,495)
Balances as of December 31, 2016	99,875	7,994	—	—	38,209	—	—	—	313	(13,996)	(13,683)
Cancellation of unvested units	—	—	—	—	(1,495)	—	—	—	—	—	—
Conversion of LLC units	(99,875)	(7,994)	7,244,412	7,994	(36,714)	—	2,661,803	3	(3)	—	—
Issuance of Series B convertible preferred stock, net of issuance costs of $309	—	—	8,999,999	9,906	—	—	—	—	3,029	—	3,029
Conversion of Notes Payable to Series B convertible preferred stock	—	—	3,334,613	6,669	—	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	109	—	109
Net loss	—	—	—	—	—	—	—	—	—	(14,334)	(14,334)
Balances as of December 31, 2017	—	—	19,579,024	24,569	—	—	2,661,803	3	3,448	(28,330)	(24,879)
Issuance of Series B preferred stock from exercise of Series B warrants	—	—	4,023,251	17,305	—	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—	—	—	331	—	331
Net loss	—	—	—	—	—	—	—	—	—	(9,448)	(9,448)
Balances as of June 30, 2018 (unaudited)	—	$ —	23,602,275	$ 41,874	—	$ —	2,661,803	$ 3	$ 3,779	$(37,778)	$ (33,996)

See accompanying notes.

Agilis Biotherapeutics, Inc.
Consolidated Statements of Cash Flows
(in thousands)

	Year Ended December 31,		Six Months Ended June 30,	
	2016	2017	2017	2018
			(unaudited)	
Cash flows from operating activities:				
Net loss	$ (6,495)	$ (14,334)	$ (2,657)	$ (9,448)
Adjustments to reconcile net loss to net cash used in operating activities:				
Stock-based compensation expense	85	109	14	331
Depreciation expense	2	6	1	19
Non-cash interest expense	302	34	34	—
Change in fair value of convertible notes	1,325	8	8	—
Share of loss in joint venture	—	463	—	—
Change in fair value of warrant liability	—	6,519	(23)	(2,012)
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	(93)	42	76	(47)
Other assets	(1)	(22)	(22)	(9)
Accounts payable	116	113	188	429
Accrued expenses	244	353	(258)	2,119
Deferred sponsored research	479	(324)	(116)	306
Net cash used in operating activities	(4,036)	(7,033)	(2,755)	(8,312)
Cash flows from investing activities:				
Purchases of property and equipment	(3)	(74)	(13)	(104)
Investment in joint venture	—	(463)	—	—
Net cash used in investing activities	(3)	(537)	(13)	(104)
Cash flows from financing activities:				
Proceeds from convertible notes	5,000	—	—	—
Proceeds from sale of Series B preferred stock, net of issuance costs	—	17,691	11,718	—
Proceeds from exercise of warrants for Series B preferred stock	—	—	—	17,305
Net cash provided by financing activities	5,000	17,691	11,718	17,305
Net increase (decrease) in cash and cash equivalents	961	10,121	8,950	8,889
Cash and cash equivalents at beginning of period	1,115	2,076	2,076	12,197
Cash and cash equivalents at end of period	$ 2,076	$ 12,197	$ 11,026	$ 21,086
Supplemental disclosure of non-cash investing and financing activities:				
Issuance of warrants in connection with issuance of Series B preferred	$ —	$ 4,757	$ 1,785	$ —
Conversion of LLC units into Series A Preferred	$ —	$ 7,994	$ 7,994	$ —
Conversion of LLC units into Common Stock	$ —	$ 3	$ 3	$ —
Conversion of convertible notes and accrued interest into Series B preferred	$ —	$ 6,669	$ 6,669	$ —
Value of warrants above exercise proceeds	$ —	$ —	$ —	$ 9,257
Beneficial conversion feature	$ —	$ 3,029	$ —	$ —

See accompanying notes.

1. Organization and Basis of Presentation

Agilis, LLC was formed in October 2013 as a limited liability company in the state of Delaware and, in February 2017, converted to a C corporation named Agilis Biotherapeutics, Inc. (collectively, with its wholly-owned subsidiaries, the "Company"). The Company is advancing innovative DNA therapeutics designed to provide long-term efficacy for patients with debilitating, often fatal, rare genetic diseases that affect the central nervous system.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and the ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize revenue from its product candidates.

The Company has funded its operations primarily with proceeds from the sale of its capital stock. The Company has incurred recurring losses since its inception, including net losses of $6,495, $14,334 and $9,448 for the years ended December 31, 2016 and 2017 and the six months ended June 30, 2018 (unaudited), respectively. In addition, as of December 31, 2017 and June 30, 2018 (unaudited), the Company had an accumulated deficit of $28,330 and $37,778, respectively. The Company expects to continue to generate operating losses for the foreseeable future. The future viability of the Company beyond that point is dependent on its ability to raise additional capital to finance its operations. The Company's inability to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies. There can be no assurances, however, that the Company's operating plans will be achieved or that additional funding will be available on terms acceptable to the Company, or at all. The Company expects that its cash and cash equivalents on hand as of June 30, 2018 (unaudited) of $11,829, plus the $10,000 received for the note payable issued to PTC Therapeutics, Inc, ("PTC"), as described below, will be sufficient to fund its operations and capital expenditure requirements into the first quarter of 2019.

These factors raise substantial doubt about the Company's ability to continue as a going concern. The Company's financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. These financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts or classifications of liabilities that might be necessary should the Company be unable to continue as a going concern.

On July 19, 2018, the Company entered into a merger agreement which provides for PTC to acquire the Company through the merger of the Company with a subsidiary of PTC, with the Company surviving the merger as a wholly owned, indirect subsidiary of PTC. The completion of the transaction is subject to customary closing conditions. In accordance with the terms of the merger agreement, PTC loaned the Company $10,000 under the Bridge Loan and Security Agreement between PTC and the Company, dated as of July 19, 2018, at a fixed interest rate of 3% per annum until maturity on July 19, 2020, at which time all principal and accrued unpaid interest becomes due and payable. Additionally, if the merger does not take place before September 2, 2018, PTC is required to loan the Company an additional $10,000. The Company has the option to prepay all or any amount of the loan at any time without any penalty or premium. Upon closing of the proposed merger, the Company must repay all outstanding term loans plus unpaid interest thereon, plus any other sums that have then become due and payable under the Bridge Loan and Security Agreement.

Basis of Presentation

The accompanying consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP").

Unaudited Interim Financial Information

The accompanying consolidated balance sheet as of June 30, 2018, the consolidated statements of operations and comprehensive loss and of cash flows for the six months ended June 30, 2017 and 2018, and the consolidated statement of convertible preferred units and stock and stockholders' deficit for the six months ended June 30, 2018 are unaudited. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual consolidated financial statements and, in the opinion of management, reflect all adjustments, which

include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of June 30, 2018 and the results of its operations and its cash flows for the six months ended June 30, 2017 and 2018. The financial data and other information disclosed in these notes related to the six months ended June 30, 2017 and 2018 are also unaudited. The results for the six months ended June 30, 2018 are not necessarily indicative of results to be expected for the year ending December 31, 2018, any other interim periods, or any future year or period.

2. Summary of Significant Accounting Policies

Principles of Consolidation

The consolidated financial statements include the accounts of Agilis Biotherapeutics, Inc. and its wholly owned subsidiaries Agilis AS, LLC, Agilis FA, LLC and Agilis RLN, all of which are Delaware limited liability companies. All intercompany transactions and balances have been eliminated.

Use of Estimates

The preparation of the Company's consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, expenses and related disclosure of contingent assets and liabilities at the date of the consolidated financial statements and reported amounts of expenses during the reporting period. On an ongoing basis, the Company's management evaluates its estimates, which include but are not limited to management's judgments of accrued expenses, fair value of common stock, valuation of share-based awards, fair value of warrants, fair value of convertible promissory notes and income taxes. Actual results could differ from those estimates.

Comprehensive Income (Loss)

Comprehensive loss includes net loss as well as other changes in shareholders' equity (deficit) that result from transactions and economic events other than those with shareholders. There was no difference between net loss and comprehensive loss for each of the periods presented in the accompanying consolidated financial statements.

Cash and Cash Equivalents

The Company considers all highly liquid investments with an original maturity of three months or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in money market mutual funds. Cash equivalents are stated at fair market value.

Concentration of Credit Risk and Off-Balance Sheet Risk

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. The Company maintains its cash and cash equivalents at a single accredited financial institution, in amounts that exceed federally insured limits. The Company generally invests its excess cash in money market funds that are subject to minimal credit and market risk.

The Company has no significant off-balance sheet risk such as foreign exchange contracts, option contracts, or other foreign hedging arrangements.

Significant Suppliers

The Company is dependent on third-party manufacturers to supply products for research and development activities of its programs, including preclinical and clinical testing. In particular, the Company relies and expects to continue to rely on a single manufacturer of its product candidates for sale upon approval and for use in clinical trials. The Company would be adversely affected by a significant interruption in the supply of product for sale or for use in clinical programs.

Fair Value Measurements

Fair value is defined as the price that would be received upon sale of an asset or paid to transfer a liability between market participants at measurement dates. Accounting Standards Codification ("ASC") Topic 820, Fair Value Measurement ("ASC 820"), establishes a six-level valuation hierarchy for instruments measured at fair value. The hierarchy is based on the transparency of inputs to the valuation of an asset or liability as of the measurement date. The hierarchy defines three levels of valuation inputs, of which the first two are considered observable and the last is considered unobservable:

(Amounts in thousands, except share, unit, per share, and per unit amounts)

Level 1	Quoted prices in active markets for identical assets or liabilities.
Level 2	Inputs other than quoted prices included within Level 1 that are either directly or indirectly observable, such as quoted market prices, interest rates and yield curves.
Level 3	Unobservable inputs developed using estimates or assumptions developed by the Company, which reflect those that a market participant would use in pricing the asset or liability.

To the extent that the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

The Company's warrant liability and convertible notes are carried at fair value, determined according to Level 3 inputs in the fair value hierarchy described above. In 2017, in connection with the Company's issuance and sale of Series B convertible preferred stock, all of the outstanding principal and accrued interest under the convertible notes was automatically converted into shares of Series B convertible preferred stock and the convertible notes liability was extinguished (see Notes 4, 8 and 10).

The carrying values of other current assets, accounts payable, and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

Property and Equipment

Property and equipment consists of computer equipment, software and leasehold improvements recorded at cost. These amounts are depreciated using the straight-line method over the estimated useful lives of the assets as follows:

	Estimated Useful Life
Computer equipment	5 years
Furniture and office equipment	7 years
Leasehold improvements	Shorter of estimated useful life or remaining lease term

Upon retirement or sale, the cost of the assets disposed of and the related accumulated depreciation are eliminated from the balance sheet and related gains or losses are reflected in the consolidated statements of operations and comprehensive loss.

Impairment of Long-Lived Assets

The Company continually evaluates long-lived assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparing the book values of the assets to the expected future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book values of the assets exceed their fair value. The Company did not recognize any impairment losses for the years ended December 31, 2016 and 2017 or the six months ended June 30, 2017 and 2018 (unaudited).

Warrant Liability

The Company classifies warrants for the purchase of shares of its series B convertible preferred stock (the "Series B Preferred") (see Note 9) as a liability on its consolidated balance sheets (included in long-term liabilities) as these warrants are freestanding financial instruments that may require the Company to transfer assets upon exercise (the "Series B Warrants"). The liability for warrants is initially recorded at fair value upon the date of their issuance and then subsequently remeasured to fair value at each reporting date. Changes in the fair value of the warrant liability are recognized as a component of other income (expense), net in the consolidated statements of operations and comprehensive loss. In February and March 2018, all but warrants to purchase 436 shares of Series B Preferred were exercised. The Company recorded other expense of $6,519 and other income of $23, and $2,012 in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2017 and the six months ended June 30, 2017 and 2018, respectively, for the revaluation of the Series B Warrants.

The Company utilized the Black-Scholes option pricing model, which incorporates assumptions and estimates, to value these warrants. The Company assesses these assumptions and estimates on a quarterly basis as additional information impacting the assumptions is obtained. Estimates and assumptions impacting the fair value measurement include the fair value per share of the underlying redeemable convertible preferred stock issuable upon exercise of the warrant, remaining contractual term of the warrants, risk-free interest rate, expected dividend yield and expected volatility of the value of the underlying convertible preferred stock.

Fair Value Option for Convertible Notes

As permitted under ASC 825, *Financial Instruments*, ("ASC 825"), the Company elected the fair value option to account for its convertible notes issued during 2016 (see Note 8). The Company recorded the convertible notes at fair value and subsequently remeasured them to fair value at each reporting date. Changes in fair value were recognized as a component of other income (expense), net in the consolidated statements of operations and comprehensive loss. As a result of applying the fair value option, direct costs and fees related to the convertible notes were recognized in earnings as incurred and were not deferred. In February 2017, in connection with the Company's issuance and sale of Series B Preferred, all of the outstanding principal and accrued interest under the convertible notes was automatically converted into shares of Series B Preferred and Series B Warrants (see Notes 8, 9 and 10). The Company recorded other expense of $1,325, $8, and $8 for changes in the fair value of the convertible notes in the consolidated statements of operations and comprehensive loss for the years ended December 31, 2016 and 2017 and the six months ended June 30, 2017 (unaudited), respectively.

Research and Development Costs and Accruals

Research and development expenses include salaries and benefits, materials and supplies, preclinical and clinical trial expenses, manufacturing expenses, stock-based compensation expense, depreciation of equipment, contract services and other outside expenses. The Company has entered into various research and development-related contracts with research institutions, contract research organization, contract manufacturers and other companies. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. Costs of certain development activities, such as manufacturing, pre-clinical and clinical trial expenses, are recognized based on an evaluation of the progress to completion of specific tasks. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the consolidated financial statements as prepaid or accrued research and development costs. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed. Costs incurred in obtaining technology licenses are charged to research and development expenses as acquired in-process research and development if the technology licensed has not reached technological feasibility and has no alternative future use.

Stock-Based Compensation

The Company accounts for its stock-based compensation in accordance with ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all share-based payments to employees and directors to be recognized as expense in the consolidated statements of operations and comprehensive loss based on their grant date fair values. The Company accounts for share-based payments to non-employees in accordance with ASC Topic 505, *Equity-Based Payments to Non-Employees* ("ASC 505"). Since the Company's non-employee awards do not contain performance commitments, ASC 505 requires that the expense be recognized in the consolidated statements of operations and comprehensive loss based on the awards' vesting date fair values. The Company estimates the fair value of options granted using the Black-Scholes option pricing model for stock option grants to both employees and non-employees. The Company believes the fair value of the stock options granted to non-employees is more reliably determinable than the fair value of the services provided.

The Black-Scholes option pricing model requires inputs based on certain subjective assumptions, including (a) the fair value of the underlying stock, (b) the expected stock price volatility, (c) the expected term of the award, (d) the risk-free interest rate and (e) expected dividends. Due to the lack of a public market for the Company's common stock and a lack of company-specific historical and implied volatility data, the Company has based its computation of expected volatility on the historical volatility of a representative group of public companies with similar characteristics to the Company, including stage of product development and life science industry focus. The historical volatility is calculated based on a period of time commensurate with the expected term assumption. The

Company uses the simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term for options granted to employees as it does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. The expected term is applied to the stock option grant group as a whole, as the Company does not expect substantially different exercise or post-vesting termination behavior among its employee population. For options granted to non-employees, the Company utilizes the contractual term of the share-based payment as the basis for the expected term assumption. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected term of the stock options. The expected dividend yield is assumed to be zero as the Company has never paid dividends and has no current plans to pay any dividends on its common stock. The Company records forfeitures as they occur in accordance with ASU 2016-09 *Improvements to Employee Share-Based Payment Accounting*.

There are significant judgments and estimates inherent in the determination of the fair value of the Company's common stock. These estimates and assumptions include a number of objective and subjective factors, including external market conditions, the prices at which the Company sold shares of preferred stock, the superior rights and preferences of securities senior to its common stock at the time of, and the likelihood of, achieving a liquidity event, such as an initial public offering or sale.

The Company expenses the fair value of its share-based compensation awards to employees on a straight-line basis over the requisite service period, which is generally the vesting period. Stock-based compensation awards to non-employees are adjusted through stock-based compensation expense at each reporting period end to reflect the current fair value of such awards and are expensed on a straight-line basis.

Profits Interests

From inception through February 14, 2017, the Company was a Delaware limited liability company. During this time, employees, directors, and consultants were granted profits interests. Profits interests are common units that are subject to vesting and are classified as equity awards for accounting purposes. Profits interests are considered issued and outstanding when granted. Stock-based compensation expense is recognized based on the fair value on the grant date and is recognized over the period of vesting. The Company does not have an obligation to repurchase any vested or unvested profits interests upon termination of the relationship with a holder of profits interests.

Equity Method Investments

Investments in non-public companies in which the Company owns less than a 50% equity interest and where it exercises significant influence over the operating and financial policies of the investee are accounted for using the equity method of accounting. The Company's proportionate share of the net income or loss of the equity method investment is shown as a separate line after other income (expense) in the consolidated statements of operations and comprehensive loss and results in a corresponding adjustment to the carrying value of the investment on the consolidated balance sheets. Dividends received reduce the carrying value of the investment. The Company periodically reviews the carrying value of its investment to determine if there has been an other-than-temporary decline in carrying value. A variety of factors are considered when determining if a decline in carrying value is other than temporary, including, among other factors, the financial condition and business prospects of the investee as well as the Company's intent with regard to the investment.

Income Taxes

From inception through February 14, 2017, the Company was a Delaware limited liability company for federal and state tax purposes and, therefore, all items of income or loss through February 14, 2017 flowed through to the members of the limited liability company. Effective February 14, 2017, the Company converted from an LLC to a C corporation for federal and state income tax purposes. Accordingly, prior to the conversion to a C corporation, the Company did not record deferred tax assets or liabilities or have net operating loss carryforwards. The Company accounts for income taxes using the asset and liability method in accordance with ASC Topic 740, *Income Taxes* ("ASC 740"), which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred tax assets and liabilities are determined on the basis of the differences between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a

portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies. At December 31, 2017 and June 30, 2018 (unaudited), the Company has concluded that a full valuation allowance is necessary for its deferred tax assets (see Note 13).

Recently Adopted Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board ("FASB") issued ASU No. 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern* (Subtopic 205-40) ("ASU 2014-15"). The amendments in this update explicitly require a company's management to assess an entity's ability to continue as a going concern and to provide related footnote disclosures in certain circumstances. The new standard is effective in the first annual period ending after December 15, 2016. The Company adopted ASU 2014-15 as of the required effective date of December 31, 2016. This guidance relates to footnote disclosure only (see Note 1), and its adoption had no impact on the Company's financial position, results of operations or cash flows.

In April 2015, the FASB issued ASU No. 2015-03, *Simplifying the Presentation of Debt Issuance Costs* ("ASU 2015-03"), which requires that debt issuance costs related to a debt liability be presented in the balance sheet as a direct reduction in the carrying amount of that debt liability. The amendments in ASU 2015-03 are effective for the annual periods ending after December 15, 2015. The Company adopted the standard retrospectively to all periods presented on the required effective date of January 1, 2016, and its adoption had no impact on the Company's financial position, results of operations or cash flows.

In November 2015, the FASB issued ASU No. 2015-17, *Balance Sheet Classification of Deferred Taxes* ("ASU 2015-17"), which simplifies the presentation of deferred income taxes by eliminating the need for entities to separate deferred income tax liabilities and assets into current and noncurrent amounts in a classified statement of financial position. For non-public entities, the guidance in this ASU is effective for annual periods beginning after December 15, 2017 and interim periods within annual periods beginning after December 15, 2018. Earlier application is permitted for all entities as of the beginning of an interim or an annual reporting period. The Company prospectively adopted this ASU as of February 14, 2017. The adoption of ASU 2015-17 did not have a material impact on the Company's consolidated balance sheets.

In March 2016, the FASB issued ASU No. 2016-09, *Improvements to Employee Share-Based Payment Accounting* ("ASU 2016-09"). The new standard involves several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities and classification on the consolidated statement of cash flows. Certain of these changes are required to be applied retrospectively, while other changes are required to be applied prospectively. The Company early adopted ASU 2016-09 effective January 1, 2016, and its adoption of ASU 2016-09 had no material impact on the Company's financial position, results of operations or cash flows.

In August 2016, the FASB issued ASU No. 2016-15, *Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments* ("ASU 2016-15"). This guidance addresses the presentation and classification of certain cash receipts and cash payments in the statement of cash flows. The standard is effective for annual periods beginning after December 15, 2017 and for interim periods within those fiscal years. Early adoption is permitted. The Company early adopted ASU 2016-15 effective January 1, 2016, and adoption of ASU 2016-15 did not have a material impact on the Company's consolidated financial statements.

In May 2017, the FASB issued ASU No. 2017-09, *Compensation-Stock Compensation (Topic 718): Scope of Modification Accounting* ("ASU 2017-09"). This update clarifies the changes to terms or conditions of a share-based payment award that require an entity to apply modification accounting. ASU 2017-09 is effective for annual reporting periods, and interim periods therein, beginning after December 15, 2017. Early application is permitted and prospective application is required. The Company early adopted ASU 2017-09 effective January 1, 2016, and adoption of this guidance did not have a significant impact on the Company's consolidated financial statements.

Recently Issued Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies and adopted by the Company as of the specified effective date. Unless otherwise discussed, the Company believes

that the impact of recently issued standards that are not yet effective will not have a material impact on its consolidated financial position or results of operations upon adoption.

In February 2016, the FASB issued ASU No. 2016-02, *Leases* (Topic 842) ("ASU 2016-02"). The new standard aims to increase transparency and comparability among organizations by requiring lessees to recognize lease assets and lease liabilities on the balance sheet and requiring disclosure of key information about leasing arrangements. ASU 2016-02 is effective for public entities for annual periods beginning after December 15, 2018 and for interim periods within those fiscal years. For all other entities, the guidance is effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. Early adoption is permitted for all entities. The Company is currently evaluating the potential impact that ASU 2016-02 may have on its financial position and results of operations.

In June 2018, the FASB issued ASU 2018-07, *Improvements to Nonemployee Share-Based Payment Accounting ("ASU 2018-07").* The new standard simplifies the accounting for share-based payments to nonemployees by aligning it with the accounting for share-based payments to employees, with certain exceptions. The new standard will be effective beginning January 1, 2019 and early adoption is permitted. The Company is currently evaluating the potential impact ASU 2018-07 may have on its results of operations upon adoption, but does not have any current options granted to non-employees and does not plan on granting options to non-employees in the near future, and does not expect this to have an impact on its consolidated financial statements.

3. Joint Venture

In July 2016, the Company entered into a memorandum of understanding ("MOU") with a Japanese company (the "JV Partner") to develop and commercialize gene therapy products. The initial focus was on forming a joint venture (the "JV") to develop and manufacture gene therapy vectors, to operate a GMP – compliant facility in Japan to manufacture gene therapy vectors and to commercialize gene therapy vectors for the treatment of AADC and Parkinson's disease. The JV was formalized in July 2017 and is owned approximately 50% by each of the Company and the JV Partner. The board of directors of the JV consists of two representatives from each of the Company and the JV Partner. The Company and the JV Partner each contributed JPY 50,000 ($444 at December 31, 2017) to the JV. The Company evaluated its investment in the JV and determined consolidation was not appropriate because it is unable to exert control over the JV. The Company's investment in the JV permits it to exert significant influence and therefore the Company accounts for its investment under the equity method.

Concurrent with the formalization of the JV in 2017, the Company entered into a manufacturing and collaboration agreement (the "Manufacturing Agreement") with the JV Partner which provides for the governance and operations and management as well as the primary activities of the JV. The terms of the Manufacturing Agreement provide for the JV Partner to be responsible for construction and development of the manufacturing facility and for securing all regulatory approvals. Further, the JV Partner has entered into a line of credit with the JV to provide loans of up to JPY 350,000 (approximately $3,500 at December 31, 2017, based upon the exchange rate at such time) through June 1, 2020. The Manufacturing Agreement also contemplates that the Company and the JV Partner will enter into a commercialization agreement which will provide each of the parties' rights to commercialize gene therapy products for the treatment of AADC and Parkinson's Disease in certain territories in exchange for future royalties. The detailed terms of the commercial relationship will be further articulated in a commercialization agreement to be negotiated and executed at a future date.

The Company has accounted for its investment in the JV under the equity method and has included its proportionate interest in the net loss of the JV of $463 for the year ended December 31, 2017 in other expense in the consolidated statements of operations and comprehensive loss. For the six months ended June 30, 2018 (unaudited), the proportionate share of the loss is in excess of the investment amount; therefore, no loss was recorded.

(Amounts in thousands, except share, unit, per share, and per unit amounts)

4. Fair Value Measurements

The following tables present information about the Company's financial assets and liabilities that have been measured at fair value at December 31, 2016 and 2017 and June 30, 2018 (unaudited) and indicates the fair value hierarchy of the valuation inputs utilized to determine such fair value:

	Fair Value Measurements as of December 31, 2016 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents - money market funds	$ —	$ 1,300	$ —	$ 1,300
	$ —	$ 1,300	$ —	$ 1,300
Liabilities:				
Convertible notes	$ —	$ —	$ 6,627	$ 6,627
	$ —	$ —	$ 6,627	$ 6,627

	Fair Value Measurements as of December 31, 2017 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents - money market funds	$ —	$ 11,887	$ —	$ 11,887
	$ —	$ 11,887	$ —	$ 11,887
Liabilities:				
Warrants for the purchase of shares subject to redemption	$ —	$ —	$ 11,276	$ 11,276
	$ —	$ —	$ 11,276	$ 11,276

	Fair Value Measurements as of June 30, 2018 (unaudited) Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents - money market funds	$ —	$ 11,026	$ —	$ 11,026
	$ —	$ 11,026	$ —	$ 11,026
Liabilities:				
Warrants for the purchase of shares subject to redemption	$ —	$ —	$ 7	$ 7
	$ —	$ —	$ 7	$ 7

At December 31, 2016 and 2017 and June 30, 2018 (unaudited), all of the Company's cash equivalents comprised money market funds.

At December 31, 2017 and June 30, 2018 (unaudited), the Company's warrants for the purchase of shares subject to redemption were the only financial instruments classified as Level 3.

There have been no changes to the valuation methods used during the years ended December 31, 2016 and 2017 and the six months ended June 30, 2018 (unaudited). There were no transfers within the fair value hierarchy during the years ended December 31, 2016 and 2017 or the six months ended June 30, 2018 (unaudited).

The carrying amounts reflected in the consolidated balance sheets for cash and cash equivalents, prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values.

Valuation of Warrant Liability

(Amounts in thousands, except share, unit, per share, and per unit amounts)

The warrant liability is related to the warrants to purchase shares of Series B Preferred (see Note 10). The fair value of the warrant liability was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy.

The Company used the Black-Scholes option-pricing model, which incorporates assumptions and estimates, to value the Series B Warrants. Estimates and assumptions impacting the fair value measurement include the fair value per share of the underlying shares of Series B Preferred, the remaining contractual term of the warrants, the risk-free interest rate, the expected dividend yield and the expected volatility of the price of the underlying preferred stock. The Company determined the fair value per share of the underlying preferred stock by taking into consideration the most recent sales of its preferred stock, results obtained from third-party valuations and additional factors that are deemed relevant. The Company is a private company and lacks company-specific historical and implied volatility information of its stock. Therefore, it estimates its expected stock volatility based on the historical volatility of publicly traded peer companies for a term equal to the remaining contractual term of the warrant. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve for time periods approximately equal to the remaining contractual term of the warrant. The Company estimated a 0% expected dividend yield based on the fact that the Company has never paid or declared dividends and does not intend to do so in the foreseeable future.

The fair value of the Series B Warrants is remeasured at each reporting date using then-current assumptions with changes in fair value recorded to other income (expense), net in the consolidated statements of operations and comprehensive loss.

The following table presents the unobservable inputs of the warrant liability:

	Year Ended December 31,	Six Months Ended June 30, (unaudited)	
	2017	2017	2018
Risk-free interest rate	2.30% - 2.42%	2.30% - 2.42%	2.31% - 2.33%
Expected term (in years)	9.75 - 10.01	9.75 - 10.00	10.00 - 10.01
Expected volatility	76% - 79%	77% - 79%	76% - 79%
Expected dividend yield	0%	0%	0%

Valuation of Convertible Notes Liability

The Company elected the fair value option to account for its convertible notes issued during 2016 (see Note 8). The fair value of the convertible notes was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy.

The Company determined the fair value of the convertible notes based on the proceeds received for the convertible notes; the terms of the convertible notes, including the rate at which the notes convert into qualified equity financing securities; the probability and timing of a qualified equity financing; and the fair value of the underlying preferred stock. Estimates and assumptions impacting the fair value measurement include the probability of a qualified equity financing as defined in the convertible notes agreements, the expected timing of such event, and the then fair value of the Company's Series A member units or Series A Preferred Stock (the "Series A Preferred"). The Company estimated the probability and timing of the qualified equity financing based on management's assumptions and knowledge of specified events at issuance and as of each reporting date. The Company determined the fair value per share of the preferred stock as described above.

The following table provides a roll forward of the aggregate fair values of the Company's warrant liability and convertible notes liability, for which fair value is determined using Level 3 inputs:

(Amounts in thousands, except share, unit, per share, and per unit amounts)

	Warrant Liability	Convertible Notes
Balance as of December 31, 2015	$ —	$ —
Convertible notes issuance	—	5,000
Interest accrued	—	302
Change in fair value	—	1,325
Balance as of December 31, 2016	—	6,627
Interest accrued	—	34
Change in fair value	—	8
Extinguishment of converitble notes and accrued interest	—	(6,669)
Issuance of warrants	4,757	—
Change in fair value	6,519	—
Balance as of December 31, 2017	11,276	—
Change in fair value	(2,012)	—
Exercise of warrants	(9,257)	—
Balance as of June 30, 2018 (unaudited)	$ 7	$ —

5. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	December 31,		June 30,
	2016	2017	2018
			(unaudited)
Prepaid rent and deposits	$ 4	$ 33	$ 22
Prepaid health insurance	—	17	28
Prepaid marketing programs	—	19	75
Deferred issuance costs for Series B financing	112	—	—
Other	12	17	8
	$ 128	$ 86	$ 133

6. Property and Equipment, Net

Property and equipment includes the following:

	December 31,		June 30,
	2016	2017	2018
			(unaudited)
Computer equipment	$ 9	$ 30	$ 46
Furniture and office equipment	—	30	56
Leasehold improvements	—	23	85
	9	83	187
Less: Accumulated depreciation and amortization	(3)	(9)	(28)
	$ 6	$ 74	$ 159

The Company recognized $2, $6, $1 and $19 of depreciation expense for the years ended December 31, 2016 and 2017 and the six months ended June 30, 2017 and 2018 (unaudited), respectively.

7. Accrued Expenses

Accrued expenses consist of the following:

(Amounts in thousands, except share, unit, per share, and per unit amounts)

	December 31,		June 30,
	2016	2017	2018
			(unaudited)
Payroll and employee related expenses	$ 242	$ 404	$ 631
Professional fees	76	175	440
Third-party research and development expenses	279	324	1,778
Other	5	52	225
	$ 602	$ 955	$ 3,074

8. Convertible Notes to Shareholders

In February and September 2016, the Company issued convertible notes to shareholders for $2,600 and $2,400, respectively, ("the Convertible Notes"). The Convertible Notes bore interest at a rate of 8% per annum and were payable on January 31, 2018. The Convertible Notes were automatically convertible upon the consummation of a qualified equity financing with gross proceeds of at least $10,000 excluding proceeds from the convertible note issuances, into the class of shares to be issued to investors participating in the fundraising, at a conversion price per share equal to 80% of the price per share paid by such investors. In the event that a qualified equity financing did not take place by the maturity date, the notes and accrued interest thereon would convert into Series A member units at the fair market value of such units.

The Company elected the fair value option to account for the Convertible Notes. The Company recorded the Convertible Notes at fair value and subsequently remeasured them to fair value at each reporting date. Changes in fair value were recognized as a component of other income (expense), net in the consolidated statements of operations and comprehensive loss. As a result of applying the fair value option, direct costs and fees related to the Convertible Notes were recognized in earnings as incurred and were not deferred.

As of December 31, 2016, the outstanding Convertible Notes are shown on the accompanying consolidated balance sheets at the fair value of $6,627, including accrued interest. The consolidated statements of operations and comprehensive loss include other expense of $1,325, $8 and $8 for changes in the fair value of the Convertible Notes from the date of issuance through December 31, 2016 and 2017 and for the six months ended June 30, 2017 (unaudited), respectively.

On February 14, 2017, the Company completed a qualified financing of Series B Preferred and Series B Warrants (Notes 9 and 10). In connection with the Series B Preferred financing, all of the outstanding principal of the Convertible Notes and $336 of accrued interest were automatically converted into 3,334,613 shares of Series B Preferred Stock and warrants to purchase 1,167,114 shares of the Series B Preferred.

9. Warrants

As of December 31, 2017, outstanding Series B Warrants consisted of the following:

	December 31, 2017				
Date Issued	Number of Shares Issuable	Exercise Price	Issuance Date Value	Classification	Expiration
February 15, 2017	2,158,336	$2.00	$1,295	Liability	February 15, 2027
March 30, 2017	175,000	$2.00	$105	Liability	March 30, 2027
June 30, 2017	641,667	$2.00	$385	Liability	June 30, 2027
September 29, 2017	1,049,998	$2.00	$2,971	Liability	September 29, 2027
	4,025,001				

In connection with the issuances of Series B Preferred in 2017 (Note 10), the Company issued warrants to purchase an aggregate 4,025,001 shares of Series B Preferred for $2.00 per share on the dates shown above. The Series B Warrants expire ten years from the date of issuance and would vest only if the Company did not achieve a specified milestone by December 31, 2020. The Series B Warrants were recorded at their issuance date fair values on the consolidated balance sheets. The Series B Warrants were remeasured to their aggregate fair value of $11,276

as of December 31, 2017, and the Company recorded a loss of $6,519 for the change in fair value of the Series B Warrant liability during the year ended December 31, 2017.

On January 31, 2018, the terms of the Series B Warrants were amended to provide the holders with the election to (1) exercise the Series B Warrants immediately or (2) the warrants would be exercisable at any time for the duration of the original term except the number of shares would be reduced to 25% of the original shares. Warrant holders representing 4,023,251 Series B Warrants exercised their warrants, pursuant to which the Company received $8,048 in proceeds. Warrants to purchase 1,750 shares of Series B Preferred were amended to be exercisable for 436 shares of Series B Preferred. Immediately prior to their exercise, all warrants outstanding were remeasured to their aggregate fair value of $9,258 for a gain of $2,012. At June 30, 2018 (unaudited) there are warrants outstanding for the purchase of 436 shares of Series B Preferred at an exercise price of $2.00 per share. The fair value of these warrants at June 30, 2018 (unaudited) of $7 is shown as a liability on the consolidated balance sheet.

10. Convertible Preferred Stock

As of December 31, 2017 and June 30, 2018 (unaudited), the Company has authorized 23,844,412 shares of Preferred Stock (the "Preferred Stock") and has designated 7,244,412 shares as Series A Preferred and 16,600,000 shares as Series B Preferred. The Preferred Stock is classified outside of stockholders' equity as the shares contain certain redemption features that are not solely within the control of the Company.

As of each balance sheet date, Series A LLC Member Units and Preferred Stock consisted of the following (in thousands, except share amounts):

	December 31, 2016		
	LLC Units Outstanding	Carrying Value	Liquidation Preference
Series A LLC Member Units	99,875	$ 7,994	$ 8,000
	99,875	$ 7,994	$ 8,000

	December 31, 2017				
	Preferred Shares Designated	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A Preferred	7,244,412	7,244,412	$ 7,994	$ 7,950	7,244,412
Series B Preferred	16,600,000	12,334,612	$ 16,575	24,669	12,334,612
	23,844,412	19,579,024	$ 24,569	$ 32,619	19,579,024

	June 30, 2018 (unaudited)				
	Preferred Shares Designated	Preferred Shares Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion
Series A Preferred	7,244,412	7,244,412	$ 7,994	$ 7,950	7,244,412
Series B Preferred	16,600,000	16,357,863	33,880	32,716	16,357,863
	23,844,412	23,602,275	$ 41,874	$ 40,666	23,602,275

Issuance of Series A Preferred

In connection with the Conversion in February 2017 (Note 11), the Company issued 7,244,412 shares of Series A Preferred in exchange for all outstanding Series A Units.

Issuance of Series B Preferred and Warrants

In 2017, the Company issued 12,334,612 shares of Series B Preferred and 4,025,001 Series B Warrants (see Note 9). The combination of one share of Series B Preferred and 35% of one Series B Warrant was sold for $2.00.

The Company has allocated the $2.00 purchase price between the Series B Preferred and the Series B Warrants based upon the value of the Series B Warrants using the residual method (see Note 9). For the 3,000,002 shares of Series B Preferred issued in September 2017, the accounting conversion price of the Series B Preferred was $1.01 per share which is less than the fair value of the Common Stock into which the Series B Preferred was convertible. This represented a beneficial conversion of $3,029 in the aggregate. The beneficial conversion feature was recorded as a reduction to convertible preferred stock and an increase to additional paid in capital on the consolidated balance sheets.

On January 31, 2018, the terms of the Series B Warrants were amended to provide the holders with the election to (1) exercise the Series B Warrants immediately or (2) the warrant would be exercisable at any time for the duration of the original term except the number of shares would be reduced to 25% of the original shares. Warrant holders representing 4,023,501 Series B Warrants exercised their warrants in February and March 2018 and the Company received $8,048 in proceeds and warrants to purchase 1,750 shares of Series B Preferred were amended to be exercisable for 436 shares of Series B Preferred.

The Preferred Stock has the following rights and preferences:

Conversion

The Preferred Stock is convertible into Series A Common Stock at any time at the option of the holder, on a 1-for-1 basis, adjustable for certain dilutive events, and is subject to mandatory conversion upon the closing of a firm commitment underwritten public offering with proceeds of at least $60,000 and at least $8.00 per share.

Voting

The holders of the Preferred Stock have voting rights equivalent to the number of shares of Common Stock into which their shares convert.

Dividends

Holders of Preferred Stock are entitled to receive, before any cash is paid out or set aside for Common Stock, dividends at 6% of the Series A Preferred and Series B Preferred issuance price, subject to adjustment for any stock dividend, stock split, or other similar recapitalization affecting such class or series of capital stock. The dividends are non-cumulative and are payable only when and if declared by the Board of Directors of the Company.

Holders of shares of Series A Preferred and Series B Preferred are then entitled to receive, before or simultaneously with Common Stock, a dividend at least equal to the amount of dividends per share received by the Common Stock. No dividends have been declared since the Company’s inception.

Liquidation Preference

In the event of any voluntary or involuntary liquidation event, dissolution, winding up of the Company or upon the occurrence of certain events designated by a majority of the holders of the Preferred Stock to be a deemed liquidation event, proceeds would be distributed in the following order:

First, the holders of the Series B Preferred would receive an amount equal to their original issuance price plus declared but unpaid dividend;

Second, the holders of Series A Preferred would receive an amount equal to their original issuance price plus declared but unpaid dividend;

Third, an aggregate of $2,050 would be distributed pro rata on an as converted to common stock basis among the holders of Series B Preferred, Series A Preferred, Series A Common Stock and Series E Common Stock;

Fourth, an aggregate of $5,000 would be distributed pro rata on an as converted to common stock basis among the holders of Series B Preferred, Series A Preferred and Series A Common Stock, Series B Common Stock and Series E Common Stock;

Fifth, an aggregate of $5,000 would be distributed pro rata on an as converted to common stock basis among the holders of Series B Preferred, Series A Preferred and Series A Common Stock, Series B Common Stock, Series C Common Stock and Series E Common Stock; and

Sixth, all remaining proceeds shall be distributed pro rata on an as converted to common stock basis among the holders of Preferred Stock and common stock.

11. Stockholders' Deficit

Issuance of LLC Units

The Company was formed as an LLC in 2013 and issued 100,000 Series A Units to investors for $80.00 per unit. The Company also issued 31,625, 12,724 and 1,724 incentive units in the form of profit interests to employees, consultants and Company founders in 2013, 2014 and 2016, respectively. The profit interests issued to employees, scientific advisory board members and directors ("Employee Units") generally vest over four years, the profit interests issued to Company consultants ("Common Units") vest over three years, and the profits interests issued to Company founders vested immediately upon issuance in 2013. The value of the Employee and Common Units is recorded as an expense in the period the service was provided, and the profit interests vested.

At December 31, 2016, there were 13,209 Employee Units outstanding. The Employee Units had no voting rights, were non-transferable and shared in distributions and proceeds upon liquidation pro rata with Series A and Common Units once distributions to the Series A and Common Units met certain hurdle rates. The hurdle rate was defined as the fair market value of the Company on the date the Employee Unit was issued plus capital contributions received from Common Unit holders subsequent to the date the Employee Unit was issued.

At December 31, 2016, there were a total of 25,000 Common Units outstanding.

At December 31, 2016, there were a total of 99,875 Series A Units outstanding.

The holders of the Series A Units and the Common Units had the following rights and priorities:

Voting Rights

The holders of Common Units were entitled to vote as a single class with the holders of the Series A Units.

Distributions

Distributions to Common Unit holders were determined by a majority of the Company's managers. The Common Unit holders were not entitled to distributions until such time as cumulative distributions to the Series A Unit holders exceeded their aggregate capital contributions of $8,000. Thereafter, the Common Unit holders shared in distributions pro rata with the Series A Unit holders and the Employee Unit holders, after the Employee Unit hurdle amounts had been met. No distributions were made to the holders of Series A Units, Common Units or Employee Units.

Liquidation

In the event of a liquidation, dissolution, or winding-up of the Company, the assets of the Company were to be distributed in accordance with the same order of priority as distributions.

Conversion to a C-Corporation from an LLC

On February 14, 2017, in connection with the sale of Series B Preferred and in accordance with an agreement and plan of conversion, the Company issued common stock for all outstanding Employee and Common Units and issued Series A Preferred for all outstanding Series A Units based upon an exchange ratio of 72.5349 shares of common stock for each Employee and Common Unit and 72.5349 shares of Series A Preferred for each Series A Unit (the Conversion). In conjunction with the Conversion, the Company issued 2,661,803 shares of common stock consisting of 1,813,372 shares of Series A Common Stock, 809,192 shares of Series B Common Stock, 7,978 shares of Series C Common Stock, and 31,261 shares of Series D Common Stock and 7,244,412 shares of Series A Preferred.

Common Stock

The Company has authorized 36,542,218 shares of Common Stock and has designated 33,600,000 shares as Series A Common Stock, 809,193 shares as Series B Common Stock, 7,978 shares as Series C Common Stock, 125,047 shares as Series D Common Stock, and 2,000,000 shares as Series E Common Stock. The following shares were outstanding as of December 31, 2017 and June 30, 2018 (unaudited):

(Amounts in thousands, except share, unit, per share, and per unit amounts)

	December 31, 2017	June 30, 2018 (unaudited)
Series A	1,813,372	1,813,372
Series B	809,192	809,192
Series C	7,978	7,978
Series D	31,261	31,261
Series E	—	—
	2,661,803	2,661,803

Common Stock Liquidation

The primary difference between the various classes of common stock is their liquidation preference. The liquidation preference is consistent with the hurdle rate as LLC profits interests. The preferred stock and common stock share in proceeds from a voluntary or involuntary liquidation, dissolution or winding up of the Company in the following manner:

First, the holders of the Series B Preferred would receive an amount equal to their original issuance price plus declared but unpaid dividend;

Second, the holders of Series A Preferred would receive an amount equal to their original issuance price plus declared but unpaid dividend;

Third, an aggregate of $2,050 would be distributed pro rata on an as converted to Common Stock basis among the holders of Series B Preferred, Series A Preferred, Series A Common and Series E Common;

Fourth, an aggregate of $5,000 would be distributed pro rata on an as converted to Common Stock basis among the holders of Series B Preferred, Series A Preferred and Series A Common, Series B Common and Series E Common;

Fifth, an aggregate of $5,000 would be distributed pro rata on an as converted to Common Stock basis among the holders of Series B Preferred, Series A Preferred and Series A Common Stock, Series B Common Stock, Series C Common Stock and Series E Common Stock; and

Sixth, all remaining proceeds shall be distributed pro rata on an as converted to common stock basis among the holders of Preferred Stock and common stock.

The Series A Common Stock has voting rights, but all other remaining series of common stock are non-voting. Common stockholders are not entitled to receive dividends, unless declared by the Board of Directors.

12. Stock-Based Compensation

On February 15, 2017, the Company adopted the 2017 Long Term Incentive Plan (“2017 Plan”). All of the Company’s employees, officers, directors, and consultants are eligible to be granted options and restricted stock under the terms of the 2017 Plan. The Company reserved an aggregate of 1,527,252 shares for issuance under the 2017 Plan.

All stock option grants are non-statutory stock options except option grants to employees (including officers and directors) intended to qualify as incentive stock options under the Internal Revenue Code of 1986, as amended. Incentive stock options may not be granted at less than the fair market value of the Company’s common stock on the date of grant, as determined in good faith by the Board of Directors at its sole discretion. Nonqualified stock options may be granted at an exercise price established by the Board of Directors at its sole discretion (which has not been less than fair market value on the date of grant) and the vesting periods may vary. Vesting periods are generally four years and are determined by the Board of Directors. Stock options become exercisable as they vest. Options granted under the 2017 Plan expire no more than ten years from the date of grant.

Stock Options

A summary of the stock option activity under the 2017 Plan is as follows:

(Amounts in thousands, except share, unit, per share, and per unit amounts)

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Term (in years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2016	—	$ —	—	$ —
Granted	1,296,000			
Exercised	—			
Forfeited	—			
Outstanding as of December 31, 2017	1,296,000	$ 1.78	9.78	$ 1,009
Granted	95,000			
Forfeited	(14,167)			
Outstanding as of June 30, 2018 (unaudited)	1,376,833	$ 1.83	9.27	$ 1,009
Options exercisable as of December 31, 2017	54,687	$ 0.24	9.36	$ 127
Options exercisable as of June 30, 2018 (unaudited)	298,308	$ 1.27	9.00	$ 386
Options unvested as of December 31, 2017	1,241,313	$ 1.85	9.79	$ 882
Options unvested as of June 30, 2018 (unaudited)	1,078,525	$ 1.98	9.34	$ 623

Stock Option Valuation

The assumptions that the Company used to determine the grant-date fair value of stock options granted to employees and directors were as follows, presented on a weighted average basis:

	Year Ended December 31,	Six Months Ended June 30, (unaudited)	
	2017	2017	2018
Risk-free interest rate	1.88%	1.88%	2.55%
Expected term (in years)	5.40	5.80	5.61
Expected volatility	57.8%	58.7%	60.0%
Expected dividend yield	0%	0%	0%

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's Series E Common Stock for those stock options that had exercise prices lower than the fair value of the Company's common stock. There were no options exercised during the years ended December 31, 2016 and 2017 or the six months ended June 30, 2018 (unaudited).

The weighted-average fair value of options granted to employees during the year ended December 31, 2017 and the six months ended June 30, 2018 (unaudited) was $1.42 per share for both periods. There were no options granted during 2016.

The total fair value of options vested during the year ended December 31, 2017 and the six months ended June 30, 2018 (unaudited) was $7 and $201, respectively.

The Company utilizes significant estimates and assumptions in determining the fair value of its common stock. The Company has utilized various valuation methodologies in accordance with the framework of the American Institute of Certified Public Accountants Technical Practice Aid, Valuation of Privately-Held Company Equity Securities Issued as Compensation (the "Practice Aid"), to estimate the fair value of its common stock. Each valuation methodology includes estimates and assumptions that require the Company's judgment. These estimates and assumptions include a number of objective and subjective factors, including external market conditions, the prices at which the Company sold shares of preferred stock, the superior rights and preferences of securities senior to the Company's common stock at the time of, and the likelihood of, achieving a liquidity event, such as an initial public offering or sale. Significant changes to the key assumptions used in the valuations could result in different fair values of common stock at each valuation date.

(Amounts in thousands, except share, unit, per share, and per unit amounts)

Common and Employee Units and Restricted Common Stock

The Company issued Common and Employee Units (collectively, the "Profits Interests"), which were subject to vesting over three to four years. In conjunction with the Conversion, the Profits Interests were converted into shares of restricted Series B Common Stock subject to the same vesting schedule. The conversion was considered a modification of the awards, which did not create any incremental value and therefore had no impact on the accounting for the awards. If any of these individuals ceased to be employed or to provide services to the Company prior to vesting, the Company had the right to repurchase any unvested common profit interests at the price paid by the holder.

A summary of the activity related to unvested Profits Interests and unvested restricted Series B Common Stock for the year ended December 31, 2017 and the six months ended June 30, 2018 (unaudited) is presented below:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested Profits Interests units as of December 31, 2016	5,641	$ 54.15
Conversion adjustment to restricted common stock	403,528	
Cancelled	(73,063)	$ 9.67
Vested	(218,904)	$ 28.97
Unvested restricted Series B Common Stock as of December 31, 2017	117,202	$ 15.51
Vested	(82,608)	$ 10.93
Unvested restricted Series B Common Stock as of June 30, 2018 (unaudited)	34,594	$ 4.58

The total expense related to the unvested restricted stock awards during the years ended December 31, 2017 and the six months ended June 30, 2018 (unaudited), based on estimated fair values of the stock underlying the restricted stock awards on the grant date, was $22 and $11, respectively.

Stock-Based Compensation

Stock-based compensation expense included in the Company's consolidated statements of operations and comprehensive loss is as follows:

	Years Ended December 31,		Six Months Ended June 30, (unaudited)	
	2016	2017	2017	2018
			(unaudited)	
Research and development expenses	$ 16	$ 75	$ 6	$ 181
General and administrative expenses	69	34	8	150
	$ 85	$ 109	$ 14	$ 331

As of December 31, 2017 and June 30, 2018 (unaudited), total unrecognized stock-based compensation expense relating to unvested stock options was $1,196 and $990, respectively. This amount is expected to be recognized over a weighted-average period of 3.6 years and 3.1 years, respectively.

As of December 31, 2017 and June 30, 2018 (unaudited), total unrecognized stock-based compensation expense relating to unvested restricted stock awards was $117 and $3, respectively. This amount is expected to be recognized over a weighted-average period of 0.9 years and 2 months, respectively.

The expense related to restricted stock awards and stock option awards granted to employees and directors for their service on the board of directors was $28, $104, $12 and $330 for the years ended December 31, 2016 and 2017 and the six months ended June 30, 2017 and 2018 (unaudited), respectively. The expense related to restricted stock awards to non-employees was $57, $3, $2 and $1 for the years ended December 31, 2016 and 2017 and the six months ended June 30, 2017 and 2018 (unaudited), respectively.

13. Income Taxes

The Company operated as an LLC from inception until February 14, 2017, when it converted to a C-corporation and has incurred net operating losses ("NOLs") from its inception. As such, income and loss for the tax periods prior to February 14, 2017 were passed through to the Company's shareholders in the LLC. All income and loss after the conversion to a C-corporation on February 14, 2017 will be included in the Company's tax return. The Company has incurred a tax net operating loss for the period ended December 31, 2017 of approximately $7,200 for which it has recorded no tax benefit. The net operating loss is available to be carried forward to reduce future taxable income through 2037. The Company also has federal research and development and orphan drug tax credits earned in 2017 of $1,200 available to reduce future tax liabilities and which expire beginning in 2037. The Company has state operating loss carryforwards of approximately $7,500 available to reduce state future taxable income, which will expire beginning in 2027.

Realization of future tax benefits is dependent on many factors, including the Company's ability to generate taxable income within the net operating loss carryforward period. Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, respectively, as well as similar state provisions. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain shareholders or public groups in the stock of a corporation by more than 50% over a three-year period. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382, during 2017 after conversion to a C-corporation, utilization of the U.S. net operating loss carryforwards or research and development tax credit carryforwards at December 31, 2017 may be subject to an annual limitation under Section 382, which is determined by first multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization.

ASC 740 requires a valuation allowance to reduce the deferred tax assets reported if, based on the weight of the evidence, it is more likely than not that some portion or all of the deferred tax assets will not be realized. After consideration of all the evidence, both positive and negative, the Company has recorded a valuation allowance against its deferred tax assets at December 31, 2017 because the Company's management has determined that it is more likely than not that the Company will not recognize the benefits of its federal and state deferred tax assets primarily due to its cumulative loss position and, as a result, a valuation allowance of approximately $3,200 and $650 has been established, respectively. The was no deferred tax asset at December 31, 2016 as the Company was an LLC at such time and was taxed similar to a partnership where all income and expenses were recorded on the tax returns of its shareholders.

The Company has no unrecognized tax benefits. The Company has not, as yet, conducted a study of its research and development credit carryforwards. 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 development credits and, if an adjustment were required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the consolidated balance sheet or consolidated statements of operations and comprehensive loss if an adjustment were required. The Company has elected to recognize interest and penalties related to income tax matters as a component of income tax expense, of which no interest or penalties were recorded for the years ended December 31, 2016 and 2017.

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

(Amounts in thousands, except share, unit, per share, and per unit amounts)

	Year Ended December 31, 2017
U.S. federal statutory income tax rate	34.0 %
Permanent differences	(16.6)
State income taxes, net of federal benefit	2.8
Other	(0.2)
Research and development and orphan drug tax credits	8.3
Change in deferred tax asset valuation allowance	(28.3)
Effective income tax rate	— %

The Company's deferred tax assets at December 31, 2017 consists of the following:

	December 31, 2017
Net operating loss carryforwards	$ 2,000
Research and development and orphan drug tax credits	1,200
Licenses	500
Other	150
Total deferred tax assets	3,850
Valuation allowance	(3,850)
Net deferred tax assets	$ —

In December 2017, the Tax Cuts and Jobs Act, or the Tax Act ("TCJA"), was signed into law. Among other things, the Tax Act permanently lowers the corporate federal income tax rate to 21% from the statutory rate of 34%, effective for tax years including or commencing January 1, 2018. As a result of the reduction of the corporate federal income tax rate to 21%, U.S. GAAP requires companies to revalue their deferred tax assets and deferred tax liabilities as of the date of enactment, with the resulting tax effects accounted for in the reporting period of enactment. This revaluation resulted in an overall reduction of deferred taxes of $1,140 and a corresponding reduction in the valuation allowance. As a result, there was no net impact to the Company's consolidated statements of operations and comprehensive loss as a result of the reduction in tax rates.

While the Company considers undistributed earnings of its foreign subsidiary to be indefinitely reinvested, currently there are no undistributed earnings.

14. Research and License Agreements

National Taiwan University

In September 2015, the Company entered into a Collaborative Research Agreement (the "Research Agreement") with National Taiwan University ("NTU") under which the Company will collaborate with and fund certain development projects at NTU. The Research Agreement runs through September 30, 2020.

The Company also entered into a License and Technology Transfer Agreement with NTU ("NTU License") in December 2015 under which the Company received an exclusive, world-wide license to any and all technology, intellectual property, and know-how pertaining to NTU's AADC gene therapy and related products with the right to sublicense. The NTU License grants the Company the right to research, develop, make, have made, manufacture, have manufactured, use, and sell products which incorporate the licensed technology. The Company made a $100 license fee payment upon signing the NTU License, is required to make $2,000 in milestone payments upon certain clinical and regulatory milestones, pay annual license maintenance fees and is required to pay a low double-digit royalty on products that incorporate the licensed technology and intellectual property and a royalty percentage ranging between the low-twenties and mid-twenties on sublicense revenues. The Company recorded the license fee payment and annual maintenance fees as research and development expense as acquired in-process research and development as the licensed technology had not reached technological feasibility and had no alternative future uses.

The Company recognized research and development expense of $65, $293, $130 and $93 under the Research Agreement in the years ended December 31, 2016 and 2017 and the six months ended June 30, 2018 (unaudited), respectively. As of December 31, 2017, and June 30, 2018 (unaudited), the Company had no accounts payable to NTU under the Research Agreement.

Angelman and Reelin Agreements

The Company has entered into a research agreement in May 2016 with a university under which it is funding research related to six specific licensed technologies. The research agreements provide for annual funding amounts and can be terminated with thirty-day's notice by either party. The Company recorded research and development expense of $133, $141, $49 and $68 under these agreements in the years ended December 31, 2016 and 2017 and the six months ended June 30, 2017 and 2018 (unaudited).

The Company has also entered into exclusive, world-wide license agreements with the research foundation of a second university which is a direct support organization for the above university (“licensor”) for patents and intellectual property related to Angelman Syndrome and Reelin. The licenses allow the Company to research, develop, and manufacture products and licensed processes that incorporate the licensed patents and intellectual property. The Company also has the right to sublicense with consent by the licensor. In consideration of the rights granted under the agreements, the Company paid initial license fees of $90, is obligated to make payments of up to $9,000 in the aggregate upon the achievement of developmental and commercial milestones under the six license agreements and is required to pay low single digit royalties on sales of products that incorporate the licensed patents and intellectual property and royalty percentages ranging from the low thirties to low fifties on sublicense revenues. In addition, the Company must pay annual minimum royalty amounts in years in which the annual royalty amounts are not reached.

Under the agreement, the licensor reserves to itself, as well as to all non-profit research institutions with which it collaborates, the right to use materials that might be covered under the licensed patents and intellectual property solely for their internal research, educational and clinical purposes and to meet all applicable governmental and peer review journal requirements.

Collaboration Agreement

The Company entered into an Exclusive Channel Collaboration Agreement (the “Channel Agreement”) with a counterparty in October 2013 to use certain of the counterparty’s technology to research, develop, use, make, have made, manufacture and commercialize Company products targeted to Friedreich’s ataxia. The Company also has the right to sublicense with consent by the counterparty. The Company paid a technology access fee of $2,500 upon signing the Channel Agreement and is obligated to make milestone payments of up to $13,000 in the aggregate based upon the attainment of certain clinical development milestones and to pay a low double-digit royalty based on the sale of products that incorporate the technology and a royalty percentage in the low fifties on sublicense revenue. The Company recorded the technology access fee as research and development expense in 2013 as acquired in-process research and development as the licensed technology had not reached technological feasibility and had no alternative future uses.

15. Research Collaboration

In December 2016, the Company entered into a Research Collaboration Agreement with a research foundation (“Angelman Collaboration”) under which the Company is receiving funding to further the Company’s research and development of a therapeutic drug to treat Angelman Syndrome. The Company is receiving funding of $125 per quarter for two years from the date of the agreement and is obligated to make milestone payments of up to $1,500 in the aggregate based upon the attainment of certain clinical milestones and a low single-digit royalty percentage on the net sales of gene therapy products for Angelman Syndrome. Funding received from the research foundation under the Angelman Collaboration is recorded as a reduction of research and development expenses incurred under the Angelman Collaboration. Amounts received in advance of incurring the expenses are recorded as a liability. The Company has recorded $325, $116 and $154 as a reduction in research and development expense for the years ended December 31, 2017 and the six months ended June 30, 2017 and 2018 (unaudited), respectively.

16. Commitments and Contingencies

Operating Lease

(Amounts in thousands, except share, unit, per share, and per unit amounts)

In February 2018, the Company and an independent third party entered into an operating lease for the Company's primary office space in Lynnfield, Massachusetts. The operating lease is a six-year lease with an option to renew for an additional six-year period. Rent expense was $71, $101, $43 and $116 for the years ended December 31, 2016 and 2017 and the six months ended June 30, 2017 and 2018 (unaudited), respectively.

The minimum aggregate future operating lease commitments at June 30, 2018 (unaudited) are as follows:

2018 (remaining six months)	$	129
2019		261
2020		263
2021		44
2022		—
	$	697

Contract Commitments

In March 2016, the Company entered into an agreement with a clinical research organization. to provide contingent and non-contingent payments based upon meeting certain milestones. As of December 31, 2017 and June 30, 2018 (unaudited), the Company had committed to minimum payments under this agreement totaling up to $456.

The Company has contracts with two contract manufacturers for the manufacture of clinical grade material to support clinical trials and for the development of the manufacturing process and for GMP manufacturing runs to support its filings with the FDA for its lead product candidate. As of June 30, 2018 (unaudited), the Company has contracts with these two contractors providing for payments of up to $6,000 over the following 12 months.

Guarantees

The Company has identified the guarantees described below as disclosable, in accordance with ASC 460, Guarantees.

As permitted under Delaware law, the Company indemnifies its officers and directors for certain events or occurrences while the officer or director is, or was, serving at the Company's request in such capacity. The maximum potential amount of future payments the Company could be required to make is unlimited; however, the Company has directors' and officers' insurance coverage that should limit its exposure and enable it to recover a portion of any future amounts paid.

The Company is a party to a number of agreements entered into in the ordinary course of business that contain typical provisions that obligate the Company to indemnify the other parties to such agreements upon the occurrence of certain events. Such indemnification obligations are usually in effect from the date of execution of the applicable agreement for a period equal to the applicable statute of limitations. The aggregate maximum potential future liability of the Company under such indemnification provisions is uncertain.

The Company leases office space under a six-year noncancelable operating lease. The Company has standard indemnification arrangements under this lease that require it to indemnify the landlord against all costs, expenses, fines, suits, claims, demands, liabilities, and actions directly resulting from any breach, violation, or nonperformance of any covenant or condition of the lease.

As of December 31, 2016, and 2017 and June 30, 2018 (unaudited), the Company had not experienced any losses related to these indemnification obligations, and no material claims with respect thereto were outstanding. The Company does not expect significant claims related to these indemnification obligations and, consequently, concluded that the fair value of these obligations is negligible, and no related reserves have been established.

17. Employee Benefit Plan

Employees of the Company are eligible to participate in the Company's 401(k) retirement plan ("401(k) Plan"). Participants may contribute up to 100% of their annual compensation to the 401(k) Plan, subject to statutory limitations. The 401(k) Plan does not allow the Company to make matching contributions.

Exhibit 99.4

UNAUDITED PRO FORMA CONDENSED COMBINED FINANCIAL STATEMENTS

On July 19, 2018, PTC Therapeutics, Inc. (the “Company”) entered into an Agreement and Plan of Merger (the “Merger Agreement”) by and among the Company, Agility Merger Sub, Inc., a Delaware corporation and a wholly owned, indirect subsidiary of the Company (“Transitory Subsidiary”), Agilis Biotherapeutics, Inc., a Delaware corporation (“Agilis”), and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, a Colorado limited liability company. The Merger Agreement provides for the acquisition of Agilis by the Company through the merger of Transitory Subsidiary into Agilis, with Agilis surviving as a wholly owned, indirect subsidiary of the Company (the “Merger”). On August 23, 2018, the Company completed its acquisition of Agilis pursuant to the Merger Agreement.

The following unaudited pro forma condensed combined financial statements (the "Statements") were prepared in accordance with Article 11 of the Securities and Exchange Commission's ("SEC") Regulation S-X and reflect adjustments to the extent they are directly attributable to the acquisition, factually supportable, expected to have a continuing impact and, for balance sheet purposes, are nonrecurring.

The unaudited pro forma condensed combined financial statements present the combination of the historical consolidated financial statements of the Company and the historical consolidated financial statements of Agilis adjusted to give effect to the August 23, 2018 acquisition of Agilis by the Company. The unaudited pro forma condensed combined balance sheet as of June 30, 2018 combines the unaudited historical consolidated balance sheet of the Company and the unaudited historical consolidated balance sheet of Agilis as of June 30, 2018, giving effect to the Merger as if it had occurred on June 30, 2018. The unaudited pro forma condensed combined statements of operations for the year ended December 31, 2017 and for the six months ended June 30, 2018 combine the audited historical consolidated statement of operations of the Company and the audited historical consolidated statement of operations of Agilis for the year ended December 31, 2017, and the unaudited historical consolidated statement of operations of the Company and the unaudited historical consolidated statement of operations of Agilis for the six months ended June 30, 2018, giving effect to the Merger as if it had occurred on January 1, 2017.

The unaudited pro forma condensed combined financial statements are presented for informational purposes only and are not intended to represent or be indicative of what the financial position or results of operations would have been had the Merger occurred on the dates indicated. The unaudited pro forma condensed combined financial statements are not intended to represent or be indicative of the financial position or results of operations for any future periods.

The "Historical PTC" column in the unaudited pro forma combined financial statements reflects the Company's historical financial statements as of and for the period ended June 30, 2018 and historical statement of operations for the year ended December 31, 2017 and does not reflect any adjustments related to the Merger.

The "Historical Agilis" column in the unaudited pro forma combined financial statements reflects Agilis's historical consolidated financial statements as of and for the period ended June 30, 2018 and historical consolidated statement of operations for the year ended December 31, 2017 and does not reflect any adjustments related to the Merger.

The "Pro Forma Adjustments" are based on the Company's assumptions and estimates that the Company believes are reasonable and which are described in the accompanying notes to the unaudited pro forma combined financial statements (the "Notes").

The assumptions used and adjustments made in preparing the Statements are described in the Notes, which should be read in conjunction with the Statements. The Statements and related Notes contained herein should be read in conjunction with the consolidated financial statements and related notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2017 filed with the SEC on March 6, 2018 and the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 filed with the SEC on August 7, 2018 and the historical consolidated financial statements and related notes of Agilis included as Exhibit 99.3 to the Company's Current Report on Form 8-K filed with the SEC on August 24, 2018 to which this Exhibit 99.4 is attached.

PTC Therapeutics, Inc.
Unaudited Pro Forma Condensed Combined Balance Sheet
In thousands

	June 30, 2018				
	Historical PTC	Historical Agilis	Pro Forma Adjustments	Notes	Pro Forma Combined
Assets					
Current assets:					
Cash and cash equivalents	$ 223,788	$ 11,829	$ (61,829)	(A)	$ 173,788
Marketable securities	72,318	—	—		72,318
Trade receivables, net	59,383	—	—		59,383
Inventory	13,852	—	—		13,852
Prepaid expenses and other current assets	6,305	133	—		6,438
Total current assets	375,646	11,962	(61,829)		325,779
Fixed assets, net	8,217	159	—		8,376
Goodwill	—	—	103,704	(B)	103,704
Intangible assets, net	126,290	—	480,000	(B)	606,290
Deposits and other assets	1,620	38	—		1,658
Total assets	$ 511,773	$ 12,159	$ 521,875		$ 1,045,807
Liabilities and stockholders' equity					
Current liabilities:					
Accounts payable and accrued expenses	$ 82,534	$ 3,813	$ 1,744	(C)	$ 88,091
Current portion of long-term debt	1,666	—	—		$ 1,666
Other current liabilities	2,274	461	—		2,735
Total current liabilities	86,474	4,274	1,744		92,492
Deferred revenue - long-term	10,540	—	—		10,540
Long-term debt	147,204	—	—		147,204
Contingent consideration	—	—	218,700	(D)	218,700
Milestone payable	—	—	40,000	(D)	40,000
Deferred tax liabilities	—	—	115,200	(E)	115,200
Other long-term liabilities	153	7	(7)	(F)	153
Total liabilities	244,371	4,281	375,637		624,289
Redeemable convertible preferred stock	—	41,874	(41,874)	(F)	—
Stockholders' equity:					
Common stock	47	3	1	(F), (A)	51
Additional paid-in capital	1,105,124	3,779	152,077	(F), (A)	1,260,980
Accumulated other comprehensive income	1,855	—	—		1,855
Accumulated deficit	(839,624)	(37,778)	36,034	(C), (F)	(841,368)
Total stockholders' equity	267,402	(33,996)	188,112		421,518
Total liabilities, convertible preferred stock and stockholders' equity	$ 511,773	$ 12,159	$ 521,875		$ 1,045,807

See accompanying notes to the Unaudited Pro Forma Condensed Combined Financial Statements.

PTC Therapeutics, Inc.
Unaudited Pro Forma Condensed Combined Statement of Operations
In thousands (except per share data)

	Year ended December 31, 2017				
	Historical PTC	Historical Agilis	Pro Forma Adjustments	Notes	Pro Forma Combined
Revenues:					
Net product revenue	$ 174,066	$ —	$ —		$ 174,066
Collaboration and grant revenue	20,326	—	—		20,326
Total revenues	194,392	—	—		194,392
Operating expenses:					
Cost of product sales, excluding amortization of acquired intangible asset	4,577	—	—		4,577
Amortization of acquired intangible asset	15,380	—	—		15,380
Research and development	117,456	4,906	—		122,362
Selling, general and administrative	121,271	2,445	—		123,716
Total operating expenses	258,684	7,351	—		266,035
Loss from operations	(64,292)	(7,351)	—		(71,643)
Interest (expense) income, net	(12,094)	7	—		(12,087)
Other expense, net	(1,279)	(6,990)	—		(8,269)
Loss before income tax expense	(77,665)	(14,334)	—		(91,999)
Income tax expense	(1,335)	—	—		(1,335)
Net loss attributable to common stockholders	$ (79,000)	$ (14,334)	$ —		$ (93,334)
Weighted-average shares outstanding:					
Basic and diluted (in shares)	39,183,073		3,500,907	(A)	42,683,980
Net loss per share—basic and diluted (in dollars per share)	$ (2.02)				$ (2.19)

See accompanying notes to the Unaudited Pro Forma Condensed Combined Financial Statements.

PTC Therapeutics, Inc.
Unaudited Pro Forma Condensed Combined Statement of Operations
In thousands (except per share data)

	Six months ended June 30, 2018				
	Historical PTC	Historical Agilis	Pro Forma Adjustments	Notes	Pro Forma Combined
Revenues:					
Net product revenue	$ 124,151	$ —	$ —		$ 124,151
Collaboration and grant revenue	654	—	—		654
Total revenues	124,805	—	—		124,805
Operating expenses:					
Cost of product sales, excluding amortization of acquired intangible asset	5,616	—	—		5,616
Amortization of acquired intangible asset	11,022	—	—		11,022
Research and development	63,970	6,782	—		70,752
Selling, general and administrative	66,514	4,745	(711)	(G)	70,548
Total operating expenses	147,122	11,527	(711)		157,938
Loss from operations	(22,317)	(11,527)	711		(33,133)
Interest expense, net	(6,187)	67	—		(6,120)
Other income (expense), net	332	2,012	—		2,344
Loss before income tax expense	(28,172)	(9,448)	711		(36,909)
Income tax expense	(610)	—	—		(610)
Net loss attributable to common stockholders	$ (28,782)	$ (9,448)	$ 711		$ (37,519)
Weighted-average shares outstanding:					
Basic and diluted (in shares)	46,257,397		3,500,907	(A)	49,758,304
Net loss per share—basic and diluted (in dollars per share)	$ (0.62)				$ (0.75)

See accompanying notes to the Unaudited Pro Forma Condensed Combined Financial Statements.

PTC Therapeutics, Inc.
Notes to Unaudited Pro Forma Condensed Combined Financial Statements
In thousands (except per share data unless otherwise noted)

1. Description of the transaction

On August 23, 2018, the Company subsequently closed the transactions contemplated by the Merger Agreement and completed its acquisition of Agilis at which time all issued and outstanding shares of the capital stock and outstanding vested options and warrants of Agilis were converted into the right to receive, subject to customary adjustments, an aggregate, of (i) approximately $50.0 million, funded with cash on hand less Agilis transaction expenses and all amounts outstanding under the Company's Bridge Loan and Security Agreement (the "Bridge Loan Agreement") with Agilis, dated as of July 19, 2018, as of the closing and subject to certain other pre- and post-closing adjustments, and (ii) 3,500,907 of shares of the Company's common stock (the "Closing Stock Consideration"), which represents approximately 7% of the shares of the Company's common stock outstanding as of the closing of the Merger. The Closing Stock Consideration was determined by dividing $150.0 million by the volume-weighted average price per share of the Company's common stock on the Nasdaq Global Select Market for the ten consecutive trading day period ending on the second trading day immediately preceding the closing of the Merger (the "10-Day VWAP").

In addition, pursuant to the Merger Agreement, Agilis equityholders will be entitled to receive contingent 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%. Under the Merger Agreement, the Company is required to pay $40.0 million of the development milestone payments no later than the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved.

2. Basis of presentation

The unaudited pro forma condensed combined financial statements are based on the historical consolidated financial statements of the Company and the historical financial statements of Agilis as adjusted to give effect to the acquisition of Agilis by the Company. Certain reclassifications have been made to the historical financial statements of Agilis to conform with the Company's presentation, primarily related to the presentation of other income (expense), net and other current liabilities.

The unaudited pro forma combined balance sheet as of June 30, 2018 gives effect to the Merger as if it had occurred on June 30, 2018.

The unaudited pro forma combined statements of operations for the year ended December 31, 2017 and for the six months ended June 30, 2018 gives effect to the Merger as if it had occurred on January 1, 2017.

The Company will account for the Merger under the acquisition method of accounting based on Accounting Standards Codification ("ASC") Topic 805, *Business Combinations*, ("ASC 805") under which, among other things, transaction costs are expensed as incurred, the value of acquired in-process research and development is capitalized and contingent payments are recorded at their estimated fair value. The total estimated purchase price, calculated as described in Note 3 to these pro forma financial statements, is allocated to the net tangible and intangible assets of Agilis based on their estimated fair values for purposes of these pro forma financial statements. Management has made a preliminary allocation of the estimated purchase price to the tangible and intangible assets acquired and liabilities assumed based on a preliminary valuation and other preliminary estimates for purposes of these pro forma financial statements. A final determination of these estimated fair values will be based on the actual net tangible and intangible assets of Agilis that exist as of the date of completion of the transaction, and upon the final purchase price.

3. Purchase price and preliminary estimate of consideration transferred

The following is a preliminary estimate of the components of the consideration transferred as part of the acquisition:

Cash consideration	$	50,000
Fair value of PTC common stock issued		155,860
Milestone payable		40,000
Estimated fair value of contingent consideration payable		218,700
Total preliminary consideration transferred	$	464,560

The following is a summary of the preliminary allocation of the purchase price reconciled to the estimate of net consideration transferred. The purchase price allocation assumes that the acquisition occurred on June 30, 2018:

Purchase price	$	464,560
Prepaid expenses and other current assets		133
Fixed assets, net		159
Other assets		38
Intangible assets - in-process research and development		480,000
Total identifiable assets acquired		480,330
Accounts payable and accrued expenses	$	3,813
Other current liabilities		461
Deferred tax liabilities		115,200
Total liabilities assumed		119,474
Net identifiable assets acquired		360,856
Goodwill		103,704
Net assets acquired	$	464,560

4. Pro forma adjustments

The pro forma adjustments are based on preliminary estimates and assumptions that are subject to change. The following adjustments have been reflected in the unaudited pro forma condensed combined financial statements:

A. Reflects the adjustment to record the upfront cash payment to Agilis equityholders of approximately $50.0 million, less cash to be retained by Agilis equity holders of $11.8 million and the issuance of common stock of the Company as Closing Stock Consideration at the close of the acquisition valued at $155.9 million.

B. Reflects the adjustment to record estimated fair value of the in-process research and development intangible assets based on the allocation of the purchase price as if the Merger had occurred on June 30, 2018 (see Note 3). The value of in-process research and development intangible assets is based upon a preliminary valuation. The Company expects to complete the allocation of the purchase price within one year from the date of the closing of the acquisition. Differences between the preliminary and final valuation could have a material impact on the accompanying unaudited pro forma condensed combined financial statement information and the Company’s future results of operations and financial position.

C. Reflects the adjustment to record the estimated transaction costs not yet incurred as of the June 30, 2018 balance sheets presented herein.

D. Reflects the adjustment to record the milestone payments and the fair value of contingent purchase consideration at the date of the closing of the acquisition in accordance with ASC 805-10. The Company expects to complete the allocation of the purchase price within one year from the date of the closing of the acquisition. Differences between the preliminary and final valuation could have a material impact on the accompanying unaudited pro forma condensed combined financial statement information and the Company’s future results of operations and financial position.

E. Reflects the adjustment to record the preliminary estimated tax impact of identifiable intangible assets recorded in connection with the Merger. The actual deferred tax liabilities recorded as a result of the acquisition could be significantly different.

F. Reflects the adjustment to eliminate historical warrants for the purchase of Series B Preferred shares, redeemable convertible preferred stock, common stock, additional paid-in capital, and accumulated deficit accounts.

G. Reflects the adjustment to eliminate legal costs included in the historical financial statements of the Company and Agilis, which are directly attributable to the Merger but which are not expected to have a continuing impact on the combined entity's results.