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As filed with the Securities and Exchange Commission on May 7, 2020

Registration No. 333-

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM S-3**  
REGISTRATION STATEMENT  
UNDER  
THE SECURITIES ACT OF 1933

**PTC THERAPEUTICS, INC.**

(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**04-3416587**  
(I.R.S. Employer  
Identification Number)

**100 Corporate Court  
South Plainfield, NJ 07080  
(908) 222-7000**

(Address, including Zip Code, and Telephone Number, including Area Code, of Registrant's Principal Executive Offices)

**Stuart W. Peltz, Ph.D.**  
**Chief Executive Officer**  
**PTC Therapeutics, Inc.**  
**100 Corporate Court**  
**South Plainfield, New Jersey 07080**  
**(908) 222-7000**

(Name, Address, including Zip Code, and Telephone Number, including Area Code, of Agent for Service)

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**100 Corporate Court**  
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**Approximate date of commencement of proposed sale to the public:**  
**From time to time after this registration statement becomes effective.**

If the only securities being registered on this Form are being offered pursuant to dividend or interest reinvestment plans, please check the following box.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, other than securities offered only in connection with dividend or interest reinvestment plans, check the following box.

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a registration statement pursuant to General Instruction I.D or a post-effective amendment thereto that shall become effective upon filing with the Commission pursuant to Rule 462(e) under the Securities Act, check the following box.

If this Form is a post-effective amendment to a registration statement filed pursuant to General Instruction I.D filed to register additional securities or additional classes of securities pursuant to Rule 413(b) under the Securities Act, check the following box.

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

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

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act.

**CALCULATION OF REGISTRATION FEE**

Title of Each Class of Securities to be Registered(1)	Amount to be Registered(1)	Proposed Maximum Offering Price Per Unit(2)	Proposed Maximum Aggregate Offering Price(2)	Amount of Registration Fee
Common Stock, par value \$0.001 per share	2,821,176	\$47.64	\$134,400,825	\$17,445.23

- (1) The shares of common stock will be offered for resale by the selling stockholders. Pursuant to Rule 416 under the Securities Act, this Registration Statement also relates to an indeterminate number of additional shares of common stock to be issued as a result of stock splits, stock dividends or similar transactions.
  - (2) Estimated solely for purposes of calculating the registration fee pursuant to Rule 457(c) under the Securities Act, based on the average of the high and low price per share of the common stock as reported on The Nasdaq Global Select Market on May 4, 2020.
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PROSPECTUS

2,821,176 Shares



**PTC Therapeutics, Inc.**

**Common Stock**

This prospectus relates to resales of shares of common stock previously issued by PTC Therapeutics, Inc. to certain former equityholders of Agilis Biotherapeutics, Inc., or Agilis, in exchange for the cancellation and forfeiture by such former equityholders of their rights to receive certain milestone-based contingent payments, which rights were previously received by such former equityholders in connection with our acquisition of Agilis in August 2018.

We will not receive any proceeds from the sale of the shares.

The selling stockholders identified in this prospectus, or their pledgees, assignees, donees, transferees or other successors-in-interest, may offer the shares from time to time through public or private transactions at prevailing market prices, at prices related to prevailing market prices or at privately negotiated prices.

Our common stock is traded on The Nasdaq Global Select Market under the symbol "PTCT." On May 6, 2020, the closing sale price of the common stock on The Nasdaq Global Select Market was \$48.22 per share. You are urged to obtain current market quotations for the common stock.

**Investing in our common stock involves significant risks. See the information contained in this prospectus under the heading "Risk Factors" beginning on page 10 of this prospectus and in the documents incorporated by reference in this prospectus for a discussion of the factors you should carefully consider before deciding to purchase our common stock.**

**Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense. The date of this prospectus is May 7, 2020.**

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## ABOUT THIS PROSPECTUS

This prospectus is part of a registration statement that we filed with the Securities and Exchange Commission, or the SEC. The selling stockholders may from time to time sell shares of common stock described in this prospectus.

This prospectus provides you with a description of the common stock selling stockholders may offer. You should read both this prospectus together with the additional information described under the heading "Where You Can Find More Information" beginning on page 26 of this prospectus.

We have not authorized anyone to provide you with information different from that contained in this prospectus, any accompanying prospectus supplement or in any related free writing prospectus filed by us with the SEC. We do not take any responsibility for, and cannot provide any assurance as to the reliability of, any information other than the information in this prospectus, any accompanying prospectus supplement or in any related free writing prospectus filed by us with the SEC. This prospectus does not constitute an offer to sell or the solicitation of an offer to buy any securities other than the securities described herein or an offer to sell or the solicitation of an offer to buy such securities in any circumstances in which such offer or solicitation is unlawful. The selling stockholders are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. You should assume that the information appearing in this prospectus, any prospectus supplement, the documents incorporated by reference and any related free writing prospectus is accurate only as of the respective dates of such documents. Our business, financial condition, results of operations and prospects may have changed materially since those dates.

Unless the context otherwise indicates, references in this prospectus to "PTC," "we," "our," "us" and "the Company" refer, collectively, to PTC Therapeutics, Inc., a Delaware corporation, and its consolidated subsidiaries.

## SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus and the information incorporated by reference in this prospectus include "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. All statements contained or incorporated by reference herein, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth, other than statements of historical facts, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "predict," "project," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this prospectus and the information incorporated by reference herein and therein include, among other things, statements about:

- expectations with respect to our gene therapy platform, including any potential regulatory submissions and potential approvals, including those related to our gene therapy for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency, or PTC-AADC, our manufacturing capabilities and the potential financial impact and benefits of our leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that we may be obligated to make;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for our products or product candidates that we commercialize or may commercialize in the future;

- our ability to maintain our marketing authorization of Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in the European Economic Area, or EEA, which is subject to the specific obligation to conduct and submit the results of Study 041 to the European Medicines Agency, or EMA, and annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA;
- our ability to enroll, fund, and complete Study 041, a multicenter, randomized, double-blind, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open label extension, according to the protocol agreed with the EMA, and by the EMA's deadline;
- the anticipated period of market exclusivity for Emflaza for the treatment of DMD in the United States under the Orphan Drug Act of 1983, the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act;
- our ability to complete any dystrophin study necessary in order to resolve the matters set forth in the United States Food and Drug Administration's, or the FDA, denial of our appeal to the Complete Response Letter we received from the FDA in connection with our New Drug Application, or NDA, for Translarna for the treatment of nmDMD, and our ability to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost;
- the timing and scope of our commercialization of our products and product candidates;
- our expectations with respect to the novel coronavirus (COVID-19) pandemic and related response measures and their effects on our business, operations, clinical trials, potential regulatory submissions and approvals, our collaborators, contract research organizations, suppliers and manufacturers;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs for our products on adequate terms, or at all;
- our expectations with respect to the development, regulatory and commercial status of our product candidates and program directed against spinal muscular atrophy in collaboration with F. Hoffmann La Roche Ltd and Hoffmann La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or the SMA Foundation, and our estimates regarding future revenues from achievement of milestones in that program;
- our expectations and the potential financial impact and benefits related to our Collaboration and Licensing Agreement with Akcea Therapeutics, Inc., or Akcea, including with respect to the timing of regulatory approval of Tegsedi™ (inotersen) and Waylivra™ (volanesorsen) in countries in which we are licensed to commercialize them, the commercialization of Tegsedi and Waylivra, and our expectations with respect to contingent payments to Akcea based on the potential achievement of certain regulatory milestones and royalty payments by us to Akcea based on our potential achievement of certain net sales thresholds;
- our estimates regarding the potential market opportunity for our products or product candidates, including the size of eligible patient populations and our ability to identify such patients;
- our estimates regarding expenses, future revenues, third-party discounts and rebates, capital requirements and needs for additional financing, including our ability to maintain the level of our expenses consistent with our internal budgets and forecasts and to secure additional funds on favorable terms or at all;
- the timing and conduct of our ongoing, planned and potential future clinical trials and studies in our gene therapy, splicing, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications, including the timing of

initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;

- our ability to realize the anticipated benefits of our acquisitions or other strategic transactions, including the possibility that the expected impact of benefits from the acquisitions or strategic transactions will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of operations and employees into our business, our ability to obtain marketing approval of our product candidates we acquired from the acquisitions or other strategic transactions and unknown liabilities;
- the rate and degree of market acceptance and clinical utility of any of our products or product candidates;
- the ability and willingness of patients and healthcare professionals to access our products and product candidates through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- our ability to complete the FDA post-marketing requirements to the marketing authorization of Emflaza and any other post-marketing requirements for our products;
- the timing of, and our ability to obtain additional marketing authorizations for our products and product candidates;
- the ability of our products and our product candidates to meet existing or future regulatory standards;
- our ability to maintain the current labeling under the marketing authorization in the EEA or expand the approved product label of Translarna for the treatment of nmDMD;
- the potential receipt of revenues from future sales of our products or product candidates;
- the potential impact that enrollment, funding and completion of Study 041 may have on our revenue growth;
- our sales, marketing and distribution capabilities and strategy, including the ability of our third-party manufacturers to manufacture and deliver our products and product candidates in clinically and commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy their other obligations to us;
- our ability to establish and maintain arrangements for the manufacture of our products and product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our ability to establish and grow our manufacturing capabilities for our gene therapy platform;
- our expectations with respect to the potential financial impact and benefits of our leased biologics manufacturing facility and our ability to satisfy our obligations under the terms of the lease agreement for such facility;
- our ability to satisfy our obligations under the terms of the credit and security agreement with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and certain other financial institutions as lenders thereunder;
- our ability to satisfy our obligations under the indenture governing our 3.00% convertible senior notes due August 15, 2022 and under the indenture governing our 1.50% convertible senior notes due September 15, 2026;
- our regulatory submissions, including with respect to timing and outcome of regulatory review;

- our plans to advance our earlier stage programs and pursue research and development of other product candidates, including our splicing, gene therapy, Bio-e and oncology programs;
- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets and our ability to successfully develop or commercialize any assets to which we may gain rights pursuant to such business development opportunities;
- the potential advantages of our products and any product candidate;
- our intellectual property position;
- the impact of government laws and regulations;
- the impact of litigation that has been or may be brought against us or of litigation that we are pursuing against others; and
- our competitive position.

We may not actually achieve the plans, intentions or expectations expressed or implied in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus and the information incorporated by reference herein, particularly the factors set forth under the caption "Risk Factors" of this prospectus, under the caption "Risk Factors" in Part I, Item 1A of our Annual Report on Form 10-K for the fiscal year ended December 31, 2019, and under the caption "Risk Factors" in Part II, Item 1A of our Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2020, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we make. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.



## PROSPECTUS SUMMARY

This summary highlights selected information contained elsewhere in this prospectus and in the documents we incorporate by reference. This summary does not contain all of the information you should consider before making an investment decision. You should read this entire prospectus carefully, especially the risks of investing in our common stock discussed under "Risk Factors" beginning on page 10 of this prospectus, under the caption "Risk Factors" in Part I, Item 1A of our [Annual Report on Form 10-K for the fiscal year ended December 31, 2019](#), and under the caption "Risk Factors" in Part II, Item 1A of our [Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2020](#), along with our consolidated financial statements and notes to those consolidated financial statements and the other information incorporated by reference in this prospectus.

### PTC Therapeutics, Inc.

We are a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Our ability to globally commercialize products is the foundation that drives our continued investment in a robust diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. The Company's strategy is to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders and to leverage its global commercial infrastructure to maximize value for its patients and other stakeholders.

### Exchange of Rights of Certain Former Equityholders of Agilis Biotherapeutics, Inc.

On August 23, 2018, we acquired Agilis, which prior to the acquisition was a privately-held biotechnology company advancing an innovative gene therapy platform for rare monogenic diseases that affect the central nervous system. The acquisition was effected pursuant to an Agreement and Plan of Merger, dated as of July 19, 2018, or the Merger Agreement, by and among us, Agility Merger Sub, Inc., a Delaware corporation and our wholly owned, indirect subsidiary, Agilis, and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, a Colorado limited liability company and resulted in Agilis becoming our wholly owned, indirect subsidiary.

Pursuant to the Merger Agreement, each former equityholder of Agilis, or the former equityholders, is entitled to receive from us its pro rata portion of contingent payments, or the milestone payments, based on (i) the achievement of certain developmental 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 time periods, ranging from 2-6%. Under the Merger Agreement, we are required to pay \$40.0 million of the developmental milestone payments no later than the second anniversary of the closing of the acquisition, regardless of whether the applicable milestones have been achieved.

On April 29, 2020, we, certain of the former equityholders, or the Participating Rightholders, and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC entered into a rights exchange agreement, or the Rights Exchange Agreement, pursuant to which we agreed to issue 2,821,176 shares of our common stock, or the Common Stock Consideration, and paid approximately \$36.9 million, or the Cash Consideration, in the aggregate, to the Participating Rightholders in exchange for the cancellation and forfeiture by the Participating Rightholders of their rights to receive certain milestone-based contingent payments under the Merger Agreement. Also on

April 29, 2020, we issued 2,723,826 shares of the Common Stock Consideration to certain of the Participating Rightholders pursuant to the Rights Exchange Agreement, with the remaining 97,350 shares of the Common Stock Consideration issued to certain of the Participating Rightholders who are former employees of Agilis or current or former employees of us on May 6, 2020.

Pursuant to the terms of the Rights Exchange Agreement, the Participating Rightholders have canceled and forfeited their rights under the Merger Agreement to receive (i) \$174.0 million, in the aggregate, of potential milestone payments based on the achievement of certain regulatory milestones and (ii) \$37.6 million, in the aggregate, of \$40.0 million in development milestone payments due upon the passing of the second anniversary of the closing of the acquisition, regardless of whether the milestones are achieved.

The Rights Exchange Agreement has no effect on the Merger Agreement other than to provide for the cancellation and forfeiture of the Participating Rightholders' rights to receive \$211.6 million, in the aggregate, of the milestone payments described above. As a result, all other rights and obligations under the Merger Agreement remain in effect pursuant to their terms, including our obligation to pay up to an aggregate maximum amount of \$22.4 million upon the achievement of certain development milestones (representing the remaining portion of potential development milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement after deducting the \$37.6 million for which rights were canceled and forfeited pursuant to the Rights Exchange Agreement from the \$40.0 million in development milestone payments that are due upon the passing of the second anniversary of the closing of the acquisition), up to an aggregate maximum amount of \$361.0 million upon the achievement of certain regulatory milestones (representing the remaining portion of potential regulatory milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement), up to a maximum aggregate amount of \$150.0 million upon the achievement of certain net sales milestones and a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2% to 6%, pursuant to the terms of the Merger Agreement.

The above descriptions of the Merger Agreement and the Rights Exchange Agreement are summaries only and are qualified in their entirety by reference to the terms of the Merger Agreement and the Rights Exchange Agreement, included as Exhibit 2.1 and Exhibit 10.1 hereto, respectively.

#### **Company Information**

We were incorporated under the laws of the State of Delaware on March 31, 1998, under the name PTC Therapeutics, Inc. Our principal executive offices are located at 100 Corporate Court, South Plainfield, New Jersey 07080. Our telephone number is (908) 222-7000. We maintain a website at [www.ptcbio.com](http://www.ptcbio.com). The information contained on, or that can be accessed through, our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

### **The Offering**

Common Stock Offered by the Selling Stockholders	2,821,176 shares
Use of Proceeds	We will not receive any proceeds from the sale of shares in this offering.  See "Use of Proceeds" for more information.
Risk Factors	You should read the "Risk Factors" section of this prospectus, as well as those risk factors incorporated by reference in this prospectus, for a discussion of factors to consider carefully before deciding to purchase shares of our common stock.
Nasdaq Global Select Market Symbol	Our common stock is listed on The Nasdaq Global Select Market under the symbol "PTCT".

## RISK FACTORS

Investing in our common stock involves significant risks. In deciding whether to invest, and in consultation with your own financial and legal advisors, you should carefully consider the risk factors and the risks described under the caption "Risk Factors" in Part I, Item 1A of our [Annual Report on Form 10-K for the fiscal year ended December 31, 2019](#), and under the caption "Risk Factors" in Part II, Item 1A of our [Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2020](#), as well as the other information contained in this prospectus and in our filings with the SEC that we have incorporated by reference in this prospectus. Any of these risks could have a material adverse effect on our business, financial condition, results of operations and prospects and cause the value of our stock to decline, which could cause you to lose all or part of your investment. The risks and uncertainties we have described are not the only ones facing us. Additional risks and uncertainties not presently known to us or that we currently deem immaterial may also affect our business operations.

## USE OF PROCEEDS

We will not receive any proceeds from the sale of shares by the selling stockholders.

The selling stockholders will pay any underwriting discounts and commissions and expenses incurred by the selling stockholders for brokerage, accounting, tax, or legal services or any other expenses incurred by the selling stockholders in disposing of the shares. We will bear all other costs, fees and expenses incurred in effecting the registration of the shares covered by this prospectus, including, without limitation, all registration and filing fees, The Nasdaq Global Select Market listing fees, and fees and expenses of our counsel and our accountants.

## SELLING STOCKHOLDERS

This prospectus relates to the resale of shares of our common stock held by the selling stockholders listed in the table below. The selling stockholders acquired these shares from us in a private offering pursuant to an exemption from registration afforded by Section 4(a)(2) of the Securities Act and/or Regulation D promulgated thereunder as partial consideration for the cancellation and forfeiture of the selling stockholders' prior rights to receive \$211.6 million, in the aggregate, of certain milestone-based contingent payments, pursuant to the Rights Exchange Agreement. Under the Rights Exchange Agreement, we agreed to file a registration statement on Form S-3 with the SEC for the purposes of registering for resale the shares of our common stock issued to the selling stockholders and to maintain the effectiveness of the registration statement until the six month anniversary date of the Rights Exchange Agreement or such earlier time as all shares of common stock covered by the registration statement have been sold, subject to certain exceptions.

The following table sets forth, to our knowledge, certain information about the selling stockholders as of May 1, 2020.

Beneficial ownership is determined in accordance with the rules of the SEC and includes voting or investment power with respect to shares. Unless otherwise indicated below, to our knowledge, all persons named in the table have sole voting and investment power with respect to their shares of common stock, except to the extent authority is shared by spouses under applicable law. The inclusion

of any shares in this table does not constitute an admission of beneficial ownership for the person named below.

Name of Selling Stockholder	Shares of Common Stock Beneficially Owned Prior to Offering		Number of Shares of Common Stock Being Offered	Shares of Common Stock to be Beneficially Owned After Offering(1)	
	Number	Percentage		Number	Percentage
1307 Ltd.	53,035(2)	*	48,035	5,000	*
Adam Gray and Kerri Miller	5,568(3)	*	1,856	0	*
Adam L. Gray Revocable Trust U/A/D 10/5/2006	3,712(4)	*	3,712	0	*
Allen Adler	59,161(5)	*	59,161	0	*
Barton Children Trust	19,947(6)	*	19,947	0	*
Joseph U. Barton	158,627(7)	*	9,973	0	*
Kathleen Wagner Barton	5,916(8)	*	5,916	0	*
Lila Barton Thomas	9,973(9)	*	9,973	0	*
Linda Barton	9,973(10)	*	9,973	0	*
Maggie Barton	9,973(11)	*	9,973	0	*
Scott Barton	9,973(12)	*	9,973	0	*
Thomas Barton	178,574(13)	*	9,973	0	*
BGR Capital, L.P.	17,839(14)	*	17,839	0	*
BioMatrix Partners, Ltd.†	335,138(15)	*	335,138	0	*
James J. Collins	2,624(16)	*	2,624	0	*
Anne Marie Conway#	10,806(17)	*	8,421	2,385	*
Jodi Ann Cook#	54,508(18)	*	28,208	26,300	*
CureFA Foundation	74,250(19)	*	74,250	0	*
Gold Bench Capital, LLC†	192,257(20)	*	192,257	0	*
Christopher Guttilla	53,192(21)	*	53,192	0	*
Benjamin Hough	42,005(22)	*	42,005	0	*
Michael R. Hough	68,684(23)	*	52,652	16,032	*
Howard Klion	5,984(24)	*	5,984	0	*
Paola Leone	2,624(25)	*	2,624	0	*
Level One Partners, LLC†	470,340(26)	*	470,340	0	*
Thomas McGahren	46,135(27)	*	27,338	18,797	*
Mark Merrill	17,730(28)	*	17,730	0	*
NRM VII Holdings I, LLC	255,873(29)	*	118,322	137,551	*
Kevin O'Brien#	6,380(30)	*	6,380	0	*
Robert William Postma	459,390(31)	*	259,390	200,000	*
Mark J. Pykett#	132,120(32)	*	93,411	38,709	*
Gregory Scott Robinson	12,734(33)	*	9,734	3,000	*
Brett Ryan	25,629(34)	*	7,790	0	*
Sands Capital Global Venture Fund I, L.P.†	470,340(35)	*	470,340	0	*
William P. Saunders	24,620(36)	*	24,620	0	*
Chris Shackelton	7,425(37)	*	7,425	0	*
Bradley Christopher Shoup	1,856(38)	*	1,856	0	*
Adrian Stecyk†	54,191(39)	*	17,730	1,000	*
Chrystyna Bedrij Stecyk	54,191(40)	*	35,461	1,000	*
Storey Revocable Trust	4,986(41)	*	4,986	0	*
Arthur Tzianabos	3,502(42)	*	3,502	0	*
White Rock Capital Partners, L.P.	148,654(43)	*	148,654	0	*
Patricia Williams	1,359(44)	*	597	762	*
Mark Zizzamia	17,730(45)	*	17,730	0	*

Name of Selling Stockholder	Shares of Common Stock Beneficially Owned Prior to Offering		Number of Shares of Common Stock Being Offered	Shares of Common Stock to be Beneficially Owned After Offering(1)	
	Number	Percentage		Number	Percentage
Christopher Silber	6,504(46)	*	3,438	3,066	*
Gregory Fuest#	13,659(47)	*	2,646	11,013	*
Jennifer Daily#	641(48)	*	641	0	*
John Canepa#	19,250(49)	*	19,250	0	*
Kee-Hong Kim#	3,689(50)	*	3,689	0	*
Kirsten Gruis#	7,425(51)	*	7,425	0	*
Markus Peters#	7,435(52)	*	7,425	10	*
Nancy Klion	6,614(53)	*	6,614	0	*
Nancy Kribbs#	3,368(54)	*	3,368	0	*
Tony Arulanandam#	3,445(55)	*	3,327	118	*
Christina Ansted#	1,088(56)	*	1,088	0	*
Curtis Woodard#	2,646(57)	*	2,646	0	*
James E. Darnell	2,624(58)	*	2,624	0	*

\* Less than 1%.

# Employee or former employee of us or Agilis.

† Former member of the board of directors of Agilis.

- (1) We do not know when or in what amounts a selling stockholder may offer shares for sale. The selling stockholders might not sell any or all of the shares offered by this prospectus. Because the selling stockholders may offer all or some of the shares pursuant to this offering, and because there are currently no agreements, arrangements or understandings with respect to the sale of any of the shares, we cannot estimate the number of the shares that will be held by the selling stockholders after completion of the offering. However, for purposes of this table, we have assumed that, after completion of the offering, none of the shares covered by this prospectus will be held by the selling stockholders and that the number of shares otherwise held by the selling stockholders and not covered by this prospectus remains the same after completion of the offering.
- (2) Consists of 5,000 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 48,035 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Cabana Management, LLC is general partner and Casey McManemin is manager of the selling stockholder. Voting and investment control over the shares is held by Casey McManemin, manager of the selling stockholder. The address of the selling stockholder is 3838 Oak Lawn Ave., Suite 425, Dallas, TX 75219.
- (3) Consists of 1,856 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby and 3,712 shares of common stock issued to Adam L. Gray Revocable Trust U/A/D 10/5/2006 pursuant to the Rights Exchange Agreement and registered hereby, over which shares Mr. Gray has investment and voting control and of which entity Mr. Gray is trustee. The address of the selling stockholder is 4 Fairgreen Lane, Old Greenwich, CT 06870.
- (4) Consists of 3,712 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Voting and investment control over the shares is held by Adam Gray, trustee. The address of the selling stockholder is 4 Fairgreen Lane, Old Greenwich, CT 06870.

- (5) Consists of 59,161 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 800 Park Ave., 3<sup>rd</sup> Floor, New York, NY 10021.
- (6) Consists of 19,947 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Voting and investment control over the shares is held by Thomas Barton, trustee. The address of the selling stockholder is 3131 Turtle Creek Blvd., Suite 800, Dallas, TX 75219.
- (7) Consists of 9,973 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby and 148,654 shares of common stock issued to White Rock Capital Partners, L.P. pursuant to the Rights Exchange Agreement and registered hereby, over which shares the selling stockholder has shared investment and voting control. The address of the selling stockholder is 1314 East Las Olas Blvd., #34, Ft. Lauderdale, FL 33301.
- (8) Consists of 5,916 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 10026 Colvin Run Rd., Great Falls, VA 22066.
- (9) Consists of 9,973 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 6518 Lakewood Blvd., Dallas, TX 75214.
- (10) Consists of 9,973 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 4340 Overhill Dr., Dallas, TX 75205.
- (11) Consists of 9,973 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 4415 Glenwood Ave., Dallas, TX 75205.
- (12) Consists of 9,973 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The selling stockholder may be deemed to be an affiliate of National Securities Corporation, a registered broker-dealer. The selling stockholder represented that he acquired the shares in the ordinary course of business and, at the time of the acquisition of the shares, had no agreements or understandings, directly or indirectly, with any person to distribute the shares. The address of the selling stockholder is 4340 Overhill Dr., Dallas, TX 75205.
- (13) Consists of 9,973 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby, 148,654 shares of common stock issued to White Rock Capital Partners, L.P. pursuant to the Rights Exchange Agreement and registered hereby, over which shares the selling stockholder has shared investment and voting control, and 19,947 shares of common stock issued to the Barton Children Trust pursuant to the Rights Exchange Agreement and registered hereby, over which shares the selling stockholder has investment and voting control. The address of the selling stockholder is 3131 Turtle Creek Blvd., Suite 800, Dallas, TX 75219.
- (14) Consists of 17,839 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Voting and investment control over the shares is held by Brett Ryan, the manager of the selling stockholder. The address of the selling stockholder is 3131 Turtle Creek Blvd., Suite 800, Dallas, TX 75219.
- (15) Consists of 335,138 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Investment and voting control over the shares is held by G. Houston Hall. Mr. Hall, the general partner of the selling stockholder, is a former

member of the board of directors of Agilis. The address of the selling stockholder is 5956 Sherry Ln., Suite 1810, Dallas, TX 75225.

- (16) Consists of 2,624 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The selling stockholder is a former member of the Scientific Advisory Board of Agilis and currently serves on our Scientific Advisory Board. The address of the selling stockholder is 118 Glen Ave., Newton, MA 02459.
- (17) Consists of 2,385 shares of common stock underlying an inducement grant for non-statutory stock options issued to the selling stockholder pursuant to the Nasdaq inducement grant exception that are exercisable as of August 23, 2018 and 8,421 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 370 South Main St., Mansfield, MA 02048. The selling stockholder served as Vice President of Clinical Operations of Agilis prior to the acquisition and currently serves as our Clinical Head of Biologics.
- (18) Consists of 18,125 shares of common stock underlying an inducement grant for non-statutory stock options issued to the selling stockholder pursuant to the Nasdaq inducement grant exception that are exercisable as of August 23, 2019, November 23, 2019 and February 23, 2020 and 8,175 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 28,208 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 20 Terry Drive, #816, Newtown, PA 18940. The selling stockholder served as Chief Operating Officer of Agilis prior to the acquisition and served as our Head of Gene Therapy Strategy until January 2020 and currently serves as our consultant.
- (19) Consists of 74,250 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Investment and voting control over the shares is held by Thomas Hamilton. The address of the selling stockholder is 211 Stuyvesant Ave., Rye, NY 10580.
- (20) Consists of 192,257 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Investment and voting control over the shares is held by Alexander M. Seaver, Bradley R. Kent, Dominic DeMarco and John Welborn Jr. Mr. Seaver, a member of the selling stockholder, is a former member of the board of directors of Agilis. The address of the selling stockholder is 199 Elm St., New Canaan, CT 06840.
- (21) Consists of 53,192 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The selling stockholder may be deemed to be an affiliate of Raymond James, a registered broker-dealer. The selling stockholder represented that he acquired the shares in the ordinary course of business and, at the time of the acquisition of the shares, had no agreements or understandings, directly or indirectly, with any person to distribute the shares. The address of the selling stockholder is 57 Colt Rd., Summit, NJ 07901.
- (22) Consists of 42,005 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The selling stockholder may be deemed to be an affiliate of South Street Securities, a registered broker-dealer. The selling stockholder represented that he acquired the shares in the ordinary course of business and, at the time of the acquisition of the shares, had no agreements or understandings, directly or indirectly, with any person to distribute the shares. The address of the selling stockholder is 839 N. Avalon Road, Winston-Salem, NC 27104.
- (23) Consists of 16,032 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 52,652 shares of common stock issued to the selling stockholder pursuant



to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 2809 Lazy Ln., Winston-Salem, NC 27106.

- (24) Consists of 5,984 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 5927 Joyce Way, Dallas, TX 75225.
- (25) Consists of 2,624 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 312 Cotswold Ln., Cherry Hill, NJ 08034.
- (26) Consists of 470,340 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Voting and investment control over the shares is held by Robert D. Hardie and Molly G. Hardie, the partners of the selling stockholder. Mr. Hardie, a member of the selling stockholder, is a former member of the board of directors of Agilis. The address of the selling stockholder is 210 Ridge McIntire Rd., Suite 350, Charlottesville, VA 22903.
- (27) Consists of 11,697 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 7,100 shares of common stock acquired by the selling stockholder after our acquisition of Agilis and 27,338 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The selling stockholder may be deemed to be an affiliate of Griffin Securities, Inc., a registered broker-dealer. The selling stockholder represented that he acquired the shares in the ordinary course of business due to his role as a member of the Agilis founding group and, at the time of the acquisition of the shares, had no agreements or understandings, directly or indirectly, with any person to distribute the shares. The address of the selling stockholder is 50 Murray St., Apt. 711, New York, NY 10007. The selling stockholder is a former member of the Agilis Scientific Advisory Board and a member of Agilis founding group.
- (28) Consists of 17,730 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 349 11<sup>th</sup> Street, Brooklyn, NY 11215. The selling stockholder currently serves as one of our independent contractors.
- (29) Consists of 137,551 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 118,322 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Investment and voting control over the shares is held by Randal J. Kirk, the manager of Third Security, LLC, which is the manager of Third Security Capital Partners VII, LLC, which is the manager of the selling stockholder. The address of the selling stockholder is c/o Third Security, LLC, Attn: Legal Department, 1881 Grove Ave., Radford, VA 24141. An affiliate of the selling stockholder was party to an Exclusive Channel Collaboration Agreement with Agilis.
- (30) Consists of 6,380 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 149 Willow St., Acton, MA 01720. The selling stockholder served as Vice President of Finance of Agilis prior to the acquisition and served as our Head of Finance Operations until March 2019.
- (31) Consists of 200,000 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 259,390 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is P.O. Box 207, 141 Mecox Rd., Water Mill, NY 11976.
- (32) Consists of 37,500 shares of common stock underlying an inducement grant for non-statutory stock options issued to the selling stockholder pursuant to the Nasdaq inducement grant exception that

are exercisable as of August 23, 2019, November 23, 2019 and February 23, 2020 and 1,209 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 93,411 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 223A Main St., Boxford, MA 01921. The selling stockholder served as President and Chief Executive Officer of Agilis prior to the acquisition, served as our Chief Innovation Officer from August 2018 to January 2019 and currently serves as our Chief Scientific Officer.

- (33) Consists of 3,000 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 9,734 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 4 Foley Farm Rd., Wilmington, MA 01887.
- (34) Consists of 7,790 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby and 17,839 shares of common stock issued to BGR Capital, L.P. pursuant to the Rights Exchange Agreement and registered hereby, over which the selling stockholder has investment and voting control. The address of the selling stockholder is 5726 Greenbrier Drive, Dallas, Texas 75209.
- (35) Consists of 470,340 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Voting and investment control over the shares is shared by Ian Ratcliffe, Frank M. Sands and Michael Graninger. Mr. Ratcliffe, an officer of the selling stockholder, previously served as a member of the board of directors of Agilis prior to the acquisition. The address of the selling stockholder is 1000 Wilson Blvd., Suite 3000, Arlington, VA 22209.
- (36) Consists of 24,620 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 1700 Hardin Ln., Powell, OH 43065.
- (37) Consists of 7,425 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 487 West Rd., New Canaan, CT 06840.
- (38) Consists of 1,856 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 5950 Cedar Springs Road #100, Dallas, TX 75235.
- (39) Consists of 1,000 shares of common stock that the selling stockholder purchased jointly in the open market with Chrystyna Bedrij Stecyk, the spouse of the selling stockholder, and 17,730 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby and 35,461 shares of common stock issued to Mrs. Stecyk pursuant to the Rights Exchange Agreement and registered hereby. The selling stockholder disclaims beneficial ownership of the shares held by his spouse. The selling stockholder may be deemed to be an affiliate of Griffin Securities, Inc., a registered broker-dealer. The selling stockholder represented that he acquired the shares in the ordinary course of business and, at the time of the acquisition of the shares, had no agreements or understandings, directly or indirectly, with any person to distribute the shares. The address of the selling stockholder is 17 State Street, 3<sup>rd</sup> Floor, New York, NY 10004. The selling stockholder served as a member of the board of directors of Agilis prior to the acquisition.
- (40) Consists of 1,000 shares of common stock that the selling stockholder purchased jointly in the open market with Adrian Stecyk, the spouse of the selling stockholder, and 35,461 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby and 17,730 shares of common stock issued to Mr. Stecyk pursuant to the Rights Exchange

Agreement and registered hereby. The selling stockholder disclaims beneficial ownership of the shares held by her spouse. The selling stockholder may be deemed to be an affiliate of Griffin Securities, Inc., a registered broker-dealer. The selling stockholder represented that she acquired the shares in the ordinary course of business and, at the time of the acquisition of the shares, had no agreements or understandings, directly or indirectly, with any person to distribute the shares. The address of the selling stockholder is 17 State Street, 3<sup>rd</sup> Floor, New York, NY 10004.

- (41) Consists of 4,986 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Voting and investment control over the shares is held by Paula Kay White Storey and Reggie Louis Storey. The address of the selling stockholder is 508 Las Lomas Dr., Heath, TX 75032.
- (42) Consists of 3,502 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 14 Duck Rd., Reading, MA 01867.
- (43) Consists of 148,654 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. Voting and investment control over the shares is held by Thomas Barton and Joseph Barton. The address of the selling stockholder is 3131 Turtle Creek Blvd., Suite 800, Dallas, TX 75219.
- (44) Consists of 762 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 597 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 3470 Pin Oak Ct., Catharpin, VA 20143.
- (45) Consists of 17,730 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 20 River Terrace, Apt. 5M, New York, NY 10282.
- (46) Consists of 3,066 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and 3,438 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 36 Forest Street, Lexington, MA 02421.
- (47) Consists of 10,816 shares of common stock underlying options held by the selling stockholder that are exercisable as of August 23, 2018 and January 22, 2019 and 197 shares of common stock underlying a restricted stock unit award held by the selling stockholder that were distributed to the selling stockholder on January 22, 2019 and 2,646 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 44 Glen Road, Winchester, MA 01890. The selling stockholder served as Vice President, Head of Global Marketing of Agilis prior to the acquisition and currently serves as our Product Owner for our commercial launch of PTC-AADC.
- (48) Consists of 641 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 333 Schermerhorn St., Apt. 45P, Brooklyn, NY 11217. The selling stockholder served as the Director of Research of Agilis prior to the acquisition.
- (49) Consists of 19,250 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 83 Church Street, Watertown, MA 02472. The selling stockholder served as the Chief Financial Officer of Agilis prior to the acquisition.
- (50) Consists of 3,689 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder

is 17 Pacific Bay Circle, Apt. 201, San Bruno, CA 94066. The selling stockholder served as the Senior Vice President, Technical Operation of Agilis prior to the acquisition.

- (51) Consists of 7,425 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 400 Walnut St., Suite 1201, Des Moines, IA, 50309. The selling stockholder served as the Chief Medical Officer of Agilis prior to the acquisition.
- (52) Consists of 10 shares of common stock purchased by the selling stockholder on October 3, 2018 and 7,425 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 72 Hollister Drive, Avon, CT 06001. The selling stockholder served as the Chief Commercial Officer of Agilis prior to the acquisition.
- (53) Consists of 6,614 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 7401 Rio Grande NW, Albuquerque, NM 87107.
- (54) Consists of 3,368 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 341 Brookline Blvd., Havertown, PA 19083. The selling stockholder served as the Vice President, Regulatory Affairs of Agilis prior to the acquisition and served as our Vice President, Global Regulatory Affairs, Gene Therapy until October 2019.
- (55) Consists of 118 shares of common stock underlying a restricted stock unit award held by the selling stockholder that were distributed on January 22, 2020 and 3,327 shares of common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 4 SkyLine Drive, Winchester, MA 01890. The selling stockholder served as the Senior Vice President and Head of Research of Agilis prior to the acquisition and served as our Vice President and Head of Gene Therapy Non-Clinical Research until May 2020.
- (56) Consists of 1,088 shares of our common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 15 Fox Hill Circle, Marshfield, MA 02050. The selling stockholder served as the Senior Director, Global Marketing of Agilis prior to the acquisition and served as our Executive Director, Global Shared Medical Services until February 2020.
- (57) Consists of 2,646 shares of our common stock issued to the selling stockholder pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 9 East Belleview Dr., Greenwood Village, CO 80121. The selling stockholder served as the Vice President of Agilis prior to the acquisition and currently serves as a consultant for our gene therapy program.
- (58) Consists of 2,624 shares of our common stock issued pursuant to the Rights Exchange Agreement and registered hereby. The address of the selling stockholder is 22 Chestnut Avenue, Larchmont, NY 10538.

#### **Relationships with Selling Stockholders**

None of the selling stockholders has had a material relationship with us or any of our subsidiaries within the past three years except as set forth below or in the table above or the notes thereto.

None of the selling stockholders has held any position or office with us or any of our subsidiaries within the past three years, except for the positions or offices held by the selling stockholders indicated below or in the table above or the notes thereto.

In connection with our acquisition of Agilis, we entered into employment agreements with Mark Pykett, Jodi Ann Cook, Anne Marie Conway, Antonio Arulanandam, Gregory Fuest, Kevin O'Brien, Nancy Kribbs and Christina Ansted, each of whom is a former Agilis employee and selling stockholder. Mr. O'Brien, Ms. Kribbs, Mr. Arulanandam and Ms. Ansted, or collectively the Former PTC Employees, are no longer employed by us. During their employments, pursuant to their respective employment agreements, each of the Former PTC Employees received an annual base salary, was eligible to receive an annual bonus and equity grants beginning in 2019 and received inducement grants for non-statutory stock options pursuant to the Nasdaq inducement grant exception on the closing date of our acquisition of Agilis. Any equity grants that were awarded to the Former PTC Employees and have not vested and/or been exercised, as applicable, have been forfeited and are no longer outstanding.

Ms. Cook is no longer employed by us and now serves as our consultant. Pursuant to her consultant agreement, Ms. Cook receives a monthly consultant fee. While Ms. Cook was employed with us, she received an annual base salary and stock option grants for an aggregate of 125,000 shares of our common stock in connection with an inducement grant for non-statutory stock options pursuant to the Nasdaq inducement grant exception on the closing date of our acquisition of Agilis and an annual equity grant of stock options. Pursuant to her consultant agreement, these stock options remain outstanding.

Mr. Pykett, Ms. Conway and Mr. Fuest are currently employed by us. Mr. Pykett is currently our Chief Scientific Officer. Under his employment agreement, Mr. Pykett receives an annual base salary and has been eligible to receive an annual bonus and equity grant since 2019. Pursuant to his employment agreement, Mr. Pykett has received stock option grants for an aggregate of 204,250 shares of our common stock in connection with an inducement grant for non-statutory stock options pursuant to the Nasdaq inducement grant exception on the closing date of our acquisition of Agilis and annual equity grants. Mr. Pykett also received 9,300 restricted stock units in connection with an annual equity grant. Ms. Conway is currently our Clinical Head of Biologics. Under her employment agreement, Ms. Conway receives an annual base salary and has been eligible to receive an annual bonus and equity grant since 2019. Pursuant to her employment agreement, Ms. Conway has received stock option grants for an aggregate of 34,742 shares of our common stock in connection with an inducement grant for non-statutory stock options pursuant to the Nasdaq inducement grant exception on the closing date of our acquisition of Agilis and annual equity grants. Ms. Conway also received 1,661 restricted stock units in connection with her annual equity grants. Mr. Fuest is currently our Product Owner for the commercial launch of PTC-AADC. Under his employment agreement, Mr. Fuest receives an annual base salary and has been eligible to receive an annual bonus and equity grant since 2019. Pursuant to his employment agreement, Mr. Fuest has received stock option grants for an aggregate of 40,727 shares of our common stock in connection with an inducement grant for non-statutory stock options pursuant to the Nasdaq inducement grant exception on the closing date of our acquisition of Agilis and annual equity grants. Mr. Fuest also received 2,676 restricted stock units in connection with his annual equity grants. The employment agreements with Mr. Pykett, Ms. Conway and Mr. Fuest and the consultant agreement with Ms. Cook also include certain other customary terms.

In addition to the shares of common stock being registered hereby, each of the selling stockholders received its pro rata portion of the cash consideration in connection with the closing of the acquisition of Agilis. In addition, each of the selling stockholders received its pro rata portion of the cash consideration in connection with the execution of the Rights Exchange Agreement and will also be entitled to receive its pro rata portion of the portions of the milestone payments and annual net sales for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement. See the discussion under the heading "Exchange of Rights of Certain Former Equityholders of Agilis Biotherapeutics, Inc." above, our Current Report on Form 8-K filed with the SEC on July 19, 2018, and our Form 8-K filed with the SEC on April 30, 2020 for more details with respect to the consideration, including contingent consideration, that has been paid or may become payable to the selling stockholders in connection with the acquisition and the consideration that has been paid to the selling stockholders in connection with the Rights Exchange Agreement.

Under the Rights Exchange Agreement, we have agreed to indemnify and hold harmless each selling stockholder against any losses, claims, damages, expenses or liabilities to which a selling stockholder may become subject by reason of any untrue statement of material fact contained in this registration statement or any omission to state in this registration statement a fact required to be stated or necessary to make the statements in this registration statement not misleading, except to the extent that such losses, claims, damages, expenses or liabilities arise out of or are based upon information furnished to us by or on behalf of us or a selling stockholder for use in this registration statement. Each selling stockholder has agreed to indemnify and hold us and each of our directors and officers harmless against any losses, claims, damages, expenses or liabilities (including reasonable attorney's fees) to which such indemnified parties may become subject by reason of any statement or omission in this registration statement made in reliance upon, or in conformity with, a written statement by such selling stockholder furnished pursuant to the Rights Exchange Agreement.

Under the Rights Exchange Agreement, we have agreed to pay all expenses incurred by us in connection with the filing of this registration statement, including all registration and filing fees, exchange listing fees, fees and expenses of our counsel, and fees and expenses of our accountants, but excluding (i) any brokerage fees, selling commissions or underwriting spread or discounts incurred by the selling stockholders in connection with sales under this registration statement and (ii) the fees and expenses of any counsel retained by or on behalf of the selling stockholders.

The foregoing descriptions of the Merger Agreement and the Rights Exchange Agreement are summaries only and are qualified in their entirety by reference to the terms of the Merger Agreement and the Rights Exchange Agreement, included as Exhibit 2.1 and Exhibit 10.1 hereto, respectively.

## DESCRIPTION OF CAPITAL STOCK

The following description of our capital stock is intended as a summary only. This description is based upon, and is qualified by reference to, our restated certificate of incorporation, our amended and restated bylaws and applicable provisions of Delaware corporate law. This summary is not complete. You should read our restated certificate of incorporation and amended and restated bylaws, which are filed as exhibits to the registration statement of which this prospectus forms a part, for the provisions that are important to you.

Our authorized capital stock consists of 125,000,000 shares of common stock and 5,000,000 shares of preferred stock. As of April 28, 2020, there were 62,875,395 shares of common stock outstanding and no shares of preferred stock outstanding.

### Common Stock

*Voting Rights.* Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. Each election of directors by our stockholders will be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. In general, except (1) for the election of directors, (2) as described below under "—Provisions of Our Certificate of Incorporation and Bylaws and Delaware Law That May Have Anti-Takeover Effects—Super-Majority Voting," (3) in the future to the extent that we have two or more classes or series of stock outstanding with separate voting rights and (4) as otherwise required by law, any matter to be voted on by our stockholders at any meeting is decided by the vote of the holders of a majority in voting power of the votes cast by the holders of shares of our stock present or represented at the meeting and voting affirmatively or negatively on such matter.

*Dividends.* Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights of outstanding preferred stock.

*Liquidation and Dissolution.* In the event of our liquidation or dissolution, the holders of our common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any of our outstanding preferred stock.

*Other Rights.* Holders of our common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of our common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of our preferred stock that we may designate and issue in the future.

*Transfer Agent and Registrar.* American Stock Transfer & Trust Company, LLC is the transfer agent and registrar for our common stock.

*Nasdaq Global Select Market.* Our common stock is listed on The Nasdaq Global Select Market under the symbol "PTCT."

## **Preferred Stock**

Under the terms of our certificate of incorporation, our board of directors is authorized to issue shares of preferred stock in one or more series without stockholder approval, subject to any limitations imposed by the Nasdaq Marketplace Rules. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock. The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock. Currently, we have no shares of preferred stock outstanding.

## **Stock Options and Warrants**

As of March 31, 2020, we had (i) issued and outstanding options to purchase 12,670,068 shares of our common stock at a weighted average exercise price of \$34.95 per share and (ii) 927,151 shares of our common stock issuable upon the vesting of outstanding restricted stock awards and restricted stock units.

## **Convertible Notes**

In August 2015, we issued, at par value, \$150.0 million aggregate principal amount of 3.0% convertible senior notes due 2022, or the 2022 Convertible Notes. The 2022 Convertible Notes bear cash interest at a rate of 3.0% per year, payable semi-annually on February 15 and August 15 of each year, beginning on February 15, 2016. The 2022 Convertible Notes will mature on August 15, 2022, unless earlier repurchased or converted. The conversion rate for the 2022 Convertible Notes was initially, and remains, 17.7487 shares of our common stock per \$1,000 principal amount of the 2022 Convertible Notes, which is equivalent to an initial conversion price of approximately \$56.34 per share of our common stock.

In September 2019, we issued, at par value, \$287.5 million aggregate principal amount of 1.5% convertible senior notes due 2026, or the 2026 Convertible Notes. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. The conversion rate for the 2026 Convertible Notes was initially, and remains, 19.0404 shares of our common stock per \$1,000 principal amount of the 2026

Convertible Notes, which is equivalent to an initial conversion price of approximately \$52.52 per share of our common stock.

### **Effects of Authorized but Unissued Stock**

We have shares of common stock and preferred stock available for future issuance without stockholder approval, subject to any limitations imposed by the listing standards of the Nasdaq Global Select Market. We may utilize these additional shares for a variety of corporate purposes, including for future public offerings to raise additional capital or facilitate corporate acquisitions or for payment as a dividend on our capital stock. The existence of unissued and unreserved common stock and preferred stock may enable our board of directors to issue shares to persons friendly to current management or to issue preferred stock with terms that could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a controlling interest in our company by means of a merger, tender offer, proxy contest or otherwise. In addition, if we issue preferred stock, the issuance could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon liquidation.

### **Provisions of Our Certificate of Incorporation and Bylaws and Delaware Law That May Have Anti-Takeover Effects**

*Delaware Law.* We are subject to Section 203 of the Delaware General Corporation Law. Subject to certain exceptions, Section 203 prevents a publicly held Delaware corporation from engaging in a "business combination" with any "interested stockholder" for three years following the date that the person became an interested stockholder, unless either the interested stockholder attained such status with the approval of our board of directors, the business combination is approved by our board of directors and stockholders in a prescribed manner or the interested stockholder acquired at least 85% of our outstanding voting stock in the transaction in which it became an interested stockholder. A "business combination" includes, among other things, a merger or consolidation involving us and the "interested stockholder" and the sale of more than 10% of our assets. In general, an "interested stockholder" is any entity or person beneficially owning 15% or more of our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person. The restrictions contained in Section 203 are not applicable to any of our existing stockholders that owned 15% or more of our outstanding voting stock upon the closing of our initial public offering.

*Staggered Board; Removal of Directors.* Our certificate of incorporation and our bylaws divide our board of directors into three classes with staggered three-year terms. In addition, our certificate of incorporation and our bylaws provide that directors may be removed only for cause and only by the affirmative vote of the holders of 75% of our shares of capital stock present in person or by proxy and entitled to vote. Under our certificate of incorporation and bylaws, any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office. Furthermore, our certificate of incorporation provides that the authorized number of directors may be changed only by the resolution of our board of directors. The classification of our board of directors and the limitations on the ability of our stockholders to remove directors, change the authorized number of directors and fill vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

*Stockholder Action; Special Meeting of Stockholders; Advance Notice Requirements for Stockholder Proposals and Director Nominations.* Our certificate of incorporation and our bylaws provide that any action required or permitted to be taken by our stockholders at an annual meeting or special meeting of stockholders may only be taken if it is properly brought before such meeting and may not be taken by written action in lieu of a meeting. Our certificate of incorporation and our bylaws also provide that, except as otherwise required by law, special meetings of the stockholders can only be called by our



chairman of the board, our president or chief executive officer or our board of directors. In addition, our bylaws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. Stockholders at an annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors, or by a stockholder of record on the record date for the meeting, who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities. These provisions also could discourage a third party from making a tender offer for our common stock, because even if it acquired a majority of our outstanding voting stock, it would be able to take action as a stockholder, such as electing new directors or approving a merger, only at a duly called stockholders meeting and not by written consent.

*Super-Majority Voting.* The Delaware General Corporation Law provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our bylaws may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in any election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate of incorporation described above.

### **Directors' Liability**

Our certificate of incorporation limits the personal liability of directors for breach of fiduciary duty to the maximum extent permitted by the Delaware General Corporation Law and provides that no director will have personal liability to us or to our stockholders for monetary damages for breach of fiduciary duty or other duty as a director. However, these provisions do not eliminate or limit the liability of any of our directors:

- for any breach of the director's duty of loyalty to us or our stockholders;
- for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law;
- for voting or assenting to unlawful payments of dividends, stock repurchases or other distributions; or
- for any transaction from which the director derived an improper personal benefit.

Any amendment to or repeal of these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to such amendment or repeal. If the Delaware General Corporation Law is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited to the greatest extent permitted by the Delaware General Corporation Law.

In addition, our certificate of incorporation provides that we must indemnify our directors and officers and we must advance expenses, including attorneys' fees, to our directors and officers in connection with legal proceedings, subject to very limited exceptions.

We maintain a general liability insurance policy that covers certain liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers. In addition, we have entered into indemnification agreements with our directors. These indemnification

agreements may require us, among other things, to indemnify each such director for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by him in any action or proceeding arising out of his service as one of our directors.

Certain of our non-employee directors may, through their relationships with their employers, be insured and/or indemnified against certain liabilities incurred in their capacity as members of our board of directors.

## PLAN OF DISTRIBUTION

The shares covered by this prospectus may be offered and sold from time to time by the selling stockholders. The term "selling stockholders" includes donees, pledgees, assignees, transferees or other successors-in-interest selling shares received after the date of this prospectus from a selling stockholder as a gift, pledge, partnership distribution or other non-sale related transfer. The selling stockholders will act independently of us in making decisions with respect to the timing, manner and size of each sale. Such sales may be made on one or more exchanges in any market or trading facility on which the shares are traded, or in the over-the-counter market or otherwise, at fixed prices, at prices and under terms then prevailing or at prices related to the then current market price or in negotiated transactions. The selling stockholders may sell their shares by one or more of, or a combination of, the following methods:

- purchases by a broker-dealer as principal and resale by such broker-dealer for its own account pursuant to this prospectus;
- ordinary brokerage transactions and transactions in which the broker solicits purchasers;
- block trades in which the broker-dealer so engaged will attempt to sell the shares as agent but may position and resell a portion of the block as principal to facilitate the transaction;
- an over-the-counter distribution in accordance with the rules of The Nasdaq Global Select Market;
- through one or more underwritten offerings on a firm commitment or best efforts basis;
- in privately negotiated transactions; and
- in options transactions.

In addition, any shares that qualify for sale pursuant to Rule 144 may be sold under Rule 144 rather than pursuant to this prospectus.

To the extent required, this prospectus may be amended or supplemented from time to time to describe a specific plan of distribution. In connection with distributions of the shares or otherwise, the selling stockholders may enter into hedging transactions with broker-dealers or other financial institutions. In connection with such transactions, broker-dealers or other financial institutions may engage in short sales of the common stock in the course of hedging the positions they assume with selling stockholders. The selling stockholders may also sell the common stock short and redeliver the shares to close out such short positions. The selling stockholders may also enter into option or other transactions with broker-dealers or other financial institutions which require the delivery to such broker-dealer or other financial institution of shares offered by this prospectus, which shares such broker-dealer or other financial institution may resell pursuant to this prospectus. The selling stockholders may also pledge or grant a security interest in shares to a broker-dealer, other financial institution or other person, and, upon a default such pledgee or secured parties may effect sales of the pledged shares pursuant to this prospectus.

In effecting sales, broker-dealers or agents engaged by the selling stockholders may arrange for other broker-dealers to participate. Broker-dealers or agents may receive commissions, discounts or

concessions from the selling stockholders in amounts to be negotiated immediately prior to the sale. Except as set forth in this prospectus under the heading "Selling Stockholders," each selling stockholder has informed us that it is not a registered broker-dealer or an affiliate of a registered broker-dealer.

In offering the shares covered by this prospectus, the selling stockholders and any broker-dealers who execute sales for the selling stockholders may be deemed to be "underwriters" within the meaning of the Securities Act in connection with such sales. Any profits realized by the selling stockholders and the compensation of any broker-dealer may be deemed to be underwriting discounts and commissions. Any selling stockholder who is an "underwriter" within the meaning of the Securities Act will be subject to the prospectus delivery requirements of the Securities Act and the provisions of the Exchange Act and the rules thereunder relating to stock manipulation.

In order to comply with the securities laws of certain states, if applicable, the shares must be sold in such jurisdictions only through registered or licensed brokers or dealers. In addition, in certain states the shares may not be sold unless they have been registered or qualified for sale in the applicable state or an exemption from the registration or qualification requirement is available and is complied with.

We have advised the selling stockholders that the anti-manipulation rules of Regulation M under the Exchange Act may apply to sales of shares in the market and to the activities of the selling stockholders and their affiliates. In addition, we will make copies of this prospectus available to the selling stockholders for the purpose of satisfying the prospectus delivery requirements of the Securities Act. The selling stockholders may indemnify any broker-dealer that participates in transactions involving the sale of the shares against certain liabilities, including liabilities arising under the Securities Act.

At the time a particular offer of shares is made, if required, a prospectus supplement will be distributed that will set forth the number of shares being offered and the terms of the offering, including the name of any underwriter, dealer or agent, the purchase price paid by any underwriter, any discount, commission and other item constituting compensation, any discount, commission or concession allowed or reallowed or paid to any dealer, and the proposed selling price to the public.

We have agreed to indemnify the selling stockholders against certain liabilities, including certain liabilities under the Securities Act.

We have agreed with the selling stockholders to keep the Registration Statement of which this prospectus constitutes a part effective until the earlier of (i) such time as all of the shares covered by this prospectus have been disposed of pursuant to and in accordance with the Registration Statement or (ii) October 29, 2020.

## LEGAL MATTERS

The validity of the shares offered by this prospectus has been passed upon by Wilmer Cutler Pickering Hale and Dorr LLP.

## EXPERTS

Ernst & Young LLP, independent registered public accounting firm, has audited our consolidated financial statements included in our [Annual Report on Form 10-K for the year ended December 31, 2019](#), and the effectiveness of our internal control over financial reporting as of December 31, 2019, as set forth in their reports, which are incorporated by reference in this prospectus and elsewhere in the registration statement. Our financial statements are incorporated by reference in reliance on Ernst & Young LLP's reports, given on their authority as experts in accounting and auditing.

The financial statements of Agilis as of and for the years ended December 31, 2017 and 2016, incorporated in this prospectus by reference from our [Current Report on Form 8-K filed August 24, 2018](#) have been audited by BDO USA, LLP, independent auditors, as stated in their report, which is incorporated herein by reference. Such financial statements have been so incorporated in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

### WHERE YOU CAN FIND MORE INFORMATION

We file annual, quarterly and current reports, proxy statements and other information with the SEC. Our SEC filings are available to the public over the Internet at the SEC's website at <http://www.sec.gov>. Copies of certain information filed by us with the SEC are also available on our website at <http://www.ptcbio.com>. Our website is not a part of this prospectus and is not incorporated by reference in this prospectus. You may also read and copy any document we file at the SEC's Public Reference Room, 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the Public Reference Room.

This prospectus is part of a registration statement we filed with the SEC. This prospectus omits some information contained in the registration statement in accordance with SEC rules and regulations. You should review the information and exhibits in the registration statement for further information on us and our consolidated subsidiaries and the securities we are offering. Statements in this prospectus concerning any document we filed as an exhibit to the registration statement or that we otherwise filed with the SEC are not intended to be comprehensive and are qualified by reference to these filings. You should review the complete document to evaluate these statements. You can obtain a copy of the registration statement from the SEC at the address listed above or from the SEC's website.

### INCORPORATION BY REFERENCE

The SEC allows us to incorporate by reference in this prospectus much of the information we file with the SEC, which means that we can disclose important information to you by referring you to those publicly available documents. The information that we incorporate by reference in this prospectus is considered to be part of this prospectus. Because we are incorporating by reference future filings with the SEC, this prospectus is continually updated and those future filings may modify or supersede some of the information included or incorporated in this prospectus. This means that you must look at all of the SEC filings that we incorporate by reference to determine if any of the statements in this prospectus or in any document previously incorporated by reference have been modified or superseded. This prospectus incorporates by reference the documents listed below (File No. 001-35969) and any future filings we make with the SEC under Sections 13(a), 13(c), 14 or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act (in each case, other than those documents or the portions of those documents not deemed to be filed) until the offering of the securities under the registration statement is terminated or completed:

- [Annual Report on Form 10-K for the fiscal year ended December 31, 2019](#), including the information specifically incorporated by reference into the Annual Report on Form 10-K from our definitive proxy [statement for the 2020 Annual Meeting of Stockholders, filed on April 27, 2020](#);
- [Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2020](#);
- Current Reports on Form 8-K filed on [August 24, 2018](#) (solely with respect to Item 9.01 and Exhibits 99.3 and 99.4), [January 17, 2020](#) (excluding Item 7.01 and Exhibit 99.1), [March 16, 2020](#) (excluding Item 7.01 and Exhibit 99.1), [April 30, 2020](#) (Film #20835722) and [May 6, 2020](#) (excluding Item 7.01 and Exhibit 99.1); and

- [The description of the securities contained in our registration statement on Form 8-A filed under the Exchange Act on June 14, 2013, including any amendment or report filed for the purpose of updating such description.](#)

A statement contained in a document incorporated by reference into this prospectus shall be deemed to be modified or superceded for purposes of this prospectus to the extent that a statement contained in this prospectus, any prospectus supplement or in any other subsequently filed document which is also incorporated in this prospectus modifies or replaces such statement. Any statements so modified or superceded shall not be deemed, except as so modified or superceded, to constitute a part of this prospectus.

You may request a copy of these filings, at no cost, by writing or telephoning us at the following address or phone number:

PTC Therapeutics, Inc.  
100 Corporate Court  
South Plainfield, New Jersey 07080  
(908) 222-7000

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**PTC THERAPEUTICS, INC.**

**Common Stock**

**PROSPECTUS**

**May 7, 2020**

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**PART II.**  
**INFORMATION NOT REQUIRED IN PROSPECTUS**

**Item 14. Other Expenses of Issuance and Distribution.**

The following table sets forth the various expenses to be incurred in connection with the sale and distribution of the securities being registered hereby, all of which will be borne by the Registrant (except any underwriting discounts and commissions and expenses incurred by the selling stockholders for brokerage, accounting, tax or legal services or any other expenses incurred by the selling stockholders in disposing of the shares). All amounts shown are estimates except the SEC registration fee.

SEC registration fee	\$ 17,445.23
Legal fees and expenses	\$ 50,000
Accounting fees and expenses	\$ 46,000
Miscellaneous expenses	\$ 6,000
Total expenses	<u>\$ 119,445.23</u>

**Item 15. Indemnification of Directors and Officers.**

Section 102 of the Delaware General Corporation Law permits a corporation to eliminate the personal liability of its directors or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his or her duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit. The Registrant's certificate of incorporation provides that no director shall be personally liable to it or its stockholders for monetary damages for any breach of fiduciary duty as a director, notwithstanding any provision of law imposing such liability, except to the extent that the Delaware General Corporation Law prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145 of the Delaware General Corporation Law provides that a corporation has the power to indemnify a director, officer, employee or agent of the corporation and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he or she is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnify for such expenses which the Court of Chancery or such other court shall deem proper.

The Registrant's certificate of incorporation provides that it will indemnify each person who was or is a party or threatened to be made a party to any threatened, pending or completed action, suit or proceeding whether civil, criminal, administrative or investigative (other than an action by or in the right of us) by reason of the fact that he or she is or was, or has agreed to become, the Registrant's director or officer, or is or was serving, or has agreed to serve, at the Registrant's request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (all such persons being referred to as an "Indemnitee"), or by

reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding and any appeal therefrom, if such Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, the Registrant's best interests, and, with respect to any criminal action or proceeding, he or she had no reasonable cause to believe his or her conduct was unlawful.

The Registrant's certificate of incorporation also provides that the Registrant will indemnify any Indemnitee who was or is a party to an action or suit by or in the right of the Registrant to procure a judgment in its favor by reason of the fact that the Indemnitee is or was, or has agreed to become, the Registrant's director or officer, or is or was serving, or has agreed to serve, at the Registrant's request as a director, officer, partner, employee or trustee or, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise, or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees) and, to the extent permitted by law, amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding, and any appeal therefrom, if the Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, the Registrant's best interests, except that no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the Registrant, unless a court determines that, despite such adjudication but in view of all of the circumstances, he or she is entitled to indemnification of such expenses. Notwithstanding the foregoing, to the extent that any Indemnitee has been successful, on the merits or otherwise, he or she will be indemnified by the Registrant against all expenses (including attorneys' fees) actually and reasonably incurred by him or her or on his or her behalf in connection therewith. If the Registrant does not assume the defense, expenses must be advanced to an Indemnitee under certain circumstances.

The Registrant has entered into indemnification agreements with its directors and executive officers. In general, these agreements provide that the Registrant will indemnify the director or executive officer to the fullest extent permitted by law for claims arising in his or her capacity as a director or officer of the Registrant or in connection with their service at the Registrant's request for another corporation or entity. The indemnification agreements also provide for procedures that will apply in the event that a director or executive officer makes a claim for indemnification and establish certain presumptions that are favorable to the director or executive officer.

The Registrant maintains a general liability insurance policy which covers certain liabilities of its directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers.

Any underwriting agreement Registrant enters into in connection with an offering of common stock being registered under this registration statement may provide that the underwriters will indemnify, under certain conditions, Registrant and Registrant's directors and officers (as well as certain other persons) against certain liabilities arising in connection with such offering.

**Item 16. Exhibits.**

<u>Exhibit No.</u>	<u>Description</u>
2.1†	<a href="#">Agreement and Plan of Merger, dated July 19, 2018, by and among PTC Therapeutics, Inc., Agility Merger Sub, Inc., Agilis Biotherapeutics, Inc. and, solely in its capacity as equityholder representative, Shareholder Representative Services LLC (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on July 19, 2018)</a>



<u>Exhibit No.</u>	<u>Description</u>
4.2	<a href="#">Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.1 to the Current Report on Form 8-K filed by the Registrant on April 21, 2017)</a>
5.1	<a href="#">Opinion of Wilmer Cutler Pickering Hale and Dorr LLP</a>
10.1†	<a href="#">Rights Exchange Agreement, dated April 29, 2020, by and among PTC Therapeutics, Inc. and the Persons listed on Schedule 1 thereto (incorporated by reference as Exhibit 10.1 to the Form 8-K filed by the Registrant on April 30, 2020)</a>
23.1	<a href="#">Consent of Ernst &amp; Young LLP, independent registered public accounting firm for the Registrant</a>
23.2	<a href="#">Consent of BDO USA, LLP</a>
23.3	<a href="#">Consent of Wilmer Cutler Pickering Hale and Dorr LLP (included in Exhibit 5.1)</a>
24.1	<a href="#">Powers of Attorney (included on the signature page to the Registration Statement)</a>

† Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

**Item 17. Undertakings.**

The undersigned Registrant hereby undertakes:

- (1) To file, during any period in which offers or sales are being made, a post-effective amendment to this registration statement:
  - (i) to include any prospectus required by Section 10(a)(3) of the Securities Act of 1933, as amended (the "Securities Act");
  - (ii) to reflect in the prospectus any facts or events arising after the effective date of this registration statement (or the most recent post-effective amendment thereof) which, individually or in the aggregate, represent a fundamental change in the information set forth in this registration statement. Notwithstanding the foregoing, any increase or decrease in volume of securities offered (if the total dollar value of securities offered would not exceed that which was registered) and any deviation from the low or high end of the estimated maximum offering range may be reflected in the form of prospectus filed with the Securities and Exchange Commission pursuant to Rule 424(b) if, in the aggregate, the changes in volume and price represent no more than a 20 percent change in the maximum aggregate offering price set forth in the "Calculation of Registration Fee" table in the effective registration statement; and
  - (iii) to include any material information with respect to the plan of distribution not previously disclosed in this registration statement or any material change to such information in this registration statement;

*provided, however,* that paragraphs (1)(i), (1)(ii) and (1)(iii) do not apply if the information required to be included in a post-effective amendment by those paragraphs is contained in reports filed with or furnished to the Securities and Exchange Commission by the Registrant pursuant to Section 13 or Section 15(d) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), that are incorporated by reference in this registration statement, or is contained in a form of prospectus filed pursuant to Rule 424(b) that is part of this registration statement.

- (2) That, for the purpose of determining any liability under the Securities Act, each such post-effective amendment shall be deemed to be a new registration statement relating to the

securities offered therein, and the offering of such securities at that time shall be deemed to be the initial *bona fide* offering thereof.

- (3) To remove from registration by means of a post-effective amendment any of the securities being registered which remain unsold at the termination of the offering.
- (4) That, for the purpose of determining liability under the Securities Act to any purchaser:
  - (i) each prospectus filed by the Registrant pursuant to Rule 424(b)(3) shall be deemed to be part of the registration statement as of the date the filed prospectus was deemed part of and included in the registration statement; and
  - (ii) each prospectus required to be filed pursuant to Rule 424(b)(2), (b)(5), or (b)(7) as part of a registration statement in reliance on Rule 430B relating to an offering made pursuant to Rule 415(a)(1)(i), (vii) or (x) for the purpose of providing the information required by Section 10(a) of the Securities Act shall be deemed to be part of and included in the registration statement as of the earlier of the date such form of prospectus is first used after effectiveness or the date of the first contract of sale of securities in the offering described in the prospectus. As provided in Rule 430B, for liability purposes of the issuer and any person that is at that date an underwriter, such date shall be deemed to be a new effective date of the registration statement relating to the securities in the registration statement to which that prospectus relates, and the offering of such securities at that time shall be deemed to be the initial *bona fide* offering thereof. *Provided, however*, that no statement made in a registration statement or prospectus that is part of the registration statement or made in a document incorporated or deemed incorporated by reference in the registration statement or prospectus that is part of the registration statement will, as to a purchaser with a time of contract of sale prior to such effective date, supersede or modify any statement that was made in the registration statement or prospectus that was part of the registration statement or made in any such document immediately prior to such effective date.
- (5) That, for purposes of determining any liability under the Securities Act, each filing of the Registrant's annual report pursuant to Section 13(a) or 15(d) of the Exchange Act (and, where applicable, each filing of an employee benefit plan's annual report pursuant to Section 15(d) of the Exchange Act) that is incorporated by reference in this registration statement shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial *bona fide* offering thereof.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the Securities and Exchange Commission such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.



<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>/s/ ALLAN JACOBSON, PH.D.</u> Allan Jacobson, Ph.D.	Director	May 7, 2020
<u>/s/ STEPHANIE S. OKEY, M.S.</u> Stephanie S. Okey, M.S.	Director	May 7, 2020
<u>/s/ EMMA REEVE</u> Emma Reeve	Director	May 7, 2020
<u>/s/ DAVID P. SOUTHWELL</u> David P. Southwell	Director	May 7, 2020
<u>/s/ GLENN D. STEELE, JR., M.D., PH.D.</u> Glenn D. Steele, Jr., M.D., Ph.D.	Director	May 7, 2020
<u>/s/ DAWN SVORONOS</u> Dawn Svoronos	Director	May 7, 2020
<u>/s/ JEROME B. ZELDIS, M.D., PH.D.</u> Jerome B. Zeldis, M.D., Ph.D.	Director	May 7, 2020



May 7, 2020

PTC Therapeutics, Inc.  
100 Corporate Court  
South Plainfield, New Jersey 07080

Re: Registration Statement on Form S-3

Ladies and Gentlemen:

This opinion is furnished to you in connection with a Registration Statement on Form S-3 (the "Registration Statement") filed by PTC Therapeutics, Inc., a Delaware corporation (the "Company"), with the Securities and Exchange Commission (the "Commission") under the Securities Act of 1933, as amended (the "Securities Act"), for the registration for resale of an aggregate of 2,821,176 shares of common stock, par value \$0.001 per share (the "Shares"), of the Company. All of the Shares are being registered on behalf of certain stockholders of the Company (the "Selling Stockholders").

We are acting as counsel for the Company in connection with the registration for resale of the Shares. We have examined and relied upon signed copies of the Registration Statement as filed with the Commission, including the exhibits thereto. We have also examined and relied upon the Certificate of Incorporation of the Company (as amended or restated from time to time, the "Certificate of Incorporation"), the Bylaws of the Company (as amended or restated from time to time, the "Bylaws") and minutes of meetings of the stockholders and the Board of Directors of the Company as provided to us by the Company, and such other documents as we have deemed necessary for purposes of rendering the opinions hereinafter set forth.

In our examination of the foregoing documents, we have assumed the genuineness of all signatures, the authenticity of all documents submitted to us as originals, the conformity to original documents of all documents submitted to us as copies, the authenticity of the originals of such latter documents and the legal competence of all signatories to such documents.

Our opinion below, insofar as it relates to the Shares being fully paid, is based solely on a certificate of the Chief Financial Officer of the Company confirming the Company's receipt of the consideration called for by the applicable resolutions authorizing the issuance of such Shares.

We assume that the appropriate action will be taken, prior to the offer and sale of the Shares, to register and qualify the Shares for sale under all applicable state securities or "blue sky" laws.

We express no opinion herein as to the laws of any state or jurisdiction other than the General Corporation Law of the State of Delaware.

Based upon and subject to the foregoing, we are of the opinion that the Shares have been duly authorized and are validly issued, fully paid and nonassessable.

It is understood that this opinion is to be used only in connection with the offer and sale of the Shares while the Registration Statement is in effect.

Please note that we are opining only as to the matters expressly set forth herein, and no opinion should be inferred as to any other matters. This opinion is based upon currently existing statutes, rules, regulations and judicial decisions, and we disclaim any obligation to advise you of any change in any of these sources of law or subsequent legal or factual developments which might affect any matters or opinions set forth herein.

We hereby consent to the filing of this opinion with the Commission as an exhibit to the Registration Statement in accordance with the requirements of Item 601(b)(5) of Regulation S-K under the Securities Act and to the use of our name therein and in the related prospectus under the caption "Legal Matters." In giving such consent, we do not hereby admit that we are in the category of persons whose consent is required under Section 7 of the Securities Act or the rules and regulations of the Commission.

Very truly yours,

WILMER CUTLER PICKERING  
HALE AND DORR LLP

By: /s/ BRIAN A. JOHNSON

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Brian A. Johnson, a Partner

QuickLinks

[Exhibit 5.1](#)



**Consent of Independent Registered Public Accounting Firm**

We consent to the reference to our firm under the caption "Experts" in the Registration Statement (Form S-3) and related Prospectus of PTC Therapeutics, Inc. for the registration of common stock and to the incorporation by reference therein of our reports dated March 2, 2020, with respect to the consolidated financial statements of PTC Therapeutics, Inc. and the effectiveness of internal control over financial reporting of PTC Therapeutics, Inc. included in its Annual Report (Form 10-K) for the year ended December 31, 2019, filed with the Securities and Exchange Commission.

/s/ Ernst & Young LLP

Iselin, New Jersey  
May 7, 2020

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QuickLinks

[Exhibit 23.1](#)

[Consent of Independent Registered Public Accounting Firm](#)

**Consent of Independent Certified Public Accounting Firm**

PTC Therapeutics, Inc.  
South Plainfield, New Jersey

We hereby consent to the incorporation by reference in the Prospectus constituting a part of this Registration Statement of PTC Therapeutics, Inc. of our report dated August 21, 2018, relating to the consolidated financial statements of Agilis Biotherapeutics, Inc. ("the Company") as of and for the years ended December 31, 2017 and 2016 appearing in PTC Therapeutics, Inc.'s Form 8-K filed on August 24, 2018. Our report contains an explanatory paragraph regarding the Company's ability to continue as a going concern.

We also consent to the reference to us under the caption "Experts" in the Prospectus.

/s/ BDO USA, LLP  
Boston, Massachusetts

May 7, 2020

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QuickLinks

[Exhibit 23.2](#)

[Consent of Independent Certified Public Accounting Firm](#)