

PTC THERAPEUTICS, INC.
Form S-3ASR
August 24, 2018

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As filed with the Securities and Exchange Commission on August 24, 2018

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)

Copies to:

**Brian A. Johnson
Wilmer Cutler Pickering Hale and Dorr LLP
7 World Trade Center
250 Greenwich Street
New York, New York 10007
Telephone: (212) 230-8800
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**Mark E. Boulding
Executive Vice President and Chief Legal Officer
PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield, New Jersey 07080-2449
Telephone: (908) 222-7000
Fax: (908) 222-1128**

**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.

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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

(Do not check if a
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 | 3,500,907 | \$43.38 | \$151,869,346 | \$18,908 |

(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 average of high and low price per share of the common stock as reported on the Nasdaq Global Select Market on August 22, 2018.

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PROSPECTUS

3,500,907 Shares

PTC Therapeutics, Inc. Common Stock

This prospectus relates to resales of shares of common stock previously issued by PTC Therapeutics, Inc. to the former stockholders of Agilis Biotherapeutics, Inc., or Agilis, in connection with our acquisition of that company.

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 August 23, 2018, the closing sale price of the common stock on the Nasdaq Global Select Market was \$44.52 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 8 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 August 24, 2018.

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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 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 19 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:

Our ability to realize the anticipated benefits of our acquisition of Agilis, including the possibility that the expected impact of benefits from the acquisition, including with respect to the business of Agilis and our expectations with respect to the potential achievement of development, regulatory and sales milestones and our contingent payments to the former Agilis equityholders with respect thereto, will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of Agilis's operations and employees into our business, our ability to obtain marketing approval of our gene therapy for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency, or GT-AADC, and other product candidates we acquired from Agilis, unknown liabilities, the risk of litigation

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and/or regulatory actions related to the acquisition, and other business effects, including the effects of industry, market, economic, political or regulatory conditions;

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 Emflaza (deflazacort) for the treatment of Duchenne muscular dystrophy, or DMD, in the United States and for Translarna (ataluren) for the treatment of nonsense mutation DMD, or nmDMD, in the European Economic Area, or EEA, and other countries in which we have or may obtain regulatory approval, or in which there exist significant reimbursed early access programs, or EAP programs;

our ability to maintain our marketing authorization of Translarna for the treatment of nmDMD in the EEA (which is subject to the specific obligation to conduct and submit the results of Study 041 to the European Medicines Agency, or EMA, and annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA);

our ability to 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 trial'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, or the Orphan Drug Act, the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act and through any grant of pediatric exclusivity;

our ability to complete the United States Food and Drug Administration, or FDA, post-marketing requirements to the marketing authorization of Emflaza or any requirements necessary to obtain any grant of pediatric exclusivity;

our expectations with respect to our acquisition of all rights to Emflaza from Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, including with respect to our ability to realize the anticipated benefits of the acquisition (including with respect to future revenue generation and contingent payments to Marathon based on annual net sales);

our ability to complete any dystrophin study necessary in order to resolve the matters set forth in the FDA's denial of our appeal to the Complete Response Letter we received from the FDA in connection with our 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 continued commercialization of Translarna as a treatment for nmDMD in the EEA or other territories outside of the United States;

our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;

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 where we are licensed to commercialize them, the potential 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;

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our estimates regarding the potential market opportunity for Translarna, Emflaza, GT-AADC, Tegsedi, Waylivra or any other product candidate, 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 of Translarna for the treatment of nmDMD, aniridia, and Dravet syndrome/CDKL5, each caused by nonsense mutations, as well as our studies in spinal muscular atrophy and our oncology program, including the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;

the rate and degree of market acceptance and clinical utility of Translarna, Emflaza, GT-AADC, Tegsedi and Waylivra, and our other product candidates;

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

the timing of, and our ability to obtain additional marketing authorizations for, Translarna and our other product candidates;

the ability of Translarna, Emflaza, GT-AADC, Tegsedi and Waylivra and our other 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 Translarna, Emflaza and other product candidates, including our ability to earn a profit from sales or licenses of Translarna for the treatment of nmDMD in the countries in which we have or may obtain regulatory approval and of Emflaza for the treatment of DMD in the United States;

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 Translarna and Emflaza and any other product candidate 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 Translarna, Emflaza and our other product candidates that are sufficient to meet clinical trial and commercial launch requirements;

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;

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our other regulatory submissions, including with respect to timing and outcome of regulatory review;

our plans to pursue development of Translarna for additional indications;

our ability to advance our earlier stage programs, including our oncology program;

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our plans to pursue research and development of other product candidates;

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 Translarna, Emflaza, GT-AADC, Tegsedi and Waylivra and any other product candidate;

our intellectual property position;

the impact of government laws and regulations;

the impact of litigation that has been brought against us and certain of our current and former officers or of litigation that we are pursuing against others;

our competitive position; and

our expectations with respect to the development and regulatory 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.

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. There are a number of important factors that could cause actual results, levels of activity, performance or events to differ materially from those expressed or implied in the forward-looking statements we make. These important factors include our "critical accounting estimates" described in Part II, Item 7 "Management's Discussion and Analysis of Financial Condition and Results of Operations Critical Accounting Policies and Significant Judgments and Estimates" of our Annual Report on Form 10-K for the fiscal year ended December 31, 2017 and the factors set forth 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, 2017, under the caption "Risk Factors" in Part II, Item 1A of our Quarterly Reports on Form 10-Q for the fiscal quarters ended March 31, 2018 and June 30, 2018, and in Exhibit 99.2 to our Current Report on Form 8-K filed on August 24, 2018. Although we may elect to update forward-looking statements in the future, we specifically disclaim any obligation to do so, even if our estimates change, and readers should not rely on those forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

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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 8 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, 2017, under the caption "Risk Factors" in Part II, Item 1A of our Quarterly Reports on Form 10-Q for the fiscal quarters ended March 31, 2018 and June 30, 2018, and in Exhibit 99.2 to our Current Report on Form 8-K filed on August 24, 2018, 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-led global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. We have launched two rare disease products and have a global commercial footprint. Our recent ability to commercialize products is the foundation that drives our continued investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. Our strategy is to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders and to leverage our global commercial infrastructure to maximize value for our patients and other stakeholders.

We have two products, Translarna (ataluren) and Emflaza (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. Translarna is currently available for the treatment of nonsense mutation DMD in over 25 countries on a commercial basis or through a reimbursed early access program, or EAP program. We hold worldwide commercialization rights to Translarna for all indications in all territories. Emflaza is approved in the United States for the treatment of DMD in patients five years and older. We also have a spinal muscular atrophy (SMA) collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. Currently, our collaboration has three clinical trials ongoing to evaluate the safety and effectiveness of risdiplam (RG7916, RO7034067), the lead compound in the SMA program. In August 2018, we acquired a pipeline of gene therapy product candidates through our acquisition of Agilis Biotherapeutics, Inc., or Agilis, and entered into a collaboration and license agreement with Akcea Therapeutics, Inc., or Akcea, for the commercialization of TEGSEDI (inotersen) and WAYLIVRA (volanesorsen) for the treatment of rare diseases in countries in Latin America and the Caribbean. In addition, we have a pipeline of product candidates that are in early clinical and pre-clinical development. Our pre-clinical and discovery programs are focused on the development of new treatments for multiple therapeutic areas, including rare diseases and oncology.

Acquisition 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, or CNS. Agilis's lead product candidate is GT-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency, a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. The Agilis product candidate pipeline also includes a gene therapy asset targeting Friedreich ataxia, a rare and life-shortening neurodegenerative disease caused by a single defect in the FXN gene which causes reduced production of the frataxin protein. Additionally, the Agilis pipeline includes two other gene therapy programs targeting CNS disorders, including Angelman syndrome, a rare, genetic, neurological

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disorder characterized by severe developmental delays. 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.

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. 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.

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The Offering

| | |
|---|---|
| Common Stock Offered by the Selling Stockholders | 3,500,907 shares |
| Use of Proceeds | We will not receive any proceeds from the sale of shares in this offering. |
| Risk Factors | You should read the "Risk Factors" section of this prospectus for a discussion of factors to consider carefully before deciding to purchase shares of our common stock. |
| Nasdaq Global Select Market Symbol | PTCT |

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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, 2017, under the caption "Risk Factors" in Part II, Item 1A of our Quarterly Reports on Form 10-Q for the fiscal quarters ended March 31, 2018 and June 30, 2018, and in Exhibit 99.2 to our Current Report on Form 8-K filed on August 24, 2018, 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, 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 our acquisition of Agilis on August 23, 2018. Under the Merger 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 closing of the acquisition 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 August 23, 2018.

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.

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| 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. | 58,100(2) | * | 58,100 | 0 | * |
| Abraxus Trust | 46,480(3) | * | 46,480 | 0 | * |
| Adam Gray and Kerri Miller | 7,155(4) | * | 2,385 | 0 | * |
| Adam L. Gray Revocable Trust U/A/D 10/5/2006 | 4,770(5) | * | 4,770 | 0 | * |
| Allen Adler | 68,775(6) | * | 68,775 | 0 | * |
| Barton Children Trust | 22,734(7) | * | 22,734 | 0 | * |
| Joseph U. Barton | 190,588(8) | * | 11,366 | 0 | * |
| Kathleen Wagner Barton | 6,877(9) | * | 6,877 | 0 | * |
| Lila Barton | 11,366(10) | * | 11,366 | 0 | * |
| Linda Barton | 11,366(11) | * | 11,366 | 0 | * |
| Maggie Barton | 11,366(12) | * | 11,366 | 0 | * |
| Scott Barton | 11,366(13) | * | 11,366 | 0 | * |
| Thomas Barton | 213,322(14) | * | 11,366 | 0 | * |
| BGR Capital, L.P. | 30,759(15) | * | 21,473 | 0 | * |
| BioMatrix Partners, Ltd. | 401,158(16) | * | 401,158 | 0 | * |
| Carruthers Family Trust UA 4/1/04 | 30,122(17) | * | 30,122 | 0 | * |
| James J. Collins | 2,495(18) | * | 2,495 | 0 | * |
| Anne Marie Conway## | 4,770(19) | * | 4,770 | 0 | * |
| Jodi Ann Cook## | 17,175(20) | * | 17,175 | 0 | * |
| Cure FA Foundation | 95,405(21) | * | 95,405 | 0 | * |
| Gold Bench Capital, LLC | 233,593(22) | * | 233,593 | 0 | * |
| Christopher Guttilla | 51,233(23) | * | 51,233 | 0 | * |
| Benjamin Hough | 53,248(24) | * | 53,248 | 0 | * |
| Michael R. Hough | 64,032(25) | * | 64,032 | 0 | * |
| Howard Klion | 6,820(26) | * | 6,820 | 0 | * |
| Paolo Leone | 2,495(27) | * | 2,495 | 0 | * |
| Level One Partners, LLC | 604,345(28) | 1.2% | 604,345 | 0 | * |
| Thomas McGahren | 28,697(29) | * | 28,697 | 0 | * |
| Mark Merrill | 17,077(30) | * | 17,077 | 0 | * |
| NRM VII Holdings I, LLC | 137,551(31) | * | 137,551 | 0 | * |
| Kevin O'Brien## | 572(32) | * | 572 | 0 | * |
| Robert William Postma | 313,742(33) | * | 313,742 | 0 | * |
| Mark J. Pykett## | 56,209(34) | * | 56,209 | 0 | * |
| Gregory Scott Robinson | 9,254(35) | * | 9,254 | 0 | * |
| Brett Ryan | 30,759(36) | * | 9,286 | 0 | * |
| Sands Capital Private Growth Fund II, L.P. | 604,345(37) | 1.2% | 604,345 | 0 | * |
| William P. Saunders | 28,739(38) | * | 28,739 | 0 | * |
| Chris Shackelton | 9,540(39) | * | 9,540 | 0 | * |
| Bradley Christopher Shoup | 2,385(40) | * | 2,385 | 0 | * |
| Adrian Stecyk | 51,232(41) | * | 17,077 | 0 | * |
| Chrystyna Bedrij Stecyk | 51,232(42) | * | 34,155 | 0 | * |
| Richard Thorn | 477(43) | * | 477 | 0 | * |
| Storey Revocable Trust | 5,683(44) | * | 5,683 | 0 | * |
| Arthur Tzianabos | 3,303(45) | * | 3,303 | 0 | * |
| Hans Utsch | 143,108(46) | * | 143,108 | 0 | * |
| White Rock Capital Partners, L.P. | 179,222(47) | * | 179,222 | 0 | * |
| Patricia Williams | 752(48) | * | 752 | 0 | * |
| Mark Zizzamia | 17,077(49) | * | 17,077 | 0 | * |
| Additional selling stockholders as a group (4 persons) | 15,975(50) | * | 15,975 | 0 | * |

* Less than 1%.

Employee or former employee of us or Agilis.

+ Received an inducement grant for non-statutory stock options from us pursuant to the Nasdaq inducement grant exception on August 23, 2018.

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(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.

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- (2) Consists of 58,100 shares of common stock issued to the selling stockholder pursuant to the Merger 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 46,480 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. Voting and investment control over the shares is held by John D. Harkey, Jr., sole trustee. The address of the selling stockholder is 12200 Stemmons Frwy, Suite 100, Dallas, TX 75234.
- (4) Consists of 2,385 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby and 4,770 shares of common stock issued to Adam L. Gray Revocable Trust U/A/D 10/5/2006 pursuant to the Merger 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 Ave., Old Greenwich, CT 06870.
- (5) Consists of 4,770 shares of common stock issued to the selling stockholder pursuant to the Merger 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 Ave., Old Greenwich, CT 06870.
- (6) Consists of 68,775 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 800 Park Ave., New York, NY 10021.
- (7) Consists of 22,734 shares of common stock issued to the selling stockholder pursuant to the Merger 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.
- (8) Consists of 11,366 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby and 179,222 shares of common stock issued to White Rock Capital Partners, L.P. pursuant to the Merger Agreement and registered hereby, over which shares the selling stockholder has shared investment and voting control. The address of the selling stockholder is 3131 Turtle Creek Blvd., Suite 800, Dallas, TX 75219.
- (9) Consists of 6,877 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 10026 Calvin Run Rd., Great Falls, VA 22066.
- (10) Consists of 11,366 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 4340 Overhill Dr., Dallas, TX 75205.
- (11) Consists of 11,366 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 4340 Overhill Dr., Dallas, TX 75205.
- (12) Consists of 11,366 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 4340 Overhill Dr., Dallas, TX 75205.
- (13) Consists of 11,366 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The selling stockholder may be deemed to be an affiliate of National Securities Corp., 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.
- (14) Consists of 11,366 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby, 179,222 shares of common stock issued to White Rock Capital Partners, L.P. pursuant to the Merger Agreement and registered hereby, over which shares the selling stockholder has shared investment and voting control, and 22,734 shares of common stock issued to the Barton Children Trust pursuant to the Merger 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.
- (15)

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Consists of 21,473 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby and 9,286 shares of common stock issued to Brett Ryan pursuant to the Merger 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.

(16)

Consists of 401,158 shares of common stock issued to the selling stockholder pursuant to the Merger 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.

(17)

Consists of 30,122 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. Voting and investment control over the shares is held by James L. Carruthers and Noreen N. Carruthers. The address of the selling stockholder is 68 Inglewood Ln., Atherton, CA 94027.

(18)

Consists of 2,495 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 118 Glen Ave., Newton, MA 02459.

(19)

Consists of 4,770 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 370 South Main St., Mansfield, MA 02048. The selling

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stockholder served as Vice President of Clinical Operations of Agilis prior to the acquisition and currently serves as our Clinical Head of Biologics.

- (20) Consists of 17,175 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 126 Hillborn Dr., Newtown, PA 18940. The selling stockholder served as Chief Operating Officer of Agilis prior to the acquisition and currently serves as our Head of Gene Therapy Strategy.
- (21) Consists of 95,405 shares of common stock issued to the selling stockholder pursuant to the Merger 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.
- (22) Consists of 233,593 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. Investment and voting control over the shares is held by Alexander M. Seaver and Bradley R. Kent. 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.
- (23) Consists of 51,233 shares of common stock issued to the selling stockholder pursuant to the Merger 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.
- (24) Consists of 53,248 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 2756 Windsor Rd., Winston-Salem, NC 27104.
- (25) Consists of 64,032 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 2809 Lazy Ln., Winston-Salem, NC 27106.
- (26) Consists of 6,820 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 5927 Joyce Way, Dallas, TX 75225.
- (27) Consists of 2,495 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 312 Cotswold Ln., Cherry Hill, NJ 08034.
- (28) Consists of 604,345 shares of common stock issued to the selling stockholder pursuant to the Merger 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. The address of the selling stockholder is 2105 Dogwood Ln., Charlottesville, VA 22901.
- (29) Consists of 28,697 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 349 11th St., Brooklyn, NY 11215. The selling stockholder is a former member of the Agilis Scientific Advisory Board and a member of Agilis's founding group.
- (30) Consists of 17,077 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 50 Murray St., Apt. 711, New York, NY 10007.
- (31) Consists of 137,551 shares of common stock issued to the selling stockholder pursuant to the Merger 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 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 is party to an Exclusive Channel Collaboration Agreement with Agilis.
- (32) Consists of 572 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 149 Willow St., Acton, MA 10720. The selling stockholder served as Vice President of Finance of Agilis prior to the acquisition and currently serves as our Head of Finance Operations.
- (33) Consists of 313,742 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is P.O. Box 2017, 141 Mecox Rd., Water Mill, NY 11976.

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- (34) Consists of 56,209 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 223A Main St., Boxford, MA 01923. The selling stockholder served as President and Chief Executive Officer of Agilis prior to the acquisition and currently serves as our Chief Innovation Officer.
- (35) Consists of 9,254 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 4 Foley Farm Rd., Wilmington, MA 01887.
- (36) Consists of 9,286 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby and 21,473 shares of common stock issued to BGR Capital, L.P. pursuant to the Merger Agreement and registered hereby, over which the selling stockholder has investment and voting control. The address of the selling stockholder is 3131 Turtle Creek Blvd., Suite 800, Dallas, TX 75219.
- (37) Consists of 604,345 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. Voting and investment control over the shares is shared by Ian Ratcliffe and Frank M. Sands.

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Mr. Ratcliffe, an officer of the selling stockholder, previously served as a member of the Agilis Board of Directors. The address of the selling stockholder is 1000 Wilson Blvd., Suite 3000, Arlington, VA 22209.

- (38) Consists of 28,739 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 1700 Hardin Ln., Powell, OH 43065.
- (39) Consists of 9,540 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 487 West Rd., New Canaan, CT 06840.
- (40) Consists of 2,385 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 6337 Northport Dr., Dallas, TX 75230.
- (41) Consists of 17,077 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby and 34,155 shares of common stock issued to Chrystyna Bedrij Stecyk, the spouse of the selling stockholder, pursuant to the Merger Agreement and registered hereby with respect to which the selling stockholder may have a beneficial ownership interest. The address of the selling stockholder is 21 S. End Ave. #603, New York, NY 10280. The selling stockholder served as a member of the Board of Directors of Agilis prior to the acquisition.
- (42) Consists of 34,155 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby and 17,077 shares of common stock issued to Adrian Stecyk, the spouse of the selling stockholder, pursuant to the Merger Agreement and registered hereby, with respect to which the selling stockholder may have a beneficial ownership interest. The address of the selling stockholder is 21 S. End Ave. #603, New York, NY 10280.
- (43) Consists of 477 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 20 Dudley Rd., Mendon, MA 01756.
- (44) Consists of 5,683 shares of common stock issued to the selling stockholder pursuant to the Merger 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.
- (45) Consists of 3,303 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 114 Duck Rd., Reading, MA 01867.
- (46) Consists of 143,108 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 15 Legare St., Charleston, SC 29401.
- (47) Consists of 179,222 shares of common stock issued to the selling stockholder pursuant to the Merger 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.
- (48) Consists of 752 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 3470 Pin Oak Ct., Catharpin, VA 20143.
- (49) Consists of 17,077 shares of common stock issued to the selling stockholder pursuant to the Merger Agreement and registered hereby. The address of the selling stockholder is 400 Chambers St., Apt. 25K, New York, NY 10282.
- (50) Consists of 15,975 shares of common stock issued to the selling stockholders pursuant to the Merger Agreement and registered hereby. The address of the selling stockholders is c/o Shareholder Representative Services LLC, 950 17th St., Suite 1400, Denver, CO 80202.

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.

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None of the selling stockholders has held any position or office with us or any of our subsidiaries within the past three years, except that the selling stockholders indicated below or in the table above or the notes thereto have been employed by us and/or Agilis. In connection with our acquisition of Agilis, we entered into employment agreements with Jodi Ann Cook, formerly Chief Operating Officer of Agilis, to serve as our Head of Gene Therapy Strategy, and Mark Pykett, formerly Chief Executive Officer of Agilis, to serve as our Chief Innovation Officer. Under her employment agreement, Ms. Cook will receive an annual base salary and will be eligible to receive an annual bonus and equity grant beginning in 2019. Pursuant to her employment agreement, Ms. Cook also received an inducement grant for non-statutory stock options pursuant to the Nasdaq inducement grant exception for options to purchase 75,000 shares of our common stock on the closing date of our acquisition of Agilis and in 2019 will receive a stock option grant for 50,000 shares of our common stock as her annual equity grant. Under his employment agreement, Mr. Pykett will receive an annual base salary

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and will be eligible to receive an annual bonus and equity grant beginning in 2019. Pursuant to his employment agreement, Mr. Pykett also received an inducement grant for non-statutory stock options pursuant to the Nasdaq inducement grant exception for options to purchase 100,000 shares of our common stock on the closing date of our acquisition of Agilis and in 2019 will receive a stock option grant for 50,000 shares of our common stock as his annual equity grant. In addition, we entered into employment agreements with Anne Marie Conway, formerly Vice President of Clinical Operations of Agilis, to serve as our Clinical Head of Biologics, and Kevin O'Brien, formerly Vice President of Finance of Agilis, to serve as our Head of Finance Operations, that provide for a base salary and the eligibility to receive an annual cash bonus and annual equity grant commensurate with and on the same terms as other comparable employees and pursuant to which Ms. Conway and Mr. O'Brien each received an inducement grant for non-statutory stock options pursuant to the Nasdaq inducement grant exception for options to purchase in the aggregate amount 50,000 shares of our common stock on the closing date of the acquisition. The employment agreements with Ms. Cook, Mr. Pykett, Ms. Conway and Mr. O'Brien 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. Under the Merger Agreement, each selling stockholder will also be entitled to receive its pro rata portion of contingent payments from the Company based on (i) the achievement of certain development milestones, (ii) the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher, (iii) the achievement of certain net sales, and (iv) a percentage of annual net sales for Friedreich ataxia and Angelman Syndrome during specified terms, and we are required to pay certain of the development milestone payments regardless of whether the applicable milestones have been achieved. See the discussion under the heading "Merger Agreement" in our Current Report on Form 8-K filed with the SEC on July 19, 2018 for more details with respect to the consideration, including contingent consideration, that has been paid or may become payable to selling stockholders in connection with the acquisition.

Under the Merger Agreement, we will indemnify each selling stockholder against any damages to which a selling stockholder may become subject by reason of any material misstatement or omission in this registration statement, except to the extent that such damages arise out of or are based upon information furnished to us by or on behalf of Agilis or an Agilis equityholder for use in this registration statement. Each selling stockholder will indemnify us and certain related persons against any damages to which such indemnified parties may become subject by reason of any material misstatement or omission in this registration statement or any violation or alleged violation of the Securities Act, the Exchange Act, or any state securities law, or any rule promulgated under any of the foregoing, in each case to the extent based upon any action or omission made in reliance upon information provided by such selling stockholder for use in this registration statement.

The foregoing description of the Merger Agreement is a summary only and is qualified in its entirety by reference to the terms of the Merger Agreement, included as Exhibit 2.1 hereto.

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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 August 23, 2018, there were 50,265,287 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

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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 June 30, 2018, we had issued and outstanding options to purchase 8,166,403 shares of our common stock at a weighted average exercise price of \$27.06 per share and warrants to purchase (i) 7,030 shares of our common stock at an exercise price of \$128.00 per share and (ii) 130 shares of our common stock at an exercise price of \$2,520.00 per share.

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 Convertible Notes. The 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 Convertible Notes will mature on August 15, 2022, unless earlier repurchased or converted. The conversion rate for the Convertible Notes was initially, and remains, 17.7487 shares of our common stock per \$1,000 principal amount of the Convertible Notes, which is equivalent to an initial conversion price of approximately \$56.34 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.

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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;

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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.

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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 (as supplemented or amended to reflect such transaction). 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 (as supplemented or amended to reflect such transaction).

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 reallocated 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.

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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) February 23, 2019.

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, 2017, and the effectiveness of our internal control over financial reporting as of December 31, 2017, 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 the Emflaza Business as of and for the years ended December 31, 2016 and 2015, incorporated in this prospectus by reference from the Current Report on Form 8-K/A dated July 3, 2017 of PTC Therapeutics, Inc., have been audited by Deloitte & Touche LLP, independent auditors, as stated in their report (which report expresses an unmodified opinion and includes an emphasis-of-matter paragraph relating to the allocation of certain assets, liabilities, expenses, and income that have historically been held at the Marathon Pharmaceutical Holdings, LLC corporate level but which are specifically identifiable or attributable to the Emflaza Business), 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.

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.

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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, 2017, including the information specifically incorporated by reference into the Annual Report on Form 10-K from our definitive proxy statement for the 2018 Annual Meeting of Stockholders;

Quarterly Reports on Form 10-Q for the fiscal quarters ended March 31, 2018 and June 30, 2018;

Current Reports on Form 8-K filed on April 20, 2017 (solely with respect to Items 2.01, 3.02 and 9.01, as amended on July 3, 2017), February 20, 2018, April 3, 2018, June 14, 2018, July 19, 2018 (solely with respect to Items 1.01, 3.02 and 9.01), August 2, 2018 (solely with respect to Item 1.01) and August 24, 2018 (solely with respect to Items 1.01, 2.01, 3.02, 8.01 and 9.01); 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

August 24, 2018

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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 | \$ 18,908 |
| Legal fees and expenses | \$ 50,000 |
| Accounting fees and expenses | \$ 61,000 |
| Miscellaneous expenses | \$ 5,000 |
| Total expenses | \$ 134,908 |

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

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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.

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Item 16. Exhibits.

| Exhibit No. | Description |
|-------------|---|
| 2.1 | <u>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)</u> |
| 4.1 | <u>Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.3 to the Registration Statement on Form S-1, as amended (File No. 333-188657), of the Registrant)</u> |
| 4.2 | <u>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)</u> |
| 5.1 | <u>Opinion of Wilmer Cutler Pickering Hale and Dorr LLP</u> |
| 23.1 | <u>Consent of Ernst & Young LLP, independent registered public accounting firm for the Registrant</u> |
| 23.2 | <u>Consent of Deloitte & Touche LLP</u> |
| 23.3 | <u>Consent of BDO USA, LLP</u> |
| 23.4 | <u>Consent of Wilmer Cutler Pickering Hale and Dorr LLP (included in Exhibit 5.1)</u> |
| 24.1 | <u>Powers of Attorney (included on the signature page to the Registration Statement)</u> |

Confidential treatment has been requested for certain portions that are omitted from this exhibit. The omitted information has been filed separately with the SEC pursuant to the registrant's application for confidential treatment. In addition, schedules have been omitted from this exhibit pursuant to Item 601(b)(2) of Regulation S-K. A copy of any omitted schedule will be furnished supplementally to the SEC upon request; provided, however, that the registrant may request confidential treatment for any document so furnished.

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

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- (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,

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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.

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| Signature | Title | Date |
|---|--------------|-----------------|
| <hr/> <i>/s/ ALLAN JACOBSON, PH.D.</i> Allan Jacobson, Ph.D. | Director | August 24, 2018 |
| <hr/> <i>/s/ GLENN D. STEELE, JR., M.D., PH.D.</i> Glenn D. Steele, Jr., M.D., Ph.D. | Director | August 24, 2018 |
| <hr/> <i>/s/ DAVID P. SOUTHWELL</i> David P. Southwell | Director | August 24, 2018 |
| <hr/> <i>/s/ JEROME B. ZELDIS, M.D., PH.D.</i> Jerome B. Zeldis, M.D., Ph.D. | Director | August 24, 2018 |

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