PTC THERAPEUTICS, INC.
Form 8-K
August 24, 2018

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549
FORM 8-K
CURRENT REPORT
Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934
Date of Report (Date of earliest event reported): August 23, 2018
PTC THERAPEUTICS, INC.
(Exact Name of Company as Specified in Charter)

Delaware	001-35969	04-3416587
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)
100 Corporate Court South Plainfield, NJ		07080
(Address of Principal Executive Offices)		(Zip Code)

Company's telephone number, including area code: (908) 222-7000
Not applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):
o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))
Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).
Emerging growth company o
If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. o

Introductory Note.

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

Item 1.01. Entry into a Material Definitive Agreement.

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

Agreements with National Taiwan University

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

Collaboration Agreement

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

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

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

Licensing Agreement

Pursuant to the Licensing Agreement, NTU granted to Agilis an exclusive, perpetual license, with the right to grant sublicenses through all tiers, to research and use the intellectual property, data, chemistry, manufacturing and controls ("CMC") records, documents, confidential information, materials and know-how pertaining to the Research, including GT-AADC for the treatment of AADC deficiency, under the Collaboration Agreement (the “Technology”) and to develop, make, manufacture, use, sell, import and market the Technology and any other products made, invented, developed or incorporated by or with the Technology (the “Licensed Products”). Subject to any regulatory delays or issues, Agilis is obligated to research, use and develop the Technology to manufacture Licensed Products by December 23, 2025. Additionally, the Licensing Agreement provides for Agilis to obtain marketing approval of GT-AADC for the treatment of AADC deficiency, either by the U.S. Food and Drug Administration (the “FDA”) or by the European Medicines Agency (the “EMA”), by December 31, 2024. The agreement also stipulates milestones in

relation to a Phase 3 trial with respect to GT-AADC for the treatment of AADC deficiency, which such Phase 3 trial the Company does not deem necessary and does not plan to conduct.

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

fees, (iii) a low double-digit percentage royalty of annual net sales of Licensed Products, and (iv) a percentage of sublicense revenue, ranging from low-twenties to mid-twenties. The annual license maintenance fees are non-refundable, but creditable against annual net sales payments.

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

The Licensing Agreement expires on December 23, 2035. Upon expiration, Agilis will have a fully paid-up, perpetual, royalty-free exclusive license to the Technology. Agilis may terminate the Licensing Agreement upon 60 days' written notice to NTU in the event of (a) the failure of a pivotal clinical study, or serious adverse event in a clinical study, with respect to GT-AADC for the treatment of AADC deficiency, that prevents continuing such clinical study under reasonable circumstances or (b) the rejection of a BLA with the FDA or a MAA with the EMA, or equivalent biologics approval application in another territory with respect to GT-AADC for the treatment of AADC. In such termination event, Agilis must pay $100,000 to NTU within 30 days of termination and NTU would retain all rights to the Technology. Agilis may terminate the Licensing Agreement for material breach by another party following a 30-day cure period. NTU may terminate the Licensing Agreement for Agilis's failure to pay any undisputed license fees or net sales or sublicensing royalty fees within the applicable deadline following a 30-day cure period.

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

Item 2.01. Completion of Acquisition or Disposition of Assets.

Agilis Acquisition

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

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

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

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

Bridge Loan Agreement

As previously disclosed, in connection with its entry into the Merger Agreement, the Company entered into a Bridge Loan and Security Agreement (the "Bridge Loan Agreement") on July 19, 2018 with Agilis and certain of Agilis's domestic subsidiaries, as guarantors. Under the Bridge Loan Agreement, the Company made a term loan advance to Agilis on July 23, 2018 in an original principal amount of $10.0 million. In connection with the closing of the Merger,

the original principal amount of $10.0 million plus all accrued and unpaid interest thereon was credited against the cash portion of the upfront consideration paid by the Company pursuant to the terms of the Merger Agreement in satisfaction of Agilis's outstanding payment obligations under the Bridge Loan Agreement, and the Company will have no further obligation to extend any further loan amounts under the Bridge Loan Agreement.

Description of Agilis' Business

Agilis is a biotechnology company advancing a gene therapy platform focused on the development of innovative therapies for rare, debilitating diseases of the central nervous system ("CNS"). Agilis's lead product candidate is GT-AADC for the treatment of AADC deficiency. AADC deficiency is a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. AADC is the enzyme responsible for the conversion of L-dopa to dopamine. Dopamine is a key neurotransmitter that acts within the striatum (caudate and putamen), a component of the brain's deep grey matter, to modulate output of neurons that project to the motor and premotor cortices of the brain that plan and execute normal motor function and is required to be present in the brain for humans to develop and maintain proper motor function.

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

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

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

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

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

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

At baseline, patients had no functional movement and failed to achieve any motor milestones, including head control, sitting or standing capabilities, consistent with the published natural history of severe AADC deficiency. Compared to

baseline, at one-year and at five-years after GT-AADC administration, patients had objective evidence of de novo dopamine production as visualized by F-DOPA PET imaging of the brain, consistent with successful and stable gene expression and enzyme activity over time.

Based on preliminary analysis, following administration of GT-AADC, the combined group of patients showed significant changes from baseline capabilities at one-year post-treatment in functional motor skills assessed with the PDMS-2 total score,

as well as locomotion, grasping, visual-motor integration and stationary subscales. Significant changes from baseline at one-year post-treatment were also observed for the combined group of patients on the AIMS total score and prone, supine, sit and stand subscales.

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

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

The ongoing clinical trial, AADC-011, is a single-center, open-label trial to assess the efficacy and safety of GT-AADC in patients with AADC deficiency. The primary outcomes for this trial include assessing a change in the PDMS-2 score and measuring the change in the neurotransmitter metabolite homovanillic acid (HVA) or 5-hydroxyindoleacetic acid (HIAA) in the cerebrospinal fluid. A total of 10 patients are planned for recruitment, of which 8 have been enrolled and treated to date.

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

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

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

If GT-AADC for the treatment of AADC deficiency receives FDA approval, we expect that GT-AADC would have a twelve-year exclusive marketing period in the United States for the approved indication, commencing on the date of FDA approval, under the provisions of the Biologics Price Competition and Innovation Act of 2009 (“BPCIA”) as well as a concurrent seven-year exclusive marketing period, which would commence on the date of FDA approval, under the provisions of the Orphan Drug Act of 1983 (the "Orphan Drug Act"). We are pursuing patent protection for GT-AADC, and, in the meantime, we expect to rely on the twelve-year BPCIA regulatory exclusivity and concurrent seven-year Orphan Drug Act exclusivity to commercialize GT-AADC in the United States, if it is approved.

The Agilis pipeline also includes a gene therapy asset targeting Friedreich ataxia, a rare and life-shortening neurodegenerative disease caused by a single defect in the FXN gene which causes reduced production of the frataxin protein. An investigational new drug ("IND") submission with the FDA for this program is expected in 2019.

Additionally, the Agilis pipeline includes two other gene therapy programs targeting CNS disorders, including Angelman syndrome, a rare, genetic, neurological disorder characterized by severe developmental delays.

Intellectual Property

As part of our acquisition of Agilis, we are acquiring a patent portfolio consisting of U.S. patents and patent applications, including original filings, continuations and divisional applications, as well as numerous foreign

counterparts to many of these patents and patent applications. We exclusively in-license these patents and patent applications with claims directed to composition of matter, formulation and methods of use, including for the target disease AADC. For a further discussion of the material agreements relating to our in-licensing of GT-AADC for the treatment of AADC deficiency, see Item 1.01 of this Current Report on Form 8-K.

Manufacturing

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

Competition

Currently, no treatment options are available for the underlying cause of AADC deficiency, and care is limited to palliative options with significant burden on caregivers. Additionally, we are not aware of any late-stage development product candidates for AADC deficiency. However, other gene therapy companies may in the future decide to utilize existing technologies to address unmet needs that could potentially compete with our product candidates.

Government Regulation of Gene Therapy

In the United States, the FDA regulates biologic products including gene therapy products under the Federal Food, Drug, and Cosmetic Act, or the FDCA, the Public Health Service Act, or the PHSA, and regulations and guidance implementing these laws. The FDCA, PHSA and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biologic products. Applications to the FDA are required before conducting human clinical testing of biologic products. Additionally, each clinical trial protocol for a gene therapy product candidate is reviewed by the FDA.

Until recently, the National Institutes of Health, or the NIH, through its Recombinant DNA Advisory Committee, or RAC, also reviewed certain proposed gene therapy trials; however, the FDA and the NIH recently proposed to change this practice so that the RAC will no longer review individual human gene transfer protocols. The NIH has stated that it will finalize this change after taking public comments. The FDA has issued a growing body of guidance documents on CMC, clinical investigations and other areas of gene therapy development, all of which are intended to facilitate the industry’s development of gene therapy products.

U.S. New Drug and Biological Product Development Process

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

Additional regulation for gene therapy clinical trials

In addition to the regulations discussed above, there are a number of additional standards that apply to clinical trials involving the use of gene therapy. The FDA has issued various guidance documents regarding gene therapies, which outline additional factors that the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical assessment of gene therapies; the CMC information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene therapy trials and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these trials.

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

Manufacturers of biologics must comply with applicable cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Manufacturers and others involved in the manufacture and distribution of such products also must register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process.

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

Concurrent with clinical trials, companies usually complete additional preclinical studies and must also develop additional information about the physical characteristics of the biologic product candidate as well as finalize a process for manufacturing the product candidate in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents or of causing other adverse events with the use of biologic products, the PHSA emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other requirements, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biologic product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biologic product candidate does not undergo unacceptable deterioration over its shelf life.

BPCIA Exclusivity

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

The 2010 Patient Protection and Affordable Care Act included the BPCIA as a subtitle. The BPCIA established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars. As of January 1, 2018, the FDA has approved nine biosimilar products for use in the United States. No interchangeable biosimilars have been approved. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars. We expect the FDA to finalize additional guidance in the near term.

Under the BPCIA, a manufacturer may submit an application for licensure of a biologic product that is “biosimilar to” or “interchangeable with” a previously approved biological product or “reference product.” In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. For the FDA to approve a biosimilar product as interchangeable with a reference product, the FDA must find that the biosimilar product can be expected to produce the same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date of approval of the reference product. The FDA may not approve a biosimilar product until 12 years from the date on which the reference product was approved. Even if a product is considered to be a reference product eligible for exclusivity, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor’s own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At present, it is unclear whether products deemed “interchangeable” by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

Government regulation of gene therapy outside of the United States

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

European Union regulation and exclusivity

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

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

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

Orphan drug designation

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

Item 3.02. Unregistered Sales of Equity Securities.

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

Item 7.01. Regulation FD Disclosure.

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

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

Item 8.01. Other Events.

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

Item 9.01. Financial Statements and Exhibits.

(a) Financial Statements of Business Acquired

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

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

(b) Pro Forma Financial Information

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

(d) Exhibits

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

Cautionary Statement Concerning Forward Looking Statements

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

Signature

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

PTC Therapeutics, Inc.

Date: August 24, 2018 By: /s/ Christine Utter
Name: Christine Utter
Title: Principal Financial Officer