PTC Therapeutics to Acquire Agilis Biotherapeutics

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- Expands and diversifies current pipeline with four gene therapy programs -
- BLA submission in AADC deficiency expected in 2019 -

SOUTH PLAINFIELD, N.J., July 19, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that it has entered into an agreement to acquire Agilis Biotherapeutics, Inc., a biotechnology company advancing an innovative gene therapy platform for rare monogenic diseases that affect the central nervous system (CNS). The transaction was approved by the Boards of both companies.

"The addition of the gene therapy platform transforms PTC and aligns with our vision of being a leader in the treatment of rare disorders," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We are impressed with the clinical results shown by the AADC program and are excited with the potential to quickly bring this therapy to patients. We look forward to advancing the Friedreich ataxia and Angelman syndrome programs into the clinic in the next two years."

"I am proud of the accomplishments achieved by Agilis culminating with this value-creating transaction," said Mark Pykett, DVM, Ph.D., President and Chief Executive Officer of Agilis Biotherapeutics, Inc. "PTC provides a global infrastructure and proven capabilities, which we believe will enable our goal of providing therapy to patients suffering from rare CNS disorders. I look forward to joining PTC and supporting the advancement of the programs to provide value to patients."

The lead gene therapy candidate, GT-AADC, has compelling clinical data in treating Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency. AADC Deficiency is a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase (DDC) gene. In its severe forms, AADC Deficiency causes the inability to develop any motor control (global muscular hypotonia/dystonia) resulting in breathing, feeding, and swallowing problems, frequent hospitalizations, and the need for life-long care. Many patients 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 results from two prospective clinical studies enrolling 18 subjects with severe AADC Deficiency indicated that treated subjects with GT-AADC exhibited de novo dopamine production. Additionally, the subjects demonstrated achievement of motor development milestones and substantial gains on motor function and cognitive scales over multiple years following the single gene therapy treatment. Data from trials that began in 2010 have demonstrated long-term evidence of durable clinical benefit. Based on multi-year data from initial clinical studies with the AADC gene therapy treatment candidate, PTC plans to submit a Biologics License Application (BLA) to the FDA in 2019.

The product 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 IND submission for this program is expected in 2019. Additionally, the acquisition brings two other gene therapy programs targeting CNS disorders, including Angelman syndrome, a rare, genetic, neurological disorder characterized by severe developmental delays.

The transaction is expected to close in the third quarter of 2018, pending successful fulfillment of all customary closing conditions. On completion, PTC plans a smooth transition of operations and the integration of Agilis' talented and dedicated employees to continue the mission of bringing the pipeline of gene therapies for CNS disorders to patients worldwide.

Transaction Details
Under the terms of the merger agreement, PTC will pay an upfront consideration of $50 million in cash and approximately $150 million in PTC common stock, subject to an estimated maximum 9.34 million share limit (with any shortfall to be made whole with additional cash consideration). In addition to the upfront payments, potential future consideration includes $60 million in development milestones to be paid over the next two years which includes the acceptance of a BLA. Additionally, the transaction includes up to $535 million in success-based milestones in connection with regulatory approvals on the three most advanced programs and receipt of a priority review voucher, as well as tiered commercial milestones of $150 million, and 2-6 % of annual net sales for Friedreich ataxia and Angelman syndrome.

Conference Call and Webcast Details
A conference call will take place on Thursday, July 19 at 4:30 pm ET and can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 8469109. A live, listen-only webcast of the conference call can be accessed at https://edge.media-server.com/m6/p/25h8j9nj. A webcast replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for two weeks.

About Agilis Biotherapeutics, Inc.
Agilis is advancing innovative gene therapies designed to provide long-term efficacy for patients with debilitating, often fatal, rare genetic diseases that affect the central nervous system. Agilis' gene therapies are engineered to impart sustainable clinical benefits by inducing persistent expression of a therapeutic gene through precise targeting and restoration of lost gene function to achieve long-term efficacy. Agilis' rare disease programs are focused on gene therapy for AADC deficiency, Friedreich ataxia, and Angelman syndrome, all rare genetic diseases that include neurological deficits and result in physically debilitating conditions.

About PTC Therapeutics
PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder

products and has a global commercial footprint. This success is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. For more information, please visit our website at www.ptcbio.com.

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

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

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

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