



## PTC Therapeutics Successfully Completes Acquisition of Agilis Biotherapeutics

August 23, 2018

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

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

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

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

### About PTC Therapeutics

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

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### Forward Looking Statements:

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

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

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

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

or other circumstances occurring after the date of this press release except as required by law.

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