



## PTC Therapeutics Announces First Patient Dosed in Phase 1 Trial for PTC857

July 8, 2020

**- First in human trial for PTC857, the second compound in the Bio-e platform to enter clinical trials -**

SOUTH PLAINFIELD, N.J., July 8, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the first subject in the Phase 1 clinical trial evaluating PTC857 in healthy volunteers has been dosed. PTC857, discovered through PTC's Bio-e platform, is an investigational small molecule drug that inhibits 15-Lipoxygenase, an enzyme that is a key regulator of the oxidative stress, protein aggregation and inflammation response pathways that underpin neurodegenerative disease pathology. The first indication planned for PTC857 is Parkinson's disease caused by a mutation in the GBA gene ("GBA Parkinson's disease"), which is one of the most common genetic subtypes of Parkinson's disease.

"We are excited about the initiation of this study for PTC857, which was developed from our novel Bio-e platform," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "PTC857 is an inhibitor of the key enzyme whose products modulate fundamental pathways that are involved in neuroinflammation and oxidative stress that lead to multiple disorders including GBA Parkinson's disease. We look forward to completing the healthy volunteer study and advancing PTC857 into patients."

The Phase 1 study will evaluate PTC857 in healthy human volunteers with single and multiple ascending doses that will inform safety and pharmacology, and the design for the Phase 2 program. PTC857 has demonstrated efficacy in a number of GBA Parkinson's disease-relevant *in vitro* and *in vivo* test systems.

### About PTC857

PTC857 is an investigational, novel small molecule in development for orphan neurodegenerative diseases of oxidative stress and inflammation from PTC's Bio-e platform. PTC857 targets the enzyme 15-lipoxygenase, a key enzymatic hub that regulates the inflammation and oxidative stress that underpin CNS pathology. PTC857 is currently being studied in GBA subtype Parkinson's disease (PD), which is one of most common genetic risk factors for PD.[i]

### About PTC Therapeutics, Inc.

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more about PTC, please visit us at [www.ptcbio.com](http://www.ptcbio.com) and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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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 contained in this release, other than statements of historic fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "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: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; significant 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 PTC's products and product candidates; PTC's scientific approach and general development progress; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and 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. You are urged to carefully consider all such factors.

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 will receive or maintain regulatory approval in any territory, or prove to be commercially successful.

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.

<sup>1</sup>GBA Mutation May Lead to Novel Therapeutics (2020). Parkinson's Foundation. Accessed June 30, 2020.

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