



## **PTC Therapeutics Reports the Initiation of the Second Stage of the FITE19 Phase 2/3 Clinical Trial Evaluating PTC299 for the Treatment of COVID-19**

February 17, 2021

**-- PTC299 is an oral therapy that has a novel dual mechanism of action that inhibits viral replication and attenuates the uncontrolled inflammatory response --**  
**-- Stage 1 of the clinical trial is complete --**

SOUTH PLAINFIELD, N.J., Feb. 17, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced the initiation of the second stage of the FITE19 clinical trial to assess PTC299 in COVID-19 patients. PTC299 is an oral investigational drug with a novel dual mechanism of action that has the potential to inhibit viral replication and attenuate the uncontrolled inflammatory response that ensues after infection. PTC299 functions by targeting a cellular enzyme, dihydroorotate dehydrogenase (DHODH). By targeting this enzyme instead of a viral protein, it makes it less likely to elicit drug-resistance. Enrollment for the second stage of the trial has been initiated in multiple centers outside of the US.

"Completion of the first stage of the FITE19 trial is an important milestone that triggers the final stage of the trial to begin," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "The lack of an effective COVID-19 treatment has significantly hampered our ability to resume our normal lives. PTC299 has a unique mechanism of action that has the potential to be an effective COVID-19 treatment for both at-home and hospitalized patients and should be effective against the wildtype virus as well as viral variants. We are working hard to bring this potential important treatment to patients as rapidly as possible."

The Phase 2/3 clinical trial is a randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the efficacy and safety of PTC299 in patients hospitalized with COVID-19. The study is being completed in two stages: the first stage enrolled a smaller cohort of 40 patients followed by an interim safety analysis before enrolling the larger cohort of approximately 340 participants in the second stage. The primary endpoint of the study is to evaluate the clinical efficacy of PTC299 compared with placebo assessed by time to respiratory improvement in adult individuals hospitalized with COVID-19.

Please visit PTC's PTC299 COVID-19 FITE19 clinical trial recruitment page, [here](#) for more information.

### **About PTC299**

PTC299 is an investigational oral small molecule that inhibits the cellular enzyme dihydroorotate dehydrogenase (DHODH). Recently, DHODH has been independently identified as one of the top-three most promising targets for intervention in COVID-19. PTC299 is being evaluated for the potential treatment of patients with COVID-19. With a novel dual-mechanism of action, PTC299 inhibits DHODH activity which leads to a reduction of pyrimidine biosynthesis and SARS-CoV-2 viral replication, and selectively modulates the immune response by attenuating the stress-induced inflammatory cytokine storm. PTC299 has broad-spectrum antiviral activity and potent inhibition of numerous RNA viruses with low nanomolar potency. PTC299 is also currently being evaluated in an ongoing clinical study in acute myeloid leukemia (AML).

### **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 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 on [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; the enrollment, conduct and results of PTC's PTC299 clinical trial for COVID-19; 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.

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