PTC Therapeutics Announces the Initiation of a Phase 2/3 Clinical Trial to Evaluate PTC299 for the Treatment of COVID-19

June 17, 2020

- Oral treatment with novel dual-mechanism of action has the potential to address the two critical elements of COVID-19; viral replication and uncontrolled inflammatory response -

- Randomized, double-blind, placebo-controlled, multi-national Phase 2/3 clinical study with two stages to initiate in the U.S. in coming days -

- CTA submitted in Spain with additional filings planned in Europe, Brazil and Australia -

- Conference call scheduled for 8:30 am ET -

SOUTH PLAINFIELD, N.J., June 17, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that the U.S. Food and Drug Administration has authorized the initiation of a Phase 2/3 trial to investigate PTC299, a dihydroorotate dehydrogenase (DHODH) inhibitor, as a potential treatment for COVID-19. PTC299 is an oral investigational drug with a novel dual-mechanism of action that has the potential to address the two critical elements of COVID-19: 1) the high viral replication and 2) the uncontrolled inflammatory response that ensues after infection. PTC will initiate an integrated Phase 2/3 study in the U.S. in the coming days with additional sites planned globally.

"We are excited about the potential of PTC299 to be part of the solution to this unprecedented global public health crisis and have made it a high priority within our organization," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "The fact that PTC299 inhibits DHODH uniquely addresses the two key issues of COVID-19, namely reducing the high viral replication and also selectively attenuating the immune response caused by the uncontrolled cytokine storm resulting from the SARS-CoV-2 infection."

PTC299 is an oral, small molecule tablet that inhibits the cellular enzyme DHODH which is used to produce the RNA building blocks for the production of SARS-CoV-2. PTC299 has demonstrated potent inhibition of viral replication in SARS-CoV-2 cell based assays, which has led to PTC moving rapidly into the clinic. A number of RNA viruses require the same building blocks, which explains why in preclinical tests PTC299 has shown broad-spectrum antiviral activity.

"Infection with SARS-CoV-2 has caused COVID-19 in millions of people around the world and the death of hundreds of thousands. In the year ahead, SARS-CoV-2 could infect and kill even greater numbers of people. As a global community, we are working together with unprecedented speed to find treatments that block the virus and the deadly consequences of COVID-19," said Dr. Jeremy Luban, Professor, University of Massachusetts Medical School. "PTC299 is among the most promising potential treatments on the immediate horizon because it potently inhibits SARS-CoV-2 in the lab, it has already been used in people, it is already formulated as a tablet, and it may attenuate the deadly inflammation observed in the most severe cases of COVID-19. Additionally, PTC299 targets a cellular enzyme, as opposed to a viral one, and is therefore less likely to elicit drug-resistant SARS-CoV-2."

The integrated Phase 2/3 study will evaluate the efficacy and safety of PTC299 in two stages, the first stage consisting of 40 patients, followed by a larger cohort of approximately 340 patients. The primary objective is to evaluate the clinical efficacy of PTC299 compared with placebo assessed by time to respiratory improvement in adult individuals hospitalized with COVID-19.

PTC299 is also being developed for treatment in oncological indications and has been extensively studied in nine clinical trials in over 300 individuals, including both oncology patients and healthy volunteers. In addition, PTC299 is currently being studied in a clinical trial in acute myeloid leukemia (AML) patients. In a PTC299 clinical study, results showed that PTC299 inhibited DHODH, confirming the mechanism of action in patients.

[1]Multi-omics study revealing tissue-dependent putative mechanisms of SARS-CoV-2 drug targets on viral infections and complex diseases. Jie Zheng, Yuemiao Zhang, Yi Liu, Denis Baird, Xingzi Liu, Lin Wang, Hong Zhang, George Davey Smith, Tom R Gaunt.

Today's Conference Call and Webcast Reminder:
Today's conference call will take place at 8:30 am (ET) and can be access by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 9174134. A live, listen-only webcast of the conference call can be accessed on the investor relations section of the PTC website at www.ptcbio.com. The accompanying slide presentation will be posted on the investor relations section of the PTC website. 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 30 days following the call.

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[1]. 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). For more information regarding

PTC299 clinical trials, visit clinicaltrials.ptcbio.com or www.clinicaltrials.gov and search PTC299.

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 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; the enrollment, conduct, and results of studies for PTC299 during, or as a result of, the studies that could delay or prevent further development under the program, including any potential regulatory submissions; 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; 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.

View original content:http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-the-initiation-of-a-phase-23-clinical-trial-to-evaluate-ptc299-for-the-treatment-of-covid-19-301078337.html

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