PTC Therapeutics Announces APHENITY Trial Achieved Primary Endpoint with Sepiapterin in PKU Patients

May 17, 2023

- Highly statistically significant and clinically meaningful results -

- 63% mean blood Phe reduction in primary analysis population (p<0.0001) -

- Conference call and webcast to be held at 8:00 AM EDT -

SOUTH PLAINFIELD, N.J., May 17, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the primary endpoint was achieved in the APHENITY, Phase 3 registration-directed clinical trial of sepiapterin in adult and pediatric patients with phenylketonuria (PKU).

"The positive results from the APHENITY trial bring us one step closer to providing a therapy that could deliver meaningful benefit to PKU patients," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "The Phe reductions observed in the placebo-controlled portion of the study are consistent with, and, in some cases, exceed the magnitude of Phe reductions recorded in the open label portion of the study. We look forward to meeting with regulatory authorities to discuss the path to approval."

The placebo-controlled portion of the study included 98 patients in the primary analysis population. The mean percent Phe reduction in sepiapterin treated patients was 63%. In the subset of classical PKU patients, the mean percent Phe reduction was 69%. Minimal reductions in Phe levels were observed in the placebo treated patients resulting in a highly statistically significant sepiapterin treatment benefit (p<0.0001). Sepiapterin was generally well tolerated with no serious adverse events.

Today's Conference Call and Webcast
PTC will hold a conference call at 8 AM EDT today to discuss this news. To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A 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 APHENITY
APHENITY was a global double-blind, placebo-controlled, registration-directed study which enrolled 156 children and adults with PKU. Participants were randomized to receive sepiapterin or placebo for six weeks with the primary endpoint being reduction in blood phenylalanine levels. The trial consisted of two parts. Part 1 was a run-in phase, during which all screened subjects received sepiapterin for two weeks. Only those subjects who demonstrated a reduction in phenylalanine levels of 15% or more from baseline in Part 1 were randomized to receive either sepiapterin or placebo in Part 2 of the clinical trial. The primary analysis population consists of those who had greater than 30% reduction in phenylalanine levels from baseline during Part 1 of the trial. The primary outcome measure is the reduction of blood phenylalanine levels from baseline compared to Weeks 5 and 6 in patients from Part 2 of the clinical trial. All patients are eligible to enroll in an open label long term clinical trial designed to further evaluate the long-term safety and durable effect of sepiapterin.

About Sepiapterin
Sepiapterin (formerly PTC923) is an oral formulation of synthetic sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in the metabolism and synthesis of numerous metabolic products. Sepiapterin is a more bioavailable precursor than exogenously administered synthetic BH4 and has the potential to treat the broad range of PKU patients.

About Phenylketonuria
Phenylketonuria (PKU) is a rare, inherited metabolic disease, which affects the brain.[1] It is caused by a defect in the gene that helps create the enzyme needed to break down phenylalanine.[1] If left untreated or poorly managed, phenylalanine – an essential amino acid found in all proteins and most foods – can build up to harmful levels in the body. This causes severe and irreversible disabilities, such as permanent intellectual disability, seizures, delayed development, memory loss, and behavioral and emotional problems.[1] Newborns with phenylketonuria initially don't have any symptoms, but symptoms are usually progressive, and damage caused by toxic levels of phenylalanine in the first few years of life is irreversible.[2,3] Diagnosis of phenylketonuria usually takes place during newborn screening programs.[4] There are an estimated 58,000 people with phenylketonuria globally.

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 innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and Twitter at @PTCBio.

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Forward-Looking Statement
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this press release, other than statements of historic fact, are forward-looking statements, including statements with respect to 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, 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; expectations with respect to potential regulatory submissions and commercialization of sepiapterin for phenylketonuria, or PKU, and potential development and regulatory milestone payments that PTC may be obligated to make with regards to sepiapterin, expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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 sepiapterin for PKU; PTC's scientific approach and general development progress; 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; and the factors discussed in the "Risk Factors" section of PTC's most recent 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, including sepiapterin.

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 presentation except as required by law.

References:

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