PTC Therapeutics to Present Recent Data for Sepiapterin at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium

August 28, 2023

- Results from the APHENITY trial in PKU and long-term extension study including Phe tolerance to be highlighted -

SOUTH PLAINFIELD, N.J., Aug. 28, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that results from the APHENITY clinical trial and long-term extension study will be presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium 2023 which is being held from Aug. 29 to Sept. 1 in Jerusalem, Israel.

Topline results of the APHENITY trial will be presented in an oral presentation, "Phase 3 APHENITY Study: Oral Sepiapterin for the Treatment of Phenylketonuria." The presentation will take place on Thursday, Aug. 31 during the Parallel Session: Phenylketonuria, beginning at 10:45 IST.

The poster "B284: Phase 3 APHENITY Long-Term Study Design: Sepiapterin for Treatment of Phenylketonuria," will also be presented on Wednesday, Aug. 30 during the poster walk at 18:35 IST. The poster contains an updated data cut of the ongoing APHENITY long-term extension study. Phe tolerance results continued to demonstrate that sepiapterin treated patients maintain mean blood Phe control (>360 µmol/L) while significantly increasing protein intake, consistent with previous results.

PTC will also be sponsoring a symposium, "Advancing the Treatment of PKU: Results of the APHENITY Trial," which will be hosted on Aug. 31 at 12:30 IST in Dulcin Hall. The symposium will bring together leading international experts to discuss the latest results from the APHENITY trial, highlight the unique features of sepiapterin, and share findings from the dietary Phe tolerance assessment in the open-label extension study.

In addition, PTC will share ongoing research for aromatic l-amino acid decarboxylase (AADC) deficiency during an oral presentation, poster and sponsored symposium.

More information about the SSIEM Annual Symposium can be found at ssiem.org.

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 percent 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 percent 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.

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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; 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.

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:

1. de Groot MJ, Hoeksma M, Blau N, et al. Mol Genet Metab 2010;99:S86–S89.
2. Phenylketonuria (PKU). Available at: https://www.mayoclinic.org/diseases-conditions/phenylketonuria/symptoms-causes/syc-20376302. Accessed October 2021.
3. Blau N, van Spronsen FJ, Levy HL. Lancet 2010;376:1417–1427.
4. Al Hafid N, Christodoulou J. Transl Pediatr 2015;4(4):304–317.

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