



PTC Therapeutics Announces Validation of Sepiapterin European MAA

May 28, 2024

- Review of European marketing application for PKU now initiated -

WARREN, N.J., May 28, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that the sepiapterin MAA for PKU has been validated and accepted for review by the EMA.

"The validation of the MAA is an important step towards making sepiapterin available to children and adults affected by PKU in Europe," said Matthew B. Klein, M.D., Chief Executive Officer of PTC Therapeutics. "The European submission is the first of several global submissions planned in 2024."

PTC expects to submit the sepiapterin NDA to the FDA no later than the third quarter of 2024. In addition, submissions are planned in a number of additional countries in 2024 including Brazil and Japan.

The sepiapterin MAA includes data from the phase 3 APHENITY trial which demonstrated a mean reduction in Phe levels of 63% in the overall treated population and 69% in the subgroup of subjects with classical PKU. The vast majority of subjects (84%) achieved Phe control in accordance with treatment guidelines of <360 µmol/L, and 22% of subjects had normalization of Phe levels. The application also includes data from the APHENITY open-label extension study which provides evidence of sepiapterin durability of effect as well as data from the Phe tolerance sub-study. Phe tolerance sub-study results collected to date demonstrate that subjects are liberalizing their diet, beyond the recommended daily allowance of protein, and maintaining control of Phe within guideline target levels of <360 µmol/L. The ability to enable patients to liberalize their highly restrictive diets and still maintain Phe control is a very meaningful sepiapterin treatment benefit for patients as well as for physicians and payers.

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. It is caused by a defect in the gene that helps create the enzyme needed to break down phenylalanine. 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. 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. Diagnosis of phenylketonuria usually takes place during newborn screening programs. There are an estimated 58,000 people with phenylketonuria globally.

About PTC Therapeutics, Inc.

PTC is a 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 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 regulatory submissions and responses, commercialization and other matters with respect to its products and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; 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 sepiapterin, including any regulatory submissions and potential approvals, commercialization, the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; 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, 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 press release except as required by law.

Acronyms:

EMA: European Medicines Agency

FDA: U.S. Food and Drug Administration

MAA: Marketing Authorization Application

NDA: New Drug Application

Phe: Phenylalanine

PKU: Phenylketonuria

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