PTC Therapeutics Receives Positive CHMP Opinion for Sephience™ (sepiapterin) for the Treatment of Children and Adults Living with Phenylketonuria (PKU)

April 25, 2025

- Label includes full spectrum of PKU patients -

- European launch preparations underway -

WARREN, N.J., April 25, 2025 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has issued a positive opinion on the marketing authorization application for Sephience™ (sepiapterin) for the treatment of children and adults living with phenylketonuria (PKU). The opinion includes a broad label inclusive of all ages and disease severities.

"This positive opinion is great news for the PKU patient community in Europe for whom there remains a significant unmet medical need," said Matthew B. Klein, M.D., Chief Executive Officer of PTC Therapeutics. "We look forward to initiating the European launch immediately following the adoption of this positive opinion by the European Commission. The data collected to date continue to support the potential for Sephience to provide a safe and effective treatment that not only lowers phenylalanine levels but also enables diet liberalization even for those with the most severe disease subtypes."

Launch planning in Europe is well underway with priorities in Germany and other key European markets where named patient access will be immediately available.

PTC expects the European Commission to ratify the marketing authorization for Sephience in approximately two months. The decision will be applicable to all 27 European Union member states, as well as Iceland, Norway and Liechtenstein.

The New Drug Application (NDA) for sepiapterin is under review by the U.S. Food and Drug Administration (FDA) with a Prescription Drug User Fee Act (PDUFA) target action date of July 29, 2025. Review of approval applications is ongoing in several other countries including Japan and Brazil.

About Sephience™ (sepiapterin)
Sephience, an oral formulation of synthetic sepiapterin, has a dual mechanism of action to increase activity of the phenylalanine hydroxylase (PAH) enzyme. First, Sephience is a precursor compound that is rapidly absorbed and converted intracellularly to tetrahydrobiopterin (BH4), a critical cofactor of PAH. Sephience also has an independent pharmacological chaperone effect, correcting PAH misfolding to enhance the enzyme function. Through this dual mechanism of action, Sephience effectively reduces blood phenylalanine (Phe) levels and has the potential to treat a 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 (Phe). If left untreated or poorly managed, Phe – 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 PKU initially don't have any symptoms, but symptoms are usually progressive, and damage caused by toxic levels of Phe in the first few years of life is irreversible. Diagnosis of PKU usually takes place during newborn screening programs. There are an estimated 58,000 people living with PKU globally.

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company that discovers, develops and commercializes clinically differentiated medicines that provide benefits to children and adults living with rare disorders. PTC's ability to innovate new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, X, and LinkedIn.

For More Information:

Investors:
Ellen Cavaleri
+1 (615) 618-6228
ecavaleri@ptcbio.com

Media:
Jeanine Clemente
+1 (908) 912-9406
jclemente@ptcbio.com

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 clinical trials and studies, availability of data, regulatory submissions and responses, commercialization and other matters with respect to its products and product candidates; expectations with respect to Sephience, including any regulatory decision made by the European Commission; 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; expectations with respect to Sephience, including any regulatory submissions and potential approvals, commercialization and the potential achievement of 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 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 Sephience.

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:https://www.prnewswire.com/news-releases/ptc-therapeutics-receives-positive-chmp-opinion-for-sephience-sepiapterin-for-the-treatment-of-children-and-adults-living-with-phenylketonuria-pku-302438377.html

SOURCE PTC Therapeutics, Inc.