Sephience™ (sepiapterin) Granted Marketing Authorization by the European Commission for the Treatment of Children and Adults Living with Phenylketonuria (PKU)

June 23, 2025

– Broad label inclusive of all ages and disease severities –

– Launch to be initiated in Germany –

WARREN, N.J., June 23, 2025 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Sephience™ (sepiapterin) was granted marketing authorization by the European Commission for the treatment of children and adults living with phenylketonuria (PKU). The authorization includes a broad label inclusive of all ages and disease severities.

"The European approval of Sephience is a great step in our efforts to bring this safe and highly effective therapy to children and adults affected by PKU worldwide," said Matthew B. Klein, M.D., Chief Executive Officer of PTC Therapeutics. "The broad label supports that potential for Sephience to address all key PKU patient segments and become the new standard of care."

The European approval is based on the highly statistically significant results from the Phase 3 APHENITY trial as well as evidence of durable treatment effect and the ability of study participants to liberalize their diet in the APHENITY long-term extension study.

The marketing authorization is applicable to all 27 European Union member states as well as Iceland, Norway and Liechtenstein. The Sephience European launch will be initiated in Germany in the first half of July.

A New Drug Application (NDA) for sepiapterin remains on schedule for its FDA 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 ™(sepiapterin) is indicated for the treatment of hyperphenylalaninaemia (HPA) in adult and pediatric patients with phenylketonuria (PKU). Sephience is a natural precursor of the enzymatic co-factor BH4, a critical co-factor for phenylalanine hydroxylase (PAH). Sephience acts as a dual pharmacological chaperone (sepiapterin and BH4 each with its own binding affinity to variant PAH), including PAH variants commonly found in PKU and known to be insensitive to BH4, to improve the activity of the defective PAH enzyme, achieving a high concentration of BH4 intracellularly. By enhancing the conformational stability of misfolded PAH enzyme and increasing the intracellular concentrations of BH4, Sephience is able to effectively reduce blood Phe levels. Sephience (sepiapterin) is approved in the European Economic Area. Sepiapterin is an investigational new drug in the United States.

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 do not 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 focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to children and adults living 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. The company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit www.ptcbio.com and follow on Facebook, X, and LinkedIn.

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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 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 timing of commercialization and of potential regulatory decisions; 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.

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