



PTC Therapeutics Announces Approval of Sephience™ (sepiapterin) for the Treatment of Children and Adults Living with Phenylketonuria (PKU) in Japan

December 22, 2025

*– Indication includes all ages and the full spectrum of disease severity –
– First Japan product approval for PTC –*

WARREN, N.J., Dec. 22, 2025 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today that Sephience™ (sepiapterin) has been approved by the Japanese Ministry of Health, Labor and Welfare (MHLW) for the treatment of children and adults living with phenylketonuria (PKU). The label includes individuals of all ages and the full spectrum of disease severity.

"The approval of Sephience in Japan is an important milestone for Japanese patients living with PKU as well as for PTC," said Matthew B. Klein, M.D., Chief Executive Officer of PTC Therapeutics. "I am proud that we have been able to achieve approvals for Sephience in the EU, US and Japan within less than six months, allowing for a true global launch. Our team in Japan is fully built and excited to bring Sephience to any individual who may benefit."

PTC will now engage in pricing discussions which are expected to conclude in Q1 2026, with launch occurring shortly thereafter.

About Sephience™ (sepiapterin)

Sephience™ is indicated for the treatment of 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). Through its mechanism of action, Sephience is able to effectively reduce blood phenylalanine (Phe) levels and has the potential to treat a broad range of PKU patients. Sephience is approved in the European Union / European Economic Area region, the United States and Japan.

About Phenylketonuria

Phenylketonuria (PKU) is a rare, inherited metabolic disease, characterized by the body's inability to break down an essential amino acid called phenylalanine (Phe) and which can result in neurological and other symptoms. If left untreated or poorly managed, Phe 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 dedicated to the discovery, development and commercialization of clinically differentiated medicines for children and adults living with rare disorders. PTC is advancing a robust and diversified pipeline of transformative medicines as part of its mission to provide access to best-in-class treatments for patients with unmet medical needs. The company's strategy is to leverage its scientific expertise and global commercial infrastructure to optimize value for patients and other stakeholders.

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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; 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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