# PTC Therapeutics Announces New Sephience™ (sepiapterin) Data Reinforcing Broad Patient Benefits Including Sustained Metabolic Control and Diet Liberalization as well as Consistent Favorable Safety Profile

August 24, 2026

*– 17 abstracts and presentations planned at SSIEM Symposium –*

*– Real-world evidence demonstrates significant diet liberalization achieved with Sephience while maintaining target blood Phe levels –*

*– Continued evidence of meaningful benefit across all patient subgroups –*

WARREN, N.J., Aug. 24, 2026 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that multiple Sephience™ (sepiapterin) scientific data presentations will be featured at the 2026 Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium, taking place in Helsinki, Finland, from Aug. 25-28. The presentations include new data from clinical trials and real-world evidence which reinforce the clinically meaningful benefits of Sephience on lowering phenylalanine (Phe), significant diet liberalization and sustained metabolic control for the full spectrum of individuals living with phenylketonuria (PKU) including those with classical PKU. In addition, Sephience studies continue to show a consistent and favorable safety profile.

"The SSIEM presentations further demonstrate the broad clinical benefits of Sephience across the full spectrum of individuals living with PKU," said Matthew B. Klein, M.D., Chief Executive Officer. "In addition, new data to be presented at the PTC symposium show treatment with Sephience led to a large number of responsive participants achieving normalization of blood Phe levels (<120 µmol/L) in a rapid timeframe, including those with classical or non-BH4-responsive PKU. These impressive data support the potential benefits Sephience can deliver for individuals affected by PKU."

**Highlights of the data to be presented at SSIEM 2026 include:**

- New analyses from the AMPLIPHY study demonstrate that Sephience treatment resulted in a 100% greater reduction in blood Phe for participants on sapropterin at screening after switching to Sephience.
- In an analysis of participants in Sephience studies with high baseline Phe levels (≥900 µmol/L), Sephience treatment resulted in clinically meaningful reductions in blood Phe levels within 14 days, with response rates comparable to the overall study population, and a safety profile consistent with prior studies. These findings support a trial of Sephience treatment in individuals with PKU regardless of severity or baseline Phe.
- An analysis of real-world data performed by a leading global key opinion leader shows that Sephience enabled significant dietary liberalization in adolescents, supporting greater independence, reduced dietary burden, and maintained metabolic control within recommended targets. These results were observed in individuals with both BH4-responsive and classical/non-BH4 responsive PKU mutations.

**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 unique dual 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 United States, the European Union/European Economic Area region, Japan and other countries.

**Indication and Important Safety Information**

**Indication**
SEPHIENCE is indicated for the treatment of hyperphenylalaninemia (HPA) in adult and pediatric patients 1 month of age and older with sepiapterin-responsive phenylketonuria (PKU). SEPHIENCE is to be used in conjunction with a phenylalanine (Phe)-restricted diet.

**Contraindications**
None.

**Important Safety Information**
Treatment with SEPHIENCE should be directed by physicians knowledgeable in the management of PKU. Biochemical response to SEPHIENCE can only be determined by a therapeutic trial with careful monitoring of ongoing dietary and nutritional balance to ensure adequate Phe control.

**Warnings and Precautions**

- Increased Bleeding: SEPHIENCE may increase the risk of bleeding. Bleeding events, including superficial hematomas, prolonged bleeding, and heavy menstrual bleeding have occurred in patients treated with SEPHIENCE. Inform patients about the risk of bleeding associated with SEPHIENCE and have patients follow up with their healthcare provider should such a bleeding event occur. Consider treatment interruption with SEPHIENCE in patients with active bleeding.
- Hypophenylalaninemia: Some pediatric patients receiving SEPHIENCE experienced hypophenylalaninemia. Monitor blood Phe levels during treatment and modify the dosage of SEPHIENCE and/or dietary protein and Phe intake as needed to ensure adequate blood Phe level control. Frequent blood monitoring is recommended in the pediatric population.
- Interaction with Levodopa: In a 10-year post-marketing safety surveillance program for a non-PKU indication using another drug that is a phenylalanine hydroxylase (PAH) activator, three patients with underlying neurological disorders experienced seizures, exacerbation of seizures, over-stimulation, and irritability during co-administration with levodopa. Monitor patients who are receiving levodopa for changes in neurological status during treatment with SEPHIENCE.

**Adverse Reactions**
Most common adverse reactions with SEPHIENCE (≥2% and > placebo) were diarrhea, headache, abdominal pain, hypophenylalaninemia, feces discoloration and oropharyngeal pain.

**Drug Interactions**
Avoid concomitant use of drugs known to inhibit folate synthesis dihydrofolate reductase (DHFR) (e.g., trimethoprim, methotrexate, trimetrexate, pemetrexed, pralatrexate, raltitrexed, and piritrexim) while taking SEPHIENCE. Concomitant administration of such drugs may reduce sepiapterin metabolism to BH4. If concomitant use is not avoidable, monitor blood Phe levels.

SEPHIENCE and PDE-5 inhibitors (e.g., sildenafil, vardenafil, or tadalafil) induce vasorelaxation and may reduce blood pressure. Monitor for signs and symptoms of hypotension.

For medical information, product complaints, or to report an adverse event, please call 1-866-562-4620 or email usmedinfo@ptcbio.com.

You may also report adverse events directly to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see Full Prescribing Information.

**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. To learn more about PTC, please visit www.ptcbio.com and follow us on LinkedIn, X, Instagram and Facebook.

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**Forward-Looking Statements:**
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; 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 commercialization and the potential achievement of 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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