



PTC Therapeutics, Inc.
500 Warren Corporate Center Drive
Warren, N.J. 07059

September 2, 2025

Update to the PKU community:

We're excited to share that our therapy, Sephience™ (sepiapterin), is now available through our specialty pharmacy network. Sephience (pronounced: seh-FIGH-ence) was recently approved by the U.S. Food and Drug Administration (FDA) with broad labeling for the treatment of hyperphenylalaninemia (HPA) in adult and pediatric patients 1 month of age and older with sepiapterin-responsive PKU. Sephience is to be used in conjunction with a phenylalanine (Phe)-restricted diet.

The FDA approval underscores Sephience's potential to address the significant unmet needs of individuals living with PKU. Sephience has been demonstrated to address the unmet need as a well-tolerated treatment that helps lower blood Phe levels in a broad range of people, including those with the most severe form of the disease.

If you have any questions about treatment with Sephience, please talk to your healthcare provider and contact our [PTC Cares™](#) team for treatment coordination questions. Our case managers are ready to provide personalized assistance and address inquiries about the therapy, prescription start forms, insurance verification, co-pay assistance, and specialty pharmacy shipments.

We extend our deepest gratitude to the PKU community for your unwavering support in reaching this significant milestone. This achievement would not have been possible without the contributions of those who participated in the Sephience clinical trials, including individuals living with PKU, their families, healthcare professionals, and our advocacy partners. Thank you for your invaluable partnership.

For more information about Sephience, visit <https://www.sephience.com/>

Please see Indication and Important Safety Information below. A link to the full Prescribing Information is available here: [Full Prescribing Information](#)

What is SEPHIENCE?

SEPHIENCE™ (sepiapterin) is a prescription medicine used to lower blood levels of phenylalanine (Phe) in adults and children 1 month of age and older with a certain type of Phenylketonuria (PKU). SEPHIENCE is used along with a Phe-restricted diet. It is not known if SEPHIENCE is safe and effective in children younger than 1 month of age.

What is the most important information I should know about SEPHIENCE?
SEPHIENCE may cause serious side effects, including:

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- **increased bleeding problems.** SEPHIENCE may increase your risk of bleeding. Tell your healthcare provider if you develop any symptoms of bleeding including:
 - bruising or red or purple skin marks
 - menstrual bleeding that is heavier than normal
 - bleeding longer than usual or that does not stop
- **low blood Phe (hypophenylalaninemia).** Low blood Phe levels are common during treatment with SEPHIENCE and can be severe.

The most common side effects of SEPHIENCE include: diarrhea, headache, stomach (abdominal) pain, yellow or orange stool (feces), and throat pain.

Tell your healthcare provider if you have any side effect that bothers you or that does not go away. These are not all the possible side effects of SEPHIENCE. Call your healthcare provider for medical advice about side effects.

Before taking or giving SEPHIENCE, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. It is not known if SEPHIENCE will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if SEPHIENCE passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby if you take SEPHIENCE.

Tell your healthcare provider about all medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. SEPHIENCE and other medicines may affect each other causing side effects. SEPHIENCE may affect the way other medicines work, and other medicines may affect how SEPHIENCE works. Especially tell your healthcare provider if you take:

- a medicine that contains levodopa
- an antifolate medicine
- another medicine also used to lower Phe
- medicines used to treat erectile dysfunction (phosphodiesterase 5 inhibitors or PDE5)

Know the medicines you take. Keep a list of them with you to show your healthcare provider and pharmacist when you get a new medicine.

How should I take or give SEPHIENCE?

- Take or give SEPHIENCE exactly as your healthcare provider tells you. Your healthcare provider will tell you how much SEPHIENCE to take or give.
- Your healthcare provider may change your or your child's dose of SEPHIENCE depending on your weight and age.



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- Take or give SEPHIENCE 1 time each day with food. You must take or give more food after the SEPHIENCE dose.
- SEPHIENCE comes as a packet containing oral powder.
 - For SEPHIENCE doses **less than** 1,000 mg: mix SEPHIENCE oral powder with water or apple juice before giving or taking.
 - For SEPHIENCE doses 1,000 mg or **greater**: SEPHIENCE oral powder can be mixed with water, apple juice, strawberry jam, or applesauce before taking or giving.
 - **See the detailed Instructions for Use that comes with SEPHIENCE for information about the correct way to prepare, take, or give a dose of SEPHIENCE oral powder.**
- If you or your child miss a dose of SEPHIENCE, take or give it as soon as possible. Do not take 2 doses in a day. Continue with your normal dosing schedule the following day.
- During treatment with SEPHIENCE, you or your child will have regular check-ups with your healthcare provider.
- Your healthcare provider will do blood tests before and during your treatment with SEPHIENCE to check your blood Phe levels.
- Your healthcare provider may change your dose, temporarily stop, or permanently stop treatment with SEPHIENCE if you have certain side effects.
- Your healthcare provider may change your diet during treatment. Follow your healthcare provider's instructions and do not make any changes to your diet without first talking to your healthcare provider.

To report an adverse event, please call 1-866-562-4620 or email at usmedinfo@ptcbio.com. You may also report side effects to FDA at 1-800-FDA-1088 or at www.fda.gov/medwatch.

Please see [Full Prescribing Information](#).