25 April 2025
EMA/CHMP/132388/2025
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Sephience

sepiapterin

On 25 April 2025, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Sephience[2], intended for the treatment of hyperphenylalaninaemia in adults and children with phenylketonuria (PKU).

The applicant for this medicinal product is PTC Therapeutics International Limited.

Sephience will be available as a 250 mg and 1000 mg oral powder. The active substance of Sephience is sepiapterin, an alimentary tract and metabolism product (ATC code: A16AX28). It is a natural precursor of tetrahydrobiopterin, a critical co-factor of phenylalanine hydroxylase. Sepiapterin has a dual pharmacological activity, acting as a chaperone in case of a misfolded phenylalanine hydroxylase improving the enzyme's conformational stability as well as by increasing the intracellular concentrations of tetrahydrobiopterin, thereby reducing blood phenylalanine levels.

The benefits of Sephience are its ability to significantly reduce blood phenylalanine levels after 6 weeks *versus* placebo, as observed in a double-blind, randomised, placebo-controlled clinical study which included patients of all ages with PKU.

The most common side effects are upper respiratory tract infection, headache, diarrhoea, abdominal pain, hypophenylalaninaemia and discoloration of faeces.

The full indication is:

> Sephience is indicated for the treatment of hyperphenylalaninaemia (HPA) in adult and paediatric patients with phenylketonuria (PKU).

Sephience must be initiated and supervised by a physician experienced in the treatment of PKU.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published on the EMA website in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union