EMA/50045/2024

European Medicines Agency decision

P/0041/2024

of 2 February 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral for sepiapterin (EMEA-003027-PIP02-23) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

European Medicines Agency decision

P/0041/2024

of 2 February 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral for sepiapterin (EMEA-003027-PIP02-23) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by PTC Therapeutics International Limited on 20 March 2023 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 19 January 2024, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the agreement of a paediatric investigation plan and on the granting of a deferral.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for sepiapterin, oral powder, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for sepiapterin, oral powder, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to PTC Therapeutics International Limited, 5th Floor, 3 Grand Canal Plaza Grand Canal Street Upper, Dublin 4, D04 EE70 – Dublin, Ireland.

EMA/PDCO/481353/2023
Amsterdam, 19 January 2024

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral

EMEA-003027-PIP02-23

Scope of the application

Active substance(s):

Sepiapterin

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of hyperphenylalaninaemia

Pharmaceutical form(s):

Oral powder

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

PTC Therapeutics International Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, PTC Therapeutics International Limited submitted for agreement to the European Medicines Agency on 20 March 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 24 April 2023.

Supplementary information was provided by the applicant on 16 October 2023. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of hyperphenylalaninaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of hyperphenylalaninaemia in patients with phenylketonuria (excluding primary BH4 deficiency)

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Oral powder

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of a 125 mg strength oral powder.
Non-clinical studies	Not applicable
Clinical studies	**Study 2 (PTC923-MD-003-PKU)** Open-label run-in (Part 1) followed by a double-blind, randomised, placebo-controlled (Part 2) study to evaluate pharmacokinetics, safety, and efficacy of sepiapterin in children from birth to less than 18 years of age (and adults) with phenylketonuria (PKU). **Study 3 (PTC923-MD-004-PKU)** Open-label, uncontrolled, long-term study to evaluate long-term safety of sepiapterin in children from birth to less than 18 years of age (and adults) with phenylketonuria (PKU). **Study 4 (PTC923-PKU-401)** Open-label study to evaluate long-term neurocognitive outcomes in children from 1 month to less than 10 years with phenylketonuria treated with sepiapterin.

Modelling and simulation studies	**Study 5 (PTC923-2021-022)** Modelling and simulation study to support dose finding in children from birth to less than 18 years of age with hyperphenylalaninaemia.
Other studies	Not applicable
Extrapolation plan	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By March 2031
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.