EMA/298061/2024

European Medicines Agency decision

P/0241/2024

of 19 July 2024

on the acceptance of a modification of an agreed paediatric investigation plan for pegvaliase (Palynziq), (EMEA-001951-PIP01-16-M03) 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.

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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 European Medicines Agency's decision P/0036/2017 issued on 31 January 2017 and the decision P/0089/2022 issued on 11 March 2022,

Having regard to the application submitted by BioMarin International Limited on 22 February 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 May 2024, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[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

Changes to the agreed paediatric investigation plan for pegvaliase (Palynziq), solution for injection, subcutaneous use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to BioMarin International Limited, Shanbally, Ringaskiddy County Cork, P43 R298 – Ringaskiddy, Ireland.

EMA/PDCO/103374/2024
Amsterdam, 31 May 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-001951-PIP01-16-M03

Scope of the application

Active substance(s):

Pegvaliase

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of hyperphenylalaninaemia

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

BioMarin International Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, BioMarin International Limited submitted to the European Medicines Agency on 22 February 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0036/2017 issued on 31 January 2017 and the decision P/0089/2022 issued on 11 March 2022.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 2 April 2024.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
    - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.
2. The measures and timelines of the 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 paediatric patients with phenylketonuria

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)

Solution for injection

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	**Study 1** A randomized (2:1), open label trial to evaluate the efficacy, safety and pharmacokinetics of subcutaneous injections of pegvaliase in adolescent patients (12 years to less than 16 years of age) with phenylketonuria against dietary only management, followed by a long term treatment extension. **Study 2** An open-label, randomized, controlled study to evaluate the efficacy, safety and pharmacokinetics of subcutaneous injections of pegvaliase in children with phenylketonuria. **Study 3** An open-label, randomized, controlled study to evaluate the efficacy, safety and pharmacokinetics of subcutaneous injections of pegvaliase in infants, toddlers and children with phenylketonuria.
Extrapolation, modelling and simulation studies	Not applicable.

Other studies	Not applicable.
Other measures	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2030
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:

Condition(s) and authorised indication(s)

1. Treatment of hyperphenylalaninaemia

Authorised indication(s):

- Treatment of patients with phenylketonuria (PKU) aged 16 years and older who have inadequate blood phenylalanine control (blood phenylalanine levels greater than 600 micromol/l) despite prior management with available treatment options.
  - Invented name(s): Palynziq
  - Authorised pharmaceutical form(s): Solution for injection
  - Authorised route(s) of administration: Subcutaneous use
  - Authorised via centralised procedure.