



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1789262

European Medicines Agency decision

EMA/PE/0000238074

of 27 November 2024

on the acceptance of a modification of an agreed paediatric investigation plan for mitapivat (Pryrukind)
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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on the acceptance of a modification of an agreed paediatric investigation plan for mitapivat (Pryrukind) 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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0150/2022 issued on 13 May 2022,

Having regard to the application submitted by Agios Netherlands B.V. on 19 November 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 25 November 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 mitapivat (Pryrukind) 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 covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0365/2020 issued on 09 September 2020, including subsequent modifications thereof.

Article 3

This decision is addressed to Agios Netherlands B.V., Zuidplein 36, 1077 XV – Amsterdam, The Netherlands.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1776051

Amsterdam, 25 November 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA/PE/0000238074

Scope of the application

Active substance(s):

Mitapivat

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of thalassaemia

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate oral solid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Agios Netherlands B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Agios Netherlands B.V. submitted to the European Medicines Agency on 19 November 2024 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0150/2022 issued on 13 May 2022.

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

The procedure started on 20 November 2024.



Scope of the modification

An administrative modification to link two paediatric investigation plans for regulatory purposes.

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.

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

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

1.1. Condition:

Treatment of thalasseamia

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- film-coated tablet, age-appropriate oral solid dosage form, oral use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of thalassaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients with thalassaemia

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

From 1 year to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate oral solid dosage form

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 (<i>Same study as EMEA-002684-PIP01-19</i>) Development of an age-appropriate oral solid dosage form (coated granules) for use in paediatric patients unable to swallow the available tablets
Non-clinical studies	Not applicable
Clinical studies	Study 2 Double-blind, randomised, placebo-controlled trial to evaluate the efficacy, pharmacokinetics and safety of mitapivat in children from 1 year to less than 18 years of age with alpha- or beta-transfusion-dependent thalassemia.

	Study 3 Double-blind, randomised, placebo-controlled trial to evaluate the efficacy, pharmacokinetics and safety of mitapivat in children from 1 year to less than 18 years of age with alpha- or beta- non-transfusion-dependent thalassemia.
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 2029
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 pyruvate kinase deficiency

Authorised indication(s):

- Pyrukynd is indicated for the treatment of pyruvate kinase deficiency (PK deficiency) in adult patients
 - Invented name(s): Pryrukind
 - Authorised pharmaceutical form(s): film-coated tablet
 - Authorised route(s) of administration: oral use
 - Authorised via centralised procedure