EMA/516252/2023

European Medicines Agency decision

P/0500/2023

of 1 December 2023

on the acceptance of a modification of an agreed paediatric investigation plan for treprostinil (sodium) (Trisuva and associated names), (EMEA-003182-PIP01-22-M01) 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/0427/2022 issued on 28 October 2022,

Having regard to the application submitted by AOP Orphan Pharmaceuticals GmbH on 3 July 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 October 2023, 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 and on the refusal of a deferral.

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

(3) It is therefore appropriate to adopt a decision refusing 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

Changes to the agreed paediatric investigation plan for treprostinil (sodium) (Trisuva and associated names), solution for infusion, subcutaneous use, intravenous 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

A deferral for treprostinil (sodium) (Trisuva and associated names), solution for infusion, subcutaneous use, intravenous use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby refused.

Article 3

This decision is addressed to AOP Orphan Pharmaceuticals GmbH, Leopold-Ungar-Platz 2, 1190 – Vienna, Austria.

EMA/PDCO/316915/2023
Amsterdam, 13 October 2023

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

EMEA-003182-PIP01-22-M01

Scope of the application

Active substance(s):

Treprostinil (sodium)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of pulmonary arterial hypertension

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Subcutaneous use

Intravenous use

Name/corporate name of the PIP applicant:

AOP Orphan Pharmaceuticals GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, AOP Orphan Pharmaceuticals GmbH submitted to the European Medicines Agency on 3 July 2023 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0427/2022 issued on 28 October 2022.

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

The procedure started on 14 August 2023.

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;
   - to refuse a deferral as it does not meet the grounds detailed in Article 20(1) 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 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 pulmonary arterial hypertension

2.1.1. Indication(s) targeted by the PIP

Treatment of severe pulmonary arterial hypertension (PAH Group 1) to improve symptoms of the disease in paediatric patients

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 infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1 - TREPed-01** Open-label, single-arm, non-controlled trial to evaluate safety and tolerability of treprostinil sodium in children from birth to less than 18 years of age with pulmonary arterial hypertension **Study 2 - TREPed-02** This study was deleted as a result of procedure EMEA-003182-PIP01-22-M01
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:	No
Date of completion of the paediatric investigation plan:	By June 2026
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of pulmonary arterial hypertension

Authorised indication(s):

- Treatment of idiopathic or heritable pulmonary arterial hypertension (PAH) to improve exercise tolerance and symptoms of the disease in patients classified as New York Heart Association (NYHA) functional class III
  - Invented name(s): Trisuva and associated names
  - Authorised pharmaceutical form(s): solution for infusion
  - Authorised route(s) of administration: subcutaneous or intravenous administration
  - Authorised via national procedure