EMA/236607/2023

European Medicines Agency decision

P/0206/2023

of 14 June 2023

on the acceptance of a modification of an agreed paediatric investigation plan for treprostinil (Remodulin), (EMEA-000207-PIP01-08-M08) 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/121/2008 issued on 1 December 2008, the decision P/0074/2012 issued on 25 April 2012, the decision P/0291/2012 issued on 18 December 2012, the decision P/0308/2013 issued on 19 December 2013, the decision P/0059/2015 issued on 1 April 2015 and the decision P/0144/2018 issued on 7 May 2018,

Having regard to the application submitted by Ferrer Internacional, S.A. on 19 January 2023 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 26 April 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 to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the 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 (Remodulin), solution for infusion, subcutaneous use, intravenous use, including changes to the deferral, 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 Ferrer Internacional, S.A., Avenida Diagonal 549, 5ª planta, 08029 – Barcelona, Spain.

EMA/PDCO/56811/2023
Amsterdam, 26 April 2023

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

EMEA-000207-PIP01-08-M08

Scope of the application

Active substance(s):

Treprostinil

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:

Ferrer Internacional, S.A.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Ferrer Internacional, S.A. submitted to the European Medicines Agency on 19 January 2023 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/121/2008 issued on 1 December 2008, the decision P/0074/2012 issued on 25 April 2012, the decision P/0291/2012 issued on 18 December 2012, the decision P/0308/2013 issued on 19 December 2013, the decision P/0059/2015 issued on 1 April 2015 and the decision P/0144/2018 issued on 7 May 2018.

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

The procedure started on 27 February 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 and to the deferral 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 pulmonary arterial hypertension

The waiver applies to:

- children from one month to less than 12 years of age;
- solution for infusion, subcutaneous use, intravenous use;
- on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the paediatric population.

2. Paediatric investigation plan

2.1. Condition:

Treatment of pulmonary arterial hypertension

2.1.1. Indication(s) targeted by the PIP

Treatment of pulmonary arterial hypertension

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

From birth to one month of age and from 12 years 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	**Study 1** Development of an intravenous formulation
Non-clinical studies	Not applicable
Clinical studies	**Study 3** A comparative study of intravenously administered treprostinil in neonates with Persistent Pulmonary Hypertension of neonates (PPHN) treated in Paediatric Intensive Care Unit.
Extrapolation, modelling and simulation studies	Not applicable
Other studies	**Study 2**

	Analyses of existing data to clarify the safety and efficacy of the parenteral formulation of treprostinil in children aged from 12 to less than 18 years, as systematic review. **Study 4** *(Study added in procedure EMEA-000207-PIP01-08-M08)* Multicentre, non-interventional, retrospective and prospective study collecting clinical data on neonates with pulmonary hypertension (PH) treated with intravenous and subcutaneous treprostinil.
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 April 2027
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 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 NYHA (New York Association) Class III
  - Invented name(s): Remodulin
  - Authorised pharmaceutical form(s): Solution for infusion
  - Authorised route(s) of administration: Subcutaneous use and intravenous use
  - Authorised via mutual recognition procedure