EMA/777575/2022

European Medicines Agency decision

P/0414/2022

of 29 September 2022

on the acceptance of a modification of an agreed paediatric investigation plan for sotatercept (EMEA-002756-PIP01-19-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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on the acceptance of a modification of an agreed paediatric investigation plan for sotatercept (EMEA-002756-PIP01-19-M01) 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 European Medicines Agency's decision P/0227/2021 issued on 8 June 2021,

Having regard to the application submitted by Merck Sharp & Dohme B.V. on 18 May 2022 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 9 September 2022, 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 sotatercept, powder for solution for injection, subcutaneous 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 Merck Sharp & Dohme B.V., Waarderweg 39, 2031 BN - Haarlem, The Netherlands.

EMA/PDCO/576491/2022 Corr
Amsterdam, 9 September 2022

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

EMEA-002756-PIP01-19-M01

Scope of the application

Active substance(s):

Sotatercept

Condition(s):

Treatment of pulmonary arterial hypertension

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Merck Sharp & Dohme B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Merck Sharp & Dohme B.V. submitted to the European Medicines Agency on 18 May 2022 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/0227/2021 issued on 8 June 2021.

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

The procedure started on 11 July 2022.

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 annex 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 (PAH)

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- powder for solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of pulmonary arterial hypertension

2.1.1. Indication(s) targeted by the PIP

Treatment of patients with PAH (WHO Group1) to improve exercise tolerance and functional class

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)

Powder for solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of an age-appropriate dosage form
Non-clinical studies	Not applicable
Clinical studies	**Study 2** Open-label, 24-week study to assess pharmacokinetics (PK), safety and pharmacodynamic effects of sotatercept as add-on therapy to standard-of-care in children from 1 year to less than 18 years of age with pulmonary arterial hypertension (PAH)

Area	Description
Extrapolation, modelling and simulation studies	**Study 3** Population pharmacokinetic/pharmacodynamic analysis to support extrapolation of efficacy of sotatercept in the treatment of pulmonary arterial hypertension in children from 1 year to less than 18 years of age **Study 4** Analysis of existing in house and literature data to support extrapolation of efficacy of sotatercept in the treatment of PAH in children from 1 year to less than 18 years of age
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 April 2029
Deferral for one or more measures contained in the paediatric investigation plan:	Yes