EMA/235349/2021

European Medicines Agency decision

P/0227/2021

of 8 June 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for Sotatercept (EMEA-002756-PIP01-19) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Acceleron Pharma on 11 July 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 April 2021, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and of its own motion in accordance with Article 12 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for sotatercept, powder for solution for injection, subcutaneous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for sotatercept, powder for solution for injection, subcutaneous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A product-specific waiver for sotatercept, powder for solution for injection, subcutaneous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Acceleron Pharma, 128 Sidney Street, 02139 - Cambridge, MA, United States.

EMA/PDCO/72399/2021
Amsterdam, 23 April 2021

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMEA-002756-PIP01-19

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:

Acceleron Pharma

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Acceleron Pharma submitted for agreement to the European Medicines Agency on 11 July 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 18 August 2020.

Supplementary information was provided by the applicant on 15 January 2021. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population of its own motion in accordance with Article 12 of said Regulation and concluded in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

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

2. The measures and timelines of the agreed 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	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age-appropriate dosage form.
Non-clinical studies	0	Not applicable.
Clinical studies	1	**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	Number of measures	Description
Extrapolation, modelling and simulation studies	2	**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	0	Not applicable.
Other measures	0	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 August 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes