EMA/628038/2021

European Medicines Agency decision

P/0509/2021

of 3 December 2021

on the acceptance of a modification of an agreed paediatric investigation plan for molgramostim, (EMEA-002282-PIP01-17-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 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 European Medicines Agency's decision P/0094/2019 issued on 22 March 2019,

Having regard to the application submitted by Savara Aps on 9 July 2021 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 15 October 2021, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for molgramostim, nebuliser solution, inhalation 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 Savara Aps, Slotsmarken 17, 2970 – Hoersholm, Denmark.

EMA/PDCO/406627/2021
Amsterdam, 15 October 2021

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

EMEA-002282-PIP01-17-M01

Scope of the application

Active substance(s):

Molgramostim

Condition(s):

Treatment of pulmonary alveolar proteinosis

Pharmaceutical form(s):

Nebuliser solution

Route(s) of administration:

Inhalation use

Name/corporate name of the PIP applicant:

Savara ApS

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Savara ApS submitted to the European Medicines Agency on 9 July 2021 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/0094/2019 issued on 22 March 2019.

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

The procedure started on 17 August 2021.

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 Norwegian Paediatric Committee member 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 alveolar proteinosis

The waiver applies to:

- the paediatric population from birth to less than 6 years;
- nebuliser solution, inhalation use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of pulmonary alveolar proteinosis

2.1.1. Indication(s) targeted by the PIP

Treatment of autoimmune pulmonary alveolar proteinosis

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

From 6 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Nebuliser solution

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	2	**Study 1** Development of lower strength formulation appropriate to the paediatric population. **Study 2** Evaluation of formulations and reduction of fill volume of delivered dose using simulated paediatric breathing pattern.
Non-clinical studies	0	Not applicable.
Clinical studies	1	**Study 3** (SAV006-04) Open-label, un-controlled multicentre study to investigate the activity of inhaled molgramostim in paediatric patients with autoimmune pulmonary alveolar proteinosis.

Extrapolation, modelling and simulation studies	1	**Study 4** Analysis of adult data (study MOL-PAP-002) and paediatric data (study SAV006-04) to extrapolate efficacy of molgramostim from adult to paediatric patients from 6 to less than 18 years of age with autoimmune pulmonary alveolar proteinosis.
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:	No
Date of completion of the paediatric investigation plan:	By June 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes