

EMA/87258/2024

European Medicines Agency decision

P/0063/2024

of 8 March 2024

on the acceptance of a modification of an agreed paediatric investigation plan for molgramostim, (EMA-002282-PIP01-17-M02) 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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0094/2019 issued on 22 March 2019 and the decision P/0509/2021 issued on 3 December 2021,

Having regard to the application submitted by Savara ApS on 28 September 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 19 January 2024, 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 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, Tuborg Boulevard 12, 4., c/o Lundgrens Advokatpartnerselskab (LP), 2900 - Hellerup Denmark.

EMA/PDCO/478614/2023
Amsterdam, 19 January 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002282-PIP01-17-M02

Scope of the application

Active substance(s):

Molgramostim

Invented name and authorisation status:

See Annex II

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 28 September 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/0094/2019 issued on 22 March 2019 and the decision P/0509/2021 issued on 3 December 2021.

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

The procedure started on 20 November 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 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 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Nebuliser solution

2.1.4. Measures

Area	Description
Quality-related studies	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	Not applicable.
Clinical studies	Study 3 (SAV006-04) Open-label, uncontrolled multicentre study to investigate the activity of inhaled molgramostim in paediatric patients from 6 years to less than 18 years of age with autoimmune pulmonary alveolar proteinosis.

Extrapolation, modelling and simulation studies	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 years to less than 18 years of age with autoimmune pulmonary alveolar proteinosis.
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 March 2028
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:

The product is not authorised anywhere in the European Community.