

EMA/130699/2019

European Medicines Agency decision P/0094/2019

of 22 March 2019

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for molgramostim (EMEA-002282-PIP01-17) 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.

European Medicines Agency decision

P/0094/2019

of 22 March 2019

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for molgramostim (EMA-002282-PIP01-17) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Savara ApS on 18 December 2017 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 1 February 2019, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 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 molgramostim, nebuliser solution, inhalation 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 molgramostim, nebuliser solution, inhalation 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 waiver for molgramostim, nebuliser solution, inhalation 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 Savara ApS, Slotsmarken 17, 2. tv, 2970 - Hørsholm, Denmark.

EMA/PDCO/780467/2018
London, 1 February 2019

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

EMA-002282-PIP01-17

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 16(1) of Regulation (EC) No 1901/2006 as amended, Savara ApS submitted for agreement to the European Medicines Agency on 18 December 2017 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 23 January 2018.

Supplementary information was provided by the applicant on 29 October 2018. The applicant proposed modifications to the paediatric investigation plan.

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 in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) 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 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 March 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes