EMA/187096/2019

European Medicines Agency decision

P/0113/2019

of 29 March 2019

on the agreement of a paediatric investigation plan and on the granting of a deferral for chemically modified recombinant human sulfamidase (EMEA-002380-PIP01-18) 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 Swedish Orphan Biovitrum AB (publ) on 20 April 2018 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 March 2019, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) of Regulation (EC) No 1901/2006, has given an opinion on the agreement of a paediatric investigation plan and on the granting of a deferral.

(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.

[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 chemically modified recombinant human sulfamidase, solution for injection/infusion, intravenous 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 chemically modified recombinant human sulfamidase, solution for injection/infusion, intravenous 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

This decision is addressed to Swedish Orphan Biovitrum AB (publ), Tomtebodavägen 23A, SE-11276 - Stockholm, Sweden.

EMA/PDCO/157598/2019
Amsterdam, 20 March 2019

Final opinion of the Paediatric Committee on the agreement of a Paediatric Investigation plan and a deferral

EMEA-002380-PIP01-18

Scope of the application

Active substance(s):

Chemically modified recombinant human sulfamidase

Condition(s):

Treatment of mucopolysaccharidosis type IIIA

Pharmaceutical form(s):

Solution for injection/infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Swedish Orphan Biovitrum AB (publ)

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Swedish Orphan Biovitrum AB (publ) submitted for agreement to the European Medicines Agency on 20 April 2018 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

An Opinion was adopted by the Paediatric Committee on 1 February 2019. Swedish Orphan Biovitrum AB (publ) received the Paediatric Committee Opinion on 11 February 2019.

On 19 February 2019 Swedish Orphan Biovitrum AB (publ) submitted to the European Medicines Agency a written request, including detailed grounds for re-examination of the Opinion.

The re-examination procedure started on 20 February 2019.

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

    1.1. to revise its opinion and

    - 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.

    1.2. following re-examination, to amend the measures of the paediatric investigation plan.

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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of mucopolysaccharidosis type IIIA (MPS IIIA)

2.1.1. Indication(s) targeted by the PIP

Treatment of mucopolysaccharidosis type IIIA (MPS IIIA)

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection/infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	1	**Study 1** Chronic toxicity study in juvenile rats
Clinical studies	5	**Study 2 (SOBI003-001)** Open-label, non-controlled, parallel, sequential ascending multiple-dose, first-in-human study to assess the dose related safety, tolerability, pharmacokinetics (PK) and pharmacodynamics (PD) of Chemically modified recombinant human sulfamidase (SOBI003) in paediatric patients with mucopolysaccharidosis type IIIA (MPS IIIA) from 1 to 6 years of age **Study 3 (SOBI003-002)** Open-label, single-arm, extension study of first-in-human study (PIP Study 2) to assess the long-term safety, tolerability and efficacy of SOBI003

		Study 4 (SOBI003-003) Open-label, single-arm, pivotal study to evaluate the safety, efficacy and PK of SOBI003 in MPS IIIA patients from birth to no greater than 30 months **Study 5 (SOBI003-004)** Open-label, single-arm study to assess the safety of SOBI003 maintenance treatment in paediatric patients who have completed PIP Study 3 (SOBI003-002) or PIP Study 4 (SOBI003-003) **Study 6 (SOBI003-005)** Open-label, single-arm study to evaluate the efficacy, safety, PK and PD of SOBI003 in patients with MPS IIIA from 6 to less than 18 years of age
Extrapolation, modelling and simulation studies	0	Not applicable
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 December 2031
Deferral for one or more measures contained in the paediatric investigation plan:	Yes