EMA/493358/2020

European Medicines Agency decision

P/0407/2020

of 23 October 2020

on the acceptance of a modification of an agreed paediatric investigation plan for velmanase alfa (Lamzede), (EMEA-001056-PIP02-12-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/0122/2014 issued on 7 May 2014,

Having regard to the application submitted by Chiesi Farmaceutici S.p.A. on 27 May 2020 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 4 September 2020, 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 velmanase alfa (Lamzede), powder for solution for infusion, intravenous 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 Chiesi Farmaceutici S.p.A., Via Palermo 26/A, 43122 – Parma, Italy.

EMA/PDCO/321756/2020
Amsterdam, 4 September 2020

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

EMEA-001056-PIP02-12-M01

Scope of the application

Active substance(s):

Velmanase alfa

Invented name:

Lamzede

Condition(s):

Treatment of alpha-mannosidosis

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Chiesi Farmaceutici S.p.A.

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Chiesi Farmaceutici S.p.A. submitted to the European Medicines Agency on 27 May 2020 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0122/2014 issued on 7 May 2014.

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

The procedure started on 6 July 2020.

Scope of the modification

Some 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 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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition: treatment of alpha-mannosidosis

2.1.1. Indication(s) targeted by the PIP

Treatment of alpha-mannosidosis.

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)

Powder for solution for infusion.

2.1.4. Measures

Area	Number of studies	Description
Quality - related studies	0	Not applicable.
Non-clinical studies	2	**Study 1: CRL 495110** Preliminary juvenile toxicity study in rats, lasting up to 11 weeks. **Study 2: CRL 495126** Main juvenile toxicity study in Sprague-Dawley rats, lasting 10 weeks.
Clinical studies	6	**Study 3: rhLAMAN-01** Twenty four-month multicentre, prospective, observational study to evaluate parameters of relevance in untreated children (and adults) with alpha-mannosidosis. **Study 4: rhLAMAN-02** Open-label, dose escalation study to evaluate pharmacokinetics and safety of intravenous recombinant human alpha-mannosidase in patients from 5 to years of age with alpha-mannosidosis. **Study 5: rhLAMAN-03** Six-month open-label, multiple dose study to evaluate efficacy and safety of intravenous recombinant human alpha-mannosidase in patients from 5 to 21 years of age with alpha-mannosidosis.

Area	Number of studies	Description
		Study 6: rhLAMAN-04 Six-month open-label, multiple dose study to evaluate efficacy and safety of intravenous recombinant human alpha-mannosidase in patients from 5 to 21 years of age with alpha-mannosidosis. **Study 7: rhLAMAN-05** Twelve-month multi-centre, double blind, randomized, active/placebo study to evaluate the efficacy and safety of intravenous recombinant human alpha-mannosidase in patients from 5 to less than 36 years of age with alpha-mannosidosis. **Study 8: rhLAMAN-08** Twenty four-month multi-centre, open label study to evaluate pharmacokinetics, safety and efficacy of intravenous recombinant human alpha-mannosidase in patients from birth to less than 6 years of age with alpha-mannosidosis.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By August 2020
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of alpha-mannosidosis

Authorised indication(s):

- Enzyme replacement therapy for the treatment of non-neurological manifestations in patients with mild to moderate alpha mannosidosis.

Authorised pharmaceutical form(s):

Powder for solution for infusion

Authorised route(s) of administration:

Intravenous use