European Medicines Agency decision

P/0459/2020

of 1 December 2020


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/0459/2020

of 1 December 2020


The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,


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 European Medicines Agency’s decision P/0053/2015 issued on 6 March 2015 and the decision P/0258/2018 issued on 15 August 2018,

Having regard to the application submitted by Genzyme Europe B.V. on 2 July 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 16 October 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.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

Has adopted this decision:

**Article 1**

Changes to the agreed paediatric investigation plan for olipudase alfa, powder for concentrate for solution for infusion, intravenous use, 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 Genzyme Europe B.V., Paasheuvelweg 25, 1105 BP - Amsterdam, The Netherlands.
Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMEA-001600-PIP01-13-M02

Scope of the application

Active substance(s):
Olipudase alfa

Condition(s):
Treatment of Niemann-Pick disease

Pharmaceutical form(s):
Powder for concentrate for solution for infusion

Route(s) of administration:
Intravenous use

Name/corporate name of the PIP applicant:
Genzyme Europe B.V.

Basis for opinion


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

The procedure started on 18 August 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 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 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 Niemann-Pick disease

2.1.1. Indication(s) targeted by the PIP

Treatment of the non-neurological manifestations of acid sphingomyelinase deficiency

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 concentrate for solution for infusion

2.1.4. Measures

<table>
<thead>
<tr>
<th>Area</th>
<th>Number of measures</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Quality-related studies</td>
<td>0</td>
<td>Not applicable</td>
</tr>
<tr>
<td>Non-clinical studies</td>
<td>0</td>
<td>Not applicable</td>
</tr>
</tbody>
</table>
| Clinical studies                       | 2                  | **Study 1: DFI13803**  
Multi-centre, open-label, ascending dose study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics and exploratory efficacy of olipudase alfa in paediatric patients below 18 years of age with acid sphingomyelinase deficiency  
**Study 2: LTS13632**  
Long-term study to assess safety, pharmacokinetics and efficacy of olipudase alfa in patients with acid sphingomyelinase deficiency (ASMD) |
| Extrapolation, modelling and simulation studies | 1                  | **Study 3:**  
Extrapolation measure to confirm extrapolability of adult results to paediatrics                                                               |
| Other studies                          | 0                  | Not applicable                                                                                                                              |
| Other measures                         | 0                  | Not applicable                                                                                                                              |
3. Follow-up, completion and deferral of PIP

| Concerns on potential long term efficacy issues in relation to paediatric use: | Yes |
| Date of completion of the paediatric investigation plan: | By January 2023 |
| Deferral for one or more measures contained in the paediatric investigation plan: | Yes |