EMA/538557/2018

European Medicines Agency decision

P/0298/2018

of 12 September 2018

on the acceptance of a modification of an agreed paediatric investigation plan for eliglustat (tartrate) (Cerdelga), (EMEA-000461-PIP02-11-M03) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Contact EMA www.ema.europa.eu/contact

European Medicines Agency decision

P/0298/2018

of 12 September 2018

on the acceptance of a modification of an agreed paediatric investigation plan for eliglustat (tartrate) (Cerdelga), (EMEA-000461-PIP02-11-M03) 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 726/2004[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/0041/2012 issued on 28 February 2012 and the decision P/0191/2015 issued on 4 September 2015,

Having regard to the application submitted by Genzyme Europe B.V. on 2 May 2018 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 27 July 2018, 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 eliglustat (tartrate) (Cerdelga), capsule, hard, oral 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 Genzyme Europe B.V., Gooimeer 10, 1411DD - Naarden, The Netherlands.

EMA/PDCO/299577/2018 Corr
London, 27 July 2018

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

EMEA-000461-PIP02-11-M03

Scope of the application

Active substance(s):

Eliglustat

Invented name:

Cerdelga

Condition(s):

Treatment of Gaucher disease Type 1 and Type 3

Treatment of Gaucher disease Type 2

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Genzyme Europe B.V.

Information about the authorised medicinal product:

See Annex II

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5510
Send a question via our website www.ema.europa.eu/contact

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Genzyme Europe B.V. submitted to the European Medicines Agency on 2 May 2018 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/0041/2012 issued on 28 February 2012 and the decision P/0191/2015 issued on 4 September 2015.

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

The procedure started on 29 May 2018.

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 Norwegian Paediatric Committee member 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 Gaucher disease Type 2

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- capsule, hard, oral use;
- on the grounds that the specific medicinal product is likely to be ineffective.

1.2. Condition: treatment of Gaucher disease Type 1 and Type 3

The waiver applies to:

- all subsets of the paediatric population from birth to less than 24 months of age;
- capsule, hard, oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan

2.1. Condition: treatment of Gaucher disease Type 1 and Type 3

2.1.1. Indication(s) targeted by the PIP

Treatment of Gaucher disease Type 1 and Type 3

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

From 24 months to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Capsule, hard

2.1.4. Studies

Area	Number of studies	Description
Quality	2	**Study 1** Development of capsule, hard, for oral use in an age-appropriate dose of 10mg or less subject to PK data. **Study 2** Data to support validated compounding of the product for children not able to swallow the capsules: including stability, reproducibility, dose uniformity and acceptability.

Area	Number of studies	Description
Non-clinical	1	**Study 3** Juvenile Rat Toxicology Study.
Clinical	1	**Study 4** Open label, two cohort (with and without imiglucerase), multi-centre, study to evaluate pharmacokinetics (PK), safety, and efficacy of eliglustat in paediatric patients with Gaucher disease type 1 (GD1) and type 3 (GD3).

3. Follow-up, completion and deferral of PIP

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

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of Gaucher disease type 1

Authorised indication(s):

- Cerdelga is indicated for the long-term treatment of adult patients with Gaucher disease type 1 (GD1), who are CYP2D6 poor metabolisers (PMs), intermediate metabolisers (IMs) or extensive metabolisers (EMs).

Authorised pharmaceutical form(s):

Capsule, hard

Authorised route(s) of administration:

Oral use