EMADOC-1700519818-1799764

European Medicines Agency decision

EMA/PE/0000223351

of 3 January 2025

on the acceptance of a modification of an agreed paediatric investigation plan for cipaglucosidase alfa (Pombiliti) 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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on the acceptance of a modification of an agreed paediatric investigation plan for cipaglucosidase alfa (Pombiliti) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0474/2020 issued on 1 December 2020, and the decision P/0204/2021 issued on 10 May 2021,

Having regard to the application submitted by Amicus Therapeutics Europe Limited on 12 August 2024 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 15 November 2024, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for cipaglucosidase alfa (Pombiliti), 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 Amicus Therapeutics Europe Limited, Block 1 Blanchardstown Corporate Park, Ballycoolen Road, Blanchardstown, Dublin 15 - D15 AKK1, Ireland.

EMADOC-1700519818-1679420
Amsterdam, 15 November 2024

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

EMA/PE/0000223351

Scope of the application

Active substance(s):

Cipaglucosidase alfa

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of glycogen storage disease Type II (Pompe's disease)

Pharmaceutical form(s):

Powder for concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Amicus Therapeutics Europe Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Amicus Therapeutics Europe Limited submitted to the European Medicines Agency on 12 August 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0474/2020 issued on 1 December 2020, and the decision P/0204/2021 issued on 10 May 2021.

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

The procedure started on 16 September 2024.

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 Paediatric Committee member of Norway 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 glycogen storage disease Type II (Pompe's disease)

2.1.1. Indication(s) targeted by the PIP

Cipaglucosidase alfa used in conjunction with miglustat is indicated for the long-term treatment of adolescent and paediatric patients with Pompe disease

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

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	**Study 1** *(Charles River Study 20201984)* Definitive juvenile toxicity study in rats to evaluate the reproductive and developmental toxicity of cipaglucosidase alfa / miglustat
Clinical studies	**Study 2** *(ATB200-04)* Open-label, uncontrolled trial to evaluate safety, pharmacokinetics (PK), efficacy, pharmacodynamics (PD) and immunogenicity of cipaglucosidase alfa/miglustat in children from birth to less than 18 years with late-onset Pompe disease (LOPD) and less severe variants of infantile-onset Pompe disease (IOPD) **Study 3** *(ATB200-16)* *Deleted during procedure EMEA-002447-PIP01-18-M01* **Study 4** *(ATB200-08)* Open-label, uncontrolled trial to evaluate pharmacokinetics (PK), safety, activity and pharmacodynamics (PD) of cipaglucosidase alfa/ miglustat in children from birth to less than 18 years with classic infantile-onset Pompe disease (IOPD)

Extrapolation, modelling and simulation studies	**Study 5** Modelling and simulation study to evaluate the use of cipaglucosidase alfa/miglustat in children from birth to less than 18 years of age with LOPD and less severe variants of IOPD **Study 6** Modelling and simulation study to evaluate the use of cipaglucosidase alfa/miglustat in children from birth to less than 18 years of age with classic IOPD **Study 7** *(Extrapolation study ATB200-04)* Analysis of existing data on cipaglucosidase alfa /miglustat in children from birth to less than 18 years of age with LOPD and less severe variants of IOPD
Other studies	Not applicable
Other measures	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 July 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of glycogen storage disease Type II (Pompe's disease)

Authorised indication(s):

- Pombiliti (cipaglucosidase alfa) is a long-term enzyme replacement therapy used in combination with the enzyme stabiliser miglustat for the treatment of adults with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency).

Invented name(s): Pombiliti

- Authorised pharmaceutical form(s): Powder for concentrate for solution for infusion (powder for concentrate)
- Authorised route(s) of administration: Intravenous infusion
- Authorised via centralised procedure