EMA/627313/2020

European Medicines Agency decision

P/0474/2020

of 1 December 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for recombinant human acid alpha-glucosidase (ATB200) (EMEA-002447-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 Amicus Therapeutics Europe Limited on 26 November 2019 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 16 October 2020, 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 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 recombinant human acid alpha-glucosidase (ATB200), powder for concentrate for solution for 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 recombinant human acid alpha-glucosidase (ATB200), powder for concentrate for solution for 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 Amicus Therapeutics Europe Limited, Block 1, Blanchardstown Corporate Park, Ballycoolen Road, Blanchardstown, D15 AKK1 – Dublin, Ireland.

EMA/PDCO/410451/2020
Amsterdam, 16 October 2020

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral

EMEA-002447-PIP01-18

Scope of the application

Active substance(s):

Recombinant human acid alpha-glucosidase (ATB200)

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 16(1) of Regulation (EC) No 1901/2006 as amended, Amicus Therapeutics Europe Limited submitted for agreement to the European Medicines Agency on 26 November 2019 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 6 January 2020.

Supplementary information was provided by the applicant on 10 July 2020. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - 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.

   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 glycogen storage disease Type II (Pompe's disease)

2.1.1. Indication(s) targeted by the PIP

Recombinant human acid alpha-glucosidase (ATB200) co-administered with N butyldeoxynojirimycin (AT2221) 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 0 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	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	1	**Study 1** *(Charles River Study 20201984)* Definitive juvenile toxicity study in rats to evaluate the reproductive and developmental toxicity of ATB200 in combination with AT2221
Clinical studies	3	**Study 2** *(ATB200-04)* Open-label, uncontrolled trial to evaluate pharmacokinetics (PK), safety and pharmacodynamics (PD) of ATB200/AT2221 in children from 12 to less than 18 years with late-onset Pompe disease (LOPD) **Study 3** *(ATB200-16)* Open-label, historical-controlled trial to evaluate pharmacokinetics (PK), safety, activity and pharmacodynamics (PD) of ATB200/AT2221 in children from 1 to less than 12 years with late-onset Pompe disease (LOPD)

		Study 4 *(ATB200-08)* Open-label, historical-controlled trial to evaluate pharmacokinetics (PK), safety, activity and pharmacodynamics (PD) of ATB200/AT2221 in children from birth to less than 18 years with infantile-onset Pompe disease (IOPD)
Extrapolation, modelling and simulation studies	3	**Study 5** *(ATB200 / AT2221 Population PK Modelling and Simulation for LOPD)* Modelling and simulation study to evaluate the use of ATB200 / AT2221 in children from 1 to less than 18 years of age with LOPD **Study 6** *(ATB200 / AT2221 Population PK Modelling and Simulation for IOPD)* Modelling and simulation study to evaluate the use of ATB200 / AT2221 in children from birth to less than 18 years of age with IOPD **Study 7** *(Extrapolation study ATB200-04 / ATB200-16)* Analysis of existing data on ATB200 / AT2221 in children from 1 to less than 18 years of age with LOPD
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 March 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes