MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-100129-PIP01-21-M01

Scope of the Application

Active Substance(s)

Cipaglucosidase alfa

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 UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Amicus Therapeutics UK Limited submitted to the licensing authority on 04/06/2021 14:03 BST an application for a Modification

The procedure started on 22/04/2022 07:49 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100129-PIP01-21-M01

Of 19/05/2022 16:56 BST

On the adopted decision for Cipaglucosidase alfa (MHRA-100129-PIP01-21-M01) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for Cipaglucosidase alfa, Powder for concentrate for solution for infusion , Intravenous use .

This decision is addressed to Amicus Therapeutics UK Limited, One Globeside Business Park, Fieldhouse Lane, Marlow, United Kingdom, SL7 1HZ

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

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

2.2 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.

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Powder for concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	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 cipaglucosidase alfa / miglustat.
Clinical Studies	2	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 MHRA-100129-PIP01-21-M01 Study 4 (ATB200-08) Open-label, uncontrolled trial to evaluate pharmacokinetics (PK), safety and pharmacodynamics (PD) of cipaglucosidase alfa /miglustat in children from birth to less than 18 years with classic infantile-onset Pompe disease (IOPD).
Extrapolation, Modeling & Simulation Studies	3	Study 5 (Cipaglucosidase alfa / miglustat Population PK Modelling and Simulation for LOPD / less severe variants of IOPD) Modelling and simulation study to evaluate the use of cipaglucosidase alfa/ miglustat in children from 1 to less than 18 years of age with LOPD and less severe variants of IOPD. Study 6 (Cipaglucosidase alfa / miglustat Population PK Modelling

		and Simulation for classic IOPD) 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	0	Not applicable
Other Measures	0	Not applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	31/03/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes