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 (MHRA-100369-PIP01-21-M01)

MHRA-100369-PIP01-21-M02

Scope of the Application

Active Substance(s)

MIGALASTAT HYDROCHLORIDE

Condition(s)

Treatment of Fabry disease

Pharmaceutical Form(s)

Capsule, hard; Dispersible tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Amicus Therapeutics UK Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Amicus Therapeutics UK Ltd submitted to the licensing authority on 03/10/2023 21:12 BST an application for a Modification

The procedure started on 13/11/2023 12:21 GMT

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-100369-PIP01-21-M02

Of 22/11/2023 10:06 GMT

On the adopted decision for MIGALASTAT HYDROCHLORIDE (MHRA-100369-PIP01-21-M02) 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.

This decision applies to a Modification for MIGALASTAT HYDROCHLORIDE, Capsule, hard; Dispersible tablet , ORAL USE .

This decision is addressed to Amicus Therapeutics UK Ltd, One Globeside, Fieldhouse Lane, Marlow, UNITED KINGDOM, SL7 1HZ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Fabry disease. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age. Pharmaceutical form(s): Capsule, hard Dispersible tablet Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Fabry disease.

2.2 Indication(s) targeted by the PIP:

Treatment of Fabry disease.

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

The paediatric population from 2 years to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Capsule, hard Dispersible tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	3	Study 1 Development of a dispersible tablet. Study 2 Development of marked packaging to show the days of week/month clearly indicating which days to dose on in order to aid compliance with the alternate day regimen (capsule). Study 5 Development of instructions clearly indicating on which days to dose in order to aid compliance with the alternate day regimen (dispersible tablet).
Non-Clinical Studies	0	Not Applicable.
Clinical Studies	2	Study 3 (AT1001-20) 2-stage, open-label, non-comparative, multicentre trial to evaluate pharmacokinetics, pharmacodynamics, safety and activity of migalastat hydrochloride in children from 12 years to less than 18 years of age with Fabry Disease and amenable GLA mutations. Study 4 (AT1001-033) 2-stage, open-label, non-comparative, multicentre trial to evaluate pharmacokinetics, pharmacodynamics, safety, and activity of migalastat hydrochloride in children from 2 years to less than 12 years of age with Fabry disease and amenable GLA mutations.
Extrapolation, Modeling & Simulation Studies	0	Not Applicable.
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:	Yes
Date of completion of the paediatric investigation plan:	31/12/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes