EMA/190110/2019

European Medicines Agency decision

P/0137/2019

of 17 April 2019

on the acceptance of a modification of an agreed paediatric investigation plan for migalastat (hydrochloride) (Galafold), (EMEA-001194-PIP01-11-M04) 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 European Medicines Agency's decision P/0174/2012 issued on 27 July 2012, the decision P/0256/2014 issued on 1 October 2014 and the decision P/0328/2016 issued on 2 December 2016,

Having regard to the application submitted by Amicus Therapeutics UK Ltd on 23 November 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 1 March 2019, 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 migalastat (hydrochloride) (Galafold), capsule, hard, dispersible tablet, 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 Amicus Therapeutics UK Limited, Phoenix House, Oxford Road, Tatling End, Gerrards Cross, SL9 7AP – Buckinghamshire, United Kingdom.

EMA/PDCO/836806/2018
London, 1 March 2019

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

EMEA-001194-PIP01-11-M04

Scope of the application

Active substance(s):

Migalastat (hydrochloride)

Invented name:

Galafold

Condition(s):

Treatment of Fabry disease

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Capsule, hard

Dispersible tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Amicus Therapeutics UK Ltd

Information about the authorised medicinal product:

See Annex II

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Amicus Therapeutics UK Ltd submitted to the European Medicines Agency on 23 November 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/0174/2012 issued on 27 July 2012, the decision P/0256/2014 issued on 1 October 2014 and the decision P/0328/2016 issued on 2 December 2016.

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

The procedure started on 3 January 2019.

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 Fabry disease

The waiver applies to:

- all subsets of the paediatric population from birth to less than 2 years of age;
- for capsule, hard, dispersible tablet, oral use;
- on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the paediatric population.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of Fabry disease

2.1.1. Indication(s) targeted by the PIP

Treatment of Fabry disease

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

From 2 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Capsule, hard, oral use

Dispersible tablet

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	2	**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 and dispersible tablet).
Non-clinical studies	0	Not applicable.

Clinical studies	2	**Study 3:** 2-stage, open-label, non-comparative, multicentre trial to evaluate pharmacokinetics, pharmacodynamics, safety and activity of migalastat hydrochloride in children from 12 to less than 18 years of age with Fabry Disease and amenable GLA mutations. (AT1001-020) **Study 4:** (added during procedure EMEA-001194-PIP01-11-M04) Open-label, non-comparative, multicentre trial to evaluate pharmacokinetics, pharmacodynamics, safety and activity of migalastat hydrochloride in children from 2 to less than 12 years of age, and from 12 to less than 16 years of age and less than 45 kg, with Fabry Disease and amenable GLA mutations. (AT1001-033)
Extrapolation, modelling and simulation studies	0	Not applicable
Other studies	0	Not applicable
Other measures	0	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2022
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 Fabry disease

Authorised indication(s):

- Long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation.

Authorised pharmaceutical form(s):

Hard capsule

Authorised route(s) of administration:

Oral use