EMA/418723/2021

European Medicines Agency decision

P/0389/2021

of 8 September 2021

on the acceptance of a modification of an agreed paediatric investigation plan for metreleptin (Myalepta), (EMEA-001701-PIP01-14-M02) 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/0194/2016 issued on 15 July 2016 and the decision P/0314/2016 issued on 25 November 2016,

Having regard to the application submitted by Amryt Pharmaceuticals DAC on 25 March 2021 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 23 July 2021, 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 metreleptin (Myalepta), powder for solution for injection, subcutaneous 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 Amryt Pharmaceuticals DAC, 45 Mespil Road, D04 W2F1 Dublin 4, Ireland.

EMA/PDCO/253041/2021
Amsterdam, 23 July 2021

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

EMEA-001701-PIP01-14-M02

Scope of the application

Active substance(s):

Metreleptin

Invented name:

Myalepta

Condition(s):

Treatment of lipodystrophy

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Amryt Pharmaceuticals DAC

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Amryt Pharmaceuticals DAC submitted to the European Medicines Agency on 25 March 2021 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0194/2016 issued on 15 July 2016 and the decision P/0314/2016 issued on 25 November 2016.

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

The procedure started on 25 May 2021.

Scope of the modification

Some 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 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 lipodystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of lipodystrophy

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 solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	2	**Study 1** Development of a 2.5 mg and of a 5 mg presentation of metreleptin to facilitate dosing of paediatric patients **Study 2** Study to select an appropriate small capacity syringe appropriate to provide accurate dosing of metreleptin to paediatric patients to be available with the appropriate presentations
Non-clinical studies	0	Not applicable
Clinical studies	3	**Study 3** (991265/20010769) Open-label uncontrolled trial to evaluate activity and safety of metreleptin in children from 1 year to less than 18 years of age (and adults) with lipodystrophy **Study 4** (FHA101) Open-label, uncontrolled trial to evaluate activity and safety of metreleptin in children from 9 years to less than 18 years of age (and adults) with lipodystrophy

		and associated diabetes mellitus and/or hypertriglyceridaemia **Study 5** Open-label, uncontrolled trial to evaluate PK, activity and safety of metreleptin in patients less than 6 years of age with generalised lipodystrophy and associated diabetes mellitus and/or hypertriglyceridaemia
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 safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of lipodystrophy

Authorised indication(s):

Myalepta is indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients:

- with confirmed congenital generalised LD (Berardinelli-Seip syndrome) or acquired generalised LD (Lawrence syndrome) in adults and children 2 years of age and above
- with confirmed familial partial LD or acquired partial LD (Barraquer-Simons syndrome), in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control.

Authorised pharmaceutical form(s):

Powder for solution for injection

Authorised route(s) of administration:

Subcutaneous use