EMA/84341/2023

European Medicines Agency decision

P/0073/2023

of 10 March 2023

on the acceptance of a modification of an agreed paediatric investigation plan for ibutamoren mesylate (EMEA-003032-PIP01-21-M01) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0145/2022 issued on 13 May 2022,

Having regard to the application submitted by Lumos Pharma, Inc. on 5 October 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 January 2023, 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.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for ibutamoren mesylate, tablet, oral use, 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 Lumos Pharma, Inc,.4200 Marathon Blvd. Suite 200,TX 78756 - Austin, Texas, United States.

EMA/PDCO/889020/2022
Amsterdam, 20 January 2023

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

EMEA-003032-PIP01-21-M01

Scope of the application

Active substance(s):

Ibutamoren mesylate

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of growth hormone deficiency

Pharmaceutical form(s):

Tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Lumos Pharma, Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Lumos Pharma, Inc. submitted to the European Medicines Agency on 5 October 2022 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0145/2022 issued on 13 May 2022.

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

The procedure started on 21 November 2022.

Scope of the modification

Some measures 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 in the scope set out in the Annex I of this opinion

   The Paediatric Committee member of Norway 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

Condition:

Treatment of growth hormone deficiency

The waiver applies to:

- the paediatric population from birth to less than 3 years of age;
- tablet, oral use;
- on the grounds that the specific medicinal product is likely to be ineffective.

2. Paediatric investigation plan

2.1. Condition:

Treatment of growth hormone deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of growth hormone deficiency

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

From 3 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	Study 1 (LUM-201-01) Randomised, open-label, active control, parallel arm study to evaluate activity pharmacokinetics (PK) and safety of ibutamoren in children from 3 years up to and including 11 years of age for girls and up to and including 12 years of age for boys with idiopathic paediatric growth hormone deficiency Study 2 (LUM-201-03) Randomised open-label parallel arm study to assess effects on endogenous growth hormone (GH) pulsatility and the pharmacokinetics (PK) of ibutamoren in naive to treatment, pre-

	pubertal children with idiopathic growth hormone deficiency from 4 years of age and up to achievement of adult height Study 3 (LUM-201-02) Open-label, multicentre safety study to assess the long-term safety of ibutamoren administration in children from PIP studies 1, 2 and 4 with idiopathic growth hormone deficiency Study 4 (LUM-201-0X) Open label randomised study of efficacy and safety of ibutamoren as compared to an appropriate approved rhGH injectable treatment in male and female children and adolescents from 3 years to up to and including 12 years of age with treatment-naive idiopathic paediatric growth hormone deficiency who test predictive enrichment marker (PEM) positive Study 5 (LUM-201-04) *New study included as part of procedure EMEA-003032-PIP01-21-M01* Open-label, multicentre study to evaluate growth of patients and safety of ibutamoren following 12 months of daily rhGH treatment in children with idiopathic growth hormone deficiency who have previously completed the LUM-201-01 trial
Extrapolation, modelling and simulation studies	Not applicable
Other studies	Not applicable
Other measures	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 December 2028
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.