EMA/206176/2022 Corr

European Medicines Agency decision

P/0151/2022

of 13 May 2022

on the agreement of a paediatric investigation plan and on the granting of a waiver for ibutamoren mesylate (EMEA-003032-PIP01-21) 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 application submitted by Lumos Pharma, Inc. on 2 June 2021 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 25 March 2022, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a waiver.

[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

A paediatric investigation plan for ibutamoren mesylate, tablet, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A waiver for ibutamoren mesylate, tablet, oral use, of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Lumos Pharma, Inc., 4200 Marathon Blvd. Suite 200, TX 78756 - Austin, Texas, USA.

EMA/PDCO/20839/2022
Amsterdam, 25 March 2022

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMEA-003032-PIP01-21

Scope of the application

Active substance(s):

Ibutamoren mesylate

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 16(1) of Regulation (EC) No 1901/2006 as amended, Lumos Pharma, Inc. submitted for agreement to the European Medicines Agency on 2 June 2021 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 12 July 2021.

Supplementary information was provided by the applicant on 20 December 2021.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 annex 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 paediatric growth hormone deficiency. Study 2 (LUM-201-03) Randomised open-label parallel arm study to assess effects on endogenous growth hormone pulsatility and the pharmacokinetics (PK) of ibutamoren in naive to treatment, pre-pubertal children from 4 years up to and including 10 years of age for girls and up to and

	including 12 years of age for boys with paediatric growth hormone deficiency. Study 3 (LUM-201-02) Open-label, multicentre safety study to assess the long-term safety of ibutamoren administration in children with growth hormone deficiency. Study 4 (LUM-201-04) 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.
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