



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/122335/2019

European Medicines Agency decision P/0095/2019

of 22 March 2019

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for (6aR,10aR)-1-Hydroxy-6,6-dimethyl-3-(2-methyl-2-octanyl)-6a,7,10,10a-tetrahydro-6H-benzo[c]chromene-9-carboxylic acid (lenabasum) (EMEA-002069-PIP02-17) 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.

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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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Corbus Pharmaceuticals Holdings Inc on 11 September 2017 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 1 February 2019, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation 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 deferral 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 deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for (6aR,10aR)-1-Hydroxy-6,6-dimethyl-3-(2-methyl-2-octanyl)-6a,7,10,10a-tetrahydro-6H-benzo[c]chromene-9-carboxylic acid (lenabasum), tablet, age-appropriate oral formulation, 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 deferral for (6aR,10aR)-1-Hydroxy-6,6-dimethyl-3-(2-methyl-2-octanyl)-6a,7,10,10a-tetrahydro-6H-benzo[c]chromene-9-carboxylic acid (lenabasum), tablet, age-appropriate oral formulation, 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 granted.

Article 3

A waiver for (6aR,10aR)-1-Hydroxy-6,6-dimethyl-3-(2-methyl-2-octanyl)-6a,7,10,10a-tetrahydro-6H-benzo[c]chromene-9-carboxylic acid (lenabasum), tablet, age-appropriate oral formulation, 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 granted.

Article 4

This decision is addressed to Corbus Pharmaceuticals Holdings Inc, 100 River Ridge Drive, MA 02062 – Norwood, USA.

EMA/PDCO/800827/2018
London, 1 February 2019

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

EMA-002069-PIP02-17

Scope of the application

Active substance(s):

(6aR,10aR)-1-Hydroxy-6,6-dimethyl-3-(2-methyl-2-octanyl)-6a,7,10,10a-tetrahydro-6H-benzo[c]chromene-9-carboxylic acid (lenabasum)

Condition(s):

Treatment of systemic sclerosis

Pharmaceutical form(s):

Tablet

Age-appropriate oral formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Corbus Pharmaceuticals Holdings Inc

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Corbus Pharmaceuticals Holdings Inc submitted for agreement to the European Medicines Agency on 11 September 2017 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 17 October 2017.

Supplementary information was provided by the applicant on 29 October 2018. The applicant proposed modifications to the paediatric investigation plan.

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 deferral in accordance with Article 21 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)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Norwegian Paediatric Committee member 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

1.1. Condition:

Treatment of systemic sclerosis

The waiver applies to:

- the paediatric population from birth to less than 8 years of age;
- tablet, age-appropriate oral formulation, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of systemic sclerosis

2.1.1. Indication(s) targeted by the PIP

Treatment of Juvenile Systemic Sclerosis (jSSc)

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

From 8 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet

Age-appropriate oral formulation

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1 Development of an age-appropriate formulation for use in a paediatric population subset and for use by older patients who are unable to swallow capsules
Non-clinical studies	1	Study 2 Definitive rat juvenile toxicity study for characterisation of juvenile toxicity of lenabasum, toxicokinetics and evaluation of reversibility of any findings

Clinical studies	1	Study 3 Single-arm, open-label trial to explore safety, PK and efficacy of lenabasum in paediatric patients from 8 to less than 18 years of age with juvenile systemic sclerosis (JSSc)
Extrapolation, modelling and simulation studies	2	Study 4 Dose finding modelling and simulation study for calculation of initial paediatric dose and confirmation of final recommended posology of lenabasum Study 5 Extrapolation study based on analysis of existing in-house data on lenabasum on systemic sclerosis to support efficacy assumptions in children aged 8 to less than 18 years of age
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 July 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes