EMA/26710/2020

European Medicines Agency decision

P/0051/2020

of 29 January 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for 2-[[8-chloro-3-[(4-chlorophenyl)methyl]-4-(difluoromethoxy)-2-ethyl-5-quinolinyl]oxy]acetic acid L-lysine salt (GB001) (EMEA-002484-PIP01-18) 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 application submitted by GB001, Inc. (A wholly-owned subsidiary of Gossamer Bio, Inc.) on 18 November 2018 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 11 December 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 2-[[8-chloro-3-[(4-chlorophenyl)methyl]-4-(difluoromethoxy)-2-ethyl-5-quinolinyl]oxy]acetic acid L-lysine salt (GB001), film-coated tablet, age appropriate oral solid 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 2-[[8-chloro-3-[(4-chlorophenyl)methyl]-4-(difluoromethoxy)-2-ethyl-5-quinolinyl]oxy]acetic acid L-lysine salt (GB001), film-coated tablet, age appropriate oral solid 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 2-[[8-chloro-3-[(4-chlorophenyl)methyl]-4-(difluoromethoxy)-2-ethyl-5-quinolinyl]oxy]acetic acid L-lysine salt (GB001), film-coated tablet, age appropriate oral solid 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 GB001, Inc (A wholly-owned subsidiary of Gossamer Bio, Inc.) 3013 Science Park Road, 92121 - San Diego, United States.

EMA/PDCO/510406/2019
Amsterdam, 11 December 2019

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

EMEA-002484-PIP01-18

Scope of the application

Active substance(s):

2-[[8-chloro-3-[(4-chlorophenyl)methyl]-4-(difluoromethoxy)-2-ethyl-5-quinolinyl]oxy]acetic acid L-lysine salt (GB001)

Condition(s):

Treatment of asthma

Pharmaceutical form(s):

Film-coated tablet

Age appropriate oral solid formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

GB001, Inc. (A wholly-owned subsidiary of Gossamer Bio, Inc.)

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, GB001, Inc. (A wholly-owned subsidiary of Gossamer Bio, Inc.) submitted for agreement to the European Medicines Agency on 18 November 2018 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 3 January 2019.

Supplementary information was provided by the applicant on 9 September 2019. 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 asthma

The waiver applies to:

- the paediatric population from birth to less than 1 year;
- film-coated tablet, age appropriate oral solid formulation, oral use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of asthma

2.1.1. Indication(s) targeted by the PIP

Add-on treatment in adolescents and children 1 year of age and older with inadequately controlled asthma

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

From 1 year to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Age appropriate oral solid formulation

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	***Study 1*** Development of an age appropriate oral solid formulation
Non-clinical studies	2	***Study 2*** Dose range finding toxicity study in juvenile SD rats ***Study 3*** Definitive juvenile toxicity study in rats to investigate the effects of GB001 in juvenile animals.

Clinical studies	6	***Study 4 (GB001-3001)*** Randomized placebo-controlled 48-week study to evaluate efficacy, safety and PK of GB001 in adolescents (and adults) with moderate to severe asthma. ***Study 5 (GB001-3002)*** Confirmatory placebo-controlled 48-week study to evaluate efficacy, safety and PK of GB001 in adolescents (and adults) with moderate to severe asthma. ***Study 6 (GB001-3004)*** Two-part study in paediatric subjects aged 6 to less than 12 years of age with moderate to severe asthma to evaluate PK, PD, safety, and tolerability of two doses of GB001 (open-label Part A) and to evaluate efficacy and safety of GB001 as maintenance therapy in 36-week placebo-controlled part B. ***Study 7 (GB001-3005)*** Two-part study in paediatric subjects aged 1 to less than 6 years of age with moderate to severe asthma to evaluate PK, PD, safety, and tolerability of two doses of GB001 (open-label Part A) and to evaluate efficacy and safety of GB001 as maintenance therapy in 26-week placebo-controlled part B. ***Study 8 (GB001-3006)*** Randomized, double-blind, placebo and active-controlled 26-week study in children and adolescents aged 6 to less than 18 years of age with mild asthma to demonstrate the non-inferiority of GB001 compared with low dose inhaled corticosteroids with a placebo group to ensure the study's assay sensitivity. ***Study 9 (GB001-3007)*** Randomized, active-controlled 26-week study in children aged 1 to less than 6 years of age with mild asthma to evaluate efficacy and safety of GB001 compared to low-dose inhaled corticosteroids
Extrapolation, modelling and simulation studies	1	Study 10 Population PK and Exposure-Response Model to predict paediatric doses to be used in clinical studies.
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:	No
Date of completion of the paediatric investigation plan:	By September 2034
Deferral for one or more measures contained in the paediatric investigation plan:	Yes