EMA/81788/2024

European Medicines Agency decision

P/0060/2024

of 7 March 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for tinlarebant (EMEA-003225-PIP01-22) 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 Belite Bio, Inc on 1 April 2022 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 19 January 2024, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for tinlarebant, 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 tinlarebant, 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 tinlarebant, 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 Belite Bio, Inc, 12750 High Bluff Drive, Suite 475, 92130 - San Diego, USA.

EMA/PDCO/484089/2023
Amsterdam, 19 January 2024

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

EMEA-003225-PIP01-22

Scope of the application

Active substance(s):

Tinlarebant

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Stargardt disease

Pharmaceutical form(s):

Tablet

Age-appropriate oral formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Belite Bio, Inc

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Belite Bio, Inc submitted for agreement to the European Medicines Agency on 1 April 2022 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 23 May 2022.

Supplementary information was provided by the applicant on 13 October 2023. 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 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 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

1.1. Condition:

Treatment of Stargardt disease

The waiver applies to:

- the paediatric population from birth to less than 3 years of age;
- tablet, age-appropriate oral 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 Stargardt disease

2.1.1. Indication(s) targeted by the PIP

Treatment of autosomal recessive Stargardt Disease (STGD1)

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

Age-appropriate oral formulation

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 (LBS-008-CTDF) Development of a liquid dosage form of Tinlarebant for dosing of paediatric subjects from 3 years of age in study LBS-008-CT0X.
Non-clinical studies	Not applicable
Clinical studies	Study 2 (LBS-008-CT02) Open-label, dose-finding followed by 2-year extension study to evaluate safety and tolerability of tinlarebant in adolescent subjects from 12 years to less than 18 years of age with Stargardt Disease.

	Study 3 (LBS-008-CT03) Multicentre, randomized, double-masked, placebo-controlled study to evaluate the safety and efficacy of tinlarebant in the treatment of autosomal recessive Stargardt Disease (STGD1) in adolescent subjects from 12 years to less than 18 years of age (and adults). Study 4 (LBS-008-CT0X) Multicentre, randomized, double-masked, placebo-controlled, study to evaluate the safety, tolerability, and efficacy of tinlarebant in children aged from 3 years to less than 12 years with autosomal recessive Stargardt Disease (STGD1).
Modelling and simulation studies	Study 5 (LBS-008-PedMS) Population PK/PD study to determine optimal dosage strength of Tinlarebant for paediatric subjects in study LBS-008-CT0X (PIP study 4)
Other studies	Not applicable
Extrapolation plan	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 October 2028
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.