EMA/289384/2023

European Medicines Agency decision

P/0249/2023

of 5 July 2023

on the acceptance of a modification of an agreed paediatric investigation plan for beremagene geperpavec (EMEA-002472-PIP03-22-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/0464/2022 issued on 28 October 2022,

Having regard to the application submitted by Krystal Biotech, Inc. on 23 February 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan and proposing a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 June 2023, in accordance with Article 22 of Regulation (EC) No 1901/2006 and Article 21 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 acceptance of changes to the agreed paediatric investigation plan and on the granting of a deferral and on the refusal of a waiver.

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

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

(4) It is therefore appropriate to adopt a decision refusing 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

Changes to the agreed paediatric investigation plan for beremagene geperpavec, gel, topical 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

A deferral for beremagene geperpavec, gel, topical use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for beremagene geperpavec, gel, topical use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby refused.

Article 4

This decision is addressed to Krystal Biotech, Inc., 2100 Wharton Street, Suite 701, 15203 – Pittsburgh, USA.

EMA/PDCO/145041/2023
Amsterdam, 23 June 2023

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

EMEA-002472-PIP03-22-M01

Scope of the application

Active substance(s):

Beremagene geperpavec

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of dystrophic epidermolysis bullosa

Pharmaceutical form(s):

Gel

Route(s) of administration:

Topical use

Name/corporate name of the PIP applicant:

Krystal Biotech, Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Krystal Biotech, Inc. submitted to the European Medicines Agency on 23 February 2023 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0464/2022 issued on 28 October 2022.

The application for modification proposed changes to the agreed paediatric investigation plan and proposed a deferral and a waiver.

The procedure started on 24 April 2023.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified. A deferral has been added to the PIP.

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;
   - to grant a deferral, the details of which are set out in the Annex I of this opinion;
   - to refuse the granting of a waiver for some of the subsets of the paediatric population and the above mentioned condition(s) as it does not meet the grounds detailed in Article 11(1) of said Regulation.

   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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of dystrophic epidermolysis bullosa

2.1.1. Indication(s) targeted by the PIP

Treatment of dystrophic epidermolysis bullosa from birth to adults

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Gel

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1** Open-label, randomised, placebo-controlled trial to evaluate efficacy and safety of beremagene geperpavec in children from 2 years to less than 18 years of age with dystrophic epidermolysis bullosa (KB103-001, GEM-1) **Study 2** Double blind, randomised, placebo-controlled trial to evaluate efficacy and safety of beremagene geperpavec in children from 6 months to less than 18 years of age with dystrophic epidermolysis bullosa (B-VEC-03, GEM-3) **Study 3** Open-label, uncontrolled trial to evaluate safety of beremagene geperpavec in patients who participated in Study 2 and in newly diagnosed patients from birth to less than 18 years of age with dystrophic epidermolysis bullosa (B-VEC-EX02)
Modelling and simulation studies	Not applicable

Other studies	Not applicable
Extrapolation plan	Added during procedure EMEA-002472-PIP03-22-M01. Study 1 (KB103-001, GEM-1), Study 2 (B-VEC-03, GEM-3) and Study 3 (B-VEC-EX02) are a part of the extrapolation plan of efficacy from older children to infants from birth to less than 2 months of age with dystrophic epidermolysis bullosa.

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 2024
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.