EMA/180518/2022

European Medicines Agency decision

P/0104/2022

of 25 March 2022

on the acceptance of a modification of an agreed paediatric investigation plan for etranacogene dezaparvovec (EMEA-002722-PIP01-19-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/0517/2020 issued on 2 January 2021,

Having regard to the application submitted by CSL Behring GmbH on 23 March 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 March 2022, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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.

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

[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 etranacogene dezaparvovec, concentrate for solution for infusion, intravenous 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

This decision is addressed to CSL Behring GmbH, 76 Emil-Von-Behring-Strasse, 35041 – Marburg, Germany.

EMA/PDCO/180475/2022
Amsterdam, 23 March 2022

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

EMEA-002722-PIP01-19-M01

Scope of the application

Active substance(s):

Etranacogene dezaparvovec

Condition(s):

Treatment of haemophilia B

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

CSL Behring GmbH

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, CSL Behring GmbH submitted to the European Medicines Agency on 23 March 2022 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0517/2020 issued on 2 January 2021.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 23 March 2022.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

The pharmaceutical form was amended.

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.

   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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of haemophilia B

2.1.1. Indication(s) targeted by the PIP

Prevention of bleeding in male haemophilia patients without history of Factor IX inhibitors and currently on stable FIX prophylaxis therapy

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)

Concentrate for solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	None	Not applicable
Non-clinical studies	3	**Study 1** Juvenile animal efficacy study in non-human primates (NHP) matching 12-year old children in age. **Study 2** Juvenile animal efficacy study in non-human primates (NHP) matching 4- to 6-year old children in age. **Study 3** Exploratory efficacy and toxicity study in very young pigs.
Clinical studies	3	**Study 4** Single dose, open-label study to evaluate the efficacy and safety of etranacogene dezaparvovec in patients from 12 years to less than 18 years of age with haemophilia B.

		Study 5 Randomised study to evaluate the efficacy and safety of 2 dose levels of etranacogene dezaparvovec in children from 4 years to less than 12 years of age with haemophilia B. **Study 6** Open label study to evaluate the efficacy and safety of 2 dose levels of etranacogene dezaparvovec in children from birth to less than 4 years of age with haemophilia B.
Extrapolation, modelling and simulation studies	None	Not applicable
Other studies	None	Not applicable
Other measures	None	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 March 2042
Deferral for one or more measures contained in the paediatric investigation plan:	Yes