MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral.

MHRA-100695-PIP01-22-M02

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

Name / Corporate name of the PIP applicant

CSL Behring GmbH

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, CSL Behring GmbH submitted to the licensing authority on 20/10/2022 17:20 BST an application for a Modification

The procedure started on 17/11/2022 13:22 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral.

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100695-PIP01-22-M02

Of 23/11/2022 20:59 GMT

On the adopted decision for Etranacogene dezaparvovec (MHRA-100695-PIP01-22-M02) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for Etranacogene dezaparvovec, Concentrate for solution for infusion , INTRAVENOUS USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of haemophilia B.

2.2 Indication(s) targeted by the PIP:

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

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

All subsets of the paediatric population from birth to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Concentrate for solution for infusion.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	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, Modeling & Simulation Studies	0	Not applicable.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	31/03/2042
Deferral of one or more studies contained in the paediatric investigation plan:	Yes