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 (MHRA-100451-PIP01-22-M02) and to the deferral

MHRA-100451-PIP01-22-M03

Scope of the Application

Active Substance(s)

fidanacogene elaparvovec

Condition(s)

Treatment of congenital factor IX deficiency (haemophilia B)

Pharmaceutical Form(s)

Concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Pfizer Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Pfizer Limited submitted to the licensing authority on 08/05/2024 14:18 BST an application for a Modification

The procedure started on 04/06/2024 16:32 BST

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-100451-PIP01-22-M03

Of 24/09/2024 07:45 BST

On the adopted decision for fidanacogene elaparvovec (MHRA-100451-PIP01-22-M03) 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 fidanacogene elaparvovec , Concentrate for solution for infusion , INTRAVENOUS USE .

This decision is addressed to Pfizer Limited, Ramsgate Road, Sandwich, Kent, UNITED KINGDOM, CT13 9NJ

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of congenital factor IX deficiency (haemophilia B)

2.2 Indication(s) targeted by the PIP:

Prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency)

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

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	2	Study 1 Juvenile pharmacology study in dogs with haemophilia to analyse a dose response in this age group and to assess the durability of FIX transgene expression over a time period that mimics the rate of growth seen in humans during the 2 to 6 and 6 to 12-year age period. Study 2 Juvenile pharmacology study in dogs with haemophilia to support dosing in children below 2 years of age and to assess the durability of FIX transgene expression over a time period that mimics the rate of growth seen in humans during the period from birth to 2 years of age.
Clinical Studies	1	Study 3 (C0371006) Open-label, single armed, intra-patient, controlled study to evaluate the safety and efficacy of a single infusion of fidanacogene elaparvovec in patients from birth to less than 18 years of age with moderately severe to severe haemophilia B in an age staggered manner.
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:	28/02/2039
Deferral of one or more studies contained in the paediatric investigation plan:	Yes