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

**gov.uk/mhra**

# Decision Cover Letter

## Decision of the licensing authority to:

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

MHRA-100499-PIP01-22-M01

# Scope of the Application

## Active Substance(s)

Giroctocogene Fitelparvovec

## Condition(s)

Treatment of haemophilia A

## Pharmaceutical Form(s)

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 31/03/2022 19:59 BST an application for a Modification

The procedure started on 19/11/2022 02:59 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-100499-PIP01-22-M01

Of 30/11/2022 20:28 GMT

On the adopted decision for Giroctocogene Fitelparvovec (MHRA-100499-PIP01-22-M01) 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 Giroctocogene Fitelparvovec, Solution for infusion , INTRAVENOUS USE .

This decision is addressed to Pfizer Limited, Ramsgate Road, Sandwich, UNITED KINGDOM, CT139NJ

## ANNEX I

### 1. Waiver

### 1.1 Condition:

| |
|---|
| Not applicable |

### 2. Paediatric Investigation Plan:

### 2.1 Condition(s):

| |
|---|
| Treatment of haemophilia A |

### 2.2 Indication(s) targeted by the PIP:

| |
|---|
| Treatment of haemophilia A (congenital FVIII deficiency) |

## 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):

| |
|---|
| Solution for infusion |

## 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 0 | Not applicable |
| **Non-Clinical Studies** | 1 | Study 1 Exploratory safety and efficacy study comparing FVIII expression levels between juvenile mice and sexually mature mice. |
| **Clinical Studies** | 1 | Study 2 (C3731005) Open-label, single-arm, single dose study to evaluate safety and efficacy of giroctocogene fitelparvovec in children from birth to less than 18 years of age with haemophilia A. |
| **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:** | 31/12/2039 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |