

EMA/121937/2024

European Medicines Agency decision

P/0115/2024

of 12 April 2024

on the acceptance of a modification of an agreed paediatric investigation plan for fidanacogene elaparvovec (EMEA-002362-PIP02-19-M03) 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.

European Medicines Agency decision

P/0115/2024

of 12 April 2024

on the acceptance of a modification of an agreed paediatric investigation plan for fidanacogene elaparvovec (EMA-002362-PIP02-19-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0222/2020 issued on 17 June 2020, the decision P/0382/2021 issued on 8 September 2021 and the decision P/0277/2022 issued on 10 August 2022,

Having regard to the application submitted by Pfizer Europe MA EEIG on 15 November 2023 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 February 2024, 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 and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

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 fidanacogene elaparvovec, concentrate for solution for infusion, intravenous use, including changes to the deferral, 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 Pfizer Europe MA EEIG, 17 Boulevard de la Plaine, 1050 – Brussels, Belgium

EMA/PDCO/537811/2023 Corr¹
Amsterdam, 23 February 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-002362-PIP02-19-M03

Scope of the application

Active substance(s):

Fidanacogene elaparvovec

Invented name and authorisation status:

See Annex II

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 Europe MA EEIG

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pfizer Europe MA EEIG submitted to the European Medicines Agency on 15 November 2023 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0222/2020 issued on 17 June 2020, the decision P/0382/2021 issued on 8 September 2021 and the decision P/0277/2022 issued on 10 August 2022.

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

The procedure started on 3 January 2024.

¹ 7 March 2024

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified and 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 and to the deferral 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 and the subset(s) of the paediatric population and condition(s) covered by the waiver 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 congenital factor IX deficiency (haemophilia B)

2.1.1. Indication(s) targeted by the PIP

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

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	Description
Quality-related studies	Not applicable
Non-clinical studies	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	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, modelling and simulation studies	Not applicable

Other studies	Not applicable
Other measures	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 February 2039
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.