EMA/808629/2022

European Medicines Agency decision

P/0431/2022

of 28 October 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512), (EMEA-003157-PIP01-21) 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 application submitted by Pfizer Europe MA EEIG on 15 December 2022 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 9 September 2022, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[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

A paediatric investigation plan for fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512), powder for solution for injection, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512), powder for solution for injection, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512), powder for solution for injection, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Pfizer Europe MA EEIG, Boulevard de la Plaine 17, 1050 – Brussels, Belgium.

EMA/PDCO/583579/2022
Amsterdam, 9 September 2022

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMEA-003157-PIP01-21

Scope of the application

Active substance(s):

Fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512)

Condition(s):

Treatment of focal segmental glomerulosclerosis

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Pfizer Europe MA EEIG

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Pfizer Europe MA EEIG submitted for agreement to the European Medicines Agency on 15 December 2021 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 31 January 2022.

Supplementary information was provided by the applicant on 27 May 2022. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 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

1.1. Condition:

Treatment of focal segmental glomerulosclerosis

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- powder for solution for injection, intravenous use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of focal segmental glomerulosclerosis

2.1.1. Indication(s) targeted by the PIP

Treatment of focal segmental glomerulosclerosis (FSGS) in addition to standard of care

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 year to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Powder for solution for injection

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable.
Non-clinical studies	Study 1: Pre- and postnatal development (PPND) study in rats to evaluate potential effects of PF-06730512 on pre- and postnatal development, including maternal function, and development of the central nervous system (CNS).
Clinical studies	Study 2 (C0221003): Double-blind, randomised, placebo-controlled trial to evaluate the efficacy and safety of PF-06730512 in participants from 12 years to less than 18 years of age (and adults) with FSGS.

	Study 3 (C0221005): Open-label, single-arm extension trial to evaluate the efficacy and safety of PF-06730512 in participants from 1 year to less than 18 years of age (and adults) with FSGS. Study 4 (C0221011): Open-label, single-arm trial to evaluate safety, tolerability, pharmacokinetics and efficacy of PF-06730512 in participants from 1 year to less than 12 years of age with FSGS.
Extrapolation, modelling and simulation studies	Study 5: Pharmacokinetic (PK) modelling and simulation study for dose justification of PF-06730512 in paediatric patients from 12 years to less than 18 years of age with FSGS. Study 6: Pharmacokinetic (PK) modelling and simulation study for dose justification of PF-06730512 in paediatric patients from 1 year to less than 12 years of age with FSGS. Study 7: Modelling and simulation study to characterise the exposure-response (ER) relationship in adult and paediatric FSGS subjects.
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:	No
Date of completion of the paediatric investigation plan:	By June 2030
Deferral for one or more measures contained in the paediatric investigation plan:	Yes