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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100403-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 Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Pfizer Limited submitted to the licensing authority on 18/01/2022 07:36 GMT an application for a Paediatric Investigation Plan

The procedure started on 10/10/2022 15:49 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 agree a paediatric investigation plan and grant a deferral and grant a waiver

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.

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

gov.uk/mhra

Final Decision Letter

MHRA-100403-PIP01-21

Of 20/10/2022 15:58 BST

On the adopted decision for Fusion protein composed of the first 2 immunoglobulin (Ig)-like domains of the human ROBO2 fused to human IgG1 Fc (PF-06730512) (MHRA-100403-PIP01-21) 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 a paediatric investigation plan

This decision applies to 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 .

This decision is addressed to Pfizer Limited, Ramsgate Road, Sandwich, Kent, United Kingdom, CT139NJ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of focal segmental glomerulosclerosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Powder for solution for injection Route(s) of administration: Intravenous use Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of focal segmental glomerulosclerosis

2.2 Indication(s) targeted by the PIP:

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

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

The paediatric population from 1 year to less than 18 years of age

2.4 Pharmaceutical Form(s):

Powder for solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	1	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	3	Study 2 (C0221003) Double-blind, randomised, placebo-controlled, trial to evaluate the efficacy and safety of PF-06730512 participants from 12 years to less than 18 years of age (and adults) with focal segmental glomerulosclerosis (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 assess the safety, tolerability, pharmacokinetics (PK) and efficacy of PF-06730512 in participants from 1 year to less than 12 years of age with FSGS.
Extrapolation, Modeling & Simulation Studies	3	Study 5 Pharmacokinetic (PK) modelling and simulation study for dose justification of PF-0673512 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-0673512 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	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:	No
Date of completion of the paediatric investigation plan:	30/06/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes