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-101451-PIP01-24)

MHRA-101451-PIP01-24-M01

Scope of the Application

Active Substance(s)

osivelotor

Condition(s)

Treatment of sickle cell disease

Pharmaceutical Form(s)

Tablet Age appropriate oral solid dosage form

Route(s) of Administration

ORAL 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 30/04/2024 13:00 BST an application for a Modification

The procedure started on 02/10/2024 11:50 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

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-101451-PIP01-24-M01

Of 04/11/2024 13:57 GMT

On the adopted decision for osivelotor (MHRA-101451-PIP01-24-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 osivelotor, Tablet Age appropriate oral solid dosage form , ORAL USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of sickle cell disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 months of age Pharmaceutical form(s): Tablet Age appropriate oral solid dosage form Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of sickle cell disease

2.2 Indication(s) targeted by the PIP:

Treatment of sickle cell disease

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

The paediatric population from 6 months to less than 18 years of age

2.4 Pharmaceutical Form(s):

Tablet Age appropriate oral solid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age appropriate oral solid dosage form.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 C5351004 (GBT021601-021) Part B Double-blind, randomised, placebo-controlled study to evaluate safety and efficacy of osivelotor in adolescents from 12 years to less than 18 years of age (and adults) with sickle cell disease. Study 3 C5351004 (GBT021601-021) Part C Open-label, uncontrolled study to evaluate pharmacokinetics and safety of osivelotor in children and adolescents from 6 months to less than 18 years of age with sickle cell disease.
Extrapolation, Modeling & Simulation Studies	3	Study 4 Modelling and simulation study to support dose finding in children from 12 years to less than 18 years of age with sickle cell disease. Study 5 Modelling and simulation study to support dose finding in children from 6 months to less than 12 years of age with sickle cell disease. Extrapolation Plan Studies 2, 3, 4 and 5 are part of the extrapolation plan of efficacy data from adult and adolescents to the paediatric population from 6

		months to less than 12 years of age with sickle cell disease.
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:	30/06/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes