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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral

MHRA-100612-PIP01-22

Scope of the Application

Active Substance(s)

Fosmanogepix

Condition(s)

Treatment of invasive fungal infections

Pharmaceutical Form(s)

Solution for infusion; Age appropriate parenteral formulation; Tablet; Age appropriate oral formulation

Route(s) of Administration

INTRAVENOUS, 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 14/07/2022 10:08 BST an application for a Paediatric Investigation Plan

The procedure started on 16/01/2023 08:26 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 agree a paediatric investigation plan and grant a 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-100612-PIP01-22

Of 16/06/2023 14:04 BST

On the adopted decision for Fosmanogepix (MHRA-100612-PIP01-22) 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 Fosmanogepix, Solution for infusion; Age appropriate parenteral formulation; Tablet; Age appropriate oral formulation , INTRAVENOUS USE; ORAL 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 invasive fungal infections

2.2 Indication(s) targeted by the PIP:

Treatment of invasive candidiasis (IC) Treatment of invasive aspergillosis (IC) and other invasive rare mould infections (IMI)

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

The paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for infusion Age appropriate parenteral formulation Tablet Age appropriate oral formulation

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of age-appropriate oral formulation. Study 2 Development of an age-appropriate parenteral formulation.
Non-Clinical Studies	1	Study 3 Toxicity study in juvenile rats to investigate the potential effects on the central nervous system (CNS).
Clinical Studies	2	Study 4 (C4791021) Open-label, single arm study to evaluate the pharmacokinetics, safety and tolerability of single dose (SD) and multiple dose (MD) fosmanogepix in paediatric patients from birth to 18 years of age who have an indication for antifungal prophylaxis. Study 5 (C4791022) Open-label, single arm study to evaluate safety, tolerability and pharmacokinetics of fosmanogepix in paediatric patients from birth to less than 18 years of age with possible, proven or probable invasive infections.
Extrapolation, Modeling & Simulation Studies	2	Study 6 Modelling and simulation study to determine the initial doses (both IV and PO) for Study 4 and Study 5 and to confirm the paediatric doses. Extrapolation Plan Studies 4 and 5 are part of an extrapolation plan covering the paediatric population from birth to less than 18 years of age.
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:	31/03/2035
Deferral of one or more studies contained in the paediatric investigation plan:	Yes