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 and grant a waiver

MHRA-100165-PIP01-21

Scope of the Application

Active Substance(s)

PF-06865571

Condition(s)

Treatment of non-alcoholic steatohepatitis

Pharmaceutical Form(s)

Tablet

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 02/08/2021 15:44 BST an application for a Paediatric Investigation Plan

The procedure started on 19/05/2022 14:48 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.

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Final Decision Letter

MHRA-100165-PIP01-21

Of 17/06/2022 15:02 BST

On the adopted decision for PF-06865571 (MHRA-100165-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 PF-06865571, Tablet , Oral use .

This decision is addressed to Pfizer Limited, Ramsgate Road, Sandwich, Kent, United Kingdom, CT13 9NJ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of non-alcoholic steatohepatitis (NASH) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 8 years of age Pharmaceutical form(s): Tablet Route(s) of administration: Oral use Reason for granting waiver: From birth to less than 2 years of age: on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s). From 2 years of age to less than 8 years of age: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments;

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of non-alcoholic steatohepatitis (NASH)

2.2 Indication(s) targeted by the PIP:

Treatment of non-cirrhotic NASH with advanced steatosis with liver fibrosis

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

The paediatric population from 8 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate tablet if necessary upon determination of the paediatric dose.
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	Study 2 (C2541018) Randomised, double-blind, placebo-controlled, dose-confirmatory study to evaluate PF-06865571 as monotherapy or to evaluate PF-06865571 in co-administration with PF-05221304 in adolescents from 16 years to less than 18 years (and adults) for the treatment of NASH with liver fibrosis (F2-F3). Study 3 (C2541015) Randomised, double blind, placebo-controlled, parallel group study to assess the efficacy, safety and pharmacokinetics (PK) of PF-06865571 as monotherapy or PF-06865571 in co-administration with PF-05221304 in paediatric participants aged 8 years to less than 18 years with non-cirrhotic NASH with liver fibrosis (F1- F3).
Extrapolation, Modeling & Simulation Studies	1	Study 4 Paediatric population pharmacokinetic (PK) / pharmacodynamic (PD) simulation study for paediatric dose selection for PF-06865571 (+/- PF-05221304)
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:	31/03/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes