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-100240-PIP01-21-M02) and to the deferral

MHRA-100240-PIP01-21-M04

Scope of the Application

Active Substance(s)

BOSUTINIB

Condition(s)

Treatment of chronic myeloid leukemia

Pharmaceutical Form(s)

Capsule, hard, Film-coated 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 25/10/2023 13:10 BST an application for a Modification

The procedure started on 09/01/2024 21:40 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 accept change(s) to the agreed paediatric investigation plan and to the 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-100240-PIP01-21-M04

Of 05/02/2024 16:46 GMT

On the adopted decision for BOSUTINIB (MHRA-100240-PIP01-21-M04) 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 BOSUTINIB, Capsule, hard, Film-coated tablet , ORAL USE .

This decision is addressed to Pfizer Limited, Ramsgate Road, Sandwich, UNITED KINGDOM, CT139NJ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of chronic myeloid leukaemia. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age. Pharmaceutical form(s): Capsule, hard Film-coated tablet Route(s) of administration: ORAL USE Reason for granting waiver: 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). Reason for Refusing Waiver: Not Applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of chronic myeloid leukaemia.

2.2 Indication(s) targeted by the PIP:

- Treatment of newly diagnosed chronic phase CML in children and adolescents (from 1 to less than 8 years of age). - Treatment of chronic, accelerated or blast phase CML in children and adolescents with resistance or intolerance to prior therapy including imatinib.

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

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

2.4 Pharmaceutical Form(s):

Capsule, hard Film-coated tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of age-appropriate hard capsules.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 removed in procedure EMEA-000727-PIP01-09-M01. Study 3 Bioequivalence study in adults with bosutinib age-appropriate hard capsules. Study 4 Two-phase study, a 6+4 dose escalation phase to determine a recommended phase 2 dose based on tolerability and PK of bosutinib and an open-label, non-controlled phase to evaluate anti-leukemic activity (determined by central laboratory analysis of cytogenetic and molecular response data), safety, tolerability, and PK of bosutinib.
Extrapolation, Modeling & Simulation Studies	0	Extrapolation plan Added in procedure MHRA-100240-PIP01-M04 Study 4 is part of the extrapolation plan of efficacy data from adult patients to the paediatric population from 1 year to less than 18 years of age with chronic myeloid leukaemia.
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:	30/09/2022
Deferral of one or more studies contained in the paediatric investigation plan:	Yes