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

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Decision Cover Letter

Decision of the licensing authority to:

grant a product specific waiver

MHRA-100234-PIP01-21

Scope of the Application

Active Substance(s)

(S)-N-(1-amino-4-(dimethylamino)-1-oxobutan-2-yl)-5-(2,4-difluorophenoxy)-1-isobutyl-1H-indazole-6-carboxamide

Condition(s)

Treatment of dilated cardiomyopathy due to lamin A/C gene mutations

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 17/08/2021 14:29 BST an application for a Paediatric Investigation Plan

The procedure started on 07/07/2022 14:08 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 grant a product specific 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-100234-PIP01-21

Of 23/08/2022 17:06 BST

On the adopted decision for (S)-N-(1-amino-4-(dimethylamino)-1-oxobutan-2-yl)-5-(2,4-difluorophenoxy)-1-isobutyl-1H-indazole-6-carboxamide (MHRA-100234-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:

Granting a waiver in all age groups for the listed condition(s)

This decision applies to a Paediatric Investigation Plan for (S)-N-(1-amino-4-(dimethylamino)-1-oxobutan-2-yl)-5-(2,4-difluorophenoxy)-1-isobutyl-1H-indazole-6-carboxamide, Tablet , Oral use .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of dilated cardiomyopathy due to lamin A/C gene mutations The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Tablet Route(s) of administration: Oral use Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Not Applicable

2.2 Indication(s) targeted by the PIP:

Not Applicable

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

Not Applicable

2.4 Pharmaceutical Form(s):

Not Applicable

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not Applicable
Non-Clinical Studies	0	Not Applicable
Clinical Studies	0	Not Applicable
Extrapolation, Modeling & Simulation Studies	0	Not Applicable
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:	
Date of completion of the paediatric investigation plan:	
Deferral of one or more studies contained in the paediatric investigation plan:	