MHRA
10 South Colonnade
Canary Wharf
London
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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-101307-PIP01-23

Scope of the Application

Active Substance(s)

inaxaplin

Condition(s)

Treatment of apolipoprotein L1 (APOL1) -mediated kidney disease

Pharmaceutical Form(s)

Film coated tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Vertex Pharmaceuticals (Europe) Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Vertex Pharmaceuticals (Europe) Limited submitted to the licensing authority on 14/12/2023 15:02 GMT an application for a Paediatric Investigation Plan

The procedure started on 06/08/2024 15:07 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-101307-PIP01-23

Of 05/09/2024 07:59 BST

On the adopted decision for inaxaplin (MHRA-101307-PIP01-23) 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 inaxaplin, Film coated tablet , ORAL USE .

This decision is addressed to Vertex Pharmaceuticals (Europe) Limited , 2 Kingdom Street, Paddington, UNITED KINGDOM, W2 6BD

ANNEX I

1. Waiver

1.1 Condition:

Treatment of apolipoprotein L1 (APOL1) -mediated kidney disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth less than 12 years of age Pharmaceutical form(s): Film coated 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):

Treatment of apolipoprotein L1 (APOL1) -mediated kidney disease

2.2 Indication(s) targeted by the PIP:

Treatment of proteinuric APOL1-mediated kidney disease (AMKD) in patients who have 2 APOL1 risk variants (G1/G1, G1/G2 or G2/G2)

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

The paediatric population from 12 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film coated tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 [Study C-1 (VX21-147-301)] Double-blind, randomised, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics of inaxaplin as add-on to standard of care in paediatric patients from 12 years to less than 18 years of age (and adults) with APOL1-mediated kidney disease (AMKD).
Extrapolation, Modeling & Simulation Studies	1	Extrapolation Plan Study 1 is part of the extrapolation plan of efficacy data from adults to the paediatric population from 12 years to less than 18 years of age with APOL1-mediated kidney disease (AMKD).
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:	31/12/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes