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 of change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-100802-PIP01-22-M01

Scope of the Application

Active Substance(s)

ROXADUSTAT

Condition(s)

Treatment of anaemia due to chronic disorders

Pharmaceutical Form(s)

Film-coated tablets

Route(s) of Administration

ORAL USE; GASTRIC USE

Name / Corporate name of the PIP applicant

Astellas Pharma Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Astellas Pharma Ltd submitted to the licensing authority on 20/12/2022 16:23 GMT an application for a Modification

The procedure started on 14/04/2023 18:40 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 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-100802-PIP01-22-M01

Of 25/04/2023 13:21 BST

On the adopted decision for ROXADUSTAT (MHRA-100802-PIP01-22-M01) 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 ROXADUSTAT, Film-coated tablets , ORAL USE .

This decision is addressed to Astellas Pharma Ltd , 300 Dashwood Lang Road, Bourne Business Park, Addlestone , UNITED KINGDOM, KT15 2NX

ANNEX I

1. Waiver

1.1 Condition:

Treatment of anaemia due to chronic disorders. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age. Pharmaceutical form(s): Film-coated tablet Route(s) of administration: ORAL USE; GASTRIC USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe. Reason for Refusing Waiver: Not Applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of anaemia due to chronic disorders.

2.2 Indication(s) targeted by the PIP:

Treatment of anaemia associated with chronic kidney disease.

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

The paediatric population from 2 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	1	Study 1 Development of lower strength of the film-coated tablet appropriate to the paediatric population not containing azo dyes
Non-Clinical Studies	1	Study 2 (ASP1517-PED-NON-CLIN-01) Definitive juvenile toxicity study.
Clinical Studies	4	Study 3 (ASP1517- PED-CLIN-01) Open label, randomised, 4-way cross-over study to evaluate the relative bioavailability, PK and palatability of the paediatric formulation versus adult tablet under fasting conditions. Study 4 Deleted during procedure MHRA-100802-PIP01-22-M01. Study 5 Deleted during procedure MHRA-100802-PIP01-22-M01. Study 6 (1517-CL-1003) (Added during procedure MHRA-100802-PIP01-22-M01) Open-label, uncontrolled study to evaluate PK/PD, safety and activity of roxadustat in paediatric patients from 2 years to less than 18 years of age with anaemia due to chronic kidney disease (CKD).
Extrapolation, Modeling & Simulation Studies	1	Study 7 (Added during procedure MHRA-100802-PIP01-22-M01) Model-based extrapolation study to evaluate efficacy of roxadustat for treatment of paediatric patients from 2 years to less than 18 years of age

		with anaemia due to chronic kidney disease (CKD).
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:	30/11/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes