MHRA
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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-100559-PIP01-22

Scope of the Application

Active Substance(s)

mitapivat

Condition(s)

Treatment of thalassaemia

Pharmaceutical Form(s)

Film-coated tablet; Age-appropriate oral solid dosage form (coated granules)

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Agios Netherlands B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Agios Netherlands B.V. submitted to the licensing authority on 01/08/2022 15:42 BST an application for a Paediatric Investigation Plan

The procedure started on 23/01/2023 08:57 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 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-100559-PIP01-22

Of 20/02/2023 15:44 GMT

On the adopted decision for mitapivat (MHRA-100559-PIP01-22) 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 mitapivat , Film-coated tablet; Age-appropriate oral solid dosage form (coated granules) , ORAL USE .

This decision is addressed to Agios Netherlands B.V., Zuidplein 36, Regus Amsterdam WTC, Amsterdam, NETHERLANDS, 1077 XV

ANNEX I

1. Waiver

1.1 Condition:

Treatment of thalassaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Film-coated tablet Age-appropriate oral solid dosage form (coated granules) Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of thalassaemia

2.2 Indication(s) targeted by the PIP:

Treatment of paediatric patients with thalassaemia

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

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

2.4 Pharmaceutical Form(s):

Film-coated tablet Age-appropriate oral solid dosage form (coated granules)

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 (Same as Study 1 of MHRA-100608-PIP01-22-M01 and subsequent modifications thereof) Development of an age-appropriate oral solid dosage form (coated granules) for use in paediatric patients unable to swallow the available tablets.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 Double-blind, randomised, placebo-controlled trial to evaluate the efficacy, pharmacokinetics and safety of mitapivat in children from 1 year to less than 18 years of age with alpha- or beta-transfusion-dependent thalassemia. Study 3 Double-blind, randomised, placebo-controlled trial to evaluate the efficacy, pharmacokinetics and safety of mitapivat in children from 1 year to less than 18 years of age with alpha- or beta- non-transfusion-dependent thalassemia.
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:	Yes

Date of completion of the paediatric investigation plan:	30/06/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes