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-100162-PIP01-21-M01)

MHRA-100162-PIP01-21-M02

Scope of the Application

Active Substance(s)

SELEXIPAG

Condition(s)

Treatment of Pulmonary arterial hypertension (PAH)

Pharmaceutical Form(s)

Film-coated tablet, Tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Janssen-Cilag Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Janssen-Cilag Limited submitted to the licensing authority on 06/03/2023 23:50 GMT an application for a

The procedure started on 05/07/2023 11:37 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

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-100162-PIP01-21-M02

Of 09/10/2023 08:39 BST

On the adopted decision for SELEXIPAG (MHRA-100162-PIP01-21-M02) 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 for SELEXIPAG, Film-coated tablet, Tablet , ORAL USE .

This decision is addressed to Janssen-Cilag Limited , 50-100 Holmers Farm Way, High Wycombe, UNITED KINGDOM, HP124EG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of pulmonary arterial hypertension (PAH) 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 Tablet Route(s) of administration: Oral use Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of pulmonary arterial hypertension (PAH)

2.2 Indication(s) targeted by the PIP:

Treatment of pulmonary arterial hypertension

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 Tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of mini tablets (diameter < 3mm).
Non-Clinical Studies	2	Study 2 28-day dose range finding toxicity study in juvenile dogs. Study 3 39-week toxicity study in juvenile dogs.
Clinical Studies	2	Study 4 This study was deleted during procedure EMEA-000997-PIP01-10-M02. Study 5 This study was deleted during procedure EMEA-000997-PIP01-10-M02. Study 6 (AC-065A203) Open-label, single-arm study to evaluate the safety, tolerability and pharmacokinetics of selexipag in children from 2 years to less than 18 years of age with pulmonary arterial hypertension (PAH). Study 7 (AC-065A310) Double-blind, randomised, placebo-controlled, parallel group study with open-label extension period to assess the efficacy and safety of selexipag as add-on to standard of care in children from 2 years to less than 18 years with pulmonary arterial hypertension (PAH).
Extrapolation, Modeling & Simulation Studies	1	Study 8 Added during procedure MRHA-100162-PIP01-21-M02. Pharmacodynamic (PD) similarity/ comparison study to compare the PD and clinical responses for efficacy based on Study 6 (AC-065A203), Study 7 (AC-065A310) and

		Study AC-065A302 in paediatric participants from 2 years to less than 18 years of age and adult participants with PAH.
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:	31/12/2023
Deferral of one or more studies contained in the paediatric investigation plan:	Yes