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-100991-PIP01-23)

MHRA-100991-PIP01-23-M01

Scope of the Application

Active Substance(s)

MARALIXIBAT CHLORIDE

Condition(s)

Progressive familial intrahepatic cholestasis (PFIC)

Pharmaceutical Form(s)

Tablet, Oral solution

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Mirum Pharmaceuticals, Inc.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Mirum Pharmaceuticals, Inc. submitted to the licensing authority on 19/01/2024 15:50 GMT an application for a Modification

The procedure started on 12/02/2024 10:51 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 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-100991-PIP01-23-M01

Of 04/06/2024 07:37 BST

On the adopted decision for MARALIXIBAT CHLORIDE (MHRA-100991-PIP01-23-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 MARALIXIBAT CHLORIDE, Tablet, Oral solution , ORAL USE .

This decision is addressed to Mirum Pharmaceuticals, Inc., 950 Tower Lane, Suite 1050, Foster City, UNITED STATES OF AMERICA, 94404

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of progressive familial intrahepatic cholestasis (PFIC)

2.2 Indication(s) targeted by the PIP:

Treatment of pruritis in progressive familial intrahepatic cholestasis

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

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

2.4 Pharmaceutical Form(s):

Oral solution Tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 1 (MRX-502) Randomised, double-blind, placebo-controlled study in children from 12 months to less than 18 years of age with PFIC. Study 2 (LUM001-501) Open label safety and efficacy study in children from 12 months to less than 18 years of age with PFIC 1 or 2. Study 3 (MRX-801) (Added during procedure EMEA-001475-PIP03-17-M01) Open label, uncontrolled safety study to evaluate the safety and tolerability of maralixibat in paediatric subjects with cholestatic liver diseases including, but not limited to, Alagille syndrome and PFIC from birth to less than 12 months of age.
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:	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