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-100432-PIP01-22)

MHRA-100432-PIP01-22-M01

Scope of the Application

Active Substance(s)

Zilurgisertib

Condition(s)

Treatment of fibrodysplasia ossificans progressiva

Pharmaceutical Form(s)

Film-coated tablet; Age-appropriate oral solid dosage form

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

Mirum Pharmaceuticals International B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Mirum Pharmaceuticals International B.V. submitted to the licensing authority on 23/07/2026 09:55 BST an application for a Modification

The procedure started on 04/08/2026 15:33 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-100432-PIP01-22-M01

Of 01/10/2026 17:10 BST

On the adopted decision for Zilurgisertib (MHRA-100432-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 Zilurgisertib, Film-coated tablet; Age-appropriate oral solid dosage form , Oral use .

This decision is addressed to Mirum Pharmaceuticals International B.V., First Floor 1, Q1 The Square, Randalls Way,, Leatherhead, UNITED KINGDOM, 1043

ANNEX I

1. Waiver

1.1 Condition:

Treatment of fibrodysplasia ossificans progressiva The waiver applies / applied to: Paediatric Subset(s): The paediatric subset from birth to less than 2 years of age Pharmaceutical form(s): Film-coated tablet Age-appropriate oral solid dosage form 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 fibrodysplasia ossificans progressiva

2.2 Indication(s) targeted by the PIP:

Treatment of fibrodysplasia ossificans progressiva

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

The paediatric population form 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet Age-appropriate oral solid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of age-appropriate oral solid dosage form, for oral use.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 (INCB 00928 201) Double-blind, randomised, placebo-controlled study to determine the efficacy, safety, and tolerability of zilurgisertib for the prevention of new heterotopic ossification in children from 2 years to less than 18 years of age (and adults) with fibrodysplasia ossificans progressiva.
Extrapolation, Modeling & Simulation Studies	1	Study 3 Compartmental pharmacokinetic model with absorption and disposition to characterise the pharmacokinetic (PK) data of zilurgisertib in children.
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:	31/01/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes