MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100673-PIP01-22

Scope of the Application

Active Substance(s)

Fidrisertib

Condition(s)

Treatment of fibrodysplasia ossificans progressiva

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

ORAL USE; GASTRIC USE

Name / Corporate name of the PIP applicant

Ipsen Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Ipsen Limited submitted to the licensing authority on 02/12/2022 13:17 GMT an application for a Paediatric Investigation Plan

The procedure started on 24/05/2023 16:52 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 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-100673-PIP01-22

Of 17/07/2023 08:30 BST

On the adopted decision for Fidrisertib (MHRA-100673-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 Fidrisertib, Capsule, hard , ORAL USE; GASTRIC USE .

This decision is addressed to Ipsen Limited, 190 Bath Road, Slough, UNITED KINGDOM, SL1 3XE

ANNEX I

1. Waiver

1.1 Condition:

Treatment of fibrodysplasia ossificans progressiva The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Capsule, hard 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.

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 (FOP)

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):

Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of an age-appropriate oral solid dosage form Study 2 Study to demonstrate feasibility of administration of the drug product through the enteral tube/feeding tube
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 3 (D-CA-60130-452) A three-part, placebo-controlled, parallel-group, double-blind study (part A and B) with an extension (part C) to assess the efficacy and safety of 2 dosage regimens of oral IPN60130 in paediatric patients from 2 years to less than 18 years of age (and adults) with fibrodysplasia ossificans progressiva
Extrapolation, Modeling & Simulation Studies	1	Study 4 (D-CA-60130-452 POP-PK and PK/PD modelling) Population pharmacokinetic (PK) modelling study to describe the PK profile of IPN60130 in D-CA-60130-452 participants (in paediatrics and adults).
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