MHRA
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Canary Wharf
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United Kingdom

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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-100432-PIP01-22

Scope of the Application

Active Substance(s)

2-Amino-N-(4-hydroxybicyclo[2.2.2]octan-1-yl)-5-(4-((1R,5S)-3- (tetrahydro-2H-pyran-4-yl)-3-azabicyclo[3.1.0]hexan-1- yl)phenyl)nicotinamide fumarate dihydrate

Condition(s)

Treatment of fibrodysplasia ossificans progressiva

Pharmaceutical Form(s)

Film-coated tablet; Age-appropriate solid dosage form

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

Incyte Biosciences UK Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Incyte Biosciences UK Ltd submitted to the licensing authority on 25/03/2022 13:51 GMT an application for a Paediatric Investigation Plan

The procedure started on 22/07/2022 07:27 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-100432-PIP01-22

Of 13/10/2022 17:21 BST

On the adopted decision for 2-Amino-N-(4-hydroxybicyclo[2.2.2]octan-1-yl)-5-(4-((1R,5S)-3-(tetrahydro-2H-pyran-4-yl)-3-azabicyclo[3.1.0]hexan-1- yl)phenyl)nicotinamide fumarate dihydrate (MHRA-100432-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 2-Amino-N-(4-hydroxybicyclo[2.2.2]octan-1-yl)-5-(4-((1R,5S)-3- (tetrahydro-2H-pyran-4-yl)-3-azabicyclo[3.1.0]hexan-1- yl)phenyl)nicotinamide fumarate dihydrate , Film-coated tablet; Age-appropriate solid dosage form , Oral use .

This decision is addressed to Incyte Biosciences UK Ltd, First Floor, The Square, Randalls Way, Leatherhead, United Kingdom, KT22 7TW

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 INCB000928 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 INCB000928 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