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 and to the deferral

MHRA-100750-PIP01-22-M01

Scope of the Application

Active Substance(s)

setrusumab

Condition(s)

Treatment of osteogenesis imperfecta

Pharmaceutical Form(s)

Powder for solution for injection

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Mereo Biopharma 3 Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Mereo Biopharma 3 Ltd submitted to the licensing authority on 14/12/2022 14:12 GMT an application for a

The procedure started on 26/04/2023 10:55 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 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-100750-PIP01-22-M01

Of 19/05/2023 11:30 BST

On the adopted decision for setrusumab (MHRA-100750-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 included in that paediatric investigation plan)

This decision applies to a for setrusumab, Powder for solution for injection , INTRAVENOUS .

This decision is addressed to Mereo Biopharma 3 Ltd, 4th Floor, 1 Cavendish Place, London, UNITED KINGDOM, W1G 0QF

ANNEX I

1. Waiver

1.1 Condition:

Treatment of osteogenesis imperfecta. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age. Pharmaceutical form(s): Powder for solution for injection Route(s) of administration: INTRAVENOUS 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 osteogenesis imperfecta.

2.2 Indication(s) targeted by the PIP:

Treatment of osteogenesis imperfecta.

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):

Powder for solution for injection.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	2	Study 1 Dose range-finding juvenile toxicity study Study 2 Definitive juvenile toxicity study
Clinical Studies	2	Study 3 (UX143-CL301) Randomised, double-blind, placebo-controlled parallel group (adaptive design) study to select a dose and to assess the efficacy, safety and pharmacokinetics of intravenous setrusumab in growing paediatric patients from 5 years to less than 18 years (and 18 years to less than 26 years) of age with osteogenesis imperfecta (OI) Types I, III, or IV. Study 4 (UX143-CL314) Randomised, open-label, active-controlled, parallel group study to assess the efficacy, safety and pharmacokinetics of intravenous setrusumab compared with standard-of-care intravenous bisphosphonates in paediatric subjects 2 years to less than 5 years of age with OI Types I, III, or IV confirmed by COL1A1 or COL1A2 mutation. Study 5 Deleted during procedure MHRA-100750-PIP01-22-M01.
Extrapolation, Modeling & Simulation Studies	1	Study 6 Modelling and simulation study to support the use of setrusumab for the treatment of osteogenesis imperfecta in children from 2 years to less than 18 years of age.

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:	31/03/2027
Deferral of one or more studies contained in the paediatric investigation plan:	No