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-100222-PIP01-21-M02) and to the deferral

MHRA-100222-PIP01-21-M03

Scope of the Application

Active Substance(s)

ROMOSOZUMAB

Condition(s)

Treatment of osteoporosis

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

UCB Pharma Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, UCB Pharma Ltd submitted to the licensing authority on 20/09/2024 14:51 BST an application for a Modification

The procedure started on 18/10/2024 17:38 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-100222-PIP01-21-M03

Of 28/10/2024 10:53 GMT

On the adopted decision for ROMOSOZUMAB (MHRA-100222-PIP01-21-M03) 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 ROMOSOZUMAB, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to UCB Pharma Ltd, 208 Bath Road, Berkshire, UNITED KINGDOM, SL1 3WE

ANNEX I

1. Waiver

1.1 Condition:

Treatment of osteoporosis. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 5 years of age. Pharmaceutical form(s): Solution for injection. Route(s) of administration: SUBCUTANEOUS 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 osteoporosis.

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 5 years of age to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Solution for injection.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Age-appropriate pharmaceutical form for parenteral use.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 Open label, single arm, ascending multiple dose study to evaluate the safety, pharmacokinetics (PK) and pharmacodynamics (PD) of romosozumab in paediatric patients from 5 years of age to less than 18 years of age with osteogenesis imperfecta (OI). Study 3 Randomised, open-label, controlled vs standard of care study to evaluate the efficacy and safety of romosozumab in children with osteoporosis imperfecta in paediatric patients from 5 years of age to less than 18 years of age. Study 4 Study deleted in MHRA-100222-PIP01-21-M01. Study 5 Study deleted in MHRA-100222-PIP01-21-M01.
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:	Yes

Date of completion of the paediatric investigation plan:	31/05/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes