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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100225-PIP01-21-M01) and to the deferral

MHRA-100225-PIP01-21-M02

Scope of the Application

Active Substance(s)

PARATHYROID HORMONE

Condition(s)

Treatment of hypoparathyroidism

Pharmaceutical Form(s)

Powder for solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Takeda UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Takeda UK Limited submitted to the licensing authority on 12/06/2023 17:00 BST an application for a Modification

The procedure started on 20/10/2023 15:43 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-100225-PIP01-21-M02

Of 02/11/2023 17:13 GMT

On the adopted decision for PARATHYROID HORMONE (MHRA-100225-PIP01-21-M02) 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 PARATHYROID HORMONE, Powder for solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Takeda UK Limited, 1 Kingdom Street, Paddington, London, UNITED KINGDOM, W2 6BD

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of hypoparathyroidism

2.2 Indication(s) targeted by the PIP:

The long-term treatment of subjects with hypoparathyroidism

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

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Powder and solvent for solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of lower strength of existing dose form, appropriate for children from birth to less than 12 years of age.
Non-Clinical Studies	1	Study 2 Repeat-dose toxicity study in juvenile rats, including bone densitometry, histomorphometry and histopathology, plus femur and tibia length.
Clinical Studies	1	Study 3 Deleted and combined with study 5 in procedure MHRA-100225-PIP01-21-M01. Study 4 Deleted and combined with study 3 in procedure EMEA-001526-PIP01-13-M03. Study 5 Open-label, non-controlled trial to assess pharmacokinetics, pharmacodynamics, safety and activity of recombinant parathyroid hormone, administered with age-appropriate delivery device, as add-on to best standard of care in children and adolescents from birth to less than 18 years of age with hypoparathyroidism. Study 6 Deleted and combined with study 5 in procedure EMEA-001526-PIP01-13-M03.
Extrapolation, Modeling & Simulation Studies	1	Study 7 Development of a population PK model and quantitative system pharmacology model (QSP) to perform simulations and to support the choice of doses for the studies in children from birth 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:	30/06/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes