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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral

MHRA-102029-PIP01-25

Scope of the Application

Active Substance(s)

Encaleret

Condition(s)

Treatment of hypoparathyroidism

Pharmaceutical Form(s)

Film-coated tablet, Age-appropriate oral dosage form;

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

BRIDGEBIO EUROPE B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, BRIDGEBIO EUROPE B.V. submitted to the licensing authority on 18/07/2025 15:40 BST an application for a Paediatric Investigation Plan

The procedure started on 24/07/2025 20:38 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 agree a paediatric investigation plan and grant a 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-102029-PIP01-25

Of 12/12/2025 08:18 GMT

On the adopted decision for Encaleret (MHRA-102029-PIP01-25) 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 Encaleret, Film-coated tablet, Age-appropriate oral dosage form; , ORAL USE .

This decision is addressed to BRIDGEBIO EUROPE B.V., Weerdestein 97, Amsterdam, NETHERLANDS, 1083GG

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:

Treatment of autosomal dominant hypocalcaemia type 1 (ADH1)

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):

Film-coated tablet Age-appropriate oral dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate oral dosage form suitable for children from birth to less than 12 years of age.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 (CLTX-305-303) Open-label trial to evaluate pharmacokinetics, safety and efficacy of encaleret in children from birth to less than 18 years of age with autosomal dominant hypocalcaemia Type 1 (ADH1).
Extrapolation, Modeling & Simulation Studies	1	Study 3 Modelling and simulation study to evaluate the use of encaleret in children from birth to less than 18 years of age with autosomal dominant hypocalcaemia Type 1 (ADH1)
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:	No
Date of completion of the paediatric investigation plan:	31/12/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes