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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral

MHRA-102026-PIP01-25

Scope of the Application

Active Substance(s)

DB-OTO

Condition(s)

Treatment of otoferlin gene-mediated hearing loss

Pharmaceutical Form(s)

suspension for infusion

Route(s) of Administration

AURICULAR USE

Name / Corporate name of the PIP applicant

Regeneron UK Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Regeneron UK Ltd submitted to the licensing authority on 15/08/2025 15:51 BST an application for a Paediatric Investigation Plan

The procedure started on 28/08/2025 12:00 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-102026-PIP01-25

Of 17/07/2026 07:29 BST

On the adopted decision for DB-OTO (MHRA-102026-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 DB-OTO, suspension for infusion , AURICULAR USE .

This decision is addressed to Regeneron UK Ltd , 3rd Floor, The Charter Building, Vine Street, Uxbridge, UNITED KINGDOM, UB8 1JG

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of otoferlin gene-mediated hearing loss

2.2 Indication(s) targeted by the PIP:

Treatment of congenital deafness caused by biallelic OTOF mutations

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):

Suspension for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 (DB-OTO-001) Open-label trial to evaluate the safety, tolerability, and efficacy of DB-OTO in children with biallelic otoferlin gene (hOTOF) mutations.
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:	No
Date of completion of the paediatric investigation plan:	31/05/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes