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-102165-PIP01-25

Scope of the Application

Active Substance(s)

adeno-associated viral vector serotype Anc80 containing the 5' portion of human OTOF gene; adeno-associated viral vector serotype Anc80 containing the 3' portion of human OTOF gene

Condition(s)

Treatment of otoferlin gene-mediated hearing loss

Pharmaceutical Form(s)

Suspension for injection

Route(s) of Administration

AURICULAR USE

Name / Corporate name of the PIP applicant

Akouos, Inc. a wholly owned subsidiary of Eli Lilly and Company

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Akouos, Inc. a wholly owned subsidiary of Eli Lilly and Company submitted to the licensing authority on 10/10/2025 17:42 BST an application for a

The procedure started on 13/10/2025 13:50 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-102165-PIP01-25

Of 28/11/2025 18:11 GMT

On the adopted decision for adeno-associated viral vector serotype Anc80 containing the 5' portion of human OTOF gene; adeno-associated viral vector serotype Anc80 containing the 3' portion of human OTOF gene (MHRA-102165-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 for adeno-associated viral vector serotype Anc80 containing the 5' portion of human OTOF gene; adeno-associated viral vector serotype Anc80 containing the 3' portion of human OTOF gene, Suspension for injection , AURICULAR USE .

This decision is addressed to Akouos, Inc. a wholly owned subsidiary of Eli Lilly and Company, 645 Summer Street, Suite 200, Boston, UNITED STATES OF AMERICA, 02210

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 otoferlin gene (OTOF)-mediated hearing loss

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 injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 1 (AK-OTOF-101 Part A) First-in-human open-label, single-arm trial to evaluate safety, tolerability, and activity of escalating doses of AAVAnc80-hOTOF in children from birth to less than 18 years of age with otoferlin gene (OTOF)-mediated hearing loss. Study 2 (AK-OTOF-101 Part B) Open-label, single-arm trial to evaluate safety and efficacy of AAVAnc80-hOTOF in children from birth to less than 18 years of age with OTOF-mediated hearing loss.
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:	31/12/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes