

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 adopted paediatric investigation plan and to the deferral

MHRA-102230-PIP01-25-M01

Scope of the Application

Active Substance(s)

Rebisufligene etisparvovec

Condition(s)

Treatment of mucopolysaccharidosis IIIA

Pharmaceutical Form(s)

Solution for infusion

Route(s) of Administration

PARENTERAL USE

Name / Corporate name of the PIP applicant

Ultragenyx Netherlands B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Ultragenyx Netherlands B.V. submitted to the licensing authority on 20/11/2025 18:05 GMT an application for a Modification

The procedure started on 09/12/2025 08:46 GMT

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-102230-PIP01-25-M01

Of 10/02/2026 11:09 GMT

On the adopted decision for Rebisufligene etisparvec (MHRA-102230-PIP01-25-M01) 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 Rebisufligene etisparvec , Solution for infusion , PARENTERAL USE .

This decision is addressed to Ultragenyx Netherlands B.V., Evert van de Beekstraat 1, Unit 104, Schiphol, NETHERLANDS, 1118 CL

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of mucopolysaccharidosis IIIA.

2.2 Indication(s) targeted by the PIP:

Treatment of mucopolysaccharidosis IIIA.

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

The paediatric population from birth to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Solution 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	3	Study 1 (ABT-001) Open-label, historical-controlled, dose-escalation trial to evaluate safety and efficacy of rebisufligene etisparvovec in children with mucopolysaccharidosis IIIA from birth to 2 years of age with no minimum Cognitive Developmental Quotient (DQ) requirement or older than 2 years of age with a minimum Cognitive Development Quotient (DQ) of 60. Study 2 (ABT-003) Open-label, uncontrolled trial to evaluate safety and efficacy of rebisufligene etisparvovec in children with mucopolysaccharidosis IIIA who have a Cognitive Development Quotient (DQ) of less than 60. Study 3 (Added during procedure MHRA-102230-PIP01-25-M01.) Long-term follow-up study to evaluate the efficacy and safety of rebisufligene etisparvovec in paediatric patients with mucopolysaccharidosis IIIA who received rebisufligene etisparvovec in a prior clinical trial.
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:	31/03/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes