

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-100178-PIP01-21) and to the deferral

MHRA-100178-PIP01-21-M01

Scope of the Application

Active Substance(s)

pariglasgene breCAParvovec (DTX401)

Condition(s)

Treatment of glycogen storage disease type 1a (GSDIa)

Pharmaceutical Form(s)

Concentrate for solution for infusion

Route(s) of Administration

Intravenous 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 17/06/2026 16:35 BST an application for a Modification

The procedure started on 01/07/2026 07:23 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-100178-PIP01-21-M01

Of 04/09/2026 17:45 BST

On the adopted decision for pariglasgene breCAParvovec (DTX401) (MHRA-100178-PIP01-21-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 pariglasgene breCAParvovec (DTX401), Concentrate for solution for infusion , Intravenous 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:

Treatment of glycogen storage disease type 1a (GSD1a) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Concentrate for solution for infusion Route(s) of administration: Intravenous use Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of glycogen storage disease type 1a (GSD1a)

2.2 Indication(s) targeted by the PIP:

Treatment of glycogen storage disease type 1a (GSD1a)

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

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	2	Study 1 (CRL Study number: 20282036) Juvenile animal efficacy study to evaluate the potential loss of vector expression and pharmacological effects at various ages to better inform paediatric dose selection. Study 4 (CRL Study Number: 20317343) Added in procedure MHRA-100178-PIP01-21-M01 Juvenile animal study to evaluate the biodistribution and potential toxicity of pariglasgene breCAPARVovec (DTX401) administered at various ages after birth.
Clinical Studies	2	Study 2 (DTX401-CL301) Randomised, double-blind, placebo-controlled Phase 3 clinical study to evaluate the efficacy and safety of pariglasgene breCAPARVovec in patients 8 years of age and older with GSD1a. In particular the reduction or elimination of dependence on exogenous glucose replacement therapy and the effect of pariglasgene breCAPARVovec on glucose control will be evaluated. Study 3 (DTX401-CL302) Open-label, non-comparative study to determine the efficacy and confirm the safety of pariglasgene

		brecaparvovec in paediatric patients with GSDIa from 2 years to less than 8 years of age, evaluating the efficacy of pariglasgene brecaparvovec in providing normal G6Pase activity, and thereby reducing dependence on exogenous glucose replacement therapy needed to maintain glucose control.
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:	30/06/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes