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-102116-PIP01-25

Scope of the Application

Active Substance(s)

Delpacibart etedesiran

Condition(s)

Treatment of myotonic disorders

Pharmaceutical Form(s)

Powder for concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Novartis Pharmaceuticals UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Novartis Pharmaceuticals UK Limited submitted to the licensing authority on 10/10/2025 17:35 BST an application for a Paediatric Investigation Plan

The procedure started on 14/10/2025 11:10 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-102116-PIP01-25

Of 12/08/2026 07:50 BST

On the adopted decision for Delpacibart etedesiran (MHRA-102116-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 Delpacibart etedesiran, Powder for concentrate for solution for infusion , INTRAVENOUS .

This decision is addressed to Novartis Pharmaceuticals UK Limited, 3020 Callan Road, San Diego, UNITED STATES OF AMERICA, W12 7FQ

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of myotonic disorders

2.2 Indication(s) targeted by the PIP:

Treatment of paediatric patients with myotonic dystrophy type I (DM1)

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):

Powder for concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	3	Study 1 (AOC 1001-TX-018) Fertility and early embryofoetal development toxicity study in CD-1 mice. Study 2 (AOC 1001-TX-019) Pre- and postnatal development toxicity study in CD-1 mice. Study 3 Definitive juvenile toxicity study in CD-1 mice.
Clinical Studies	2	Study 4 (AOC 1001-CS3) Double-blind, randomised, placebo-controlled trial to evaluate the safety and efficacy of delpacibart etedesiran in paediatric patients from 16 years to less 18 years of age (and adults) with myotonic dystrophy type 1. Study 5 (AOC 1001-CS5) Open-label, single-arm study to evaluate the safety, activity, and pharmacokinetics of delpacibart etedesiran in paediatric patients from birth to less than 16 years of age with myotonic dystrophy type 1.
Extrapolation, Modeling & Simulation Studies	2	Study 6 Modelling and simulation study based on PopPK modelling from adult and emerging paediatric data to inform paediatric dosing and support the paediatric extrapolation. Extrapolation Plan Studies 4, 5, 6, and natural history study END-DM1 are part of the extrapolation plan of efficacy data from adult and adolescents from 16 years of age to the paediatric population from birth to less than 18 years of age with myotonic dystrophy type 1.

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:	31/12/2036
Deferral of one or more studies contained in the paediatric investigation plan:	Yes