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-100734-PIP01-22

Scope of the Application

Active Substance(s)

tideglusib

Condition(s)

Treatment of myotonic dystrophy

Pharmaceutical Form(s)

Powder for oral suspension

Route(s) of Administration

ORAL USE ENTERAL USE

Name / Corporate name of the PIP applicant

AMO Pharma Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, AMO Pharma Ltd submitted to the licensing authority on 01/03/2023 13:36 GMT an application for a Paediatric Investigation Plan

The procedure started on 21/11/2023 13:30 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 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-100734-PIP01-22

Of 03/03/2025 08:22 GMT

On the adopted decision for tideglusib (MHRA-100734-PIP01-22) 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 tideglusib, Powder for oral suspension , ORAL USE ENTERAL USE .

This decision is addressed to AMO Pharma Ltd, Braeburn, Grove Road, Godalming, Surrey, UNITED KINGDOM, GU7 1RE

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of myotonic dystrophy

2.2 Indication(s) targeted by the PIP:

Treatment of myotonic dystrophy type 1

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 oral suspension

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Study to evaluate the feasibility of tideglusib powder for oral suspension via feeding tubes. Study 2 Development of a lower strength powder for oral suspension.
Non-Clinical Studies	4	Study 3 (2809-20292) Dose range-finding juvenile toxicity study in rats. Study 4 (2809-22147) Juvenile toxicity study in rats Study 5 (2809-211365) Definitive juvenile toxicity study in rats. Study 6 Enhanced pre- and postnatal development study in rats.
Clinical Studies	4	Study 7 (AMO-02-MD-2-001) Single blinded, placebo controlled study to evaluate safety, activity and pharmacokinetics of 2 doses of tideglusib in adolescents from 12 years to less than 18 years of age (and adults) with congenital or juvenile (also known as childhood) onset myotonic dystrophy (DM-1). Study 8 (AMO-02-MD-2-003 [REACH CDM]) Double-blind, randomised, placebo-controlled study to evaluate safety and efficacy of tideglusib in children from 6 years to less than or equal to 16 years of age with congenital DM-1. Study 9 (AMO-02-MD-2-005) Single arm, open label trial to evaluate safety, activity and pharmacokinetics of tideglusib in children from birth to less than 6 years of age with congenital and childhood onset myotonic dystrophy. Study 10

		(AMO-02-MD-2-004 [REACH CDM X]) Open-label, single arm study to evaluate the long-term safety, tolerability and activity of tideglusib in participants with congenital or childhood onset myotonic dystrophy from 6 years to less the 18 years of age (and adults) .
Extrapolation, Modeling & Simulation Studies	1	Study 11 (AMO-02-MD-2-005 Modelling) Modelling and simulation study to confirm the paediatric dose to be used in children from birth to less than 18 years of age with congenital or childhood onset myotonic dystrophy.
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:	31/05/2034
Deferral of one or more studies contained in the paediatric investigation plan:	Yes