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 and grant a waiver

MHRA-102040-PIP01-25

Scope of the Application

Active Substance(s)

Brogidirsen

Condition(s)

Treatment of Duchenne muscular dystrophy

Pharmaceutical Form(s)

Solution for injection/infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

NS Pharma, Inc.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, NS Pharma, Inc. submitted to the licensing authority on 17/09/2025 17:01 BST an application for a Paediatric Investigation Plan

The procedure started on 04/11/2025 10:37 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 and grant a waiver

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-102040-PIP01-25

Of 10/04/2026 17:30 BST

On the adopted decision for Brogidirsen (MHRA-102040-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 Brogidirsen , Solution for injection/infusion , INTRAVENOUS USE .

This decision is addressed to NS Pharma, Inc., 140 East Ridgewood Ave, Suite 280S, Paramus, New Jersey, UNITED STATES OF AMERICA, 07652

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Duchenne muscular dystrophy The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 months of age Pharmaceutical form(s): Solution for injection/infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Duchenne muscular dystrophy

2.2 Indication(s) targeted by the PIP:

Treatment of Duchenne muscular dystrophy in patients amenable to exon 44 skipping

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

The paediatric population from 6 months to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for injection/infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Assessment of suitability of the current formulation of brogidirsen for the use in children from 6 months to 2 years of age.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	6	Study 2 (NS089/NCNP-02-P2OE) Open-label, parallel-group comparison (2 doses) extension study to evaluate the safety, tolerability and efficacy of brogidirsen in patients with Duchenne muscular dystrophy (DMD) from 4 years to less than 17 years of age. Study 3 (NS-089/NCNP-02-201) Open-label, externally controlled 2-part study to evaluate safety and tolerability of brogidirsen administered by intravenous (IV) infusion once weekly to ambulant boys from 4 years to less than 15 years of age with DMD due to mutations amenable to exon 44 skipping. Study 4 (NS-089/NCNP-02-202) Open-label extension study to evaluate safety and efficacy of brogidirsen administered for 120 weeks to ambulant boys with DMD who completed PIP Study 3 (NS-089/NCNP-02-201). Study 5 (NS-089/NCNP-02-301) Double-blind, randomised, placebo-controlled, study to evaluate the safety and efficacy of brogidirsen

		in ambulant boys from 7 years to less than 18 years with DMD at the dose determined in PIP Study 3 (NS-089/NCNP-02-201). Study 6 (NS-089/NCNP-02-311) Double-blind, randomised, placebo-controlled, study to evaluate the safety, tolerability and efficacy of brogidirsen in non-ambulant boys from 10 years to less than 18 years of age with DMD at the dose determined in PIP Study 3 (NS-089/NCNP-02-201). Study 7 (NS-089/NCNP-01-221) Single arm study to evaluate the safety, tolerability, and activity of brogidirsen in paediatric participants from 6 months to less than 4 years of age with DMD.
Extrapolation, Modeling & Simulation Studies	2	Study 8 Population pharmacokinetic model and exposure- response model to guide dose selection in patients in NS-089/NCNP-01-221 and analyse PK/PD relationship. Extrapolation plan Studies NCNP/DMT02, PIP Study 2 (NS-089/NCNP02-P2OE), PIP study 3 (NS-089/NCNP-02-201), PIP study 4 (NS-089/NCNP-02-202) and PIP study 5 (NS 089/NCNP-02-301) are part of an extrapolation plan covering the paediatric population from 6 months to less than 4 years of age.
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:	30/06/2039
Deferral of one or more studies contained in the paediatric investigation plan:	Yes