

MHRA
10 South Colonnade
Canary Wharf
London
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-101389-PIP01-24-M01) and to the deferral

MHRA-101389-PIP01-24-M02

Scope of the Application

Active Substance(s)

OMAVELOXOLONE

Condition(s)

Treatment of Friedreich's ataxia

Pharmaceutical Form(s)

Capsule, hard Age appropriate oral formulation

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Biogen Netherlands B.V

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Biogen Netherlands B.V submitted to the licensing authority on 13/02/2026 09:08 GMT an application for a Modification

The procedure started on 03/03/2026 17:15 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-101389-PIP01-24-M02

Of 19/05/2026 09:08 BST

On the adopted decision for OMAVELOXOLONE (MHRA-101389-PIP01-24-M02) 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 OMAVELOXOLONE, Capsule, hard Age appropriate oral formulation , ORAL USE .

This decision is addressed to Biogen Netherlands B.V, Prins Mauritslaan 13, Badhoevedorp, NETHERLANDS, 1170

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Friedreich's ataxia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Capsule, hard Age appropriate oral formulation Route(s) of administration: ORAL USE GASTRIC 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 Friedreich's ataxia

2.2 Indication(s) targeted by the PIP:

Treatment of Friedreich's ataxia

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):

Capsule, hard Age appropriate oral formulation

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 (PED-FORM-DEV-1) Development of an age appropriate oral capsule formulation.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	4	Study 2 (408-C-1402 [Part 1]) Double-blind, randomised, placebo-controlled, dose ranging trial to evaluate pharmacokinetics, pharmacodynamics, safety and efficacy of omaveloxolone in adolescents from 16 years to less than 18 years of age (and adults) with genetically confirmed Friedreich's ataxia. Study 3 (408-C-1402 [Part 2]) Double-blind, randomised, placebo-controlled trial to evaluate safety and efficacy of omaveloxolone in adolescents from 16 years to less than 18 years of age (and adults) with genetically confirmed Friedreich's ataxia. Study 4 (408-C-2001) Open-label, single-arm trial to identify the appropriate dose and evaluate pharmacokinetics, pharmacodynamics, safety activity and acceptability/palatability of omaveloxolone in children from 2 years to less than 16 years of age with genetically confirmed Friedreich's ataxia. Study 6 (296FA301) Added during

		procedure MHRA-101389-PIP01-24-M01 Randomised, double-blind, placebo-controlled study to evaluate the efficacy, safety, PK, and PD of omaveloxolone in children from 2 years to less than 16 years of age with genetically confirmed Friedreich's ataxia, with a long term extension
Extrapolation, Modeling & Simulation Studies	1	Study 5 Modelling and simulation study to support the use of omaveloxolone in children from 2 years to less than 16 years of age with genetically confirmed Friedreich's ataxia.
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:	31/07/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes