MHRA
10 South Colonnade
Canary Wharf
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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-101389-PIP01-24

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 GASTRIC 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 22/03/2024 13:46 GMT an application for a Paediatric Investigation Plan

The procedure started on 27/03/2024 17:29 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-101389-PIP01-24

Of 21/05/2024 07:48 BST

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

This decision is addressed to Biogen Netherlands B.V., Prins Mauritslaan 13, Badhoevedorp, NETHERLANDS, 1171LP

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	3	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, historical controlled, two part trial to identify the appropriate dose (part one) 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.
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:	30/06/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes