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-101550-PIP01-24

Scope of the Application

Active Substance(s)

Troriluzole Hydrochloride

Condition(s)

Treatment of hereditary spinocerebellar ataxia

Pharmaceutical Form(s)

Capsule, hard Age appropriate oral dosage formulation

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Biohaven Bioscience Ireland Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Biohaven Bioscience Ireland Limited submitted to the licensing authority on 20/12/2024 19:00 GMT an application for a Paediatric Investigation Plan

The procedure started on 13/01/2025 15:12 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-101550-PIP01-24

Of 22/04/2025 15:51 BST

On the adopted decision for Troriluzole Hydrochloride (MHRA-101550-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 Troriluzole Hydrochloride, Capsule, hard Age appropriate oral dosage formulation , ORAL USE .

This decision is addressed to Biohaven Bioscience Ireland Limited, 6th Floor, South Bank House, Barrow Street, Dublin 4, IRELAND, D04 TR29

ANNEX I

1. Waiver

1.1 Condition:

Treatment of hereditary spinocerebellar ataxia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 11 years of age Pharmaceutical form(s): Capsule, hard Age appropriate oral dosage formulation Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of hereditary spinocerebellar ataxia

2.2 Indication(s) targeted by the PIP:

Treatment of spinocerebellar ataxia (SCA) in children and adolescents from 11 years to less than 18 years of age.

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

The paediatric population from 11 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Capsule, hard Age appropriate oral dosage formulation

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate formulation (e.g. mini-tablets, granules, solution or suspension) suitable to paediatric patients unable to swallow the available hard capsules.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 Multicentre, open-label trial to evaluate the pharmacokinetics (PK), safety and activity of troriluzole in children and adolescents from 11 years to less than 18 years of age with hereditary spinocerebellar ataxia and to provide PK/pharmacodynamic (PD) data to support the extrapolation of efficacy from adults.
Extrapolation, Modeling & Simulation Studies	2	Study 3 Modelling and simulation study and exposure-response study to determine the dose to be used in the paediatric population from 11 years to less than 18 years of age with hereditary spinocerebellar ataxia and to support the extrapolation of efficacy from adults. Extrapolation Plan Studies 2 and Study 3 are part of the extrapolation plan of efficacy data from adults to paediatric patients from 11 years to less than 18 years of age with hereditary spinocerebellar 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:	No
Date of completion of the paediatric investigation plan:	31/08/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes