MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
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-101171-PIP01-23) and to the deferral

MHRA-101171-PIP01-23-M01

Scope of the Application

Active Substance(s)

fosigotifator sodium tromethamine

Condition(s)

Treatment of vanishing white matter disease

Pharmaceutical Form(s)

Granules; Age appropriate dosage form

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

AbbVie Ltd.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, AbbVie Ltd. submitted to the licensing authority on 09/08/2024 14:50 BST an application for a Modification

The procedure started on 06/09/2024 12:18 BST

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-101171-PIP01-23-M01

Of 08/11/2024 07:53 GMT

On the adopted decision for fosigotifator sodium tromethamine (MHRA-101171-PIP01-23-M01) 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 fosigotifator sodium tromethamine, Granules; Age appropriate dosage form , ORAL USE .

This decision is addressed to AbbVie Ltd., AbbVie House, Vanwall Business Park, Vanwall Road, Maidenhead, UNITED KINGDOM, SL6 4UB

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of vanishing white matter disease

2.2 Indication(s) targeted by the PIP:

Treatment of vanishing white matter disease

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):

Granules Age appropriate dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of granule formulation for paediatric use in children from 6 months of age. Study 2 Feasibility assessment and formulation development of an age-appropriate formulation of ABBV-CLS-7262 for use in infants from birth to less than 6 months of age.
Non-Clinical Studies	1	Study 3 (TA21-009) Definitive juvenile toxicity study to support the evaluation of the use of ABBV-CLS-7262 in the paediatric population from birth to less than 18 years of age.
Clinical Studies	3	Study 4 (M23-523) Open-label, uncontrolled trial to evaluate pharmacokinetics, safety, activity and acceptability/ palatability of ABBV-CLS-7262 in children from 6 months to less than 18 years of age (and adults) with vanishing white matter (VWM) disease. Study 5 (M20-474) Double-blind, randomised, placebo controlled trial to evaluate pharmacokinetics, safety and efficacy of ABBV-CLS-7262 in children from 6 years to less than 18 years of age (and adults) with vanishing white matter (VWM) disease. Study 6 (M20-475) Open-label, historical controlled trial to evaluate pharmacokinetics, safety, efficacy, acceptability/ palatability of ABBV-CLS-7262 in children from birth to less than 6 years of age

		with vanishing white matter (VWM) disease.
Extrapolation, Modeling & Simulation Studies	1	Study 7 Modelling and simulation physiologically based pharmacokinetic (PBPK) study, to evaluate the use of the product in the treatment of vanishing white matter disease in children from birth to less than 18 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:	30/09/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes