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-100128-PIP01-21-M01

Scope of the Application

Active Substance(s)

bis-choline tetrathiomolybdate

Condition(s)

Treatment of Wilson disease

Pharmaceutical Form(s)

Coated tablet; Age-appropriate oral solid dosage form

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

Alexion Europe SAS

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Alexion Europe SAS submitted to the licensing authority on 04/06/2021 16:06 BST an application for a Modification

The procedure started on 27/10/2021 14:57 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-100128-PIP01-21-M01

Of 23/11/2021 15:15 GMT

On the adopted decision for bis-choline tetrathiomolybdate (MHRA-100128-PIP01-21-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 bis-choline tetrathiomolybdate, Coated tablet; Age-appropriate oral solid dosage form , Oral use .

This decision is addressed to Alexion Europe SAS, 103-105 Rue Anatole France, Levallois Perret, France, 92300

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Wilson disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 3 years of age Pharmaceutical form(s): Coated tablet; Age-appropriate oral solid dosage form 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 over existing treatments

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Wilson disease

2.2 Indication(s) targeted by the PIP:

Treatment of Wilson disease in the paediatric population from 3 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 3 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Coated tablet; Age-appropriate oral solid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1:_ Development of an age appropriate oral solid dosage form.
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	Study 2 (WTX101-301):_Randomised, rater-blinded efficacy and safety study of ALXN1840 compared with standard of care in adolescents 12 years to less than 18 years (and adults) with Wilson disease; Study 3 (ALXN1840-WD-302):_Open-label, randomised, controlled, efficacy and safety study of ALXN1840 compared with standard of care in children from 3 years to less than 18 years with Wilson disease.
Extrapolation, Modeling & Simulation Studies	1	Study 4 (ALXN1840-WD-303):_ Extrapolation study to evaluate the efficacy of ALXN1840 in paediatric Wilson disease patients from 3 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:	30/11/2024
Deferral of one or more studies contained in the paediatric investigation plan:	Yes