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-100544-PIP01-22-M01) and to the deferral

MHRA-100544-PIP01-22-M02

Scope of the Application

Active Substance(s)

Sebetralstat

Condition(s)

Treatment of Hereditary Angioedema

Pharmaceutical Form(s)

FILM COATED TABLET; AGE-APPROPRIATE ORAL SOLID DOSAGE FORM

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

KalVista Pharmaceuticals Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, KalVista Pharmaceuticals Ltd submitted to the licensing authority on 28/06/2023 14:28 BST an application for a Modification

The procedure started on 09/11/2023 07:33 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-100544-PIP01-22-M02

Of 29/11/2023 16:27 GMT

On the adopted decision for Sebetralstat (MHRA-100544-PIP01-22-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 Sebetralstat, FILM COATED TABLET; AGE-APPROPRIATE ORAL SOLID DOSAGE FORM , ORAL USE .

This decision is addressed to KalVista Pharmaceuticals Ltd, Porton Science Park, Bybrook Road, Porton Down, Salisbury, UNITED KINGDOM, SP4 0BF

ANNEX I

1. Waiver

1.1 Condition:

Treatment of hereditary angioedema The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): AGE-APPROPRIATE ORAL SOLID DOSAGE FORM. FILM-COATED TABLET Route(s) of administration: ORAL 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 hereditary angioedema

2.2 Indication(s) targeted by the PIP:

Treatment of hereditary angioedema in adolescents and children from 2 years of age.

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):

AGE-APPROPRIATE ORAL SOLID DOSAGE FORM. FILM-COATED TABLET

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate oral solid dosage form for children from 2 years to less than 12 years of age.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 2 (KVD900-301) Double blind, randomised, placebo-controlled, 3-way crossover trial to evaluate the efficacy and safety of two dose levels of KVD900 in adolescents from 12 years to less than 18 years of age (and adults) with hereditary angioedema (HAE). Study 3 (KVD900-302) Open-label extension trial to evaluate long-term safety of KVD900 in adolescents from 12 years to less than 18 years of age (and adults) with hereditary angioedema (HAE), including a PK sub-trial in adolescents. Study 4 (KVD900-303) Open-label trial to evaluate PK and safety of KVD900 in children from 2 years to less than 12 years of age with hereditary angioedema (HAE).
Extrapolation, Modeling & Simulation Studies	2	Study 5 (3244-007) Population PK model. Study 6 (KVD900 Extrapolation) Analysis of existing in house and literature data on KVD900 on hereditary angioedema (HAE) in order to provide efficacy assumptions

		in the paediatric population from 2 years to less than 12 years based on extrapolation from adults and adolescents.
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:	31/07/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes