

**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-M02)  
MHRA-100544-PIP01-22-M03

### **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 (Orodispersible tablet)

#### **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 10/12/2024 13:59 GMT an application for a Modification

The procedure started on 13/01/2025 14:52 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

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-M03

Of 02/04/2025 09:43 BST

On the adopted decision for Sebetralstat (MHRA-100544-PIP01-22-M03) 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 (Orodispersible tablet) , 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 (Orodispersible tablet) 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 (Orodispersible tablet) 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.
<b>Other Studies</b>	0	Not applicable.
<b>Other Measures</b>	0	Not applicable.

### 3. Follow-up, completion and deferral of a PIP:

<b>Concerns on potential long term safety and efficacy issues in relation to paediatric use:</b>	No
<b>Date of completion of the paediatric investigation plan:</b>	31/07/2028
<b>Deferral of one or more studies contained in the paediatric investigation plan:</b>	Yes