MHRA
10 South Colonnade
Canary Wharf
London
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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-101731-PIP01-24

Scope of the Application

Active Substance(s)

pridopidine hydrochloride

Condition(s)

Treatment of Huntington disease (HD)

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Prilenia Therapeutics B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Prilenia Therapeutics B.V. submitted to the licensing authority on 20/12/2024 11:40 GMT an application for a Paediatric Investigation Plan

The procedure started on 13/01/2025 15:17 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-101731-PIP01-24

Of 16/04/2025 08:53 BST

On the adopted decision for pridopidine hydrochloride (MHRA-101731-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 pridopidine hydrochloride, Capsule, hard , ORAL USE .

This decision is addressed to Prilenia Therapeutics B.V., Gooimeer 2/35, DC Naarden, NETHERLANDS, 1411

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Huntington disease (HD) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Capsule, hard 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 Huntington disease (HD)

2.2 Indication(s) targeted by the PIP:

Treatment of juvenile-onset HD (JHD)

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):

Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of age-appropriate oral formulation for the paediatric population.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 (PL101-JHDPK201) Open label, uncontrolled trial to evaluate pharmacokinetics (PK) and safety of pridopidine in children with Huntington's disease to contribute to modelling of the PK in children from 10 years to less than 18 years of age. Study 3 (PL101-JHD202) Double-blind, placebo controlled trial to evaluate safety, efficacy and pharmacokinetics (PK) of pridopidine in paediatric patients from 2 years to less than 18 years of age with Huntington's disease.
Extrapolation, Modeling & Simulation Studies	1	Study 4 Modelling and simulation population pharmacokinetic (PK) study, to evaluate the use of the product in the treatment of juvenile Huntington's disease in children from 2 years to less than 18 years of age with Huntington's disease.
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:	31/03/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes