MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
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-100075-PIP02-24

Scope of the Application

Active Substance(s)

leniolisib phosphate

Condition(s)

Treatment of common variable immunodeficiency

Pharmaceutical Form(s)

Film coated tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Pharming Technologies B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Pharming Technologies B.V. submitted to the licensing authority on 06/09/2024 23:11 BST an application for a Paediatric Investigation Plan

The procedure started on 25/10/2024 13:31 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 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-100075-PIP02-24

Of 14/08/2025 11:29 BST

On the adopted decision for leniolisib phosphate (MHRA-100075-PIP02-24) 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 leniolisib phosphate, Film coated tablet , ORAL USE .

This decision is addressed to Pharming Technologies B.V., Darwinweg 24, Leiden, NETHERLANDS, 2333 CR

ANNEX I

1. Waiver

1.1 Condition:

Treatment of common variable immunodeficiency The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 4 years of age Pharmaceutical form(s): Film coated tablet Route(s) of administration: ORAL USE Reason for granting waiver: For the paediatric population from birth to less than 1 year of age • the grounds are that the specific medicinal product is likely to be unsafe. For the paediatric population from 1 year to less than 4 years of age • the grounds are 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 common variable immunodeficiency

2.2 Indication(s) targeted by the PIP:

Treatment of common variable immunodeficiency

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 4 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film coated tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	4	Study 1 (LE 8201) Open-label, single arm, dose finding trial to evaluate the pharmacokinetics, safety, tolerability and activity of leniolisib in adolescents from 12 years to less than 18 years of age (and adults) with common variable immunodeficiency (CVID). Study 2 Open-label, single-arm extension trial to evaluate the long-term safety, tolerability, and activity of leniolisib in male and female patients, from 4 years to less than 18 years of age (and adults) who participated in PIP studies 1 (LE 8201), 3 or 4. Study 3 Randomised control trial to evaluate efficacy and safety of leniolisib in male and female adolescent patients from 12 years to less than 18 years of age (and adults) with CVID and immune dysregulation. Study 4 Open label, single arm trial to evaluate safety, tolerability, efficacy, and pharmacokinetics of leniolisib in children from 4 years to less than 12 years of age with CVID.
Extrapolation, Modeling & Simulation Studies	1	Study 5 Modelling and simulation study to support the use of leniolisib

		in paediatric patients from 4 years to less than 18 years of age with CVID.
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:	Yes
Date of completion of the paediatric investigation plan:	31/12/2033
Deferral of one or more studies contained in the paediatric investigation plan:	Yes