EMA/628925/2022

European Medicines Agency decision

P/0259/2022

of 15 July 2022

on the acceptance of a modification of an agreed paediatric investigation plan for leniolisib phosphate (EMEA-002989-PIP01-21-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0556/2021 issued on 31 December 2021,

Having regard to the application submitted by Pharming Technologies B.V. on 25 April 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 24 June 2022, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for leniolisib phosphate, capsule, hard, film-coated tablet, granules, oral use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

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

EMA/PDCO/245065/2022
Amsterdam, 24 June 2022

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-002989-PIP01-21-M01

Scope of the application

Active substance(s):

Leniolisib phosphate

Condition(s):

Treatment of activated phosphoinositide 3-kinase delta syndrome

Pharmaceutical form(s):

Capsule, hard

Film-coated tablet

Granules

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Pharming Technologies B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pharming Technologies B.V. submitted to the European Medicines Agency on 25 April 2022 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0556/2021 issued on 31 December 2021.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 23 May 2022.

Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

Annex I

The subset of the paediatric population and condition covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of activated phosphoinositide 3-kinase delta syndrome

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- capsule, hard, film-coated tablet, granules; oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of activated phosphoinositide 3-kinase delta syndrome (APDS)

2.1.1. Indication(s) targeted by the PIP

Treatment of activated phosphoinositide 3-kinase delta (δ) syndrome (APDS)

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 year to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Capsule, hard, film-coated tablet, granules

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1 (PIPLform)** Development of an oral age-appropriate formulation
Non-clinical studies	Not applicable.
Clinical studies	**Study 2 (CCDZ173X2201 - part II)** Double-blind, randomised, assessor-blind, placebo-controlled trial to evaluate safety and efficacy of leniolisib compared to placebo in children from 12 years to less than 18 years of age (and adults) with documented activated phosphoinositide 3-kinase delta syndrome (APDS).

Area	Description
	Study 3 (CCDZ173X2201 - part I) Open label, single arm, dose-finding trial to evaluate pharmacokinetics, safety, activity of leniolisib in children from 12 years to less than 18 years of age (and adults) with documented APDS. **Study 4 (CCDZ173X2201E1)** Open-label, long term safety and tolerability trial of leniolisib in children from 12 years to less than 18 years of age (and adults) who participated in studies 2 or 3. **Study 5 (PIPCL1)** Open-label, single arm, two part trial to evaluate pharmacokinetics, safety and efficacy of leniolisib in children from 4 years to less than 12 years of age with documented APDS. **Study 6 (PIPCL2)** Open-label, single arm, two-part trial to evaluate pharmacokinetics, safety and efficacy of leniolisib in children from 1 year to less than 7 years of age with documented APDS.
Extrapolation, modelling and simulation studies	**Study 7 (PIPLMS)** Modelling and simulation study to support the use of leniolisib from 1 year to less than 18 years of age with documented APDS.
Other studies	Not applicable.
Other measures	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By September 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes