EMA/413895/2024

European Medicines Agency decision

P/0330/2024

of 13 September 2024

on the acceptance of a modification of an agreed paediatric investigation plan for leriglitazone (EMEA-002106-PIP01-16-M03) 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/0152/2020 issued on 18 April 2020, the decision P/0412/2021 issued on 29 October 2021 and the decision P/0115/2022 issued on 13 April 2022,

Having regard to the application submitted by Minoryx Therapeutics S.L. on 24 May 2024 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 26 July 2024, 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 and to the deferral.

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

[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 leriglitazone, oral suspension, oral use, including changes to the deferral, 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 Minoryx Therapeutics S.L., ecnoCampus Mataró-Maresme Av Ernest Lluch 32 TCM3, 08302 – Mataró, Spain.

EMA/PDCO/279464/2024
Amsterdam, 26 July 2024

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

EMEA-002106-PIP01-16-M03

Scope of the application

Active substance(s):

Leriglitazone

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of adrenoleukodystrophy

Pharmaceutical form(s):

Oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Minoryx Therapeutics S.L.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Minoryx Therapeutics S.L. submitted to the European Medicines Agency on 24 May 2024 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/0152/2020 issued on 18 April 2020, the decision P/0412/2021 issued on 29 October 2021 and the decision P/0115/2022 issued on 13 April 2022.

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

The procedure started on 8 July 2024.

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 and to the deferral 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(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of adrenoleukodystrophy

The waiver applies to:

- girls from birth to less than 18 years of age, and boys from birth to less than 2 years of age;
- oral suspension, oral use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of adrenoleukodystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of adrenoleukodystrophy

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

Boys from 2 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Oral suspension

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1** Open-label PK, efficacy and safety study to evaluate whether leriglitazone can arrest disease progression of cerebral adrenoleukodystrophy (cALD) if administered prior to hematopoietic stem-cell transplantation (HSCT), as determined by serial clinical and magnetic resonance imaging (MRI) investigations in male paediatric patients from 2 to less than 12 years of age (MT-2-02).

	Study 2 Safety analysis from double-blind, randomised, parallel-group, placebo-controlled study to evaluate the effect of 48 weeks of treatment with leriglitazone on biochemical, imaging, neurophysiological and clinical markers in paediatric patients from 12 to less than 18 years of age (and adults) with Friedreich's Ataxia (MT-2-03).
Extrapolation, modelling and simulation studies	**Study 3** Population pharmacokinetic analysis to explore the influence of covariates, especially weight and age on the pharmacokinetics of leriglitazone and to predict the dose (range) that meets the target AUC in adolescents. Preceding modelling: Physiologically based pharmacokinetic (PBPK) model for leriglitazone that incorporates CYP3A4 and CYP2C8-mediated metabolism as well as biliary clearance derived from in-vitro data to derive an appropriate starting dose for paediatric patients. **Study 4** Disease progression/exposure-response analysis (i.e. PKPD modelling) to confirm/establish the effective AUC target in adolescents.
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 July 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.