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 and to the deferral.

MHRA-102685-PIP01-26-M01

Scope of the Application

Active Substance(s)

leriglitazone

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 the Decision

Pursuant to the Human Medicines Regulations 2012, Minoryx Therapeutics S.L. submitted to the licensing authority on 05/10/2026 12:47 BST an application for a Modification

The procedure started on 12/10/2026 15:41 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 accept change(s) to the agreed paediatric investigation plan and to the deferral.

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-102685-PIP01-26-M01

Of 16/10/2026 10:48 BST

On the adopted decision for leriglitazone (MHRA-102685-PIP01-26-M01) 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 leriglitazone, Oral suspension , ORAL USE .

This decision is addressed to Minoryx Therapeutics S.L., AVENIDA ERNEST LLUCH 32, TCM3, MATARO , SPAIN, 08302

ANNEX I

1. Waiver

1.1 Condition:

Treatment of adrenoleukodystrophy. The waiver applies / applied to: Paediatric Subset(s): Girls from birth to less than 18 years of age, and boys from birth to less than 2 years of age. Pharmaceutical form(s): Oral suspension Route(s) of administration: ORAL USE Reason for granting waiver: 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(s):

Treatment of adrenoleukodystrophy.

2.2 Indication(s) targeted by the PIP:

Treatment of adrenoleukodystrophy.

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

Boys from 2 years to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Oral suspension

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	Study 1 (MT-2-02) 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. Study 2 (MT-2-03) 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.
Extrapolation, Modeling & Simulation Studies	2	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	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:	31/07/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes