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-101894-PIP01-25

Scope of the Application

Active Substance(s)

nomlabofusp

Condition(s)

Treatment of Friedreich’s ataxia

Pharmaceutical Form(s)

Powder for solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Larimar Therapeutics, Inc.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Larimar Therapeutics, Inc. submitted to the licensing authority on 16/04/2025 01:53 BST an application for a Paediatric Investigation Plan

The procedure started on 29/06/2025 17:21 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-101894-PIP01-25

Of 05/03/2026 08:25 GMT

On the adopted decision for nomlabofusp (MHRA-101894-PIP01-25) 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 nomlabofusp, Powder for solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Larimar Therapeutics, Inc., Three Bala Plaza East, Suite 506, Bala Cynwyd, UNITED STATES OF AMERICA, 19004

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Friedreich's ataxia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Powder for solution for injection Route(s) of administration: SUBCUTANEOUS 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 Friedreich's ataxia

2.2 Indication(s) targeted by the PIP:

Treatment of Friedreich's ataxia

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):

Powder for solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	1	Study 1 (Tox-1601-18) Toxicity study in juvenile rats.
Clinical Studies	3	Study 2 (CLIN-1601-103) Double-blind, randomised, placebo-controlled study to evaluate safety, tolerability, pharmacokinetics and pharmacodynamics of subcutaneous nomlabofusp in adolescents from 12 years to less than 18 years of age with Friedreich's ataxia. Study 3 (CLIN-1601-201) Open-label study to optimise exposure-matching doses and to evaluate the safety and tolerability of long-term subcutaneous administration in children and adolescents from 2 years to less than 18 years of age (and adults) with Friedreich's ataxia. Study 4 (CLIN-1601-301) Double-blind, randomised, placebo-controlled trial to evaluate the efficacy and safety of subcutaneous nomlabofusp in children and adolescents from 2 years to less than 18 years of age (and adults) with Friedreich's ataxia.
Extrapolation, Modeling & Simulation Studies	2	Study 5 (PK-1601-10 & PK-1601-12) Modelling and simulation analysis (pop-PK model) to evaluate the use of nomlabofusp and to support dose selection in the paediatric population from 2

		years to less than 18 years of age with Friedreich's ataxia. Study 6 (PK-1601-11) Modelling and simulation analysis (pop-PK model) to evaluate the use of nomlabofusp in the paediatric population from 2 years to less than 18 years of age with Friedreich's ataxia.
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:	29/02/2028
Deferral of one or more studies contained in the paediatric investigation plan:	No