

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

MHRA-101824-PIP01-25

Scope of the Application

Active Substance(s)

AAV8-RK-AIPL1

Condition(s)

Treatment of Leber congenital amaurosis

Pharmaceutical Form(s)

Suspension for injection

Route(s) of Administration

INTRAOCULAR USE

Name / Corporate name of the PIP applicant

MeiraGTx UK II Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, MeiraGTx UK II Ltd submitted to the licensing authority on 21/03/2025 15:22 GMT an application for a Paediatric Investigation Plan

The procedure started on 17/04/2025 21:50 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

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-101824-PIP01-25

Of 11/11/2025 09:24 GMT

On the adopted decision for AAV8-RK-AIPL1 (MHRA-101824-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 AAV8-RK-AIPL1, Suspension for injection , INTRAOCULAR USE .

This decision is addressed to MeiraGTx UK II Ltd, 92 Britannia Walk, London, UNITED KINGDOM, N1 7NQ

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Leber congenital amaurosis

2.2 Indication(s) targeted by the PIP:

Treatment of paediatric patients with Leber congenital amaurosis (LCA) owing to genetic deficiency of aryl-hydrocarbon-interacting protein-like 1 (AIPL1).

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

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Suspension for injection

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 Open-label, single arm study to evaluate the safety and activity of AAV8-RK-AIPL1 in children from 1 year to less than 12 years of age with AIPL1-associated Leber congenital amaurosis. Study 2 Open label, single arm non-randomised, observational study to evaluate long term efficacy and safety in children from birth to less than 18 years of age diagnosed with Leber congenital amaurosis, who are scheduled to have or have recently been treated with AAV8-RK-AIPL1.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
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:	No
Date of completion of the paediatric investigation plan:	31/12/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes

