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-100348-PIP01-21-M01

Scope of the Application

Active Substance(s)

botaretigene sparoparvovec

Condition(s)

Treatment of retinitis pigmentosa

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

OPHTHALMIC USE

Name / Corporate name of the PIP applicant

Janssen-Cilag Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Janssen-Cilag Ltd submitted to the licensing authority on 04/12/2023 21:44 GMT an application for a Modification

The procedure started on 05/03/2024 15:17 GMT

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-100348-PIP01-21-M01

Of 26/03/2024 15:45 GMT

On the adopted decision for botaretigene sparoparvovec (MHRA-100348-PIP01-21-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 botaretigene sparoparvovec , Solution for injection , OPHTHALMIC USE .

This decision is addressed to Janssen-Cilag Ltd, 50-100 Holmers Farm Way , High Wycombe, UNITED KINGDOM, HP12 4EG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of retinitis pigmentosa The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 5 years of age. Pharmaceutical form(s): Solution for injection Route(s) of administration: OPHTHALMIC USE Reason for granting waiver: On the grounds the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of retinitis pigmentosa

2.2 Indication(s) targeted by the PIP:

Treatment of GTPase regulator (RPGR) mutation-associated X-linked retinitis pigmentosa.

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

The paediatric population from 5 years to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Solution 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	5	Study 1 (MGT009) Open-label, multi-centre dose escalation in adults, followed by dose confirmation in children 5 years to less than 18 years of age to assess the safety of the recombinant adeno-associated virus vector AAV5-hRKp.RPGR for gene therapy of individuals with X-linked Retinitis Pigmentosa (XLRP) owing to defects in Retinitis Pigmentosa GTPase Regulator (RPGR). Study 2 (MTG010) Long term follow-up study of participants of study MGT009 to assess the longer-term safety and efficacy of AAV5-hRKp.RPGR. Study 3 (MTG011) Observational natural history study in children and adolescents 5 years to less than 18 years with X-linked Retinal Dystrophy Associated with Mutations in Retinitis Pigmentosa GTPase Regulator (RPGR). Study 4 (MGT-RPGR-021) Randomised, controlled, 52-week efficacy and safety study of bilateral subretinal treatment with AAV5-hRKp.RPGR gene therapy in children and adolescents 8 years to less than 18

		years of age (and adults) with RPGR-XLRP. Study 5 (MGT-RPGR-022) Long-term safety follow-up study of paediatric (and adult) participants previously enrolled in study MGT-RPGR-021 to assess the long-term safety of AAV5-hRKp.RPGR.
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:	Yes
Date of completion of the paediatric investigation plan:	31/12/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes