EMA/328869/2024

European Medicines Agency decision

P/0269/2024

of 17 July 2024

on the acceptance of a modification of an agreed paediatric investigation plan for botaretigene sparoparvovec (EMEA-002827-PIP01-20-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/0125/2021 issued on 17 March 2021 and the decision P/0389/2023 issued on 8 September 2023,

Having regard to the application submitted by Janssen-Cilag International NV on 22 February 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 31 May 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.

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

[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 botaretigene sparoparvovec, solution for injection, ophthalmic use, 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 Janssen-Cilag International NV Turnhoutseweg 30, Turnhoutseweg 30, B-2340 – Beerse, Belgium.

EMA/PDCO/107434/2024 Corr[i]
Amsterdam, 31 May 2024

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

EMEA-002827-PIP01-20-M03

Scope of the application

Active substance(s):

Botaretigene sparoparvovec

Invented name and authorisation status:

See Annex II

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 International NV

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Janssen-Cilag International NV submitted to the European Medicines Agency on 22 February 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/0125/2021 issued on 17 March 2021 and the decision P/0389/2023 issued on 8 September 2023.

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

The procedure started on 2 April 2024.

Scope of the modification

Some measures 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 in the scope set out in the Annex I of this opinion.
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 annexes 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 retinitis pigmentosa

The waiver applies to:

- the paediatric population from birth to less than 5 years of age;
- solution for injection, ophthalmic use;
- 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:

Treatment of retinitis pigmentosa

2.1.1. Indication(s) targeted by the PIP

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

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

From 5 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**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)** Randomized, controlled, 52-week efficacy and safety study of bilateral subretinal treatment with AAV5-hRKp.RPGR gene therapy in children and adolescents from 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 adult and paediatric participants who participated in study MGT-RPGR-021 to assess the long-term safety of AAV5-hRKp.RPGR
Extrapolation, modelling and simulation studies	Not applicable
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 December 2029
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.

[i] Corr 18 July 2024