

EMA/97583/2021

European Medicines Agency decision P/0125/2021

of 17 March 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV5-hRKp.RPGR), (EMEA-002827-PIP01-20) 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 726/2004¹,

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency²,

Having regard to the application submitted by MeiraGTx UK II Ltd on 20 April 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 29 January 2021, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV5-hRKp.RPGR), solution for injection, ophthalmic use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV5-hRKp.RPGR), solution for injection, ophthalmic use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV5-hRKp.RPGR), solution for injection, ophthalmic use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to MeiraGTx UK II Ltd, 92 Britannia Walk, N1 7NQ – London, United Kingdom.

EMA/PDCO/597588/2020
Amsterdam, 29 January 2021

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMA-002827-PIP01-20

Scope of the application

Active substance(s):

Linear single strand of deoxyribonucleic acid (encoding human retinitis pigmentosa GTPase regulator [RPGR]) packaged in a recombinant adeno-associated virus protein capsid of serotype 5 (AAV5-hRKp.RPGR)

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:

MeiraGTx UK II Ltd

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, MeiraGTx UK II Ltd submitted for agreement to the European Medicines Agency on 20 April 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 26 May 2020.

Supplementary information was provided by the applicant on 22 October 2020. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
 - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
 - to grant a deferral in accordance with Article 21 of said Regulation;
 - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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 annex 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 3 years;
- 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 3 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	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) Randomized, controlled, 52-week efficacy and safety study of bilateral subretinal treatment with AAV5-hRKp.RPGR gene therapy in children and adolescents 3 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	0	Not applicable
Other studies	0	Not applicable
Other measures	0	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 September 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes