EMA/413764/2020

European Medicines Agency decision

P/0309/2020

of 14 August 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for resamirigene bilparvovec (EMEA-002571-PIP01-19) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Audentes Therapeutics, Inc. on 16 May 2019 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 June 2020, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[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 resamirigene bilparvovec, solution for infusion, intravenous 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 resamirigene bilparvovec, solution for infusion, intravenous 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

This decision is addressed to Audentes Therapeutics, Inc., 600 California Street, 17th Floor, 94108 - San Francisco, California, United States.

EMA/PDCO/207104/2020
Amsterdam, 26 June 2020

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

EMEA-002571-PIP01-19

Scope of the application

Active substance(s):

Resamirigene bilparvovec

Condition(s):

Treatment of X-linked myotubular myopathy

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Audentes Therapeutics, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Audentes Therapeutics, Inc. submitted for agreement to the European Medicines Agency on 16 May 2019 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation .

The procedure started on 25 June 2019.

Supplementary information was provided by the applicant on 24 March 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.

   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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of X-linked myotubular myopathy

2.1.1. Indication(s) targeted by the PIP

Treatment of X-linked myotubular myopathy

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

From birth to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	3	**Study 1:** Randomised, open-label, ascending-dose, delayed-treatment concurrent control trial to evaluate the safety and efficacy of resamirigene bilparvovec (hereafter referred to as AT132) in male patients with X-linked myotubular myopathy (XLMTM) who are less than 5 years of age at Day 1, or who participated in Study 3, and who require mechanical ventilatory support (ASPIRO) **Study 2:** Open-label, single arm, uncontrolled trial to evaluate the safety and efficacy of AT132 in male patients with XLMTM who are from 7 to less than 18 years of age at Day 1, or who participated in Study 3 (LIBERI) **Study 3:** Prospective, non-interventional natural history study in subjects less than 4 years of age with XLMTM (INCEPTUS)
Extrapolation, modelling and simulation studies	0	Not applicable.

Other studies	1	**Study 4:** Retrospective medical chart review of patients with XLMTM (RECENSUS)
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 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes