EMA/624102/2021

European Medicines Agency decision

P/0504/2021

of 3 December 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA (ATL1102), (EMEA-002981-PIP01-21) 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 Antisense Therapeutics Limited on 15 February 2021 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 15 October 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 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA (ATL1102), solution for injection, subcutaneous 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 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA (ATL1102), solution for injection, subcutaneous 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 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA (ATL1102), solution for injection, subcutaneous 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 Antisense Therapeutics Limited, Level 1, 14 Wallace Avenue Toorak, 3142 – Victoria, Australia.

EMA/PDCO/435036/2021
Amsterdam, 15 October 2021

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

EMEA-002981-PIP01-21

Scope of the application

Active substance(s):

2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA (ATL1102)

Condition(s):

Treatment of Duchenne muscular dystrophy

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Antisense Therapeutics Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Antisense Therapeutics Limited submitted for agreement to the European Medicines Agency on 15 February 2021 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 23 March 2021.

Supplementary information was provided by the applicant on 12 July 2021. 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 Duchenne muscular dystrophy

The waiver applies to:

- the paediatric population from birth to less than 2 years;
- Solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of Duchenne muscular dystrophy.

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne muscular dystrophy.

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

From 2 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	4	Study 1 Randomised, double-blind, placebo-controlled study to assess the efficacy, safety, and pharmacokinetic profile of two dose levels of ATL1102 administered by subcutaneous injection in non-ambulatory paediatric patients from 10 years to less than 18 years of age with Duchenne Muscular Dystrophy (DMD) (1102-DMD-CT03)

		Study 2 An open label extension study to assess the long-term profile of ATL1102 administered by subcutaneous injection in non-ambulatory paediatric patients from 10 years to less than 18 years of age with Duchenne Muscular Dystrophy (DMD) (and young adults) (1102-DMD-CT04). Study 3 Randomised, double-blind, placebo-controlled study to assess the efficacy, safety, and pharmacokinetic profile of ATL1102 administered by subcutaneous injection in ambulatory paediatric patients from 5 years to less than 11 years of age with Duchenne Muscular Dystrophy (DMD) (1102-DMD-CT05) Study 4 Single arm, open label study to assess the safety, and pharmacokinetic profile of ATL1102 administered by subcutaneous injection in paediatric patients from 2 years to less than 5 years of age with Duchenne Muscular Dystrophy (DMD) (1102-DMD-CT08).
Extrapolation, modelling and simulation studies	1	Study 5 Population pharmacokinetic (PK) model and exposure-response correlation analysis developed based on all data including adolescents and children with DMD, and (healthy adult volunteers) to define the body weight and age effect to predict the dose regimen for Study 3 and 4 and analysis of exposure and response for boys aged from 2 years to less than 5 years (1102-DMD-CT06).
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 May 2030
Deferral for one or more measures contained in the paediatric investigation plan:	Yes