MHRA
10 South Colonnade
Canary Wharf
London
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100060-PIP01-21

Scope of the Application

Active Substance(s)

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

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, Australia

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Antisense Therapeutics Limited, Australia submitted to the licensing authority on 17/03/2021 06:57 GMT an application for a Paediatric Investigation Plan

The procedure started on 25/11/2021 10:27 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 agree a paediatric investigation plan and grant a deferral and grant a waiver

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-100060-PIP01-21

Of 10/12/2021 10:46 GMT

On the adopted decision for 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA (MHRA-100060-PIP01-21) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for 2'-O-(2-methoxyethyl) phosphorothioate antisense oligonucleotide targeting CD49d RNA, Solution for injection , Subcutaneous use .

This decision is addressed to Antisense Therapeutics Limited, Australia, Level 1, 14 Wallace Avenue Toorak, Victoria, Australia, 3142

ANNEX I

1. Waiver

1.1 Condition:

1.1 Condition: Treatment of Duchenne muscular dystrophy The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: Subcutaneous use Reason for granting waiver: 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(s):

Treatment of Duchenne muscular dystrophy

2.2 Indication(s) targeted by the PIP:

Treatment of Duchenne muscular dystrophy

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

The paediatric population from 2 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	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 participants with Duchenne Muscular Dystrophy (DMD) (1102-DMD-CT03) . Study 2 Open label extension study to assess the long term safety and efficacy profile of ATL1102 administered by subcutaneous injection in non-ambulatory paediatric patients from 10 years to less than 18 years of age (and young adults) with Duchenne Muscular Dystrophy (DMD) (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 paediatric patients from 5 years to less than 11 years 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 with Duchenne Muscular Dystrophy (DMD) (1102-DMD-CT08).

Extrapolation, Modeling & Simulation Studies	1	Study 5 Population 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 in Study 3 and Study 4, and analysis of exposure and response for boys aged 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 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:	31/05/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes