MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
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-102004-PIP01-25

Scope of the Application

Active Substance(s)

olezarsen sodium

Condition(s)

Treatment of familial chylomicronaemia syndrome

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Swedish Orphan Biovitrum AB

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Swedish Orphan Biovitrum AB submitted to the licensing authority on 31/07/2025 18:24 BST an application for a Paediatric Investigation Plan

The procedure started on 02/09/2025 19:18 BST

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-102004-PIP01-25

Of 04/12/2025 08:53 GMT

On the adopted decision for olezarsen sodium (MHRA-102004-PIP01-25) 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 olezarsen sodium , Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Swedish Orphan Biovitrum AB, -, Stockholm, SWEDEN, SE-112 76

ANNEX I

1. Waiver

1.1 Condition:

Treatment of familial chylomicronaemia syndrome 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 clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of familial chylomicronaemia syndrome

2.2 Indication(s) targeted by the PIP:

Treatment of familial chylomicronaemia syndrome

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	1	Study 1 (ISIS 678354-CS21) Open-label study to evaluate pharmacokinetics, pharmacodynamics, and safety of olezarsen in children from 2 years to less than 18 years of age with familial chylomicronaemia syndrome (FCS).
Extrapolation, Modeling & Simulation Studies	2	Study 2 (ISIS 678354-PPK03) Modelling and simulation study to update an existing population PK/PD model using data from adults and adolescents, to determine the appropriate dose in children from 2 years to less than 12 years of age with FCS. Study 3 Extrapolation study to support dose selection of olezarsen in children from 2 years of age to less than 18 years of age, and to compare PK, PD and PK/PD across age groups.
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:	No
Date of completion of the paediatric investigation plan:	31/01/2031

Deferral of one or more studies contained in the paediatric investigation plan:	Yes