MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral.

MHRA-100982-PIP01-23-M01

Scope of the Application

Active Substance(s)

pegunigalsidase alfa

Condition(s)

Treatment of Fabry disease

Pharmaceutical Form(s)

Solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Chiesi Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Chiesi Ltd submitted to the licensing authority on 14/04/2023 15:50 BST an application for a Modification

The procedure started on 17/08/2023 15:48 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 accept change(s) to the agreed paediatric investigation plan and to the deferral.

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-100982-PIP01-23-M01

Of 21/09/2023 10:55 BST

On the adopted decision for pegunigalsidase alfa (MHRA-100982-PIP01-23-M01) 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 modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for pegunigalsidase alfa, Solution for infusion , INTRAVENOUS USE .

This decision is addressed to Chiesi Ltd, 333 Styal Road, Manchester, UNITED KINGDOM, M22 5LG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Fabry disease. 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 infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: On the grounds 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 Fabry disease.

2.2 Indication(s) targeted by the PIP:

Treatment of Fabry disease.

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 infusion

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 Multi-centre, open-label trial to assess the safety, pharmacodynamics, efficacy and pharmacokinetics of pegunigalsidase alfa in patients from 2 years to less than 18 years of age with confirmed Fabry disease.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
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:	31/10/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes