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

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Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-100182-PIP01-21-M01

Scope of the Application

Active Substance(s)

Avalglucosidase alfa

Condition(s)

Treatment of Pompe disease

Pharmaceutical Form(s)

Powder for concentrate for solution for infusion

Route(s) of Administration

Intravenous use

Name / Corporate name of the PIP applicant

Genzyme Europe B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Genzyme Europe B.V. submitted to the licensing authority on 01/10/2021 12:22 BST an application for a Modification

The procedure started on 29/07/2022 14:38 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-100182-PIP01-21-M01

Of 12/08/2022 14:04 BST

On the adopted decision for Avalglucosidase alfa (MHRA-100182-PIP01-21-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 Avalglucosidase alfa, Powder for concentrate for solution for infusion , Intravenous use .

This decision is addressed to Genzyme Europe B.V., Paasheuvelweg 25, Amsterdam, Netherlands, 1105 BP

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Pompe disease

2.2 Indication(s) targeted by the PIP:

Long-term use as an ERT for the treatment of patients with Pompe disease (acid #-glucosidase deficiency)

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

The paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Powder for concentrate for 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	2	Study 1 Deleted in EMEA-001945-PIP01-16-M02 Study 2 (ACT14132) Open-label, multicentre, multinational, ascending dose, repeated intravenous infusion study of avalglucosidase alfa in treatment experienced paediatric patients from 6 months to less than 18 years of age with infantile-onset Pompe disease (IOPD) to evaluate the safety profile of and the pharmacokinetic profile of avalglucosidase alfa and to evaluate the preliminary efficacy of avalglucosidase alfa in comparison to alglucosidase alfa. Study 3 (EFC14462) Open-label, multinational, multicentre study of avalglucosidase alfa in treatment-naïve paediatric patients from birth to less than 12 months of age with infantile onset Pompe disease (IOPD) to determine the safety, tolerability and effect of avalglucosidase alfa treatment.
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:	No
Date of completion of the paediatric investigation plan:	31/12/2024

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