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 (MHRA-100274-PIP01-21-M01) and to the deferral

MHRA-100274-PIP01-21-M02

Scope of the Application

Active Substance(s)

ELIGLUSTAT

Condition(s)

Treatment of Gaucher Disease Type 2, Treatment of Gaucher Disease Type 1 and Type 3

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

ORAL 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 20/10/2022 10:53 BST an application for a Modification

The procedure started on 28/11/2022 12:21 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 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-100274-PIP01-21-M02

Of 09/12/2022 11:08 GMT

On the adopted decision for ELIGLUSTAT (MHRA-100274-PIP01-21-M02) 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 ELIGLUSTAT, Capsule, hard , ORAL USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Condition 1: Treatment of Gaucher Disease Type 2 The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Capsule, hard Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be ineffective. Condition 2: Treatment of Gaucher Disease Type 1 and Type 3 The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 24 months of age Pharmaceutical form(s): Capsule, hard Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe. Reason for Refusing Waiver:

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Gaucher Disease Type 1 and Type 3

2.2 Indication(s) targeted by the PIP:

Treatment of Gaucher Disease Type 1 and Type 3

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

The paediatric population from 24 months to less than 18 years of age

2.4 Pharmaceutical Form(s):

Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of 21 mg capsule strength, capsule, hard, for oral use in an age-appropriate dose based on PK data. Study 2 Data to support validated compounding of the product for children not able to swallow the capsules: including stability, reproducibility, dose uniformity and acceptability.
Non-Clinical Studies	1	Study 3 Juvenile Rat Toxicity Study
Clinical Studies	1	Study 4 Open label, two cohort (with and without imiglucerase), multi-centre, study to evaluate pharmacokinetics (PK), safety, and efficacy of eliglustat in paediatric patients with Gaucher disease type 1 (GD1) and type 3 (GD3).
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:	31/07/2023

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