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-100342-PIP01-21-M01

Scope of the Application

Active Substance(s)

arimoclomol citrate

Condition(s)

Treatment of Niemann-Pick Disease, type C

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

Orphazyme A/S

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Orphazyme A/S submitted to the licensing authority on 24/11/2021 16:25 GMT an application for a Modification

The procedure started on 18/01/2022 09:39 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-100342-PIP01-21-M01

Of 21/01/2022 10:32 GMT

On the adopted decision for arimoclomol citrate (MHRA-100342-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 arimoclomol citrate, Capsule, hard , Oral use .

This decision is addressed to Orphazyme A/S, Ole Maaloes Vej 3, Copenhagen N, Denmark, 2200

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Niemann-Pick Disease, type C The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 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.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Niemann-Pick Disease, type C

2.2 Indication(s) targeted by the PIP:

Treatment of Niemann-Pick Disease, type C

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

The paediatric population from 6 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	3	Study 1 In-use stability of drug product in beverages Study 2 In-use stability of drug product dispersed in food Study 3 Dose recovery by administration through feeding tube of drug product dispersed in water
Non-Clinical Studies	3	Study 4 Juvenile toxicity study Study 5 Reprotoxicity (enhanced) pre- and postnatal developmental study Study 6 Juvenile toxicity study
Clinical Studies	2	Study 7 Randomised, double-blind, placebo-controlled study to evaluate safety and efficacy of arimoclomol, in addition to best standard of care, in patients diagnosed with Niemann-Pick disease type C Study 9 Open-label study to assess safety and tolerability of arimoclomol, in addition to best standard of care, in patients aged 6 months to less than 24 months at study enrolment with confirmed diagnosis of NPC 1 or NPC 2
Extrapolation, Modeling & Simulation Studies	1	Study 8 Modelling study for optimisation of study 9
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:	30/06/2023
Deferral of one or more studies contained in the paediatric investigation plan:	Yes