MHRA
10 South Colonnade
Canary Wharf
London
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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-101016-PIP01-23

Scope of the Application

Active Substance(s)

ublituximab

Condition(s)

Treatment of multiple sclerosis

Pharmaceutical Form(s)

Concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

ProPharma Group The Netherlands B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, ProPharma Group The Netherlands B.V. submitted to the licensing authority on 17/05/2023 09:45 BST an application for a Paediatric Investigation Plan

The procedure started on 18/05/2023 10:53 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-101016-PIP01-23

Of 26/05/2023 12:26 BST

On the adopted decision for ublituximab (MHRA-101016-PIP01-23) 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 ublituximab, Concentrate for solution for infusion , INTRAVENOUS USE .

This decision is addressed to ProPharma Group The Netherlands B.V. , Schipholweg 73, Leiden, NETHERLANDS, 2316 ZL

ANNEX I

1. Waiver

1.1 Condition:

Treatment of multiple sclerosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 10 years of age Pharmaceutical form(s): Concentrate for solution for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of multiple sclerosis

2.2 Indication(s) targeted by the PIP:

Treatments of relapsing forms of multiple sclerosis

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

The paediatric population from 10 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

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 Open-label, uncontrolled trial with two cohorts to evaluate pharmacokinetics, pharmacodynamics, safety and activity of ublituximab in children from 10 years to less than 18 years of age with relapsing forms of multiple sclerosis. Study 2 Double-blind, double dummy, randomised, non-inferiority trial to evaluate safety and efficacy of ublituximab compared to fingolimod in children from 10 years to less than 18 years of age with relapsing forms of multiple sclerosis.
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:	31/12/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes