MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
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-100103-PIP01-21

Scope of the Application

Active Substance(s)

rozanolixizumab

Condition(s)

Treatment of myelin oligodendrocyte glycoprotein antibody-associated disease

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

Subcutaneous use

Name / Corporate name of the PIP applicant

UCB Pharma Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, UCB Pharma Limited submitted to the licensing authority on 03/06/2021 15:39 BST an application for a Paediatric Investigation Plan

The procedure started on 22/04/2022 07:27 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-100103-PIP01-21

Of 18/12/2023 16:56 GMT

On the adopted decision for rozanolixizumab (MHRA-100103-PIP01-21) 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 rozanolixizumab, Solution for injection , Subcutaneous use .

This decision is addressed to UCB Pharma Limited, 208 Bath Road, Slough, United Kingdom, SL1 3WE Berkshire

ANNEX I

1. Waiver

1.1 Condition:

Treatment of myelin oligodendrocyte glycoprotein antibody-associated 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 injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that 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 myelin oligodendrocyte glycoprotein antibody-associated disease

2.2 Indication(s) targeted by the PIP:

Treatment of children from 2 years to less than 18 years of age with relapsing myelin oligodendrocyte glycoprotein antibody-associated 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 injection

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, single-arm trial to evaluate pharmacokinetics, pharmacodynamics, safety, and tolerability of rozanolixizumab in adolescents from 12 years to less than 18 years of age with relapsing myelin oligodendrocyte glycoprotein antibody-associated disease (MOG-AD). Study 2 Open-label, single-arm trial to evaluate pharmacokinetics, pharmacodynamics, safety, and tolerability of rozanolixizumab in children from 2 years to less than 12 years of age with relapsing myelin oligodendrocyte glycoprotein antibody-associated disease (MOG-AD).
Extrapolation, Modeling & Simulation Studies	2	Study 3 Modelling and simulation study to support the dose selection of rozanolixizumab in children from 2 years to less than 18 years of age with relapsing myelin oligodendrocyte glycoprotein antibody-associated disease (MOG-AD). Extrapolation Plan Studies 1, 2 and 3 are part of the extrapolation plan of efficacy data from adult to the paediatric population from 2 years to less than 18 years of age with condition relapsing myelin

		oligodendrocyte glycoprotein antibody-associated disease (MOG-AD).
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:	31/12/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes