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-100709-PIP01-22-M01)

MHRA-100709-PIP01-22-M02

Scope of the Application

Active Substance(s)

rozanolixizumab

Condition(s)

Treatment of Myasthenia Gravis

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

UCB Pharma Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, UCB Pharma Ltd submitted to the licensing authority on 20/03/2023 14:50 GMT an application for a Modification

The procedure started on 27/03/2023 09:56 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

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-100709-PIP01-22-M02

Of 31/03/2023 13:53 BST

On the adopted decision for rozanolixizumab (MHRA-100709-PIP01-22-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 rozanolixizumab, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to UCB Pharma Ltd, 208 Bath Road, Slough, UNITED KINGDOM, SL1 3WE

ANNEX I

1. Waiver

1.1 Condition:

Treatment of myasthenia gravis 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 the specific medicinal product is likely to be ineffective.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of myasthenia gravis

2.2 Indication(s) targeted by the PIP:

Treatment of myasthenia gravis

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	1	Study 1 Open-label, single arm activity, safety and pharmacokinetic study of rozanolixizumab in paediatric patients from 2 years to less than 18 years of age with generalised myasthenia gravis (gMG) with moderate to severe symptoms.
Extrapolation, Modeling & Simulation Studies	1	Study 2 Development of an exposure response model to support the dose selection of rozanolixizumab for Study 1 and confirmation of final paediatric recommended dose.
Other Studies	1	Study 3 Systematic review of literature to map existing evidence on the effect of age, maturation, disease type, severity, comorbidity for myasthenia gravis, supporting extrapolation of efficacy data from adults to paediatric patients.
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:	31/12/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes