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 of change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-101233-PIP01-23-M01

Scope of the Application

Active Substance(s)

inebilizumab

Condition(s)

Treatment of neuromyelitis optica spectrum disorders

Pharmaceutical Form(s)

Solution for infusion Concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Amgen Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Amgen Limited submitted to the licensing authority on 16/11/2023 15:08 GMT an application for a

The procedure started on 13/03/2024 11:43 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-101233-PIP01-23-M01

Of 04/04/2024 11:18 BST

On the adopted decision for inebilizumab (MHRA-101233-PIP01-23-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 for inebilizumab, Solution for infusion Concentrate for solution for infusion , INTRAVENOUS .

This decision is addressed to Amgen Limited, 216 Cambridge Science Park, Milton Road, Cambridge, UNITED KINGDOM, CB4 0WA

ANNEX I

1. Waiver

1.1 Condition:

Treatment of neuromyelitis optica spectrum disorders. The waiver applies to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for infusion 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 neuromyelitis optica spectrum disorders

2.2 Indication(s) targeted by the PIP:

Maintenance treatment of patients with neuromyelitis optica spectrum disorders (NMOSD)

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

From 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for infusion Concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Study 1 Deleted during procedure MHRA-101233-PIP01-23-M01
Non-Clinical Studies	1	Study 2 Reprotox: enhanced pre- and postnatal development in mice.
Clinical Studies	1	Study 3 Open-label, uncontrolled trial to evaluate PK/PD and safety of inebilizumab in children from 2 to less than 18 years of age with neuromyelitis optica spectrum disorders.
Extrapolation, Modeling & Simulation Studies	2	Study 4 Modelling and simulation study to determine the dose of inebilizumab in the treatment of neuromyelitis optica spectrum disorders in children from 2 to less than 18 years of age. Study 5 Analysis of existing data on efficacy, safety, pharmacodynamics and pharmacokinetics of inebilizumab to evaluate the use of the product in the treatment of neuromyelitis optica spectrum disorders in children from 2 to less than 18 years of age.
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:	30/04/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes