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

MHRA-101359-PIP01-24

Scope of the Application

Active Substance(s)

SPESOLIMAB

Condition(s)

Treatment of Netherton syndrome

Pharmaceutical Form(s)

Solution for infusion, Solution for injection

Route(s) of Administration

INTRAVENOUS USE; SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Boehringer Ingelheim International GmbH

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Boehringer Ingelheim International GmbH submitted to the licensing authority on 09/02/2024 13:04 GMT an application for a Paediatric Investigation Plan

The procedure started on 15/03/2024 14:39 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 agree a paediatric investigation plan and grant a 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-101359-PIP01-24

Of 07/06/2024 13:29 BST

On the adopted decision for SPESOLIMAB (MHRA-101359-PIP01-24) 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 SPESOLIMAB, Solution for infusion, Solution for injection , INTRAVENOUS USE; SUBCUTANEOUS USE .

This decision is addressed to Boehringer Ingelheim International GmbH, Binger Strasse 173, Ingelheim am Rhein, GERMANY, 55216

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Netherton syndrome

2.2 Indication(s) targeted by the PIP:

Treatment of patients with Netherton syndrome

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

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

2.4 Pharmaceutical Form(s):

Solution for infusion 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 (1368-0104) Double-blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, safety, and efficacy of spesolimab in children from 12 years to less than 18 years of age (and adults) with Netherton syndrome. Study 2 (1368-NS-Ped1) Open-label non-randomised uncontrolled trial to evaluate safety, pharmacokinetics and immunogenicity of spesolimab in children from birth to less than 12 years of age with Netherton syndrome.
Extrapolation, Modeling & Simulation Studies	4	Study 3 Modelling and simulation study to evaluate the adequacy of dose of spesolimab in children from 12 years to less than 18 years of age with Netherton syndrome. Study 4 Modelling and simulation study to inform the design of Study 2 (open label study of spesolimab in children from birth to less than 12 years of age with Netherton syndrome). Study 5 Modelling and simulation study to evaluate the adequacy of dose of spesolimab in children from birth to less than 12 years of age with Netherton syndrome. Extrapolation Plan Studies 1, 2 ,3, 4, 5 are part of an extrapolation plan covering the paediatric population from birth to less than 12 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:	No
Date of completion of the paediatric investigation plan:	30/09/2033
Deferral of one or more studies contained in the paediatric investigation plan:	Yes