MHRA
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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to grant a product specific waiver

MHRA-100645-PIP01-22-M01

Scope of the Application

Active Substance(s)

AMIKACIN SULFATE

Condition(s)

Treatment of Pseudomonas aeruginosa lung infection/colonisation in cystic fibrosis patients, Treatment of non-tuberculous mycobacterial lung infection

Pharmaceutical Form(s)

Nebuliser dispersion

Route(s) of Administration

INHALATION USE

Name / Corporate name of the PIP applicant

Insmed Netherlands B.V.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Insmed Netherlands B.V. submitted to the licensing authority on 17/08/2022 13:45 BST an application for a Modification

The procedure started on 07/02/2023 13:17 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 grant a product specific 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-100645-PIP01-22-M01

Of 24/03/2023 15:38 GMT

On the adopted decision for AMIKACIN SULFATE (MHRA-100645-PIP01-22-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 Modification for AMIKACIN SULFATE, Nebuliser dispersion , INHALATION USE .

This decision is addressed to Insmed Netherlands B.V., Stadsplateau 7, Utrecht, NETHERLANDS, 3521 AZ

ANNEX I

1. Waiver

1.1 Condition:

Condition 1: Treatment of Pseudomonas aeruginosa lung infection/colonisation in cystic fibrosis patients The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Nebuliser dispersion Route(s) of administration: INHALATION USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be ineffective. Condition 2: Treatment of non-tuberculous mycobacterial lung infection The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 years of age Pharmaceutical form(s): Nebuliser dispersion Route(s) of administration: INHALATION 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):

Condition 2: Treatment of non-tuberculous mycobacterial lung infection

2.2 Indication(s) targeted by the PIP:

Condition 2: Treatment of non-tuberculous mycobacterial lung infection

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

Condition 2: The paediatric population from 6 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Condition 2: Nebuliser dispersion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	2	Study 1 2-year inhalation carcinogenicity study in rats. Study 2 Juvenile rodent inhalation toxicity and toxicokinetic study.
Clinical Studies	0	Study 11 Deleted during modification MHRA-100645-PIP01-22-M01.
Extrapolation, Modeling & Simulation Studies	1	Study 12 Extrapolation study of pharmacokinetic (PK) data of liposomal amikacin for inhalation (LAI) from adult and paediatric patients with Pseudomonas infection/ colonisation in cystic fibrosis (CF) to paediatric patients with CF and non-tuberculous mycobacterial (NTM) lung disease.
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:	30/04/2016

Deferral of one or more studies contained in the paediatric investigation plan:	Yes