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-100315-PIP01-21) and to the deferral

MHRA-100315-PIP01-21-M01

Scope of the Application

Active Substance(s)

Brensocatib

Condition(s)

Treatment of non#cystic fibrosis bronchiectasis

Pharmaceutical Form(s)

Film-coated tablet; Age-appropriate oral liquid dosage form

Route(s) of Administration

ORAL 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 18/10/2024 14:14 BST an application for a Modification

The procedure started on 02/12/2024 08:20 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-100315-PIP01-21-M01

Of 22/01/2025 17:57 GMT

On the adopted decision for Brensocatib (MHRA-100315-PIP01-21-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 Brensocatib, Film-coated tablet; Age-appropriate oral liquid dosage form , ORAL USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of non#cystic fibrosis bronchiectasis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 years of age Pharmaceutical form(s): Film-coated tablet Age-appropriate oral liquid dosage form Route(s) of administration: ORAL 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 non#cystic fibrosis bronchiectasis

2.2 Indication(s) targeted by the PIP:

Treatment of non#cystic fibrosis bronchiectasis (NCFBE) for reducing exacerbations

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

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

2.4 Pharmaceutical Form(s):

Film-coated tablet Age-appropriate oral liquid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	1	Study 1 (QLT-DOC-001231) Development of an age-appropriate oral liquid dosage form.
Clinical Studies	0	Not applicable.
Extrapolation, Modeling & Simulation Studies	2	Study 2 (INS1007-301) Double blind, randomised, placebo controlled study to evaluate the safety and efficacy of brensocatib in adolescents (and adults) from 12 years to less than 18 years of age with NCFBE. Study 3 (INS1007-202) Open label single arm multicentre study to evaluate pharmacokinetics, pharmacodynamics, efficacy, and safety of brensocatib in children from 6 years to less than 12 years of age with NCFBE.
Other Studies	2	Study 4 (VV-CLIN-005036) Modelling and simulation study to evaluate the doses of brensocatib for investigation in children from 6 years to less than 12 years of age with NCFBE. Study 5 (VV-CLIN-005037) Modelling and simulation study to evaluate the use of brensocatib in children from 6 years to less than 12 years of age with NCFBE.
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:	30/04/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes