

MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

grant a product specific waiver

MHRA-101019-PIP01-23

Scope of the Application

Active Substance(s)

Treprostinil Palmitil

Condition(s)

Treatment of pulmonary hypertension due to lung disease and/or hypoxia.

Pharmaceutical Form(s)

Inhalation powder

Route(s) of Administration

INHALATION USE

Name / Corporate name of the PIP applicant

INSMED NETHERLANDS BV

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, INSMED NETHERLANDS BV submitted to the licensing authority on 25/05/2023 15:24 BST an application for a Paediatric Investigation Plan

The procedure started on 05/10/2023 12:10 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 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-101019-PIP01-23

Of 20/10/2023 16:09 BST

On the adopted decision for Treprostinil Palmitil (MHRA-101019-PIP01-23) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Granting a waiver in all age groups for the listed condition(s)

This decision applies to a Paediatric Investigation Plan for Treprostinil Palmitil, Inhalation powder , INHALATION USE .

This decision is addressed to INSMED NETHERLANDS BV, Stadsplateau 7, Utrecht, NETHERLANDS, 3521 AZ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of pulmonary hypertension due to lung disease and/or hypoxia. 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): Inhalation powder 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):

Not applicable

2.2 Indication(s) targeted by the PIP:

| |
|----------------|
| Not applicable |
|----------------|

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

| |
|----------------|
| Not applicable |
|----------------|

2.4 Pharmaceutical Form(s):

| |
|----------------|
| Not applicable |
|----------------|

2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|--------------------------|--------------------------|
| Quality Measures | 0 | Not applicable |
| Non-Clinical Studies | 0 | Not applicable |
| Clinical Studies | 0 | Not applicable |
| Extrapolation, Modeling & Simulation Studies | 0 | Not applicable |
| 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: | |
| Date of completion of the paediatric investigation plan: | |
| Deferral of one or more studies contained in the paediatric investigation plan: | |