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

**gov.uk/mhra**

# Decision Cover Letter

## Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100332-PIP01-21

# Scope of the Application

## Active Substance(s)

sotatercept

## Condition(s)

Treatment of pulmonary arterial hypertension

## Pharmaceutical Form(s)

Powder for solution for injection

## Route(s) of Administration

Subcutaneous use

## Name / Corporate name of the PIP applicant

Merck Sharp & Dohme (UK) Limited

## Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Merck Sharp & Dohme (UK) Limited submitted to the licensing authority on 26/01/2022 18:16 GMT an application for a Paediatric Investigation Plan

The procedure started on 27/05/2022 10:00 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 agree a paediatric investigation plan and grant a deferral and grant a 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-100332-PIP01-21

Of 07/07/2022 15:52 BST

On the adopted decision for sotatercept (MHRA-100332-PIP01-21) 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 sotatercept, Powder for solution for injection , Parenteral use .

This decision is addressed to Merck Sharp & Dohme (UK) Limited, 128 Sidney Street, cambridge, MA, United States, EC2M 6UR

# ANNEX I

## 1. Waiver

### 1.1 Condition:

| |
|---|
| Treatment of pulmonary arterial hypertension The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Powder for solution for injection Route(s) of administration: Subcutaneous use Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe. |

## 2. Paediatric Investigation Plan:

### 2.1 Condition(s):

| |
|---|
| Treatment of pulmonary arterial hypertension (PAH) |

### 2.2 Indication(s) targeted by the PIP:

| |
|---|
| Treatment of PAH, World Health Organisation (WHO) Group I to improve exercise tolerance and functional class. |

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

| |
|---|
| The paediatric population from 1 year to less than 18 years of age |

### 2.4 Pharmaceutical Form(s):

| |
|---|
| Powder for solution for injection |

### 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 1 | Study 1 Development of an age-appropriate dosage form. |
| **Non-Clinical Studies** | 0 | Not applicable |
| **Clinical Studies** | 1 | Study 2 Open label, 24-week study to assess pharmacokinetics (PK), safety and pharmacodynamic effects of sotatercept as add-on therapy to standard-of-care in children from 1 year to less than 18 years of age with pulmonary arterial hypertension (PAH). |
| **Extrapolation, Modeling & Simulation Studies** | 2 | Study 3 Population pharmacokinetic/ pharmacodynamic analysis to support the extrapolation of efficacy of sotatercept in the treatment of PAH in children from 1 year to less than 18 years of age. Study 4 Analysis of existing inhouse and literature data to support extrapolation of efficacy of sotatercept in the treatment of PAH in children from 1-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:** | 31/08/2023 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |