**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-101058-PIP01-23

# Scope of the Application

## Active Substance(s)

SIROLIMUS

## Condition(s)

Treatment of Tuberous Sclerosis

## Pharmaceutical Form(s)

Cream

## Route(s) of Administration

TOPICAL USE

## Name / Corporate name of the PIP applicant

AFT Pharma UK Limited

## Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, AFT Pharma UK Limited submitted to the licensing authority on 20/06/2023 05:52 BST an application for a Paediatric Investigation Plan

The procedure started on 24/10/2023 08:03 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.

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

**gov.uk/mhra**

# Final Decision Letter

MHRA-101058-PIP01-23

Of 09/11/2023 11:46 GMT

On the adopted decision for SIROLIMUS (MHRA-101058-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:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for SIROLIMUS, Cream , TOPICAL USE .

This decision is addressed to AFT Pharma UK Limited, 14 Manchester Square, London, UNITED KINGDOM, W1U 3PP

## ANNEX I

### 1. Waiver

### 1.1 Condition:

| |
|---|
| Treatment of Tuberous Sclerosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Cream Route(s) of administration: TOPICAL 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 Tuberous Sclerosis |

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

| |
|---|
| Treatment of facial angiofibromas associated with tuberous sclerosis |

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

| |
|---|
| The paediatric population from 2 years to less than 18 years of age |

### 2.4 Pharmaceutical Form(s):

| |
|---|
| Cream |

### 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 (DSLP-01) Double blind, randomised, placebo-controlled trial to evaluate safety and efficacy of sirolimus in children from 6 years to less than 18 years (and adults) with facial angiofibromas associated with tuberous sclerosis. Study 2 (DSLP-02) Double-blind, randomised, placebo-controlled 12 week study to evaluate pharmacokinetics, safety and efficacy of sirolimus in children from 2 years to less than 18 years of age (and adults) with facial angiofibromas associated with tuberous sclerosis, followed by an open label safety part. |
| **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:** | No |
| **Date of completion of the paediatric investigation plan:** | 31/12/2026 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |