MHRA
10 South Colonnade
Canary Wharf
London
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-100689-PIP01-22

Scope of the Application

Active Substance(s)

Dersimelagon

Condition(s)

Treatment of systemic sclerosis

Pharmaceutical Form(s)

Film-coated tablet; Age-appropriate oral formulation

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Mitsubishi Tanabe Pharma Europe

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Mitsubishi Tanabe Pharma Europe submitted to the licensing authority on 18/10/2022 10:41 BST an application for a Paediatric Investigation Plan

The procedure started on 13/03/2023 16:51 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 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-100689-PIP01-22

Of 18/12/2023 16:24 GMT

On the adopted decision for Dersimelagon (MHRA-100689-PIP01-22) 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 Dersimelagon, Film-coated tablet; Age-appropriate oral formulation , ORAL USE .

This decision is addressed to Mitsubishi Tanabe Pharma Europe, 69 Dashwood House, London, UNITED KINGDOM, EC2M1QS

ANNEX I

1. Waiver

1.1 Condition:

Treatment of systemic sclerosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 5 years of age Pharmaceutical form(s): Film-coated tablet Age-appropriate oral formulation 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 systemic sclerosis

2.2 Indication(s) targeted by the PIP:

Treatment of juvenile diffuse cutaneous systemic sclerosis

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

The paediatric population from 5 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet Age-appropriate oral formulation

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Generation of data on suitability of crushing existing film-coated tablets for use in the paediatric population from 5 years to less than 12 years and in children not able to swallow tablets. Study 2 Development of an age-appropriate oral formulation (oral solid dosage form or oral liquid dosage form) for use in the paediatric population from 5 years to less than 12 years of age and in children not able to swallow the existing film-coated tablets, and where results of study 1 demonstrate that crushing existing film coated tablets is not appropriate.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 3 (MT-7117-A0Y) Open-label, single arm, multicentre study to evaluate the pharmacokinetics, pharmacodynamics, safety, and tolerability of dersimelagon in children from 5 years to less than 18 years of age with diffuse cutaneous systemic sclerosis (dcSSc).
Extrapolation, Modeling & Simulation Studies	2	Study 4 Modelling and simulation dose finding study in children from 5 years to less than 18 years of age with diffuse cutaneous systemic sclerosis. Extrapolation Plan Study 3 and 4 are part of the extrapolation of efficacy from adult and children

		to the paediatric population form 5 years to 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:	No
Date of completion of the paediatric investigation plan:	31/03/2032
Deferral of one or more studies contained in the paediatric investigation plan:	Yes