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-100115-PIP01-21

Scope of the Application

Active Substance(s)

Dersimelagon

Condition(s)

Treatment of erythropoietic protoporphyria , Treatment of X-linked protoporphyria

Pharmaceutical Form(s)

Film coated tablet, Age-appropriate dosage form

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

Mitsubishi Tanabe Pharma Europe Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Mitsubishi Tanabe Pharma Europe Limited submitted to the licensing authority on 27/05/2021 13:08 BST an application for a

The procedure started on 02/03/2022 17:40 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-100115-PIP01-21

Of 07/07/2022 09:16 BST

On the adopted decision for Dersimelagon (MHRA-100115-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 for Dersimelagon, Film coated tablet, Age-appropriate dosage form , Oral use .

This decision is addressed to Mitsubishi Tanabe Pharma Europe Limited, Dashwood House, 69 Old Broad Street, London, United Kingdom, EC2M 1QS

ANNEX I

1. Waiver

1.1 Condition:

Treatment of erythropoietic protoporphyria (EPP) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Film coated tablet; Age-appropriate dosage form Route(s) of administration: Oral use Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). 1.2 Condition: Treatment of X-linked protoporphyria (XLP) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Film coated tablet; Age-appropriate dosage form Route(s) of administration: Oral use Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Condition 1: Treatment of erythropoietic protoporphyria (EPP); Condition 2: Treatment of X-linked protoporphyria (XLP)

2.2 Indication(s) targeted by the PIP:

Condition 1: Treatment of erythropoietic protoporphyria; Condition 2: Treatment of X-linked protoporphyria

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

For both conditions: The paediatric population from 1 year to less than 18 years of age

2.4 Pharmaceutical Form(s):

For both conditions: Film coated tablet; Age-appropriate dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	(Same for both conditions) Study 1 Generation of data on suitability of crushing existing film-coated tablets for use in the paediatric population from 1 year to less than 12 years and in children not able to swallow tablets. Study 2 Development of an age-appropriate dosage from (oral solid dosage form or oral liquid dosage form) for use in the paediatric population from 1 year to less than 12 years of age and in children not able to swallow 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	2	(Same for both conditions) Study 3 (MT-7117-G01) Randomised, double-blind, placebo-controlled study to assess the efficacy, tolerability, safety, pharmacokinetics and dose determination of dersimelagon in adolescents from 12 years to less than 18 years of age (and adults) with erythropoietic protoporphyria (EPP) or X-linked protoporphyria (XLP). Study 4 (MT-7117-A0X) Open-label,

		single arm study to assess the pharmacokinetics, tolerability, safety and clinical activity of dersimelagon in children from 1 year to less than 12 years of age with erythropoietic protoporphyria (EPP) and X-linked protoporphyria (XLP).
Extrapolation, Modeling & Simulation Studies	2	(Same for both conditions) Study 5 Population PK modelling and PK/ PD exposure-response study to select doses of dersimelagon across weight bands and age groups to be used in children from 1 year to less than 6 years of age, and from 6 years to less than 12 years of age with erythropoietic protoporphyria (EPP) and X-linked protoporphyria (XLP). Study 6 Analysis of existing data on efficacy, safety and pharmacokinetics of dersimegalon to evaluate the use of the product in the treatment of erythropoietic protoporphyria (EPP) and X-linked protoporphyria (XLP) in children from 1 year to less than 12 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:	31/12/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes