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

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100115-PIP01-21) and to the deferral

MHRA-100115-PIP01-21-M01

Scope of the Application

Active Substance(s)

Dersimelagon

Condition(s)

Treatment of erythropoetic 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

Tanabe Pharma Europe Ltd.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Tanabe Pharma Europe Ltd. submitted to the licensing authority on 15/01/2026 12:06 GMT an application for a Modification

The procedure started on 02/03/2026 09:49 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 accept change(s) to the agreed paediatric investigation plan and to the deferral

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-M01

Of 24/04/2026 08:45 BST

On the adopted decision for Dersimelagon (MHRA-100115-PIP01-21-M01) 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 modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for Dersimelagon, Film coated tablet; Age-appropriate dosage form , ORAL USE .

This decision is addressed to Tanabe Pharma Europe Ltd., Dashwood House, 69 Old Broad Street, London, UNITED KINGDOM, EC2M 1QS

ANNEX I

1. Waiver

1.1 Condition:

Condition 1: 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). Condition 2: 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 studies 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	3	(Same studies 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-A-30X) 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). Study 7 (MT-7117-A-302) Added during procedure MHRA-100115-PIP01-21-M01 Randomised, double-blind, placebo-controlled trial to evaluate efficacy, safety, and tolerability of dersimelagon in adolescents aged from 12 years to less than 18 years of age (and adults) with erythropoietic protoporphyria or X-Linked protoporphyria.
Extrapolation, Modeling & Simulation Studies	2	(Same studies 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 Deleted during procedure MHRA-100115-PIP01-21-M01 Extrapolation plan Added during procedure MHRA-100115-PIP01-21-M01 Studies MT-7117-E01, MT-7117-Z-102, MT-7117-A01, MT-7117-G01, MT-7117-A-302 and MT-7117-A-30X are part of the extrapolation plan covering the paediatric population 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:	31/10/2030

Deferral of one or more studies contained in the paediatric investigation plan:	Yes