EMA/366139/2022

European Medicines Agency decision

P/0216/2022

of 10 June 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for dersimelagon (EMEA-002850-PIP02-21) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Mitsubishi Tanabe Pharma GmBH on 16 April 2021 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 22 April 2022, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for dersimelagon, film-coated tablet, age-appropriate dosage form, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for dersimelagon, film-coated tablet, age-appropriate dosage form, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for dersimelagon, film-coated tablet, age-appropriate dosage form, oral use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Mitsubishi Tanabe Pharma GmbH, Willstätter Straße 30, 40549 – Düsseldorf, Germany.

EMA/74553/2022
Amsterdam, 22 April 2022

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMEA-002850-PIP02-21

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 GmBH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Mitsubishi Tanabe Pharma GmBH submitted for agreement to the European Medicines Agency on 16 April 2021 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 25 May 2021.

Supplementary information was provided by the applicant on 19 January 2022.

The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of erythropoietic protoporphyria

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- film-coated tablet, age-appropriate dosage form, oral use;
- 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

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- film-coated tablet, age-appropriate dosage form, oral use;
- 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:

Treatment of erythropoietic protoporphyria

2.1.1. Indication(s) targeted by the PIP

Treatment of erythropoietic protoporphyria in children from 1 year to less than 18 years of age

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 year to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate dosage form

2.1.4. Measures

Area	Description
Quality-related studies	**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 children not able to swallow the existing film-coated tablets **Study 2** Development of an age-appropriate dosage form (oral solid dosage form or oral liquid dosage form) for use in the paediatric population from 1 year to less than 12 years and children not able to swallow the existing film-coated tablets and results of study 1 demonstrate that crushing existing film-coated tablets is not appropriate
Non-clinical studies	Not applicable.
Clinical studies	**Study 3 (MT-7117-G01)** Randomised, double-blind, placebo-controlled study to assess the efficacy, tolerability, safety and pharmacokinetics and dose determination of dersimelagon in adolescents from 12 years to less than 18 years of age (and in adults) with erythropoietic protoporphyria (EPP) or X-linked protoporphyria (XLP) **Study 4 (MT-117-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) or X-linked protoporphyria (XLP)
Extrapolation, modelling and simulation studies	**Study 5** Population PK modelling and PK/PD exposure-response study to select the 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 dersimelagon 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	Not applicable.
Other measures	Not applicable.

2.2. Condition:

Treatment of X-linked protoporphyria

2.2.1. Indication(s) targeted by the PIP

Treatment of X-linked protoporphyria in children from 1 year to less than 18 years of age

2.2.2. Subset(s) of the paediatric population concerned by the paediatric development

From 1 year to less than 18 years of age

2.2.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate dosage form

2.2.4. Measures

Area	Description
Quality-related studies	***Study 1*** same as study 1 in condition erythropoietic protoporphyria ***Study 2*** same as study 2 in condition erythropoietic protoporphyria
Non-clinical studies	Not applicable.
Clinical studies	***Study 3*** same as study 3 in condition erythropoietic protoporphyria ***Study 4*** same as study 1 in condition erythropoietic protoporphyria
Extrapolation, modelling and simulation studies	***Study 5*** same as study 5 in condition erythropoietic protoporphyria ***Study 6*** same as study 6 in condition erythropoietic protoporphyria
Other studies	Not applicable.
Other measures	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By December 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes