EMA/275720/2022

European Medicines Agency decision

P/0204/2022

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 omaveloxolone (EMEA-002487-PIP01-18) 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 Reata Ireland Limited on 26 October 2020 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 omaveloxolone, capsule, hard, age appropriate oral formulation, oral use, gastric 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 omaveloxolone, capsule, hard, age appropriate oral formulation, oral use, gastric 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 omaveloxolone, capsule, hard, age appropriate oral formulation, oral use, gastric 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 Reata Ireland Limited, 32 Merrion Street Upper, D02 KW80 – DUBLIN, Ireland.

EMA/PDCO/54744/2022
Amsterdam, 22 April 2022

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

EMEA-002487-PIP01-18

Scope of the application

Active substance(s):

Omaveloxolone

Condition(s):

Treatment of Friedreich's ataxia

Pharmaceutical form(s):

Capsule, hard

Age appropriate oral formulation

Route(s) of administration:

Oral use

Gastric use

Name/corporate name of the PIP applicant:

Reata Ireland Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Reata Ireland Limited submitted for agreement to the European Medicines Agency on 26 October 2020 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 1 December 2020.

Supplementary information was provided by the applicant on 21 January 2022. The applicant proposed modifications to the paediatric investigation plan and waiver.

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 Friedreich's ataxia

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- capsule, hard, age appropriate oral formulation, 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 Friedreich's ataxia

2.1.1. Indication(s) targeted by the PIP

Treatment of Friedreich’s ataxia

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

From 2 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Capsule, hard

Age appropriate oral formulation

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1 (PED-FORM-DEV-1)** Development of an age appropriate oral capsule formulation
Non-clinical studies	Not applicable
Clinical studies	**Study 2 (408-C-1402 [Part 1])** Double-blind, randomised, placebo-controlled, dose ranging trial to evaluate pharmacokinetics, pharmacodynamics, safety and efficacy of omaveloxolone in adolescents from 16 years to less than 18 years of age (and adults) with genetically confirmed Friedreich's ataxia.

	Study 3 (408-C-1402 [Part 2]) Double-blind, randomised, placebo controlled trial to evaluate safety and efficacy of omaveloxolone in adolescents from 16 years to less than 18 years of age (and adults) with genetically confirmed Friedreich's ataxia. **Study 4 (408-C-2001)** Open-label, single arm, historical controlled, two part trial to identify the appropriate dose (part one) and evaluate pharmacokinetics, pharmacodynamics, safety, activity and acceptability/palatability of omaveloxolone in children from 2 years to less than 16 years of age with genetically confirmed Friedreich's ataxia.
Extrapolation, modelling and simulation studies	**Study 5** Modelling and simulation study to support the use of omaveloxolone in children from 2 years to less than 16 years of age with genetically confirmed Friedreich's ataxia.
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:	Yes
Date of completion of the paediatric investigation plan:	By June 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes