EMA/699703/2021 corr

European Medicines Agency decision

P/0543/2021

of 31 December 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral for vatiquinone (EMEA-001238-PIP03-21) 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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on the agreement of a paediatric investigation plan and on the granting of a deferral for vatiquinone (EMEA-001238-PIP03-21) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by PTC Therapeutics International on 11 February 2021 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 12 November 2021, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

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

Has adopted this decision:

Article 1

A paediatric investigation plan for vatiquinone, capsule, hard, oral solution, 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 vatiquinone, capsule, hard, oral solution, 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

This decision is addressed to PTC Therapeutics International, 5th Floor, 3 Grand Canal Plaza Grand Canal Street Upper, Dublin 4, D04 EE70 – Dublin, Ireland.

EMA/PDCO/463016/2021 Corr
Amsterdam, 12 November 2021

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

EMEA-001238-PIP03-21

Scope of the application

Active substance(s):

Vatiquinone

Condition(s):

Treatment of Friedreich's ataxia

Pharmaceutical form(s):

Capsule, hard

Oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

PTC Therapeutics International

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, PTC Therapeutics International submitted for agreement to the European Medicines Agency on 11 February 2021 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 23 March 2021.

Supplementary information was provided by the applicant on 6 August 2021. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a 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 of its own motion in accordance with Article 21 of said Regulation.

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

   The measures and timelines of the agreed paediatric investigation plan 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

Not applicable

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 birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Capsule, hard; oral solution

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	1	**Study 1:** Development of an oral solution
Non-clinical studies	0	Not applicable
Clinical studies	2	**Study 2:** Double-blind, randomised, placebo-controlled trial to evaluate efficacy and safety of vatiquinone in children from 7 years to less than 18 years of age (and adults) with Friedreich's ataxia. (PTC743-NEU-003-FA; MOVE-FA) **Study 3:** Open-label trial to evaluate pharmacokinetics, safety and efficacy in children from birth to less than 7 years of age with Friedreich's ataxia

Extrapolation, modelling and simulation studies	2	**Study 4:** Modelling and simulation study to determine population pharmacokinetic parameters, inter-subject and intra-subject variability and potential covariates, to support extrapolation and evaluate the use of vatiquinone in children from 7 years to less than 18 years of age with Friedreich's ataxia **Study 5:** Modelling and simulation study to determine population pharmacokinetic parameters, inter-subject and intra-subject variability and potential covariates, to support extrapolation and evaluate the use of vatiquinone in children from birth to less than 7 years of age with Friedreich's ataxia
Other studies	0	Not applicable
Other measures	0	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 March 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes