

EMA/186780/2023

European Medicines Agency decision P/0192/2023

of 26 May 2023

on the acceptance of a modification of an agreed paediatric investigation plan for vatiquinone (EMA-001238-PIP03-21-M01) 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 acceptance of a modification of an agreed paediatric investigation plan for vatiquinone (EMA-001238-PIP03-21-M01) 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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0543/2021 issued on 31 December 2021,

Having regard to the application submitted by PTC Therapeutics International on 16 December 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 March 2023, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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

Changes to the agreed paediatric investigation plan for vatiquinone, capsule, hard, oral solution, oral use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

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/6850/2023
Amsterdam, 31 March 2023

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001238-PIP03-21-M01

Scope of the application

Active substance(s):

Vatiquinone

Invented name and authorisation status:

See Annex II

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 22 of Regulation (EC) No 1901/2006 as amended, PTC Therapeutics International submitted to the European Medicines Agency on 16 December 2022 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0543/2021 issued on 31 December 2021.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 30 January 2023.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
 - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

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

2. The measures and timelines of the 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 annexes 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	Description
Quality-related studies	Study 1: Development of an oral solution
Non-clinical studies	Not applicable
Clinical studies	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. (PTC743-NEU-005-FA)
Extrapolation, modelling and simulation studies	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	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 March 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.