EMA/427603/2021

European Medicines Agency decision

P/0318/2021

of 11 August 2021

on the agreement of a paediatric investigation plan for vatiquinone (EMEA-001238-PIP02-20) 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 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 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,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 25 June 2021, in accordance with Article 17 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 agreement of a paediatric investigation plan.

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

[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, 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

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

EMA/PDCO/221601/2021 Corr
Amsterdam, 25 June 2021

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

EMEA-001238-PIP02-20

Scope of the application

Active substance(s):

Vatiquinone

Condition(s):

Treatment of mitochondrial disease

Pharmaceutical form(s):

Oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

PTC Therapeutics International Limited

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Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, PTC Therapeutics International 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.

The procedure started on 1 December 2020.

Supplementary information was provided by the applicant on 19 March 2021. The applicant proposed modifications to the paediatric investigation plan and withdrew their request for a deferral.

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.
2. 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 mitochondrial disease

2.1.1. Indication(s) targeted by the PIP

Treatment of seizures in patients with mitochondrial diseases

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)

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	1	Study 2 Randomized, double blind, placebo-controlled study to assess the efficacy and safety of vatiquinone compared to placebo for the treatment of refractory epilepsy in paediatric subjects from birth to less than 18 years of age with mitochondrial disease (PTC743-MIT-001-EP).
Extrapolation, modelling and simulation studies	1	Study 3 Population modelling and simulation study to describe population PK and/or PD of vatiquinone in paediatric patients.
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 October 2023
Deferral for one or more measures contained in the paediatric investigation plan:	No