

Notification of discontinuation of a paediatric development which is covered by an agreed PIP Decisionⁱ

Actives substances(s): Vatiquinone

Latest Decision number(s): 1) P/0318/2021

Corresponding PIP number(s): 1) EMEA-001238-PIP02-20

If the PIP has been submitted as part of a marketing authorisation application in order to comply with the requirements of Article 7 of the Paediatric Regulation (as a condition of the validation of the respective application) and a marketing authorisation was granted based on this application, then there is a legal obligation to complete that PIP. The same applies if there has been a successful post-authorisation application, where the PIP was included in order to comply with the requirements of Article 8 of the Paediatric Regulation.

Please confirm if any of the above applies:

Yes No

If yes, it means that based on the Marketing Authorisation obtained at the end of that initial procedure or the successful post-authorisation application, as applicable, you are obliged to complete that PIP. That obligation cannot be cancelled by a unilateral decision, including by withdrawing the MA. Such PIP must be completed, unless it is modified in agreement with the PDCO by removing all outstanding PIP measures or granting a full product-specific waiver instead (upon relevant circumstances in accordance with the Paediatric Regulation). Non-completion of a binding PIP establishes noncompliance with the requirements of the Paediatric Regulation, which the European Medicines Agency has an obligation to report to the European Commission.

Please note that development of the medicinal product above in the following **condition(s)/indication(s)**:

Treatment of mitochondrial disease

has been discontinued

for the following reason(s): (tick all that apply)

(possible) lack of efficacy in adults

(possible) lack of efficacy in children

(possible) unsatisfactory safety profile in adults

(possible) unsatisfactory safety profile in children

commercial reasons (please specify: PTC made the strategic decision not to continue the development of vatiquinone in this indication and patient population. The reason for premature study termination was not based on safety concerns, but rather without any results suggesting clear clinical efficacy, there does not seem to be a potential benefit-risk to subjects.)

manufacturing / quality problems

other regulatory action (please specify:)

other reason (please specify:)

Please add a brief description (max 2000 characters) of the reason(s) for the discontinuation:

PTC made the strategic decision not to continue the development of vatiquinone in this indication and patient population. The reason for premature study termination was not based on safety concerns, but rather without any results suggesting clear clinical efficacy, there does not seem to be a potential benefit-risk to subjects. Vatiquinone did not meet the primary endpoint of reducing the frequency of observable motor seizures per 28 days during the placebo-controlled, double-blind phase relative to placebo, but directional improvements were observed in subjects with Leigh syndrome. Likewise, there were no statistically significant or clinically relevant treatment-related changes in key and other secondary efficacy endpoints over the 24-week double-blind treatment period or overall treatment period. No new or unexpected safety findings were identified.

Date: 19 January 2026

Contact for inquiries from interested parties: MedInfo PTC

Telephone: +353 (0)1 447 5165

Email: medinfo@ptcbio.com

ⁱ This form will be published to the corresponding decision available on the website of the European Medicines Agency.