Notification of discontinuation of a paediatric development which is covered by an agreed PIP Decision[i]

Actives substances(s): ganaxolone

Latest Decision number(s): 1) P/0183/2024

Corresponding PIP number(s): 1) EMEA-002341-PIP02-23

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 tuberous sclerosis complex

☒ 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:)

☐ 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:

The ganaxolone Phase 3, double-blind, (DB) study in TSC (Study 1042-TSC-3001) did not meet its primary endpoint of percentage change in 28-day TSC-associated seizure frequency. While reductions in seizure frequency favoured the ganaxolone arm, the primary endpoint did not achieve statistical significance. Ganaxolone was generally well-tolerated in this study, with a safety profile consistent with previous clinical trials.

Given these findings, Marinus, the developer of ganaxolone, has made the decision to stop development in TSC and will not be pursuing a registration in this indication. Hence, this request to withdraw the ganaxolone TSC PIP (EMEA-002341-PIP02-23).

Marinus plans to close out the Phase 3 DB study and is evaluating what is needed to stop the ongoing study in paediatric patients (1042-TSC-3002) while maintaining patient safety.

Date:	22 January 2025
Contact for inquiries from interested parties:	Marinus Pharmaceuticals Inc.
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[i] This form will be published to the corresponding decision available on the website of the European Medicines Agency.