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

Actives substances(s): Pamrevlumab

Latest Decision number(s): 1) P/ 0143/2022

Corresponding PIP number(s): 1) EMEA-002979-PIP01-21

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 Duchenne muscular dystrophy

☒ 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 PIP withdrawal at the request of FibroGen management is based on topline data from the Phase 3 LELANTOS-1 and LELANTOS-2 trials. These randomised, double-blind global Phase 3 trials were designed to evaluate the efficacy and safety of pamrevlumab in combination with systemic corticosteroids in patients with either non-ambulatory or ambulatory DMD. Pamrevlumab was generally safe and well tolerated and the majority of treatment emergent adverse events were mild or moderate, however, the studies did not meet the primary endpoints of Performance of the Upper Limb 2.0 (PUL 2.0) score at week 52 compared to baseline (LELANTOS-1) and change in the North Star Ambulatory Assessment (NSAA) total score from baseline to week 52 (LELANTOS-2), respectively. Therefore, FibroGen Inc. has made the decision to discontinue development of pamrevlumab in the indication of DMD.

Date: 31 October 2024

Contact for inquiries from interested parties:

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