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

Actives substances(s): eteplirsen

Latest Decision number(s): 1) P/0286/20191

Corresponding PIP number(s): 1) EMEA-001722-PIP01-14-M02

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: Please see details listed below)

☐ other reason (please specify:)

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

The EU MAA for eteplirsen 30mg/kg was refused by the EMA in 2018. The current focus of the program is the ongoing Study 4658-402 (MIS5ION) for eteplirsen which is looking at higher doses (100mg/kg and 200mg/kg). The development of eteplirsen is currently focused on 4658-402 and this study is not part of the PIP. Once study 4658-402 concludes, the clinical development plan and potential for an MAA resubmission in the EU will be reevaluated by the sponsor. The sponsor proposes to withdraw the existing PIP and to submit a new PIP when these further clinical data are available from Study 4658-402) (e.g. suspension, revocation of M.A.)

Date: 3 February 2026

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