To:

Head of Paediatric Medicines
European Medicines Agency

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

Actives substances(s): Bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (RO7239361)

Invented name:

Latest Decision number(s): 1) P/0043/2019

Corresponding PIP number(s): 1) EMEA-001793-PIP01-15-M03

Date of initial marketing authorisation granted: N/A

Date of authorisation of new indication, pharmaceutical form or route of administration: N/A

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

Treatment of Duchenne Muscular Dystrophy

☒ has been discontinued

☐ has been suspended/put on long-term hold (with possible re-start at a later time)

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:) (e.g. suspension, revocation of M.A.)

☐ other reason (please specify:)

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

On 04 November 2019, Roche made the decision to discontinue the on-going RO7239361 clinical trials WN40226 and WN40227 investigating RO7239361 in Duchenne Muscular Dystrophy (DMD) (PIP measures 3 and 4).

On 05 November 2019, this decision was comminicated to the EMA, including the PDCO. Affected National Authorities were subsquently infomed shortly after.

A pre-planned futility analysis assessing the efficacy of the Phase II/III study WN40227 was conducted after approximately 30% of patients had completed 48 weeks of study drug treatment. The decision to discontinue was based on a very low likelihood of meeting the primary endpoint of change from baseline at Week 48 in North Star Ambulatory Assessment (NSAA) total score.

Due to these results, the company will not be pursuing further clinical development for anti-myostatin adnectin in DMD.

Please note that 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 to the PIP in question:

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.

Name and signature of the PIP contact point: Signature on file

Date: 24/04/2020

Contact for inquiries from interested parties: Roche Registration GmbH

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