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

Actives substances(s): Adeno-associated viral vector serotype 8 containing the human RPGR gene (AAVS-RPGR)

Latest Decision number(s): P/0229/2020

Corresponding PIP number(s): EMEA-002601-PIP0 1-19

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 X-linked retinitis pigmentosa

☒ 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:

Study 1 in the PIP (XIRIUS (NSR-RPGR-01)) was a multi-centre, dose-escalation interventional study of AAV8-RPGR in male subjects from 10 years to less than 18 years and adults with genetically confirmed XLRP. Unfortunately, XIRIUS (NSR-RPGR-01) did not meet its primary endpoint of demonstrating a statistically significant improvement in the proportion of treated study eyes with ≥7 dB improvement from baseline at ≥5 of the 16 central loci of the 10-2 grid assessed by Macular Integrity Assessment (MAIA) microperimetry. This assessment was performed at 12 months and compared to the study eye of patients randomized to the untreated control group. Positive trends were observed across several clinically relevant prespecified secondary endpoints. Following further evaluation of these data, the Sponsor decided to discontinue the BIIB112 program.

Name and signature of the PIP contact point:

Date: 23 March 2026

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.