

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

Actives substances(s): Adeno-associated viral vector serotype 9 containing human sulfamidase gene

Latest Decision number(s): 1) P/0149/2014

Corresponding PIP number(s): 1) EMEA-001502-PIP01-13

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 mucopolysaccharidosis type IIIA (Sanfilippo A syndrome)

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 intracerebroventricular (ICV) administration of AAV9-CAG-coh-SGSH to MPSIIIA patients with at least one missense mutation has demonstrated an acceptable safety and tolerability profile of the product and of the mode of administration (ICV infusion). The thorough immunological evaluation performed does not show evidence of any unexpected response to the AAV9 nor to the SGSH. The exhaustive biomarker assessment indicates directionality in the reduction of Heparan Sulfate in plasma and cerebrospinal fluid, although a more pronounced reduction is required to bring it closer to the normal values. In relation to efficacy, the results do not suggest any benefits in behavioural skills or cognitive function and the assessment of brain volume also showed a generalized reduction, both in the cortical and subcortical grey matter volume, efficacy profiles that would be comparable to the natural history of the disease.

Date: 20 January 2026

Contact for inquiries from interested parties:

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¹ This form will be published to the corresponding decision available on the website of the European Medicines Agency.