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

Actives substances(s): Autologous CD34+ cells transduced with lentiviral vector containing the human Wiskott Aldrich

Latest Decision number(s): 1) P/0260/2016

Corresponding PIP number(s): 1) EMEA-000786-PIP01-09-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 Wiskott-Aldrich 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: Strategic reason)

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

Results of the phase I/II studies in U.K. (EUDRACT n° 2007-004308-11) and France (EUDRACT n° 2009-011152-22) in ten patients showed sustained clinical benefits of the product with an acceptable safety profile. Similar results were observed in a U.S. clinical trial (NCT01410825) with 5 treated patients.

However, due to the extreme rarity of the Wiskott-Aldrich Syndrome and considering that another gene therapy (Etuvetidigene autotemcel, Fondazione Telethon) is being developed in the indication, the applicant has decided to stop the development of the product.

European participants who received the gene therapy are followed-up for 15 years in the ongoing long term follow-up study (EudraCT N°2014-000274-20).

References:

- Hacein-Bey Abina, Salima et al. "Outcomes following gene therapy in patients with severe Wiskott-Aldrich syndrome." JAMA vol. 313,15 (2015): 1550-63. doi:10.1001/jama.2015.3253

- Labrosse, Roxane et al. "Outcomes of hematopoietic stem cell gene therapy for Wiskott-Aldrich syndrome." Blood vol. 142,15 (2023): 1281-1296. doi:10.1182/blood.2022019117

- Magnani, A et al. "Long-term safety and efficacy of lentiviral hematopoietic stem/progenitor cell gene therapy for Wiskott-Aldrich syndrome." Nature medicine vol. 28,1 (2022): 71-80. doi:10.1038/s41591-021-01641-x

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

Date: 2 May 2024

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l be published to the corresponding decision available on the website of the European Medicines