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): Susoctocog alfa (INN)

Invented name: OBIZUR 500 U powder and solvent for solution for injection

Latest Decision number(s): 1) P/0381/2017

Corresponding PIP number(s): 1) EMEA-000753-PIP02-16

Date of initial marketing authorisation granted: 11.11.2015

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)**:

Condition: Treatment of congenital haemophilia A with antibodies (inhibitors) to human factor VIII

Indication targeted by the PIP: Peri-operative management in patients with congenital haemophilia A with antibodies (inhibitors) to human factor VIII.

☒ 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:

Obizur is a medicine authorized for used as the treatment of bleeding episodes in adults with acquired haemophilia, a bleeding disorder caused by the spontaneous development of antibodies that inactivate factor VIII. Factor VIII is one of the proteins needed for normal clotting of the blood.

On 04-Jan-2017, the MAH submitted the EMEA-000753-PIP02-16, in line with Article 7 and Article 8 of the Paediatric Regulation with the intention to submit an application for a new indication (Peri-operative management in patients with congenital haemophilia A with antibodies (inhibitors) to human FVIII) in both paediatric population (from 12 to 18 years of age) and adult population. This PIP has as single measure the CHAWI study 241502 (Eudract No. 2015-005521-39) and can be reference with the decision number P/0381/2017.

The MAH would like to discontinue the PIP EMEA-000753-PIP02-16 as a result of stoping the CHAWI study 241502 (Eudract No. 2015-005521-39). The study was placed on temporary halt in January 2020 to allow time for the Sponsor to determine the feasibility and specifics of an anticipated protocol amendment to modify the enrolment criteria and improve safety oversight measures. After internal assessment and consideration, the Sponsor has determined that the risks of anamnestic reaction outweigh the benefit in the study population and have decided to terminate the study. In addition, during the OBIZUR 5-year Renewal of Marketing Authorization process (EMEA/H/C/002792/R/0033), the European Medicines Agency (EMA) recommended that the MAH should evaluate the risk for anamnestic reaction in the ongoing clinical trial in the indication of congenital haemophilia A with inhibitors (CHAWI) and conclude on a potential premature termination of the study because of undue risks to the participants.

This post-authorisation application for a new indication has not been submitted and is not intended to be submitted for neither adult nor paediatric population due to safety concerns. The MAH would also like to note that in the CHAWI study no paediatric patient was treated with susoctocog alfa so far.

The MAH will continue the PIP EMEA-000753-PIP01-11; P/0040/2012 with the waiver for 0-18 years, for the treatment of bleeding episodes in patients with acquired haemophilia caused by antibodies to factor VIII.

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: 1.6.2021

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