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

Actives substances(s): r (1R,3S,5R)-2-(2-(3-acetyl-5-(2-methylpyrimidin-5-yl)-1H-indazol-1-yl)acetyl)- N-(6-bromo-3-methylpyridin-2-yl)-5-methyl-2-azabicyclo[3.1.0]hexane-3-carboxamide

Latest Decision number(s): 1) P/0324/2021

Corresponding PIP number(s): 1) EMEA-002863-PIP01-20

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)**:

Paroxysmal Nocturnal Haemoglobinuria

☒ 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:

Alexion has decided to terminate the ALXN2050 program in Paroxysmal Nocturnal Haemoglobinuria, as such no marketing authorisation application will be submitted by Alexion for ALXN2050 (INN: vemircopan).

The decision was based off of interim analysis results of Phase 2 study that showed positive proof of concept for vemircopan in patients with PNH with topline data showing improvement in markers of hemolysis and QoL, as well as an acceptable overall safety profile. However, vemircopan's ability to appropriately control intravascular hemolysis has not been adequately shown due to significantly increased rates of breakthrough hemolysis and LDH excursions (LDH excursions is defined by LDH values > 2XULN). The decision not to progress the ALXN2050 program in PNH was not related to any safety signals.

Name and signature of the PIP contact point:	Signature on file Regulatory Affairs Specialist
Date:	24 May 2024
Contact for inquiries from interested parties:	Alexion Europe S.A.S
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[i] This form will be published to the corresponding decision available on the website of the European Medicines Agency.