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General AML

FDA grants Orphan Drug Designation to PCM-075 for the treatment of AML

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On 9th October 2017, the US Food and Drug Administration (FDA) granted Orphan Drug Designation to PCM-075, a Polo-like Kinase 1 (PLK1) inhibitor, for the treatment of patients with Acute Myeloid Leukemia (AML).[1] This comes after the FDA recently approved the Investigational Drug application for PCM-075, which the AGP reported in July 2017.

PLK1 is expressed at very low levels in most normal tissues. Nevertheless, it has been found to be overexpressed in multiple AML cell lines and also in leukemia blasts from AML patients. Overexpression of PLK1 in AML patients has been reported to be associated with poor prognosis in these group of patients.[2] PCM-075 is an oral, highly selective Adenosine Tri-phosphate (ATP) competitive inhibitor which inhibits the PLK1 enzyme thereby inducing cell cycle arrest and apoptosis in cancer cells.[1,3]

Currently, PCM-075 is being evaluated in a phase Ib/II study (NCT03303339), which is assessing the safety and efficacy of PCM-075 in combination with decitabine or low-dose cytarabine in adult patients with AML, who are in relapse or refractory. The primary outcomes of the phase Ib portion of the study were the number of patients with Dose Limiting Toxicity (DLTs) and Adverse Events (AEs). The primary outcome of the phase II portion of the study is the rate of Complete Response (CR) plus CR with Incomplete Blood Count Recovery (CRi).

References

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2. Brandwein J. M. Targeting polo-like kinase 1 in acute myeloid leukemia. Ther Adv Hematol. 2015 Apr; 6(2): 80–87. DOI: 10.1177/2040620715571077.
3. NCI Drug Dictionary: Polo-like kinase 1 inhibitor NMS-1286937. https://www.cancer.gov/publications/dictionaries/cancer-drug?cdrid=660210 [Accessed 2017 Oct 10].