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General AML

AB8939 receives orphan drug designation for acute myeloid leukemia from the FDA

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The U. S. Food and Drug Administration (FDA) have granted orphan drug designation to AB8939 (AB Science) for the treatment of patients with acute myeloid leukemia (AML).[1] AB8939 was shown to offer therapeutic benefit in refractory/relapsed AML.

AB8939 is a novel tubulin inhibitor which seems able to overcome drug resistance induced by P-glycoprotein (Pgp). It acts by binding to the colchicine-binding site of the beta-subunit of tubulin, leading to apoptosis *via* mitotic cell cycle arrest at the G2/M phase. Unlike vinca alkaloids (e.g. vincristine or vinblastine), AB8939 is not deactivated by myeloperoxidase and has been shown to have a strong anti-proliferative effect in refractory/resistant AML cell lines.[2] Data on this will be presented at the 61st Annual Meeting and Exposition of the American Society of Hematology which will take place in Orlando, U.S., on the 7–10th December 2019.

Orphan drug status grants a company with a seven-year exclusivity period for marketing and allows the company to apply for funding for phase I and II clinical trials, providing some financial benefits and a quicker regulatory process further down the line.

References

1. Global newswire. AB Science announces that AB8939 receives Orphan Drug Designation for Acute Myeloid Leukemia from FDA. https://www.globenewswire.com/news-release/2019/11/07/1942829/0/en/AB-Science-announces-that-AB8939-receives-Orphan-Drug-Designation-for-Acute-Myeloid-Leukemia-from-FDA.html [Accessed 2019 Nov 12]
2. Humbert M. *et al.*, 2075 Anticancer Activity of a Highly Potent Small Molecule Tubulin Polymerization Inhibitor, AB8939; 2019 Dec 7. Oral Abstract #2075. ASH 61st Annual Meeting and Exposition, Orlando, FL