AMLGlobalPortal

IDH1/2

FDA grants ivosidenib (Tibsovo®), in combination with azacitidine, Breakthrough Therapy Designation for the treatment of patients with newly diagnosed *IDH1*-mutant AML

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On 26 March 2019, the US Food and Drug Administration (FDA) granted a Breakthrough Therapy Designation to ivosidenib, a first-in-class, oral, selective inhibitor of mutations in isocitrate dehydrogenase-1 (*IDH1*), in combination with azacitidine, for the treatment of patients with newly diagnosed *IDH1*-mutant acute myeloid leukemia (AML). This comes after ivosidenib received FDA approval in July 2018 for the treatment of patients with *IDH1*-mutant relapsed/refractory AML.

The Breakthrough Therapy Designation for ivosidenib and azacitidine was based on the results from a phase I/II study, which were presented at the Acute Leukemias XVII Biology and Treatment Strategies biennial symposium. In the ivosidenib and azacitidine arm, the overall response rate was 78%, with a complete response rate of 57%. Moreover, the median duration of response in the ivosidenib and azacitidine arm was not reached (95% CI, 7.7–not reached).

According to the drug manufacturers, the combination of ivosidenib and azacitidine for patients with newly diagnosed AML "has the potential to be a compelling treatment option", because at present in the front-line setting there are "no approved options specifically for patients with an *IDH1* mutation."

References

1. GlobalNewswire. Agios receives FDA Breakthrough Therapy Designation for TIBSOVO® (ivosidenib) in combination with azacitidine for the treatment of newly diagnosed acute myeloid leukemia (AML) with an IDH1 mutation in adult patients ineligible for intensive chemotherapy. https://www.globenewswire.com/news-release/2019/03/26/1772973/0/en/Agios-Receives-FDA-Breakthrough-Therapy-Designation-for-TIBSOVO-ivosidenib-in-Combination-with-Azacitidine-for-the-Treatment-of-Newly-Diagnosed-Acute-Myeloid-Leukemia-AML-with-an-I.html [Accessed 2019 Apr 02]