New Treatment for Relapsed or Refractory Acute Myeloid Leukemia

A clinical trial showed that 21 percent of participants achieved complete remission.

November 28, 2018 By Food and Drug Administration (FDA)

FDA approves treatment for adult patients who have relapsed or refractory acute myeloid leukemia (AML) with a certain genetic mutation

The U.S. Food and Drug Administration today approved Xospata (gilteritinib) tablets for the treatment of adult patients who have relapsed or refractory acute myeloid leukemia (AML) with a FLT3 mutation as detected by an FDA-approved test. The FDA also approved an expanded indication for a companion diagnostic, to include use with Xospata. The LeukoStrat CDx FLT3 Mutation Assay, developed by Invivoscribe Technologies, Inc., is used to detect the FLT3 mutation in patients with AML.

“Approximately 25 to 30 percent of patients with AML have a mutation in the FLT3 gene. These mutations are associated with a particularly aggressive form of the disease and a higher risk of relapse,” said Richard Pazdur, MD, director of the FDA’s Oncology Center of Excellence and acting director of the Office of Hematology and Oncology Products in the FDA’s Center for Drug Evaluation and Research. “Xospata targets this gene and is the first drug to be approved that can be used alone in treating patients with AML having a FLT3 mutation who have relapsed or who don’t respond to initial treatment.”

AML is a rapidly progressing cancer that crowds out normal cells in the bone marrow and bloodstream, resulting in low numbers of normal blood cells and a continuous need for transfusions. The National Cancer Institute estimates that approximately 19,520 people will be diagnosed with AML this year; approximately 10,670 patients with AML will die of the disease in 2018.

The efficiency of Xospata was studied in a clinical trial of 138 patients with relapsed or refractory AML having a confirmed FLT3 mutation. Twenty-one percent of patients achieved complete remission (no evidence of disease and full recovery of blood counts) or complete remission with partial hematologic recovery (no evidence of disease and partial recovery of blood counts) with treatment. Of the 106 patients who required red blood cell or platelet transfusions at the start of treatment with Xospata, 31 percent became transfusion-free for at least 56 days.

Common side effects reported by patients in clinical trials were muscle and joint pain

(myalgia/arthralgia), fatigue and elevated liver enzymes (liver transaminase). Health care providers are advised to monitor patients for posterior reversible encephalopathy syndrome (a syndrome characterized by headache, confusion, seizures and visual loss), prolonged QT interval (a heart rhythm condition that can potentially cause fast, chaotic heartbeats) and pancreatitis (inflammation in the pancreas). Rare cases of differentiation syndrome (symptoms of which may include fever, cough, trouble breathing, fluid around the lungs or heart, rapid weight gain, swelling, and renal or hepatic dysfunction) have been seen in patients taking Xospata. Women who are pregnant or breastfeeding should not take Xospata because it may cause harm to a developing fetus or newborn baby.

The FDA granted this application Fast Track and Priority Review designation. Xospata also received Orphan Drug designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The FDA granted the approval of Xospata to Astellas Pharma.

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