FDA News Release

FDA approves new targeted treatment for relapsed or refractory acute myeloid leukemia

For Immediate Release

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Release

The U.S. Food and Drug Administration today approved Idhifa (enasidenib) for the treatment of adult patients with relapsed or refractory acute myeloid leukemia (AML) who have a specific genetic mutation. The drug is approved for use with a companion diagnostic, the RealTime IDH2 Assay, which is used to detect specific mutations in the IDH2 gene in patients with AML.

“Idhifa is a targeted therapy that fills an unmet need for patients with relapsed or refractory AML who have an IDH2 mutation,” said Richard Pazdur, M.D., 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. “The use of Idhifa was associated with a complete remission in some patients and a reduction in the need for both red cell and platelet transfusions.”

AML is a rapidly progressing cancer that forms in the bone marrow and results in an increased number of abnormal white blood cells in the bloodstream and bone marrow. The National Cancer Institute at the National Institutes of Health estimates that approximately 21,380 people will be diagnosed with AML this year; approximately 10,590 patients with AML will die of the disease in 2017.

Idhifa is an isocitrate dehydrogenase-2 inhibitor that works by blocking several enzymes that promote cell growth. If the IDH2 mutation is detected in blood or bone marrow samples using the RealTime IDH2 Assay, the patient may be eligible for treatment with Idhifa.

The efficacy of Idhifa was studied in a single-arm trial of 199 patients with relapsed or refractory AML who had IDH2 mutations as detected by the RealTime IDH2 Assay. The trial measured the percentage of patients with no evidence of disease and full recovery of blood counts after treatment (complete remission or CR), as well as patients with no evidence of disease and partial recovery of blood counts after treatment (complete remission with partial hematologic recovery or CRh). With a minimum of six months of treatment, 19 percent of patients experienced CR for a median 8.2 months, and 4 percent of patients experienced CRh for a median 9.6 months. Of the 157 patients who required transfusions of blood or platelets due to AML at the start of the study, 34 percent no longer required transfusions after treatment with Idhifa.

Common side effects of Idhifa include nausea, vomiting, diarrhea, increased levels of bilirubin (substance found in bile) and decreased appetite. Women who are pregnant or breastfeeding should not take Idhifa because it may cause harm to a developing fetus or a newborn baby.

The prescribing information for Idhifa includes a boxed warning that an adverse reaction known as differentiation syndrome can occur and can be fatal if not treated. Sign and symptoms of differentiation syndrome may include fever, difficulty breathing (dyspnea), acute respiratory distress, inflammation in the lungs (radiographic pulmonary infiltrates), fluid around the lungs or heart (pleural or pericardial effusions), rapid weight gain, swelling (peripheral edema) or liver (hepatic), kidney (renal) or multi-organ dysfunction. At first suspicion of symptoms, doctors should treat patients with corticosteroids and monitor patients closely until symptoms go away.

Idhifa was granted **Priority Review (/ForPatients/Approvals/Fast/ucm405405.htm)** designation, under which the FDA's goal is to take action on an application within six months where the agency determines that the drug, if approved, would significantly improve the safety or effectiveness of treating, diagnosing or preventing a serious condition. Idhifa also received **Orphan Drug (/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/default.htm)** designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The FDA granted the approval of Idhifa to Celgene Corporation. The FDA granted the approval of the RealTime IDH2 Assay to Abbott Laboratories.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

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Related Information

- **FDA: Office of Hematology and Oncology Products (/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm091745.htm)**
- **FDA: Approved Drugs: Questions and Answers (/Drugs/ResourcesForYou/Consumers/ucm054420.htm)**
- **FDA: Fast Track, Breakthrough Therapy, Accelerated Approval, and Priority Review (/ForPatients/Approvals/Fast/default.htm)**
- **FDA: Companion Diagnostics (/MedicalDevices/ProductsandMedicalProcedures/InVitroDiagnostics/ucm407297.htm)**
- **NCI: Adult AML (https://www.cancer.gov/types/leukemia/patient/adult-aml-treatment-pdq)**

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