Idhifa approved for some with acute myeloid leukemia

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(HealthDay)—Idhifa (enasidenib) has been approved by the U.S. Food and Drug Administration to treat adults with a specific genetic mutation that leads to relapsed or refractory acute myeloid leukemia (AML).

The mutation in the IDH2 gene can be diagnosed with a newly approved companion diagnostic, the RealTime IDH2 Assay, the agency said in a news release Tuesday.

"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," said Dr. Richard Pazdur, director of the FDA's Oncology Center of Excellence.

AML is a rapidly progressing cancer that begins in the bone marrow and causes an abnormally high number of white blood cells. More than 21,000 people in the United States are projected to be diagnosed with the disease this year, and more than 10,000 are likely to die from it, the U.S. National Cancer Institute estimates.

Idhifa is designed to block enzymes that foster cell growth. The drug was clinically evaluated in a trial of nearly 200 people with relapsed or refractory AML whose IDH2 mutations were detected by the newly approved diagnostic. After a minimum of six months of treatment, 34 percent of trial participants no longer required blood transfusions, the FDA said.

Common side effects of the drug included nausea, vomiting, diarrhea, elevated levels of bilirubin (a byproduct of the liver as red blood cells are broken down) and loss of appetite.

The drug's label will contain a boxed warning of a deadly side effect called differentiation syndrome, with possible symptoms including fever, difficulty breathing, lung inflammation and rapid weight gain .

Women who are pregnant or breast-feeding shouldn't take the drug, the agency warned, as it could harm a developing fetus or newborn.

Idhifa is produced by Celgene Corp., in Summit, N.J. The RealTime IDH2 Assay is produced by Chicago-based Abbott Laboratories.

More information: The FDA has more about these approvals.