FDA News Release

FDA approves new treatment for adults with relapsed or refractory acute lymphoblastic leukemia

For Immediate Release

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Release

The U.S. Food and Drug Administration today approved Besponsa (inotuzumab ozogamicin) for the treatment of adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL).

“For adult patients with B-cell ALL whose cancer has not responded to initial treatment or has returned after treatment, life expectancy is typically low,” 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. “These patients have few treatments available and today’s approval provides a new, targeted treatment option.”

B-cell precursor ALL is a rapidly progressing type of cancer in which the bone marrow makes too many B-cell lymphocytes, an immature type of white blood cell. The National Cancer Institute estimates that approximately 5,970 people in the United States will be diagnosed with ALL this year and approximately 1,440 will die from the disease.

Besponsa is a targeted therapy that is thought to work by binding to B-cell ALL cancer cells that express the CD22 antigen, blocking the growth of cancerous cells.

The safety and efficacy of Besponsa were studied in a randomized trial of 326 patients with relapsed or refractory B-cell ALL who had received one or two prior treatments. Patients were randomized to receive treatment with Besponsa or an alternative chemotherapy regimen. The trial measured the percentage of patients with no evidence of disease and full recovery of blood counts after treatment (complete remission or CR). Of the 218 evaluated patients, 35.8 percent who received Besponsa experienced CR for a median 8.0 months; of the patients who received alternative chemotherapy, 17.4 percent experienced CR for a median 4.9 months.

Common side effects of Besponsa include low levels of platelets (thrombocytopenia), low levels of certain white blood cells (neutropenia, leukopenia), infection, low levels of red blood cells (anemia), fatigue, severe bleeding (hemorrhage), fever (pyrexia), nausea, headache, low levels of white blood cells with fever (febrile neutropenia), liver damage (transaminases and/or gamma-glutamyltransferase increased), abdominal pain and high levels of bilirubin in the blood (hyperbilirubinemia).

The prescribing information for Besponsa includes a boxed warning that severe liver damage (hepatotoxicity), including blockage of veins in the liver (veno-occlusive disease [VOD] or sinusoidal obstruction syndrome), occurred in some patients who took Besponsa. If hepatotoxicity occurs, doctors should pause treatment or reduce the dose of Besponsa. If VOD occurs, patients should stop taking Besponsa and be given standard VOD treatment, if severe.The boxed warning also includes an increased risk of death for patients who take Besponsa after receiving a certain type of stem cell transplant.

Other serious side effects of Besponsa include a decrease in blood cell and platelet production (myelosuppression), infusion-related reactions and problems with the heart's electrical pulses (QT interval prolongation). Women who are pregnant or breastfeeding should not take Besponsa because it may cause harm to a developing fetus or a newborn baby.

The FDA granted this application **Priority Review (/ForPatients/Approvals/Fast/ucm405405.htm)** and **Breakthrough Therapy (/ForPatients/Approvals/Fast/ucm405397.htm)** designations. Besponsa also received **Orphan Drug (/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/TipsforApplyingforOrphanProductDesignation/default.htm)** designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The FDA granted the approval of Besponsa to Pfizer Inc.

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)**
- **NCI: Adult ALL (https://www.cancer.gov/types/leukemia/patient/adult-all-treatment-pdq)**

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