FDA News Release

FDA approves first treatment for certain types of poor-prognosis acute myeloid leukemia

For Immediate Release

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Release

The U.S. Food and Drug Administration today approved Vyxeos for the treatment of adults with two types of acute myeloid leukemia (AML): newly diagnosed therapy-related AML (t-AML) or AML with myelodysplasia-related changes (AML-MRC). Vyxeos is a fixed-combination of chemotherapy drugs daunorubicin and cytarabine.

“This is the first approved treatment specifically for patients with certain types of high-risk AML,” 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. “Vyxeos combines two commonly used chemotherapies into a single formulation that may help some patients live longer than if they were to receive the two therapies separately.”

AML is a rapidly progressing cancer that forms in the bone marrow and results in an increased number of white blood cells in the bloodstream. 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. T-AML occurs as a complication of chemotherapy or radiation in approximately 8 to10 percent of all patients treated for cancer within an average of five years after treatment. AML-MRC is characterized by a history of certain blood disorders and other significant mutations within cancer cells. Patients with t-AML or AML-MRC have very low life expectancies.

The safety and efficacy of Vyxeos were studied in 309 patients with newly diagnosed t-AML or AML-MRC who were randomized to receive Vyxeos or separately administered treatments of daunorubicin and cytarabine. The trial measured how long patients lived from the date they started the trial (overall survival). Patients who received Vyxeos lived longer than patients who received separate treatments of daunorubicin and cytarabine (median overall survival 9.56 months vs. 5.95 months).

Common side effects of Vyxeos include bleeding events (hemorrhage), fever with low white blood cell count (febrile neutropenia), rash, swelling of the tissues (edema), nausea, inflammation of the mucous membranes (mucositis), diarrhea, constipation, musculoskeletal pain, fatigue, abdominal pain, shortness of breath (dyspnea), headache, cough, decreased appetite, abnormal heart rhythm (arrhythmia), lung infection (pneumonia), blood infection (bacteremia), chills, sleep disorders and vomiting.

Patients who have a history of serious hypersensitivity to daunorubicin, cytarabine or any component of the formulation should not use Vyxeos. Patients taking Vyxeos should be monitored for hypersensitivity reactions and decreased cardiac function. Vyxeos has been associated with serious or fatal bleeding events. Daunorubicin has been associated with severe damage (necrosis) where the drug leaks into the skin and subcutaneous tissue from the intravenous infusion (extravasation). Women who are pregnant or breastfeeding should not take Vyxeos, because it may cause harm to a developing fetus or a newborn baby.

The prescribing information for Vyxeos includes a boxed warning not to interchange Vyxeos with other daunorubicin- and/or cytarabine-containing products.

The FDA granted this application **Priority Review (/ForPatients/Approvals/Fast/ucm405405.htm)** and **Breakthrough Therapy (/ForPatients/Approvals/Fast/ucm405397.htm)** designations. Vyxeos 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 Vyxeos to Jazz Pharmaceuticals.

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, Priority Review (/ForPatients/Approvals/Fast/default.htm)**
- **NCI: Adult AML (https://www.cancer.gov/types/leukemia/patient/adult-aml-treatment-pdq)**

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