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FDA approves Defitelio to treat hepatic veno-occlusive disease in adults, children

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The U.S. Food and Drug Administration today approved Defitelio (defibrotide sodium) to treat adults and children who develop hepatic veno-occlusive disease (VOD) with additional kidney or lung abnormalities after they receive a stem cell transplant from blood or bone marrow called hematopoietic stem cell transplantation (HSCT). This is the first FDA-approved therapy for treatment of severe hepatic VOD, a rare and life-threatening liver condition.

HSCT is a procedure performed in some patients to treat certain blood or bone marrow cancers. Immediately before an HSCT procedure, a patient receives chemotherapy. Hepatic VOD can occur in patients who receive chemotherapy and HSCT. Hepatic VOD is a condition in which some of the veins in the liver become blocked, causing swelling and a decrease in blood flow inside the liver, which may lead to liver damage. In the most severe form of hepatic VOD, the patient may also develop failure of the kidneys and lungs. Fewer than 2 percent of patients develop severe hepatic VOD after HSCT, but as many as 80 percent of patients who develop severe hepatic VOD do not survive.

"The approval of Defitelio fills a significant need in the transplantation community to treat this rare but frequently fatal complication in patients who receive chemotherapy and HSCT," said Richard Pazdur, M.D., director of the Office of Hematology and Oncology Products in the FDA's Center for Drug Evaluation and Research.

The efficacy of Defitelio was investigated in 528 patients treated in three studies: two prospective clinical trials and an expanded access study. The patients enrolled in all three studies had a diagnosis of hepatic VOD with liver or kidney abnormalities after HSCT. The studies measured the percentage of patients who were still alive 100 days after HSCT (overall survival). In the three studies, 38 to 45 percent of patients treated with Defitelio were alive 100 days after HSCT. Based on published reports and analyses of patient-level data, the expected survival rates 100 days after HSCT would be 21 to 31 percent for patients with severe hepatic VOD who received only supportive care or interventions other than Defitelio.

The most common side effects of Defitelio include abnormally low blood pressure (hypotension), diarrhea, vomiting, nausea and nosebleeds (epistaxis). Serious potential side effects of Defitelio that were identified include bleeding (hemorrhage) and allergic reactions. Defitelio should not be used in patients who are having bleeding complications or who are taking blood thinners or other medicines that reduce the body's ability to form clots.

The FDA granted the Defitelio application priority review status, which facilitates and expedites the development and review of certain drugs in light of their potential to benefit patients with serious or life-threatening conditions. Defitelio also received orphan drug designation, which provides incentives such as tax credits, user fee waivers and eligibility for exclusivity to assist and encourage the development of drugs for rare diseases.

Defitelio is marketed by Jazz Pharmaceuticals based in Palo Alto, California.

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