

Title: Defibrotide for Patients with Hepatic Veno-Occlusive Disease (VOD): A Treatment IND Study

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Synopsis:

This research study is evaluating an investigational drug called Defibrotide as a possible treatment for hepatic VOD. Defibrotide is not approved by the U.S. Food and Drug Administration for use outside of a research study.

Hepatic VOD can be a complication of bone marrow and/or stem cell transplant or high-dose chemotherapy, which may be life threatening. This condition is characterized by damage to blood vessels in the liver and surrounding liver cells, leading to pain, fluid retention and abnormal liver function. Severe VOD has been associated with high mortality.

Defibrotide is a complex form of DNA (a component of genetic material) derived from porcine, or pig, tissue. It is prepared in such a way that there is no known risk of microbial infection from the drug. When administered to humans, Defibrotide appears to interact with surface structures, called receptors, on blood vessels that are important in controlling inflammation, blood clotting and scar formation (fibrosis). It is believed that Defibrotide works by inhibiting or reversing blood clotting and blood vessel inflammation in several ways. This drug has been used for kidney graft rejection after kidney transplant, blood vessel inflammation (vasculitis), and related disorders, and information from those other research studies suggests that Defibrotide may help to resolve hepatic VOD in this research study.