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Title: *Identification of Pediatric Stem Cell Transplant Patients at High Risk for Hepatic Veno-Occlusive Disease*

Abstract

Introduction:

Venoocclusive disease (VOD) is a complication of hematopoietic stem cell transplants (HSCT) that may cause liver failure. In the United States, defibrotide is the only approved agent for the treatment of VOD; however, there is no indication for prophylactically treating patients with high risk factors undergoing HSCT. The purpose of this study is to retrospectively analyze patients who were diagnosed with VOD and determine if they met the criteria for defibrotide prophylaxis as stated by the British Society for Blood and Marrow Transplant (BSBMT).

Methods:

Patients included in this study were treated for hematopoietic stem cell transplant at Cardinal Glennon Children's Hospital (CGCH) from January 1, 2010 through May 6, 2019. Data was collected retrospectively by looking at patient risk factors prior to transplant and following patients for up to day +100 post-transplant for development of VOD.

Results:

This study shows potential benefit of prophylactically treating VOD with a number needed to treat of 2. Every patient that developed VOD met criteria for prophylaxis according to the BSBMT. Risk factors in this study that were prevalent in patients who developed VOD included: conditioning treatment with Busulfan, baseline liver disease, umbilical cord transplants, patients diagnosed with SCID or myeloproliferative disease, and transplants with an unmatched donor.

Conclusion:

Defibrotide should not only be approved for the treatment of VOD but for prophylaxis as well. This study shows that health care providers can predict which patients are at risk of developing this complication by utilizing the BSBMT prophylaxis criteria. Also, Cardinal Glennon Children's Hospital should start diagnosing VOD with EBMT criteria since it is the most inclusive.