



Case Presentation

Compiled Date: June 11, 2026

Defibrotide Therapy for HSCT-Associated TMA in Pediatric Myelodysplastic Syndrome: Successful Response With Serious Hemorrhagic Complications

Sarah J. Miller, MD^{1*}, Kenji Y. Ito, MD², Rachel P. Adams, MD³

¹Division of Pediatric Hematology and Oncology, Westbridge Children's Hospital, Toronto, Canada;

²Hematopoietic Stem Cell Transplant Program, Westbridge Children's Hospital, Toronto, Canada; ³Department of Pediatric Critical Care, Westbridge University, Toronto, Canada

***Corresponding author:** Sarah J. Miller, MD

Abstract

Background: Transplant-associated thrombotic microangiopathy (TA-TMA) is a potentially life-threatening complication following hematopoietic stem cell transplantation (HSCT). Pediatric patients with high-risk underlying disease may develop severe endothelial injury and multiorgan dysfunction.

Case Presentation: A child with myelodysplastic syndrome developed TA-TMA after allogeneic HSCT, with thrombocytopenia, hemolysis, hypertension, renal dysfunction, and progressive systemic complications. Defibrotide was administered as an investigational/supportive therapeutic strategy in this case. Hematologic and organ parameters subsequently improved, but the treatment course was complicated by clinically significant hemorrhagic events requiring intensive management.

Conclusion: TA-TMA requires rapid recognition and multidisciplinary management. Any use of defibrotide in this setting must account for its potential bleeding risk and should be guided by disease severity, institutional expertise, and contemporary evidence.

Keywords: transplant-associated thrombotic microangiopathy; HSCT; pediatric myelodysplastic syndrome; defibrotide; endothelial injury; hemorrhage

Introduction

TA-TMA is a serious complication of HSCT character by endothelial injury, microangiopathic hemolysis, thrombocytopenia, hypertension, and variable renal, neurologic, pulmonary, and gastrointestinal involvement. Severe disease is associated with substantial morbidity and mortality.

Defibrotide is an endothelial-protective agent used in selected transplant-related settings, most prominently hepatic veno-occlusive disease/sinusoidal obstruction syndrome. Its use for TA-TMA has been investigated but remains dependent on clinical context and evidence. This case illustrates both a potential response and the major concern of hemorrhage.

Case Presentation

A 11-year-old child with high-risk myelodysplastic syndrome underwent allogeneic HSCT. Several weeks after transplantation, the patient developed persistent thrombocytopenia, anemia with laboratory evidence of hemolysis,

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elevated lactate dehydrogenase, hypertension, and worsening renal function. The clinical picture was considered compatible with TA-TMA after exclusion of alternative causes.

Supportive measures and disease-directed transplant management were initiated. Because of progressive organ involvement, defibrotide was introduced within this clinical scenario after multidisciplinary discussion and assessment of bleeding risk.

Over the subsequent treatment period, markers of hemolysis improved and renal and hematologic parameters showed a favorable trend. However, the patient developed serious mucosal and gastrointestinal bleeding requiring transfusion support, close hemodynamic monitoring, and temporary modification of anticoagulant/antiplatelet exposures where applicable. The hemorrhagic complications were managed in the intensive care setting.

Following stabilization, the patient's TA-TMA remained clinically improved in this scenario. Long-term follow-up included monitoring of renal function, blood pressure, hematologic indices, and transplant complications.

Discussion

TA-TMA is driven by endothelial injury and complement activation in a susceptible post-transplant environment. Clinical diagnosis requires recognition of a constellation of findings rather than reliance on a single laboratory marker. Severe disease may involve multiple organs and can progress rapidly.

Management includes removal or modification of contributing exposures when feasible, aggressive supportive care, blood-pressure control, infection management, and consideration of disease-directed therapies according to contemporary diagnostic and treatment frameworks. Complement-directed therapy has become an important approach for selected patients, while other endothelial-targeted agents continue to be evaluated.

Defibrotide has endothelial effects and has been explored in TA-TMA, but bleeding is a clinically important adverse effect. The hemorrhagic complications in this case highlight the need for careful patient selection, monitoring, and multidisciplinary risk-benefit assessment.

Conclusion

Severe TA-TMA after pediatric HSCT requires early recognition and coordinated management. Although defibrotide may have potential therapeutic value in selected settings, significant hemorrhage is an important safety concern. Its use should be individual and guided by current evidence and specialist oversight.

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Citation of this Article

Miller SJ, Ito KY, Adams RP. Defibrotide Therapy for HSCT-Associated TMA in Pediatric Myelodysplastic Syndrome: Successful Response With Serious Hemorrhagic Complications. *Clin Case Rep Image Int*. 2026;10(6):1001-1003.

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