FAST FACTS

Protocol Title: A Phase 2, Prospective, Randomized, Open-Label Study on the Efficacy of Defibrotide Added to Standard of Care Immunoprophylaxis for the Prevention of Acute Graft-versus-Host-Disease in Adult and Pediatric Patients After Allogeneic Hematopoietic Stem Cell Transplant

Target Population: Acute Graft Versus Host Disease (aGvHD)

Summary: This is a study comparing the Defibrotide prophylaxis arm vs standard of care arm for the prevention of aGvHD.

Key Inclusion Criteria:

- Patient must be ≥ 1 Year and < 75 Years of age at screening and undergoing allogeneic HSCT.
- Patient must be diagnosed with acute leukemia in morphologic complete remission (CR1 or CR2) or with MDS with no circulating blasts and with less than 5% blasts in the bone marrow.
- Patient must have planned to receive either a myeloablative or reduced-intensity conditioning regimen and have an unrelated donor who is HLA matched or single-allele mismatched.
- Patient must receive the following medical regimen as part of standard of care immunoprophylaxis for GvHD in either study arm at doses and regimen determined by local institutional guidelines, physician preference, and patient need:
    - MTX or MMF + Calcineurin Inhibitor (CSA or TAC) +/- ATG (ATG use is limited to 30% of patients)
- Graft must be a CD3+ T-cell replete PBSC graft or non-manipulated BM graft.
- Adult patients must be able to understand and sign a written informed consent.
    - For pediatric patients, the parent / legal guardian or representative must be able to understand and sign a written informed consent. Assent, when appropriate, will be obtained according to institutional guidelines.

Key Exclusion Criteria:

- Patient has had a prior autologous or allogeneic HSCT.
- Patient is using or plans to use an investigational agent for the prevention of GvHD.
- Patient is receiving or plans to receive other investigational therapy and/or is enrolled or plans to enroll in a separate clinical study.
- Patient, in the opinion of the investigator, may not be able to comply with the safety monitoring requirements of the study.
- Patient has a psychiatric illness that would prevent the patient or legal guardian or representative from giving informed consent and/or assent.
- Patient has a serious active disease or co-morbid medical condition, as judged by the investigator, which would interfere with the conduct of this study.
- Patient is pregnant or lactating and does not agree to stop breastfeeding.
- Any other condition that would cause a risk to the patient if he/she participated in the trial.
- Patient has a known history of hypersensitivity to defibrotide or any of the excipients.

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For additional information: **https://clinicaltrials.gov/ct2/show/NCT03339297**

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