

MT2013-31:Allogeneic Hematopoietic Cell Transplantation for Inherited Metabolic Disorders and Severe Osteopetrosis following Conditioning with Busulfan (Therapeutic Drug Monitoring), Fludarabine +/- ATG

Status: Recruiting

Eligibility Criteria

Conditions & Interventions

Keywords:

Clinics and Surgery Center (CSC)

More Information

Description: To evaluate the ability to achieve high-level donor hematopoietic engraftment (defined as neutrophil recovery by Day +42 post-transplant and $\geq 80\%$ donor cells on the myeloid fraction of peripheral blood at Day +100 post-transplant) using related and unrelated BM, PBSC, or UCB grafts following a reduced intensity conditioning regimen based on targeted-exposure busulfan, fludarabine +/- serotherapy in patients with inherited metabolic disorders and severe osteopetrosis.

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IRB

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