PROTOCOL SYNOPSIS

Hematopoietic Cell Transplant and Gene Therapy for Non-Malignant Blood Disorders Biobank Resource (the 'HOPE Resource')

Co-Principal Investigators:	**Leslie Kean, M.D., PhD and Jane Churpek, M.D. MS**
Study Design:	This is a prospective, multicenter, study that will establish a repository of biospecimens and clinical data from patients undergoing hematopoietic stem cell transplant (HCT) or gene therapy (GT) for treatment of non-malignant blood diseases. The enrollment goal is 375 participants and consenting related donors (approximately 100 are anticipated), accrued over 4 years. Participants will be followed until the end of study, but continued collection of specimens and clinical data may continue to allow up to 15 years of follow-up for all enrolled participants if funds are available.
Eligibility Criteria:	Participants with a diagnosis of Aplastic Anemia Hemoglobinopathies or bone marrow failure undergoing HCT or GT for management of their underlying disease.
Treatment Description:	There is no treatment intervention in this study. Treatment will be at the provider's discretion. Participants will provide blood and other biospecimens pre and post-intervention at specified intervals.
Accrual Objective:	375 participants and approximately 100 related donors will be enrolled from 35 sites
Accrual Period:	The estimated accrual period is 4 years.
Study Duration:	Participants will be followed until the end of study, at a minimum through March 30, 2031, but continued collection of specimens and clinical data may continue to allow up to 15 years of follow-up for all enrolled participants if funds are available. Additional long-term clinical data will be collected through usual procedures of the Center for International Blood and Marrow Transplant Research (CIBMTR).