IRB Proposal: Using the Hematopoietic Cell Transplantation Comorbidity Index In Pediatric Patients Undergoing BMT following busulfan based regimens

A. Study Purpose and Rationale

Bone marrow transplants are done more frequently compared to several decades ago. Ideally discussing prognosis with patients and their families would happen before transplant. In the adult population, the hematopoietic cell transplantation comorbitidy index (HCT-CI) has been used to predict overall survival and mortality based on patient's co-morbidities prior to transplant. The purpose of our study is to apply the HCT-CI on pediatric patients who received busulfan based conditioning regimens and see if it still predicts survival and mortality.

B. Study Design and Statistical Analysis

We will conduct a retrospective chart review of patients at Morgan Stanley Children's Hospital who underwent bone marrow transplant with busulfan based conditioning regimens only.
We will have two endpoints: transplant related mortality (TRM) and overall survival (OS) at 1 year.
We will use the same scoring system used by Smith et al (Blood 2011).

- **1 point** (HR 1.3 – 2.0): Arrhythmia, CV, IBD, DM, CVA, depression, mild hepatic, obesity, infection
- **2 points** (HR 2.1 – 3.0): Rheumatologic, peptic ulcer, renal, moderate pulmonary
- **3 points** (HR > 3): Severe pulmonary, heart valve disease, prior solid malignancy, moderate/severe hepatic
- Cumulative integer scores: HCT-CI 0 → Low risk; HCT-CI 1-2 → Intermediate risk; HCT-CI ≥3 →High risk

Statistical analysis includes the following: Continuous variables will be summarized by mean standard deviation; categorical variables will be summarized by rates; the Chi-square test / Fisher's exact test was used to compare categorical variables among groups. Probability of TRM and OS at 1y calculated using the Kaplan-Meier estimator. The p-value 0.05 was used as the significant level

C. **Study Subjects**

We will include patients ages 0.1 to 22 years from 1996 to 2016. Our goal is to review at least 200 patients. By choosing patients with busulfan based regimens we aim to have a more homogenous population. We also avoid looking at other regimens that have higher complication rates and can thus skew results.