Design and Implementation of the Sickle Cell Disease Hematopoietic Cell Transplantation Evaluating Long Term and Late Outcomes Registry (STELLAR) to Compare Long Term Outcomes after Hematopoietic Cell Transplantation to that in Siblings without Sickle Cell Disease and in Non-Transplanted Individuals with Sickle Cell Disease

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Abstract

Background There are sparse data on long-term and late effects of hematopoietic cell transplantation (HCT) for sickle cell disease (SCD) Objectives To establish an international registry of long-term outcomes post-HCT for SCD and demonstrate the feasibility of recruitment at a single site in the US. Methods The STELLAR registry is designed to enroll SCD patients [?] 1-year post-HCT, their siblings without SCD, and non-transplanted SCD controls to collect participant self-report of health status and practices using the BMT survivor study surveys, HRQOL using PROMIS 25 or 29, cGVHD using the symptom scale survey, daily pain using an electronic pain diary, economic impact of HCT using the financial hardship survey, and sexual function using PROMIS SexFSv2.0. We also piloted retrieval of clinical data previously submitted to CIBMTR, recorded demographics, height, weight, BP, hip and waist circumference, timed-up-and-go, and handgrip test, and obtained blood for

metabolic screening, gonadal function, fertility potential, and biorepository of plasma, serum, RNA, and DNA. Results Among 100 eligible post-HCT patients, we enrolled 72 participants 9-38 (median 17) years age. We also enrolled 19 siblings 5-32 (median10)years age and 28 non-transplanted SCD controls 4-46 (median 22) years age. Of 119 participants, 73 completed 85 sets of surveys and 41 contributed samples to the biorepository. We successfully piloted retrieval of data submitted to CIBMTR and expanded recruitment to seven US, Canada, UK, and Nigeria sites. Conclusions It is feasible to recruit subjects and conduct study procedures for the STELLAR registry of long-term and late effects of HCT for SCD.

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