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Title: Peripheral blood Stem Cell Collection for Sickle Cell Disease (SCD) Patients using plerixafor

Principal Investigator: John Tisdale, MD

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Detailed description:

Hematopoietic stem cell (HSC) gene therapy is potentially curative for sickle cell disease (SCD);[1] however, options for HSC collection are limited in this population,[2-4] and investigation of the collection, efficiency, and safety of peripheral blood (PB) mobilization with plerixafor from start to finish is needed. Here we describe consistent, safe, and sufficient PB HSC collection and processing after plerixafor mobilization from the greatest number of participants reported to date and the first two-institutional study. Our data suggest plerixafor mobilized HSCs in SCD are enriched for long-term engrafting HSCs, which is not true of HSCs from SCD bone marrow (BM),[5] supporting a paradigm shift in the optimal HSC source for patients with SCD.

This open-label phase I study was sponsored by NHLBI at NIH and was conducted at the NIH Clinical Center and St. Jude Children's Research Hospital (SJCRH) (NCT03226691). All patients provided written informed consent for a protocol approved by each institution's Institutional Review Board. Hydroxyurea (HU) was stopped at least 2 weeks prior to mobilization, and all participants received red blood cell exchange the day prior to mobilization and collection to target <30% sickle hemoglobin (HbS).

Statistics

Adverse events and severe adverse events were collected in accordance with CTCAE grading. Both types of events were broadly separated as pain or non-pain related events. Clinical details on the SAEs were used to determine if stopping rules were met. Spearman's correlation test was used to assess the relationship between baseline and pre-apheresis CD34/μL, total CD34+ cells/kg collected, and total blood volume processed.