

Unrelated Donor Hematopoietic Cell Transplantation for Children with Severe Sickle Cell Disease Using a Reduced Intensity Conditioning Regimen (BMTCTN 0601)

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Synopsis:

The Blood and Marrow Transplant Clinical Trials Network (BMT CTN) develops clinical trials, also called research studies, about bone marrow and cord blood transplantation. The BMT CTN works to find the best treatment options and improve transplant results.

The goal of this study: Bone marrow or cord blood transplantation from unrelated donors is a developing treatment for sickle cell disease (SCD). In the past, most children with SCD who had a transplant received cells from a sister or brother who had the same tissue type (HLA-identical).

This study will look at whether a new type of preparation for transplantation (the combination of drugs that prepares the patient to receive the donor cells) that uses a less intense combination of drugs is better than the standard preparation. It will also look at whether a patient with severe SCD who receives bone marrow or cord blood cells from a person who is not related to them is effective. The results may help doctors learn more about how well this type of preparation and transplantation will cure SCD.