Application Title: A multi-center, prospective, minimal intensity BMT trial for dyskeratosis congenita

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Bone marrow transplantation (BMT) is a life-saving therapy for many blood diseases, but the chemotherapy and radiation typically used in BMT cause life-threatening complications. For patients with the rare disease dyskeratosis congenita (DC) who require BMT to cure their failing blood system, the toxicity and risks of BMT can be even more severe. We are conducting a clinical trial that asks whether DC patients can undergo BMT without using traditional “DNA-damaging” chemotherapy and radiation, in order to reduce the toxicity of the procedure and improve survival. The idea that this might work comes from an emerging understanding of the genetics and biology of DC. So far, a series of DC patients treated at one institution have successfully undergone BMT on this trial, making them the first group to obtain a cure for their failing blood systems without exposure to DNA damaging radiation and chemotherapy. Now, we aim to expand this trial to other major centers in the United States, as the first prospective multi-center BMT trial for DC. The 2016 Million Dollar Bike Ride Pilot Award from Team Josh and the DCO Riders will enable multi-center expansion of this clinical study, which promises to change the approach and outcome of BMT for DC patients.