Nucleoside Therapy in Patients With Telomere Biology Disorders

NCT06817590

Status	RECRUITING
Phase	Phase 1
Sponsor	Suneet Agarwal
Enrollment	36 participants

Key Eligibility Criteria

Inclusion (10)

- Age e 1 year and d 70 years
- Karnofsky performance status e 50 for participants e16 years of age and Lansky performance status e 50 for participants \<16 years of age
- Diagnosis requirement. Participants must meet at least one of the following requirements for a diagnosis of a telomere biology disorder:
- Age-adjusted mean telomere length \< 1%ile in peripheral blood lymphocytes by flow cytometry-fluorescence in situ hybridization (flow-FISH), as reported by a Clinical Laboratory Improvement Amendments (CLIA)-approved laboratory
- OR

... and 5 more (see full listing online)

Exclusion (9)

- Participants must not otherwise be expected to undergo bone marrow transplantation within 6 months of enrollment.
- Participants must not be taking concurrent medications intended to improve hematopoiesis such as androgens or growth factors, including granulocyte colony stimulating factor, erythropoietin, or thrombopoietin mimetics. If any of these therapies were taken previously, patients must wait 30 days after cessation of the therapy before enrollment on this trial.
- Participants must not have chronic diarrhea or an average baseline stool output of more than 4 stools per day.
- Participants must not have gastrointestinal disorders that may impair enteral absorption of dC/dT, such as inflammatory bowel disease or short bowel syndrome.
- Participants must not have chronic kidney disease with an estimated glomerular filtration rate \< 60 mL/min/1.73 m2.

... and 4 more (see full listing online)

Locations (1 total)

Boston Childrens Hospital, Boston, Massachusetts, United States