Low-Dose Sirolimus to Increase Hematopoietic Function in Patients With RUNX1 Familial Platelet Disorder

NCT06261060

Status	RECRUITING
Phase	Phase 2
Sponsor	M.D. Anderson Cancer Center
Enrollment	6 participants

Key Eligibility Criteria

Inclusion (8)

- Participants has provided signed, informed consent before initiation of any study specific procedures
- Aged e18 years at the time of signing the informed consent
- Confirmed P/LP germline RUNX1 variant per ClinGen Myeloid Malignancy Variant Curation Expert Panel (MM-VCEP) RUNX1-specific variant curation rules80
- Participants must be willing to provide bone marrow sample at time of screening and at the end of treatment with sirolimus
- Platelet count of e50,000/µL

... and 3 more (see full listing online)

Exclusion (14)

- Known allergy to sirolimus
- History of lymphoma or other hematologic malignancies
- Uncontrolled bleeding
- Any prior diagnosis of myelodysplastic syndrome or other hematologic malignancy using International Working Group criteria
- Prior treatment with sirolimus or a rapalog, mTOR inhibitor, or B-cell-depleting therapy within 28 days before study day 1

... and 9 more (see full listing online)

Locations (1 total)

MD Anderson Cancer Center, Houston, Texas, United States