Imatinib to Increase RUNX1 Activity in Participants With Germline RUNX1 Deficiency

NCT06090669

Status	RECRUITING
Phase	Phase 1
Sponsor	National Cancer Institute (NCI)
Enrollment	75 participants

Key Eligibility Criteria

Inclusion (24)

- Affected participants must have a confirmed pathogenic or likely pathogenic germline RUNX1 variant by history. ClinGen expert variant curation panel criteria for pathogenicity will be utilized.
- Affected participants must have a history of clinically significant bleeding as defined by history of abnormal ISTH-BAT score, use of anti-bleeding medications (e.g., amicar), history of platelet transfusion, abnormal PFA screen, abnormal TEG, abnormal platelet aggregation or abnormal platelet electron microscopy.
- Bone marrow morphology, flow cytometry and cytogenetics confirmed by the NIH Department of Laboratory Medicine (DLM) at least within 12 months of initiating imatinib.
- TSO500 performed by NCI Lab of Pathology within 12 months of initiating imatinib.
- Substantial GI malabsorption is not suspected.

... and 19 more (see full listing online)

Exclusion (12)

- Participants who are receiving any other investigational agents.
- Participants who received prior hematologic malignancy directed therapy
- Participants receiving medication that would affect platelet number or function (e.g., aspirin and anti-platelet medications
- Participants without access to medical care at home.
- Pregnancy (confirmed with beta-HCG serum or urine pregnancy test performed in females of childbearing potential at screening).

... and 7 more (see full listing online)

Locations (1 total)

National Institutes of Health Clinical Center, Bethesda, Maryland, United States