IMATINIB CLINICAL TRIAL FOR *RUNX1*-FPD PATIENTS

"PHASE 1B STUDY OF IMATINIB TO INCREASE RUNX1 ACTIVITY IN PARTICIPANTS WITH GERMLINE RUNX1 DEFICIENCY"

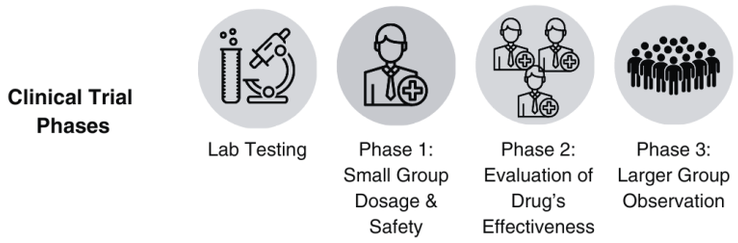
BACKGROUND

The National Cancer Institute (NCI) is conducting a clinical trial to treat *RUNX1*-FPD patients with a medicine called imatinib. The study team believes that this medicine may be effective in increasing RUNX1 protein levels in individuals with *RUNX1*-FPD who have low RUNX1 protein levels.

STUDY PURPOSE

The main focus of this clinical trial is to determine the safest dose of imatinib that can be comfortably tolerated by participants. Earlier laboratory studies have shown that imatinib could help raise RUNX1 protein levels, which is important for making healthy blood cells. This could be particularly helpful for people with *RUNX1*-FPD as it might improve the way their blood cells develop, lowering the risks linked to having too few platelets or platelets that don't work properly.

The findings from this study will help guide whether this medicine should be further studied. The ultimate goal is to determine whether this medicine can improve the health of patients' blood systems and reduce blood cancer risk.



Remember, studying a medicine in a new disease for the first time will take time and multiple steps.

WHO CAN PARTICIPATE

- Male and female adults aged 18 and older diagnosed with *RUNX1*-FPD.
- Healthy volunteers, including family members of *RUNX1*-FPD patients, are also needed to make helpful comparisons.

STUDY PARTICIPANT PROCESS OVERVIEW

Initial Health Screenings: Include physical exams, blood and urine tests, heart function assessments, and a potential bone marrow biopsy at the NIH in Bethesda, Maryland.

Starting Treatment: Following screening and enrollment, participants will begin imatinib treatment, with an estimated 48-hour stay for observation at the NIH.

Continued Treatment: Daily doses of imatinib taken at home. Depending on your assigned study group, the treatment duration may be either 28 days or 84 days.

Regular Check-Ins: Weekly telehealth visits during the first 28 days, followed by visit every other week for participants on the 84-day treatment plan. There will also be an in-person clinic visit on the last day of treatment for both groups.

Ongoing Monitoring: Repeat tests of blood, urine and heart function; an optional bone marrow biopsy may be conducted after treatment at the NIH.

Follow-Up: A telehealth visit 30 days after the final dose of imatinib.

For more details on this study, click here.

CONTACT

For more details about the study, including eligibility and enrollment questions, please contact:

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