MT2022-54 A MULTINATIONAL, MULTICENTER, DOSE ESCALATION STUDY TO EVALUATE THE SAFETY, TOLERABILITY, PHARMACOKINETICS, AND PRELIMINARY ACTIVITY OF FP-045 IN PATIENTS WITH FANCONI ANEMIA (FuschiA Study)

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: Not specified
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- 3 to 25 years old - documented Fanconi anemia by chromosome breakage analysis - women of child-bearing potential and males required to use highly effective birth control

Exclusion Criteria:

- history of any cancer except squamous cell or basal cell carcinoma of the skin or carcinoma in situ of cervix - myelodysplastic syndrome or acute leukemia - history of any significant medical conditions - history of bone marrow or stem cell transplant - see link to clinicaltrials.gov for complete criteria

Conditions & Interventions

Interventions:
Drug: FP-045
Conditions:
Blood Disorders, Rare Diseases
Keywords:
Fanconi Anemia

More Information

Description: The purpose of this research study is to determine the best dose of FP-045 for Fanconi anemia pediatric and adolescent participants. The study will look at whether the participants have any side effects and if there are any possible changes in something called “biomarkers,” which are blood proteins that will be checked to see if they change when taking FP-045 and that may indicate if FP-045 can delay or prevent disease symptoms. Every participant will receive FP-045.
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