A Study to Assess the Effectiveness and Safety of Pacritinib in Patients With VEXAS Syndrome (PAXIS)

NCT06782373

Status	RECRUITING
Phase	Phase 2
Sponsor	Swedish Orphan Biovitrum
Enrollment	78 participants

Key Eligibility Criteria

Inclusion (16)

- Documented evidence of a pathogenic mutation at methionine-41 (M41) or neighboring splice site mutation (c.118-1, c.118-2) position in UBA1 mutation based on myeloid next-generation sequencing (NGS) droplet digital polymerase chain reaction (ddPCR), or Sanger sequencing in peripheral blood or bone marrow samples.
- Current or documented evidence of past inflammatory involvement within 6 months prior to enrollment of at least one of the following organ systems by VEXAS syndrome: cutaneous (e.g., neutrophilic dermatosis, cutaneous vasculitis), vasculature (e.g., vasculitis), musculoskeletal (e.g., chondritis, arthritis), ocular (e.g., uveitis, scleritis), periorbital (e.g. periorbital edema), genitourinary (e.g., epididymitis), or pulmonary (e.g., alveolitis).
- Receiving ongoing GC therapy (stable prednisone or prednisolone dose of 15-45 mg/day) leading up to enrollment.
- Karnofsky Performance Status e50%
- Adequate organ function, meeting all the following criteria within 30 days prior to enrollment:

... and 11 more (see full listing online)

Exclusion (25)

- Prior allogenic hematopoietic stem cell transplant (allo-HSCT) or solid organ transplant (other than corneal).
- Current use of systemic GCs for conditions other than VEXAS syndrome, which, in the opinion of the Investigator, would interfere with adherence to a GC taper regimen and/or assessment of efficacy.
- More than one prior admission to an intensive care unit due to a VEXAS Syndrome flare within the prior 6 months.
- Received e9 units of intensive red blood cell (RBC) transfusions in the 90 days prior to enrollment.
- Known concurrent myelodysplastic syndrome (MDS) requiring antineoplastic treatment, or allo-HSCT, or known high-risk or very high-risk MDS based on the Revised International Prognostic Scoring System (IPSS-R). Participants with MDS who do not meet these criteria may enroll.

... and 20 more (see full listing online)

Locations (40 total)

Mayo Clinic - Scottsdale, Scottsdale, Arizona, United States
University of Maryland Medical Center Midtown Campus, Baltimore, Maryland, United States
Dana Farber Cancer Institute, Boston, Massachusetts, United States
... and 37 more locations