Safety and Efficacy of PMT Therapy of hPAP

NCT05761899

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Children's Hospital Medical Center, Cincinnati
Enrollment	3 participants

Key Eligibility Criteria

Inclusion (13)

- Patients must meet all of the following conditions to be eligible for participation in this study:
- Male or female with a confirmed diagnosis of hPAP defined as:
- Homozygous or compound heterozygous CSF2RA mutations - AND -
- A normal GM-CSF autoantibody test result - AND -
- An abnormal STAT5-PI test result - OR -

... and 8 more (see full listing online)

Exclusion (18)

- Patients who meet any of the following conditions will not be eligible for participation in this study:
- History of a confirmed diagnosis of any other PAP-causing disease defined as:
- PAP caused by function-altering mutations in CSF2RB, adenosine triphosphate (ATP)-binding cassette subfamily A member 3 (ABCA3), SFTPB, SFTPC, Thyroid Transcription Factor 1 (TTF-1), GATA-binding factor 2 (GATA2), SLC7A7, and methionyl-transfer RNA (tRNA) synthetase (MARS), or other genes demonstrated to cause PAP other than CSF2RA
- PAP associated with an abnormal GM-CSF autoantibody test
- PAP associated with hematologic disorders including but not limited to myelodysplasia, aplastic anemia, leukemia, multiple myeloma, lymphoma

... and 13 more (see full listing online)

Locations (1 total)

Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio, United States