

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

<https://clinicaltrials.gov/study/NCT05761899>

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