

A Study of Emapalumab for Pediatric Aplastic Anemia

NCT06430788

Status	RECRUITING
Phase	Phase 2
Sponsor	Memorial Sloan Kettering Cancer Center
Enrollment	35 participants

Key Eligibility Criteria

Inclusion (7)

- Patients undergoing workup for suspected newly diagnosed sAA:
- Patients with severe cytopenias and a hypocellular marrow concerning for sAA
- Patients that meet the definition for suspected sAA (Camitta Criteria) as follows:
 - Marrow Cellularity: $\leq 25\%$, or 25-50% with $\leq 30\%$ residual hematopoietic cells
 - Peripheral cytopenias (at least 2 of 3) Absolute neutrophil count (ANC): $\leq 500 \times 10^9/L$ Platelets: $\leq 20 \times 10^9/L$ Absolute Reticulocyte Count: $\leq 60 \times 10^9/L$
- Patients that do not have evidence of leukemia or MDS

... and 2 more (see full listing online)

Exclusion (6)

- Uncontrolled infection at presentation.
- Patients who have undergone previous treatment for sAA.
- Patients with known inherited bone marrow failure
- Patient who has completed a full workup for sAA including having results back from telomere testing, DEB and genetics (when applicable), as well as having an appropriate willing and available donor and would otherwise be admitted for HSCT within 2 weeks of enrolling on the trial
- Patients with leukemia or MDS

... and 1 more (see full listing online)

Locations (6 total)

Memorial Sloan Kettering Cancer Center (All Protocol Activities), New York, New York, United States

Cincinnati Children's Hospital Medical Center (Data collection only), Cincinnati, Ohio, United States

Children's Hospital of Philadelphia (Data Collection AND Specimen Analysis), Philadelphia, Pennsylvania, United States

... and 3 more locations