

# A Study of Emapalumab for Pediatric Aplastic Anemia

**Status:** RECRUITING

## Eligibility Criteria

**Age:** 0 years to 25 years old

This study is also accepting healthy

**Healthy Volunteers:** volunteers

### Inclusion Criteria:

\* 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 \* Patients  $\leq 25$  years of age at time of diagnosis \* Able to tolerate emapalumab and IST (with standard institutional organ function criteria)

### Exclusion Criteria:

\* 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 \* Patient or parent or guardian unable to give informed consent or unable to comply with the treatment protocol including research tests.

## Conditions & Interventions

### Interventions:

BIOLOGICAL: Emapalumab

### Conditions:

Aplastic Anemia, Cytopenia, Hypocellular Marrow

### Keywords:

pediatric aplastic anemia, aplastic anemia, cytopenia, hypocellular marrow, Emapalumab, Memorial Sloan Kettering Cancer Center, 23-278

## More Information

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**Principal Investigator:**

**Phase:** PHASE2

**IRB**

**Number:**

**System ID:** NCT06430788

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