A Study of Emapalumab for Pediatric Aplastic Anemia

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 0 years to 25 years old
Healthy Volunteers: This study is also accepting healthy 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: <25%, or 25-50% with <30% residual hematopoietic cells Peripheral cytopenias (at least 2 of 3) Absolute neutrophil count (ANC): <500 x 10^9/L Platelets: <20 x 10^9/L Absolute Reticulocyte Count: <60 x 10^9/L

- Patients that do not have evidence of leukemia or MDS
- Patients < 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

Description:
The purpose of this study is to find out whether upfront emapalumab treatment can help in sAA (Aplastic Anemia) treatment planning and increase the effectiveness of standard treatment options. Funding Source- FDA OOPD

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