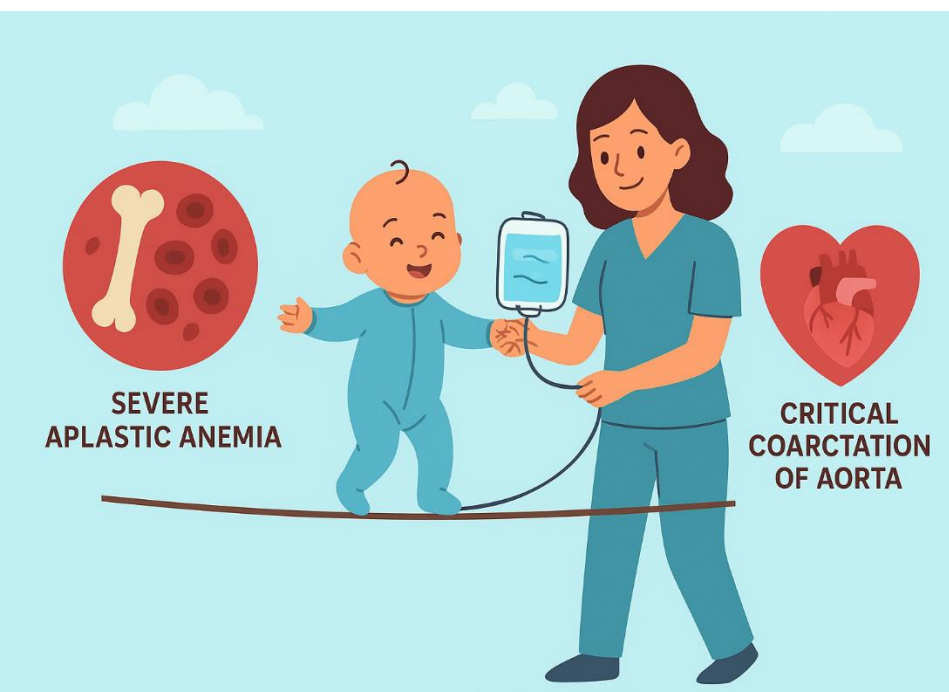


Walking a Tightrope: Haploidentical TCR $\alpha\beta$ Depleted HSCT in an Infant with Severe Aplastic Anemia & Critical Coarctation of Aorta

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Case Overview

The patient VV is a one-year-old male 3rd born of non consanguineous marriage diagnosed with severe aplastic anemia and coarctation of the aorta

History of gold based nutritional supplements

Coarctation of the aorta - underwent balloon angioplasty in November 2024: Persistent coarctation detected; surgery planned but deferred

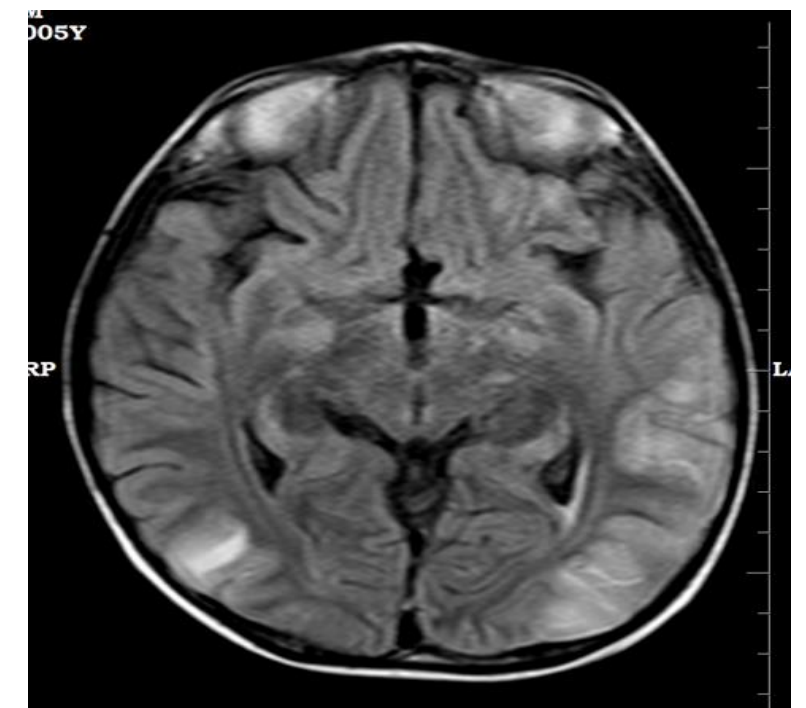
On arrival - refractory to platelet transfusions – hemorrhagic, labile blood pressure - coarctation of aorta

Haploidentical TCR $\alpha\beta$ depleted HSCT using his father as donor in March 2025

Conditioning protocol - Thiotepa, Treosulfan, Fludarabine, rabbit ATG, and rituximab

Early Complications

- Grade IV mucositis with loose stools and volume loss - managed with minimal trophic feeds and fluid replacement
- Treosulfan-induced skin rash over the abdomen - treated conservatively
- Hypertension - sodium nitroprusside infusion , labetalol infusion and multiple oral antihypertensives, 2D Echo: significant left ventricular dysfunction
- Day +4 - neurological deterioration -PRES - anticonvulsants and intracranial pressure control
- Day + 14 WBC engrafted



Fever with no focus

- Continuous fever on Day 40
- All investigations clear including MRI brain and SCF ME panel
- Same time – drop in chimerism to 99.8% with poor immune reconstitution
- Stem cell top up – Day 42
- Day 62 – 100% donor and clinically well

Follow up

T and B cell recovery demonstrated gradual improvement by Day +90.

Day	Chimerism (%)	CD4+	CD8+	B cells	NK cells
+14	100	15	8	0	-
+27	100	-	-	0	-
+42	99.8	-	-	-	-
+62	100	4	3	0	75
+90	100	35	10	0	68
6 months	100%	47	24	0	180

- Immunosuppression (Neoral), antimicrobial prophylaxis, and supportive medications.
- IVIg monthly
- CMV,EBV and Adenovirus reactivations-negative
- Blood pressure - stable on four oral antihypertensives (MAP target ~70)
- Currently 9 months post transplant
- Readmitted with loose stools – rota, astro, norovirus

Questions to the experts

1. What are the best strategies to accelerate immune reconstitution after TCR $\alpha\beta$ -depleted HSCT?
2. Infants require a lot of supportive care – how can we plan and work with PICU team?