REscuing bone marrow function in patients with aplaStic anaEmia and bone marrow faiLure post allogEneiC Transplantation (RESE-LECT)

Phase I/II single arm with historical control study assessing the efficacy and safety of Atorvastatin and N-Acetyl Cysteine in the treatment of Poor Graft Function post allogeneic transplantation and relapsed/refractory aplastic anaemia

ACTRN12620001339943

Status	RECRUITING
Phase	Phase 2
Sponsor	The Royal Melbourne Hospital
Enrollment	20 participants

Plain Language Summary

After a bone marrow or stem cell transplant, it's essential that the donor cells "take root" and begin producing healthy blood cells. When this doesn't happen well — a problem called "poor graft function" — patients develop dangerously low blood counts and face serious risks. A related condition, aplastic anaemia, involves the body's immune system attacking its own bone marrow, leading to similar outcomes. Both conditions are hard to treat once standard approaches fail.

This trial is testing a combination of two repurposed medications: atorvastatin (commonly used to lower cholesterol) and N-acetylcysteine (NAC, used to treat paracetamol overdose). Lab research suggests these drugs may help restore the bone marrow's microenvironment — the biological ecosystem that blood-producing cells depend on — which appears to be abnormal in both poor graft function and aplastic anaemia.

You may be eligible if you are 17 or older with poor graft function after a stem cell transplant or relapsed/refractory aplastic anaemia, have reasonable physical function, and do not have active severe graft-versus-host disease or other active cancers. The study will measure whether blood counts improve over the treatment period, and whether the drugs are safe in this patient group.

Key Eligibility Criteria

Inclusion (14)

- Poor Graft function OR Relapsed/refractory AA defined as the following
- Poor Graft Function:
- Two Lineage cytopenias defined as
- Thrombocytopenia
- i)Less than or equal to 30x10^9 /L from D40-D60 OR

... and 9 more (see full listing online)

Exclusion (8)

- Active Grade 3-4 acute GVHD
- Relapsed or progressive disease on screening bone marrow biopsy or most recent PET imaging.
- Active second malignancy currently requiring treatment
- Human Immuno-deficiency Virus (HIV) infection

• Any coexisting medical or psychological condition that would preclude participation in the required study procedures.

... and 3 more (see full listing online)

Locations (1 total)

NSW,WA,VIC, Australia