FANCONI ANEMIA RESEARCH FUND

LET'S TALK ABOUT

STEM CELL TRANSPLANTS

WHAT

BONE MARROW FAILURE

Bone marrow failure is one of the manifestations of the DNA repair problem in Fanconi anemia (FA), and leads to the need for a Hematopoietic Stem Cell Transplant (HSCT). This is currently the only long-term treatment for fixing bone marrow failure in FA.

THE TRANSPLANT

In this process, the patient's (non-functioning) stem cells are replaced with healthy stem cells from a donor. This donor can be related or unrelated, and the cells can come from the bone marrow, cord blood or peripheral blood. When choosing donor cells, the goal is to find a donor whose cells "match" the recipient's as closely as possible. The closer the match, the more likely the transplanted cells will grow and the less risk there is for a complication known as graft-versus-host-disease (GvHD).

WHEN

TIMING OF TRANSPLANT

It is important to note that not every person with FA will need a transplant. Although likely, it is not a certainty. Someone needs a transplant when his/her counts are low enough to require it. Criteria may include:

- A consistent **downward trend in counts** over a few readings. This is determined by assessing blood counts every 1 to 3 months, depending on how low counts are. Infection can cause a decline in counts, so it's important to take this into consideration, looking for a consistent trend over time. Ideally, patients should be transplanted before requiring blood or platelet transfusions.
- **Leukemia or Myelodysplastic Syndrome** (MDS)
- An **abnormal clone** (changes in the chromosomal number or structure of certain bone marrow cells). Some of these clonal changes can be monitored over time and some require immediate attention.
- Diagnosis of a **BRCA2 mutation** (this presents a high risk for the quick development of leukemia without warning signs).

WHERE

FA COMPREHENSIVE CARE CENTERS

FA is very unique and complex, and the best chance at positive outcomes are when patients are treated at centers that have a deep understanding of and experience with treating FA. This is important for long-term follow-up as well. If a patient is unable to travel to an FA center, physician-to-physician communication and collaboration is vital. The transplant process and follow-up require a team approach to care for all aspects of the patient's needs. An effective team will include sub-specialists that are invested in FA and who approach their patients through an FA lens.

Thank you to Dr. Margaret MacMillan (University of Minnesota) for help in developing this information

PROVEN FA CENTERS

- University of Minnesota Masonic Children's Hospital (Minneapolis, MN)
- Cincinnati Children's Hospital Medical Center (Cincinnati, OH)
- Memorial Sloan Kettering Cancer Center (New York, NY)

MORE INFORMATION

Visit www.fanconi.org for more information about transplants, including frequently asked questions, as well as specific medical guidelines about when and how to proceed with treatment.