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.

**WHAT**
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**
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.

**WHERE**

- **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).

**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.