Transplant outcomes for children and young adults with chronic myeloid leukemia (CML)

What were researchers trying to learn?
Researchers wanted to find out how well blood or marrow transplant (BMT) works in children and young adults with CML. CML is a cancer of the white blood cells.

There are 2 main treatments for CML:
1. Medicines called tyrosine kinase inhibitors (TKIs)
2. BMT

Imatinib (also called Gleevec®) is the most common TKI. Most of the time, TKIs are the first treatment for patients with CML. But sometimes patients with CML have transplant first.

CML is rare in children (under 18 years) and young adults (age 18 to 29 years). And less is known about CML in these patients. So the researchers studied children and young adults to find out whether:
- The patient’s age affects how well transplant works
- Treatment with TKIs before transplant affects how well the transplant works
- Transplant works better using bone marrow or using peripheral blood stem cells (PBSC)
- Transplant works better when the donor is a sibling (brother or sister) or someone unrelated to the patient

What did they find?
The researchers studied about 450 children and young adults with CML who had transplant between 2001 and 2010. They measured how many patients were alive at a specific time after transplant. In this study, 75% of patients (3 out of 4) were alive 5 years after transplant. The patient’s age didn’t affect how well transplant worked.

The researchers found that TKIs didn’t affect how well the transplant worked either. Patients who took TKIs before transplant did as well as patients who had transplant without taking TKIs first.

But the type of cells and the type of donor did affect how well the transplant worked. The CML came back (relapsed) less often in patients who got bone marrow compared to patients who got PBSC. And patients who had a sibling donor lived longer than patients who had an unrelated donor.

Important Points:
- Transplant outcomes are similar in children and young adults with CML.
- Transplant works better with bone marrow from sibling donors.
- Taking TKIs before transplant doesn’t affect how well transplant works.
**Why is this important?**
Doctors can now share more information about transplant outcomes for CML with patients and families. This can help patients and families make treatment decisions. Doctors also now know what factors, such as type of cells and type of donor, may help transplant for CML work better.

**What else should I keep in mind about this study?**
The results of research studies are always limited in what they can and can’t tell you. With this study, one drawback is that researchers only studied patients who got standard intensity preparative regimens. The preparative regimen is the chemotherapy with or without radiation given to prepare a patient’s body for transplant. For children and young adults, doctors now mostly use reduced-intensity preparative regimens, which use less chemotherapy. Doctors don’t know if these results will also apply to patients getting reduced-intensity transplants.

**Questions to ask your doctor**
If you or your child needs treatment for CML, you may want to ask your doctor:
- Do you recommend TKI therapy or transplant?
- Are there any long-term effects of TKIs that I should know about?
- What are my options if I don’t have a sibling donor for my transplant?

**Learn more about**
- This research study
- Blood and marrow transplant
- Transplants for CML

**Source:**

**About this research summary**
Ground-breaking research into blood and marrow transplant is happening every day. That research is having a significant impact on the survival and quality of life of thousands of transplant patients. But the research is written by scientists for scientists. By providing research news in an easy-to-understand way, patients, caregivers, and families have access to useful information that can help them make treatment decisions.

This information is provided on behalf of the Consumer Advocacy Committee of the CIBMTR® (Center for International Blood and Marrow Transplant Research®). The CIBMTR is a research collaboration between the National Marrow Donor Program®/Be The Match® and the Medical College of Wisconsin.