
Reduced-intensity transplant prep helps young people with immune system disorders

Immune disorders are serious and can be life-threatening. Reduced-intensity treatment before transplant helps people with serious immune system disorders live longer.

In this clinical trial, doctors treated people with 5 rare immune disorders:

- Hemophagocytic lymphohistiocytosis (HLH)
- Chronic active Epstein-Barr virus (CAEBV)
- Chronic granulomatous disease (CGD)
- Hyperimmunoglobulin syndrome (HIGM1)
- Immune dysregulation, polyendocrinopathy, enteropathy and X-linked syndrome (IPEX)

A blood or marrow transplant (BMT) using donated cells, also called an allogeneic BMT, is the only cure for these disorders. To prepare the body for the donated cells, people get medicines to destroy the broken immune system. These medicines include chemotherapy, or chemo, and can make people sick.

In this study, people got a reduced-intensity preparation, with medicines and doses less likely to make them sick. Over about 2 weeks before transplant, they got:

- Alemtuzumab, given 5 times as shots under the skin
- Fludarabine, a chemo drug, given 5 times as an intravenous (IV) infusion
- Melphalan, a chemo drug, given 1 time as an IV infusion

Doctors saw that a higher percentage of patients were alive at 1 year compared to more intense transplant preparative approaches. However, about 40% (4 of 10 people) needed more treatment, such as a second transplant or white blood cells from their donor (also called a donor lymphocyte infusion, or DLI).

Keep in mind

Because these immune system disorders are rare, the study included only 46 people from the U.S. and Canada. People's ages ranged from 5 months old to 28 years old. More research is needed to improve treatment of immune disorders.

Consider asking your doctor

- What is the best way for me (or my child) to prepare for transplant?

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Center for International Blood and Marrow Transplant Research® • Froedtert and the Medical College of Wisconsin • Clinical Cancer Center •
9200 West Wisconsin Avenue • Suite C5500 • Milwaukee, WI 53226 • 414-805-0700 • Fax: 414-805-0714

The Emmes Corporation® • 401 N. Washington Street • Suite 700 • Rockville MD 20850 • 301-251-1161 • Fax: 301-251-1355

National Marrow Donor Program® • 500 N 5th Street • Minneapolis MN 55401 • 612-627-5800 • Fax: 763-406-4370

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Learn more about

- [This research study from its publication](#)
- [This research study on ClinicalTrials.gov](#)
- [Transplant for HLH](#)

Source

Allen CE, Marsh R, Dawson P, et al. Reduced intensity conditioning for hematopoietic cell transplant for HLH and primary immune deficiencies BMT CTN 1204. Blood. 2018 Sep 27; 132 (13): 1438-1451. doi:10.1182/blood-2018-01-828277. Epub 2018 Jul 11. PMC6161764.

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.

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