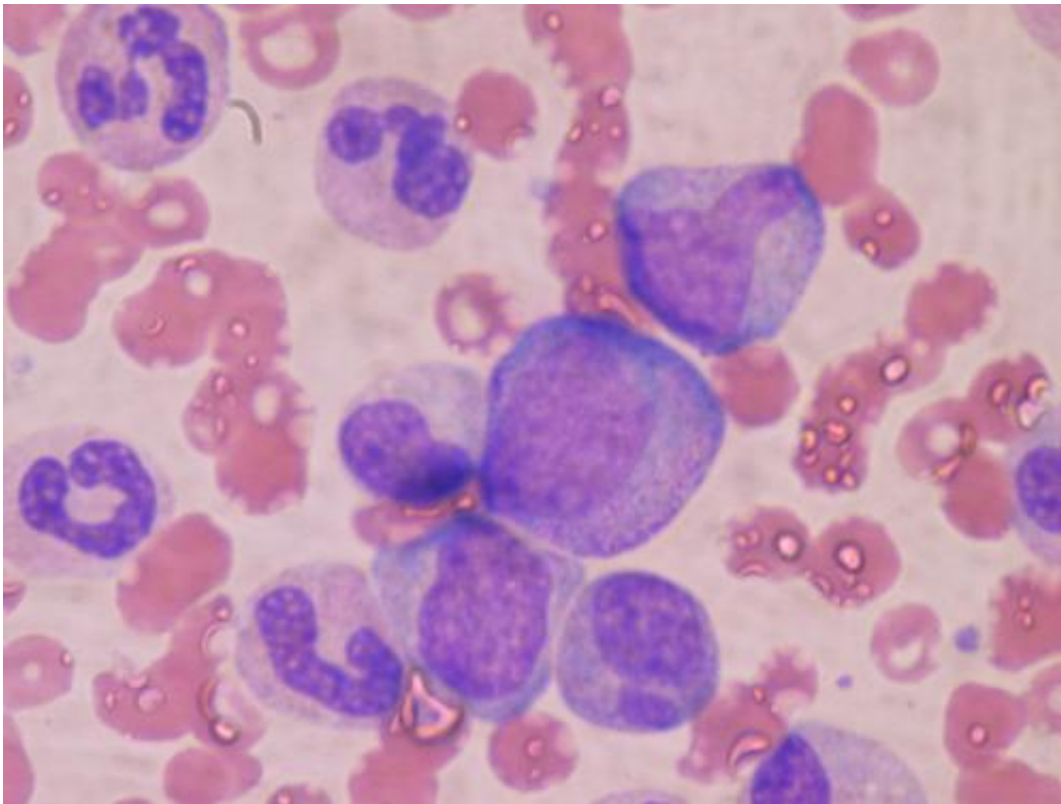


# Researchers find new, safer treatment for serious side effect of bone marrow transplant

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Hematopoietic precursor cells: promyelocyte in the center, two metamyelocytes next to it and band cells from a bone marrow aspirate. Credit: Bobjgalindo/Wikipedia

Researchers at The Tisch Cancer Institute at Mount Sinai have identified a treatment that is effective and safer than the standard of care for a serious, and sometimes fatal, side effect of bone marrow transplant in

cancer patients. Results from a phase 2 clinical trial were presented at the annual meeting of the American Society of Hematology (ASH) in December.

The clinical trial in adolescents and adults found that a drug that inhibits the [immune system](#) in patients who have graft-versus-host disease (GvHD) is safer than steroids, the current standard treatment. GvHD is a side effect seen in patients who have received a bone marrow transplant from a donor to treat blood cancers. The study used a blood test developed at Mount Sinai to identify patients with GvHD most likely to benefit from the new treatment.

"Steroids are known to cause numerous complications in bone marrow transplant recipients who require treatment for GvHD such as serious infections, bone and muscle damage, poor sleep, and poor quality of life," said Aaron Etra, MD, Assistant Professor of Medicine (Hematology and Medical Oncology) at The Tisch Cancer Institute, who presented the study at the ASH meeting.

"A steroid-free treatment for GvHD would be an important advance in improving transplant outcomes, but it's not likely to be the best choice for all patients. Being able to use GvHD biomarkers to personalize treatment intensity was key to the success of this trial," added John Levine, MD, Professor of Medicine (Hematology and Medical Oncology) at The Tisch Cancer Institute, and co-senior author for this research. Alexandra Capellini, a Mount Sinai [medical student](#), was co-first author.

GvHD occurs when the cells from a donor attack the healthy organs of the recipient. It results in the release of certain tissue proteins into the bloodstream; these proteins can be used as biomarkers to quantify the severity of the tissue damage. Patients with low levels of these biomarkers tend to respond well to treatment in general, but before now

there was no way to identify them. A research laboratory at Mount Sinai was able to quantify the GvHD biomarkers in blood samples obtained from patients within 30 hours, which made it feasible to study this treatment in patients who need to start treatment quickly.

Results from the trial were compared to a matched control group of patients who were treated with steroids. This research found that a short course of itacitinib, a JAK1 inhibitor that can tamp down the immune system, produced very high response rates that occurred faster than treatment with steroids. Responses to itacitinib were as durable as steroids and long-term outcomes were equally good.

Both itacitinib and steroids were effective in 86 percent of patients. The one-year survival rate for itacitinib was 88 percent compared to 80 percent for steroids. Patients treated with itacitinib also had significantly fewer severe infections, likely a result of dramatically less exposure to systemic steroids.

"This was the first time anyone was able to use real-time GvHD biomarkers to identify low-risk patients whose treatment could be de-escalated," said James L Ferrara, MD, Professor of Medicine (Hematology and Medical Oncology) at The Tisch Cancer Institute, and co-senior author for this research. "Mount Sinai developed this biomarker approach, which allowed us to present this new and meaningful finding for patients and their quality of life."

**More information:** Conference:  
[www.hematology.org/meetings/annual-meeting](http://www.hematology.org/meetings/annual-meeting)

Provided by The Mount Sinai Hospital

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