

# Stem cell transplantation does not provide significant improvement for Crohn's disease

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Among adults with difficult to treat Crohn disease not amenable to surgery, hematopoietic stem cell transplantation, compared with conventional therapy, did not result in significant improvement in sustained disease remission at 1 year and was associated with significant toxicity, according to a study in the December 15 issue of *JAMA*.

Crohn disease is a chronic relapsing inflammatory condition of the gastrointestinal tract that can result in life-long ill health, impaired quality of life, and reduced life expectancy. Immunosuppressive drugs are standard of care for Crohn disease, but some patients do not respond or lose response to treatment. Case reports and series suggest hematopoietic (blood) stem cell transplantation (HSCT) may benefit some patients with Crohn disease, according to background information in the article.

Christopher J. Hawkey, F.Med.Sci., of Queens Medical Centre, Nottingham, United Kingdom, and colleagues randomly assigned 45 patients with impaired quality of life from refractory (not responsive to treatment) Crohn disease not amenable to surgery to autologous (the use of one's own cells) HSCT (n = 23) or control treatment (HSCT deferred for 1 year [n = 22]). All were given standard Crohn disease treatment as needed. The trial was conducted in 11 European transplant units from July 2007 to September 2011, with follow-up through March 2013. Patients were ages 18 to 50 years.

The researchers found that there was no statistically significant between-

group difference in the proportion of patients who met the study definition of sustained disease remission (2 [8.7 percent] in the HSCT group vs 1 [4.5 percent] in the control group); or on a certain measure on the Crohn Disease Activity Index in the last 3 months; or freedom from active disease. There was a statistically significant difference among patients able to discontinue active treatment in the last 3 months (HSCT group, 61 percent; control group, 23 percent).

There were 76 serious adverse events in patients undergoing HSCT vs 38 in controls; 1 patient undergoing HSCT died.

"Because very few patients achieved sustained [disease remission](#), we conclude that HSCT is unlikely to alter the natural history of Crohn disease, and our findings argue against extension of HSCT to a wider group of patients outside of future additional trials," the authors write.

The researchers add that based on these findings, further study of HSCT in patients with refractory Crohn disease may be warranted. "It is possible that optimal sustained remission after HSCT may require maintenance immunosuppressive therapy. It is also possible that patients will regain responsiveness to treatments to which they were previously refractory. Therefore, future trials should assess the benefit of maintenance therapy. Toxicity will remain the most significant barrier to HSCT in [patients](#) with Crohn [disease](#). Therefore, identification of factors that predict either the risk of adverse effects or response to treatment will enhance the utility of this [treatment](#) in clinical practice."

**More information:** *JAMA*, [DOI: 10.1001/jama.2015.16700](https://doi.org/10.1001/jama.2015.16700)

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