

Study suggests hope for a longer life for patients with rare autoimmune disorder

17 January 2018, by Josh Barney

An unusual autoimmune disease that causes skin and lung damage can be treated effectively by stem cell transplant, a new study in the *New England Journal of Medicine* has found. The approach could represent the first new treatment to improve survival in patients with severe scleroderma in more than four decades.

The experimental procedure uses chemotherapy and radiation to destroy the body's malfunctioning immune system, then replaces it via a [stem-cell transplant](#). The [stem cells](#) are taken from the patient's own blood and given back to the patient after the chemotherapy.

In a multisite clinical trial, the approach proved more successful than the existing treatment, significantly improving survival and reported quality of life. "This is a major advance in the treatment of severe [scleroderma](#)," said Dr. Karen Ballen, a co-investigator on the study and the director of stem cell transplantation at the University of Virginia Cancer Center.

Scleroderma affects about 240 people per million in the U.S., mostly women, and is slightly more prevalent among African-Americans than among white Americans.

Scleroderma Study

The study compared the most effective existing treatment, the drug cyclophosphamide, with the new approach. Thirty-four trial participants received cyclophosphamide, while 33 received the stem [cell transplant](#) treatment. After 72 months, 86 percent of those who received the [stem-cell transplant](#) remained alive, compared with only 51 percent of those who received infusions of cyclophosphamide.

In the *New England Journal of Medicine* paper detailing their findings, the researchers conclude: "At four and a half years of follow up, participants

who received a transplant experienced significantly better outcomes overall than those who received cyclophosphamide. In addition, 44 percent of participants who received cyclophosphamide had begun taking anti-rheumatic drugs for progression of their scleroderma, compared to only 9 percent of those who received a transplant."

Both [treatment](#) options carried risks of infections and low blood-cell counts, the researchers reported. The overall infection rates were similar.

All participants had severe scleroderma that affected their lungs or kidneys. The researchers noted that their study had limitations that suggest the findings may not apply to all patients with scleroderma.

Provided by University of Virginia

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