Rapid skin improvement seen after treating systemic sclerosis patients with fresolimumab

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A major treatment breakthrough for total body scarring of the skin that occurs in patients with systemic sclerosis (SSc), also known as scleroderma, may soon be available for the estimated 300,000 Americans who suffer with this condition. Currently, no treatment is available.

Boston University School of Medicine (BUSM) researchers worked with 15 SSc patients who were treated with either one or two doses of fresolimumab, a new, unapproved drug therapy that targets a chemical mediator in the body known as TGF-beta. After seven weeks of treatment, the researchers examined the effect on skin scarring and on expression of molecular markers in the skin. In both clinical and molecular evaluations these patients showed profound decreases in skin scarring.

The researchers found that TGF-beta plays a critical role in skin scarring in patients with SSc. Although TGF-beta has long been implicated in causing scarring, this is the first clinical study to clearly show its impact on humans. The study appears online in the *Journal of Clinical Investigation*.

SSc is a chronic connective tissue disease generally classified as one of the autoimmune rheumatic diseases. The disease may affect the connective tissue in many parts of the body including the skin, esophagus, gastrointestinal tract (stomach and bowels), lungs, kidneys, heart and other internal organs. It also can affect blood vessels, muscles

and joints. The tissues of involved organs become hard and fibrous, causing them to function less efficiently.

"Our study shows that TGF-beta plays a critical role in skin scarring in patients with systemic sclerosis," explained corresponding author Robert Lafyatis, MD, professor of medicine at BUSM and Director, BU Scleroderma Center of Research Translation. "Our results strongly indicate that targeting the TGF-beta in these patients will block skin scarring."

According to the researchers proving that TGF-beta can block scarring may provide a major treatment advance for scarring-mediated organ dysfunction common to many diseases including lung fibrosis in idiopathic or radiation induced pulmonary fibrosis, liver fibrosis from viruses or toxins, kidney fibrosis from diabetes and other less common kidney diseases, and heart fibrosis that occurs after heart attacks and during heart failure.

A larger study with a placebo-treated group of patients is needed to confirm these results.

Provided by Boston University Medical Center