

# Sutimlimab shows promise for hard-to-treat, rare blood disorder

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In a first-in-human clinical trial reported today in *Blood*, the investigational drug sutimlimab appeared to be effective in treating cold agglutinin disease, a rare chronic blood disorder for which there are currently no approved treatments.

Cold agglutinin disease is caused by a malfunction in the [immune system](#) that causes antibodies—components of the immune system that are produced in the [blood](#) and help the body fight off disease—to mistakenly latch onto and kill [red blood cells](#). The disease is a type of hemolytic anemia, a condition that occurs when the [bone marrow](#) can't produce red blood cells as quickly as they are destroyed.

In the study, sutimlimab, a specific C1s inhibitor, rapidly halted the destruction of red blood cells, increased [hemoglobin levels](#), eliminated patients' need for blood transfusions, and caused no serious adverse effects.

"The drug was well tolerated, produced clinically meaningful increases in hemoglobin levels, and precluded the need for transfusions, even in patients for whom multiple prior therapies had failed," said senior author Bernd Jilma, MD, of the Medical University of Vienna.

Cold agglutinin disease is thought to affect about 10,000 people in the United States and Europe. Most patients with the disease are over 50 years old.

To date, there are no U.S. Food and Drug Administration (FDA)-approved treatments for cold agglutinin disease, though rituximab—a treatment for certain blood cancers—has been used with or without chemotherapy with limited success.

This study included 10 patients 56-76 years old. Patients had cold agglutinin disease for a median of five years, and many had received multiple prior treatments that had been unsuccessful. At enrollment, all patients had below-normal levels of hemoglobin, a component of red blood cells that is responsible for transporting oxygen to cells and organs throughout the body. Six patients were receiving regular blood transfusions to control their symptoms.

Of those patients who responded, within the first week of treatment with a full dose of sutimlimab, destruction of red blood cells stopped and patients' hemoglobin levels significantly increased, said Dr. Gilma. Seven patients responded and had a median increase in hemoglobin levels of 4 grams per deciliter (g/dL) over a baseline of 7.5 g/dL within six weeks. Four patients had their hemoglobin levels return to normal.

When sutimlimab treatment was discontinued and the drug had left the patients' blood, [hemoglobin](#) levels dropped and destruction of red blood cells began again. However, when treatment resumed, these effects were once again reversed. The six patients who had been sustained by regular blood transfusions remained transfusion-free for up to 18 months while receiving sutimlimab treatment.

"Provided that safety results remain positive, sutimlimab could become the first approved treatment for cold agglutinin disease," Dr. Gilma said. "The drug clearly addresses an unmet medical need, as we have seen rapid, strong responses in [patients](#) for whom multiple prior therapies have failed."

**More information:** *Blood* 2018 :blood-2018-06-856930; [DOI: 10.1182/blood-2018-06-856930](https://doi.org/10.1182/blood-2018-06-856930)

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