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Drug Represents First Potential Treatment for Common Anemia

(WASHINGTON, August, 27, 2014) – An experimental drug designed to help regulate the blood's iron supply shows promise as a viable first treatment for anemia of inflammation, according to results from the first human study of the treatment published online today in *Blood*, the Journal of the American Society of Hematology (ASH).

Anemia is a condition that occurs when red blood cells are in short supply or do not function properly. When an individual has anemia, the body does not get enough oxygen, since there are fewer red blood cells to carry the iron-rich protein hemoglobin that helps distribute oxygen throughout the body. This can result in symptoms such as weakness and fatigue.

The most common form of anemia in the hospital setting is anemia of inflammation, which occurs when the body's immune response is activated during illness or infection. When the body fights a disease, it deploys an inflammatory response that triggers increased secretion of a hormone called hepcidin that reduces the amount of iron available in the bloodstream. As iron is needed for the production of red blood cells in the bone marrow, many patients develop anemia.

The only current treatment strategy for anemia of inflammation involves targeting the underlying disease or infection; however, recent research has sought to explore additional options for patients whose inflammation is difficult to control or when the cause of inflammation is unknown. As the principal regulator of iron, hepcidin has become a target for researchers developing novel therapies for blood disorders. One hepcidin inhibitor, called lexaptepid pegol (lexaptepid), has demonstrated efficacy in treating anemia of inflammation in animal studies. Lexaptepid inactivates hepcidin, thereby maintaining the transport of iron to the bloodstream.

In order to evaluate lexaptepid's potential in humans, investigators induced a safe and temporary model of anemia of inflammation in 24 healthy male adults and randomized them to receive lexaptepid or placebo. Volunteers received a low dose of *Escherichia coli* (*E. coli*) endotoxin to induce controlled inflammation and received either lexaptepid or placebo 30 minutes later. After nine hours, iron in the blood stream had decreased in the placebo group, whereas this decrease could be prevented by treatment with lexaptepid.

In addition to determining whether lexaptepid interfered with hepcidin production, researchers also sought to determine whether the drug influenced the immune response. All volunteers experienced similar flu-like symptoms, increased body temperature and white blood cell count, and higher concentrations of inflammatory and signaling proteins, demonstrating to investigators that lexaptepid did not interfere with the immune response process.

"It is quite encouraging that lexaptepid helped maintain appropriate levels of iron in the bloodstream of healthy volunteers without compromising the immune response," said lead study author Lucas van Eijk, MD, of Radboud University Medical Center in Nijmegen, Netherlands. "We are hopeful that, with further study, this first-of-its-kind therapy could significantly improve quality of life for patients suffering from chronic illnesses."

Reporters who wish to receive a copy of the study or arrange an interview with the authors may contact Amanda Szabo at 202-552-4914 or email aszabo@hematology.org.

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Blood (www.bloodjournal.org), the most cited peer-reviewed publication in the field of hematology, is available weekly in print and online. *Blood* is the official journal of the American Society of Hematology (ASH) (www.hematology.org), the world's largest professional society concerned with the causes and treatment of blood disorders.

ASH's mission is to further the understanding, diagnosis, treatment, and prevention of disorders affecting blood, bone marrow, and the immunologic, hemostatic, and vascular systems by promoting research, clinical care, education, training, and advocacy in hematology.