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Researchers discover treatment for rare blood cancer

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University of British Columbia researchers have discovered a potential new treatment for a rare blood cancer that may also point the way to treating other more common diseases.

Paroxysmal nocturnal hemoglobinemia (PNH) is a rare form of cancer characterized by episodic rupture of red blood cells and the danger of blood clots forming in the vascular system. The condition results in red blood cells becoming vulnerable to attacks by the body's own complement immune system and can lead to complications such as anemia, kidney disease and fatal thromboses.

In a clinical study published today in *PLOS ONE*, the UBC team, led by Prof. Patrick McGeer, applied aurin tricarboxylic acid (ATA), a non-toxic drug, to blood samples of five patients with PNH who were undergoing standard treatment with antibodies administered through biweekly infusions.

The researchers found the addition of ATA restored blood cell resistance to complement system attacks, while the antibodies alone did not offer full protection.

"Our study suggests that ATA could offer more complete protection as an oral treatment for PNH while eliminating the need for infusions," says Prof. McGeer, professor emeritus in UBC's Department of Psychiatry. "PNH is a disease that may happen to anyone through a chance mutation, and if nature were to design a perfect fix for this

mutation, it would be ATA."

McGeer adds that since many diseases are caused or worsened by an overactive complement immune system, the discovery of ATA's effectiveness in this rare disease could have wide-reaching implications for conditions such as Alzheimer's and Parkinson disease, macular degeneration, ALS, multiple sclerosis and rheumatoid arthritis.

The team is now proceeding with further testing and McGeer hopes the treatment may be available in clinics within a year.

Provided by University of British Columbia