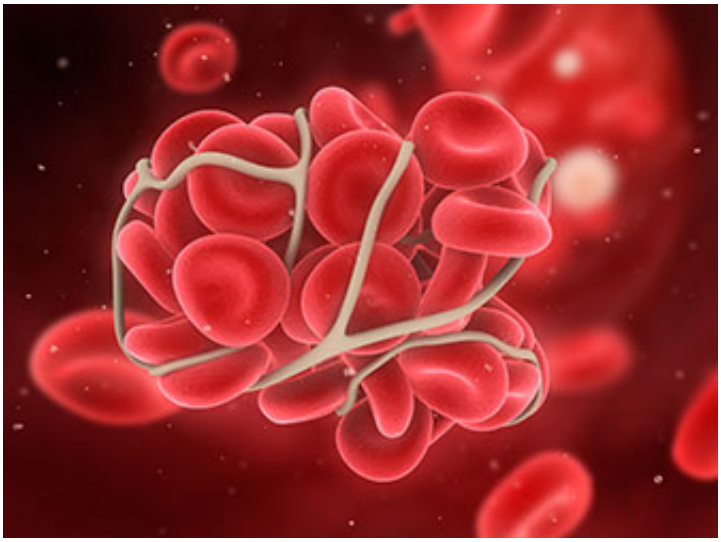
New treatment option for the acute phase of the rare disease TTP

February 11 2016



New treatment option for the acute phase of the rare disease TTP

Thrombotic Thrombocytopenic Purpura (TTP) is a rare disorder caused by an enzyme deficiency. This can be heriditary or can be acquired as an autoimmune condition. Due to the associated excessive activity of a certain protein, blood clots enriched with blood platelets form and block the smallest blood vessels. The disorder is life-threatening and very difficult to treat, particularly in the acute phase. Researchers at the Clinical Department of Haematology and Haemostaseology and the University Department of Clinical Pharmacology at MedUni Vienna have developed a treatment strategy to prevent this clotting in the blood vessels.

In its acute phase, TTP is life-threatening. Blocking of blood vessels with thrombi and blood clots, which aggregate on the overactive von Willebrand protein results in dangerous secondary diseases such as kidney failure, strokes or heart attacks, which can prove fatal.

"If patients can survive this acute phase, which has to be treated by plasma exchange, then the autoimmune disease can be easily treated by immunosuppression. Even to the point that it disappears completely," explains Paul Knöbl of the Clinical Department of Haematology and Haemostaseology at MedUni Vienna. However, approximately 10 – 20% of patients do not survive the acute phase of this disease. Every year, up to ten patients with TTP are treated at MedUni Vienna or in Vienna General Hospital. Viennese doctors and scientists are regarded as world leaders in the clinical trialing of new treatments for this disease and since 2009 they have published a total of six clinical studies into the therapeutic inhibition of the von Willebrand protein in patients with TTP.

In a study published in the leading magazine *New England Journal of Medicine*, the MedUni Vienna researchers have now been able to show that a newly developed antibody prevents overactivity of the von Willebrand protein and is therefore able to stop the life-threatening clotting of blood platelets more quickly. This would mean that even the acute phase of the disease would be easier to treat. The biotech product caplacizumab is similar to an antibody and blocks the interaction of platelets with the von Willebrand factor in a very targeted way. The drug is administered as a subcutaneous injection – a bit like an injection for preventing or treating thromboses.

"In future this drug might also be used to treat similar diseases, which are jointly caused by overactivity of the von Willebrand factor, and therefore represents a new treatment option," says Bernd Jilma of the University Department of Clinical Pharmacology at MedUni Vienna.

Another drug for treating hereditary TTP is currently being tested.

More information: "Caplacizumab for Acquired Thrombotic Thrombocytopenic Purpura", F. Peyvandi, M. Scully, J. A. Kremer Hovinga, S. Cataland, P. Knöbl, H. Wu, A. Artoni, J-P. Westwood, M. Mansouri Taleghani, B. Jilma, F. Callewaert, H. Ulrichts, C. Duby, D. Tersago. *New England Journal of Medicine*; DOI: 10.1056/NEJMoa1505533

Provided by Medical University of Vienna