Media Report

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Survival without lasting damage

Anyone who survives the rare blood disorder TTP is then left fighting the lingering neurological damage. So this does not remain the case, the Department of Hematology of Bern University Hospital is developing a faster treatment that is less likely to result in a relapse.

The rare and life-threatening blood disorder thrombotic thrombocytopenic purpura (TTP) mainly affects young, otherwise healthy people aged between 20 and 50. Only 2-3 people out of every million will fall ill with TTP, which forms blood clots in the small blood vessels. The decreased blood flow damages the heart, brain and kidneys in particular and can lead to strokes and heart attacks. The disorder mainly affects women and will lead to death in over 90 percent of cases if it is not treated within a few days.

Autoimmune disorder with lasting consequences

TTP is an autoimmune disorder with antibodies against the ADAMTS13 protein enzyme. Through the acute lack of ADAMTS13, the von Willebrand factor is no longer regulated in terms of size and blood platelets spontaneously adhere to it. For this reason, the standard treatment at the moment consists of daily plasma exchange (removal of antibodies, supply of ADAMTS13) and immunosuppressive drugs.

Despite the treatment, 10-20 percent of patients die during the acute episode. More than half of patents are left with neurological damage as a result of the decreased blood flow, such as impaired concentration, attention deficit and visual problems, numbness in an arm or leg and paralysis of these. With almost half, the disorder flares up again within 1-2 years.

Rapid control of the disorder with lower rate of relapses

In a multicentre clinical study (CH, AU, IT, BE, USA) with the involvement of the Department of Hematology of the Bern University Hospital, it has now been possible to treat TTP with an anti-von Willebrand factor nanobody, which prevents the blood platelets from adhering. As a result, TTP can be forced back within a few days and the organs are protected from further decreased blood flow – which prevents at least some of the remaining damage.

75 patients were involved in the study. Together with the standard treatment, 36 patients received the new active ingredient and the 39 patients of the control group received a placebo. Bern-based haematologist Johanna Kremer Hovinga analysed all the blood samples and found that in 95

percent of patients, who received the new active ingredient, the acute phase of TTP had ended after scarcely 4 days, making this almost 40 percent faster than with the traditional treatment.

The side-effects were generally comparable, but light bleeding occurred more frequently with the new active ingredient (54% of patients in comparison with 38% with the placebo). As long as the medication was given, no patient had a relapse, although the autoimmune reaction continued to be active in at least 20 percent of patients. Another advantage: Because the new active ingredient can be injected subcutaneously, it was possible to treat patients on an outpatient basis after just a few days.

Long-term special subject at the Bern University Hospital and Bern University.
The Department of Hematology at the Bern University Hospital and the University of Bern have been researching TTP and ADAMTS13 since the mid-1990s and have published extensive work in this. This study in the New England Journal of Medicine is the first major randomised patient study in the field of the rare disorder of TTP and shows a promising new treatment approach based on expanded knowledge of TTP.

Link to study: http://www.nejm.org/doi/full/10.1056/NEJMoa1505533

Caption:Schematic presentation of platelets (purple) which stick to the over-long Von Willebrand factor (brown).
©Massachusetts Medical Society, excerpt of illustrating video.

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27th February: Rare Disease Day

The International Rare Disease Day is taking place for the 6th time in Switzerland in 2016. The organiser is the ProRaris Swiss Alliance for Rare Diseases. On the event website http://www.proraris.ch/de/journees-internationale-maladies-rares/, heart surgeon Thierry Carrel explains why the support and attention of the public is so important for rare diseases.