

PRESS RELEASE

Haemophilia: New milestones in treatment

New findings from two important studies with the participation of the University Hospital Bonn contribute to a better quality of life for haemophiliacs

Bonn, 9 February 2023 - Haemophilia A is the most common severe form of haemophilia. It affects almost exclusively the male sex. The disease can usually be treated well, but the medicines known so far do not help all of those affected. Now, two important studies involving the Haemophilia Centre of the University Hospital Bonn have been published in the New England Journal of Medicine and in Lancet Haematology, which represent new milestones for the treatment of haemophilia.

Patients with haemophilia A lack coagulation factor VIII which needs to be substituted so that the blood clots and the patients do not bleed. In a clinical phase 3 trial with the participation of the Haemophilia Centre of the University Hospital Bonn (UKB), a new drug with the complicated name, Efanesoctocog Alpha, has now been investigated for the treatment of haemophilia A for the first time. The new findings represent a milestone in haemophilia treatment and have now been published in the renowned New England Journal of Medicine.

Efanesoctocog Alpha is the first drug to contain a genetically engineered clotting factor VIII, which has a significantly prolonged duration of action of 47 hours. The study with 133 patients shows that blood clotting with the new drug is almost normalised over four days and provides very good protection against bleeding over seven days. The number of bleedings is reduced from three bleedings in 12 months with the classical treatment to only one bleeding in 18 months with Efanesoctocog Alpha. The long duration of action is achieved by a series of genetic modifications of the factor VIII protein, the most important

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of which is blocking the binding to the Von Willebrand factor, which otherwise determines the speed of factor VIII clearance.

In parallel, a study on the prophylaxis of less severe forms of haemophilia A with the monoclonal antibody emicizumab was published in Lancet Haematology. These patients have comparatively few bleedings, so that the classic bleeding-preventive therapy with multiple intravenous coagulation factor VIII administrations per week does not seem feasible for many patients. Nevertheless, the majority of patients in adulthood show significant bleeding sequelae, especially in the joints. Until now, emicizumab was only approved for the treatment of patients with severe haemophilia A. The current study now shows very good results, including a very good efficacy also in patients with a less severe form of haemophilia A. Thus, the patients treated with emicizumab, which was given subcutaneously only once every one to two weeks, had on average less than one bleeding episode per year. As with the earlier approval studies on emicizumab, the haemophilia centre of the UKB was also significantly involved in this study. Almost simultaneously with the publication, the approval of emicizumab for haemophilia A patients with a moderately severe course was granted.

Prof. Johannes Oldenburg, Director of the Institute for Experimental Haematology and Transfusion Medicine at the UKB, which also includes the Haemophilia Centre, states: "Efanesoctocog alpha with its prolonged duration of action and emicizumab for patients with a moderate course of the disease represent important milestones in the treatment of haemophilia A patients. This prevents bleeding and its consequences, especially joint damage and severe pain, so that patients with haemophilia A continue to have a good quality of life in adulthood."

The Haemophilia Centre at the University Hospital Bonn (UKB) is one of the largest centres in Europe. About 15 percent of haemophilia patients in Germany are treated here.

Publication:

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Picture:



Caption: Prof. Johannes Oldenburg is Director of the Institute for Experimental Haematology and Transfusion Medicine at the University Hospital Bonn (UKB)

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About the University Hospital Bonn: The UKB cares for about 500,000 patients per year, employs 8,800 people and has a balance sheet total of 1.5 billion euros. In addition to the more than 3,300 medical and dental students, a further 580 people are trained in numerous health professions each year. The UKB is ranked first among university hospitals in North Rhine-Westphalia in the science ranking and in the Focus clinic list and has the third-highest case mix index (severity of cases) in Germany.