US approval for bleeding disorder drug

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"VONVENDI [von Willebrand factor (Recombinant)]", the new drug from Baxalta Incorporated, a global biopharmaceutical company recently spun off from Baxter International, has just been approved by the United States Food and Drug Administration. It is the first, and so far the only, recombinant protein for the treatment of von Willebrand disease, and

offers those affected a new therapeutic option for the first time in more than 10 years.

VONVENDI relates to a substance patented by the Max Delbrück Center for Molecular Medicine (MDC) in the Helmholtz Association that arose from the work of Prof. Michael Bader and Dr Diego Walter. The scientists investigated serotonin-modulating agents that could be used for the treatment of bleeding disorders, including von Willebrand factor.

Some years ago Ascenion GmbH, the MDC's technology transfer partner, negotiated a licensing agreement between the MDC and Baxter that gave the company exclusive rights to use the recombinant factor for the treatment of bleeding disorders. In exchange, Baxter agreed to pay the MDC milestone payments and licensing fees. Beyond the licensing of the substance patent, the MDC was not involved in the development of the drug.

"Following the approval of Amgen's cancer drug Blincyto, this is the second product based on MDC patents to reach the market within the space of a few months," says Dr Christian Stein, CEO of Ascenion. "This is a great success for patients worldwide - and for technology transfer." The licensing revenues could enable the MDC to initiate further pioneering research projects.

The MDC's interim scientific director, Prof. Dr Thomas Sommer, says: "The transfer of our molecular biology research findings into application has been part of our mission since the MDC was founded. I'm all the more delighted to see two drugs reach the market in such a short space of time. This success underlines once again the value of basic research."

Von Willebrand disease is the most common inherited bleeding disorder. Genetic mutations result in changes in von Willebrand factor - a protein

required in blood clotting - that result in it being produced at reduced levels, or not at all. The result is an increased tendency to bleed which, depending on disease severity, can result in massive bleeding. It affects up to one in every 100 people worldwide, although only an estimated one in one million people suffers the most severe form of the disease.

Provided by Helmholtz Association of German Research Centres

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