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New $8.5M research grant aims to address patients affected by severe hemophilia A

Published on December 23, 2015 at 3:34 AM

People with hemophilia have prolonged abnormal bleeding as a result of trauma. Hemophilia A, also called factor VIII (FVIII) deficiency, is the most common form of the genetic disorder caused by missing or defective blood clotting protein called factor VIII. Severe hemophilia occurs in about 60% of cases where the deficiency of Factor VIII is less than 1% of normal blood concentration. While it is passed down from parents to children, about 1/3 of cases are caused by a spontaneous change in the gene.

A new $8.5M (CAD) research grant award by the European commission via its Horizon 2020 program aims to address patients affected by this condition. The funds are earmarked to the HemAcure consortium with the goal to bring treatment of hemophilia A, also called factor VIII (FVIII) deficiency, the most common form of hemophilia A into the clinic for human trials. The genetic disorder is caused by missing or defective factor VIII, a blood clotting protein. The consortium, consisting of Canadian-based Sernova Corp. and five European academic and private partners, will be working jointly to advance development of a GMP clinical grade Factor VIII releasing therapeutic cell product via Sernova's signature technology called the Cell Pouch™ for the treatment of severe hemophilia A.

Delivering the patient's own cells to Treat Hemophilia A via Cell Pouch System
The therapy being developed by the HemAcure consortium is expected to be highly disruptive to the current standard of care treatments for hemophilia A. The therapeutic uses the patient's own cells which have been corrected for the factor VIII gene. Center to the therapy is Sernova's Cell Pouch System™, a novel implantable and scalable medical device which forms a natural environment in the body for the housing and long-term survival and function of therapeutic cells. These therapeutic cells release necessary proteins or hormones missing from the body to treat chronic diseases as an alternative to daily administration of drugs.

The patients corrected cells placed in the implanted Cell Pouch™ will release factor VIII on a continual basis at a rate that would be expected to significantly reduce disease-associated hemorrhaging and joint damage. The constant delivery of factor VIII is also expected to reduce or eliminate the need for multiple weekly infusions which is the current standard of care using plasma-derived or recombinant, genetically engineered factor VIII for the prophylactic treatment of hemophilia A.
According to Dr. David Lillicrap, MD, FRCPC Professor Department of Pathology and Molecular Medicine Queens University, Canada Research Chair in Molecular Hemostasis and member of the HemAcure Scientific Advisory Board. "The therapeutic potential to have a constant release of factor VIII from a Hemophilia A patient's own genetically corrected cells placed within the implanted Cell Pouch™ would be a very significant advance in the treatment of hemophilia A. Sernova's Cell Pouch™ with its vascularized tissue lined chambers for therapeutic cells, which has already been proven for islet safety and survival in human clinical assessment of diabetes, is an ideal, fully scalable first-in-class medical device suitable for the potential treatment of hemophilia," said Dr. Lillicrap.

Global Problem of Hemophilia
According to the US Centers for Disease Control and Prevention, hemophilia occurs in about 1 in 5,000 births. If the prolonged bleeding occurs in the brain of a person with hemophilia, it can be fatal. Prolonged bleeding in joints can cause inflammatory responses and permanent joint damage. Approximately 20,000 people in the United States and 10,000 in Europe have the moderate or severe form of hemophilia A, as well as approximately 2,500 in Canada. All races and ethnic groups are equally affected by hemophilia A. Though there is no cure for the disease, it can be controlled with regular infusions of recombinant clotting Factor VIII.

The condition has an economical toll on patients. Annual costs for the treatment of the disease for each patient may range from $60,000 to $260,000 US for a total cost of between $2-5B per year in North America and Europe. The current standard-of-care involves regular infusions of factor VIII, which achieves normal factor VIII blood levels for only a few hours at a time. The product being developed by the HemAcure consortium will seek to provide constant delivery of Factor VIII to normalize blood levels in an effort to significantly improve the quality of life of patients suffering from hemophilia A.

According to Delfina Siroen, Sr. Director of Sernova's Research and Development team. "In a very short time, Sernova's hemophilia program has achieved great strides and the addition of this European grant and team will

ensure the best possible outcome for this program to the clinic." The preliminary preclinical proof of concept data used as a basis to support the foundation of the grant was generated in a collaborative agreement between Medicyte GmbH under the FP7 ReLiver project (No. 304961) and Sernova Corp where cryopreserved cells with the ex vivo inserted corrected gene for factor VIII were successfully shipped and assessed in Sernova's Cell Pouch™ at its headquarters in Canada.

Source:
Sernova Corp.