



Gene Therapy for Hemophilia A

University of Florida researchers have developed a novel treatment for Hemophilia A using adeno-associated virus (AAV) mediated gene transfer. Hemophilia A is a sex-linked recessive bleeding disorder caused by deficiency in clotting factor VIII. Approximately 17,000 Americans have Hemophilia A, which is currently treated by replacement therapy using plasma-derived or recombinant factor VIII. However, limitations to replacement therapy include cost, inconvenience, availability of factor VIII, and the short half-life of factor VIII in vivo. The most severe limitation to replacement therapy occurs when individuals build resistance to treatment due to immune response to the recombinant factor. The method developed by University of Florida researchers should require only one treatment with the AAV vector to enable lifetime disease correction, providing an alternative form of treatment for those patients who are resistant to recombinant factor VIII.

Applications

Long-term treatment of Hemophilia A

Advantages

- ◆ Optimally, a single delivery of AAV vector should result in disease correction for the lifetime of the individual, saving time, money, and the health of the afflicted individual
- ◆ Offers increased efficacy over standard treatment options, improving patient quality of life
- ◆ Provides effective treatment for those patients who are resistant to current therapies, offering an solution to more patients currently suffering from this disease

The Technology

This invention provides a novel treatment for Hemophilia A by Factor VII delivery via adeno-associated virus (AAV)-mediated gene transfer. Activated factor VII acts through the extrinsic pathway of the clotting cascade and circumvents the necessity for functional factor VIII. This therapy should provide long lasting treatment for Hemophilia A, and is particularly useful for those patients for whom conventional therapies are no longer effective.

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