Gene therapy successfully cures hemophilia in dogs

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis.

Hemophilia was once popularly referred to as “the royal disease.” It was passed through the blood line of Queen Victoria to her progeny, which affected several royal families in Spain, Germany and Russia.

Hemophilia is an inherited bleeding disorder that can be either X-linked, meaning passed along on the X chromosome of female carriers primarily affecting male offspring, since males inherit only one copy of the X chromosome containing the defective gene. Or they can be autosomal recessive, where two bad copies need to be inherited for the disease to manifest. Sufferers of this disorder run the potential risk of life-threatening bleeding, as their blood cannot clot properly.

A study published in the journal, *Blood* reveals how researchers were able to use a single gene therapy injection in dogs to successfully correct a rare type of hemophilia. Factor VII deficiency is an autosomal recessive bleeding disorder that affects about one in 500,000 people.

The research team used the adenovirus (virus that causes the common cold) as a carrier to inject the dogs with the corrected version of the Factor VII gene. Within three months of receiving the injection, the dogs had a level of Factor VII in their blood that would be considered therapeutic in humans. Additionally, the injection had a very safe side-effect profile, as evidenced by the dogs’ normal kidney and liver enzymes – no toxicity was observed, nor were there any issues with excessive clotting.

Read full, original post: Successful Gene Therapy for Rare Hemophelia