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Gene therapy for the X-linked bleeding disorder hemophilia

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Clinical gene therapy has enjoyed enormous progress in recent years, documenting successful treatments in patients with primary immune deficiencies, inherited forms of blindness, and other diseases. Cancer gene therapies are now also showing remarkable successes. After more than a decade of translational research toward gene therapy for the X-linked bleeding disorder hemophilia B (coagulation factor IX deficiencies) using *in vivo* gene transfer with adeno-associated viral (AAV) vector, Nathwani, Davidoff, and collaborators reported sustained therapeutic expression in the New England Journal of Medicine in 2011. Our own research had documented >8 years of therapy in hemophilia B dogs after a single hepatic administration of an AAV vector. Importantly, this approach induces immune tolerance to the therapeutic gene product, which critically depends on induction of regulatory T cells. However, pre-existing immunity to AAV capsid hampers gene therapy in humans because of neutralizing antibodies and memory CD8+ T cells. Our center is developing vectors with reduced MHC I presentation of capsid antigen and superior transduction efficiency of hepatocytes. In addition, we are optimizing vectors and gene transfer protocols to reliably achieve immune tolerance to factors VIII and IX upon hepatic gene transfer. This approach is also useful to eradicate humoral immune responses that may have developed during conventional factor replacement therapy. These efforts are expected to result in superior and safer gene therapy protocols for hemophilia in the future.

Biography

Dr. Herzog received a Ph.D. in Microbiology from Auburn University in 1996. He performed postdoctoral research in gene therapy at the Children's Hospital of Philadelphia until becoming faculty member at the University of Pennsylvania. In 2005, he joined the University of Florida, where he is currently Professor of Pediatrics, Molecular Genetics and Microbiology. Dr. Herzog's research has received several prestigious awards from foundations and societies. He served as chair of the immunology and education committees of the American Society of Gene Therapy and edited two textbooks. He is currently Deputy Editor of the journal Molecular Therapy and Executive Editor of the Journal of Genetic Syndromes and Gene Therapy, and member of the NIH study section Hemostasis and Thrombosis. His laboratory is funded by multiple grants from the NIH and other agencies.

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