‘One shot’ treatment for hemophilia B uses CRISPR and stem cells

Scientists at the Salk Institute have combined CRISPR-Cas9 gene editing with stem cell technology to generate a one-time, autologous cell therapy for the genetic blood clotting disorder hemophilia B. In vivo tests showed that gene-edited, stem cell–derived liver cells remained viable and functional in hemophiliac mice for nearly a year, after just a single injection.

Headed by Suvasini Ramaswamy, Ph.D., and Inder M. Verma, Ph.D., the Salk Institute team’s results offer proof of concept for the potential use of autologous cell therapy in the treatment of hemophilia B and potentially other liver disorders that are similarly caused by defects in a single gene.

Hemophilia B is an X-linked clotting disorder that affects 1 in 30,000 male births, the researchers explain. The blood clotting disorder is caused by lack of clotting factor IX (FIX), due to mutations in the FIX gene, and can manifest as either mild, moderate, or severe, dependent upon the extent of FIX activity remaining in patients. Current treatment involves giving patients frequent intravenous doses of recombinant FIX supplements.

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Dr. Ramaswamy acknowledges that “a lot of things have to happen before this can go into humans.” Nevertheless, as the authors state, the study demonstrates the “feasibility of autologous and heterologous cell therapy for treatment of hemophilia B.”

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