Hemophilia cure? First attempt at in vivo human genome editing

Researchers have edited the human genome before, but always in cells outside the body. Now, biotech company Sangamo Therapeutics is recruiting participants for clinical trials in which patients with hemophilia B, Hurler syndrome, or Hunter syndrome will have the gene coding for one of the enzymes that is non-functional in them stitched into their genomes at double-stranded DNA breaks caused by zinc finger nucleases.

“This is the first time someone could have a new gene put into their liver,” said Sangamo President and CEO Sandy Macrae….

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Hemophilia B “is a logical first disease to target because the disease physiology is pretty straightforward,” said Andrew Davidoff, a pediatric surgeon at St. Jude Children’s Research Hospital in Memphis, Tennessee. “If you just replace the missing or defective clotting factor you will essentially cure the patients,” he explained.

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Paula Cannon, a gene therapy researcher at the University of Southern California’s Keck School of Medicine in Los Angeles, [stated]…that there are advantages for zinc finger nucleases in developing clinical therapies [like]…their history and approval as investigational new drugs by the US Food and Drug Administration.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: First In Vivo Human Genome Editing to Be Tested in New Clinical Trial