Gene Repair For Hemophilia A

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SUMMARY

Researchers at UCLA from the Departments of Medicine and Urology have developed a treatment for hemophilia A that utilizes non-viral gene editing technologies for *ex vivo* repair of the mutation in the gene F8.

BACKGROUND

Hemophilia affects about 400,000 people worldwide, and approximately 80% of these cases are hemophilia A. Hemophilia A is an X-linked genetic disease caused by a mutation of the F8 gene. This mutation results in a deficiency of the blood coagulation protein factor VIII. Patients with the disease typically have spontaneous bleeding, as well as joint and muscle damage. These injuries can result in crippling arthropathy and physical disability. The current treatment for hemophilia involves the administration of clotting factors, either from a plasma infusion or recombinant protein production. Although these methods effectively manage hemophilia A, they are not curative. In addition, long-term treatment with recombinant proteins can cause immune responses due to contaminants from the source of purification. Plasma infusions possess risks associated with blood transfusions, such as viral transmission, alloimmunization, excessive intravascular volume, and acute lung injury. Ongoing efforts have attempted to use viral vectors, such as adeno-associated virus or lentivirus, to treat hemophilia patients. While there is great merit in these approaches, none has demonstrated efficacy for treating hemophilia A in humans. Both viral techniques have safety risks, including immunogenicity or incorporation into the genome, which results in dysregulation of normal cell differentiation. Thus, a safe and curative treatment for hemophilia A is greatly needed.

INNOVATION

Researchers at UCLA from the Departments of Medicine and Urology have developed a treatment for hemophilia A that utilizes non-viral gene editing technologies, such as transcriptional activator-like effector nucleases (TALENs), zinc finger nucleases (ZFNs), and CRISPR-Cas, for *ex vivo* repair of the mutation in the gene F8. This autologous treatment is expected to restore a full length and functional Factor VIII and avoid the safety risks associated with viral-mediated gene repair.

APPLICATIONS

The treatment method suggested by the inventor offers a novel, curative treatment for hemophilia A

- Patients will no longer require administration of blood clotting factors and other life-long forms of treatment

ADVANTAGES

- All techniques utilized in this method for treating hemophilia A are established techniques
- Curative – fully repairs gene mutation and associated protein function
- Safer than using viral vectors to repair genetic mutations
- If successful, the strategy scheme proposed by the inventor can be applied to other genetic diseases

STATE OF DEVELOPMENT

This technology is at the conceptual phase. The inventors plan to perform several key *in vivo* experiments using canine animal models and *ex vivo* experiments in patient samples.

PATENT STATUS

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OTHER INFORMATION

KEYWORDS

Hemophilia A, gene editing, gene therapy, F8, Factor VIII, blood coagulation protein, transcriptional activator-like effector nucleases, TALEN, Zinc Finger Nucleases ZFN, CRISPR-Cas

CATEGORIZED AS

- **Medical**
  - Gene Therapy
  - Therapeutics

RELATED CASES

2014-016-0

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	11,083,801	08/10/2021	2014-016
United States Of America	Issued Patent	10,272,163	04/30/2019	2014-016
European Patent Office	Published Application	2928303	10/14/2015	2014-016

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