November 18, 2019

CRISPR Gene Editing Eliminates Herpes Simplex Virus and JC Virus, Demonstrating Feasibility of a Potential Functional Cure

Excision BioTherapeutics showed that CRISPR excised herpes simplex virus (HSV) and JC Virus genomes from cell lines to establish proof-of-concept for clinical applications

Oakland, CA, Nov. 18, 2019 (GLOBE NEWSWIRE) -- At the2019 International Symposium on Neurovirology, Excision BioTherapeutics, a gene therapy company focusing on curing viral infectious diseases, presented multiple preclinical abstracts, which revealed that gene editing strategies are promising tools for eradicating viral genomes and possible cures for HSV and JC virus-induced progressive multifocal leukoencephalopathy (PML). Entitled *"Inhibition of HSV-1 Replication In-Vitro and In-Vivo by a Gene Editing Strategy,"* and *"CRISPR/Cas9 System as an Agent for Inhibition of Polyomavirus JC Infection,"*the studies demonstrate the power of gene editing as a potential curative therapy.

There are no current effective therapies for JC virus-induced progressive multifocal leukoencephalopathy (PML). Similarly, patients with HSV have limited treatment options. Researchers working with collaborators at the Lewis Katz School of Medicine at Temple University (LKSOM) demonstrated the ability to remove JC Virus and HSV from cell lines and animals using CRISPR, a powerful gene editing technology. The teams combined multiple guide RNAs (gRNAs) to cut multiple locations to deactivate the viral genomes. The team used AAV9, AAV6 and AAV2 mediated delivery of CRISPR/Cas9, which led to viral suppression.

"For the first time, the team led by Drs. Jennifer Gordon, Ilker Sariyer, and Hassen Wollebo at Temple, have demonstrated proof-of-concept in cell culture using CRISPR to suppress these devastating infections," said Dr. Kamel Khalili, Chair of the Department of Neuroscience and Director of the Center for Neurovirology at LKSOM and principal scientific advisor at Excision BioTherapeutics. "Excision's technology leverages multiple gRNAs to generate multiple excision sites within viral genomes, thereby deactivating viral infections."

The scientists at LKSOM who are associated with Excision BioTherapeutics were the first to demonstrate a functional cure for HIV in animals, in collaboration with Dr. Howard Gendelman and his team at the University of Nebraska Medical Center, published in *Nature Communications* in 2019.

"The Excision team envisions a future where the world's most devastating viral diseases have functionally curative treatments," commented Daniel Dornbusch, Excision's CEO. He continued, "Both JC virus-induced PML and HSV require vastly improved therapies to treat

many patients with significant unmet needs.”

Excision is developing CRISPR-based gene therapies delivered by a single intravenous infusion to cure viral infectious diseases. The company’s lead compound, EBT-101, a treatment for HIV, will enter human clinical trials in 2020.

About Excision BioTherapeutics

Excision BioTherapeutics, Inc. is a biotechnology company developing CRISPR-based therapies to cure viral infectious diseases. Excision is focused on improving the lives of chronically ill patients by eliminating viral genomes from infected individuals. By using CRISPR in unique ways, the company has already demonstrated the first functional cure for HIV in animals. Excision is developing technologies and IP developed at Temple University and U.C. Berkeley. Excision is located in Oakland, California and is supported by ARTIS Ventures, Norwest Venture Partners, SilverRidge Venture Partners, Oakhouse Ventures, and Gaingels. For more information, please visit www.excisionbio.com.

Editor’s Note: Dr. Khalili is a named inventor on patents that cover the CRISPR/CAS9 system viral gene editing technology. In addition, Dr. Khalili is a co-founder, scientific advisor, and has received equity and monetary compensation from Excision BioTherapeutics, a biotech start-up which has licensed the viral gene editing technology from Temple University for commercial development and clinical trials.

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Source: Excision BioTherapeutics