HIV Eliminated from the Genomes of Living Animals; Excision BioTherapeutics Advancing the Innovation to Clinical Development

Study published in Nature Communications marks a critical step toward the development of a possible cure for human HIV infection

Philadelphia, July 2, 2019 -- Researchers at the Lewis Katz School of Medicine at Temple University and the University of Nebraska Medical Center (UNMC) have for the first time in history eliminated replication-competent HIV-1 DNA – the virus responsible for AIDS – from the genomes of living animals. The study, reported today in the journal Nature Communications, marks a critical step toward the development of a possible cure for human HIV infection. Excision BioTherapeutics holds the exclusive license for commercial application of these advancements as it works on the development and commercialization of advanced gene editing therapeutics for the treatment of life-threatening diseases caused by viruses.

The next step is for the company to enter into clinical trials with its HIV-1 targeted CRISPR platform, giving Excision BioTherapeutics the first effort to fully remove/excise the HIV-1 genome from all human cells and tissues.

“We are incredibly proud of the medical breakthroughs made by this team of scientists. As the exclusive license holders from Temple on the technology and patents behind these innovations, Excision is working closely with the team to bring these advances to human clinical trials and to patients in need around the world," said Kamel Khalili Ph.D., Professor and Chair of Neuroscience, Lewis Katz School of Medicine at Temple University and founder and Principal Scientific Advisor to Excision BioTherapeutics.

The results of the study validate the use of a novel approach to using the powerful CRISPR-Cas9 gene editing and gene therapy platform in order to remove HIV DNA from genomes harboring the virus. Using the proprietary approach to CRISPR-Cas9 gene editing, HIV-infected mice were able to be removed from antiretroviral therapy without viral rebound from previously-infected cells.

Added Kamel: ”The results further validate the efforts of Excision to take the product forward to patients, and it’s one of the primary reasons I became a founding member of the company. The next step is to bring this from the bench to the clinic, and to make this life-changing development commercially available.”

For the new study, Dr. Khalili and colleagues combined their gene editing system with long-acting slow-effective release (LASER) ART, co-developed by Dr. Gendelman and Benson Edagwa, PhD, Assistant Professor of Pharmacology at UNMC. LASER ART packages HIV antiretroviral drugs into nanocrystals which reduces the frequency of ART administration and enables more effective HIV studies in animal models.

HIV is a global epidemic, affecting almost 37 million people. The disease most commonly affects patients during their most productive years, with about one third of new infections occurring in people aged 15-24. In the United States alone approximately 38,000 new infections occur annually. Although the virus is treatable, resistant strains have arisen against some medications causing concern in the medical community, making advancements more urgent than ever.

Excision BioTherapeutics is privately held and has raised funding by ARTIS Ventures, Oakhouse Partners, and Gaingels.

About Excision BioTherapeutics Inc.
Excision BioTherapeutics Inc. is a life science company focused on the development and commercialization of advanced gene editing therapeutics for the treatment and eradication of life-threatening disease caused by neurotropic viruses (viral infections).

The company was founded in 2015 out of Temple University's Lewis Katz School of Medicine in Philadelphia, PA, and has licensed the viral gene editing technology from Temple University and UC Berkeley for commercial development and clinical trials. The team has progressed to produce a considerable intellectual property portfolio covering the use of the CRISPR and other gene editing tools in relation to a targeted pipeline of indications, as well as innovative companion diagnostics. For more information 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. Drs. Gendelman and Edagwa are named inventors on patents that cover the LASER ART technology. Dr. Gendelman also is the founder and chief scientific officer of Brain First, Inc. Drs. Khalili and Gendelman have not received financial compensation from any other third parties for any aspects of this published work.