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Excision BioTherapeutics Doses First Participant in EBT-101 Phase 1/2 Trial Evaluating EBT-101 as a Potential Cure for HIV

- *EBT-101 is an in vivo CRISPR-based therapeutic designed to remove HIV proviral DNA from affected cell reservoirs*
- *First-in-human study will evaluate the safety and efficacy of EBT-101 in participants who chronically suppress HIV with daily antiretroviral therapy*
- *EBT-101 has been well tolerated to-date and the trial is proceeding as planned*

SAN FRANCISCO, Sept. 15, 2022 (GLOBE NEWSWIRE) -- Excision BioTherapeutics, Inc., a clinical-stage biotechnology company developing CRISPR-based therapies intended to cure viral infectious diseases, today announced that the first participant has been dosed in the Phase 1/2 clinical trial of EBT-101 for human immunodeficiency virus type 1 (HIV-1). The participant was dosed in July 2022, with initial findings indicating EBT-101 has been well tolerated to-date. The participant continues to be monitored for safety and is expected to qualify for analytical treatment interruption (ATI) of their background anti-retroviral therapy (ART) in an evaluation of a potential cure.

“Dosing the first participant with EBT-101 is a landmark event that solidifies Excision’s position as a pioneer in gene editing,” said Daniel Dornbusch, Chief Executive Officer of Excision. “It is the first time a CRISPR-based therapy targeting an infectious disease has been administered to a patient and is expected to enable the first ever clinical assessment of a multiplexed, *in vivo* gene editing approach. We were able to reach this watershed moment thanks to years of innovative work by leading scientists and physicians, to whom we are immensely grateful. With this achievement, Excision has taken a major step forward in developing a one-time treatment that could transform the HIV pandemic by freeing affected people from life-long disease management and the stigma of disease.”

EBT-101 is a unique, clinical-stage *in vivo* CRISPR-based therapeutic designed to cure HIV infections after a single intravenous infusion. EBT-101 employs an adeno-associated virus (AAV) to deliver CRISPR-Cas9 and dual guide RNAs, enabling a multiplex editing approach that simultaneously targets three distinct sites within the HIV genome. This allows for the excision of large portions of the HIV genome, thereby minimizing potential viral escape.

Rachel M. Presti, M.D., Ph.D., Associate Professor of Medicine at Washington University School of Medicine in St. Louis and Principal Investigator of the Phase 1/2 trial added, “Using gene editing to target the HIV DNA reservoirs in human cells is a novel investigational approach, designed to cure HIV. We are well-positioned to collect key data in this first-in-human study, to determine whether we can duplicate the success seen using this approach in animal models.”

Kamel Khalili, Ph.D., Chair of the Department of Microbiology, Immunology and Inflammation, Director of the Center for Neurovirology and Gene Editing, and Director of the Comprehensive NeuroAIDS Center at the Lewis Katz School of Medicine at Temple University and Co-founder of Excision said, “There are nearly 40 million people worldwide suffering from the effects of HIV with no curative treatments available more than 40 years after its discovery. We believe EBT-101 can address the long-standing unmet needs of people living with HIV by removing HIV DNA from their cells, thereby eradicating their infections. We believe we are well-positioned to collect key data that will enable our efforts to translate the success this approach has shown in animal models to human clinical trial participants. We look forward to investigating this hypothesis through the EBT-101 clinical program and are pleased that the EBT-101 Phase 1/2 trial is proceeding as planned.”

About the EBT-101 Clinical Program

The EBT-101 Phase 1/2 trial is an open-label, multi-center single ascending dose study designed to evaluate the safety, tolerability and preliminary efficacy of EBT-101 in approximately nine participants with HIV-1 who are suppressed on antiretroviral therapy. The clinical program is supported by preclinical studies that included positive long-term non-human primate safety data and efficacy data in transgenic mice showing the potential to cure HIV when treated with EBT-101. The primary objective of the trial is to assess the safety and tolerability of a single dose of EBT-101 in study participants with undetectable viral load on antiretroviral therapy (ART). Biodistribution, pharmacodynamic, and efficacy assessments will also be conducted. All participants will be assessed for eligibility for an analytical treatment interruption (ATI) of their background ART at Week 12. Following the initial 48-week follow up period, all participants will be enrolled into a long-term follow up protocol. For more information, see ClinicalTrials.gov identifiers NCT05144386 (Phase 1/2 trial) and NCT05143307 (long-term follow up protocol).

About Excision BioTherapeutics, Inc.

Excision BioTherapeutics, Inc. is a clinical-stage biotechnology company developing CRISPR-based therapies as potential cures for viral infectious diseases. EBT-101, the Company’s lead program, is an *in vivo* CRISPR-based therapeutic designed to cure HIV infections after a single intravenous infusion. Excision’s pipeline unites next-generation CRISPR nucleases with a novel gene editing approach to develop curative therapies for Herpes Virus, JC Virus, which causes PML, and Hepatitis B virus. Excision’s foundational technologies were developed in the laboratories of Dr. Kamel Khalili at Temple University and Dr. Jennifer Doudna at the University of California, Berkeley. For more information, please visit www.excision.bio.

Editor’s Note: Dr. Kamel Khalili is Co-Founder and Chief Scientific Consultant and holds equity in Excision BioTherapeutics, which has licensed the viral gene-editing technology from Temple University. Kamel Khalili is a named inventor on patents that cover the viral gene-editing technology. Dr. Tricia Burdo holds equity in Excision BioTherapeutics. These named researchers are employed by Temple University and conduct research activities sponsored by the company. Temple University also holds an equity interest in Excision. Temple University could ultimately potentially benefit financially from the outcome of this research. These interests have been reviewed and approved by Temple University in accordance with its Institutional Conflict of Interest policy. Questions about affiliations or interests can be directed to coitemple@temple.edu.

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