Source: *Excision BioTherapeutics*

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Excision BioTherapeutics Receives FDA Fast Track Designation for EBT-101, a First-in-Class CRISPR-Based Gene Therapy Candidate to Functionally Cure HIV-1

- EBT-101 is a potentially curative, one-time CRISPR-based treatment which makes two cuts in integrated retroviral DNA to remove large portions of the HIV genome and prevent HIV from escaping and reproducing
- Excision's ongoing Phase 1/2 trial is the first clinical evaluation of a CRISPR-based therapy targeting an infectious disease using a multiplexed *in vivo* gene editing approach
- HIV-1 affects 38 million people worldwide, including 1.1 million in the US, with no available cures

SAN FRANCISCO, July 20, 2023 (GLOBE NEWSWIRE) -- Excision BioTherapeutics, Inc., a clinical-stage biotechnology company developing CRISPR-based therapies to cure viral infectious diseases, today announced that the US Food and Drug Administration (FDA) has granted Fast Track designation for its CRISPR-based therapy, EBT-101, a dual-cut CRISPR-based gene therapy for the treatment of human immunodeficiency virus type 1 (HIV-1). EBT-101 is being tested in an ongoing Phase 1/2 trial to assess its safety and tolerability in adults (18 to 65 years old) with HIV-1 who are on continuous antiretroviral therapy with HIV RNA below the level of detection.

Fast Track designation is intended to facilitate the development and expedite the review of potentially important new drugs to treat serious conditions with unmet medical needs. Therapies granted this designation are given the opportunity for more frequent interactions with the FDA and may qualify for other designations including accelerated approval and priority review.

Daniel Dornbusch, Chief Executive Officer of Excision, said, "We are pleased with the FDA's decision to grant Fast Track designation to EBT-101. This designation underscores the importance of finding a cure for people living with HIV and bolsters Excision's efforts to rapidly develop potentially curative therapies for significant unmet medical needs."

HIV-1 affects 38 million people worldwide, including 1.1 million in the US, many of whom face significant health complications and the challenge of social stigma from the disease. Without a cure, the current standard of care is antiretroviral therapy (ART) that must be taken for the duration of an individual's life. ART effectively reduces viral loads and can reduce the risk of HIV transmission but, it does not eliminate latent HIV in the body which can cause significant medical conditions.

About EBT-101

EBT-101 is a unique, *in vivo* CRISPR-based therapeutic designed to cure HIV infection after a single intravenous infusion. EBT-101 employs an adeno-associated virus (AAV) to deliver CRISPR-Cas9 and dual guide RNAs, enabling a multiplexed *in vivo* 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.

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 primary objective of the trial is to assess the safety and tolerability of a single dose of EBT-101 in study participants with an undetectable viral load on antiretroviral therapy. 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 post EBT-101 administration.

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 infection 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.

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