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Excision BioTherapeutics Awarded California Institute for Regenerative Medicine (CIRM) Grant to Support Ongoing Phase 1/2 Trial Evaluating EBT-101 as a Potential Cure for HIV

- *CIRM Grant of $6.85 million supports the first-in-human clinical study evaluating the safety and efficacy of EBT-101 in participants people living with HIV*
- *EBT-101 is an in vivo CRISPR-based therapeutic designed to remove HIV proviral DNA*

SAN FRANCISCO, Sept. 29, 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 California Institute for Regenerative Medicine (CIRM) has awarded Excision a $6.85 million grant to support the clinical development of the EBT-101 program for human immunodeficiency virus type 1 (HIV-1).

Daniel Dornbusch, Chief Executive Officer of Excision, commented, “We are honored that CIRM has recognized the potential value of the EBT-101 program and our dual-guide RNA CRISPR approach to developing curative therapies for HIV-1 as well as other serious viral diseases with significant unmet needs. The CIRM grant provides further validation for the EBT-101 clinical trial, which is the first ever to evaluate an *in vivo* CRISPR-based therapy in an infectious disease. The grant will provide Excision with important funding to advance the trial and potentially demonstrate the safety and efficacy of removing viral DNA from people affected by the HIV pandemic.”

Excision recently reported the first participant in the EBT-101 Phase 1/2 clinical trial was dosed in July 2022, with initial findings indicating the therapeutic 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.

“To date only a handful of people have been cured of HIV/AIDS, so this proposal of using gene editing to eliminate the virus could be transformative,” says Maria T. Millan, MD, President and CEO of CIRM. “In California alone there are almost 140,000 people living with HIV. HIV infection continues to disproportionately impact marginalized populations, many of whom are unable to access the medications that keep the virus under control. A functional cure for HIV would have an enormous impact on these communities, and others around the world.”

About EBT-101
EBT-101 is a unique, *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.

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 humanized 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 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 CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission. To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies. With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality. For more information go to www.cirm.ca.gov.

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.

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