May 13, 2024

Excision BioTherapeutics Announces Data from the Phase 1/2 Trial of EBT-101 in HIV And In Vivo Efficacy Data in Herpes Virus and Hepatitis B

- *EBT-101 successfully met the primary endpoint of safety and secondary endpoint of biodistribution/immunogenicity in a Phase 1/2 study (EBT-101-001)*
- *EBT-104 successfully reduced Herpes Virus DNA and biomarkers in a rabbit model of herpes keratitis with application to HSV-1 and HSV-2*
- *EBT-107 successfully reduced Hepatitis B DNA and biomarkers in a mouse model of Hepatitis B*

As First in Human Study, EBT-101-001 provided invaluable insights on the potential of CRISPR to address infectious diseases impacting millions of people

SAN FRANCISCO, May 13, 2024 (GLOBE NEWSWIRE) -- Excision BioTherapeutics, Inc. (“Excision”, the “Company”), a clinical-stage biotechnology company developing CRISPR-based therapies to cure latent viral infectious diseases, summarized the novel presentation of multiple studies at this week’s American Society of Gene and Cell Therapy (ASGCT) meeting. Excision's HIV program was the first *in vivo* CRISPR-based systemic treatment evaluated in a clinical trial in the United States. The EBT-101-001 clinical study was an open-label design to assess safety and tolerability as its primary endpoint.

“Evaluating any new molecular entity begins by first and foremost assessing participant safety, and this is particularly true when exploring the potential of new treatment modalities such as CRISPR-based therapeutics,” said Daniel Dornbusch, Chief Executive Officer of Excision. As a First-in-Human trial, EBT-101-001 was designed for the specific purpose of assessing the safety and tolerability of systemically administered CRISPR. The primary endpoint of the study was safety and tolerability, and the secondary endpoints of the study were biodistribution and immunogenicity.

“We know that many people were hopeful that a first trial could provide evidence of a possible cure for HIV because the field has been waiting over 20 years for a cure. However, it was essential that this clinical trial establish safety for EBT-101 as a gene therapy product as well as safety related to the use of CRISPR for the field,” stated William Kennedy, M.D., Senior Vice President, Clinical at Excision.

Rachel M. Presti, M.D., Ph.D., Professor of Medicine at Washington University School of Medicine in St. Louis and a Principal Investigator of the Phase 1/2 trial added, “Initial data from the EBT-101-001 trial provides important clinical evidence that a gene editing treatment modality can be safely delivered for targeting the HIV DNA reservoirs in human cells. This study provides researchers with invaluable insights for how CRISPR technology can be

applied for addressing infectious disease and was an important first step towards additional programs designed to optimize this treatment modality for treating the millions of individuals who are impacted by HIV and other infectious disease."

Specifics about the issued data:

- In the EBT-101 clinical trial, there were no serious adverse events and only grade 1 adverse events were associated with the therapy. All events, including those not deemed associated with the therapy, were either grade 1 or 2 (out of 5) and resolved without treatment.
- In the EBT-104 preclinical studies, a single dose of therapy reduced Herpes Virus DNA by over 99.99% in Vero cells and nearly eliminated (11 out of 12) viral shedding in the rabbit keratitis model.
- In the EBT-107 preclinical studies, a single dose of a lipid nanoparticle (LNP) delivered CRISPR compound reduced HBV DNA, HBsAg (s-antigen), and HBeAg (e-antigen) by 98%, 97%, and 92% respectively in a mouse model of HBV. Because EBT-107 is delivered by LNP, the compound can be re-dosed to potentially achieve therapeutic target efficacy.

"We are thrilled with the substantial and diverse advances the Excision Research and Development team have shown in these multiple studies," noted Dr. Jennifer Gordon, Senior Vice President, R&D at Excision. She added, "We have very quickly demonstrated the power and potential of CRISPR-based therapies to treat viral infectious diseases."

About the EBT-101-001 Clinical Study
EBT-101-001 is a First in Human open-label, sequential cohort, single ascending dose study of EBT-101 administered intravenously to HIV-1 infected adults on stable antiretroviral therapy (ART). The primary outcome measure from the trial was to assess the safety and tolerability of a single dose of EBT-101 in study participants with undetectable viral load on antiretroviral therapy. The secondary endpoint of the trial was assessing EBT-101 biodistribution and immunogenicity.

About Excision BioTherapeutics, Inc.
Excision BioTherapeutics, Inc. develops CRISPR-based medicines as potential cures for serious chronic viral infectious diseases based on its proprietary multiplexed gene editing platform that unites next-generation CRISPR nucleases with a novel gene editing approach to develop curative therapies. The Company's pipeline targets large, underserved markets including herpes virus (HSV-1 and HSV-2), hepatitis B virus (HBV), and human immunodeficiency virus-1 (HIV-1). 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.

Contact:
John Fraunces
LifeSci Advisors
917-355-2395
jfraunces@lifesciadvisors.com

Source: Excision BioTherapeutics