European Medicines Agency Grants Orphan Drug Designation to AlloVir's Viralym-M, an Allogeneic, Off-the-Shelf, Multi-Virus Specific T-Cell Therapy

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Viralym-M recently received EMA PRIME and U.S. FDA RMAT designations

Phase 3 pivotal and Phase 2 proof-of-concept studies to be initiated for Viralym-M in 2020 targeting six devastating and life-threatening viral pathogens in immunocompromised patients

Cambridge, MA, March 26, 2020 – AlloVir, a late-clinical stage allogeneic T-cell immunotherapy company, today announced it has been granted Orphan Drug Designation from the European Medicines Agency (EMA) for Viralym-M (ALVR105) as a potential treatment of viral diseases and infections in patients undergoing hematopoietic stem cell transplantation (HSCT). Viralym-M is the company's lead allogeneic, off-the-shelf, multi-virus specific T-cell therapy, being developed for the treatment and prevention of serious viral diseases caused by six commonly occurring, devastating viral pathogens in immunocompromised individuals: BK virus, cytomegalovirus, human herpes virus-6, Epstein Barr virus, adenovirus, and JC virus. Viral diseases are a primary reason for poor outcomes in transplant patients, resulting in potentially devastating and life-threatening consequences.

In addition to Orphan Drug Designation, Viralym-M has been granted PRIority MEdicines (PRIME) designation from the EMA and Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA). Viralym-M is one of only seven investigational therapies, to date, to receive both PRIME and RMAT designations from the EMA and FDA, respectively. AlloVir plans to initiate Phase 3 pivotal and Phase 2 proof-of-concept studies with Viralym-M in 2020 targeting six commonly occurring, devastating and life-threatening viral pathogens.

"At AlloVir, we are committed to advancing allogeneic, off-the-shelf novel T-cell therapies with the potential to improve the way we treat and prevent devastating viral diseases," said Agustin Melian, MD, Chief Medical Officer and Head of Global Medical Sciences of AlloVir. "The Orphan Drug Designation by the EMA acknowledges the critical need for new treatment options for patients who have undergone stem cell transplant and are at risk of the serious consequences of viral diseases. Also, leveraging PRIME and RMAT designations, we are working to quickly advance Viralym-M through late-stage clinical development to bring, what we believe to be a transformative new therapy, to patients in the U.S., European Union and eventually around the world."

The EMA grants Orphan Drug Designation status for products intended for the treatment, prevention or diagnosis of life-threatening or chronically debilitating conditions that affect no more than five in 10,000 people in the European Union, and where the product represents a significant benefit over existing treatments. Orphan Drug Designation provides companies with certain benefits and incentives in the EU, including a 10-year period of market exclusivity after product approval, reduced regulatory fees and protocol assistance.

About Opportunistic Viral Diseases

In healthy individuals, virus-specific T-cells from the body's natural defense system provide protection against numerous disease-causing viruses. However, in patients with a weakened immune system these viruses may be uncontrolled. Viral diseases are common, with potentially devastating and life-threatening consequences in immunocompromised patients. For example, up to 90% of patients will reactivate at least one virus following an allogeneic HSCT and two-thirds of these patients reactivate more than one virus, resulting in significant and prolonged morbidity, hospitalization and premature death. Typically, when viruses infect immunocompromised patients, standard antiviral treatment does not address the underlying problem of a weakened immune system and therefore, many patients suffer with life-threatening outcomes such as multi-organ damage and failure, and even death.

About Viralym-M (ALVR105)

AlloVir's lead product Viralym-M (ALVR105) is in late-stage clinical development as an allogeneic, off-the-shelf, multi-virus specific T-cell therapy targeting six common viral pathogens in immunocompromised individuals: BK virus, cytomegalovirus, adenovirus, Epstein-Barr virus, human herpesvirus 6, and JC virus. In a positive Phase 2 proof-of-concept study, published in the *Journal of Clinical Oncology* (Tzannou, JCO, 2017), greater than 90% of patients who failed conventional treatment and received Viralym-M, demonstrated a predefined criteria for a complete or partial clinical response, most with complete elimination of detectable virus in the blood and resolution of major clinical symptoms. The company plans to initiate pivotal and proof-of-concept studies with Viralym-M in 2020 for treatment and prevention of severe and life-threatening viral diseases.

Viralym-M has received Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA) and PRIority MEdicines (PRIME) designation from European Medicines Agency (EMA).

About AlloVir

AlloVir, formerly ViraCyte, is an ElevateBio portfolio company that was founded in 2013 and is the leader in the development of novel cell therapies with a focus on restoring natural immunity against life-threatening viral diseases in patients with severely weakened immune systems. The company's technology platforms deliver commercially scalable solutions by leveraging off-the-shelf, allogeneic, multi-virus specific T cells targeting devastating viral pathogens for immunocompromised patients under viral attack. AlloVir's technology and manufacturing process enables the potential for the treatment and prevention of a spectrum of devastating viruses with each single allogeneic cell therapy. The company is advancing multiple mid- and late-stage clinical trials across its product portfolio.

AlloVir's investors include Fidelity Research and Management Company, Gilead Sciences, F2 Ventures, The Invus Group, Redmile Group, EcoR1, Samsara Biocapital, and Leerink Partners Co-investment Fund, LLC.

AlloVir is an ElevateBio portfolio company. More information can be found at www.allovir.com.

About ElevateBio

ElevateBio, LLC, is a Cambridge-based creator and operator of a portfolio of innovative cell and gene therapy companies. It begins with an environment where scientific inventors can transform their visions for cell and gene therapies into reality for patients with devastating diseases. Working with leading academic researchers, medical centers, and corporate partners, ElevateBio's team of scientists, drug developers, and company builders are creating a portfolio of therapeutics companies that are changing the face of cell and gene therapy and regenerative medicine. Core to ElevateBio's vision is BaseCamp, a centralized state-of-the-art innovation and manufacturing center, providing fully integrated capabilities, including basic and transitional research, process development, clinical development, cGMP manufacturing, and regulatory affairs across multiple cell and gene therapy and regenerative medicine technology platforms. ElevateBio portfolio companies, as well as select strategic partners are supported by ElevateBio BaseCamp in the advancement of novel cell and gene therapies.

ElevateBio's investors include F2 Ventures, MPM Capital, EcoR1 Capital, Redmile Group, Samsara BioCapital, Emerson Collective, The Invus Group, Surveyor Capital (A Citadel company), EDBI, and Vertex Ventures.

ElevateBio is headquartered in Cambridge, Mass, with ElevateBio BaseCamp located in Waltham, Mass. For more information, please visit www.elevate.bio.

Media contact:
Courtney Heath
ScientPR
AlloVirPR@scientpr.com
617-872-2462