FDA Grants Regenerative Medicine Advanced Therapy (RMAT) Designation to AlloVir's Posoleucel for the Treatment of Adenovirus (AdV) Infections in Adults and Children Post-Allogeneic Stem Cell Transplantation

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Treatment of AdV infection is posoleucel's second potential indication to receive RMAT designation

Phase 3 registrational study of posoleucel for AdV treatment is now open for enrollment

Proof-of-concept study of ALVR106 for the treatment of multiple respiratory viral infections is also open for enrollment

WALTHAM, Mass.--(BUSINESS WIRE)--Jan. 5, 2022-- AlloVir, a late-clinical stage allogeneic T-cell immunotherapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted its lead multi-virus specific T cell (VST) therapy, posoleucel (Viralym-M, ALVR105), Regenerative Medicine Advanced Therapy (RMAT) designation for the treatment of adenovirus (AdV) infection following allogeneic hematopoietic stem cell transplant (allo-HCT). The designation is based on positive results from the Phase 2 CHARMS study.

RMAT designation recognizes the potential for posoleucel to address the unmet medical need posed by AdV, a potentially life-threatening condition with no approved treatment options. This designation enables early interactions with the FDA to discuss clinical trial design and other actions to expedite development and review. The FDA previously granted RMAT designation to posoleucel for the treatment of hemorrhagic cystitis (HC) caused by BK virus in adults and children following allo-HCT.

"We are pleased that the FDA has granted posoleucel RMAT designation for a second treatment-related indication," said Ercem Atillasoy, M.D., Chief Regulatory and Safety Officer, AlloVir. "The two RMAT designations reinforce the potential of posoleucel in areas of urgent patient need. We look forward to working closely with FDA as we continue to advance posoleucel through late-stage clinical development."

A Phase 3 registrational study (NCT05179057) of posoleucel for the treatment of AdV viremia is now open and enrolling pediatric and adult patients following allo-HCT. This study is the second Phase 3 registrational study of posoleucel, following the initiation of the Phase 3 study for the treatment of virus-associated HC last year.

Separately, the company also announced the initiation of a Phase 1/2 clinical trial (NCT04933968) of ALVR106, its investigational, allogeneic, off-the-shelf, multi-VST therapy for the treatment of infections caused by human metapneumovirus (hMPV), influenza, parainfluenza virus (PIV) and respiratory syncytial virus (RSV). This trial extends AlloVir's VST platform to tackle respiratory viruses that pose a considerable risk for autologous and allo-HCT patients.

"The initiation of these posoleucel and ALVR106 studies represents important progress in our effort to bring forward much needed treatment options for immunocompromised patients," said Richard Riese, M.D., Ph.D., Senior Vice President, Clinical Research, AlloVir. "These investigational multi-VST therapies aim to restore immunity against viruses that, if left untreated, can have devastating consequences. We look forward to working with study investigators to enroll these studies and demonstrate the potential for VST therapy in areas of urgent unmet need."

About Adenovirus and the Posoleucel Phase 3 Study

AdV is a potentially life-threatening viral infection that has no approved treatments. AdV viremia occurs in approximately one third (32%) of pediatric allo-HCT patients and 6% of adult allo-HCT patients. The spectrum of AdV-associated disease in HCT patients ranges from mild gastroenteric or respiratory symptoms to pneumonia, hepatitis, severe hemorrhagic enteritis or cystitis, multi-organ failure or death. The current standard of care is off-label use of an antiviral that has demonstrated limited efficacy and significant toxicity.

The Phase 3 registrational trial (NCT05179057) of posoleucel is a randomized, double-blind, placebo-controlled study assessing the efficacy and safety of posoleucel for the treatment of AdV. The study is enrolling 80 pediatric and adult allo-HCT patients with high-level viremia (viral load ≥10,000 copies/mL) or with consistently rising viral load and immune deficiency (viral load ≥1,000 copies/mL and <180 lymphocytes/mm^3 or T cell depletion). The primary efficacy endpoint is the reduction in viral load at Week 4.

About Respiratory Viral Infections and the ALVR106 Phase 1/2 Study

Upper respiratory tract infections due to hMPV, influenza, PIV and RSV are detected in up to 40% of allo-HCT patients. In approximately half of these patients, these viral infections progress to lower respiratory tract infections and present a 20-45% mortality rate. There are no approved treatments or vaccines for hMPV and PIV, and there is no vaccine for RSV; treatments for RSV and influenza have limitations.

The ALVR106 Phase 1/2 proof-of-concept trial (NCT04933968) is a double-blind, placebo-controlled study to assess the safety and dose selection of ALVR106 in addition to standard of care for the treatment of high-risk patients with respiratory viral infections following HCT. The clinical trial consists of two parts. Part A will evaluate multiple ascending doses of ALVR106 in up to 32 patients. Part B will expand the sample size of the selected Part A dose cohort by enrolling an additional 45 high-risk patients. The primary efficacy endpoint is the change in viral load at Week 4.

About Posoleucel

AlloVir's lead product, posoleucel, is in late-stage clinical development as an allogeneic, off-the-shelf, multi-virus specific T-cell therapy targeting six viral pathogens in immunocompromised individuals: adenovirus (AdV), BK virus (BKV), cytomegalovirus (CMV), Epstein-Barr virus (EBV), human herpes virus-6 (HHV-6) and JC virus (JCV). In the positive Phase 2, proof-of-concept CHARMS study, more than 90% of patients who failed conventional treatment and received posoleucel, demonstrated a complete or partial clinical response based on predefined criteria, most with complete elimination of detectable virus in the blood and resolution of major clinical symptoms.

A Phase 3 multicenter, double-blind, placebo-controlled trial of posoleucel for the treatment of virus-associated HC is ongoing. A Phase 2 trial to assess the safety and efficacy of posoleucel for the prevention of infections and disease from the six target viruses is also ongoing. Based on initial data from this Phase 2 multi-virus prevention study, the company plans to initiate a Phase 3 registrational trial for multi-virus prevention in the first half of 2022, following FDA review of the final protocol. A Phase 2 proof-of-concept trial with posoleucel for the preemptive treatment of BKV in adult kidney transplant recipients is also ongoing.

In addition to the RMAT designations for AdV and virus-associated HC treatment, the FDA has also granted posoleucel Orphan Drug Designation for the treatment of virus-associated HC. The European Medicines Agency has granted posoleucel PRIority Medicines (PRIME) designation for the treatment of serious infections with AdV, BKV, CMV, EBV and HHV-6, and Orphan Medicinal Product designation as a potential treatment of viral diseases and infections in patients undergoing HCT.

About ALVR106

ALVR106 is an allogeneic, off-the-shelf, multi-virus specific VST therapy candidate designed to target diseases caused by respiratory syncytial virus (RSV), influenza, parainfluenza virus (PIV) and human metapneumovirus (hMPV). *In vitro* data demonstrate that ALVR106 reactive cells have antiviral activity against each of the target viruses with minimal or no activity against non-virus-infected cells.

About AlloVir

AlloVir is a leading late clinical-stage cell therapy company with a focus on restoring natural immunity against life-threatening viral diseases in pediatric and adult patients with weakened immune systems. The company's innovative and proprietary technology platforms leverage off-the-shelf, allogeneic, single- and multi-virus-specific T cells for patients with T cell deficiencies who are at risk from the life-threatening consequences of viral diseases. AlloVir's technology and manufacturing process enable 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. For more information, visit www.allovir.com or follow us on Twitter or LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding AlloVir's development and regulatory status of our product candidates, the planned conduct of its preclinical studies, and clinical trials and its prospects for success in those studies and trials, and its strategy, business plans and focus. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties, and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, those related to AlloVir's financial results, the timing for the initiation and successful completion of AlloVir's clinical trials of its product candidates, whether and when, if at all, AlloVir's product candidates will receive approval from the U.S. Food and Drug Administration, or FDA, or other foreign regulatory authorities, competition from other biopharmaceutical companies, the impact of the COVID-19 pandemic on AlloVir's product development plans, supply chain, and business operations and other risks identified in AlloVir's SEC filings. AlloVir cautions you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. AlloVir disclaims any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions, or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent AlloVir's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date.

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