FDA Grants Regenerative Medicine Advanced Therapy (RMAT) Designation to AlloVir's Posoleucel for Prevention of Multiple Life-Threatening Infections from Six Viruses in Allogeneic Hematopoietic Cell Transplant Patients

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Posoleucel's third RMAT designation marks an unprecedented regulatory distinction among cell and gene therapies

Global Phase 3 multi-virus prevention trial initiated in March 2022 and is enrolling patients

WALTHAM, Mass.--(BUSINESS WIRE)--Apr. 20, 2022-- AlloVir (Nasdaq: ALVR), a late clinical-stage allogeneic T cell immunotherapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to its lead investigational multi-virus-specific T cell therapy, posoleucel, for the prevention of clinically significant infections and disease from six devastating viruses that commonly impact high-risk adult and pediatric patients following allogeneic hematopoietic cell transplant (allo-HCT) – adenovirus (AdV), BK virus (BKV), cytomegalovirus (CMV), Epstein-Barr virus (EBV), human herpes virus-6 (HHV-6) and JC virus (JCV). This is the third RMAT designation that FDA has granted to posoleucel, in recognition of the therapy's transformative potential to address significant unmet medical needs facing immunocompromised allo-HCT patients.

The FDA previously granted RMAT designation to posoleucel for the treatment of hemorrhagic cystitis (HC) caused by BKV in adults and children following allo-HCT and for the treatment of adenovirus infection following allo-HCT. RMAT designation enables early interactions with the FDA to discuss clinical trial design and other actions to expedite development and review. Outside of the United States, 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.

"The receipt of three RMAT designations for a single therapy is unprecedented. Posoleucel's three RMAT designations reflect the strength of AlloVir's multi-virus platform and its potential both to deliver an important treatment option for immunocompromised patients who currently have none, and to transform the management of allo-HCT patients with a multi-virus prevention approach," said Ercem Atillasoy, M.D., Chief Regulatory and Safety Officer, AlloVir.

Posoleucel has the potential to fundamentally transform the landscape for allo-HCT by preventing life-threatening viral diseases and infections, either as a prophylactic therapy in high-risk patients or as a preemptive therapy in patients who have already reactivated one or more of the six viruses targeted by posoleucel. As 90% of allo-HCT patients reactivate at least one of these viruses, there is a large global market opportunity for the prevention of devastating viral diseases, with an estimated addressable patient population of 40,000 allo-HCT patients annually.

The new RMAT designation was based on initial data from an open-label Phase 2 study evaluating the potential for posoleucel to prevent life-threatening infections from six common viruses following allo-HCT. Initial data from this study were most recently presented at the 48th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT) in March 2022. Out of 26 patients who received at least one dose of posoleucel in the ongoing Phase 2 trial, and including those who completed, discontinued or are continuing posoleucel, only three clinically significant infections were observed through Week 14, as of the data cut-off for this analysis. Of the 24 patients who had reached the Week 14 primary endpoint, 21 remained free of clinically significant infections. Repeat dosing was generally well-tolerated. Final results of the Phase 2 study are expected to be available at the end of this year.

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.

Posoleucel is being studied in three Phase 3 clinical trials for three distinct indications - the treatment of virus-associated HC, the treatment of AdV infection, and the prevention of infections and disease caused by posoleucel's six target viruses. 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 multi-virus prevention and for the treatment of AdV and virus-associated HC, 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 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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