



FDA Grants Orphan Drug Designation (ODD) to AlloVir's Posoleucel, an Allogeneic, Off-the-Shelf, Multi-Virus Specific T-Cell Therapy, for the Treatment of Virus-Associated Hemorrhagic Cystitis

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ODD for posoleucel adds to RMAT designation by FDA and OMP and PRIME designations by EMA

Phase 3 registrational study of posoleucel in virus-associated hemorrhagic cystitis is ongoing

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 4, 2021-- AlloVir, Inc. (Nasdaq: ALVR), a late clinical-stage cell therapy company, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to posoleucel (Viralym-M, ALVR105) for the treatment of virus-associated hemorrhagic cystitis (HC). Posoleucel is an investigational, allogeneic, off-the-shelf, multi-virus specific T cell therapy under development for the treatment and prevention of serious diseases caused by six devastating viral pathogens: BK virus (BKV) and the related polyomavirus JC virus (JCV), cytomegalovirus (CMV), human herpes virus-6 (HHV-6), Epstein Barr virus (EBV) and adenovirus (AdV).

An inflammatory disease of the bladder, virus-associated HC is a serious complication of hematopoietic stem cell transplantation (HSCT) that can significantly prolong hospitalization and increase mortality and for which there are currently no approved or effective antiviral treatment options. The company's Phase 3 study of posoleucel for the treatment of virus-associated HC is ongoing and enrolling adult and pediatric patients following allogeneic HSCT (allo-HSCT).

"This Orphan Drug Designation acknowledges the urgent need for new treatment options for patients who have undergone hematopoietic stem cell transplantation and are at risk for developing viral infections and hemorrhagic cystitis," said Ercem Atillasoy, M.D., Chief Regulatory and Safety Officer, AlloVir. "We look forward to working with the FDA and regulators around the globe as we advance this therapy for patients in need."

In addition to this ODD, posoleucel has been granted Regenerative Medicine Advanced Therapy (RMAT) designation from the FDA, and Orphan Medicinal Product (OMP) and PRiority MEDicines (PRIME) designations from the European Medicines Agency (EMA). Posoleucel is one of the first seven investigational therapies to receive both PRIME and RMAT designations.

ODD is granted by the FDA to drugs or biological products intended for the treatment of diseases or conditions that impact fewer than 200,000 people in the U.S. The designation acts as a stimulus for the development of drugs for rare diseases through several incentives, including research and development tax credits, waiver of filing fees and the potential for a seven-year marketing exclusivity period after FDA approval. Drugs with this designation undergo the same rigorous scientific evaluation as other investigational medicines seeking FDA approval.

About Virus-Associated Hemorrhagic Cystitis (HC)

Virus-associated HC is a well-described complication after HSCT in which a viral infection causes the bladder lining to become inflamed, resulting in hematuria, or blood in the urine. More than half of patients with HC experience clot formation and/or severe bladder hemorrhage with renal impairment. Bleeding may be life-threatening, requiring urologic interventions including cystectomy, or the removal of the urinary bladder.

Clinical manifestations of HC include kidney dysfunction or failure, blood in the urine and severe abdominal pain frequently requiring narcotics. With no approved or effective antiviral treatments, virus-associated HC is primarily managed with supportive care, including forced diuresis, continuous bladder irrigation, platelet transfusion and anti-spasmodics, in addition to urinary and opioid analgesics.

Newly published data¹ demonstrate that patients with virus-associated HC had a 70% higher risk of mortality, and the viral disease was associated with significantly prolonged hospitalization, including increased intensive care unit stay and hospital readmission rates.

HC can be caused by several viruses, including AdV and CMV; however, up to 90% of HC cases are caused by BKV. HC is the primary clinical manifestation associated with BKV following HSCT, occurring in up to 25% of pediatric patients and up to 54% of adult patients.

About Posoleucel (Viralym-M, ALVR105)

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: BKV, CMV, AdV, EBV, HHV-6 and 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.

About AlloVir's Ongoing Clinical Studies with Posoleucel

- A Phase 3 ([NCT04390113](#)), multicenter, double-blind, placebo-controlled study to assess the efficacy and safety of posoleucel for the treatment of patients with virus-associated HC following allo-HSCT is open and recruiting patients.
- A proof-of-concept study ([NCT04693637](#)) targeting the prevention of clinically significant disease caused by BKV, CMV, AdV, EBV, HHV-6 and JCV in patients following allo-HSCT is open and recruiting patients. Approximately 90% of all allo-HSCT patients experience at least one infection associated with BKV, CMV, AdV, EBV or HHV-6, and more than 60% of these patients experience infections caused by two or more of these five viruses within 100 days post allo-HSCT. Data from the open-label phase of the study are anticipated before year-end.

- A Phase 2 proof-of-concept study ([NCT04605484](https://clinicaltrials.gov/ct2/show/study/NCT04605484)) for the preemptive treatment of BK viremia in adult kidney transplant recipients is also ongoing and recruiting patients. BK viremia, which is detected in up to 20% of kidney transplant patients, can lead to decreased kidney survival and a return to end-stage renal disease and dialysis.

For more information on clinical trials of posoleucel, visit www.clinicaltrials.gov.

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](https://twitter.com/allovir) or [LinkedIn](https://www.linkedin.com/company/allovir).

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 the development and regulatory status of our product candidates, the planned conduct of its 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, including but not limited to the risks discussed in AlloVir's Quarterly Report on Form 10-Q for the quarter ended June 30, 2021 and in our other filings with the SEC. 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.

References

1. Economic and Clinical Burden of Virus-Associated Hemorrhagic Cystitis in Patients Following Allogeneic Hematopoietic Stem Cell Transplantation in the United States; McGuirk, Joseph et al.; Transplantation and Cellular Therapy, Official Publication of the American Society for Transplantation and Cellular Therapy, Volume 27, Issue 6, 505.e1 - 505.e9; [https://www.astctjournal.org/article/S2666-6367\(21\)00708-9/fulltext](https://www.astctjournal.org/article/S2666-6367(21)00708-9/fulltext).

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