LAVA Therapeutics Receives FDA Orphan Drug Designation for LAVA-051 for the Treatment of Chronic Lymphocytic Leukemia

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UTRECHT, The Netherlands and PHILADELPHIA, Oct. 15, 2021 (GLOBE NEWSWIRE) -- LAVA Therapeutics N.V. (Nasdaq: LVTX), a clinical-stage biotechnology company, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) for the company's CD1d targeted Gammabody™, LAVA-051, for the treatment of chronic lymphocytic leukemia (CLL). CLL is a form of leukemia characterized by progressive accumulation of abnormal lymphocytes in the peripheral blood, bone marrow and lymphoid tissues.

"We are excited to receive our first orphan drug designation from the FDA for LAVA-051, our most advanced product candidate from our off-the-shelf Gammabody™ platform that is designed to unlock the full anti-cancer potential of this specialized effector cell population," said Stephen Hurly, president and chief executive officer, LAVA Therapeutics. "This designation will be helpful in enhancing our communication with the FDA on our development of LAVA-051. We are grateful to the FDA for highlighting the need for new and improved therapies to address the unmet needs in CLL."

The Gammabody™ LAVA-051 is a bispecific antibody that recruits the immune system to attack CD1d-expressing cancer cells via the preferential activation of both Gamma Delta T cells and type 1 natural killer T (NKT) cells. Enrollment is underway in the company's open-label, multi-center, Phase 1/2a clinical trial for the treatment of relapsed and/or refractory CLL, multiple myeloma (MM) and, later in the trial, acute myeloid leukemia (AML) (NCT04887259). Initiated in July 2021, the trial is designed to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics, immunogenicity and preliminary antitumor activity of LAVA-051. Data from the Phase 1 dose escalation phase of the study are expected in the first half of 2022 with top line clinical data from the Phase 2a expansion cohorts expected in the second half of 2022.

About Orphan Drug Designation

The term orphan drug refers to pharmaceutical products developed for the prevention, diagnosis and treatment of rare diseases or conditions. In the U.S., an orphan disease is defined as a disease or condition with a U.S. prevalence of less than 200,000 people. Supporting the development and evaluation of new treatments for rare diseases is a key priority for the FDA. Since the Orphan Drug Act was passed in 1983, the U.S. government has provided incentives and policy support to encourage development of orphan drugs to meet medical unmet needs. This orphan drug designation from the FDA qualifies LAVA for various incentives related to the development of LAVA-051, including tax credits for qualified clinical trials, exemption from user fees and the potential for seven years of U.S. market exclusivity for the treatment of CLL.

About LAVA-051

LAVA-051 is a Gammabody™ designed to activate both Vγ9Vδ2 T cells and type 1 NKT cells to kill CD1d expressing tumor cells. LAVA-051 consists of two single domain antibodies linked via a short five amino acid glycine-serine linker. One domain recognizes the Vδ2 chain of the Vγ9Vδ2 T cell receptor, and the other domain is specific for CD1d, a glycoprotein involved in the presentation of (glyco)lipid antigens to distinct T cell populations including type 1 NKT cells, and that can be expressed on a wide range of hematological malignancies, including CLL, MM and AML.

An open-label, Phase 1/2a study of LAVA-051 as monotherapy in patients with relapsed or refractory CD1d-positive CLL, MM or AML is currently ongoing (NCT04887259). The Phase 1 dose-escalation portion of the study will determine an optimal Phase 2 dose of LAVA-051. Once a recommended Phase 2 dose has been established, the trial will expand into the Phase 2a portion, which will enroll patients in three disease specific cohorts for relapsed and/or refractory CLL, MM and AML, to confirm safety and evaluate preliminary antitumor activity in each disease cohort. The trial is initially being conducted in Europe and is planned to later expand to the U.S. The company expects to report data from the Phase 1 dose escalation phase of the study in the first half of 2022 with top line clinical data from the Phase 2a expansion cohorts expected in the second half of 2022.

About LAVA Therapeutics

LAVA Therapeutics N.V. is a clinical-stage biotechnology company utilizing its proprietary Gammabody™ platform to develop a portfolio of bispecific gamma delta T cell engagers (gamma delta bsTCEs) for the treatment of solid tumors and hematological malignancies. The company's innovative approach utilizes bispecific antibodies engineered to selectively kill cancer cells via the triggering of Vγ9Vδ2 T cell antitumor effector functions upon cross-linking to tumor associated antigens. A Phase 1/2a clinical study evaluating LAVA-051 in patients with certain hematological malignancies is enrolling patients (NCT04887259). The company plans to initiate a Phase 1/2a clinical study to evaluate LAVA-1207 in patients with prostate cancer in the second half of 2021. For more information, please visit www.lavatherapeutics.com and follow us on LinkedIn and Twitter.

LAVA's Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements, including in respect of the company's anticipated growth and clinical developments plans, including the timing of clinical trials. Words such as "anticipate," "believe," "could," "will," "may," "expect," "should," "plan," "intend," "estimate," "potential" and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements. These forward-looking statements are based on LAVA's expectations and assumptions as of the date of this press release and are subject to various risks and uncertainties that may cause actual results to differ materially from these forward-looking statements. Forward-looking statements contained in this press release include, but are not limited to, statements about the preclinical data, clinical development and scope of clinical trials, and the potential use of our product candidates to treat various tumor targets. Many factors, risks and uncertainties may cause differences between current expectations and actual results including, among other things, the timing and results of our research and development programs and preclinical and clinical trials, our ability to obtain regulatory approval for and commercialize our product candidates, our ability to leverage our initial programs to develop additional product candidates using our Gammabody™ platform, and the, failure of LAVA's collaborators to support or advance collaborations or our product candidates. In addition, the COVID-19 pandemic may disrupt our business and that of the third parties on which we depend, including delaying or otherwise disrupting our clinical trials and preclinical studies, manufacturing and supply chain, or impairing employee productivity. LAVA assumes no obligation to update any forward-looking statements contained herein to reflect any change in expectations, even as new information becomes available.

CONTACT
Edward Smith
Chief Financial Officer
ir@lavatherapeutics.com

Catherine Day
+1-917-763-2709
Catherine@newdaybioconsulting.com