Verve Therapeutics Receives U.S. FDA Fast Track Designation for VERVE-102, an In Vivo Base Editing Medicine Targeting PCSK9

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BOSTON, April 11, 2025 (GLOBE NEWSWIRE) -- Verve Therapeutics, a clinical-stage company developing a new class of genetic medicines for cardiovascular disease, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for VERVE-102 for the treatment of patient groups with hyperlipidemia and high lifetime cardiovascular risk to reduce low-density lipoprotein cholesterol (LDL-C). VERVE-102 is the company's novel, *in vivo*, investigational base editing medicine designed to be a single-course treatment that permanently turns off the *PCSK9* gene in the liver and durably reduces disease-driving LDL-C. VERVE-102 is currently being tested in the Phase 1b Heart-2 clinical trial, which is designed to evaluate the safety and tolerability of VERVE-102 administration in adult patients with heterozygous familial hypercholesterolemia (HeFH) and/or premature coronary artery disease (CAD) who require additional lowering of LDL-C.

"Despite available treatment options to lower LDL-C, there remains a pressing need to provide sustained LDL-C lowering and thereby, improve efficacy," said Sekar Kathiresan, M.D., co-founder and chief executive officer of Verve Therapeutics. "Nearly 50% of patients discontinue prescribed LDL-C-lowering therapy within a year. Verve has long understood that profound, sustained LDL-C lowering is key to transforming the treatment of atherosclerotic cardiovascular disease. As a potential one-dose treatment, we believe VERVE-102 is uniquely designed to address this unmet need. We look forward to collaborating with the FDA as we work to deliver this paradigm-shifting treatment to patients."

The FDA's Fast Track designation is designed to facilitate the development and expedite the review of drugs that are intended to treat serious or life-threatening conditions and demonstrate the potential to address an unmet medical need. A drug that receives Fast Track designation may be eligible for more frequent meetings and communications with the FDA and rolling review of any application for marketing approval. A drug receiving Fast Track designation also may be eligible for Priority Review if relevant criteria are met.

In the second quarter of 2025, Verve expects to announce demographic and initial safety and efficacy data from the Heart-2 clinical trial. The initial data set is expected to include participants across the first three dose cohorts (0.3 mg/kg, 0.45 mg/kg, and 0.6 mg/kg) with at least 28 days of follow-up for each participant.

In addition, in the second half of 2025, Verve remains on track to report the final data for the dose escalation portion of the Heart-2 clinical trial, deliver the opt-in package for the PCSK9 program to Eli Lilly and Company (Lilly), and initiate the Phase 2 clinical trial for the PCSK9 program.

About Verve Therapeutics
Verve Therapeutics, Inc. (Nasdaq: VERV) is a clinical-stage company developing a new class of genetic medicines for cardiovascular disease with the potential to transform treatment from chronic therapies to single-course gene editing medicines. The company's lead programs – VERVE-102, VERVE-201, and VERVE-301 – target the three cholesterol drivers of atherosclerosis: LDL-C, remnant cholesterol, and Lp(a). VERVE-102 is designed to permanently turn off the *PCSK9* gene in the liver and is being developed initially for heterozygous familial hypercholesterolemia (HeFH) and ultimately to treat patients with established atherosclerotic cardiovascular disease (ASCVD) who continue to be impacted by high LDL-C levels. VERVE-201 is designed to permanently turn off the *ANGPTL3* gene in the liver and is initially being developed for refractory hypercholesterolemia, where patients still have high LDL-C despite treatment with maximally tolerated standard of care therapies, and homozygous familial hypercholesterolemia (HoFH). VERVE-301 is designed to permanently turn off the *LPA* gene to reduce Lp(a) levels. Lp(a) is a genetically validated, independent risk factor for ASCVD, ischemic stroke, thrombosis, and aortic stenosis. For more information, please visit www.VerveTx.com.

Cautionary Note Regarding Forward Looking Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 that involve substantial risks and uncertainties, including statements regarding the company's ongoing Heart-2 clinical trial; the timing and availability of data for the Heart-2 trial and timing for initiation of the Phase 2 clinical trial for the PCSK9 program; the timing of delivery of the opt-in package to Lilly; the potential advantages and therapeutic potential of the company's programs; and expectations regarding the potential benefits of Fast Track designation. All statements, other than statements of historical facts, contained in this press release, including statements regarding the company's strategy, future operations, future financial position, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the company's limited operating history; the company's ability to timely submit and receive approvals of regulatory applications for its product candidates; advance its product candidates in clinical trials; initiate, enroll and complete its ongoing and future clinical trials on the timeline expected or at all; correctly estimate the potential patient population and/or market for the company's product candidates; replicate in clinical trials positive results found in preclinical studies and/or earlier-stage clinical trials of VERVE-101, VERVE-102, and VERVE-201; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; obtain, maintain or protect intellectual property rights related to its product candidates; manage expenses; and raise the substantial additional capital needed to achieve its business objectives. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the company's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in the company's most recent filings with the Securities and Exchange Commission and in other filings that the company makes with the Securities and Exchange Commission in the future. In addition, the forward-looking statements included in this press release represent the company's views as of the date hereof and should not be relied upon as representing the company's views as of any date subsequent to the date hereof. The company anticipates that subsequent events and developments will cause the company's views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so.

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