Verve Therapeutics Announces VERVE-101 Awarded Innovation Passport by the UK MHRA for the Treatment of Heterozygous Familial Hypercholesterolemia

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BOSTON, Feb. 14, 2023 (GLOBE NEWSWIRE) -- Verve Therapeutics, Inc., a clinical-stage biotechnology company pioneering a new approach to the care of cardiovascular disease with single-course gene editing medicines, today announced that VERVE-101 has been awarded the Innovation Passport for the treatment of heterozygous familial hypercholesterolemia (HeFH) under the Innovative Licensing and Access Pathway (ILAP) by the Medicines and Healthcare products Regulatory Agency (MHRA), the regulatory body of the United Kingdom (UK). The ILAP aims to accelerate time to market and facilitate patient access to innovative medicines. VERVE-101 is a novel, investigational gene editing medicine designed to be a single-course treatment that permanently turns off the PCSK9 gene in the liver to durably lower disease-driving low-density lipoprotein cholesterol (LDL-C). Innovation Passport is the first step in the ILAP process and is awarded for a medicinal product addressing a condition that is life-threatening or seriously debilitating and where there is a significant patient or public health need.

“People suffering with HeFH face the life-threatening consequences of lifelong very high levels of LDL-C and only about 3% of HeFH patients worldwide are actually at treatment goal. This is often due to the heavy treatment burden of the chronic care model requiring daily statins and/or intermittent injections, often over decades,” said Sekar Kathiresan, M.D., co-founder and chief executive officer of Verve. “There is a critical need for a new treatment approach such as a once-and-done medicine for these patients, and we are pleased that VERVE-101 has received the Innovation Passport from MHRA, which could help to accelerate its clinical development. Our heart-1 clinical trial is ongoing in both New Zealand and the UK, and we are committed to our continued partnership with regulatory authorities so that we may bring VERVE-101 to patients as efficiently as possible.”

Heart-1 is designed to evaluate the safety and tolerability of VERVE-101 administration in patients with HeFH, with additional analyses for pharmacokinetics and reductions in blood PCSK9 protein and LDL-C. Verve expects to report initial clinical data from the dose escalation portion of the heart-1 clinical trial including safety parameters, blood PCSK9 level, and blood LDL-C level in the second half of 2023. For more information, please visit clinicaltrials.gov.

ILAP was launched by the MHRA in January 2021 with an aim to accelerate the development of and facilitate patient access to medicines. It comprises the Innovation Passport designation as well as a Target Development Profile (TDP) and provides applicants with access to a toolkit to support all stages of the design, development and approvals process. The Innovation Passport is granted by the UK's ILAP Steering Group, which consists of representatives from MHRA, the National Institute for Health and Care Excellence (NICE), the Scottish Medicines Consortium (SMC), and the All Wales Therapeutics and Toxicology Centre (AWTTC).

About VERVE-101
VERVE-101 is a novel, investigational gene editing medicine designed to be a single-course treatment that permanently turns off the PCSK9 gene in the liver to reduce disease-driving low-density lipoprotein cholesterol (LDL-C). VERVE-101 is being developed initially as a treatment for patients with heterozygous familial hypercholesterolemia (HeFH), a prevalent and potentially life-threatening genetic subtype of atherosclerotic cardiovascular disease (ASCVD). VERVE-101 consists of an adenine base editor messenger RNA (licensed from Beam Therapeutics Inc.) and an optimized guide RNA targeting the PCSK9 gene packaged in an engineered lipid nanoparticle. By making a single A-to-G change in the DNA genetic sequence of PCSK9, VERVE-101 aims to inactivate the target gene. Inactivation of the PCSK9 gene has been shown to up-regulate LDL receptor expression, which leads to lower LDL-C levels, thereby reducing the risk for ASCVD.

About Verve Therapeutics
Verve Therapeutics, Inc. (Nasdaq: VERV) is a clinical-stage genetic medicines company pioneering a new approach to the care of cardiovascular disease, potentially transforming treatment from chronic management to single-course gene editing medicines. The company’s initial two programs – VERVE-101 and VERVE-201 – target genes that have been extensively validated as targets for lowering low-density lipoprotein cholesterol (LDL-C), a root cause of cardiovascular disease, in order to durably reduce blood LDL-C levels. VERVE-101 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 atherosclerotic cardiovascular disease (ASCVD) patients not at goal on oral therapy. VERVE-201 is designed to permanently turn off the *ANGPTL3* gene in the liver and is initially being developed in homozygous familial hypercholesterolemia (HoFH) and ultimately to treat patients with refractory hypercholesterolemia. For more information, please visit www.VerveTx.com

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 timing and availability of clinical trial data from its ongoing heart-1 clinical trial, its research and development plans, the potential advantages and therapeutic potential of the company’s programs, including VERVE-101 and VERVE-201,. 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 timing of and the company’s ability to submit 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 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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