Verve Therapeutics to Present Preclinical Data from Gene Editing Programs for the Treatment of Atherosclerotic Cardiovascular Disease at ASGCT

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CAMBRIDGE, Mass. — May 11, 2021— Verve Therapeutics, a biotech company pioneering a new approach to the care of cardiovascular disease with single-course gene editing medicines, today announced the presentation of additional preclinical data from the company's gene editing programs for the treatment of atherosclerotic cardiovascular disease (ASCVD), the most common form of cardiovascular disease, at the American Society of Gene and Cell Therapy (ASGCT) 24th Virtual Annual Meeting. The data will be presented today at 10 a.m. ET in an oral session, entitled "*In Vivo* CRISPR Base Editing of PCSK9 in Primates and Durable Cholesterol Reduction," by Andrew Bellinger, M.D., Ph.D., chief scientific officer of Verve.

Verve is advancing a pipeline of single-course *in vivo* gene editing programs, each designed to mimic the benefit of natural disease resistance mutations and turn off specific genes in order to lower blood lipids, thereby reducing the risk of ASCVD. These programs are focused on two genes, PCSK9 and ANGPTL3, which are well-validated targets involved in the control of blood lipids, such as low-density lipoprotein cholesterol (LDL-C).

"We're pleased to present these preclinical data at ASGCT highlighting the progress made across our lead programs targeting PCSK9 and ANGPTL3. The findings support our approach of utilizing optimized base editors delivered to the liver via lipid nanoparticles, which are designed to potently and durably edit root causal genes for the leading cause of death in the world," said Dr. Bellinger. "We look forward to advancing these programs to help change how ASCVD is treated."

Verve's pipeline is led by VERVE-101, which is designed to permanently turn off the PCSK9 gene in the liver and is being developed initially for the treatment of heterozygous familial hypercholesterolemia (HeFH), a genetic disorder caused by life-long severely elevated blood cholesterol that can lead to increased risk of early-onset ASCVD.

Preclinical data reported today show that in a disease model of HeFH, the well-established LDLR knockout mouse model, the company's mouse surrogate version of VERVE-101 produced similar and robust amounts of PCSK9 editing in the liver, above 50%, at doses of 0.05, 0.1 and 0.5 mg/kg administered as a single dose to wild-type and heterozygous LDLR knockout mice.

In addition, data from Verve's ongoing preclinical study in non-human primates (NHPs) showed potent and durable lowering of both blood PCSK9 protein and LDL-C levels of approximately 90% and 60%, respectively, following administration of a single PCSK9-targeted gene editor. These reductions were maintained out to 10 months after the single treatment administration at the most recent analysis.

The company also reported data from its second gene editing program, which is designed to target the ANGPTL3 gene, a regulator of cholesterol and triglyceride metabolism. In a preclinical proof-of-concept study with an ANGPTL3-gene editor administered as a single dose to NHPs, Verve observed a 95% average reduction of blood ANGPTL3 protein levels. These reductions were maintained, and at 10 months after the single treatment administration, the NHPs exhibited an average reduction of 96% in blood ANGPTL3 protein.

About Verve Therapeutics

Verve Therapeutics is a genetic medicines company pioneering a new approach to the care of cardiovascular disease, transforming treatment from chronic management to single-course gene editing medicines. The company's initial two programs target PCSK9 and ANGPTL3, genes that have been extensively validated as targets for lowering blood lipids such as low-density lipoprotein cholesterol (LDL-C), a root cause of cardiovascular disease. Verve's lead product candidate, VERVE-101, is designed to turn off the PCSK9 gene in the liver in order to disrupt blood PCSK9 protein production and thereby reduce blood LDL-C levels, with the goal of reducing a patient's risk for cardiovascular disease. VERVE-101, currently in IND-enabling studies, is being developed initially for the treatment of patients with heterozygous familial hypercholesterolemia, a potentially fatal genetic heart disease. For more information, please visit www.VerveTx.com.

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