Verve Therapeutics Announces Pipeline Progress and Reports Third Quarter 2021 Financial Results

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VERVE-101 Preclinical Data Announced at TIDES 2021 Demonstrated Potent, Durable PCSK9 Protein and LDL-C Reductions, Supporting Planned Clinical Initiation in 2022

New Preclinical Data from ANGPTL3 Program Highlight Potent Editing with Proprietary GalNAc-LNP Delivery in a Novel NHP Model of Homozygous Familial Hypercholesterolemia

Ended Third Quarter of 2021 with $389.2 million in Capital

CAMBRIDGE, Mass., Nov. 10, 2021 (GLOBE NEWSWIRE) -- Verve Therapeutics, Inc., (Nasdaq: VERV), a biotech company pioneering a new approach to the care of cardiovascular disease with single-course gene editing medicines, today reported pipeline and business highlights and third quarter 2021 financial results.

"We're thrilled with the progress we've made thus far in 2021, exemplified by recent preclinical data readouts from our lead programs targeting PCSK9 and ANGPTL3," said Sekar Kathiresan, M.D., co-founder and chief executive officer of Verve. "Data from our VERVE-101 program targeting PCSK9 demonstrate its potential to safely and durably lower disease-causing high LDL-C, a key driver of atherosclerotic cardiovascular disease, and support our plans to initiate clinical development in 2022. Preclinical data from our ANGPTL3 program, including exciting data highlighting our proprietary GalNAc-LNP delivery technology, could allow us to reach even more patients with our single-course gene editing medicines. Backed by an expert team and a strong balance sheet, we stand well positioned to advance our gene editing therapies and fundamentally change the way cardiovascular disease is treated."

Pipeline Highlights

- **VERVE-101 Clinical Initiation on Track for 2022.** Data presented during an oral session at the TIDES USA Oligonucleotide & Peptide Therapeutics Conference (TIDES 2021) demonstrated that administration of VERVE-101, Verve's lead gene editing therapy targeting PCSK9, led to potent and durable lowering of both blood PCSK9 protein and disease-causing low-density lipoprotein cholesterol (LDL-C), with no evidence of adverse events or significant off-target, bystander or germline editing, in non-human primates (NHPs). Based on these data, Verve plans to pursue regulatory submissions and begin clinical development of VERVE-101 in 2022, initially focused on patients with heterozygous familial hypercholesterolemia (HeFH), a prevalent form of atherosclerotic cardiovascular disease (ASCVD).
- **Preclinical Data Highlight Liver-Targeted Delivery with Proprietary GalNAc-LNP Technology.** Verve recently announced preclinical data highlighting the development of a potent, *in vivo* liver-targeting lipid nanoparticle (LNP) delivery technology using a novel GalNAc-targeting ligand. Findings show that Verve's ANGPTL3-targeted base editor, delivered via its GalNAc-LNP, was taken up in the livers of NHPs, reduced blood ANGPTL3 protein by 94-97% and lowered LDL-C in an internally developed NHP model of homozygous familial hypercholesterolemia (HoFH), a rare form of ASCVD. These findings build upon *in vivo* data presented at TIDES 2021 showing that the company's proprietary GalNAc-LNP achieved high efficiency liver delivery and base editing in mouse models of HoFH. Verve is advancing development of its GalNAc-LNP technology and plans to select the development candidate for its ANGPTL3 program in 2022.

Upcoming Investor Conference Presentation

- **Jefferies 2021 Virtual London Healthcare Conference.** Dr. Kathiresan and Andrew Bellinger, M.D., Ph.D., chief scientific officer and chief medical officer, will participate in a fireside chat during the Jefferies 2021 Virtual London Healthcare Conference, being held November 16-19, 2021. The fireside chat will be available to registered participants on-demand between 8:00 a.m. GMT on November 18, 2021, and 5:00 p.m. GMT on November 19, 2021. The webcast will be available in the investor section of the company's website at www.vervetx.com, and will be archived for 60 days following the presentation.

Third Quarter 2021 Financial Results

- **Cash Position**: Cash, cash equivalents and marketable securities were $389.2 million as of September 30, 2021, as compared to $72.1 million as of December 31, 2020.
- **Research & Development (R&D) Expenses**: R&D expenses were $17.5 million for the third quarter of 2021, as compared to $7.6 million for the third quarter of 2020. This increase was primarily due to additional investment in preclinical studies and manufacturing activities to support future clinical trials, and higher personnel costs (including noncash stock-based compensation).
- **General & Administrative (G&A) Expenses**: G&A expenses were $6.0 million for the third quarter of 2021, as compared to $1.4 million for the third quarter of 2020. This increase was primarily due to higher personnel costs (including non-cash stock-based compensation), legal and other professional fees, and facilities costs.
- **Net Loss**: Net loss attributable to common stockholders was $22.7 million, or $0.47 per share, for the third quarter of 2021, as compared to $9.0 million, or $3.81 per share, for the third quarter of 2020.

About Verve Therapeutics

Verve Therapeutics, Inc. (Nasdaq: VERV) 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 permanently turn off the PCSK9 gene in the liver in order to disrupt blood PCSK9 protein production and thereby durably 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.

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 initiation, and timing, of the Company's planned regulatory submissions, including an Investigational New Drug application, and future clinical trials, its research and development plans and the potential advantages and therapeutic potential of the Company's programs. 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 and enroll 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 its other product candidates; 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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Verve Therapeutics, Inc.
Selected Condensed Financial Information
(in thousands, except share and per share amounts)
(unaudited)

Condensed consolidated statements of operations	**Three months ended September 30,**		**Nine months ended September 30,**	
	2021	**2020**	**2021**	**2020**
Operating expenses:				
Research and development	$ 17,495	$ 7,618	$ 42,263	$ 19,795
General and administrative	6,007	1,354	12,264	3,232
Total operating expenses	23,502	8,972	54,527	23,027
Loss from operations	(23,502)	(8,972)	(54,527)	(23,027)
Other income (expense):				
Change in fair value of preferred stock tranche liability	-	-	-	2,507
Change in fair value of antidilution rights liability	-	(59)	(25,574)	(1,804)
Change in fair value of success payment liability	700	4	(8,954)	(13)
Interest income and other income (expense), net	53	37	78	164
Total other income (expense), net	753	(18)	(34,450)	854
Net loss	$ (22,749)	$ (8,990)	$ (88,977)	$ (22,173)
Net loss per common share, attributable to common stockholders, basic and diluted	$ (0.47)	$ (3.81)	$ (4.52)	$ (10.23)
Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	47,992,773	2,358,924	19,698,450	2,167,842

Condensed consolidated balance sheets	**September 30, 2021**	**December 31, 2020**

Assets		
Current assets:		
Cash and cash equivalents	$ 148,134	$ 8,993
Marketable securities	241,076	63,119
Prepaid expenses and other current assets	5,227	1,854
Total current assets	394,437	73,966
Property and equipment, net	6,574	3,984
Restricted cash	5,237	463
Operating lease right-of-use assets	2,335	-
Other long term assets	2,294	-
Total assets	$ 410,877	$ 78,413
Liabilities, convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable and accrued liabilities	$ 11,706	$ 7,225
Success payment liability, current portion	6,250	-
Operating lease obligations, current portion	2,295	-
Deferred rent, current portion	-	90
Total current liabilities	20,251	7,315
Operating lease obligations, net of current portion	181	-
Deferred rent, net of current portion	-	125
Success payment liability, net of current portion	5,510	2,806
Antidilution rights liability	-	6,916
Total liabilitites	25,942	17,162
Convertible preferred stock	-	125,160
Stockholders' equity (deficit)	384,935	(63,909)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 410,877	$ 78,413

Source: Verve Therapeutics