



## Verve Therapeutics Announces Pipeline Progress and Reports Fourth Quarter and Full Year 2021 Financial Results

March 14, 2022

*Significant Progress Anticipated in 2022 with Initiation of Clinical Trial for VERVE-101 and IND-enabling Studies for ANGPTL3 Program*

*Presentations at Upcoming Scientific Conferences to Highlight Novel Base Editing Approach and Proprietary Lipid Nanoparticle Delivery System*

*Cash, Cash Equivalents and Marketable Securities of \$360.4 Million with Cash Runway into 2024*

CAMBRIDGE, Mass., March 14, 2022 (GLOBE NEWSWIRE) -- [Verve Therapeutics](#), Inc., (Nasdaq: VERV), a biotechnology company pioneering a new approach to the care of cardiovascular disease with single-course gene editing medicines, today reported pipeline highlights and fourth quarter and full year 2021 financial results. Verve's programs are designed to mimic natural disease resistance mutations and turn off specific genes, such as PCSK9 and ANGPTL3, in order to lower blood lipids and treat atherosclerotic cardiovascular disease (ASCVD).

"The relationship between the lowering of cumulative LDL-C exposure and reduction in the risk of ASCVD is among the best understood relationships in medicine," said Sekar Kathiresan, M.D., co-founder and chief executive officer of Verve. "Today's chronic care model for lowering LDL-C is sub-optimal as it requires continuous, life-long treatment, and cumulative exposure to LDL-C for many patients with ASCVD often remains insufficiently controlled. Our lead program, VERVE-101, has demonstrated the ability to meaningfully and durably lower LDL-C with a well-tolerated safety profile in numerous non-human primate (NHP) studies after one-time dosing. Among many activities and initiatives planned in 2022, we are preparing for our most meaningful milestone yet – treating patients in our first clinical trial of VERVE-101 later this year. We are excited to report progress and execution against our goals."

### Pipeline Highlights

- **VERVE-101 Clinical Initiation on Track for the Second Half of 2022:** Verve is advancing its lead program, VERVE-101, initially for the treatment of patients with heterozygous familial hypercholesterolemia (HeFH). VERVE-101 is designed to permanently turn off the PCSK9 gene in the liver to reduce disease-driving LDL-C. The company has initiated IND-enabling studies and expects to complete its submissions of clinical trial applications (CTAs) to certain foreign regulatory agencies and an investigational new drug (IND) application to the U.S. Food and Drug Administration (FDA) in the second half of 2022, followed by initiation of clinical development for patients with HeFH.
- **ANGPTL3 Program IND-Enabling Studies Anticipated to Begin in the Second Half of 2022:** Verve's second program is designed to permanently turn off the ANGPTL3 gene, a key regulator of cholesterol and triglyceride metabolism. Verve plans to develop this program initially for the treatment of patients with homozygous familial hypercholesterolemia (HoFH) as well as ASCVD patients who require additional LDL-C lowering. The company continues to advance this program toward the selection of a development candidate and plans to initiate IND-enabling studies in the second half of 2022.
- **Preclinical Data in Non-Human Primates Demonstrate Potential to Re-Dose or Sequentially Dose Base Editing Programs:** Given the complexities of ASCVD indications, the company believes some patients may benefit from additional lipid lowering after treatment with any single agent. In January 2022, Verve [announced data from multiple preclinical assessments](#) in NHPs to explore the potential to re-dose or sequentially dose its gene editing treatments. These data suggest that repeat low doses of a PCSK9 base editor could achieve a high level of liver editing, and that sequential dosing of a PCSK9 base editor followed by an ANGPTL3 base editor may be able to efficiently edit two genes that control key lipid pathways.
- **Development of Proprietary GalNAc-LNP Delivery System:** Verve has developed [GalNAc-LNP technology](#) and plans to use this technology to deliver a base editor targeting the ANGPTL3 gene to the liver. Delivery of the ANGPTL3 base editor using Verve's GalNAc-LNP delivery system led to potent editing of the ANGPTL3 gene in NHPs with LDL receptor-deficient livers, with over a 90% reduction in blood ANGPTL3 protein levels. Verve plans to develop its GalNAc-LNP delivery system as a leading technology for *in vivo* liver delivery of gene editors.
- **Expansion of pipeline to include additional *in vivo* liver gene editing treatments:** Verve plans to expand beyond its PCSK9 and ANGPTL3 programs to develop a suite of single-course gene editing medicines that address root causes of disease.

## Upcoming Medical Meeting Presentations

Verve today announced that the company will be presenting at multiple upcoming scientific conferences. Details of the presentations are as follows:

- **American College of Cardiology's 71st Annual Scientific Session & Expo (ACC 2022):** Verve is scheduled to present in an oral session at ACC 2022 being held April 2 – April 4, 2022 in Washington, D.C and virtually.

**Title:** Sequential *In Vivo* Crispr Base Editing of the PCSK9 and ANGPTL3 Genes in Non-Human Primates

**Track:** Highlighted Original Research: Ischemic Heart Disease and the Year in Review

**Session:** 913

**Date/Time:** Monday, April 4, 2022, 9:06 a.m. – 9:16 a.m. ET

- **TIDES 2022:** Verve is scheduled to present in an oral session at TIDES 2022 being held May 9 – 12, 2022 in Boston, MA and virtually.

**Title:** *In Vivo* CRISPR Base Editing to Treat ASCVD

**Track:** Genome Editing and mRNA

**Date/Time:** Wednesday, May 11, 2022, 8:30 a.m. – 9:00 a.m. ET

- **American Society of Gene and Cell Therapy (ASGCT) 25<sup>th</sup> Annual Meeting:** Verve is scheduled to present in an education session at the ASGCT 25<sup>th</sup> Annual Meeting being held May 16 – 18, 2022 in Washington, D.C. and virtually.

**Session Title:** *In Vivo* CRISPR Base Editing of PCSK9 Durably Lowers Cholesterol in Primates

**Track:** CRISPR/CAS9 Gene Editing - Concepts to In-Vivo Editing

**Date/Time:** Tuesday, May 17, 2022, 8:00 a.m. – 9:45 a.m. ET

## Fourth Quarter and Full Year 2021 Financial Results

- **Cash Position:** Cash, cash equivalents and marketable securities were \$360.4 million as of December 31, 2021, compared with \$72.1 million as of December 31, 2020. Based on current operating plans, Verve expects its existing cash, cash equivalents and marketable securities will enable the company to fund its operating expenses and capital expenditure requirements into 2024.
- **R&D Expenses:** Research and development (R&D) expenses were \$25.9 million for the quarter ended December 31, 2021, and \$68.2 million for the year ended December 31, 2021, compared to \$15.6 million for the quarter ended December 31, 2020, and \$35.4 million for the year ended December 31, 2020.
- **G&A Expenses:** General and administrative (G&A) expenses were \$6.6 million for the quarter ended December 31, 2021, and \$18.9 million for the year ended December 31, 2021, compared to \$2.0 million for the quarter ended December 31, 2020, and \$5.3 million for the year ended December 31, 2020.
- **Net Loss:** Net loss was \$31.3 million, or \$0.65 basic and diluted net loss per share, for the quarter ended December 31, 2021, and \$120.3 million, or \$4.48 basic and diluted net loss per share, for the year ended December 31, 2021, compared to a net loss of \$23.5 million, or \$9.43 basic and diluted net loss per share, for the quarter ended December 31, 2020 and \$45.7 million, or \$20.31 basic and diluted net loss per share, for the year ended December 31, 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](http://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 regulatory submissions, future clinical trials, its research and development plans, the potential advantages and therapeutic potential of the Company's programs, and the period over which the Company believes that its existing cash, cash equivalents and marketable securities will be sufficient to fund its operating expenses. 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 December 31,		Year ended December 31,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	\$ 25,939	\$ 15,576	\$ 68,202	\$ 35,371
General and administrative	6,601	2,024	18,865	5,256
Total operating expenses	32,540	17,600	87,067	40,627
Loss from operations	(32,540)	(17,600)	(87,067)	(40,627)
Other income (expense):				
Change in fair value of preferred stock tranche liability	-	-	-	2,507
Change in fair value of antidilution rights liability	-	(3,555)	(25,574)	(5,359)
Change in fair value of success payment liability	1,139	(2,374)	(7,815)	(2,387)
Interest income and other income (expense), net	64	(2)	142	162
Total other income (expense), net	1,203	(5,931)	(33,247)	(5,077)
Net loss	\$ (31,337)	\$ (23,531)	\$ (120,314)	\$ (45,704)
Net loss per common share, attributable to common stockholders, basic and diluted	\$ (0.65)	\$ (9.43)	\$ (4.48)	\$ (20.31)
Weighted-average common shares used in net loss per share attributable to common stockholders, basic and diluted	48,026,078	2,495,056	26,872,036	2,250,093

#### Condensed consolidated balance sheets

##### Assets

##### Current assets:

	December 31, 2021	December 31, 2020
Cash and cash equivalents	\$ 64,330	\$ 8,993
Marketable securities	296,112	63,119
Prepaid expenses and other current assets	6,686	1,854
Total current assets	367,128	73,966
Property and equipment, net	7,224	3,984
Restricted cash	5,237	463
Operating lease right-of-use assets	1,839	-

Other long term assets	2,696	-
Total assets	<u>\$ 384,124</u>	<u>\$ 78,413</u>
<b>Liabilities, convertible preferred stock and stockholders' equity (deficit)</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 20,069	\$ 7,225
Operating lease obligations, current portion	1,955	-
Deferred rent, current portion	-	90
Total current liabilities	<u>22,024</u>	<u>7,315</u>
Deferred rent, net of current portion	-	125
Success payment liability, net of current portion	4,371	2,806
Antidilution rights liability	-	6,916
Other long term liabilities	<u>377</u>	<u>-</u>
Total liabilities	<u>26,772</u>	<u>17,162</u>
Convertible preferred stock	-	125,160
Stockholders' equity (deficit)	<u>357,352</u>	<u>(63,909)</u>
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 384,124</u>	<u>\$ 78,413</u>