Verve Therapeutics Highlights Recent Company Progress and Reports Second Quarter 2023 Financial Results

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heart-1 Initial Clinical Data Expected in the Fourth Quarter of 2023

Preclinical Studies and Clinical Operations Activities Underway to Support Initiation of Clinical Trials for VERVE-102 and VERVE-201 in 2024

Lp(a) Program Advancing in Collaboration with Lilly; Received $60 Million in Combined Upfront Payment and Equity Investment in August 2023

Well-capitalized with Cash Runway into 2026

BOSTON, Aug. 10, 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 reported recent company progress and financial results for the second quarter of 2023.

“We are dedicated to bringing life-changing, once-and-done medicines to patients with cardiovascular disease,” said Sekar Kathiresan, M.D., co-founder and chief executive officer of Verve. “Our primary focus is on developing medicines targeting three pillars of cardiovascular risk: low-density lipoproteins (LDL), triglyceride-rich lipoproteins (TRL) and lipoprotein(a) (Lp(a)). We are making meaningful progress across the portfolio, and we expect to report initial clinical data from the heart-1 Phase 1b clinical trial of VERVE-101, our first-in-class base editor targeting *PCSK9*, in the fourth quarter of this year. In addition, we are focused on completing the preclinical activities and executing the clinical operations activities necessary to start clinical trials for our second PCSK9 program, VERVE-102, and our ANGPTL3 program, VERVE-201, next year. Finally, we are thrilled to advance our Lp(a) program in collaboration with Lilly, an industry leader in cardiometabolic disease. As Verve continues to evolve with multiple assets in the clinic and a robust pipeline, our efforts are supported by a strong financial position with a cash runway extending into 2026.”

VERVE-101 Progressing in heart-1 Clinical Trial

- VERVE-101, an *in vivo* base editing medicine delivered as a one-time intravenous infusion, is designed to inactivate the *PCSK9* gene in liver cells, turning off liver production of blood PCSK9 and thereby durably reducing LDL-C. VERVE-101 is being developed initially for the treatment of heterozygous familial hypercholesterolemia (HeFH).
- VERVE-101 is being evaluated in the Phase 1b heart-1 clinical trial. Enrollment efforts are ongoing in New Zealand and the United Kingdom.
    - heart-1 is designed to evaluate the safety and tolerability of VERVE-101 initially with dose escalation in cohorts of the highest risk HeFH patients. In addition, the trial is designed to measure PCSK9 protein changes in participants, and thereby assess early proof of concept of the ability to base edit in the liver. In the fourth quarter of 2023, Verve expects to report the initial safety, pharmacodynamic, PCSK9, and LDL-C data for the four cohorts in the dose-escalation portion of the heart-1 clinical trial.
- Verve continues to work with the U.S. Food and Drug Administration to resolve the hold on its Investigational New Drug Application for VERVE-101. Based on the progress of the heart-1 clinical trial, the company expects enrollment to be completed outside the United States.

VERVE-102 On-Track for Clinical Trial Initiation in First Half of 2024

- VERVE-102 is an *in vivo* base editing medicine that aims to inactivate the *PCSK9* gene in a similar way to VERVE-101. VERVE-101 and VERVE-102 share an identical guide RNA targeting *PCSK9* as well as similar mRNA expressing an adenine base editor; however, VERVE-102 is delivered using the company’s proprietary GalNAc-LNP delivery technology. Preclinical studies in mice and non-human primates using VERVE-102 with the company’s GalNAc-LNP delivery technology demonstrated effective *in vivo* liver gene editing and significant PCSK9 protein reduction.
- Preclinical development to support a regulatory submission for VERVE-102 began in early 2022, and, following regulatory clearance, Verve expects to initiate a Phase 1b clinical trial with VERVE-102 for patients with HeFH in the first half of 2024.

VERVE-201 On-Track for Clinical Trial Initiation in Second Half of 2024

- VERVE-201, an *in vivo* base editing medicine delivered as a one-time intravenous infusion, is designed to inactivate the *ANGPTL3* gene in liver cells, turning off liver production of blood ANGPTL3 and thereby durably reducing LDL-C and TRLs. VERVE-201 is being developed initially for the treatment of homozygous familial hypercholesterolemia (HoFH), a rare and often fatal genetic subtype of premature atherosclerotic cardiovascular disease (ASCVD) characterized by extremely high blood LDL-C. VERVE-201 aims to reduce the heavy treatment burden associated with available therapies for HoFH including the requirement for multiple oral, injectable, and intravenous infusions, often administered over decades.
- Preclinical data supporting a regulatory submission for the initiation of Phase 1b clinical trial of VERVE-201 will be presented at the European Society of Cardiology (ESC) 2023 Congress. Details of the oral presentation are as follows:
    - **Title:** An investigational in vivo base editing medicine targeting ANGPTL3, VERVE-201, achieves potent and LDLR-independent liver editing in mouse models
    - **Session:** What’s new in lipid lowering
    - **Data and Time:** August 27, 2023 from 10:25-10:35 a.m. CEST
- Preclinical studies to support a regulatory submission for clinical development of VERVE-201 are ongoing, and, following regulatory clearance, Verve expects to initiate a Phase 1b clinical trial with VERVE-201 in the second half of 2024.

Business Development

- In June 2023, Verve and Eli Lilly and Company (Lilly) entered into a global research collaboration focused on advancing Verve's preclinical stage *in vivo* gene editing program targeting Lp(a). Elevated Lp(a) is an established and genetically validated, independent risk factor for ASCVD, ischemic stroke, thrombosis, and aortic stenosis.

  Under the terms of the agreement, Verve received a combined upfront payment and equity investment totaling $60 million. Research program costs through Phase 1 clinical trials will be funded by Lilly. Verve is also eligible to receive up to $465 million in research, development, and commercial milestones, as well as tiered royalties on global net sales. In addition, following the completion of Phase 1 clinical trials, Verve has the right to opt-in to co-fund and share margins globally on the Lp(a) program (in lieu of receipt of milestones and royalties). The collaboration became effective in July following expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act, and Verve expects research activities associated with the Lp(a) collaboration to commence in the third quarter.

Upcoming Investor Events
Verve plans to participate in the following upcoming events:

- Canaccord Genuity 43rd Annual Growth Conference, August 10, Boston, MA
- Stifel Biotech Executive Summit, August 15, Newport, RI

Second Quarter 2023 Financial Results

- **Cash Position:** Verve ended the second quarter of 2023 with $462.5 million in cash, cash equivalents, and marketable securities. Verve expects its existing cash, cash equivalents, and marketable securities, including the additional $60.0 million upfront payment and equity investment from Lilly in August 2023, to be sufficient to fund its operations into 2026.
- **Collaboration Revenue:** Collaboration revenue was $2.1 million for the second quarter of 2023, which was related to the collaboration agreement with Vertex Pharmaceuticals Incorporated. There was no collaboration revenue in the second quarter of 2022.
- **Research & Development (R&D) Expenses:** R&D expenses were $47.3 million for the second quarter of 2023, compared to $33.1 million for the second quarter of 2022.
- **General & Administrative (G&A) Expenses:** G&A expenses were $13.4 million for the second quarter of 2023, compared to $9.1 million for the second quarter of 2022.
- **Net Loss:** Net loss was $54.0 million, or $0.87 basic and diluted net loss per share, for the second quarter of 2023, compared to $40.9 million, or $0.84 basic and diluted net loss per share, for the second quarter of 2022.

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 three programs – VERVE-101, VERVE-102, 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 and VERVE-102 are designed to permanently turn off the *PCSK9* gene in the liver and are 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 for homozygous familial hypercholesterolemia (HoFH) and ultimately to treat patients with refractory hypercholesterolemia. 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 ability to enroll patients in its ongoing heart-1 trial; the timing and availability of clinical data from its heart-1 trial; the company's expectations related to the clinical hold on the IND for VERVE-101; the expected timing of initiating clinical trials of VERVE-102 and VERVE-201; the company's research activities under the Lilly collaboration; its research and development plans; the potential advantages and therapeutic potential of the company's programs, including VERVE-101, VERVE-102, and VERVE-201; 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 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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Verve Therapeutics, Inc.
Selected Condensed Financial Information
(in thousands, except share and per share amounts)
(unaudited)

	Three months ended June 30,		Six months ended June 30,	
Condensed consolidated statements of operations	**2023**	**2022**	**2023**	**2022**
Collaboration revenue	$ 2,093	$ —	$ 3,497	$ —
Operating expenses:				
Research and development	47,260	33,125	94,370	57,614
General and administrative	13,416	9,067	25,969	16,503
Total operating expenses	60,676	42,192	120,339	74,117
Loss from operations	(58,583)	(42,192)	(116,842)	(74,117)
Other income (expense):				
Change in fair value of success payment liability	(662)	938	76	2,615
Interest and other income, net	5,438	308	10,984	390
Total other income, net	4,776	1,246	11,060	3,005
Loss before provision for income taxes	(53,807)	(40,946)	(105,782)	(71,112)
Provision for income taxes	(176)	-	(176)	-
Net loss	$ (53,983)	$ (40,946)	$ (105,958)	$ (71,112)
Net loss per common share, basic and diluted	$ (0.87)	$ (0.84)	$ (1.71)	$ (1.46)
Weighted-average common shares used in net loss per share, basic and diluted	61,953,992	48,674,873	61,871,158	48,623,330

Condensed consolidated balance sheet data	**June 30, 2023**	**December 31, 2022**
Cash, cash equivalents and marketable securities	$ 462,476	$ 554,808
Total assets	$ 589,131	$ 679,223
Total liabilities	$ 123,909	$ 128,291
Total stockholders' equity	$ 465,222	$ 550,932