Tenaya Therapeutics Reports Second Quarter 2023 Financial Results and Provides Business Update

August 9, 2023

Dosing of First Patient in Phase 1b Trial of TN-201 On Track to Occur in Q3 2023

Data from Phase 1 Clinical Trial of TN-301 Accepted for Presentation at HFSA 2023

IND Application for TN-401 Anticipated in Second Half 2023

Second Quarter Cash and Investments of $152 Million;
Runway to Fund Operations into First Half 2025

SOUTH SAN FRANCISCO, Calif., Aug. 09, 2023 (GLOBE NEWSWIRE) -- Tenaya Therapeutics, Inc. (NASDAQ: TNYA), a clinical-stage biotechnology company with a mission to discover, develop and deliver potentially curative therapies that address the underlying causes of heart disease, today reported financial results and provided a corporate update for the second quarter ended June 30, 2023.

“We are looking ahead to important near-term milestones across all our programs. Dosing the first patient in our first clinical trial for TN-201 will be a significant moment for our company and bring us one step closer to bringing much-needed solutions to those impacted by genetic HCM,” said Faraz Ali, Chief Executive Officer of Tenaya. “We are also looking forward to presenting healthy participant data from our TN-301 small molecule program and filing the IND for our TN-401 gene therapy candidate later this year. Our continued progress is supported by our unwavering commitment to fight heart disease on behalf of individuals and families.”

Business and Program Updates
TN-201 – Gene Therapy for MYBPC3-Associated Hypertrophic Cardiomyopathy (HCM)

- In April 2023, the U.S. Food and Drug Administration (FDA) granted Fast Track designation for Tenaya’s lead gene therapy program, TN-201. Companies with therapies that receive the Fast Track designation are eligible for more frequent communication with the agency and may qualify for Accelerated Approval and Priority Review if relevant criteria are met.
- Tenaya anticipates dosing its first patient in the “MyPeak-1” Phase 1b multi-center, open-label, dose-escalation clinical trial of TN-201 during the third quarter of 2023 (ClinicalTrials.gov #NCT05836259).
    - The Phase 1b trial is designed to assess the safety, tolerability and pharmacodynamics of a one-time intravenous infusion of TN-201 in symptomatic adults with *MYBPC3*-associated HCM. Initial data from the MyPeak-1 clinical trial are anticipated in 2024.
- In July 2023, Tenaya received notification from the U.S. Patent and Trademarks Office (USPTO) that a notice of allowance has been issued for patent application No. 17/581,576. The allowed patent application includes claims covering a method of treating HCM or cardiomyopathy caused by a *MYBPC3* mutation and expands upon Tenaya’s existing composition of matter protections by extending coverage to methods of use as the company continues to enhance its protection around different aspects of the TN-201 gene therapy program. The new patent issuing from the allowed patent application is expected to expire no earlier than February 2041.

TN-301 – Small Molecule HDAC6 Inhibitor for Heart Failure with Preserved Ejection Fraction (HFpEF)

- Data from Tenaya’s Phase 1 clinical trial of TN-301 in healthy volunteers have been accepted for presentation at the Heart Failure Society of America’s (HFSA) Annual Scientific Meeting, taking place October 6-9, 2023.
    - Tenaya has completed the Phase 1 clinical trial of TN-301 in healthy participants. No dose-limiting toxicities were reported across a wide range of doses and initial target engagement, measured by the biomarker tubulin acetylation, was achieved.
    - The randomized, double-blind, placebo-controlled clinical trial was designed to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of escalating oral doses of TN-301 to help guide dosing in future studies.
- Results from preclinical studies testing the combination of HDAC6 inhibition with SGLT2 inhibition in a validated model of HFpEF have also been accepted for presentation at the upcoming HFSA meeting.
- Preclinical data from the TN-301 program presented on August 1, 2023, at the American Heart Association’s Basic Cardiovascular Sciences (BCVS) Scientific Session suggest HDAC6 inhibition may improve undesirable HFpEF phenotype

characteristics such as dysregulated metabolism, fibrosis and hypertrophy.

TN-401 – Gene Therapy for PKP2-Associated Arrhythmogenic Right Ventricular Cardiomyopathy (ARVC)

- Tenaya plans to submit an Investigational New Drug application (IND) to the FDA for TN-401 for the potential treatment of ARVC caused by *PKP2* genetic mutations in the second half of 2023. A single dose of TN-401 is designed to deliver a fully functional *PKP2* gene to cardiomyocytes in the heart and restore production of the plakophilin 2 protein in order to prevent arrhythmia and reverse disease following a single dose.
- Tenaya presented data from a new preclinical mouse model at BCVS on August 1, 2023, showing that a single dose of gene therapy prevented ARVC development and significantly prolonged survival.
- In June 2023, Tenaya received notification from the USPTO that a notice of allowance has been issued for patent application No. 17/390,395. The new patent issuing from the allowed patent application will be the first patent issued by the USPTO to cover an AAV gene therapy for *PKP2* and is expected to expire no earlier than July 2041.

Research

- Tenaya researchers also presented new data at BCVS on August 1, 2023, detailing the company's drug discovery capabilities for identifying human genetic targets in models of human heart conditions. Tenaya's novel high throughput screening methods in human induced pluripotent stem cells derived cardiomyocytes were applied to identifying genes of interest in heart muscle cells.

Second Quarter 2023 Financial Highlights

- **Cash Position and guidance:** As of June 30, 2023, cash, cash equivalents and investments in marketable securities were $151.6 million. Tenaya expects current cash, cash equivalents and investments in marketable securities (current and noncurrent) will be sufficient to fund the company into the first half of 2025.
- **Research & Development (R&D) Expenses:** R&D expenses for the quarter ended June 30, 2023, were $26.5 million. Non-cash stock-based compensation included in R&D expense was $1.8 million for the quarter ended June 30, 2023.
- **General & Administrative (G&A) Expenses:** G&A expenses for the quarter ended June 30, 2023, were $8.6 million. Non-cash stock-based compensation included in G&A expense was $2.1 million for the quarter ended June 30, 2023.
- **Net Loss:** Net loss for the quarter ended June 30, 2023, was $33.3 million, or $0.45 per share.

About Tenaya Therapeutics
Tenaya Therapeutics is a clinical-stage biotechnology company committed to a bold mission: to discover, develop and deliver potentially curative therapies that address the underlying drivers of heart disease. Leveraging its integrated and interrelated Gene Therapy, Cellular Regeneration and Precision Medicine platforms and proprietary core capabilities, the company is advancing a pipeline of novel therapies with diverse treatment modalities for rare genetic cardiovascular disorders and more prevalent heart conditions. Tenaya's most advanced candidates include TN-201, a gene therapy for *MYBPC3*-associated hypertrophic cardiomyopathy (HCM), TN-401, a gene therapy for *PKP2*-associated arrhythmogenic right ventricular cardiomyopathy (ARVC), and TN-301, a small molecule HDAC6 inhibitor being initially developed for heart failure with preserved ejection fraction (HFpEF). Tenaya also has multiple early-stage programs progressing through preclinical development. For more information, visit www.tenayatherapeutics.com.

Forward-looking Statement
This press release contains forward-looking statements as that term is defined in Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. Statements in this press release that are not purely historical are forward-looking statements. Words such as "anticipated," "looking ahead," "looking forward," "may," "expected," "plans," "will," and similar expressions are intended to identify forward-looking statements. Such forward-looking statements include, among other things, statements regarding 2023 development and regulatory milestones; Tenaya's plan to present data from the Phase 1 clinical trial evaluating TN-301, as well as results from preclinical studies, at the upcoming HFSA conference; expected timing of the submission of the TN-401 IND; eligibility for increased communication with the FDA and potential qualification for accelerated approval and priority review for TN-201; expected timing for, commencement of dosing in the Phase 1b clinical trial evaluating TN-201 and availability of initial data from the trial; the expected expiration dates of new patents; the potential for Tenaya's future programs; the sufficiency of Tenaya's cash resources to fund the company into the first half 2025; and statements by Tenaya's chief executive officer. The forward-looking statements contained herein are based upon Tenaya's current expectations and involve assumptions that may never materialize or may prove to be incorrect. These forward-looking statements are neither promises nor guarantees and are subject to a variety of risks and uncertainties, including but not limited to: Tenaya's ability to develop, initiate or complete preclinical studies and clinical trials for its product candidates; the timing, scope and likelihood of regulatory filings and approvals; the availability of data at the referenced times; risks associated with the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics and operating as an early stage company; Tenaya's ability to raise any additional funding it will need to continue to pursue its business and product development plans; the potential for any clinical trial results to differ from preclinical, interim, preliminary, topline or expected results; Tenaya's ability to successfully operate a manufacturing facility for clinical or commercial supply; Tenaya's commercialization and marketing capabilities and strategy; the loss of key scientific or management personnel; competition in the industry in which Tenaya operates; Tenaya's reliance on third parties; Tenaya's ability to obtain and maintain intellectual property protection for its product candidates; general economic and market conditions; and other risks. Information regarding the foregoing and additional risks may be found in the section entitled "Risk Factors" in documents that Tenaya files from time to time with the Securities and Exchange Commission. These forward-looking statements are made as of the date of this press release, and Tenaya assumes no obligation to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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TENAYA THERAPEUTICS, INC.

Condensed Statements of Operations
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	**2022**	**2023**	**2022**
Operating expenses:				
Research and development	$ 26,477	$ 20,876	$ 52,082	$ 45,031
General and administrative	8,627	7,743	16,745	14,742
Total operating expenses	35,104	28,619	68,827	59,773
Loss from operations	(35,104)	(28,619)	(68,827)	(59,773)
Other income (expense), net:				
Interest income	1,837	222	3,810	321
Other income (expense), net	(2)	—	11	(1)
Total other income (expense), net	1,835	222	3,821	320
Net loss before income tax expense	(33,269)	(28,397)	(65,006)	(59,453)
Income tax expense	—	—	—	—
Net loss	$ (33,269)	$ (28,397)	$ (65,006)	$ (59,453)
Net loss per share, basic and diluted	$ (0.45)	$ (0.69)	$ (0.89)	$ (1.44)
Weighted-average shares used in computing net loss per share, basic and diluted	73,399,847	41,302,157	73,249,702	41,285,168

Condensed Balance Sheet Data
(In thousands)
(Unaudited)

	June 30, 2023	**December 31, 2022**
Cash, cash equivalents and marketable securities	$ 151,641	$ 204,230
Total assets	$ 220,754	$ 278,945
Total liabilities	$ 30,814	$ 35,569
Total liabilities and stockholders' equity	$ 220,754	$ 278,945