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

May 10, 2023

TN-201 Received Fast Track Designation from the FDA

Dosing Complete in Phase 1 Clinical Trial of TN-301; Data Expected in Second Half 2023

Renowned Researchers Drs. Christine Seidman and Alex Marson Join Scientific Advisory Board

Announces Formation of Technical Advisory Board with Deep Expertise in Gene Therapy Manufacturing

SOUTH SAN FRANCISCO, Calif., May 10, 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 first quarter ended March 31, 2023.

"Tenaya is making meaningful progress across multiple fronts of our business while maintaining a solid cash position. We are pleased to have recently received Fast Track designation for TN-201 and completed dosing in our Phase 1 trial of TN-301. We remain on track to achieve our 2023 development and regulatory milestones for TN-201, TN-301 and TN-401, including the release of clinical data for TN-301 in the second half of 2023," said Faraz Ali, Chief Executive Officer of Tenaya. "In addition to our focus on achieving near-term catalysts, we continue to invest in our future. We look forward to highlighting the depth and breadth of our core capabilities at the upcoming ASGCT conference. We have also added leading industry and academic experts to our Scientific Advisory Board and to our newly created Technical Advisory Board. These efforts will collectively help Tenaya lay the foundation for commercial-ready manufacturing for our existing product candidates and support the expansion of Tenaya's pipeline into new modalities, including gene editing, so that we can bring the promise of genetic medicines to individuals and families fighting heart disease."

Business and Program Updates

TN-201 – Gene Therapy for MYBPC3-Associated Hypertrophic Cardiomyopathy (HCM)

Tenaya received notification that the U.S. Food and Drug Administration (FDA) granted Fast Track designation to TN-201. The FDA Fast Track program is designed to facilitate the development and expedite the review of drug candidates intended to treat serious conditions and for which nonclinical data demonstrates the potential to address unmet medical need.

- In January 2023, the FDA notified Tenaya that clinical testing of TN-201 may proceed based on review of the company's Investigational New Drug (IND) application.
- Tenaya plans to commence a Phase 1b multi-center, open-label, dose-escalation clinical trial in the third quarter of 2023. The Phase 1b clinical 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 Phase 1b clinical trial are anticipated in 2024.

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

- Tenaya recently completed dosing of healthy participants in its Phase 1 clinical trial. Initial target engagement, measured by the biomarker tubulin acetylation, was achieved and no dose-limiting toxicities were reported. The Phase 1 clinical trial was designed to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of escalating oral doses of TN-301.
- Tenaya expects to report results from both the single and multiple ascending dose stages of the Phase 1 clinical trial in the second half of 2023.

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

- Tenaya plans to submit an IND application for TN-401 to the FDA in the second half of 2023. TN-401 is being developed for the potential treatment of ARVC caused by *PKP2* genetic mutations and is designed to deliver a fully functional *PKP2* gene to prevent arrhythmia and reverse disease following a single dose.

Research Updates

- In May 2023, Tenaya researchers will present six abstracts at the American Society of Gene and Cell Therapy (ASGCT) 26th Annual Meeting detailing the company's expanded capabilities for manufacturing and discovering genetic medicines for heart disease. Tenaya's continued growth and optimization across its enabling technologies supports current pipeline candidates and is expected to increase opportunities for future programs. The presentations will highlight innovations in manufacturing, capsid engineering and gene editing.

Leadership

- Tenaya expanded its Scientific Advisory Board with the appointments of Christine Edry Seidman, M.D., and Alexander Marson, M.D., Ph.D.
    - Dr. Seidman leads a laboratory that aims to define genetic causes, mechanisms, treatments and cures for human heart disease. Her work enabled development of the first mechanism-based treatment for hypertrophic cardiomyopathy. Among her current roles, she serves as the Director of the Cardiovascular Genetics Center at Brigham and Women's Hospital, and a professor of genetics and medicine at Harvard Medical School. Previously, Dr. Seidman was a scientific co-founder of MyoKardia, a precision medicine cardiovascular company focused on cardiomyopathies (acquired by BMS).
    - Dr. Marson's research focuses on understanding the genetic circuits that control human immune cell function and deploying genome editing technologies such as CRISPR to create engineered cells capable of detecting, resisting and eliminating disease. He currently serves as Director of the Gladstone-UCSF Institute of Genomic Immunology and professor of medicine at UCSF. He is also co-founder of Spotlight Therapeutics, an *in vivo* genome editing company using non-viral delivery.

- Tenaya announced the formation of a Technical Advisory Board (TAB) to provide expert perspective on manufacturing, quality, regulatory process development and analytical development strategies across a range of modalities. Members of the TAB each bring significant expertise in the manufacture of genetic medicines and include:
    - Mark Angelino, Ph.D., Venture Partner at Third Rock Ventures. Dr. Angelino brings broad operational experience, most recently as co-founder and Chief Operating Officer of Generation Bio, a non-viral delivery genetics medicine company, and prior as the Senior Vice President of Pharmaceutical Sciences at bluebird bio, an *ex vivo* autologous lentiviral vector gene therapy company.
    - Guangping Gao, Ph.D., Director of the Li Weibo Institute for Rare Diseases and professor at UMass Chan Medical School. Dr. Gao is widely recognized for his significant contributions to the field of discovery and manufacturing of viral vector gene therapies for rare diseases.
    - Victoria Sluzky, Ph.D., is founder and principal of Lucid Biotechnology Advisors with more than 30 years of biotechnology industry experience. Dr. Sluzky formerly served as the Senior Vice President of Technical Development at BioMarin Pharmaceutical where she was directly involved in advancing product development in many rare diseases, and providing expertise in quality, process and assay development, formulation and regulatory strategy.
    - Angela Thedinga, MBA, MPH, is presently a Board member of Nkarta, Inc. Previously, Ms. Thedinga was the Chief Technology Officer at Adverum and led manufacturing strategy and program management at AveXis (now Novartis Gene Therapies).

First Quarter 2023 Financial Highlights

- **Cash Position and updated cash guidance:** As of March 31, 2023, cash, cash equivalents and investments in marketable securities were $173.6 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 March 31, 2023, were $25.6 million. Non-cash stock-based compensation included in R&D expense was $1.6 million for the quarter ended March 31, 2023.
- **General & Administrative (G&A) Expenses:** G&A expenses for the quarter ended March 31, 2023, were $8.1 million. Non-cash stock-based compensation included in G&A expense was $1.9 million for the quarter ended March 31, 2023.
- **Net Loss:** Net loss for the quarter ended March 31, 2023, was $31.7 million, or $0.43 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 "expected," "look forward," "plans," "anticipated," "expects," "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 abstracts covering manufacturing, capsid engineering and gene editing at the upcoming ASGCT conference; expected timing for, commencement of dosing in the Phase 1b clinical trial evaluating TN-201 and availability of initial data from the trial, data from the SAD and MAD stages of the Phase 1 clinical trial evaluating TN-301 and the submission of the TN-401 IND; 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 March 31,	
	2023	**2022**
Operating expenses:		
Research and development	$ 25,605	$ 24,155
General and administrative	8,118	6,999
Total operating expenses	33,723	31,154
Loss from operations	(33,723)	(31,154)
Other income (expense), net:		
Interest income	1,973	99
Other income (expense), net	13	(1)
Total other income (expense), net	1,986	98
Net loss before income tax expense	(31,737)	(31,056)
Income tax expense	—	—
Net loss	$ (31,737)	$ (31,056)
Net loss per share, basic and diluted	$ (0.43)	$ (0.75)
Weighted-average shares used in computing net loss per share, basic and diluted	73,097,889	41,267,990

TENAYA THERAPEUTICS, INC.

Condensed Balance Sheet Data
(In thousands)
(Unaudited)

	March 31, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 173,635	$ 204,230
Total assets	$ 245,341	$ 278,945
Total liabilities	$ 29,929	$ 35,569
Total liabilities and stockholders' equity	$ 245,341	$ 278,945