Tenaya Therapeutics Receives FDA Fast Track Designation for TN-201

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TN-201 Being Developed for the Potential Treatment of MYBPC3-associated HCM

Dosing in Phase 1 Clinical Trial Expected to Commence in Q3 2023

SOUTH SAN FRANCISCO, Calif., May 02, 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 announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for its gene therapy product candidate, TN-201, being developed for the treatment of Myosin Binding Protein C3 *(MYBPC3)*-associated hypertrophic cardiomyopathy (HCM).

TN-201 is Tenaya's potential first-in-class adeno-associated virus (AAV)-based investigational gene therapy for the treatment of HCM caused by mutations in the *MYBPC3* gene, the most common genetic cause of HCM. TN-201 is designed to deliver a fully functional *MYBPC3* gene to restore normal levels of myosin-binding protein, which regulates the contraction and relaxation of the heart muscle. In preclinical studies of *MYBPC3* knock-out models, TN-201 has been shown to halt disease progression and demonstrated significant and durable disease reversal and survival benefit after a single dose.

"Receipt of Fast Track designation for TN-201 reflects the pressing unmet need among HCM patients whose disease is caused by MYBPC3 genetic mutations," said Whit Tingley, M.D., Ph.D., Chief Medical Officer of Tenaya. "As we prepare to begin dosing patients later this year, we look forward to continued close collaboration with the FDA under this designation in support of TN-201's development."

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. Companies with therapies that receive the Fast Track designation from the FDA are eligible for increased communication with the agency and may qualify for accelerated approval and priority review if relevant criteria are met. The goal of the program is to deliver approved treatments to patients with a serious or life-threatening condition as quickly as possible. This designation is not an assurance that regulatory approval will be received. TN-201 also has received Orphan Drug Designation from the FDA and Orphan Medicinal Product designation from the European Commission for the treatment of HCM due to mutations in the *MYBPC3* gene.

In January 2023, Tenaya announced that the FDA cleared its Investigational New Drug application (IND) for TN-201. Tenaya is initiating the MyPeak-1 Phase 1b clinical trial to assess the safety, tolerability and clinical efficacy of a one-time intravenous infusion of TN-201. The multi-center, open-label study will enroll adults diagnosed with *MYBPC3*-associated nonobstructive HCM. Tenaya anticipates the first patient will be dosed in this clinical trial during the third quarter of 2023. Tenaya is also conducting two non-interventional studies to support the development of TN-201: a study evaluating seroprevalence to AAV9 antibodies among adults with *MYBPC3*-associated HCM, and MyClimb, a natural history study of pediatric patients with *MYBPC3*-associated HCM.

About *MYBPC3*-Associated Hypertrophic Cardiomyopathy
Hypertrophic cardiomyopathy (HCM) is the most common inherited cardiac disorder and variants in the Myosin Binding Protein C3 (*MYBPC3)* gene are the most common genetic cause of HCM. *MYBPC3*-associated HCM is estimated to account for approximately 20 percent of the overall HCM population and to affect approximately 115,000 patients in the United States alone. MYBPC3-associated HCM is a chronic, progressive condition characterized by left ventricular thickening, hypercontractility, fibrosis, abnormal heart rhythms, cardiac dysfunction and impaired diastolic relaxation. This in turn leads to serious complications including debilitating symptoms such as shortness of breath, fainting and palpitations; heart failure; significant impairment in overall quality of life; and sudden cardiac death in some adults and children. There are currently no approved therapeutics addressing the underlying genetic cause of HCM. TN-201 is Tenaya's first-in-class adeno-associated virus (AAV)-based gene therapy candidate being developed to treat hypertrophic cardiomyopathy (HCM) due to disease-causing variants in the *MYBPC3* gene.

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 Statements
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 "potential," "look forward," "eligible," "may," "anticipates," and similar expressions are intended to identify forward-looking statements. Such forward-looking statements include, among other things, statements regarding the therapeutic potential of TN-201 for *MYBPC3*-associated HCM patients; the timing for dosing of patients in the Phase 1b clinical trial evaluating TN-201; eligibility for increased communication with the FDA and potential qualification for accelerated approval and priority review for TN-201; and statements by Tenaya's Chief Medical 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: the timing, scope and likelihood of regulatory filings and approvals; Tenaya's ability to initiate and complete clinical trials for its product candidates; 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; the potential for any clinical trial results to differ from preclinical, interim, preliminary, topline or expected results; the level of costs associated with Tenaya's research, development, manufacturing and other activities and the company's ability to raise any additional funding it will need to continue to pursue its business and product development plans; 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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