Tenaya Therapeutics Receives Orphan Drug Designation and Presents Pre-Clinical Data for its Most Advanced Gene Therapy Product Candidate for Genetic Hypertrophic Cardiomyopathy

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- TN-201 is a potentially curative gene therapy intended to address genetic hypertrophic cardiomyopathy (gHCM) caused by mutations in the Myosin Binding Protein C3 (*MYBPC3*) gene, that is currently in IND-enabling studies.
- TN-201 has been granted Orphan Drug Designation by the U.S. Food and Drug Administration.
- Preclinical data supporting TN-201 presented at the American Society of Gene & Cell Therapy conference demonstrated significant and durable disease reversal in a severe murine model of disease.

SOUTH SAN FRANCISCO, Calif – May 20, 2021 – Tenaya Therapeutics, a biotechnology company with a mission to discover, develop and deliver curative therapies that address the underlying causes of heart disease, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) for TN-201, its most advanced product candidate from its proprietary Gene Therapy Platform.

TN-201 is a potentially curative, one-time, adeno-associated virus (AAV)-based gene therapy to address genetic hypertrophic cardiomyopathy (gHCM) caused by Myosin Binding Protein C3 (*MYBPC3*) gene mutations. TN-201 is in IND-enabling studies following candidate selection in 2020 and having obtained feedback from multiple regulatory agencies, including the FDA, to guide its path to clinical development.

Mutations of the *MYBPC3* gene cause the heart walls of affected individuals to become significantly thickened, leading to abnormal heart rhythms, cardiac dysfunction, heart failure and sudden cardiac death in some adults and children. Mutations of this gene are the most common genetic cause of HCM, estimated to represent approximately 19% of the overall HCM population and to affect approximately 115,000 patients in the United States alone. Current treatments do not address the underlying genetic cause, do not address all the symptoms of the disease, and do not appear to affect disease progression.

Tenaya presented pre-clinical data supporting TN-201 at the American Society of Gene & Cell Therapy (ASGCT) 24th Annual Meeting (see Abstract 523 titled "*Reversal of Cardiac Hypertrophy with an Optimized MYBPC3 Gene Therapy*"). These data demonstrated significant reversal of left ventricular hypertrophy, cardiac dysfunction, and electrophysiological deficits using a murine orthologue of TN-201 in a severe murine model of the disease. This disease reversal was dose-dependent and stable for more than one year following a single infusion, and a survival benefit was observed vs untreated controls. No safety signals have been observed to date.

"We are pleased that the FDA has granted orphan drug designation for TN-201," said Faraz Ali, CEO of Tenaya. "The feedback we have received from multiple regulatory agencies supports our IND-enabling studies, planned scale-up of AAV manufacturing at our new cGMP facility, and the design of planned first-in-human clinical studies. The data presented at ASGCT provides new hope for individuals with HCM due to *MYBPC3* mutations who lack therapies to address the underlying genetic cause of their disease."

Orphan designation qualifies Tenaya for various development incentives as part of the Orphan Drug Act, including tax credits for certain clinical trials. This designation is not an assurance that regulatory approval will be received but would grant TN-201 with 7 years of market exclusivity if such approval is achieved.

About Tenaya Therapeutics

Tenaya Therapeutics is a biotechnology company committed to a bold mission: to discover, develop and deliver curative therapies that address the underlying drivers of heart disease. Tenaya is developing therapies for rare genetic disorders as well as for more prevalent heart conditions through three distinct but interrelated product platforms: Gene Therapy, Cellular Regeneration and Precision Medicine. Founded by leading cardiovascular scientists from the J. David Gladstone Institutes and the University of Texas Southwestern Medical Center, Tenaya is backed by an established syndicate of investors. For more information, please visit www.TenayaTherapeutics.com and follow us on LinkedIn.

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