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Tenaya Therapeutics - Developing Gene Therapies for both Rare and Prevalent Forms of Heart Disease that Affect Millions of People

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CEOCFO: ***Mr. Ali, Tenaya Therapeutics is focused on developing gene therapies for hereditary cardiomyopathies, which is a space that has been historically underserved. What makes your approach unique and why are these diseases such an important target for innovation; why are diseases important in general?***

Mr. Ali: Tenaya Therapeutics was founded in 2016. We are 100% focused on heart disease. A big part of our focus is on genetic forms of heart disease and striving to target the underlying cause to create more effective and durable treatments. One of the things that makes us unique is that we're developing therapies for both rare and prevalent forms of heart disease that affect millions of people.

Another thing that I think makes us unique is that we're not just focused on one modality for our treatments. In addition to our two clinical-stage gene therapies for genetic cardiomyopathies, we've discovered a promising small molecule, and our early-stage pipeline includes gene editing and gene silencing technologies. Our singular focus on the heart allows us to "follow the science" and tailor solutions to the biology of a given condition. This ability to go after rare and prevalent cardiovascular conditions targeting the underlying genetic cause of disease using the treatment modality best suited to the disease gives us multiple horizons for growth, innovation and value creation.

CEOCFO: ***Would you tell us more about the gene therapies?***

Mr. Ali: TN-201 is our lead gene therapy designed to address the leading genetic cause of hypertrophic cardiomyopathy (HCM), a debilitating condition that affects over 600,000 people in the U.S. alone. Among these, approximately 20% – or more than 120,000 individuals – have a specific mutation in the *MYBPC3* gene, which is the target for our therapy.

What sets TN-201 apart is that it's the first and only clinical-stage candidate aimed at correcting the underlying genetic cause of the disease. Patients with this mutation produce insufficient levels of MyBP-C protein, which is essential for the proper regulation of contractions of the heart. Without enough of this protein, the heart becomes hyper contractile, and its function deteriorates over time, leading to thickening of the ventricular walls, fibrosis, scarring arrhythmias, and ultimately heart failure.

Initial symptoms may be mild – shortness of breath, dizziness or fainting—but can progress to severe limitations in daily life. In advanced cases, patients may require open-heart surgery to remove excess tissue or even face heart transplantation. By addressing the root cause of the disease, our goal is to halt its relentless progression and potentially restore lost cardiac function.

This approach is fundamentally different from currently approved therapies, which are primarily small molecules that manage symptoms rather than modify the course of disease. We believe TN-201 represents a transformative opportunity for patients with genetic HCM.

"In short, the next twelve months will be defined by deeper clinical insights, potential regulatory alignment, and the opportunity to transition into pivotal trials for one or both of our gene therapy candidates – bringing us closer to delivering transformative therapies to patients and unlocking significant value for stakeholders." Faraz Ali

CEOCFO: ***Why has no one tried this approach before? What do you understand at Tenaya that makes you think it is feasible to get there?***
Mr. Ali: What's enabling our approach today is a fundamental shift in our understanding of the genetics of heart disease. It's very similar to the transformation we saw in oncology. In the past, a diagnosis like breast cancer was treated as a single disease. Now, thanks to advances in genetic profiling, we can identify specific subtypes – like HER2-positive or triple-negative breast cancer—and targeted therapies can be prescribed accordingly. That precision medicine revolution in cancer, which has been successful both in terms of impacting human life and in creating multiple thriving businesses, is now coming to cardiology.

One of the key drivers of this shift is our improved ability to identify and understand the genetic mutations that cause heart disease. We're not alone in this vision—there's a growing body of research and clinical momentum behind it.

The second major breakthrough is in delivery. Gene therapy has evolved to the point where we can now deliver genetic material directly to the heart, and even to specific cells within the heart. That means we can add a healthy copy of a gene, silence a defective one, or even edit it at the source.

At Tenaya, we're operating at the intersection of these two revolutions: deeper genetic insight and advanced delivery technology. This convergence allows us to pursue true precision medicine for heart disease—targeting the root causes rather than just managing symptoms. We believe this is the future of cardiology, and Tenaya is at the vanguard of that movement, much like the pioneers who reshaped oncology over the past few decades.

CEOCFO: ***Would you tell us about the other programs in your pipeline?***
Mr. Ali: TN-201 is one example of how Tenaya is applying precision medicine to cardiology. Another is our clinical-stage gene therapy candidate, TN-401, which targets arrhythmogenic right ventricular cardiomyopathy (ARVC). ARVC is a serious, progressive condition characterized by electrical instability. While HCM primarily affects the heart's muscle, ARVC disrupts its rhythm, often leading to life-threatening arrhythmias.

This is a high-risk patient population. Nearly all of the individuals we're treating already have implantable cardioverter defibrillators (ICDs) to prevent sudden cardiac arrest. Alarmingly, for about 25% of patients, the first indication of disease is sudden cardiac death. That statistic underscores the urgency and unmet need in this space.

TN-401 is designed to address the leading genetic cause of ARVC, a mutation in the PKP2 gene, which accounts for roughly 40% of cases. Our approach involves delivering a full-length, functional copy of the PKP2 gene directly to

cardiomyocytes. By restoring the missing protein, we aim to correct the underlying defect, stabilize the disease, and improve cardiac rhythm and function.

This program reflects the same core strategy as TN-201: target the root genetic cause, prevent disease progression, and restore heart health. But it also expands our reach into another underserved and high-impact area of cardiology. Together, these programs demonstrate the breadth and scalability of our platform – and the potential to drive meaningful clinical and commercial value across multiple indications.

CEOCFO: ***What do you see ahead for the next twelve months or so?***
Mr. Ali: We're entering a pivotal period over the next twelve months. Both of our gene therapy programs – TN-201 and TN-401 – are actively dosing patients, and we've already shared early clinical data for TN-201 including a presentation at the American College of Cardiology Congress. We've committed to providing additional data updates in Q4, which will include safety data—critical in these first-in-human studies—as well as biopsy results that help us assess transgene expression and protein restoration.

From the initial dose cohort of our TN-201 program, we've seen encouraging signs. All three patients showed improvements in blood-based biomarkers and biopsy parameters. Two out of three demonstrated improvements in one or more measures of hypertrophy, meaning that the thickening of the heart that is a hallmark of the disease showed signs of reduction. All three improved in their New York Heart Association (NYHA) classification – from experiencing functional limitations that impacted their ability to perform tasks of daily living to Class I, meaning no limitations due to their disease. That's a remarkable outcome for the first patients treated at the lowest dose.

Looking ahead to Q4, we'll expand on that dataset with longer-term follow-up and introduce early data from the high-dose cohort. The goal is to maintain safety while increasing protein expression and potentially enhancing clinical benefit. If the data continue to be compelling, we believe we'll be in a position to engage the FDA in discussions about initiating a pivotal study. We're also exploring pediatric indications for TN-201. Some children with the MYBPC3 mutation experience severe disease from infancy, and current treatment options are extremely limited.

In gene therapy, regulators have shown a willingness to advance programs into pivotal trials based on strong early data, even from small patient cohorts. That opens the door for Tenaya to pursue alignment with the agency and get the green light to move forward into a pivotal study within the next year, which would be an exciting inflection point for the company. If the FDA supports pediatric development, it could enable a faster, smaller pivotal study with high impact and urgency.

It's important to note that much of what I've said about TN-201 applies to TN-401. We will have our first look at data from that clinical trial later this year, focused on safety and biopsy data for the lower dose of TN-401 that we're testing. Additional safety and efficacy data will be coming over the next twelve months for both programs, and both have the potential to reach a point where we can engage with the FDA about initiating pivotal studies. That's a rare and exciting position for a company at our stage – two programs with near-term regulatory and commercial potential.

In short, the next twelve months will be defined by deeper clinical insights, potential regulatory alignment, and the opportunity to transition into pivotal trials for one or both of our gene therapy candidates – bringing us closer to delivering transformative therapies to patients and unlocking significant value for stakeholders.

CEOCFO: ***With so much opportunity, how do you decide what to work on related to the heart and how do you balance the scientific innovation with potential profitability?***
Mr. Ali: We have more things than we could possibly move forward with the capital that is available to us today. Like any well-run company, we have to make disciplined choices based on available capital and the potential for near-term impact.

We've chosen to prioritize TN-201 and TN-401 because they represent our fastest path to FDA approval and commercial launch. Achieving approval could transform our profile—from a clinical-stage biotech to a revenue-generating company. That shift would significantly change how investors view and value the business, as it would de-risk the science and open the door to broader financial support.

At the same time, we're actively exploring partnerships and non-dilutive funding opportunities – such as grants and business development deals – to advance other promising programs in our portfolio. These discussions include both large and small companies who see the potential in our science. Through collaborations, we can continue to participate in the upside of those programs while maintaining focus on our lead assets.

In short, our strategy balances scientific innovation with capital efficiency. We're laser-focused on advancing TN-201 and TN-401 toward approval, while laying the groundwork for the next wave of value creation through partnerships and strategic funding.

CEOCFO: ***There are different medical conditions that are more in favor with the investment community at different periods; where do heart problems sit today?***
Mr. Ali: We're at the beginning of a major shift in how investors view heart disease. Historically, even though heart disease is the number one cause of death in the world, cardiology hasn't received the same attention as other indications, such as oncology. But that's changing. Recent successes – like MyoKardia's approval of a small molecule for HCM and its subsequent acquisition by Bristol Myers Squibb – have demonstrated that precision approaches to treating heart disease can deliver both clinical impact and strong financial returns. That is just one recent example. In addition to BMS's acquisition of MyoKardia, Eli Lilly recently acquired Verve Therapeutics, which is developing gene editing therapies for genetically driven cholesterol disorders. Other companies have shown that precision approaches to cardiovascular disease – whether through small molecules, gene therapies, or gene editing – can generate significant interest and value for investors.

The concept of precision medicine in cardiology is resonating. Investors are increasingly looking beyond traditional therapies for cholesterol or hypertension and toward solutions that address severe, genetically defined conditions. The parallels to oncology are clear: as our understanding of cardiac genetics deepens, so does the opportunity to develop targeted, disease-modifying therapies.

So, while cardiology may not yet rival oncology in terms of investor volume, the momentum is building. With each success, investor confidence grows, and the sector becomes increasingly attractive. At Tenaya, we believe we're well-positioned to lead in this next wave of precision cardiology innovation.

CEOCFO: ***Why consider Tenaya Therapeutics?***
Mr. Ali: Tenaya Therapeutics is a first-in-class and best-in-class company at the forefront of a transformative shift in precision medicine for heart disease. We're leading the charge in applying genetic insights and advanced delivery technologies to develop therapies that target the root causes of serious cardiac conditions – something no one else is doing at this scale.

Our lead programs, TN-201 and TN-401, are generating clinical data and setting the stage for regulatory engagement that could pave the way for FDA approvals in the coming years. These programs represent significant value inflection points, and behind them is a robust pipeline of additional assets and the expertise and platform capabilities that will drive future growth.

From an investment perspective, Tenaya offers a compelling opportunity. In today's market, we're trading at an attractive valuation, giving investors the chance to get in early on a company that's positioned to lead in a high-impact, high-growth therapeutic area. It's akin to investing in a major oncology innovator in its early days—before the field matured into one of biotech's most lucrative sectors.

We're not just building a company – we're helping shape the future of cardiology. For investors looking for differentiated science, near-term catalysts, and long-term upside, Tenaya is a company to watch.