INVEST IN **MYOSANA THERAPEUTICS**

Get a Piece of Myosana Therapeutics

myosanatherapeutics.com Seattle, WA

Highlights

1. Developing a gene therapy treatment for Duchenne muscular dystrophy (DMD).
2. Designed to deliver full-length dystrophin to skeletal and cardiac muscle.
3. Uses proprietary GLUT4-targeting antibodies to deliver DNA to muscle cells efficiently.
4. Non-viral design allows repeat dosing, unlike most viral gene therapies.
5. Investment from Parent Project Muscular Dystrophy, CureDuchenne Ventures, angel investor.
6. Preclinical studies show significant dystrophin expression in DMD animal models.
7. DMD treatment market projected to reach about $10B by 2030.
8. A founding team with extensive experience in neuromuscular disease research and drug development.

Featured Investors

John Ballantyne Follow Invested $50,000

John Ballantyne is a founder of Agathos Biologics and a Partner in the Kineticos Disruptor Fund. He co-founded Aldevron with Michael Chambers in 1998 and was the Chief Scientific Officer from inception through to the end of 2021.

""Built on decades of expertise in neuromuscular diseases, Myosana's muscle-specific non-viral gene therapy platform is poised to change the way patients are treated for neuromuscular and cardiac diseases. ... "Myosana's progress building on the platform's promising in vivo data is an essential next step. I'm excited to partner with the company as it pushes ahead toward a therapy that will reach patients and improve their lives."

I have seen across the course of my career the massive difference formulation and delivery platforms can make to the efficacy of nucleic acid-based (and other) therapies and prophylactics. I strongly believe the GLUT4 entry target will allow for the precise delivery of full-length genes to address DMD and other diseases.

Furthermore, the safety of non viral and non immunogenic delivery modes in situations where chronic treatment is needed, make the platform binder adaptability a potentially utilitarian approach across multiple cell types."

View Investment Memo

Parent Project Muscular Dystrophy Follow

Parent Project Muscular Dystrophy fights to end Duchenne. We accelerate research, raise our voices to impact policy, demand optimal care for every single family, and strive to ensure access to approved therapies.
parentprojectmd.org

Eric Camino, PhD, Vice President of Research and Clinical Innovation

"With this programmatic investment in Myosana, PPMD continues our cutting-edge approach to accelerate treatments that have the potential to end Duchenne for every single person impacted by the disease," said Eric Camino, PhD, PPMD's Vice President of Research and Clinical Innovation. "There is compelling preliminary evidence showing that Myosana's non-viral gene delivery platform complex can deliver full-length dystrophin to muscle tissue. This investment from PPMD will enable the Myosana team to further advance the development of their platform complex in the hopes of improving the health and function of dystrophic muscle in all people living with Duchenne."

Muscular Dystrophy Association

Follow

Being diagnosed with a neuromuscular disease can bring fear, frustration, and far too many unknowns. MDA brings something else: access to expert care, science that moves theory to therapy, and programs built to support life beyond the diagnosis.

Sharon Hesterlee, PhD, interim President & CEO

"Full-length gene delivery of dystrophin has the potential to restore full functionality of the dystrophin protein at the sarcolemma which may achieve a better result than current microdystrophin gene replacement approaches,""

Cure Duchenne

Follow

CureDuchenne breaks the traditional charitable mold and balances passion with business acumen. We will fulfill our mission to cure Duchenne muscular dystrophy with our innovative venture philanthropy model that funds groundbreaking research, early diagnosis and treatment access. With pioneering education and support programs, our organization drives real change for those with living with the disease and their loved ones.
cureduchenne.org

Debra Miller, Chief Executive Officer and Founder of CureDuchenne

"The strength of Myosana's team and early work made this an obvious investment on behalf of the Duchenne community," said Debra Miller, CEO and Founder of CureDuchenne. "We have been a leader in funding solutions for future problems like immunogenicity to traditional AAV gene therapy programs. We're very excited at the prospect of Myosana's non-viral gene therapy offering full-length dystrophin to potentially treat 100% of the Duchenne population."

Team

Nicholas Whitehead CEO, Co-Founder and CSO

Expert in muscle physiology and DMD research for more than 20 years. PhD from Monash University, Australia. Associate Professor at the University of Washington. Pivotal research elucidated novel roles of dystrophin. Inventor of Myosana's technology.

Stan Froehner Co-Founder and Chairman

Educated at UT Austin, Caltech, and Harvard. Served as department chair at UNC-CH and UWash. Elected Fellow of AAAS. Conducted research on DMD for more than 40 years. Pioneered the concept of the dystrophin complex as a scaffold for signaling proteins.

Matt Lumley Advisor and early investor

Physician scientist with nearly 20 years of experience in academic medicine and drug development. Earned his medical degree from Imperial College and PhD from Kings College. Worked at Pfizer, Moderna and Medicxi. Father to Myles, who suffers with DMD.

E

Eitan Charnoff

Igor Zvagelsky

Shelley Golan

Kristina Tabakharniuk

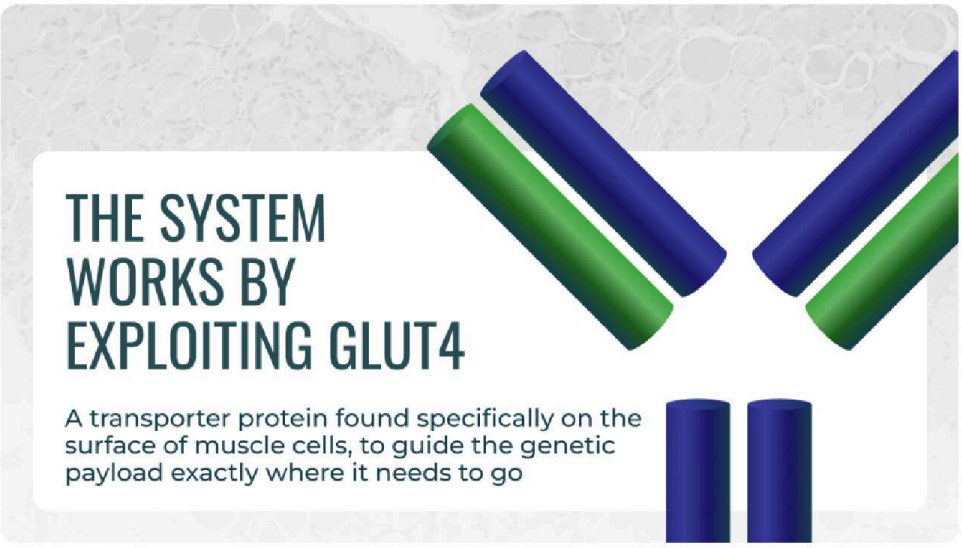
A

Anara B

Breakthrough Non-Viral Gene Therapy Platform for Muscle and Heart Diseases.

ABOUT COMPANY

The Myosana Therapeutics gene therapy platform uses a proprietary non-viral delivery system that transports the full-length dystrophin gene directly into muscle and heart cells via a standard intravenous infusion - no virus is required.



Because no virus is involved, the body does not mount an immune response to the treatment. That means it can be administered again as the patient grows and their condition changes, something no existing gene therapy can offer.

Duchenne Muscular Dystrophy (DMD) is a rare genetic disease caused by mutations in the gene that produces dystrophin, a protein that helps keep muscle cells healthy. Without dystrophin, muscle cells become damaged and progressively weaken over time. As the condition is caused by a mutation on the X chromosome, it primarily affects boys. Boys with DMD lose the ability to walk by their early teens. Their hearts and respiratory systems weaken over time, and most do not survive past their late twenties. A gene therapy for DMD costs $3.2 million per child and delivers only a shortened, partial version of the protein that boys with DMD are missing. Families who manage to access it often see limited improvement, reflecting the limitations of current approaches.

The Myosana Therapeutics gene therapy platform bypasses the constraints of viral delivery. It uses a proprietary non-viral delivery system that transports the full-length dystrophin gene directly into muscle and heart cells via a standard intravenous infusion, no virus required. The system works by exploiting GLUT4, a transporter protein found specifically on the surface of muscle cells, to guide the genetic payload exactly where it needs to go.

The team behind Myosana Therapeutics has extensive experience in

the neuromuscular and cardiac disease space. The CEO and Chief Science Officer invented the GLUT4-based delivery mechanism after more than two decades of DMD muscle research at the University of Sydney and the University of Washington. The Chairman has studied DMD for more than 40 years.

The Chief Medical Advisor brings nearly 20 years of drug development experience at Pfizer and Moderna. His son has Duchenne muscular dystrophy, which is why this work is personal for him.

The leading Muscular Dystrophy Organizations in the world, Parent Project Muscular Dystrophy, the Muscular Dystrophy Association, and CureDuchenne, have each independently reviewed Myosana's Duchenne gene therapy research and invested in the company.

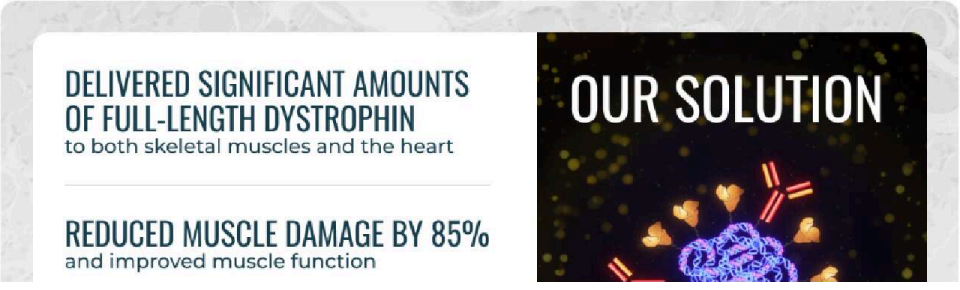
The Problem

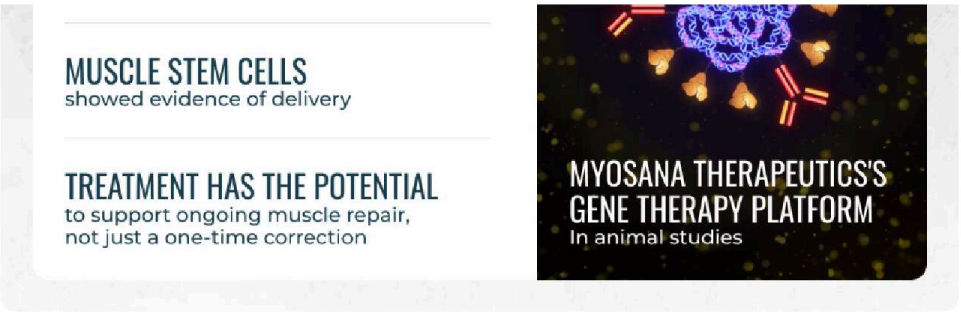
Every muscle and heart cell in the human body contains a protein called dystrophin. It acts as a scaffold for other important proteins that regulate critical functions of the muscle. Boys with Duchenne Muscular Dystrophy produce none of it. Without dystrophin, muscles break down, scar, and progressively fail, starting with the legs, then the arms, and finally the heart.. By age 12, most boys with DMD can no longer walk. By their late twenties, most are gone.

The core problem with every viral gene therapy developed so far comes down to a packaging constraint. The viral capsules used to carry genetic material into cells, known as AAV vectors (adeno-associated viruses, a type of engineered viral shell used to deliver genetic material) , have a fixed size limit. The dystrophin gene is far too large to fit. So every viral approach delivers a cut-down fragment, not the real thing. The field has spent years trying to optimize a compromised AAV solution rather than solving the underlying problem.

The Solution

The Myosana Therapeutics platform removes the packaging constraint entirely. Because it uses no virus, there is no size limit on what it can carry. The full-length dystrophin gene, the complete, natural version, travels through the bloodstream and enters muscle cells directly, guided by antibodies that target a transporter called GLUT4, which sits specifically on the surface of skeletal and cardiac muscle cells. The treatment goes where it is needed. It does not require the immune suppression protocols that viral therapies demand. And because no viral capsid is involved, the immune system is far less likely to attack it, which means the treatment can be administered multiple times as the patient grows and their muscles develop.





The result is a therapy designed to be administered repeatedly, at lower doses, with muscle-specific precision.

Three failures define every approach that came before: incomplete protein, one-time delivery, and immune system conflict. The Myosana Therapeutics platform addresses all three.

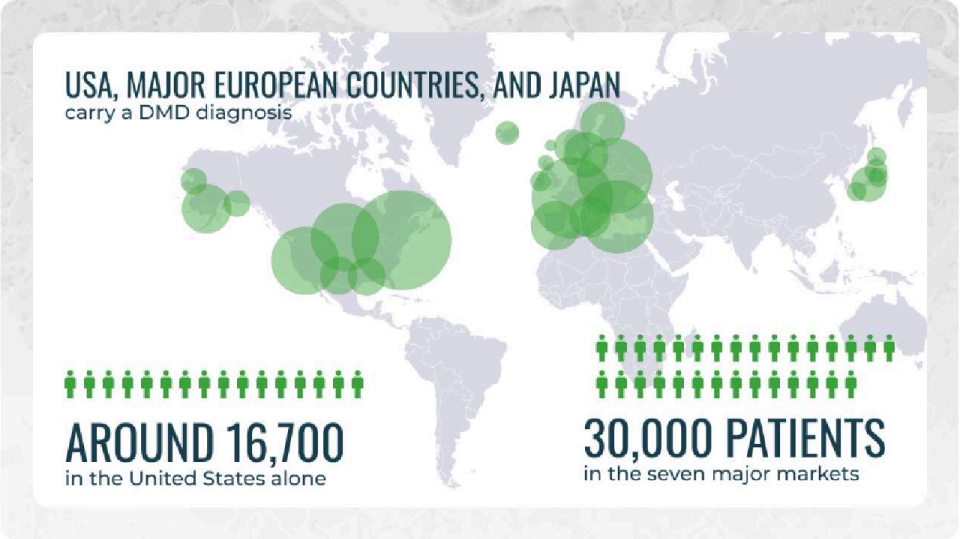
MARKET

Rare diseases are not small markets. When a condition has no adequate treatment and a high unmet need, the economics shift dramatically. Patients and their families will spend extraordinary amounts for something that works. Payers and insurers will reimburse it. Governments will fast-track its approval. That dynamic describes Duchenne Muscular Dystrophy exactly , and it explains why the global DMD drug market, currently valued at roughly $3 to $3.5 billion, is forecast to reach between $10 and $12 billion by 2030, growing at a compound annual rate in the high teens to mid-twenties.

Some forecasts push higher. Projections that account for the

adoption of high-priced AAV gene therapies at scale place the market between $18 and $27 billion by 2030. The wide range reflects genuine uncertainty about how quickly effective treatments will reach patients and how aggressively they will be priced, but the direction is not in dispute. Every scenario points to a market that is growing fast, starved of real solutions, and willing to pay for a treatment that is clinically effective.

The patient population that drives this market is relatively small but concentrated in high-income countries with established reimbursement systems.

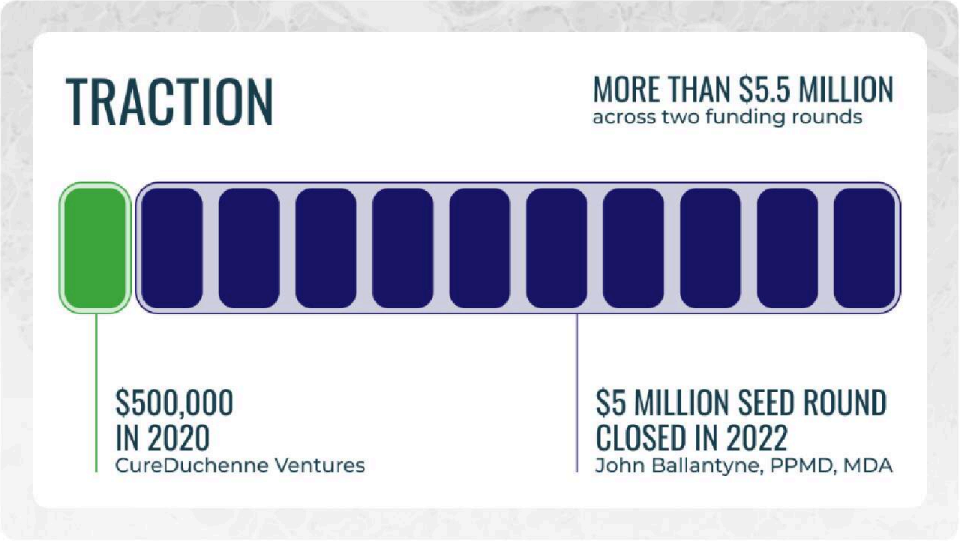


Because DMD affects boys almost exclusively and follows a predictable disease course, the patient population is well characterized and easy to reach clinically. A therapy that works does not need to chase patients. The patients are already identified, diagnosed, and waiting.

The platform's reach extends well beyond DMD. The same GLUT4-based delivery system that targets skeletal and cardiac muscle in DMD patients applies to Limb-Girdle Muscular Dystrophies, X-linked Myotubular Myopathy, and various cardiomyopathies. Each represents an additional patient population with similarly high unmet needs and limited treatment options. Myosana Therapeutics enters the market through its strongest indication and carries the infrastructure to address multiple diseases from the same technology base.

TRACTION

Since 2020 Myosana Therapeutics has raised more than $5.5 million across two funding rounds. The company is currently preclinical and has not generated product revenue.



On the science side, to the best of Myosana Therapeutics' knowledge, the company is the first to successfully deliver full-length dystrophin to both skeletal muscles and the heart of animals via intravenous injection using a single non-viral gene therapy. Animal studies showed an 84% reduction in muscle damage, significantly improved muscle function, and confirmed delivery into muscle stem cells, which are responsible for long-term muscle repair. The team is now finalizing candidate selection ahead of clinical trials.

This Wefunder campaign launched in a soft, invite-only phase before becoming publicly available. In that early phase, the campaign drew over $250,000 in commitments from investors with prior relationships with the company or deep ties to the DMD community. Every one of those early investors knew the science before they wrote the check. The public launch now opens that opportunity to a wider group of investors for the first time.

HOW WE MAKE MONEY

Myosana Therapeutics does not generate revenue today. The company is preclinical, and all capital raised through this campaign goes directly into research and development. The path to revenue in biotech is sequential: clinical results unlock commercial value, and commercial value in rare disease gene therapy has historically been substantial. The existing approved DMD gene therapy is priced at

$3.2 million per patient. That context frames what Myosana Therapeutics is building toward, not what it earns today.

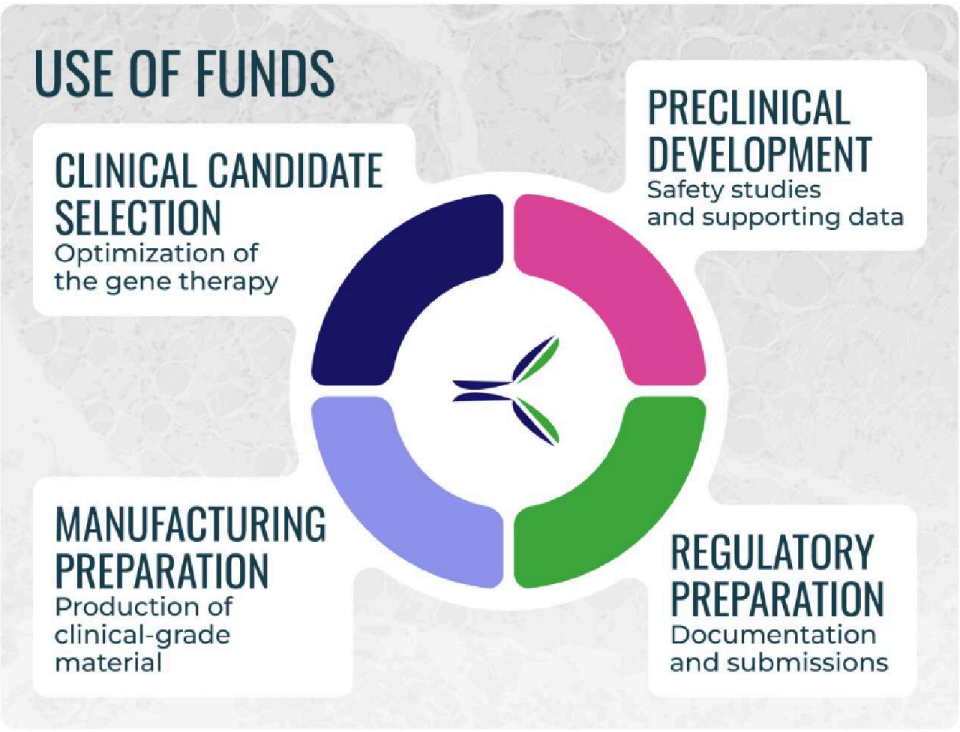
There are two credible routes through which Myosana Therapeutics expects to generate value for investors, and both are consistent with how similar companies in this space have operated. Capital raised supports research and development of the company's gene therapy for Duchenne muscular dystrophy (DMD).

Investing at this stage means accepting that revenue lies several years away, contingent on clinical outcomes that cannot be guaranteed. The opportunity reflects that risk. Early investors in similar rare disease gene therapy companies have seen significant returns when those programs progressed, and significant losses when they did not. This is high-risk, early-stage investing, and no one should invest more than they can afford to lose.

USE OF FUNDS

Myosana Therapeutics is raising between $1.5 and $2 million through this campaign. Every dollar goes into research and development. There are no sales teams to build, no commercial infrastructure to fund, and no overhead expansion at this stage. The work that this capital enables is specific, sequential, and directly

tied to advancing the platform toward human trials.



Forward-looking statements are not guaranteed. Investing in early-stage companies involves significant risk, including the potential loss of your entire investment.

The science behind Myosana Therapeutics has taken more than two decades to develop. The animal data is in. The credibility has been earned. The path to clinical trials is mapped. What stands between this platform and the first human study is capital, and this campaign is how that gap closes.

This offering is made under Regulation CF. Investments are speculative, illiquid, and involve a high degree of risk. You should not invest unless you can afford to lose your entire investment. Please

invest unless you can afford to lose your entire investment. Please review all offering materials available on Wefunder before investing in Myosana Therapeutics