



INVEST IN MYOSANA THERAPEUTICS

Novel gene therapy for treatment of Duchenne Muscular Dystrophy and other muscle diseases

myosanatherapeutics.com Seattle, WA 

Highlights

- 1 Three non-profit organizations - PPMD, MDA, and Cure Duchenne - have invested in Myosana
- 2 The Myosana team has a combined experience of over 200 years in treating muscular dystrophy.
- The platform overcomes the limitations of viral vector-delivered gene

- 3 The platform overcomes the limitations of viral vector delivered gene therapy for muscle disease.
- 4 Targeted muscle delivery, large gene capacity, repeat dosing, and immune neutrality made possible.
- 5 Precision targeting: Our delivery hits muscle cells only, avoiding harmful side effects
- 6 \$4+ billion market where current \$3.2M treatment barely works - massive opportunity
- 7 One of the only companies delivering the complete dystrophin protein - not just fragments
- 8 Patients with low amounts of the full dystrophin protein lived up to age 73 vs typical 20s lifespan

Featured Investors



John Ballantyne

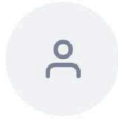
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Invested \$4,050,000 

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

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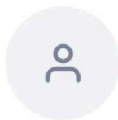
Invested \$850,480 

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

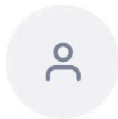
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Invested \$650,000 

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

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Invested \$500,000 

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."

Our 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

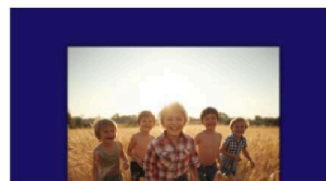
Educated at UT Austin, Caltech, and Harvard. Served as department chair at UNC-CH and UWash. Elected Fellow of AAAS. Conducted research on DMD for more than 40 years. Pioneered the concept of the dystrophin complex as a scaffold for signaling proteins.



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.

A Game-Changing Treatment for Boys with Duchenne Muscular Dystrophy





Every 2.5 Hours, Another Family's World Shatters

"Your son has Duchenne muscular dystrophy."

300,000 families. One devastating reality:

- Boys lose the ability to walk by age 12
- Most don't see their 30th birthday
- Current gene therapy "treatments" cost \$3.2M and barely work

But Everything Is About To Change

WE CRACKED THE CODE EVERYONE ELSE MISSED.

To the best of our knowledge, Myosana is the first and only company to successfully deliver the natural, full-length dystrophin to muscles and the heart of animals via intravenous delivery using a single, non-viral gene therapy.

While others fumble with broken approaches, we've engineered the breakthrough the entire industry has been searching for.

IT'S PERSONAL.

Our Board Member's son has DMD. This isn't just business, it's a father's mission to save his child and 300,000 others.

THIS IS REVOLUTIONARY SCIENCE.

Complete protein delivery. Heart muscle treatment. Stem cell repair. Game-changing results.

This Is The Future. Where boys with DMD don't just survive —they THRIVE. HIGH SCHOOL. COLLEGE. CAREERS. FAMILIES. FULL LIVES.

- **Muscles get weaker over time** because they're missing a crucial protein called dystrophin
- **Boys usually need wheelchairs by age 12** as their leg muscles fail
- **Hearts and lungs weaken** because they're controlled by muscle too
- **Most don't live past their 20s** without treatment

300,000 Families Worldwide Face This Nightmare

Right now, there's only one gene therapy available - it costs **\$3.2 million per child** and barely works. Most families can't afford it, and even those who can see little improvement.



Our Breakthrough: The Missing Piece

Here's the key insight that everyone else missed:

Think of dystrophin like the frame of a house. Current treatments try to fix the house with a tiny, incomplete frame that's missing most of its parts. That's why they don't work well.

We believe Myosana is the ONLY company that can deliver the complete, full-size frame.

What Makes Us Different

High Costs and viral treatments that deliver only partial protein and have severe side-effects. We deliver the protein safely and repeatedly at accessibly affordable pricing.

The Science Made Simple

GLUT4 Targeting



Every muscle cell has special protein "doors" called GLUT4 that only open in muscle cells. We've figured out how to use these doors to deliver our treatment directly where it's needed, like having a key that only opens muscle cell doors.

Why This Matters:

- Gets treatment exactly where it needs to go
- Avoids affecting other parts of the body
- Uses lower doses because we're not wasting medicine
- Much safer approach

Proof It Works: The Evidence

This platform is designed to treat Duchenne muscular dystrophy (DMD) by delivering the full version of the dystrophin gene directly into muscles, including the heart, without using viruses. It uses special antibodies to target muscle cells, where it restored dystrophin in DMD mice, **reduced muscle damage by 85%, and improved muscle strength**. Unlike other approaches, it also works in muscle stem cells, which means it can support long-term muscle repair. The team is now close to selecting the final version for human trials.

Our Laboratory Breakthrough

To the best of our knowledge, Myosana is the first and only company to successfully deliver the **natural, full-length dystrophin** to muscles and the heart of animals via intravenous delivery using a **single, non-viral gene therapy**. We also have evidence of delivery of full-length dystrophin to muscle satellite (stem) cells.

The Team: Personal Mission

Why We Believe We Can Win

Dr. Nick Whitehead (CEO and CSO) - 20+ years of muscle research.
Discovered how to use GLUT4 as our delivery system.

Dr. Stanley Froehner (Chairman of the Board) - Has studied this disease for over 20 years. Attends every major DMD family conference because he's committed to the community.

Dr. Matt Lumley (Chief Medical Advisor) - Former Moderna and Pfizer executive. His own son has DMD. This isn't just business, it's personal.

Trusted by the Community

The biggest DMD organizations have invested in us:

- Parent Project Muscular Dystrophy (PPMD)
- CureDuchenne
- Muscular Dystrophy Association (MDA)

These organizations evaluate dozens of potential treatments. They chose us.

The Market: Massive Opportunity

Size of the Opportunity

- 300,000+ boys worldwide need treatment
- \$4+ billion market for effective DMD therapy

- Current treatment costs \$3.2 million per patient
- Growing market as awareness increases

Why Now is the Perfect Time

1. Current treatment is failing - families are desperate for something better
2. Gene therapy is accepted - doctors and insurers understand the value
3. We have the breakthrough that others missed
4. Experienced team knows how to get treatments approved

Beyond DMD: Platform for Multiple Diseases

Our technology doesn't just work for DMD. The same approach can treat:

- Limb-Girdle Muscular Dystrophies and X-linked Myotubular Myopathy (other muscle diseases)
- Various heart muscle diseases
- Other genetic muscle conditions

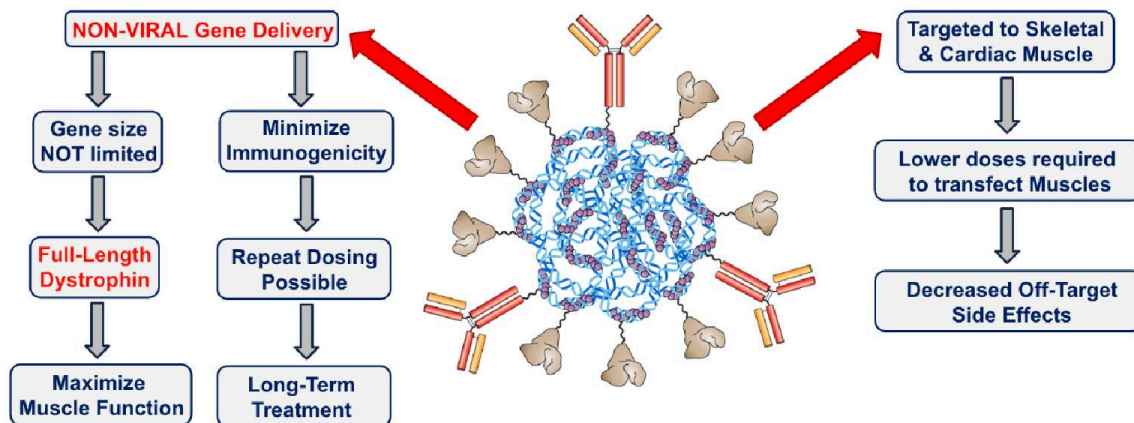
One breakthrough technology = multiple ways to help patients and create value

Investment Opportunity: Be Part Of The Cure

What We're Building

Myosana's development plan spans three phases: **Phase 1 (next 2 years)** will focus on early clinical trials to test safety in boys with DMD and measure protein levels in muscles; **Phase 2 (years 3 and 4)** will expand to larger studies comparing results with current treatments to show improved strength, mobility, and readiness for approval; and **Phase 3 (years 5 and 6)** will complete final approval studies and launch the treatment worldwide, bringing hope to thousands of boys and their families.

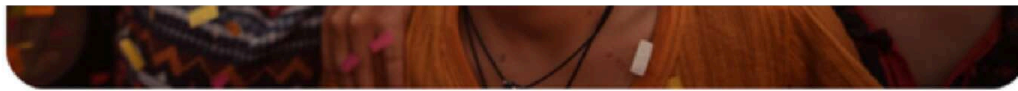
Forward-looking statements are not guaranteed.



Your Investment Impact

Investments of any size make a direct impact: \$100 helps fund lab research and trial preparation, \$1,000 supports clinical trial costs and treatment manufacturing, and \$10,000+ provides major support that accelerates approval, offers potential returns as the company grows, and directly changes the lives of boys with DMD.





Why This Is Huge

Gene therapy and rare disease treatments have historically achieved billions in sales, with first-to-market therapies often dominating their field and commanding premium prices.

Our Advantages:

Myosana stands out by offering a complete solution, backed by an experienced team committed to success, driven by personal motivation, and supported by the Duchenne community from day one.

Change The World

What Success Looks Like

Today: A DMD diagnosis is devastating news. Family life is permanently altered.

With Myosana, Boys With DMD Could:

- Walk into high school and college
- Live independent adult lives
- Have careers and families
- See their 30s, 40s, and beyond

You're Investing In:

- Saving boys' lives
- Ending family heartbreak
- Revolutionary science
- A team that won't give up
- Technology that can help multiple diseases

Ready To Join Us?

What happens next:

1. Small investments help fund research and trials
2. Clinical trials prove the treatment works
3. FDA approval makes treatment available

4. **Global launch helps families worldwide**
5. **Platform expansion addresses other diseases**
6. **Investors benefit from breakthrough success**

Every investment, large or small, brings us closer to the day when no family has to hear "your son has DMD and there's nothing we can do."

Frequently Asked Questions

Q: How do I know this will work?

A: There is scientific literature (not published by Mysosana) for DMD patients supporting that even tiny amounts of the full protein can dramatically improve patient outcomes. Additionally, we have our own unpublished data in animal studies supporting this.

Q: What if other companies develop something similar?

A: We have a 20+ year head start in understanding this disease, plus patent protection on our approach. Other companies are still stuck trying to fix the broken virus approach.

Q: When will patients get treated?

A: Clinical trials are planned to start soon. If successful, treatment could be available in 5 to 6 years - much faster than starting from scratch.

Q: What's my potential return?

A: Gene therapy companies often sell for billions. Rare disease treatments can generate massive returns. But remember - this is high-risk, high-reward investing. Returns are not guaranteed and never invest more than you can afford to lose.

Q: Is this safe?

A: Our approach is potentially much safer than virus-based treatments because we avoid immune reactions and can use lower doses with precise targeting.

Q: Can I invest a small amount?

A: Yes! We believe everyone should have the chance to be part of

breakthrough treatments. Small investments add up and every dollar helps.

TAKE ACTION TODAY

Ready to be part of the solution?

Remember: Behind every investment is a boy like Matt's son, and a family hoping for a miracle.

Your investment could be the difference between despair and hope.

Between a shortened life in a wheelchair and a whole life of possibilities.

Between "there's nothing we can do" and "we have a cure."

This is not just an investment opportunity. This is your chance to change the world.