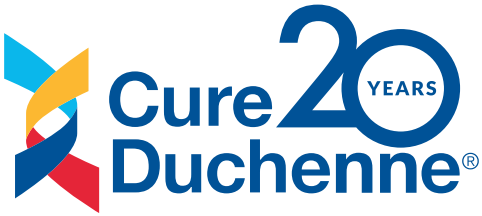
Cure
20
YEARS
Duchenne®

Our vision is our name:
TO CURE DUCHENNE

A Message From Our Founders

We are immensely grateful for your support of CureDuchenne as we celebrate our 20th anniversary. Your generosity has had a tangible impact on the lives of boys and young men affected by Duchenne muscular dystrophy, adding years and improving their quality of life.

More than two decades ago, when our son Hawken received his Duchenne diagnosis, the future seemed bleak. In response to this unacceptable prognosis, we established CureDuchenne with a clear mission: to find a cure and take research from the lab to human clinical trials. With your support, we have made remarkable progress in uncovering promising scientific advancements and improving resources for families and caregivers.

We are proud of our early funding of the company that is now Sarepta, who has developed three FDA-approved exon-skipping drugs for Duchenne, as well as a recently approved gene therapy drug **(page 12).** This recent approval was momentous, opening the door for more research and better treatments. Additionally, this year, we have funded new early-stage research with investments in Locanabio, Insmed, hC Bioscience, MyoGeneBio, and a collaborative research grant to the University of Florida **(page 9).**

Together, we have brought 18 potential treatments to human clinical trials. However, the clock is ticking for those who need treatments now, and your support is crucial in our quest for a cure.

While we advance a cure, we are committed to improving care for all, including underserved individuals with Duchenne muscular dystrophy. We are delighted to share the opening of the first CureDuchenne Clinic in Greater Dallas, serving uninsured and underinsured children and adults with Duchenne, in both Spanish and English **(page 17).**

Thanks again for your support. YOU are driving transformative research and support programs that offer hope to thousands worldwide.

Together, we are counting down to a cure. It's within our reach.

Debra and Paul Miller

Jamie Gullikson; Summer Yates; Vinh Ha; Ryan T. Meardon; Lianna Orlando, PhD; Ryan Esquibel; Okairy Townsend; Andrea Pike; Kathlene Balisy; Bailey Anderson; Chelsea Gillen; Kerry Johnson; Savannah Schmier; Kelly Crow; Ashley Glockner; Emilie Perkins; Karadee Chamley; Jennifer Wallace Valdes, PT; Debra Miller; Paul Miller; Hawken Miller; Monica Utley; Erica Rudoff; Jordan Lynch; Sheryl Marrazzo; Po-Ting Liu, PhD; Heather Medlin; Michael Kelly, PhD; Jessica Hakim; Leslie Porter; Doug Levine, PT

Not pictured: Laura Hameed; Lexi Mathes; Kristen Morris

2022-2023
CureDuchenne Staff

2022-2023
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Ryan Williams
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Elaine Wu
Chief Operations Officer of WHGC, PLC

Our Mission: We are committed to improving the lives of everyone affected by Duchenne through accelerating research to find the cure, improving care and empowering the Duchenne community.

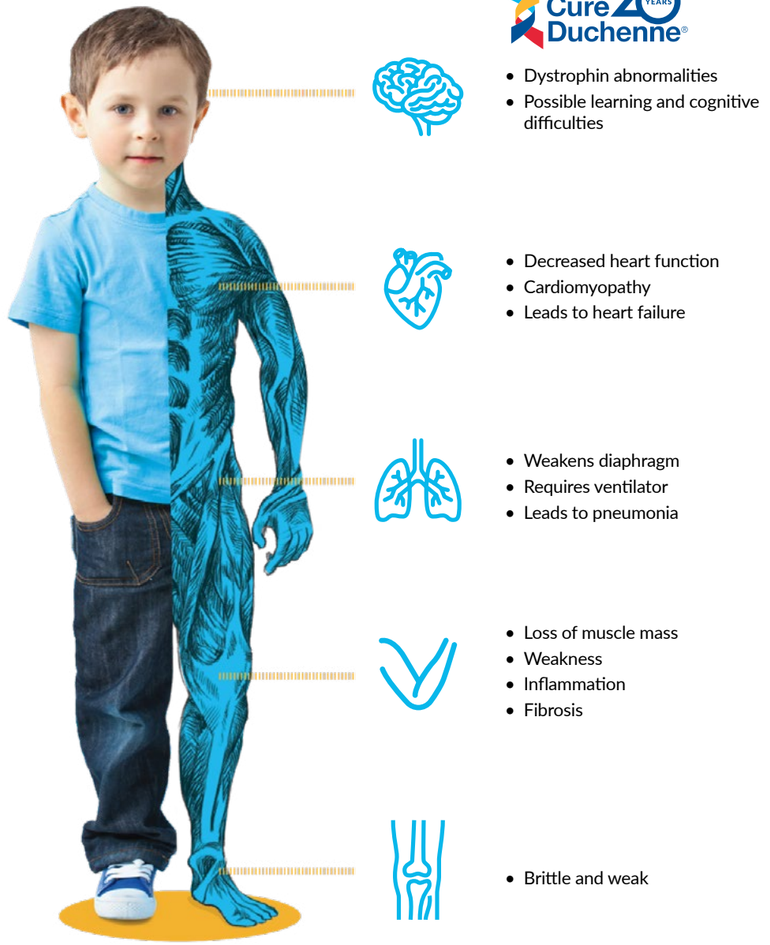
Understanding Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a degenerative neuromuscular disease that is 100% fatal. It is the most common and severe form of muscular dystrophy, affecting roughly **1 in 5,000 male births.**

Duchenne is caused by mutations in the gene that encodes dystrophin, a key muscle protein that plays a critical role in maintaining muscle health. Without dystrophin, cells become damaged and die easily, resulting in heart and breathing failure.

Children with Duchenne have significant delays reaching early development milestones, and **most will require the use of a wheelchair by ages 10–12.** In late teens, their heart and breathing muscles are severely impacted, and many Duchenne patients do not survive beyond their late 20s. The disorder knows no cultural, economic, or social boundaries.

The Impact of Duchenne on the Body



Accelerating the CURE

Venture Philanthropy: A Model of Innovation

CureDuchenne Ventures enables research and development of new therapies in Duchenne by identifying the most promising opportunities and lending support, both via funding and strategic guidance. Our investment approach allows us to reinvest the profits from successful ventures into the creation of new drug development opportunities. Our robust portfolio of companies and projects spans a diverse array of cutting-edge technologies aimed at targeting the most pressing needs in Duchenne.

The Team

Michael G. Kelly, PhD
Chief Scientific Officer

Lianna R. Orlando, PhD
Vice President of Research

Po-Ting Liu, PhD
Ventures Associate

Multiple Therapies to Combat a Multifaceted Disease

CureDuchenne's 2023 investments reflect our commitment to support and invest in a diverse array of promising research to bring solutions to everyone living with Duchenne.

CureDuchenne has made several key investments in 2023, including:

- **hC Bioscience:** Developing a t-RNA-based therapeutic to target premature stop codon mutations, and allow for restoration of full-length dystrophin
- **Insmed Incorporated:** Next-generation gene therapy with a targeted delivery approach designed with the potential to address some of the current limitations of gene therapy, at notably lower doses than other programs. The clinical trial is likely to start later this year.
- **Locanabio:** AAV-delivered snRNAs to induce high levels of exon skipping and restore dystrophin expression in people living with DMD.
- **MyoGene Bio:** AAV-delivered CRISPR/Cas9 gene editing approach that targets a common hot-spot of gene mutations and would be applicable to up to 50 percent of all DMD patients.
- **Collaborative Research Grant for DMD:** CureDuchenne, Muscular Dystrophy Association and Parent Project Muscular Dystrophy announced a collaborative project to focus on overcoming pre-existing antibodies to AAV, which currently limits who can receive gene therapy in Duchenne muscular dystrophy and other diseases. If successful, this approach might also be used in conjunction with other agents to allow for redosing of gene therapies.

> Our singular focus at Insmed has always been on patients—developing and delivering therapies that will make a transformational impact on their lives, regardless of modality or therapeutic area.
>
> **—WILL LEWIS,** *Chair and Chief Executive Officer of Insmed*

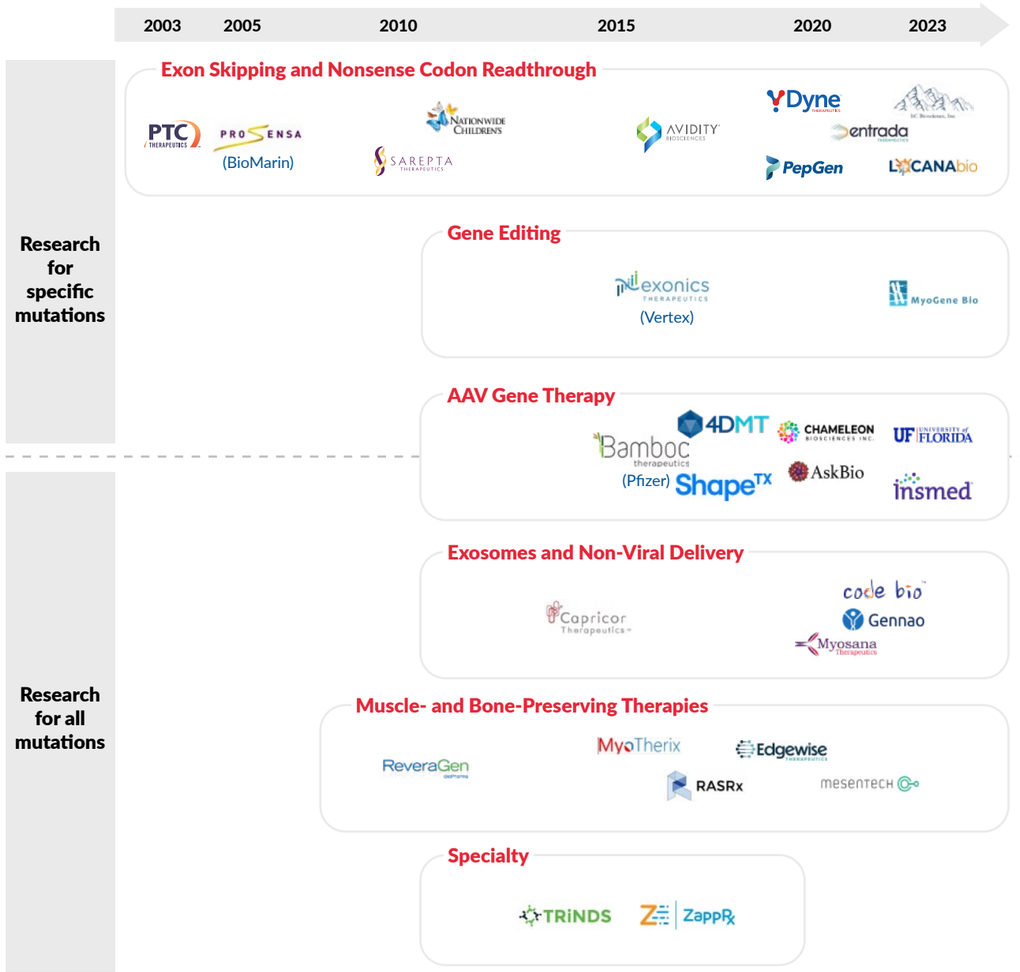
Leading the Charge: Our Investments Drive Credibility and Investor Interest

CureDuchenne has invested more than $26M in research through grants and investments. Of that $26M, $22.2M in biotech investments has led to follow-on funding of more than $3B from other investors and companies to advance these programs further. CureDuchenne has a track record of successful investments, and its "stamp of approval" attracts future investments in these projects from VC firms, biotech companies, and pharmaceutical Companies.

Celebrating CureDuchenne's 20th anniversary, here are some key accomplishments:

- Contributed early funding to the first FDA approved Duchenne drug
- Funded 49 research projects, including investments in 29 companies
- Funded 18 research projects that have advanced to human clinical trials, with more starting soon
- Invested more than $26M toward research and research-related activities, including a biobank and newborn-screening program
- Leveraged more than $3B in follow-on investments from venture capital, biotech, and pharmaceutical companies to advance Duchenne therapies
- Reinvested 100% of investment returns into research, resources, and support for families living with Duchenne

CureDuchenne's Investment Timeline



Early CureDuchenne Research Funding Drives Progress

Use this QR code to watch a video about the Elevidys approval.

> The recent approval of (Elevydis) is an extraordinary moment. For me personally, and for all of my colleagues at Sarepta, it's not only an extraordinary achievement but frankly it's a humbling moment. I really thank CureDuchenne for fighting for patients that have Duchenne.
>
> **—DOUG INGRAM,** *President and CEO, Sarepta Therapeutics Inc*

FDA Approves First Gene Therapy for Duchenne

Sarepta Therapeutics' gene therapy for Duchenne, Elevidys, was recently granted Accelerated Approval by the FDA for individuals with Duchenne ages 4-5. It's the first approved gene therapy for Duchenne, marking an important milestone that keeps us on a path toward identifying truly transformative treatments for all those living with Duchenne muscular dystrophy.

Aligned with our mission of driving early and critical Duchenne research, CureDuchenne is proud to have provided early funding to the company which later became Sarepta, helping them move forward and become the company they are today, with three FDA approved exon-skipping drugs for Duchenne and now an approved gene therapy drug for Duchenne.

While this marks important progress, we recognize this is just a first step, and our work is far from done. There are still many individuals waiting for treatments, and we will continue to support and invest in promising research to bring solutions to everyone in need.

Hudson, age 5, was one of the first individuals in the country to be dosed with Elevidys at the CureDuchenne Clinic. The Clinic provides services to families with Duchenne.
Read more pg 17.

> Unlike some medicines, which just treat symptoms, gene therapy aims to correct the root cause of the disease. This isn't just an advance for Duchenne, it's in advance in all of genetic medicine.
>
> **—LIANNA ORLANDO, PHD,** *Vice President of Research & Partner, CureDuchenne Ventures*

Empowering Research with CureDuchenne Link

CureDuchenne Link is a data-integrated biobank- open to any mutation, age or mobility status - can give numerous scientists the keys to unlocking new treatments.

CureDuchenne Link facilitates critical data sharing to advance our understanding of Duchenne and Becker muscular dystrophy to accelerate research for a cure. We gather quality data and biosamples from as large and diverse a population as possible to create one accessible resource for researchers.

"Our partnership will give members of the muscular dystrophy community the peace of mind that they can easily access and share their complete medical record with their entire care team while helping advance research to facilitate the next generation of therapies for muscular dystrophy."

—Noga Leviner, PicnicHealth founder and CEO

CureDuchenne Link has made significant advancements this year:

- Completed initial research pilots with pharma and biotech partners
- Established partnership with The Akari Foundation to provide full access to CureDuchenne Link in Spanish
- Launched partnership with PicnicHealth to bolster real-world evidence-generation capabilities to accelerate research. The collaboration offers participating individuals a comprehensive portal to access their medical records, streamlining the process of managing their care.
- Activated research sites to ease burden for participants

Championing Newborn Screening: Leading Efforts Across States

CureDuchenne supports a Newborn Screening program for Duchenne at Brigham and Women's Hospital in Boston, MA, led by Dr. Richard Parad. Data from our program was included in a submission to add Duchenne to the Recommended Uniform Screening Panel (RUSP). Although it wasn't initially approved, the Secretary's Advisory Committee on Heritable Disorders in Newborns and Children has since advanced the nomination to the Evidence-based Review stage, a crucial phase in which the committee delves deeper into the nomination to ensure its readiness for a recommendation to the RUSP. While this is in process, we are actively supporting other states across the country considering Duchenne newborn screening.

Redefining CARE

Delivering Unparalleled Care: Setting a New Standard of Excellence

We serve families throughout the Duchenne journey by providing dependable resources and expert support.

The CureDuchenne Clinic Opens in Dallas, Texas

Providing Specialized Care to All

The CureDuchenne Clinic at the Neurology & Neuromuscular Care Center, led by Dr. Diana Castro provides high-quality care for patients living with Duchenne or Becker muscular dystrophy from childhood through adulthood and never deny care due to lack of insurance. Services include neurology, cardiology, pulmonology, nutrition, physical therapy, durable medical equipment, diagnostics, and chest radiography for both pediatric and adult Duchenne and Becker muscular dystrophy patients. The clinic provides care and resources in both Spanish and English, for insured and uninsured patients.

Texas has the highest percentage of uninsured residents in the nation, with Dallas being the second least-insured large city in the country. Large racial disparities also exist in insurance rates with Hispanic individuals being uninsured at a rate of nearly 38 percent, nearly double the state average.

> The CureDuchenne Clinic will bring compassionate care to everyone with Duchenne and Becker and would not be possible without CureDuchenne, who is not only focused on accelerating a cure, but also improving the health and wellbeing of patients living with Duchenne. **—DR. DIANA CASTRO**

Pioneers in Professional Training

The CureDuchenne Physical Therapy and professional training program has added Occupational Therapy Certification to its offerings. Our programs are the first of their kind uniquely designed to empower individuals living with Duchenne by prolonging overall independence including ambulation, and delay disease progression. It's also the only program that offers certification for treating people with Duchenne.

CureDuchenne now supports the ongoing education of over 100 Certified Physical and Occupational Therapists across the country with the first and only PT and OT certification program for Duchenne. Healthcare professionals and families affected by Duchenne. Courses have also been taught internationally in countries from Hungary to India. Grant-awarded funds are being used currently to develop more online educational materials for therapists and families affected by Duchenne around the world.

Therapy at a Glance

119

physical and occupational therapists educated in 2022–2023

1,601

course attendees since inception

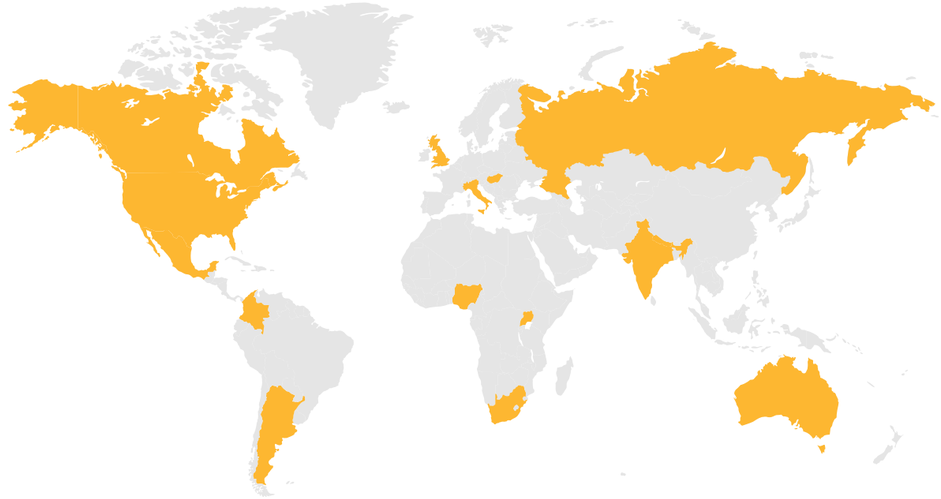
The Team

Doug Levine, PT
Physical Therapist

Jennifer Wallace, PT
Physical Therapist

Our Global Reach

Our team trains allied healthcare professionals around the world, helping people maintain mobility, flexibility, and quality of life at all ages and stages of their journey with Duchenne. Below is a list of countries we have provided training in:



Argentina, Australia, Canada, Colombia, England, Hungary, India, Italy, Mexico, Nepal, Nigeria, Russia, South Africa, Uganda, United States

> The CureDuchenne Cares program is one of the most impactful courses I've ever taken. This program has empowered me to become the best physical therapist that my Duchenne patients and their families need.
>
> **—DANI TREES, PT, TEXAS**

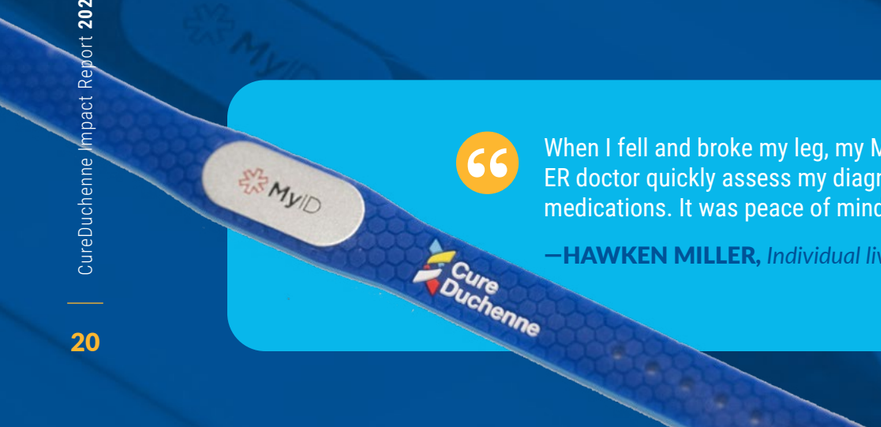
> The CureDuchenne Certified Physical Therapy program was fundamental in empowering me with knowledge and confidence to be the founding physical therapist in our multi-disciplinary DMD clinic at the Vanderbilt Children's Hospital... I am very grateful for the program and the role it has served in my growth as a DMD specialist and the quality of care I can provide to my patients.
>
> **—KARA ARPS, PT, DPT, PCS, ATP**

Celebrating 20 Years of Empowering Families

CureDuchenne Cares, an interactive education and outreach program, continues to be a beacon of support for parents, caregivers, physical therapists, and allied health professionals dedicated to serving patients living with Duchenne. For two decades, we have been fulfilling the unmet need for comprehensive information, resources, and best practices in managing the challenges of Duchenne, leading to enhanced quality of life for patients.

CureDuchenne provides resources to families through:

- Caregiver Dinner Sessions: Intimate dinner events for caregivers to provide connectivity in local communities
- Workshops: Full-day educational workshops covering the latest in care and research
- CureDuchenne 1:1: Individualized, unbiased information and advice from experts
- Medical ID Bracelets: Wristband that communicates important information about Duchenne to EMS professionals in urgent medical situations
- CureDuchenne Advocacy: Champion causes and influence policies by sponsoring legislation and educating key decision-makers
- Virtual Events: Educational webinars and social events that reach families around the globe
- Clinic Busy Bags: Information and resources for families as well as engaging activities for kids to keep them engaged for long hours at clinics



> When I fell and broke my leg, my Medical ID Bracelet helped the ER doctor quickly assess my diagnosis and administer the right medications. It was peace of mind for us in a stressful time.
>
> —**HAWKEN MILLER,** *Individual living with Duchenne*

Family Fridays

Engaging the Duchenne Community

New in 2023, CureDuchenne's "Family Friday Blogs" have been a pivotal resource to empower and support the Duchenne community. As families and caregivers navigate the multifaceted challenges of Duchenne muscular dystrophy, these articles provide invaluable information, expert insights, and a supportive community.

Key Impact Metrics:

- **Audience Engagement:** 5,550 family members receive our weekly emails, creating a vibrant community dedicated to shared experiences and information.
- **Quality Content:** Through collaboration with industry leaders and specialists, our blogs are trusted source for credible insights and advice.
- **Community Building:** More than just a source of information, our blogs foster spirit of camaraderie and mutual support within the Duchenne community.

Building COMMUNITY

CureDuchenne brings the Duchenne community together, empowering them to live joyful lives while we accelerate treatments and enhance care.

Our conferences, workshops, dinner session and events, create a supportive network, fueling hope and progress. CureDuchenne is making a difference in the lives of those affected by Duchenne. Together, we strive for a brighter future and a cure.

> “When I’m here, with my community, I have a strong belief that things will be better for my son.”
>
> **—CLINT PORTER,** *Father of child living with Duchenne*

Community

2023 Futures National Conference

The CureDuchenne FUTURES National Conference is an annual event focused on bringing education, connection, and hope to the entire Duchenne community. This year's theme was BRIGHT FUTURES, placing importance on the acceleration of critical research, improvement of care and quality of life for all, and fostering a community that supports one another and takes on the future together.

860

Total attendees
(587 in-person, 273 virtual)

56

Total families that received travel assistance
(85 children, 107 adults)

"FUTURES is an amazing conference. You learn a lot on what's new in the Duchenne community, from equipment to drugs to exercises. I always leave FUTURES inspired and wanting to get more involved in the Duchenne community." **—ADRIAN,** *age 18 living with Duchenne*

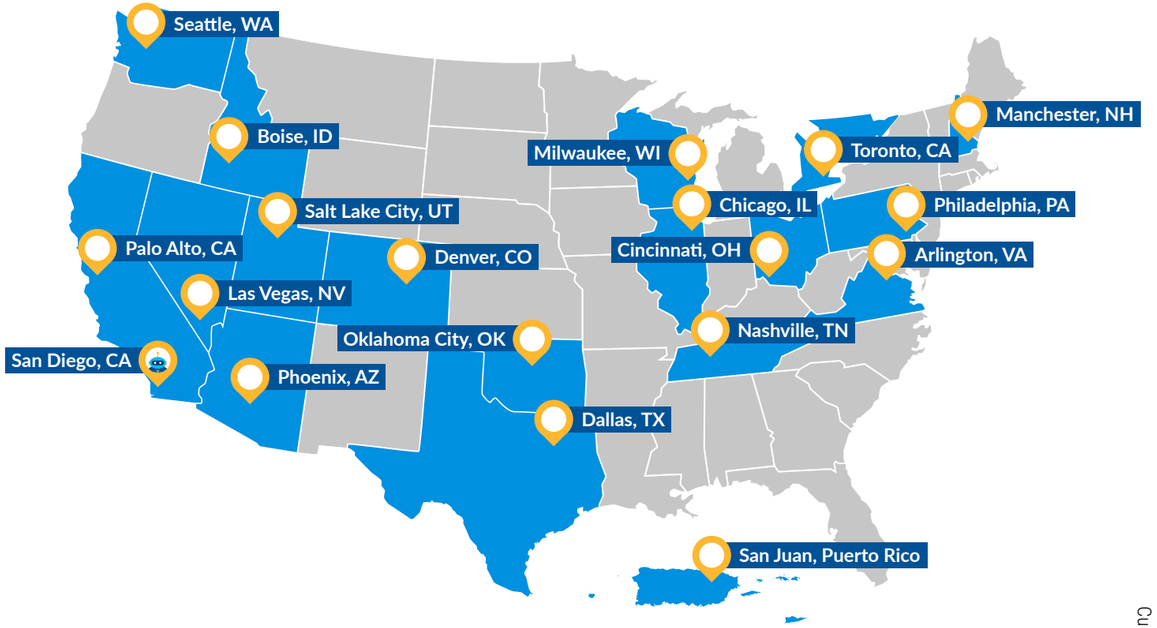
Family Workshops and Dinner Sessions

Our community programs raised awareness, shared information, and provided invaluable educational resources to various stakeholders across the Duchenne community.

Eight workshops and 10 sessions took place across the US, hosting families, healthcare providers, industry partners, advocates, equipment vendors and local community resources.

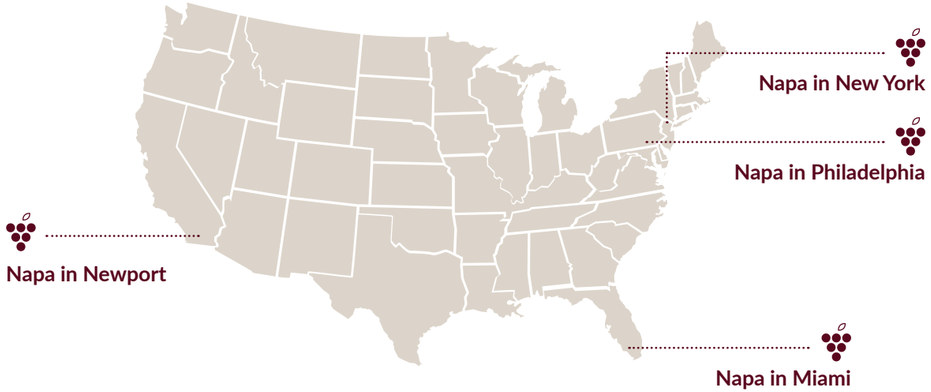
In 2023, **more than 200 family members** have attended workshops and dinner sessions.

2023 CureDuchenne Workshops and Dinner Sessions



The Napa Wine Series

Napa Wine Series events are held throughout the US to raise funds for CureDuchenne. The events feature grand tastings with Napa's best wineries and vintner-hosted dinners curated by celebrity chefs. First held in 2015, Napa in Newport quickly became a signature event in Orange County. The concept then expanded to cities across the country, with wine enthusiasts enjoying the best of Napa Valley while supporting a worthy cause.



> It's an incredible night with impeccable wine and an exquisite meal from a Michelin-starred chef, but most importantly, it was for a wonderful cause. Every year, I look forward to this opportunity to make a positive impact and move research forward for people who need a cure for Duchenne now – and I am so grateful to everyone who came out to support us.
>
> **—SARAH MCELROY,** *CureDuchenne Board Member and Napa In Newport Committee Member*

$12.8m

Total raised to date for CureDuchenne through Napa Wine Series events.

Driving a Cure at the 12th Annual Getzlaf Golf Shootout

The Getzlaf Golf Shootout stands as a shining example of unwavering support from the community, bringing together elite athletes, influential figures, and community leaders who have come together to champion the cause of CureDuchenne. Hosted by NHL All-Star and retired Anaheim Ducks captain Ryan Getzlaf and his wife Paige, the event has raised millions to fund early-stage research and bring new therapies for Duchenne muscular dystrophy.

Held at the picturesque Monarch Beach Golf Links in Dana Point, California, this year's event was truly memorable. A heartfelt thank you to every participant, family and sponsor.

$5.8m

Total raised to support CureDuchenne's mission since inception.

> We are truly humbled by the generosity we receive year after year and the impact we're able to make on the Duchenne community. We are closer than ever before to a cure for this devastating disease and the support of the community means more now than ever before. Together, we will find a cure. **—RYAN GETZLAF,** *Retired Anaheim Ducks Captain, NHL All-Star and 2007 Stanley Cup Champion*

Champions Drive Progress

CureDuchenne Champions and community partners host a variety of fundraising events, big and small, across the country to raise funds for CureDuchenne's mission. Highlighted below are a few events from 2023.

Champions Ladies Luncheon

Austin, TX

This year's event brought some of the most influential women in Austin together to experience a luxurious "Afternoon in Palm Beach" complete with fine dining, inspiring speakers, raffles, networking opportunities, and a fall fashion show preview presented by Estilo. The event was started by Austin residents Tim and Laura Revell and hosted by Venus Strawn and Jennifer Stevens.

Total Raised in 2023: Over $68,000

14th Annual Champions to CureDuchenne Gala

Austin, TX

Champions to CureDuchenne is an annual fundraising gala hosted by the Revell family, whose two sons live with Duchenne, that brings together Austin's finest for a spectacular evening under the stars. The event features gourmet food, cocktails, casino games, and live and silent auctions for a night of fun and purpose.

Total Raised in 2023: Over $310,000

Cali-RAD Festival (Reggae Against Duchenne)

San Diego, CA

Christian Hogan and his 22-year-old son Micah, who has Duchenne, created this event to combine their shared love of reggae with their desire to raise funds to accelerate research toward a cure for Duchenne. This year, an unforgettable lineup with reggae artists performed at the iconic Beach House in San Diego's Mission Beach.

Total Raised in 2023: Over $10,000

Dealing for Duchenne San Antonio

San Antonio, TX

Dealing for Duchenne San Antonio is a "Night of Hope" that connects the San Antonio community to a global network of families, caretakers, doctors, researchers and philanthropic-minded people, all while enjoying an evening filled with casino games, food, drinks & music. This annual event is organized by the Munoz family, whose son Joshua was diagnosed with Duchenne at age 5.

Total Raised in 2023: Over $90,000

Calves to Cure
Billings, MT

Paul and Laura Heaton, whose son Grant lives with Duchenne, lead this yearly cattle sale that takes place in Billings, MT. The event generates funds through contributions from livestock sales and a raffle.

Total Raised in 2023: Over $28,000

Dealing for Duchenne Philadelphia
Philadelphia, PA

This event, with a speakeasy theme, featured high-stakes casino games, signature cocktails, and delectable cuisine. The event was hosted by the Gambhir and Lehr families, both of whose lives have been profoundly impacted by Duchenne.

Total raised in 2023: Over $320,000

Beneficiary Events

Stars and Stripes
June 22-25, Cabo San Lucas, MX

The Stars & Stripes Tournament is an established golf tournament, fishing tournament and music festival. CureDuchenne is proud to be a beneficiary of this world-class charity fundraiser, held yearly in Cabo San Lucas, Mexico.

Total raised in 2023: Over $5M for all beneficiaries

MJ&M
April 27-28, Austin, TX

Mack, Jack & McConaughey (MJ&M) is the annual joint fundraising effort of Academy Award-winning actor Matthew McConaughey, ACM Award-winning recording artist Jack Ingram, and coaching legend Mack Brown. MJ&M's goal is to empower kids.

Total raised in 2023: Over $11M for all beneficiaries

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