

**Parent
Project
Muscular
Dystrophy**

JOIN THE FIGHT.

END DUCHENNE.

ENDDUCHENNE.ORG

#ENDDUCHENNE

RESEARCH



\$50 million+

invested by PPMD into Duchenne research & therapy development to date



Over 260 research grants

awarded, supporting nearly every major therapeutic approach



~ 35 clinical trials

in Duchenne at any given time



2 US drug approvals

with additional promising therapies in development

ADVOCACY



4 bills

signed into law, reshaping the Duchenne landscape



12000 messages & action alerts

sent to Members of Congress



Over \$600 million

in federal funding leveraged by PPMD community into research



More than 2000 meetings

face-to-face with Members of Congress

CARE



10 years

added to average lifespan due to PPMD-led advances in care



25 clinics

awarded certification by PPMD across the US as of June 2019



700 care providers

in PPMD network of certified clinics



20 years

of identifying & addressing gaps in care through specialty workshops & consensus meetings

ENGAGEMENT



26 PPMD Connect locations

across the United States, providing local families outreach & mentoring



Over 5000 patients & carriers

across the globe have registered in The Duchenne Registry since launch



\$36 million+

raised through Race to End Duchenne & family-led grassroots events since 1994



14310 families

reached in person, through 25 Annual Conferences, End Duchenne Tour stops, Roundtable discussions, & Advocacy Conferences

PPMD: YOUR STRENGTH, YOUR FAMILY

I had no intention of being part of the Duchenne community this long.

When we started PPMD in 1994, I naively thought we would have a cure and I would be spending these years with my sons and daughters and a ton of grandkids I would spoil rotten.

And here we are 25 years later. My sons are no longer here, I worry about my daughters being carriers, I worry about my granddaughter being a carrier, and our community has never had to work harder and the losses have never felt more profound.

But, once we dove in and began to understand Duchenne — while simultaneously educating those around us — we understood that progress wasn't going to come in the form of a magic wand or silver bullet. With time, we have had to redefine what progress is. And while it may be too late for my boys and all of the children we have lost along the way, *there is progress*. With every victory, large or small, we honor our brave loved ones.

PPMD started with two basic ideas in mind:

- We must give parents a community to turn to and a voice to advocate for their child's healthcare.
- We must end Duchenne.

Both of these goals have gone through their own redefinition.

More than a Parent Project

When my sons were diagnosed in 1984, I went to the library to find everything I could on Duchenne. This was before the internet. We're talking microfiche and a card catalog! My husband and I are both medically trained — he's a retired family doc and I was in nursing — but Duchenne was something we maybe covered in a half-day course on neurological diseases if that. It was hard to find a community, to build an army to fight this horrible disease. We needed to unite all of the families that, like ours, felt alone.

The Duchenne community has never been stronger. It continues to grow and strengthen and is a force of nature in the international rare disease community. We have become a model for other diseases and a case study in how parent advocacy can unite and get things done.

But we also realized that this isn't just a 'parents' project.' There are many voices joining us in this fight — entire families, whole communities, friends from work, our child's school, not to mention regulatory agencies, pharmaceutical companies, biotechs, universities, and clinics.



PPMD's Pat Furlong with her sons, Chris & Patrick

But perhaps the most important voice we hear today is that of the people living with Duchenne. A voice that for years had been left out of the conversation and today is often leading the narrative.

Parent Project Muscular Dystrophy is a community-wide effort that we strive to lead, incorporating the priorities and passion of every stakeholder.

Redefining an End to Duchenne

To me, success was going to be when we could turn off the lights at PPMD, close the door, and not look back. Because Duchenne would be done. Maybe it would be a vaccination you got as a child, a quick shot to guarantee you don't 'catch' Duchenne, like the Polio vaccine. It would maybe take 5, 10 years at the most because nothing happens quickly with government agencies. That was my plan in 1994.

What we have learned is that Duchenne is complicated. I know, sounds simplistic and it almost feels ridiculous to write. But it's true — if it was easy, PPMD wouldn't be turning 25.

Duchenne is a progressive disorder that needs to be treated with a progressive attack plan. We didn't realize in those early days that it was going to take a combination of therapies to treat Duchenne. We know that now. We also know that the fight won't end when we have treatments. We have to make sure that our loved ones can access these treatments and that insurance companies will cover them.

In my 25 years at PPMD, I realize that this fight is an evolution: as our understanding of Duchenne evolves, so does our strategy to end it.

Pat Furlong, *Founding President & CEO*

EVERY DAY, EVERY ANGLE

PPMD realizes it will take a combination of therapies to treat Duchenne, we realize it will take a combination of approaches to fight Duchenne. We are the only Duchenne-specific nonprofit in the United States that takes a truly comprehensive approach to address the full impact and progression of Duchenne.

We will continue to relentlessly attack Duchenne from every angle, using every weapon in our arsenal — the greatest of which is you.

Research

PPMD has contributed to almost every Duchenne research strategy that is currently in the pipeline, including an initial investment in 1997 in the very first **Duchenne Muscular Dystrophy Research Center (DMDRC)** led by Dr. Eric Hoffman. Since that moment, the Duchenne research community (and rare diseases as a whole) would learn to work together to build ideas, to test theories, and to motivate each other. PPMD brought urgency and a willingness to take chances to the table. We broke down established barriers and pushed boundaries. We still do.

PPMD continues to drive change in the research arena. Our grant cycles offer opportunities to investigators and help us keep our finger on the pulse of research. **PPMD's Scientific Advisory Board** expertly reviews every grant that comes in and offers funding or advice on how to proceed to garner funding in the future. Our collaboration with academic research institutes and industry partners, helps us push potential treatments to clinic faster, which in turn provides access to the Duchenne community and patients faster.

Remaining idle is not an option for PPMD. When we see a gap we fill it. If we see a need, we address it. And where we see the path, we fearlessly and strategically work toward clearing it.

Advocacy

Like the creation of PPMD itself, our Advocacy program evolved from parents navigating their child's diagnosis, frustrated at the limited federal support for research and services. Frustration turned to ambition and we headed to our nation's capital, ready to speak up and demand that the federal government acknowledge the significant impact of muscular dystrophy on our families.

People with Duchenne and their families use their voice to remind policy makers that they are contributing members of society and that Duchenne deserves the same federal priority and investment as any other disease.

As advocates, our most important role is to speak up on behalf of those who can't. We must remind Congress that even when budgets are tight, funding Duchenne research is still a national priority. We must remind regulatory agencies that people with Duchenne and their families are willing to take risks if there is any potential benefit

to come from an experimental treatment. And we must remind all policy makers and regulators that the clock is ticking.

We don't have time for bureaucracy. Our children's lives depend on it.

Care

Perhaps the greatest advance in the fight to end Duchenne over the last several decades has been in the way we care for people diagnosed with Duchenne. In our 25 history, the average lifespan of people with Duchenne has increased from late teens/early-20s to mid-20s/early 30s. This incredible leap is because our community has pushed to advance care for the last quarter of a century.

When PPMD first began, there were no **standards of care**. Doctors and clinicians were left comparing notes, taking chances, and using a "wait and see" approach. Early in our history, PPMD insisted that steroids be considered as an early intervention and labeled as the "gold standard" due to their ability to slow the progression of muscle deterioration.

For us, it's always been about getting you the information you need to demand the care you deserve, from the best clinics available. Care is evolving and PPMD will continue to make sure we stay on top of critical information you need to know, when you need to know it.

Engagement

Receiving the diagnosis of Duchenne is a devastating and isolating moment for a family. It is our goal to provide families with lifelines to the Duchenne community so that no one ever feels alone or uninformed.

To do so, PPMD hosts numerous in-person and virtual events throughout the year, including our **Annual Conference, Annual Advocacy Conference, End Duchenne Tour, and educational webinar series**. These events convene the leading experts in Duchenne research and care who provide updates on the most promising therapies in development and the most critical updates in care.

Participating in **The Duchenne Registry** is one of the most powerful ways you can stay updated on clinical trials and the latest advances in research. Plus, when you join the Registry, you become a citizen scientist by contributing to real scientific research.

Not only do we host impactful in-person conferences and tour stops, we also provide local opportunities for families to gather. Whether through **PPMD's Connect program** — our regional, parent-led outreach program — or at one of **PPMD's Race to End Duchenne events or a grassroots fundraiser**, we are always looking for opportunities to bring families together for face-to-face interaction.

Community. Nothing is more important than knowing you are not alone. Let PPMD help connect you.

YOUR IMPACT: 25 YEARS OF PROGRESS

We invest funds raised as responsibly, quickly and efficiently as possible, to maintain our comprehensive approach. We firmly believe that we must continue to identify gaps and address them in order to attain our goal: to end Duchenne. Without the unwavering commitment and investment of the community, PPMD would not be the Duchenne champion we are today.

87 cents of every dollar raised directly supports PPMD's projects and initiatives.



From personal fundraising sites to six-figure gifts, and everything in between, we know that no one person, company, or organization will end Duchenne. We must work together to continue the fight. Every dollar makes a difference and brings us one-step closer to the end.

We are grateful to our collaborators — individuals, foundations, corporations, organizations, and industry partners — who, by joining forces with PPMD help expand the Duchenne community and make us all stronger.

Thank you for trusting PPMD to be the steward of your funds. Your dollars will become that much more impactful, when combined with others, in a strategic approach to maximize each investment.

The opportunities for partnership are limitless with PPMD, and we welcome your continued support as we lead the fight to end Duchenne.

Individual Donors and Foundation Grants Individual donor and foundation support comes in many forms and is a testament to the generosity and support of the greater community. Whether it is a single donation, sustaining gift, funds raised through a grassroots or race event, or those who have left a legacy gift to commemorate their dedication to PPMD, you enable us to attack Duchenne from every angle.

Corporate Sponsorships Our corporate sponsors are true partners and work closely with us to achieve our mission. Each of PPMD's fundraising programs has corporate sponsorship opportunities that are fully customizable. These companies make an investment in PPMD and receive recognition and customized deliverables in return.

Industry Partners As a result of PPMD's efforts, in recent years we have witnessed a surge in industry investment in Duchenne and stronger collaboration toward drug development and approval. PPMD recognizes the strength and importance of these relationships. Through educational and program grants, industry partners help fund community engagement and outreach initiatives, enabling more families to learn about the Duchenne research landscape, their particular disease mutation, and clinical trial opportunities.

Organizational Partners PPMD maintains formal and informal partnerships with organizations around the nation and globe who share a similar mission, tactics, and ideology for the purposes of cooperation on investment strategy, research, sharing data, and raising awareness for the entire rare disease community.



Your investment empowers our impact and you have our unwavering commitment. Thank you for joining us in the fight to end Duchenne.

To learn more about partnering with Parent Project Muscular Dystrophy, please contact Erin Dresnick, PPMD Development Director at 201-250-8440, ext. 108 or erin@parentprojectmd.org.

25 MOMENTS IN PPMD HISTORY

■ Research
■ Advocacy
■ Care
■ Engagement

1994

PPMD founded and hosts 1st Annual Conference

1997

PPMD invests in first Duchenne Muscular Dystrophy Research Center

1998

Steroids become gold standard of care

2000

PPMD hosts 1st Annual Advocacy Conference

2001

MD-CARE Act signed into law (reauthorized in 2008, amended in 2014)

2003

PPMD funds first Duchenne Drug Discovery Program & begins convening consensus meetings that would cover topics like pulmonary, cardiac, and endocrine/steroid use

2005

PPMD's endurance program, Race to End Duchenne, participates in first race

2005

Provided seed funding for Preclinical Advancing Therapeutics Lab for the exploration and validation of compounds and biologics

2006

PPMD and CDC convene community thought leaders to develop Care Considerations

2007

The Duchenne Registry (aka DuchenneConnect) is born

2009

Care Considerations is published (updated in 2018)

2009

PPMD creates PPMD Connect program (aka FACES) uniting Duchenne families

2011

PPMD launches Cardiac Care Initiative which, to date, has invested over \$5 million

2012

PPMD leads way for FDASIA to be passed by Congress mandating regulatory flexibility for rare diseases

2013

PPMD announces two \$1 million RFAs for exon skipping and late stage preclinical/early stage clinical projects

2013

PPMD leads community in drafting guidance for FDA, presented to FDA in June 2014

2014

PPMD launches Certified Duchenne Care Center Program

2015

PPMD creates and convenes first Duchenne Drug Development Roundtable

2015

PPMD Adult Advisory Committee is formed to help guide the organization's mission

2016

PPMD launches Gene Therapy Initiative which, to date, has invested over \$4 million

2016

FDA approves EXONDYS 51 (eteplirsen) for treatment of Duchenne

2017

FDA approves Emflaza (deflazacort) for treatment of Duchenne

2018

New ICD 10 Code issued for Duchenne/Becker, resulting from PPMD-led evidence review & nomination

2019

PPMD launches Duchenne Newborn Screening in New York state

2019

PPMD announces 25th Certified Duchenne Care Centers and plans to certify adult care centers and mentor global certification program



H. Lee Sweeney, PhD, launches PPMD funded Preclinical Advancing Therapeutics Lab



Connecting Families — PPMD's first Annual Conference in Orlando, Florida 1994



PPMD's first national fundraising campaign — the Convoy of Hope — included families traveling across the country in WW2 vehicles

RESEARCH

For 25 years, Parent Project Muscular Dystrophy has been committed to exploring and supporting every single therapeutic possibility. We take a cutting-edge approach to accelerate finding treatments that will end Duchenne for every single person impacted by the disease. The therapeutic pipeline of potential treatments for Duchenne has never been so full of promise. And it's never been more important to ensure that the path to progress is clear, so that safe and effective therapies can reach the people who need them quickly and affordably.



invested by PPMD
into Duchenne
research & therapy
development to date



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nearly every major
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in Duchenne
at any given time



with additional
promising therapies
in development

DRIVING THERAPIES FORWARD

We take a comprehensive view of the entire landscape to identify opportunities to accelerate the development of therapies and to ensure nothing is missed. Often we start by convening leading experts in the field to advise us on how to develop and test emerging therapies that will help end Duchenne.

We partner with key stakeholders (patients, families, researchers, clinicians, pharmaceutical companies, biotechs, other advocacy organizations, etc.) to execute these strategies and push potential treatments into clinical trials more quickly.

PPMD's Duchenne Drug Development Roundtable incorporates the knowledge and support of large and small pharmaceutical companies and biotechs (our industry partners) with the goal of accelerating the development of meaningful treatments for Duchenne through collaborative efforts on pre-competitive initiatives, thus reducing duplication and gaining resource efficiencies to tackle many of the challenges in drug development. PPMD was the first Duchenne organization to convene this broad representation of industry in 2015.

The Duchenne Registry ensures all research and clinical trial strategies developed for individuals with Duchenne are patient powered and focused. Your anonymous data provides researchers and industry insight into the patient perspective and helps speed the clinical trial process. Launched in 2007, The Duchenne Registry is the single largest Duchenne patient registry in the world.

INVESTING IN INNOVATION

We invest in innovative research to fight the disease, no matter the mutation, age, or stage of progression. Our primary goal is to identify opportunities to accelerate therapies for all. This means keeping our eyes focused on emerging therapies, identifying gaps, and establishing collaborations to drive meaningful therapies that benefit the entire Duchenne population. Not only does PPMD explore and support each of these therapies, we want to ensure that the pathway to approval and the road to access after approval is as easy to navigate as possible.

The Drug Development Pipeline is full of potential treatments that are being tested. These include therapeutic approaches that restore or replace dystrophin and those that treat Duchenne symptoms (such as those that protect muscles by reducing fibrosis and inflammation). The goal? To test combinations of these therapies to create the best “cocktail” for each patient.



**Restoring or
Replacing
Dystrophin**



**Combating
Fibrosis**



**Regulating
Calcium
Balance**



**Reducing
Inflammation**



**Improving
Muscle Growth
& Protection**



**Restoring
Cellular Energy**



**Improving
Heart Function**

OUR ONGOING INVESTMENTS

- **PPMD's Gene Therapy Initiative** is a long-term, multi-million dollar approach that seeks to accelerate the potential of gene therapy as a therapeutic for Duchenne.
- PPMD has been providing support to the **Preclinical Assessment Laboratory** for the last 15 years, serving industry by replicating or confirming results of animal studies, de-risking their entry into clinical trials for Duchenne.
- **PPMD's Cardiac Initiative** is our ongoing commitment to cardiac care, because the heart is a muscle too. Heart issues don't just affect some people with Duchenne—they affect ALL people with Duchenne.
- Because female carriers of the dystrophin gene are at risk for heart disease, PPMD has initiated a **first-of-its-kind carrier study with Nationwide Children's Hospital** to characterize manifesting carriers, women who display varying ranges of Duchenne symptoms as part of our efforts to better understand and support carrier issues.
- PPMD has been an early funder of several robotic programs that strive to improve the quality of life for people with Duchenne. Our **Robotics Initiative** is about building strength in a disease that robs people of the ability to perform everyday tasks that most of us take for granted.

ACCELERATING DRUG DEVELOPMENT

We develop clinical trial methodologies and tools that address current roadblocks in Duchenne drug development. This includes innovative multi-sponsor clinical trial designs that test multiple drugs, in multiple arms at the same time, enabling participation from infants to adults to participate, minimizing exposure to placebo, and establishing success or failure more quickly.

- PPMD is thoughtfully exploring the potential of a **Master Protocol** in Duchenne that could accelerate clinical trials by standardizing and streamlining many processes that are repeated over and over for each trial and maximizing flexible trial design that is disease focused, allowing for most individuals with Duchenne to participate in a trial, decreasing risk of placebo, regardless of age or ambulatory status, broader labeling, and finding answers to whether a drug is working or not more quickly.

To optimize drug development in Duchenne, PPMD participates in two collaborations:

- The goal of the **Duchenne Regulatory Science Consortium (DRSC)** is to develop a regulatory-ready, clinical trial simulation tool to accelerate clinical trials for new drugs to treat Duchenne.
- The goal of the **Collaborative Trajectory Analysis Project (C-TAP)** is to develop a prognostic model to explain variation in Duchenne disease progression to accelerate drug development. PPMD is an early funder.

OUR PROMISE TO YOU

PPMD is the largest Duchenne-specific organization that focuses on high potential research opportunities that will impact every single person with Duchenne. Our return on investment is simple—to End Duchenne.

LEARN MORE: [ENDDUCHENNE.ORG/RESEARCH](https://endduchenne.org/research)

ADVOCACY

When Parent Project Muscular Dystrophy started in 1994, we knew that one of our first priorities was to travel to Washington, D.C. to give a voice to the thousands of families affected by Duchenne. We had to educate our nation's leaders to get the federal support needed to make real progress in research and care. Today, we continue the education of our nation's leaders. We provide advocates with the tools and information they need to advance legislation and regulatory efforts that will impact their lives and ensure Duchenne is a priority across all government agencies.

Perhaps no other rare disease health organization has come so far and so fast in the realm of Congressional advocacy than PPMD. Since our advocacy efforts began in 2000, our community has helped to leverage over \$800 million in federal funding into muscular dystrophy research, with over \$600 million specifically for Duchenne, and achieved two FDA product approvals.



4 bills

signed into law,
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Over \$600 million

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community into research



More than 2000
meetings

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Members of Congress

OUR DUCHENNE ADVOCACY PRIORITIES

Our advocacy efforts are focused on advancing the research that will lead to treatments by:

- Increasing federal investment in Duchenne research
- Accelerating therapy development & product review processes
- Ensuring that patient experience is formally integrated into the drug development life cycle, from protocol design through payer determinations
- Improving healthcare outcomes, access, & delivery
- Developing newborn screening system & infrastructure
- Ensuring access & coverage to approved products
- Ensuring that regulatory & public policies reflect the priorities of adults with Duchenne

LEGISLATION & PUBLIC POLICY ADVOCACY

PPMD drives innovative, paradigm-shifting federal legislation and has changed the Duchenne landscape through the passage of four federal bills and annual Duchenne-specific Appropriations Report Language for more than a decade. Our **Advocacy Conference** is the only officially organized gathering on Capitol Hill for the Duchenne community. We also provide the community with the opportunity to raise their voices year-round through Action Alerts—specific actions to take that influence legislators.

Ongoing Legislative & Public Policy Initiatives

- Federal Funding for Duchenne Research & Programs
- Healthcare Reform
- Duchenne Newborn Screening
- Newborn Screening Saves Lives Act Reauthorization
- Access & Coverage

By galvanizing the Duchenne-patient community and bringing our unified voice to Washington, D.C., we have fostered relationships with elected officials who became unwavering Duchenne champions on Capitol Hill, passed banner legislation, and established collaborations with every federal agency that touches Duchenne. As therapies have moved through development, we've told our personal stories to regulators and transformed those stories into quantifiable data. We have catalyzed research funding, and built a regulatory infrastructure for rare disease products that has reshaped the therapeutic, care, and trial environment.



REGULATORY ADVOCACY

PPMD works to ensure that patient experience is central in product reviews. We convene families and other Duchenne advocacy organizations for regulatory events, including federal Advisory Committee meetings and summits, designed to inform regulators about the Duchenne patient experience.

With the urging and leadership of PPMD, the FDA embarked on drafting a **Duchenne guidance** in January 2018. This was the first time a patient community took on drafting their own guidance, creating a model for other rare disease groups to follow.

Our Regulatory Leadership In Action

- Patient-Focused Drug Development
- Patient Preference Studies & Data Collection—including multiple publications
- White Paper Publications:
Putting Patients First & Patients Are Waiting
- Duchenne Patient-Focused COMPASS Meeting
- FDA Guidance

ACCESS & COVERAGE ADVOCACY

With the first **two FDA approvals** in Duchenne therapies, the Duchenne community has entered a new environment for access and coverage. PPMD has been leading the effort to educate and guide the community through these uncharted waters.

Access & Coverage Resources

PPMD began assessing the landscape and working to develop resources that would help ensure as favorable an access environment as possible, well before our first approved therapies. Our access efforts have included:

- Data gathering through The Duchenne Registry and in partnership with the CDC surveillance program, MD STARnet
- Leading health economic studies
- Establishing a distinct ICD-10 code for Duchenne and Becker
- Engaging with public and private payers
- Leading national newborn screening efforts in Duchenne
- Building resources for the patient and clinical community to support navigation of access processes, including access to unapproved investigational therapies
- Duchenne Outcomes Meeting—first ever convening of payers, regulators, clinicians, patients, methodologists, and HTAs

CARE

Care. It's at the heart of everything Parent Project Muscular Dystrophy does. We strive to ensure that people living with Duchenne are living longer, stronger lives, by helping you access expert healthcare providers, a comprehensive team of sub-specialists, and cutting edge treatments.

Because of PPMD's push to advance care, people with Duchenne are living more productive lives. Our community members hold important jobs, impact policies in Washington, get married and raise families—things we would not have thought possible even 10 years ago.



added to average
lifespan due to
PPMD-led advances
in care



awarded certification
by PPMD across the US
as of June 2019



in PPMD's network
of certified clinics



of identifying & addressing
gaps in care through
specialty workshops
& consensus meetings

STANDARDIZING CARE

Since PPMD began in 1994, the average lifespan of people with Duchenne has increased from late teens/early-20s to mid-20s/early-30s. We've seen the greatest advancements in the fight to end Duchenne around standards of care, resulting in greatly improved quantity and quality of life.



PPMD's Certified Duchenne Care Center network has now grown to 25 incredible centers across the United States, serving more than 3,300 patients and their families.

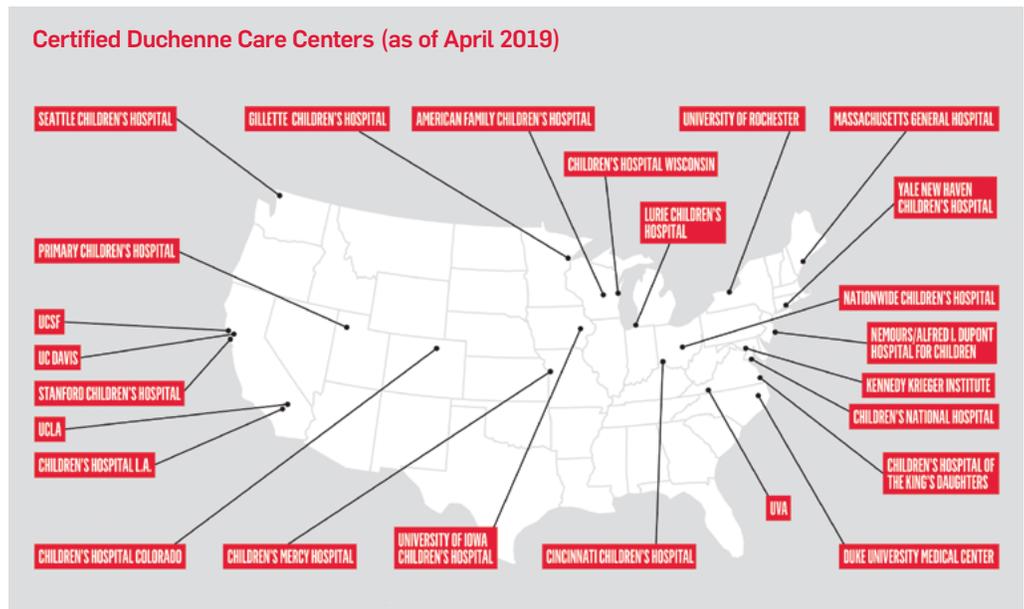
- In partnership with the CDC, PPMD advocates for and participates in the development of standards of care for Duchenne. These guidelines, updated in 2018, outline the accepted treatments and therapies known to address Duchenne symptoms and improve quality of life.
- These guidelines are also included in the "Imperatives for DUCHENNE MD," a one-page snapshot of recommended Duchenne care.
- PPMD was instrumental in the development of an ICD-10 code for Duchenne/Becker muscular dystrophy. This code will allow us to identify patients, to evaluate the care and services provided, and to evaluate the impact that care and services have on quality and quantity of life.
- PPMD helps people with Duchenne and their families understand the value and importance of standardized optimal care. We advocate for improvements in care and access for all families regardless of geography or socioeconomic status.

LEARN MORE: [ENDDUCHENNE.ORG/CARE](https://endduchenne.org/care)

ENSURING ACCESS TO OPTIMAL CARE

PPMD strives to ensure that people living with Duchenne are living longer, stronger lives, by helping them access expert healthcare providers, a comprehensive team of sub-specialists, and cutting edge treatments.

- We help families access Duchenne care and services via the **PPMD Certified Duchenne Care Center Program**. Centers that qualify for PPMD's certification must meet and maintain the highest standards—complying with CDC care guidelines and applying new, evidence-based knowledge and care as it emerges. Next, PPMD will begin certifying clinics that specialize in adult Duchenne care and we will mentor international organizations in the expansion of global certification.



- PPMD connects the Duchenne community through **The Duchenne Registry**—the only registry connecting Duchenne and Becker patients and families with clinical trials, care, and research. Many of these trials offer hope through treatments that improve function, prognosis, and quality of life.
- **Decode Duchenne** provides free genetic testing and counseling to people with Duchenne or Becker muscular dystrophy who otherwise could not afford genetic testing. Decode Duchenne is administered by The Duchenne Registry, a program of PPMD, and is supported by Sarepta Therapeutics and PTC Therapeutics.

KNOWLEDGE IS POWER

We take our leadership role in the Duchenne community very seriously and we know that people turn to us first when they have questions about research, advocacy, and most often, care. Over the last few years, PPMD has worked diligently with experts in our community to ensure that **our website is up-to-date**. We know that in urgent situations, families need important and accurate information at their fingertips. Not only does our website contain the critical information you need, but we introduced an **Emergency Care Card** and the first **Duchenne-specific mobile app** in the U.S. so you always have information you need when and where you need it. Our website **provides comprehensive care handouts**, separated by both stage of disease and body system that can be printed from home and taken to your medical team so that they have the latest Duchenne information.

Visit our website for additional Care resources, including:

- A “New Diagnosis” section of our website to help families in the first year of diagnosis
- The Duchenne Family Guide, a more accessible version of the standards of care
- Educational videos and webinars, including topics like physical therapy and pulmonary awareness
- Emergency resources available in both English and Spanish
- The PJ Nicholoff Steroid Protocol, offering emergency information on steroid replacement and stress dosing, in both English and Spanish

THE DUCHENNE REGISTRY

The Duchenne Registry began in 2007, when a group of thought leaders in the Duchenne muscular dystrophy community, led by Parent Project Muscular Dystrophy, began discussing the need for a new kind of resource that would connect and serve the needs of the entire community. What we envisioned was a central hub that would bring together those living with Duchenne or Becker, along with their families and caregivers, to connect them with medical research, clinical care, clinical trials, and each other.

At the same time, it would also be a resource for researchers and industries with an interest in Duchenne, allowing access to aggregate, de-identified data provided by patients and their families—information that could prove vital to advances in care and treatment.

Today, the result of this endeavor is The Duchenne Registry, the largest, most comprehensive registry for Duchenne and Becker muscular dystrophy.



across the globe
have registered in
The Duchenne Registry
since launch



countries
represented



of longitudinal data



trials & studies
recruited

WHY JOIN THE DUCHENNE REGISTRY

- **Advance Research & Speed Development of New Treatments**

If you have Duchenne or Becker muscular dystrophy or if you are a female carrier of Duchenne or Becker, join The Duchenne Registry and your data will help fuel the fight to end Duchenne. We share your anonymous Registry data with researchers to speed the development of new therapies.

- **You Have the Power to Make a Difference**

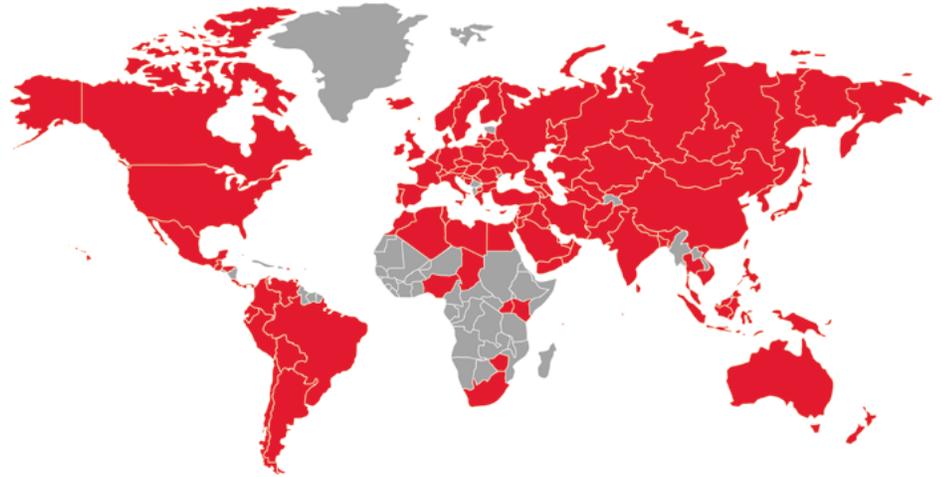
When you join and update your account in The Duchenne Registry, you are strengthening the power of a more than 12-year-old network of patient-powered data that will be used to improve care for people living with Duchenne and increase our understanding of the disorder. You become a citizen scientist by contributing to real scientific research.

- **Find out About Research Studies & Clinical Trials**

Once you register and complete your Medical Surveys, we will let you know when you might be a good fit for research studies and clinical trials. Your data also helps drug developers know the size of the Duchenne population available for trials and helps identify new trial sites, increasing our community's access to trials and potential therapies.

- **Join Patients Around the World**

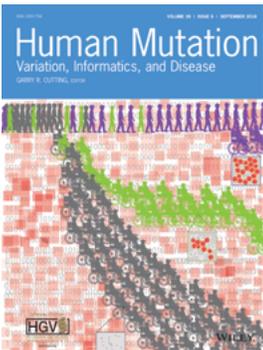
No one should have to navigate a Duchenne diagnosis alone. Be part of the global community to end Duchenne. The data you enter is not only shared with researchers in the United States, but is also shared with the TREAT-NMD International Neuromuscular Registry, which pools data from thousands of patients worldwide and enables more powerful data analysis and discovery.



STUDYING THE PAST WHILE LOOKING AHEAD



When the Registry began in 2007, we knew that building the largest, most comprehensive registry in Duchenne would be a marathon, not a sprint. In 2018, PPMD was proud to publish a ten-year report, representing an analysis of approximately 4,000 Duchenne and Becker individuals and carrier females. RTI International performed all statistical analyses.



The Registry allows researchers to have access to thousands of patients with Duchenne without ever having to see a patient in person, and at a fraction of the cost of a traditional natural history study. In the September 2018 issue of *Human Mutation*, a team of researchers at UCLA, led by Richard Wang, PhD, found a correlation between certain dystrophin gene deletions and the age at loss of

ambulation. Another study of genotype-phenotype correlations using Registry data is currently pending publication.

In 2019, a study using the Registry data was published in a well-known international journal, *BMC Neurology*. This study was led by researchers at Catabasis Pharmaceuticals and focused on the Registry's corticosteroid data. The Catabasis team wanted to gain a better understanding of corticosteroid use in the United States since the establishment of the Duchenne Care Guidelines. The researchers performed a retrospective analysis of corticosteroid use

in ambulatory and nonambulatory males with Duchenne or Becker muscular dystrophy who enrolled in The Duchenne Registry from 2007 to 2016.



In order to expand on the Registry's important work, and to make the Registry experience less burdensome and more family-friendly, we are launching a new mobile app platform in 2019. The Registry team is working with developers at THREAD Research and Invitae (the host of our current web-based platform) to make this transition as seamless as possible. We are excited about the next phase of the Registry and strongly encourage continued

participation from all of our Duchenne and Becker families.

In conjunction with our transition of the Registry to a new platform, PPMD has joined with Prometheus Research to form a Duchenne data hub—the Duchenne Outcomes Research Initiative—that will ingest data from multiple sources in order to enhance collaboration and speed research. Sources of data include our Duchenne Registry, electronic health record (EHR) data from several hospitals and clinics across the United States, claims data, and post-marketing surveillance data from industry. Our first industry partner into the Interchange is Sarepta Therapeutics, who will be contributing their EXONDYS 51 post-marketing surveillance data.

These enhancements to the Registry, we believe, will expand the breadth of the data we are able to collect, while reducing the time it takes for participants to complete surveys and update records. It is our hope that this combined data will help accelerate Duchenne research and the path to therapies.

RACE TO END DUCHENNE



\$13 million

in funds raised by
participants since the
program began



5500
participants

in Race to
End Duchenne
since 2005



Over 200
signature races

completed by
our team
since 2005



Over 6000
miles

run by Race to End
Duchenne team
members each year

Race to End Duchenne is about more than running races and winning medals. It's about friends and family members taking action in the fight to end Duchenne; it's about caregivers relieving stress and taking care of themselves physically and mentally; and it's about meeting others in the Duchenne community.

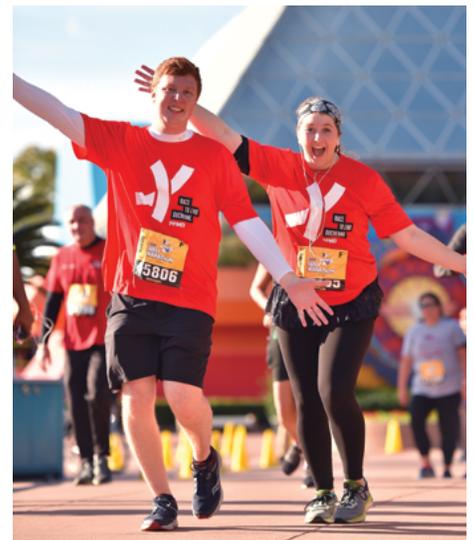
Since the **Race to End Duchenne** program began 15 years ago, more than 5,500 participants have raised over \$13 million to advance research, advocacy and care to help those with Duchenne live stronger, longer lives.

From Big Sur to New York City and countless cities in between, **Race to End Duchenne** team members have participated in nearly every major marathon in the country, as well as several other endurance events, including bike races, triathlons, mud runs, and more.

The **Race to End Duchenne** calendar offers about 15 races per year, giving participants many opportunities to find a race that's right for them, whether it's their first or their 21st race!

Annual races include:

- TCS New York City Marathon
- Bank of America Chicago Marathon
- Chevron Houston Marathon & Aramco Houston Half Marathon
- Walt Disney World Marathon Weekend
- Bank of America Shamrock Shuffle 8K
- TD Five Boro Bike Tour
- Several Rock 'n' Roll Marathons & Half Marathons



LEARN MORE: [RACETOENDDUCHENNE.ORG](https://www.racetoendduchenne.org)

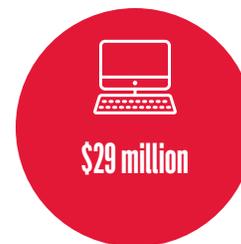
DIY FUNDRAISING TO END DUCHENNE



raised by
fundraising events
since 1994



held throughout
the country
annually



raised through
personal fundraising
pages

HONOR A LOVED ONE

Did you know you can create a fundraising page in honor or in memory of a loved one? You'll be able to personalize the page with a story and photo, track donations, and your supporters will receive tax receipts automatically. Visit EndDuchenne.org/DIY to start yours now.

FUNDRAISE ON FACEBOOK

Celebrate your birthday, wedding, or other special day by asking for donations to help fund the fight to end Duchenne. Visit PPMD's Facebook page and click on the "Create Fundraiser" button.

Fund the fight to end Duchenne *your* way and have fun encouraging your community to support you!

From fun runs, bake sales, and game nights, to full scale golf tournaments and live auctions, the Parent Project Muscular Dystrophy team will help make your ideal fundraising event a reality.



We are here to help you host an event that is meaningful to you and that your community would love! PPMD will guide you every step of the way, providing all of the tools you need to make your event a success:

- We'll create a webpage for your event, designed to track donations, registrants, and more.
- We will send all the materials you need for your DIY event including informational postcards, wristbands, balloons, and more.
- We'll help you with logistics, provide insurance, and help you identify resources in your community.

Email us at events@parentprojectmd.org to get started with planning your event!

COACH TO CURE MD



On the last Saturday of each September, the American Football Coaches Association and Parent Project Muscular Dystrophy gear up to **#TackleDuchenne**...and you can join in the game!

Coaches and fans nationwide promote Coach To Cure MD by wearing armbands, mentioning Coach To Cure MD during on and off-field interviews, holding tailgate parties, and even joining their favorite teams on the field.



Over 108000
coaches

have participated
over the past 11 years



1400 high school &
college teams

across the country
have participated



Over 100 Duchenne
families

attend games
each year!

ABOUT COACH TO CURE MD

Coach To Cure MD is a partnership between the American Football Coaches Association (AFCA) and Parent Project Muscular Dystrophy. In 2008 the AFCA adopted PPMD's Coach To Cure MD program as one of their charity efforts, in part because of the unique parallels between Duchenne, a disorder which robs young men of precious muscle strength and college football, a game where young men are at the peak of their muscle strength. For more information and to get involved visit CoachToCureMD.org.

Hundreds of college football teams across the country embrace Coach To Cure MD, raising awareness and much-needed funds, on and off the field.

GET IN THE GAME!

You can help PPMD **#TackleDuchenne**:

- Visit CoachToCureMD.org to see if your favorite college or university is involved. If they're not, email us at info@coachtocuremd.org to see how you can get them signed up.
- Set up a fundraising event such as a tailgate party or set up a personal fundraising page and ask your friends to help **#TackleDuchenne**
- Represent PPMD at a game — opportunities include tickets, and in some cases VIP experiences such as meeting the team, being on field during the game, or even participating in the coin toss!

STAFF

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Kathryn R. Wagner, MD, PhD

Greg Wilde, MD

STATEMENTS OF FINANCIAL POSITION **

Years Ended December 31, 2018 and 2017

	2018	2017
Assets		
Current Assets	5,675,952	4,727,383
Property and Equipment	34,210	38,803
Other Assets	22,607	22,607
	5,732,769	4,788,793
Liabilities and Net Assets		
Current Liabilities	1,963,776	1,402,724
Net Assets Without Donor Restrictions	3,768,993	3,386,069
	5,732,769	4,788,793

STATEMENTS OF ACTIVITIES **

Years Ended December 31, 2018 and 2017

	2018	2017
Public Support		
Contributions	6,293,312	6,019,600
Grants	1,317,052	1,703,581
Conference income	319,960	294,431
Fees for service	123,539	172,000
Special events		
Gross income	1,400,568	1,413,098
Direct expense	(383,625)	(271,515)
Investment Income		
Interest and dividend income	1,654	1,560
Loss on sale of donated securities	(1,301)	(314)
Total Income	9,071,159	9,332,441
Functional Expenses		
Program services	7,533,047	7,896,174
Management and general	463,572	511,511
Fundraising	691,616	695,074
Total Expenses	8,688,235	9,102,759
Increase (decrease) in net assets	382,924	229,682
Net assets — beginning of year	3,386,069	3,156,387
Net assets — end of year	3,768,993	3,386,069

**The audited financial statements with accompanying independent auditor's notes are available upon request, or on the PPMD website at endduchenne.org

About Duchenne

Duchenne muscular dystrophy is the most common muscular dystrophy in children. It is a progressive disorder that causes muscles to become weaker over time until it affects the whole body. Duchenne is not contagious. About one out of every 5,000 boys has Duchenne, and about 20,000 babies worldwide are born with it each year. Duchenne mostly affects males and reaches across all races and cultures. Parent Project Muscular Dystrophy (PPMD) estimates that there are about 15,000 young men, as well as a few young women, living with Duchenne today in the United States.

Duchenne progresses differently for every person. Even siblings with the same mutation may have a very different progression of symptoms. The progression of symptoms through Duchenne are on a spectrum, from late onset/very mild symptoms to early onset/severe symptoms. Regular visits with a neuromuscular team help families monitor the progression of this disease, and how it can best be treated along the way.

To learn more about Duchenne muscular dystrophy, visit EndDuchenne.org/about.

About Parent Project Muscular Dystrophy

Parent Project Muscular Dystrophy (PPMD) is the largest most comprehensive nonprofit organization in the United States focused on finding a cure for Duchenne muscular dystrophy—our mission is to end Duchenne.

We demand optimal care standards and strive to ensure every family has access to expert healthcare providers, cutting edge treatments, and a community of support. We invest deeply in treatments for this generation of Duchenne patients and in research that will benefit future generations. Our advocacy efforts have secured hundreds of millions of dollars in funding and won two FDA approvals.

Everything we do—and everything we have done since our founding in 1994—helps those with Duchenne live longer, stronger lives. We will not rest until we end Duchenne for every single person affected by the disease. Join our fight against Duchenne at EndDuchenne.org.

Parent Project Muscular Dystrophy

JOIN THE FIGHT.
END DUCHENNE.

401 Hackensack Avenue, 9th Floor
Hackensack, NJ 07601
800.714.5437

ENDDUCHENNE.ORG

