ENDDUCHENNE.ORG
#ENDDUCHENNE

Parent Project Muscular Dystrophy

JOIN THE FIGHT.
END DUCHENNE.

RESEARCH

$54 million+
invested by PPMD into Duchenne research & therapy development to date

Over 275 research grants
awarded, supporting nearly every major therapeutic approach

~ 35 clinical trials
in Duchenne at any given time

5 US drug approvals
with additional promising therapies in development

ADVOCACY

Over $600 million
in federal funding leveraged by PPMD community into research

4 bills
signed into law, reshaping the Duchenne landscape

13000 messages & action alerts
sent to Congressional offices

More than 2500 meetings
with Members of Congress

CARE

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

30+ clinics
awarded certification by PPMD across the US

750 care providers
in PPMD network of certified clinics

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

ENGAGEMENT

29 PPMD Connect groups
across the United States, providing local families with resources, outreach, & mentoring

Over 5500 patients & carriers
across the globe have registered in The Duchenne Registry since launch

$40 million+
raised through Race to End Duchenne & family-led grassroots events since 1994

14000+ families
reached in person, through 28 Annual Conferences, End Duchenne Tour stops, Roundtable discussions, & Advocacy Conferences

2021 ANNUAL REPORT

Dear Duchenne community,

These past few years, in the face of some very challenging times, we have accomplished incredible things, and I am proud of the power we have when we work together. The fight to end Duchenne is arduous, but our achievements are nothing short of remarkable.

Over the past year, PPMD has committed over $4.5 million to Duchenne research, continuing to focus on our three key initiatives: Cardiac, Gene Therapy and Biomarkers, as well as community-driven research such as The Duchenne Registry and newborn screening. We built the infrastructure to launch our Electronic Health Records (EHR) Study, which will extract data from several of our Certified Duchenne Care Centers across the country. This study will enable PPMD's Duchenne Outcomes Research Interchange to house 15+ years of patient-reported data from The Duchenne Registry alongside new clinician-reported data. Our New York State Newborn Screening Pilot recently completed two years of ground-breaking recruitment with more than 36,000 babies screened for Duchenne. As a result of screening, four babies with Duchenne or Becker and one carrier female were diagnosed and provided with optimal early care.

Our Care team has continued to bring together experts across the medical field in order to provide information and support throughout the pandemic. Through our network of Certified Duchenne Care Centers and the remarkable providers at them, we have increased our focus on cardiac care, psychosocial issues in Duchenne, and treatment of carrier females.

We saw the largest number of advocates ever participate in our Advocacy Conference, with 435 individuals representing 43 states. Advocates participated in over 300 virtual meetings with their local representatives. We are currently updating the community-led Guidance to the FDA to ensure it reflects the progress and scientific advancements made since the first Guidance in 2014. New this year is the addition of a Pharmaceutical Advisory Board and a Cardiac and Gene Therapy Working Group. The work we are doing in Washington, D.C., will have a significant impact on the future of drug development for Duchenne and lead the way for other rare diseases.

We demonstrated our impressive ability to come together again this year, even if it was virtually. We had over 1,700 attendees, representing 51 countries at our Annual Conference. Our PPMD's Connect groups, Newly Diagnosed families and Teen/Adult community have also benefited significantly from virtual participation opportunities. We launched our first-ever official group for siblings, as well as a Podcast series which serves as another space for us to talk about the Duchenne journey.

The collective power and resiliency of our community will keep us moving forward. We turn our challenges into opportunities, we fight to end Duchenne, and we never give up.

Each step we take brings us closer to the day we will end Duchenne and closer to each other as a community. When my boys were first diagnosed, life felt hopeless. But we found one another, and we built PPMD together. It was the power of each of us that made today a possibility for our Duchenne community.

The Power of Us is stronger thanks to each of you!

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 Committee** 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 Duchenne deserves the same federal priority and investment as any other disease.

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 on some level of risk and uncertainty in exchange for stopping or even slowing disease progression. 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+ year 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, 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 events, we also provide local opportunities for families to gather. Whether through **PPMD's Connect program** — our regional, parent-led outreach program — at one of **PPMD's Race to End Duchenne events or grassroots fundraisers**, or in our virtual **Newly Diagnosed Families Meet & Greets**, we are always looking for opportunities to bring families together for face-to-face interaction.

Connecting with peers is critical on the Duchenne journey. **PPMD's Adult Advisory Committee (PAAC)** brings together teens and young adults with Duchenne & Becker to drive policy initiatives and programs to create and improve pathways to a better quality of life. Our **Tweens program** brings together the younger generation to connect socially through both virtual opportunities and in-person gatherings at our Annual Conference.

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

YOUR IMPACT

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.

84 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 partnership@parentprojectmd.org or call 201-250-8440.

RESEARCH

For over 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.

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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 and Becker 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 of Duchenne Therapeutics Laboratory** for the last 15 years, serving academia and 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 expanded our support to a number of **natural history studies on carriers of Duchenne and Becker** to better understand their cardiac risks and help drive towards better care.
- **PPMD's Biomarker Initiative** seeks to enhance our understanding of Duchenne so we can better predict progression of the disease and develop more sensitive tools for determining if drugs are changing disease course.
- In addition to significant pre-clinical and academic research funding, PPMD makes **investments in early-stage biopharmaceutical companies** to catalyze development of novel therapies to treat Duchenne and Becker. This type of early stage industry investment (often referred to as venture philanthropy) allows companies to complete critical studies needed to advance investigational products to the clinic, while also providing the potential to create a financial return for PPMD that can then be reinvested into additional Duchenne and Becker research programs in academia or industry.

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 (such as a **Master Protocol**) that test multiple drugs, in multiple arms at the same time, enabling participation from infants to adults, minimizing exposure to placebo, and establishing success or failure more quickly.

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

- A major goal of the **Duchenne Regulatory Science Consortium (DRSC)**, which PPMD helped to found in 2015, is to develop a regulatory-ready, clinical trial simulation tool to accelerate clinical trials for new drugs to treat Duchenne.
- PPMD also supports the **Collaborative Trajectory Analysis Project (C-TAP)**, where one of their major aims is to develop prognostic models to explain variation in Duchenne disease progression, which can help researchers to find answers about drug efficacy.

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.

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 $600 million in federal funding for Duchenne and Becker, and achieved five FDA product approvals.

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4 bills
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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
- Prescription Drug User Fee Act
- 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.

In 2014, PPMD submitted a **community-led guidance document** to the FDA for developing therapies in Duchenne which resulted in FDA producing their own version in 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. In late 2021, PPMD set out to update the 2014 guidance with the goal of submission by mid 2022.

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 **five 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 unchartered 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.

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

30+ clinics
awarded certification by PPMD across the US

750 care providers
in PPMD's network of certified clinics

20 years
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 over 30 incredible centers across the United States, serving more than 3,700 patients and their families.

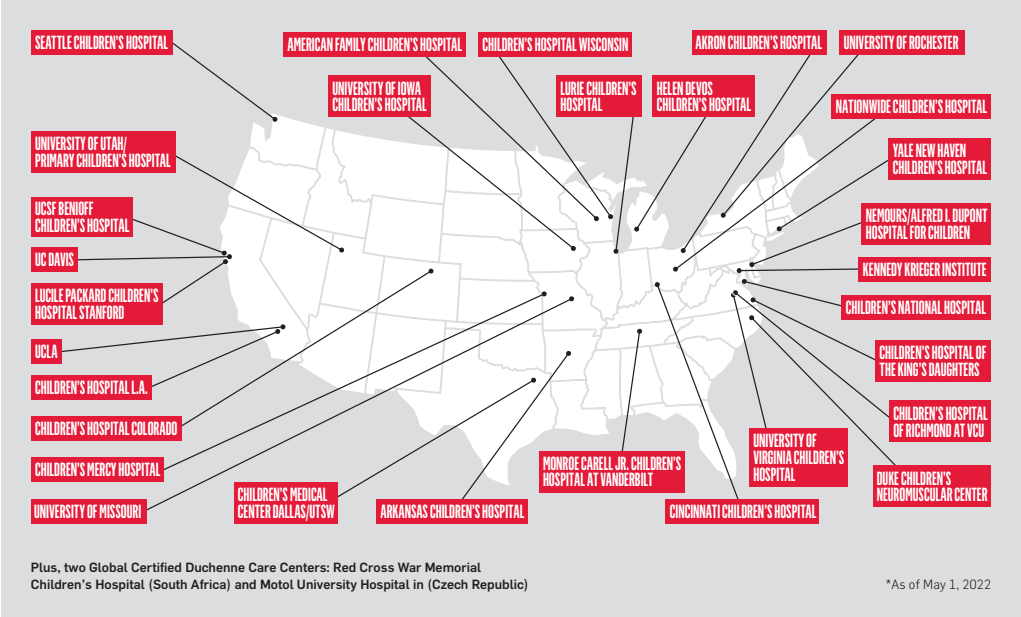
- In partnership with the CDC, PPMD advocates for and participates in the development of **standards of care for Duchenne**. These guidelines, updated in 2018, outline the accepted treatments and therapies known to address Duchenne symptoms and improve quality of life.
- These guidelines are also included 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.

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. PPMD has also begun to certify clinics that specialize in adult care, as more and more teens and adults living with Duchenne transition from pediatric care and look for quality clinicians and resources that address their needs as they age. In addition, we continue to mentor international organizations in the expansion of global certification.

Certified Duchenne Care Centers*



Plus, two Global Certified Duchenne Care Centers: Red Cross War Memorial Children's Hospital (South Africa) and Motol University Hospital in (Czech Republic)

*As of May 1, 2022

- 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 studies 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 and females at risk to be carriers. Decode Duchenne is administered by PPMD's genetic counselors, and is supported by Sarepta Therapeutics, PTC Therapeutics, Vertex Pharmaceuticals, and NS Pharma.

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. PPMD works 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.

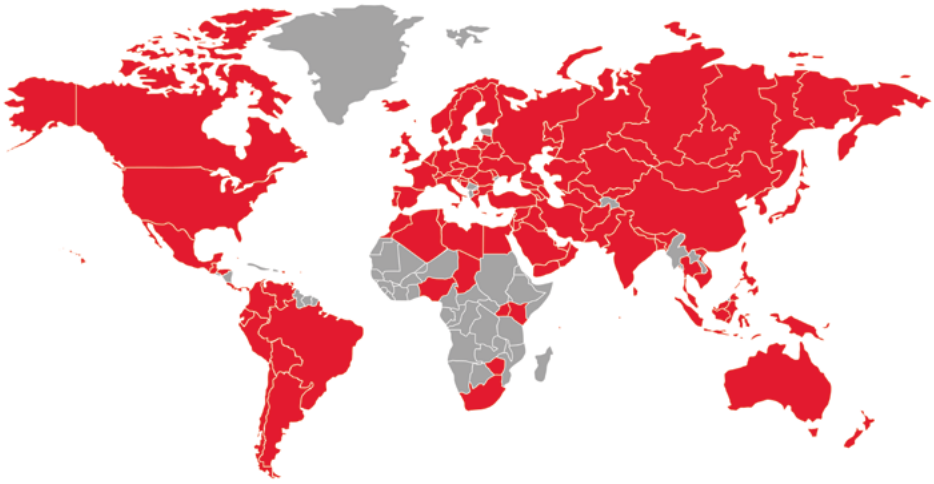
What we envisioned was a central hub that would bring together those living with Duchenne or Becker, along with their families and caregivers, to connect them with medical research, clinical care, clinical trials, and each other. At the same time, it would also be a resource for researchers and industries 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.

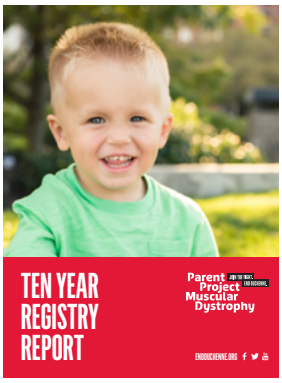
WHY JOIN THE DUCHENNE REGISTRY

- **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 15-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. As we approach our 15 year anniversary this year, we are looking forward to publishing another community report that will highlight the data we have collected since 2007 and what we have learned.

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 was published in the *Journal of Neuromuscular Diseases* in 2020 and the researchers, led by Kevin Flanigan, MD at Nationwide Children's Hospital, studied the clinical features of individuals with Duchenne gene deletions that are the same as the deletions produced by exon 51 skipping therapy.

In order to expand on the Registry's important work, and to make the Registry experience less burdensome and more family-friendly, we launched a mobile app platform in 2019. The Duchenne Registry App currently has over 1,300 active users contributing data from the palm of their hand!

In conjunction with our transition of the Registry to a mobile app platform, PPMD also formed the Duchenne Outcomes Research Interchange, a central data hub that combines data from multiple sources in order to enhance collaboration and speed research. All data from The Duchenne Registry flows into the Interchange, and can be combined with electronic health record (EHR) data from several hospitals and clinics, claims data, post-marketing surveillance data, and other research-driven data sets. PPMD's EHR Study is officially underway, extracting data from several of our Certified Duchenne Care Centers across the country. We plan to consent patients at additional centers in the coming months and years.

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.

CONNECT WITH THE COMMUNITY

No one needs to go through the Duchenne and Becker journey alone. Parent Project Muscular Dystrophy provides numerous ways to connect with others in the community, build your support network, and surround yourself with people who understand your journey.

PPMD's Connect

PPMD's Connect is the official family outreach program of PPMD. Led by volunteer parents and grandparents, PPMD's Connect groups serve as regional points of contact for families and individuals affected by Duchenne and Becker. With 29 groups across the country, including a new Sibs Connect group just for siblings, we're providing a way for families to connect with local resources, help them better navigate their diagnosis, and ways to connect with other families both in-person and online.
endduchenne.org/connect

PPMD's Annual Conference

PPMD's Annual Conference has grown to be recognized worldwide as the foremost Duchenne muscular dystrophy meeting. Families, physicians, researchers, caregivers, industry partners, and those living with Duchenne and Becker gather—in-person or virtually—to connect, share information, and learn the latest progress in the fight to end Duchenne.
endduchenne.org/conference

End Duchenne Tour

In an effort to reach every single family facing a Duchenne diagnosis in the U.S., PPMD has launched a multi-year community experience called the End Duchenne Tour. Combining each of the pillars that make up PPMD's mission into one-day meetings, the End Duchenne Tour brings updates on research, advocacy, and care to cities across the country, featuring a roster of leading experts in the Duchenne space.
endduchenne.org/tour

PPMD's Teen & Adult Community

Led by PPMD's Adult Advisory Committee (PAAC), our Teen & Adult community is growing. Together, our teens, young adults, parents, and community members are working to ensure that people with Duchenne don't just SURVIVE into adulthood with greater frequency than ever before—but that they also THRIVE in adulthood. Ways to connect include a Teen & Adult Facebook group, our new PAAC Chats social networking platform, and a Teen & Adult Track at our Annual Conference.
endduchenne.org/youngadults

Newly Diagnosed Families Meet & Greets

Held virtually several times a year, our Newly Diagnosed Families Meet & Greets provide an opportunity for families who've recently received a diagnosis to connect with members of PPMD's team and with each other.
endduchenne.org/newlydiagnosed

Tween Socials

Our virtual Tween Socials, held several times a year, are an opportunity for people with Duchenne ages 9-13 to get together, play some games and have some fun!
endduchenne.org/tweens

29 PPMD Connect groups

across the United States, providing local families with resources, outreach, & mentoring

14,000+ Families

reached in person, through 28 Annual Conferences, End Duchenne Tour stops, Roundtable discussions, & Advocacy Conferences

40 Tour Stops

as part of PPMD's End Duchenne Tour

For more information about our Community Engagement programs, please visit our website at **endduchenne.org/get-involved** or contact **connect@parentprojectmd.org**.

RACE TO END DUCHENNE

$15 million
in funds raised by participants since the program began

6000 participants
in Race to End Duchenne since 2005

Over 225 signature races
completed by our team since 2005

Over 8000 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 in 2005, more than 6,000 participants have raised over $15 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. In 2020, PPMD introduced our first-ever virtual race series, which will continue on so that families across the country (and globe!) can participate with ease!

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
- TD Five Boro Bike Tour
- Several Rock 'n' Roll Marathons & Half Marathons
- Regional races across the country

DIY FUNDRAISING TO END DUCHENNE

$25 million

raised by fundraising events since 1994

Over 100 fundraisers

held throughout the country annually

$30 million

raised through personal fundraising pages

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.

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.

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

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.

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 109000 coaches

have participated since 2008

1400 high school & college teams

across the country have participated

Over 100 Duchenne families

attend games each year!

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 you 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!

ENDDUCHENNE.ORG

Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.

STAFF

Pat Furlong — *President & CEO*

Ryan Fischer — *Chief Advocacy Officer*

Kaylan Moitoso — *Chief Business Officer*

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Eric Camino, PHD — *Vice President, Research & Clinical Innovation*

Jennifer Capobianco — *Grants Management Coordinator*

Ally Cripe — *Development Coordinator*

Brian Denger — *Community Engagement Coordinator*

Erin Dresnick — *Director, Development*

Megan Freed — *Director, Data and Technology Strategy*

Jody Gabbard — *Senior Administrative Coordinator*

Kimberly Galberaith — *Creative Director*

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Kayla Quirin, MS, CGC — *Community Research Project Manager*

Mena Scavina, DO — *Advisor, Certified Duchenne Care Center Program*

Rachel Schrader, MS, APRN, CPNP-PC — *Vice President, Clinical Care & Education*

Lauren Stanford — *Director of Advocacy*

Ellen Wagner — *Community Centricity Manager*

Amanda Wilkison, RN, BSN — *Senior Director, Clinical Care & Education*

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Donna Saccomanno — *Co-Founder & Lifetime Trustee*

STATEMENTS OF FINANCIAL POSITION

Years Ended December 31, 2021 and 2020	2021	2020
ASSETS		
Current Assets		
Cash and Cash Equivalents	7,780,724	5,472,535
Grants Receivable	37,500	—
Pledges and Accounts Receivable	793,289	355,746
Investment Securities	927,294	260,308
Total Financial Assets	**9,538,807**	**6,088,589**
Employee Advances	11,527	16,444
Merchandise Inventory	52,041	29,158
Prepaid Expenses	217,861	105,167
Total Current Assets	**9,820,236**	**6,239,358**
Property & Equipment		
Office Equipment	75,879	132,959
Office Furniture	16,019	37,810
Leasehold Improvements	—	6,180
Less Accumulated Depreciation	91,898	176,949
	(64,692)	(152,368)
	27,206	24,581
Other Assets		
Investments at Cost	350,480	948,880
Security Deposit	5,938	22,607
	356,418	872,487
Total Assets	**10,203,860**	**7,136,426**
LIABILITIES AND NET ASSETS		
Current Liabilities		
Accounts Payable and Accrued Expenses	396,783	115,946
Grant Received with Conditions	150,000	—
Research Grants Payable	531,753	517,387
Loan Payable to Small Business Administration	425,852	447,660
Total Current Liabilities	**1,504,388**	**1,080,993**
Net Assets	8,699,472	6,055,433
Total Liabilities and Net Assets	**10,203,860**	**7,136,426**

STATEMENTS OF ACTIVITIES

Years Ended December 31, 2021 and 2020	2021	2020
REVENUES		
Contributions and Grants	6,628,789	7,353,657
Conference Income	313,650	329,350
Earned Income	172,339	73,920
Special Events Revenue, Net		
Gross income	2,050,953	1,536,341
Less Direct Expense	(138,175)	(133,757)
	1,912,778	1,402,584
Revenue Sharing	997,500	—
Employee Retention Credit	329,826	—
PPP Loan	447,660	—
Investment Income	(315,549)	12,089
Other Income	410	409,888
Total Revenues	**10,487,403**	**9,569,399**
EXPENSES		
Program Services		
Research	4,761,246	5,341,667
Education	861,049	873,169
Advocacy	770,492	364,011
	6,392,787	6,578,847
Supporting Services		
Management and General	770,970	566,080
Fundraising	679,607	720,711
	1,450,577	1,286,791
Total Expenses	**7,843,364**	**7,865,638**
Change in Net Assets	2,644,039	1,703,761
Net Assets — Beginning of Year	6,055,433	4,351,672
Net Assets — End of Year	**8,699,472**	**6,055,433**

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 five 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.

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