

HONOR EVERY JOURNEY

Parent Project
Muscular Dystrophy

JOIN THE FIGHT.
END DUCHENNE.



DEAR DUCHENNE AND BECKER COMMUNITY,

Each year, I reflect on how far we've come and the strength of the community that makes it possible. Every journey in Duchenne and Becker is both unique and deeply connected, and each one drives our shared mission forward.

We have seen extraordinary progress—from therapeutic advances to expanded newborn screening and stronger standards of care. But behind every milestone is a person, a family, a story. Honoring every journey means continuing to listen, to act with urgency, and to ensure that no one is left behind as the landscape evolves.

Together, we are building a future where every individual has access to the care and support they deserve. I am endlessly grateful for this community and all that we will continue to achieve, together.

With gratitude,

Pat Furlong

Founding President



We are experiencing exciting momentum across the Duchenne and Becker landscape—in research labs, clinics, capitol buildings, and throughout our community. At PPMD, we are focused on identifying gaps across research, advocacy, care, and access, investing in the most promising solutions, and ensuring progress translates into meaningful outcomes for every individual.

From driving funding and supporting clinical trials to shaping regulatory pathways and expanding access, we are committed to moving the entire field forward. This work is grounded in data, collaboration, and a commitment to equity so that the innovation we're driving honors every journey, reaching the people who need it.

I am proud of the progress this community is making every day. Together, we are ensuring that progress is not only made, but shared.

With appreciation,

Katherine Beaverson, MS

Chief Executive Officer



HONOR EVERY JOURNEY

THE PPMD JOURNEY

Parent Project Muscular Dystrophy (PPMD) fights to end Duchenne and Becker. We accelerate research, raise our voices to impact policy, demand optimal care for every single family, and strive to ensure access to approved therapies. Through our initiatives, we listen to our community's needs and address gaps in the field so everyone diagnosed can live stronger lives.

For more than 30 years, we've played a key role in many significant victories for the Duchenne and Becker community, including securing millions of dollars in funding for research and clinical trials and winning eight FDA approvals. Today's cutting-edge therapies are possible because of PPMD's dedication and tireless work.

Together with our community, we will continue to honor every journey, improving outcomes for people living with Duchenne and Becker through research, advocacy, care, and community.

RESEARCH

Research and funding gaps across the field can slow progress towards ending Duchenne and Becker. PPMD identifies these gaps and invests in the most promising therapies across the drug development pipeline to accelerate potential treatments for all people with Duchenne and Becker.

CARE

Navigating the healthcare system and accessing expert care providers, a comprehensive team of sub-specialists, and cutting-edge therapies can be complex. PPMD breaks down barriers, and works to improve and standardize care for all.

ADVOCACY

Advocacy efforts are needed to advance the research that will lead to therapies and, ultimately, an end to Duchenne and Becker. PPMD provides advocates with the tools and information they need to drive legislation and regulatory efforts that will impact their lives and ensure Duchenne and Becker are a priority across all government agencies.

COMMUNITY

No one should have to face Duchenne and Becker alone. PPMD builds community through various programs and events, connecting individuals and families to learn from and support each other.



HONOR EVERY RESEARCH JOURNEY



OUR MISSION TO END DUCHENNE starts with research and innovation. For 30 years and counting, PPMD has pushed the landscape forward by identifying and investing in the most promising studies and therapeutic strategies to treat Duchenne and Becker muscular dystrophy. We fund research across the translational spectrum and convene the brightest minds to share knowledge, build on early work, respond to gaps in the field, and accelerate therapies

INVESTING IN CUTTING-EDGE RESEARCH

We accelerate safe and effective treatments with the goal of ending Duchenne and Becker for every single person diagnosed. To achieve this, we invest in cutting-edge research to address the full impact of Duchenne and Becker, no matter the variant, age, or stage of progression. To date, we've invested over \$60 million to support novel and emerging therapies that bring hope to our community. PPMD funds research across the broad spectrum of stages, from basic research to better understand Duchenne to translational near-term therapeutics through our Venture Pathways program.

GENE THERAPY INITIATIVE

PPMD has supported gene therapy as a potential therapeutic for Duchenne since its early stages, funding critical research to better understand gene therapy and related approaches, as well as its effects on the heart and immune system. Through this initiative we continue to fund research to address gaps and critical needs for the community, such as identifying improved gene therapy products and supporting non-viral delivery for redosing.

BIOMARKER INITIATIVE

PPMD's Biomarker Initiative, launched in 2020 thanks to the support of the community, aims to validate the use of biomarkers as the most effective ways to monitor the progression of Duchenne, improve drug development, and analyze the effects of potential new therapies. Our targeted funding has yielded qualified safety biomarkers and advanced novel biomarkers being incorporated into current clinical trial design.

CARDIAC INITIATIVE

PPMD's Cardiac Initiative is part of our ongoing commitment to cardiac care and addressing cardiomyopathy. This effort is now focused on improving our understanding of progression and how we can develop meaningful outcome measures to explore novel cardiac therapeutics. Our \$2M in funding to ACTION and other carefully curated projects has led to harmonization in delivery of cardiac care and elucidated our understanding of cardiomyopathy. We've also supported near-term therapeutics to ensure novel therapies are explored to treat cardiomyopathy.

PPMD VENTURE PATHWAYS

PPMD Venture Pathways, launched in 2020, identifies promising therapeutic strategies and provides venture philanthropy investments to help companies complete critical studies needed to secure even more significant funding and advance investigational products to the clinic. Through this strategic effort, PPMD offers pivotal support and ensures the development of a robust pipeline of potential therapeutics to treat Duchenne and Becker. Through 2025 and into 2026 PPMD has invested in five companies, spanning multiple approaches to treat Duchenne and Becker.

THE
PPMD
JOURNEY

\$60+ MILLION Invested in Research



275+ Grants Awarded by PPMD



20+ Companies with Active Trials or Approved Products



8 U.S. Drug Approvals



6,000+ Citizen Scientists in The Duchenne Registry



ADVANCING PROMISING NEW TREATMENTS



PPMD partners with researchers, clinicians, and industry partners to support innovative therapies from the early, discovery stage of the process through approval and the complicated access environment that follows.



PPMD'S DUCHENNE DRUG DEVELOPMENT ROUNDTABLE

PPMD brings industry partners together to accelerate the development of meaningful treatments for all people living with Duchenne and Becker through collaborative efforts on initiatives. This allows us to reduce duplication and gain resource efficiencies to tackle many of the challenges in drug development, leading to faster therapeutic options for our community. Our focus in 2025 and 2026 has been on data sharing with patients and reducing clinical trial burden.



PRECLINICAL ASSESSMENT LABORATORY

PPMD supports the preclinical assessment of potential new therapies by replicating and confirming results, evaluating their effectiveness, and offering guidance to help drug developers prioritize which treatments can move into the clinic.



WELLSTONE CENTERS

For over 25 years, the Wellstone Muscular Dystrophy Cooperative Research Centers, initiated through advocacy efforts of the MD-CARE Act, have led the field not only in research, but also in the training and development of researchers leading the fight to end Duchenne and Becker. PPMD provides supplemental funding to Wellstone Centers and currently funds two Wellstone Centers across the country. The Wellstones have been directly involved in supporting research that has led to current approved therapies.



PPMD "THINK TANK" APPROACH

As the Duchenne and Becker therapeutic landscape rapidly evolves, PPMD thinks ahead and convenes with industry experts to tackle the next challenges in drug development. Current "think tank" areas include:

- **Using additive therapies** so we can apply the best learnings as we pursue combination therapies.
- **Evaluating the cardiac implications** of gene therapy.
- **Understanding the regulatory and infrastructure gaps** in developing personalized therapies for individuals with rare exon variants or non-sense variants through the use of Platform Designation.



DRUG DEVELOPMENT DATA MODELING COLLABORATIONS

PPMD provides funding to and participates in two collaborations to optimize drug development:

- **The Duchenne Regulatory Science Consortium (DRSC)** at the Critical Path Institute develops regulatory ready tools to accelerate clinical trials for new drugs to treat Duchenne.
- **The Collaborative Trajectory Analysis Project (cTAP)** develops prognostic models to explain variation in Duchenne symptom progression to accelerate drug development.



PEDIATRIC GENE THERAPY MEDICAL ETHICS

PPMD funds the Pediatric Gene Therapy and Medical Ethics Working Group (PGTME) and supports their mission to advance research, policy, and education, by promoting improved understanding of the challenges and best practices for ethical research across the evolving landscape of genetic therapies.

ACCELERATING RESEARCH THROUGH DATA



In order to advance research and develop treatments for all people living with Duchenne and Becker, we must first have a full picture of the dystrophinopathy spectrum to understand its complexities and how it affects each individual. Since our founding, PPMD has launched several initiatives to collect patient data with the goal of helping researchers and clinicians improve care and develop treatments faster.

THE DUCHENNE REGISTRY

The Duchenne Registry remains the largest patient-reported registry for Duchenne, Becker and carrier females. Over 6,000 people from more than 125 countries have joined since 2007, contributing over 18 years of data. Registry data has been exported for researchers over 80 times, and the Registry has been used to identify and connect people with Duchenne and Becker to more than 100 clinical trials and research studies.

PPMD's Registry team initiated three studies in 2025 utilizing our data. The first study was presented at the Newborn Screening Symposium in March and focused on analyzing the impact of age of diagnosis on the age of steroid initiation. The second study utilizing Registry data looked at how the diagnosis an individual is given may impact their care, and this was accepted as a poster presentation at the MDA Conference in March. The third study was done with our international partners at the TREAT-NMD Neuromuscular Network. This study analyzed the distribution of different variant types around the world, and was presented as a poster session at the World Muscle Society Congress in October.

We created a new group in 2025 called the Registry Champions. These individuals are actively engaged with the Registry and are helping to spread the word about the importance of participation, with the goal of growing Registry engagement within the community. The highlight of this effort was a community webinar we hosted in May.

In 2025, we also began a platform migration with THREAD Research, the vendor for the patient-facing side of our Registry. We are moving to a more robust app platform which will enhance the user experience, and we will be launching a new web portal for those users who prefer the web experience over the app.

PPMD AND REAL WORLD DATA

THE DUCHENNE OUTCOMES RESEARCH INTERCHANGE (THE INTERCHANGE)

The Interchange provides a central patient- and clinician-reported data warehouse so all types of Duchenne data from various sources, including The Duchenne Registry, academic consortia, real-world data from Electronic Health Records (EHRs), and post-market studies from industry partners, are collected and analyzed in one place. By de-siloing data, the Interchange delivers a more complete picture of Duchenne and Becker and helps clinical researchers and partners in therapy development address common research challenges. Thus, researchers can effectively conduct their work and accelerate scientific discovery and patient access to new therapies.

PPMD'S ELECTRONIC HEALTH RECORD (EHR) STUDY

The EHR Study uses advanced technologies to extract data from several of PPMD's Certified Duchenne Care Centers across the country to improve care and answer research questions without requiring additional effort from patients or healthcare professionals. Once a patient consents to participate in the study, key data fields collected during clinic visits are automatically and securely sent to the Interchange, where they can be combined with the patient's Registry data. Over 200 patients have consented to participate in the EHR Study.

COLLABORATION WITH CINRG

Through our novel role as the sole advocacy partner to the Cooperative International Neuromuscular Research Group (CINRG) expanded Duchenne Natural History Study (eDNHS), we are helping redefine Duchenne natural history in a rapidly evolving therapeutic landscape. By leveraging existing global study infrastructure, established clinical networks, and emerging technologies, the partnership is designed to maximize efficiency in longitudinal data collection while minimizing unnecessary duplication and burden on patients, families, and sites. This collaborative approach enables the integration of contemporary standards of care, therapeutic exposure, and real-world outcomes data to better characterize disease progression and support future research, regulatory, and clinical decision-making.

EXPANDING ACCESS TO CLINICAL TRIALS

Clinical trials allow people with Duchenne and Becker to access novel treatments, but data shows that there is a lack of racial, geographic, and socioeconomic representation in trials. As part of our mission to make sure every individual diagnosed can receive optimal treatment, PPMD aims to break down barriers, expand access, and improve representation in clinical trials.

COLLECTING DATA TO ASSESS TRIAL PARTICIPATION

In 2022, PPMD started collecting data to compare the demographics of people currently participating in Duchenne clinical trials versus those who are not. The data will help inform efforts to expand clinical trial eligibility for people of color, people from various geographic areas, and people of diverse socioeconomic status.

EXPLORE CLINICAL TRIALS

PPMD updated their online trial finder tool, Explore Clinical Trials, to provide automatic updates on clinical trials and research studies directly from ClinicalTrials.gov. This tool allows all individuals with dystrophinopathy to easily search for trials and studies that may be appropriate for them or their loved one.

ADDRESSING HEALTH DISPARITIES

PPMD continues to build on research to pinpoint where barriers exist and inform how to improve access to quality care, education, and engagement.

HONOR EVERY ADVOCACY JOURNEY



AS PART OF OUR MISSION to end Duchenne, we advocate for people diagnosed by leveraging government resources, building partnerships, and transforming policies to meet people's needs. No other organization specializing in rare genetic conditions has come so far in the realm of congressional and federal agency advocacy than PPMD. By raising our voices we've amplified awareness of Duchenne and Becker, advanced therapies, improved care, and championed legislation and regulatory policy leading to lasting change for the community.

RALLYING FOR DUCHENNE AND BECKER POLICIES & FUNDING

Our advocacy initiatives prioritize the Duchenne and Becker community's needs, securing federal funding for research and identifying gaps in care to make meaningful advances both in the field and the community. We educate members of Congress about Duchenne and Becker and build support for policies that address those gaps, like including more patient perspectives in the drug approval process. As a community, we've held over 2,500 meetings with members of Congress and written over 13,000 letters, and helped secure over \$850 million in federal funding for research. Through advocacy, PPMD is able to accelerate treatments to end Duchenne and Becker.

PPMD ADVOCACY CONFERENCE

PPMD's Advocacy Conference is the longest-running advocacy event for a rare genetic condition in the United States. In 2026, PPMD celebrated 25 years of Advocacy by hosting a Congressional Briefing and honoring our long standing congressional champions. The collective efforts of our champions and our advocates have culminated in the enactment of the Muscular Dystrophy Community Assistance, Research and Education (MD-CARE) Act in 2001 and two legislative updates in 2008 and 2014. Over two decades, advocacy efforts have secured over 850 million in federal funding, established a standard of care for all diagnosed, and accelerated a robust drug development pipeline.

THE BENEFIT ACT

In collaboration with congressional champions, PPMD conceived the Better Empowerment Now to Enhance Framework and Improve Treatments (BENEFIT) Act, bipartisan legislation aimed at prioritizing the patient voice in the FDA's review process. The legislation would disclose how patient experience or patient-focused drug development (PFDD) data is used by FDA when deciding whether or not to approve medications and treatments. This legislation builds on the Patient-Focused Impact Assessment Act that PPMD led to initiate the PFDD process at FDA.

FEDERAL FUNDING

Every year, PPMD Advocates from across the country head to the nation's capital to ensure the agencies that matter most to Duchenne and Becker are funded at appropriate levels. We also provide specific appropriations language to guide these agencies when making decisions about Duchenne and Becker programs and priorities. Our efforts have resulted in significant allocations, with over \$850 million dedicated to Duchenne and Becker. Notably, through the MD-CARE Act and PPMD's continued advocacy, the Centers for Disease Control and Prevention (CDC) receives around \$8 million per year, which has resulted in products like the Care Considerations. Following our annual advocacy efforts, the Congressionally Directed Medical Research Program at the Department of Defense allocates \$12.5M a year to fund Duchenne and Becker research projects, which was increased from \$10M in a year where many other health programs experienced drastic cuts. This funding has contributed to significant advancements in research, including the development of an FDA-approved therapy for Duchenne and Becker.

\$850 MILLION Leveraged in Federal Funding



13,000 Letters to Congress



4 Bills Signed into Law



THE
PPMD
JOURNEY

2,500 Meetings with Congress



2 States Currently Screening for Duchenne with Newborn Screening





HEALTHCARE REFORM

Alongside our colleagues in the patient advocacy community, PPMD fights for affordable, high-quality health care for people with Duchenne and Becker, including affordable out-of-pocket costs, coverage for pre-existing conditions, coverage for dependents over 26 years old, and the elimination of annual and lifetime benefit caps.

STATE ADVOCACY PROGRAM

Advocacy efforts at both the federal and state levels are crucial for addressing the diverse needs of the Duchenne and Becker community. In 2024, PPMD launched a pilot program aimed at advancing more targeted legislative progress for Duchenne in eight states. Learnings from this program will help PPMD expand state advocacy efforts across the country.

INFORMING REGULATORS THROUGH PATIENT EXPERIENCE

We believe patient experience is critical for regulators and the drug development process. Basic lab research is leading to breakthrough clinical trials, and PPMD is leveraging the Prescription Drug User Fee Acts and the 21 Century Cures Act to evolve patient input and advance the field of Patient-Focused Drug Development, including centering the priorities of the Duchenne and Becker community.

PATIENT STUDIES & DATA COLLECTION

PPMD conducts patient and caregiver preference studies to collect qualitative and quantitative evidence of their views on the benefits and risks of emerging therapies. Results from these studies show that patients and caregivers are willing to take a considerable risk for a therapy that stops or slows the progression of Duchenne. Findings from our studies have been useful in both the regulatory and therapeutic access to many of the FDA-approved drugs for Duchenne.

AWARENESS

PPMD works with government advocates and partner organizations to increase Duchenne and Becker's recognition and educate and engage the global community in combating Duchenne and Becker. Most recently, PPMD celebrated when the United Nations designated September 7th as World Duchenne Awareness Day.

FDA GUIDANCE SUBMISSION

PPMD submitted the first-ever patient-initiated draft guidance for Duchenne drug development to the FDA. The groundbreaking initiative was the foundation for the FDA's own draft guidance published in 2018 and provided a model for other rare disorder organizations. PPMD developed a revised community-led draft following advancements in research, care, and clinical trials and published the updated guidance document in 2024 for broad community access.

IMPROVING PATIENT ACCESS AND COVERAGE

PPMD accelerates the development of novel therapies, and as more treatments are approved, PPMD works to expand access to them, helping everyone diagnosed equitably attain the therapies they need. We also offer roadmaps so patients and their caregivers have essential information to build their understanding of the complexities around treatments and clinical trials and receive quality care.

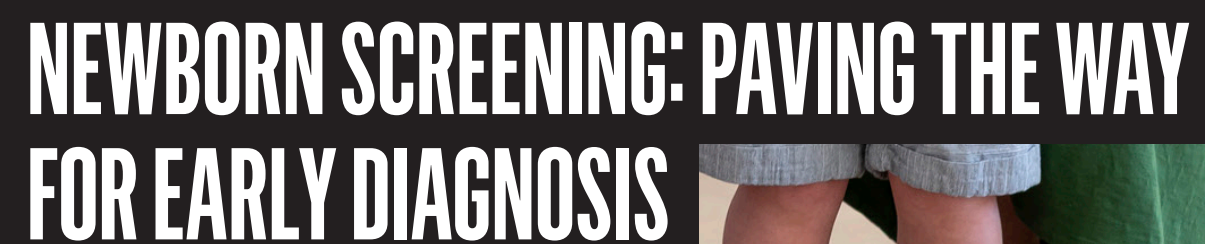
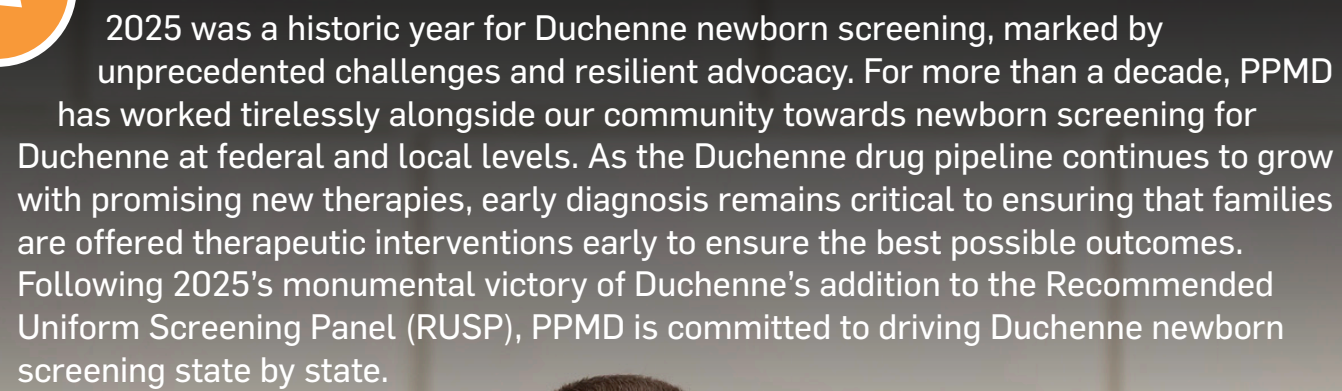
IMPROVED DISABILITY INCLUSION

PPMD collaborates with federal partners and other disability-focused organizations to advance legislation that promotes accessibility. Efforts include expanding access to telehealth services, enhancing airline staff training on handling wheelchairs and other mobility assistance devices, and improving Social Security Disability Income benefits. We continue to engage with the broader rare disease and disability community to drive further progress in this area.

IMPROVING HEALTHCARE OUTCOMES

An ICD-10 code specific to Duchenne and Becker enables PPMD to better identify patients eligible for novel therapies and clinical trials, longitudinally evaluate the impact of those therapies, improve quality of life, and increase life expectancy for those living with Duchenne and Becker.





In 2022, PPMD led the nomination of In 2022, PPMD led the nomination of Duchenne to the RUSP, the federal recommendation for which conditions all babies in the United States should be screened for at birth. In 2025, less than a month from making a decision, the Health Resources and Services Administration (HRSA) committee that reviews condition nominations and makes recommendations to the Secretary of the Department of Health and Human Services (HHS) was dissolved. PPMD and other advocates worked with HRSA and HHS to help the department complete and publish the Duchenne evidence review. EveryLife Foundation hosted a public workshop to

In December 2025, HHS reported that HRSA recommended adding Duchenne to the RUSP and the Health Secretary formally approved its addition on December 16, 2025. This rapidly paves the way for new states to consider Duchenne's addition to their state panels, including 13 states that have "RUSP-alignment" laws dictating a timeline in which they must review or add all RUSP conditions.

Prior to the RUSP addition, PPMd had already made strides in expanding access to Duchenne newborn screening in 2025. Beyond states that will move toward review or addition of Duchenne due to RUSP-alignment legislation, Duchenne newborn screening has now started in Ohio and Minnesota and is approved to start in New York, Arizona, Texas, Florida, Illinois, and Massachusetts. Additionally, in Indiana, South Dakota, Tennessee, and Nebraska, the state newborn screening advisory

Parent Project Muscular Dystrophy

HONOR EVERY CARE JOURNEY



STANDARDIZING CARE

In partnership with the Centers for Disease Control and Prevention (CDC), PPMD advocates for and participates in developing standards of care to help people with Duchenne live healthier, longer lives regardless of geography or socioeconomic status. The standards of care include recommendations across critical areas, from diagnostics to the transition of care into adulthood. These standards have played a pivotal role in extending the average lifespan by 10 years and guiding PPMD's network of Certified Duchenne Care Centers, helping to ensure that families receive optimal, standardized care and services.



About 15,000 male babies around the world are born with Duchenne each year. The evolution of Duchenne care has led to stronger, longer lives for all those living with the condition. PPMD works with Duchenne experts and partner organizations to identify gaps in care, develop standardized care practices, connect families and individuals to the best specialists, and improve quality of life. Over the past 30 years, our initiatives have advanced and expanded access to care, helping to increase the lifespan for people with Duchenne by 10 years.



CARRIER CARE

Duchenne carriers can pass the genetic variant to their children and exhibit symptoms such as muscle weakness and heart problems. To meet carriers' unique needs, PPMD invested \$50,000 in the University of Pennsylvania's Center for Inherited Cardiovascular Disease to support the development of the first dedicated carrier clinic for women with dystrophinopathy. The pilot clinic remains ongoing, continuing to refine and implement best care practices to carriers. Additionally, PPMD is actively working to build a network of providers interested and experienced with carriers to better support the community's needs.



ADULT CARE

Transitioning from adolescent to adult care varies widely by institution, state, and individual situation, underscoring the need for careful and comprehensive planning. PPMD's future work will focus on better understanding and addressing these differences. PPMD engages adult neuromuscular providers to strengthen the bridge between pediatric and adult care models and help teens and adults living with Duchenne and Becker access the care they need to live thriving, healthy lives. PPMD also works alongside individuals living with Duchenne and Becker to understand and support their goals for education, employment, housing, public transportation or driving, and finding and maintaining active social lives and relationships.

THE
PPMD
JOURNEY

10 Years added to average lifespan



39 Certified Duchenne Care Centers (CDCC)



4,700 Patients Receiving Care at a CDCC



750 Care providers in our CDCC network



15 Care Publications





DUCHENNE AND THE BRAIN

PPMD's Brain Initiative addresses barriers to obtaining diagnostic and therapeutic care for neurobehavioral conditions in Duchenne. We're developing care standards to ensure all people living with Duchenne have access to appropriate neurobehavioral care. To do this, we're expanding our understanding of neurobehavioral conditions, gathering clinician and caregiver insight, and establishing more effective ways to diagnose neurobehavioral conditions. In 2025, PPMD convened key experts from across the globe to understand advancements in the understanding of the brain and behavior and mapped out a path to continue to advocate for better neurobehavioral care, including continued funding of the BELS Tool to validate its use and bring accessible screening tools to patients regardless of where or how they receive care.

♥ CARDIAC INITIATIVE

Cardiac care is imperative for Duchenne, which is why it remains a critical element of PPMD's efforts to optimize care and research. With over \$7 million invested through our ongoing Cardiac Initiative, we're committed to advancing cardiac care standards. Since 2022, PPMD has hosted a series of 5 workshops aimed at advancing cardiac care standards and growing a network of robust cardiologists committed to the care of individuals living with Duchenne, including a \$2 million investment over three years to bolster our Cardiac Initiative and support the ACTION-DMD Network. This funding drives data-driven research across a network of sites, many of which are also Certified Duchenne Care Centers.

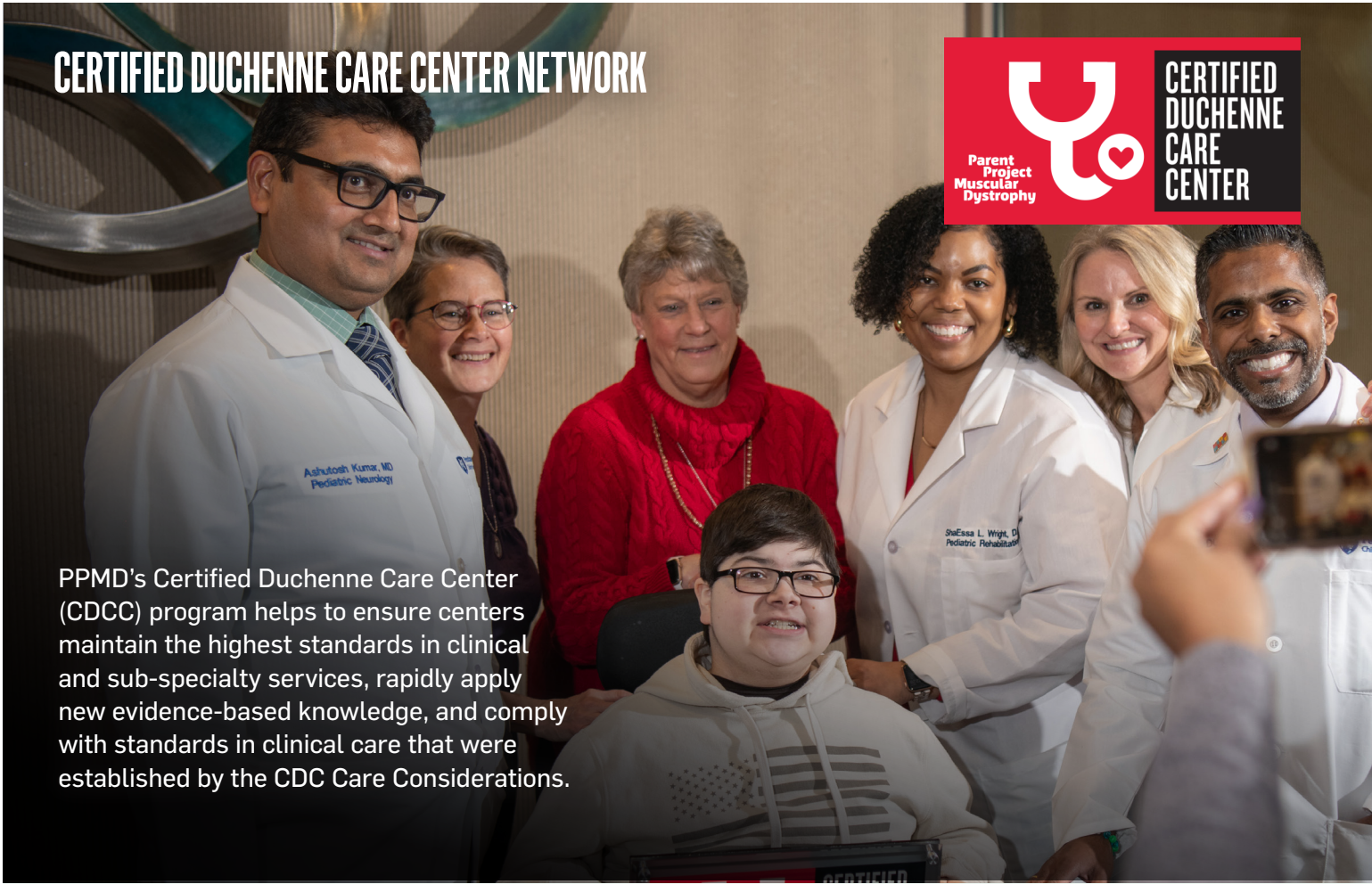


ENDOCRINOLOGY CARE INITIATIVE

Endocrine issues and weak bones are common among people with Duchenne, especially if they are taking steroids to treat their condition. PPMD is proud to support the OPTIMIZE-DMD Consortium, a network of endocrinologists across the globe focused on advancing endocrine standards of care in Duchenne. Since the establishment of the consortium in 2023, PPMD has partnered in the development of a series of manuscripts that will be published in 2026 and provide updated recommendations for care across five key endocrine-related domains. Additionally, an updated PJ Nicholoff Steroid Protocol was launched in 2025, building upon an existing effort to expand recommendations for steroid stress dosing, which can save lives in times of illness or injury. In an effort to maximize reach, the protocol has been translated into Spanish and Italian, with additional translations currently underway.

OPTIMIZING CARE FOR EVERY JOURNEY

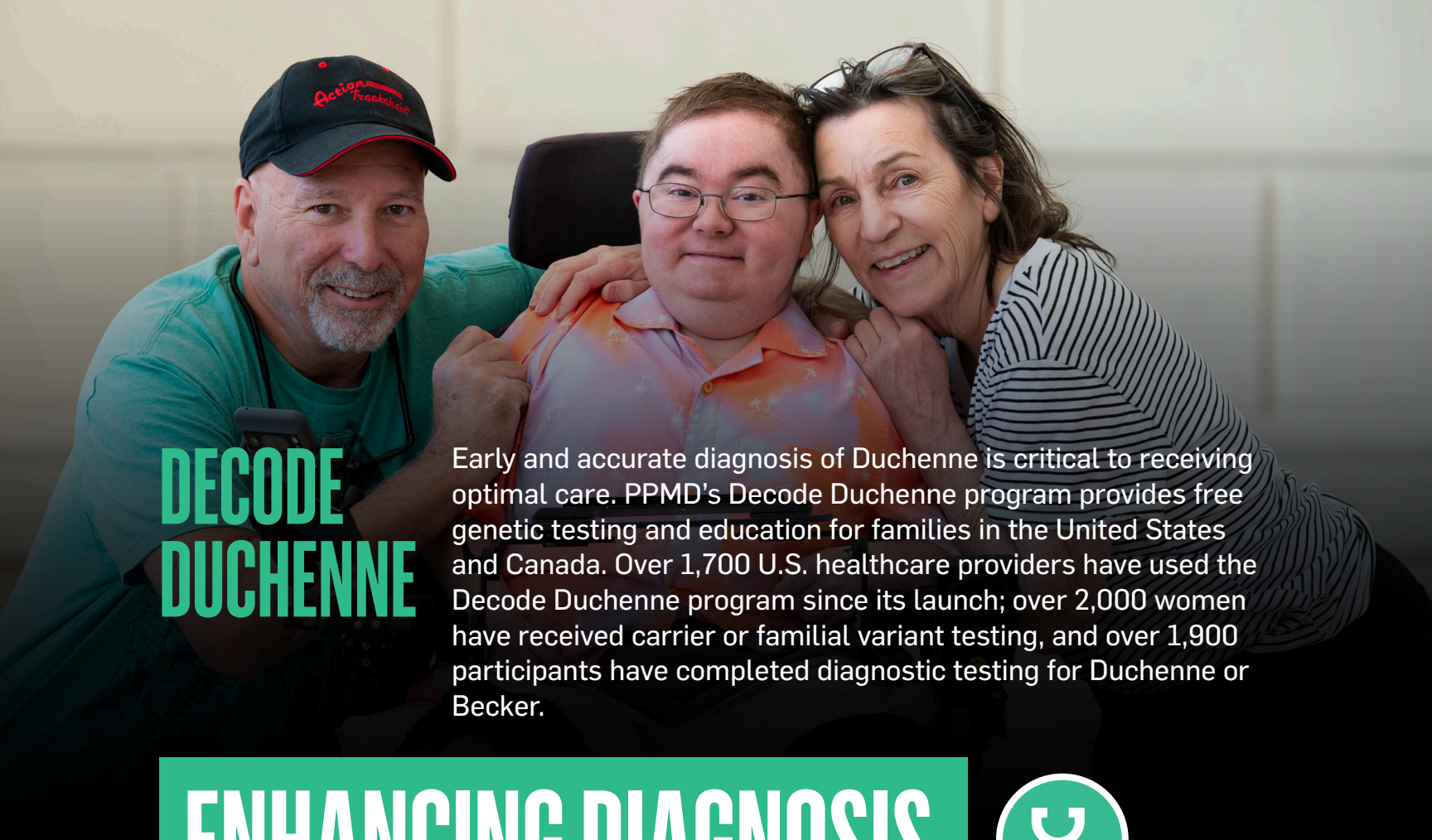
Every person living with Duchenne and Becker deserves the best care possible. PPMD helps families access expert healthcare providers, sub-specialists, and cutting-edge treatments so that families can feel confident knowing they are receiving optimal, standardized care and services that align with the Duchenne Care Guidelines.



PROGRAM IMPACT

Since its inception more than 10 years ago, PPMD's Certified Duchenne Care Center program has grown, encompassing 39 care centers across the country, working with 750 care providers with significant knowledge of Duchenne, and serving more than 5,000 patients with the best care possible. Healthcare providers at CDCCs are available to provide medical advice, answer questions about clinical trials, and support the Duchenne community.

2025 also saw the launch of the CDCC Program's Clinical Research Designation, a new optional designation for centers to reflect ongoing participation in research at the institution. The addition of this designation will allow for both recognition of centers who engage in clinical research for Duchenne and Becker, and help families know what to expect from their neuromuscular team.



DECODE DUCHENNE

Early and accurate diagnosis of Duchenne is critical to receiving optimal care. PPMD's Decode Duchenne program provides free genetic testing and education for families in the United States and Canada. Over 1,700 U.S. healthcare providers have used the Decode Duchenne program since its launch; over 2,000 women have received carrier or familial variant testing, and over 1,900 participants have completed diagnostic testing for Duchenne or Becker.

ENHANCING DIAGNOSIS



GENETIC TESTING

PPMD's partner laboratory, Revvity Omics, provides genetic testing to detect deletions, duplications, and smaller point variations in the DMD gene through next generation sequencing (NGS). The program also provides targeted testing for individuals with a positive family history, and repeat testing for individuals tested with older molecular technologies.

GENETICS EDUCATION AND SUPPORT

PPMD's genetics team is available to both ordering providers and families to answer questions about the process of genetic testing, along with connecting them to resources and support for positive test results.

CARRIER OR ASYMPTOMATIC FAMILIAL VARIANT TESTING

Decode Duchenne offers free familial variant testing to individuals who are asymptomatic and have a relative with Duchenne or Becker or have a family member with a known variant from previous genetic testing.

IDENTIFYING SIGNS OF DUCHENNE

To help drive an earlier diagnosis for Duchenne and other neuromuscular conditions, PPMD supports the ChildMuscleWeakness.org website, a resource for pediatric healthcare professionals to assist them in evaluating a child with muscle weakness and developmental delays. The website's tools, including an algorithm and videos, help guide clinicians so a neuromuscular disorder such as Duchenne can be diagnosed as early in childhood as possible.

CONNECTING FAMILIES WITH RESOURCES

A diagnosis of Duchenne, Becker, or as a carrier can leave families & individuals feeling overwhelmed, isolated and uncertain. PPMD connects families to a wide range of trusted resources, developed by leading care experts and community advocates, designed to support & honor every journey.

EDUCATIONAL RESOURCES

PPMD offers a wide variety of care resources available to order or download on our website, giving families everything they need to learn more about Duchenne and Becker, adjust to the diagnosis, find support, understand up to date care recommendations, medications & therapies and explore care options.



PPMD FOR YOU

PPMD supports every family and individual throughout their dystrophinopathy journey — from initial diagnosis to questions about genetics and clinical trials, and navigating the school system or home modifications. In 2025, our expert staff met with over 120 families and individuals to provide support surrounding the complexities of Duchenne and Becker and navigating daily life.

UPDATED PJ NICHOLOFF STEROID PROTOCOL

With the ever changing Duchenne landscape and development of new therapies, PPMD, alongside OPTIMIZE-DMD, released the updated PJ Nicholoff Steroid protocol - an even more comprehensive version of the original protocol that incorporates a decade of learning, collaboration, and feedback from families and clinicians alike. PJ's Protocol is designed to provide clinicians with critical information for managing emergency situations involving patients on chronic corticosteroids.

ACCESSIBILITY

PPMD strives to ensure every family has access to educational resources and support. We continue to translate care resources into a variety of languages to ensure families have access to resources & dystrophinopathy education.

ACCESS RESOURCES FOR APPROVED THERAPIES

PPMD has developed resources to help the Duchenne community understand which therapies have been FDA approved and gain favorable access to these therapies. The resources provide accurate information and guidance on navigating the healthcare system and finding the right path to new medications.

INCLUSIVE EMERGENCY RESOURCES

Emergency care recommendations are critical for anyone with dystrophinopathy (Duchenne, Becker and carriers); which is why we have updated our emergency care resources to be inclusive for all individuals with dystrophinopathy. This updated terminology aims to remove barriers for those in emergency situations and provide critical guidance for healthcare professionals who may not be familiar with care recommendations.

COLLABORATING TO CLOSE GAPS IN CARE



PPMD convenes researchers, industry partners, and experts in Duchenne and Becker to share learnings, navigate challenges, and explore solutions that push the Duchenne and Becker landscape forward and continually improve care for every person and every family with Duchenne and Becker.

CARE CONSENSUS MEETINGS

Hosted by PPMD and expert committees, Care Consensus Meetings bring together the brightest minds in Duchenne to identify and assess gaps in care, and further our education and understanding of the complexities of Duchenne. PPMD and Duchenne experts aim to answer the most urgent questions from the patient and family community and publish recommendations on improving care or standards of practice in Duchenne. PPMD's most recent areas of focus include care related to the heart and lungs, brain and behavior, and endocrinology and obesity.

DUCHENNE PROFESSIONALS MASTERCLASS & CEUS

In 2025, PPMD launched a completely revamped Duchenne Professionals Masterclass, offering twelve stand-alone, self-paced continuing education courses. This program enables healthcare professionals to build a personalized curriculum tailored to their specific learning needs, whether they are new to Duchenne care or experienced specialists in the field. In addition, the Duchenne Educators Masterclass is available to community educators, equipping them with the tools to promote a safe and inclusive learning environment.

DYSTROPHINOPATHY CARE LANDSCAPE ANALYSIS

Beginning in 2025, PPMD convened a series of working groups across 13 domains of care to conduct a comprehensive analysis of care in the current and rapidly evolving landscape. Leveraging the 2026 Healthcare Professionals Summit, working groups, CDCC physician leaders and other key personnel gathered in New York City for three days of collaboration to refine draft recommendations for care in the current care environment. Work continues across each of these working groups, with a set of 14 manuscripts set to be published in Muscle & Nerve as a special issue in early 2027.



PPMD HEALTHCARE PROFESSIONALS SUMMIT

Early in each new, PPMD convenes hundreds of healthcare professionals in the Duchenne and Becker space to address real-time gaps in care and potential solutions. In early 2025, PPMD convened over 360 healthcare professionals, researchers, and industry partners, including representatives from the FDA, NIH, CDC, and international representatives in one room to discuss the latest updates in dystrophinopathy care and research. Additionally, 30 adult care providers were in attendance, growing PPMD's reach into the adult care landscape. Key topics included gene therapy, developing clinical trials that work, approved therapies and care throughout the lifespan, all in an effort to move the needle forward in dystrophinopathy care and research.

HONOR EVERY JOURNEY COMMUNITY



COMMUNITY IS AT THE HEART of what we do. No one affected by Duchenne or Becker muscular dystrophy should have to go through the journey alone. PPMD honors every family and individual by connecting them with the resources they need to build a network of support and surround themselves with people who understand their hopes and fears. We foster a community of families, friends, and advocates who are committed to honoring every journey by walking alongside one another so no one has to navigate the complexities of this diagnosis alone.

PPMD is committed to honoring every journey because no two diagnoses, no two trajectories, and no two families are the same. Rather than taking a one-size-fits-all approach, we have made it our mission to meet the diverse and evolving needs of the entire Duchenne and Becker community.



PPMD'S ANNUAL CONFERENCE

Convening the community has been central to PPMD's mission since our inception. Each year, PPMD brings together the Duchenne and Becker community through our Annual Conference, the largest and most comprehensive international gathering dedicated entirely to Duchenne and Becker.

This conference provides a vital opportunity for families, caregivers, researchers, healthcare professionals, industry partners, and individuals living with Duchenne and Becker to come together. Attendees learn about the latest research, standards of care, clinical trials, and important updates shaping the future of the community.

Equally important, it is a space where our community can connect on a deeper level, sharing hopes and fears, building relationships, and working together to address the most urgent needs facing those affected by Duchenne and Becker.

PPMD CONNECT

Our official family outreach program, PPMD Connect, was created to support everyone affected by Duchenne and Becker. Launched in 2008, we now have groups serving 36 states, providing resources, community, and opportunities to engage in advocacy and fundraising efforts and connect in person or online. We also have a group specifically for Siblings to connect, forge relationships, and support one another in their unique journey.

NEWLY DIAGNOSED

No one should feel alone after receiving a diagnosis of Duchenne or Becker. PPMD supports newly diagnosed families through dedicated resources & connections, one-on-one personal meetings with the care & genetics team via our PPMD For You program, quarterly virtual meet & greets, and our Newly Diagnosed Families track at our Annual Conference. Our team provides several touchpoints for new families to ensure they receive the necessary resources to build a support network they can rely on throughout their journey with Duchenne or Becker.

THE
PPMD
JOURNEY

31

PPMD
Connect
Groups



70

PPMD Adult
Advisory
Committee
Members



40+

Fundraising
Events
Annually



\$47+ MILLION

Community
Fundraising



17,000

Families Reached
Through PPMD
Gatherings





COMMUNITY FUNDRAISING & DEVELOPMENT

Thirty years of progress is possible because of the support from our community and donors who are committed to make lasting change for people with Duchenne and Becker. To date, community fundraising efforts have resulted in over \$475 million to advance therapies and improve care.



PPMD'S ADULT ADVISORY COMMITTEE (PAAC)

This group places patients at the center. Amplifying the voices of teenagers and adults living with this rare disease, we advocate in Washington D.C., program year round community outreach (nothing about us without us!) run social groups, and confer with pharmaceutical companies to make the adult voice heard. We are the adult population standing on the shoulders of those that came before us, and educating those that come after us.

VIRTUAL SOCIALS

PPMD brings groups together to share experiences and support one another in their journeys with Duchenne and Becker. Grandparents, Carriers, Dads, and Tweens meet virtually throughout the year to socialize, connect, and interact with experts on a variety of relevant topics.

PPMD LIGHTHOUSE

The Lighthouse Workshop series were developed by PPMD to provide a safe and supportive space to build stronger connections and deeper insights about life and identity that go beyond diagnosis and management.

PPMD TOGETHER

PPMD hosted regional meetings in Minneapolis, MN and Charlotte, NC, in 2025 and Philadelphia, PA in early 2026, bringing families and community members together through connection and shared knowledge & experiences. We will end the 2026 series in San Francisco, CA in the fall followed by a virtual meeting in the winter. The PPMD Together meeting series offers the Duchenne and Becker community an opportunity to address evolving and dynamic needs by fostering collaboration between families, individuals, industry partners,

PPMD'S LIVING DUCHENNE

PPMD's podcast brings together the Duchenne community to share their experiences. In each episode, PPMD highlights individual voices and hears from Duchenne experts as they explore topics that impact families on a daily basis, and provide insight, guidance, and wisdom about living Duchenne.



RACE TO END DUCHENNE

Through PPMD's signature endurance fundraising program, anyone can friends and family take action to end Duchenne by racing in cities all over the country. Since 2005, over 76,000 participants have raised more than \$175 million to accelerate treatments and improve care, and have had a great time doing it!



COACH TO CURE MD

Started in 2008 as a partnership with the American Football Coaches Association (AFCA) and PPMD to raise awareness and funds to tackle Duchenne, Coach To Cure MD has expanded to include sports beyond football. Families are encouraged to promote community involvement in the fight to end Duchenne by holding fundraising events, going to games to cheer on their favorite teams, and serving as ambassadors of Coach To Cure MD.



DIY FUNDRAISING

Across the Duchenne and Becker community, families, friends, and caregivers engage in Do-It-Yourself Fundraising efforts – from bake sales to golf tournaments – to help make meaningful change. DIY Fundraising has produced over \$27 million towards advancing PPMD's mission to end Duchenne.



JOIN US

From our Race to End Duchenne program to our Do-It-Yourself Fundraising efforts to becoming a monthly donor, there are lots of ways for families and loved ones to lend a hand and raise funds and awareness. \$0.85 of every dollar raised directly supports PPMD's projects and initiatives, moving the landscape forward and achieving meaningful change for every individual living with Duchenne and Becker.

