Parent Project Muscular Dystrophy 30

30

years

COMMUNITY

PPMD Hosts 1st Annual Conference

PPMD is born. Our first Annual Conference is held, connecting families with clinicians, researchers, and stakeholders to advance our mission to end Duchenne.

RESEARCH

PPMD Invests in 1st Duchenne Research Center

1995

This investment breaks down barriers and encourages the research community to build ideas, test theories, and motivate each other.

CARE

Steroids Become Gold Standard

1998

ADVOCACY

1st Annual Advocacy Conference

PPMD and the Duchenne community advocate in Washington D.C., cultivating ties with elected officials who become champions for Duchenne on Capitol Hill. Annual efforts have helped leverage $800 million+ in federal funding for Duchenne-related programs.

ADVOCACY

MD-CARE Act Signed into Law

2001

Our community achieves a stirring victory: the first legislation in the history of the U.S. Congress focused on muscular dystrophy.

RESEARCH

PPMD Funds 1st Drug Discovery Program

2003

PPMD's leadership continues to transform the research landscape. Today our community has eight approved therapies and 35+ ongoing clinical trials. PPMD has invested $55 million+ into research to date.

RESEARCH

PPMD Launches 1st Duchenne-Specific Registry

The Duchenne Registry gathers data and drives research, and has connected patients to 175+ clinical trials and research studies over 15+ years. It empowers families to be citizen scientists, advancing research and understanding of the disorder.

1st Industry-Sponsored Duchenne Clinical Trial

CARE

Duchenne Standards of Care Are Published

2010

PPMD spearheads significant advancements in Duchenne care, collaborating with the Centers for Disease Control and Prevention (CDC) to advocate and participate in the development of standards that improve quality of life.

COMMUNITY

PPMD Creates Family Outreach Program

2008

PPMD's Connect now has 30+ groups across the country. Our engagement initiatives unify, amplify, and support the voices of every individual within our community.

COMMUNITY

PPMD Adult Advisory Committee Established

The PAAC amplifies the teen and adult voices of the Duchenne and Becker community as an extension of PPMD.

ADVOCACY

FDASIA Signed into Law

2012

PPMD helps lead the way for groundbreaking legislation: the Food and Drug Administration Safety and Innovation Act (FDASIA), which mandates that the FDA take a more structured and transparent approach to incorporating the perspectives of patients and families.

CARE

PPMD Launches Free Genetic Testing Program

2013

PPMD's Decode Duchenne program offers essential diagnostic and carrier testing. With existing and possible new therapies that rely on precise genetic information, understanding the genetic changes in each family is crucial.

ADVOCACY

PPMD-Led Draft Guidance Submitted to FDA

2014

Together, we submit to the FDA the first-ever patient advocacy-initiated Duchenne Guidance to accelerate drug development. The FDA later finalized its own Duchenne Guidance, expanding the drug development pipeline.

CARE

PPMD's 1st Certified Duchenne Care Center

This program makes comprehensive care and services even more accessible for families, with 35+ care centers currently certified across the country.

ADVOCACY

Specific ICD 10 Code Established

2018

The ICD 10 code for Duchenne and Becker eliminates barriers to diagnosis, care, surveillance, research, and access.

FDA Publishes Guidance

RESEARCH

1st Duchenne Patient Dosed in Gene Therapy Trial

ADVOCACY

21st Century Cures Signed into Law

PPMD drives efforts to advance the development and use of patient experience data, supporting provisions requiring the FDA to disclose how patient experience data is factored into the review of all approved products.

RESEARCH

1st U.S. Drug Approval for Duchenne

PPMD's Gene Therapy Initiative Launches With $2.2 Million Grant

This initiative aims to accelerate gene therapy's potential for treating Duchenne. Since launch, PPMD has invested $7 million+ in gene therapy approaches, including early research that resulted in the first approved Duchenne gene therapy.

COMMUNITY

Progress & Support Amid the Pandemic

PPMD unites the community virtually during the pandemic, offering support and education via virtual conferences and social events. We also ensure continued progress in the Duchenne landscape by developing bridge grants to serve as emergency temporary funding sources to support key projects.

CARE

PPMD Expands Cardiac Initiative With $2 Million Grant

Reinforcing our dedication to addressing cardiac issues in Duchenne, we announce our commitment of $2 million to support the Advanced Cardiac Therapies Improving Outcomes Network (ACTION). Overall, PPMD has invested over $7 million to manage and prevent heart failure in Duchenne through our Cardiac Initiative.

RESEARCH

PPMD Launches Electronic Health Record (EHR) Study

PPMD launches study to combine data from The Duchenne Registry with EHR data from clinics in one central place, the Duchenne Outcomes Research Interchange.

PPMD Celebrates 30 Years of Progress

PPMD commits to fighting for every future, until the day 100% of those diagnosed have access to a treatment to end Duchenne.

RESEARCH

1st Gene Therapy Approval

Our community's unwavering determination leads us to an extraordinary achievement: the accelerated approval of the first gene therapy for Duchenne. It's a moment of celebration, but we haven't reached the finish line yet.

ADVOCACY

Duchenne Newborn Screening Milestone

With universal newborn screening for Duchenne approved at the state level in Ohio, New York, and Minnesota, PPMD continues to work towards inclusion on the federal Recommended Uniform Screening Panel (RUSP) and promoting newborn screening for Duchenne in other states to ensure that all babies have the same opportunities for diagnosis and care.

Pat with her sons, Chris and Patrick

In 1984, the word “Duchenne” entered PPMD founder Pat Furlong’s world, a life-changing moment when doctors could only offer the advice to go home and love her sons, because they believed nothing more could be done.

Since PPMD’s founding in 1994, we’ve made significant progress together as a community. PPMD has tirelessly pursued research and worked to unravel the complexities of Duchenne and Becker muscular dystrophy. While there have been many moments to celebrate, we are not yet at the finish line. There is an urgent need to further accelerate the development of new therapies and increase access so that everyone can get these treatments.

We are committed to *Fighting for Every Future.*

Parent Project Muscular Dystrophy 30

DEAR DUCHENNE COMMUNITY,

In 1994, a small group of parents refused to accept “there is no hope and little help.” We united to take action, determined to fight against Duchenne, advocating for our children and making sure Duchenne became a priority for legislators and researchers across the globe. Thirty years later, novel therapies that were just a dream are real treatments improving both the quantity and quality of life for people living with Duchenne and Becker. This progress is a testament to PPMD’s unwavering commitment to advancing research, shaping policy, improving care, and connecting the community. Together, we’re fighting for every future.

As I reflect on all the work we’ve accomplished together, I know that every win — no matter how small or large — brings us closer to a world where all people living with Duchenne and Becker can access therapies. Since our founding, PPMD has invested tens of millions of dollars in cutting-edge research, convened experts across the field, and witnessed eight FDA approvals for Duchenne therapies. We’ve also celebrated 15 years of data collected through The Duchenne Registry, which enables the community to become citizen scientists and connects patients and families with research studies and clinical trials. In our efforts to ensure that all babies have the same opportunities for early diagnosis and optimal care, we’ve made significant advances in newborn screening, which is now approved at the state level in New York, Ohio, and Minnesota. We are at a critical point in our fight to end Duchenne, and we must continue to build on this momentum to accelerate therapies for every person living with Duchenne and Becker.

Each year, we see record attendance at our Annual Conference, fundraising events, and meetings, and I am thankful for the resilient community we’ve built. Through our collective power, we’ve navigated many challenges and achieved significant milestones, transforming the Duchenne landscape and creating real change for families. While there is much more work yet to do, I am immensely proud, deeply grateful, and I know that as a community, we will continue to be forward-thinking and remain steadfast in our commitment to fight for every future and end Duchenne.

Sincerely,

Pat Furlong

Founding President & CEO

30 YEARS OF FIGHTING *for every future*

THE PPMD WAY

Parent Project Muscular Dystrophy (PPMD) fights to end Duchenne. We accelerate research, raise our voices to impact policy, demand optimal care for every single family, and strive to ensure access to approved therapies. Through our initiatives, we listen to our community's needs and address gaps in the field so everyone diagnosed can live stronger lives. In the last 30 years, we've played a key role in many significant victories for the Duchenne community, including securing millions of dollars in funding for research and clinical trials and winning eight FDA approvals. Treatments like gene therapy are possible because of PPMD's dedication and tireless work. Together with our community, we will continue to fight for every future, 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. PPMD identifies these gaps and invests in the most promising therapies to accelerate potential treatments for all people with Duchenne and Becker.

ADVOCACY

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

CARE

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

COMMUNITY

No one should have to face Duchenne and Becker alone, and we know that no one person, company, or organization will end Duchenne. PPMD builds community through various programs and events, connecting individuals and families to learn from and support each other. Together, we will end Duchenne.

Research FOR EVERY FUTURE

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 for every single person diagnosed. To achieve this, we invest in cutting-edge research to address the full impact of Duchenne, no matter the variant, age, or stage of progression. To date, we've invested over $55 million to support novel and emerging therapies that bring hope to our community.

GENE THERAPY INITIATIVE

PPMD has supported gene therapy as a potential therapeutic for Duchenne since its early stages, funding over $7 million in research to better understand gene therapy and related approaches, as well as its effects on the heart and immune system. In 2023, our collective community celebrated the first accelerated approval of a gene therapy for Duchenne. PPMD is proud to have provided a $2.2 million research grant to Dr. Jerry Mendell at the Abigail Wexner Research Institute at Nationwide Children's Hospital that led to the development of this therapy.

BIOMARKER INITIATIVE

PPMD's Biomarker Initiative 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.

CARDIAC INITIATIVE

PPMD's Cardiac Initiative is part of our ongoing commitment to cardiac care, with over $7 million invested into research pertaining to the prevention and monitoring of 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.

PPMD VENTURE PATHWAYS

PPMD Venture Pathways 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.

THE PPMD EFFECT

$55+ MILLION PPMD Invested in Research

275+ Grants Awarded by PPMD

20+ Companies with Active Trials or Approved Products

8 U.S. Drug Approvals

5,500 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 our community's first approvals 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 pre-competitive 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.

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 20 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. PPMD provides supplemental funding to Wellstone Centers and currently funds three Wellstone Centers across the country focused on expanding our understanding of the role of microdystrophin in the heart and the role of steroids to modify muscle metabolism.

PPMD "THINK TANK" APPROACH

As the Duchenne 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.
- **Establishing a master protocol** to accelerate clinical trials by standardizing processes and maximizing flexible trial design to expand eligibility criteria and quickly evaluate a drug's effectiveness.

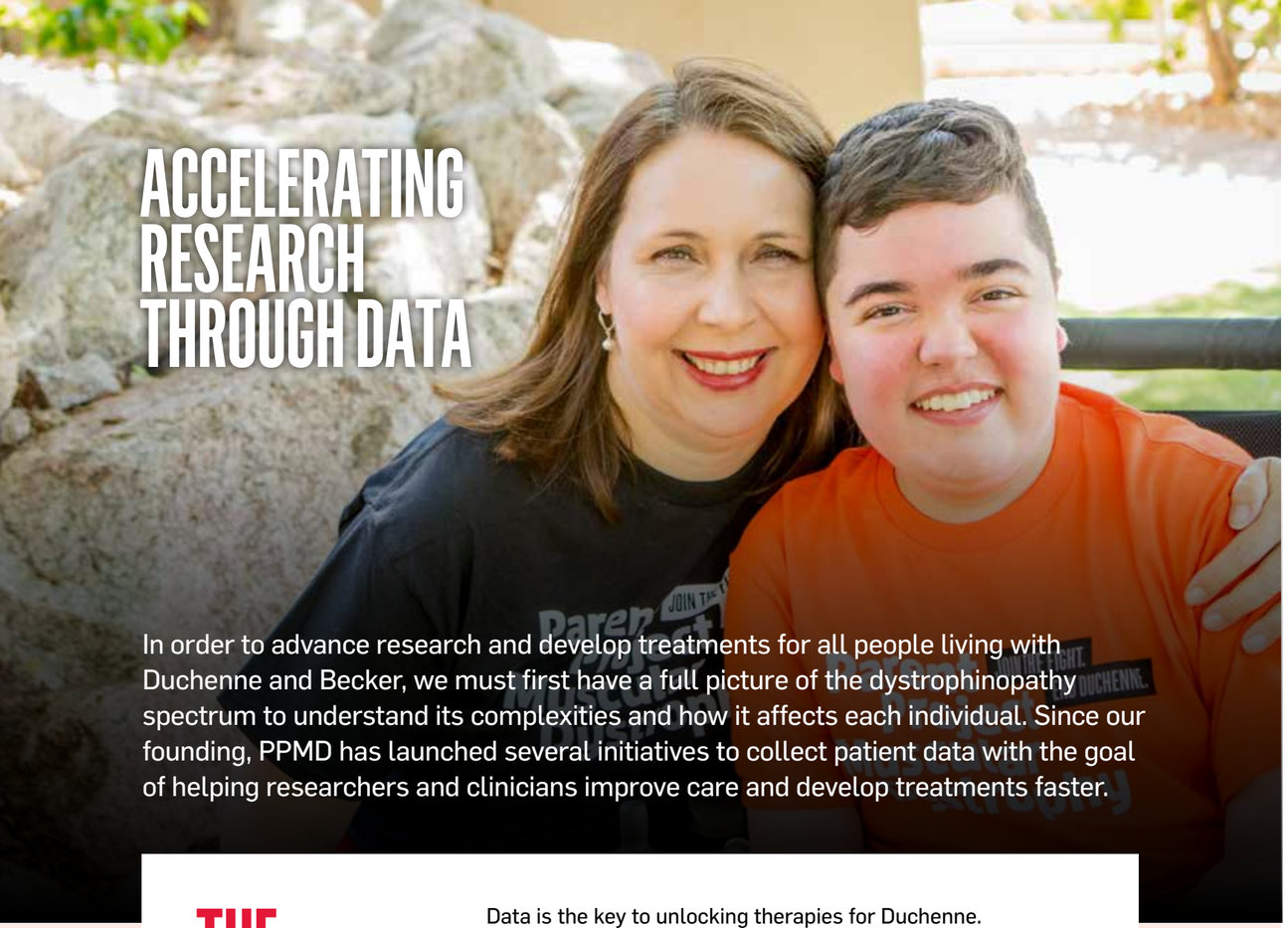
PEDIATRIC GENE THERAPY MEDICAL ETHICS

PPMD funds the Pediatric Gene Therapy and Medical Ethics Working Group 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.

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.



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

Data is the key to unlocking therapies for Duchenne. The Duchenne Registry is the world's largest, most comprehensive patient-reported registry for Duchenne and Becker muscular dystrophy. When families join the Registry, their de-identified data provides academic and industry researchers insight into the patient perspective and helps speed the clinical trial process.

CELEBRATING 15 YEARS

The Registry is powered by over 15 years of robust data shared by patients and families with Duchenne and Becker, and carriers. Since its launch, data has been referenced in 13 publications and exported for researchers 65 times. The Registry has also been used to identify and connect people with Duchenne and Becker to over 80 actively recruiting trials.

WHAT'S NEXT

PPMD has upgraded the Registry to be more accessible by creating a Spanish version of the app available on iOS and Android. A web portal is also being created for users who prefer using their desktop over a mobile app.

THE DUCHENNE OUTCOMES RESEARCH INTERCHANGE (THE INTERCHANGE)

The Interchange provides a central patient- and clinician-reported data warehouse so all types of Duchenne data and 'real world evidence' from various sources, including The Duchenne Registry, Electronic Health Records (EHRs), academic studies, and 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 efficiently 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 doctor visits are automatically and securely sent to the Interchange, where they can be combined with the patient's Registry data.

DIVERSITY, EQUITY, INCLUSION: 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.

UNDERSTANDING THE POPULATION GENETICS OF DUCHENNE

PPMD supports data-driven collaborations to better understand the relationship between the population genetics of Duchenne and its prevalence among racial groups.

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 through various diversity, equity, and inclusion (DEI) initiatives.

COLLABORATING WITH THE CDC

PPMD partners with the Centers for Disease Control and Prevention (CDC) to conduct a prevalence study using the data from the CDC's Muscular Dystrophy Surveillance, Tracking, and Research Network (MD-STARnet) which collects data on Duchenne patients from several states via medical chart review in order to get a more complete picture of Duchenne and improve care.

Advocacy FOR EVERY FUTURE

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, advanced treatments, improved care, and championed legislation and regulatory policy leading to lasting change for the community.

RALLYING FOR DUCHENNE POLICIES & FUNDING

Our advocacy initiatives prioritize the Duchenne 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. Through advocacy, PPMD is able to accelerate treatments to end Duchenne.

PPMD ADVOCACY CONFERENCE

PPMD's Advocacy Conference is the longest-running advocacy event for a rare genetic condition in the United States. Each year, we convene the Duchenne community in Washington, D.C., to ensure Duchenne and Becker muscular dystrophy are a congressional priority. Our collective efforts 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 millions 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 are funded at appropriate levels. We also provide specific appropriations language to guide these agencies when making decisions about Duchenne programs and priorities. Our efforts have resulted in significant allocations, with over $800 million dedicated to Duchenne. 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 $10M a year to fund Duchenne research projects. This funding has contributed to significant advancements in research, including the development of an FDA-approved therapy for Duchenne.

THE PPMD EFFECT

- **$800 MILLION** Leveraged in Federal Funding
- **13,000** Letters to Congress
- **4** Bills Signed into Law
- **2,500** Meetings with Congress
- **3** States 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, 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.

AWARENESS

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

STATE ADVOCACY PROGRAM

Advocacy efforts at both the federal and state levels are crucial for addressing the diverse needs of the Duchenne 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 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.

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.

FUELING THE FIGHT THROUGH *Advocacy*

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.

ESTABLISHING AN ICD-10 CODE

PPMD led efforts to create a distinct International Classification of Diseases (ICD) code for Duchenne and Becker, which expands access to therapies and improves diagnosis, care, surveillance, and research.

ENSURING ACCESS & COVERAGE

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

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.

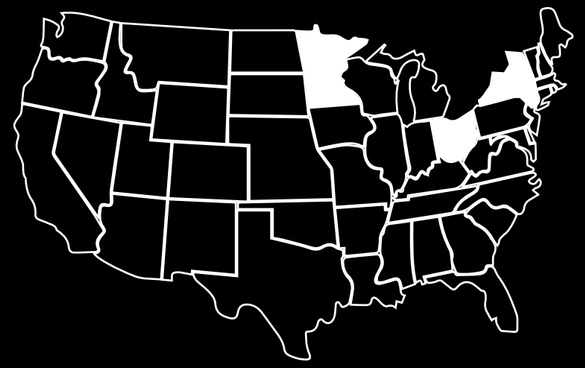
NEWBORN SCREENING: PAVING THE WAY FOR EARLY DIAGNOSIS

Right now, the Duchenne drug pipeline is full of promising new therapies, and because of the progressive nature of Duchenne, it's imperative that people diagnosed are offered therapeutic interventions as early as possible to ensure the best possible outcomes. For over a decade, PPMD has spearheaded efforts to establish nationwide newborn screening for Duchenne to address this need. Newborn screening aims to prevent families from experiencing diagnostic delays and ensure that every family receives timely, supportive, accurate resources at the time of diagnosis.

NEWBORN SCREENING PILOT

In 2019, PPMD launched a pilot newborn screening program in New York. The program screened infants born at high birth rate hospitals and validated the need for early diagnosis and intervention in Duchenne. The pilot was completed in 2022 and PPMD submitted a nomination package to the Recommended Uniform Screening Panel (RUSP), requesting that Duchenne be a condition recommended for nationwide newborn screening. In 2023, the package moved into evidence review.

NEWBORN SCREENING POLICY UPDATES



PPMD has made significant advances in newborn screening, which is now approved at the state level in Ohio, New York, and Minnesota. We continue to engage in states where newborn screening legislation is progressing. At the federal level, we are working with our partners at the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) during the ongoing evidence review process.

Care FOR EVERY FUTURE

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.

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.

EARLY/NEWBORN CARE

PPMD prioritizes early detection and newborn screening for Duchenne, ensuring timely diagnosis and comprehensive care access. In January 2023, PPMD convened a Duchenne Early Care meeting to discuss optimal care for infants and toddlers, aligning with expanded federal and state newborn screening efforts. This meeting also explored new ways to support families and parents navigating early diagnoses. A published report summarizes these discussions, facilitating broader learning among healthcare providers. Emphasizing the necessity of specific care guidelines for babies and toddlers, PPMD is committed to collaborating with clinicians leading this field to advance the development of these standards.

CARRIER CARE

Duchenne carriers can pass the genetic variant to their children and exhibit symptoms of Duchenne like 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 clinic provides and develops standardized care practices to improve the long-term physical and emotional health of carriers. PPMD hopes this pilot clinic will lead to a network of care centers for carriers.

ADULT CARE

Transitioning from adolescent to adult care requires careful and comprehensive planning. PPMD engages adult neuromuscular providers to streamline the bridge between pediatric and adult care models and help teens and adults living with Duchenne and Becker get the care they need to have thriving, healthy lives. PPMD also works alongside individuals living with Duchenne and Becker to understand and support their goals for education, employment, housing, public transport or driving, and finding and maintaining active social lives and relationships.

THE PPMD EFFECT

- **10** Years added to average lifespan
- **38** Certified Duchenne Care Centers (CDCC)
- **4,700** Patients Receiving Care at a CDCC
- **750** Care providers in our CDCC network
- **8** Care Publications

BECKER CONSIDERATIONS

As a leader in dystrophinopathy research and advocacy, PPMD's mission and work encompasses both Duchenne and Becker. With over three decades of experience, we're dedicated to providing support, education, and research opportunities for individuals and families impacted by both conditions. By leveraging our established infrastructure and community networks, we are working to ensure that individuals with Becker, like those with Duchenne, receive tailored resources and support.

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.

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. Our three-part Cardiac Workshop Series has been instrumental, facilitating collaboration and an exchange of knowledge to enhance data-driven approaches. In 2022, PPMD pledged $2 million 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, in partnership with Defeat Duchenne Canada, have jointly awarded $300,000 to support two Clinical Fellowships in Duchenne Endocrinology and Bone Fragility. The clinical research will explore solutions to tackle changes in the body's hormones and the risk of bone injury in people with Duchenne. In 2023, PPMD sponsored a workshop series, "Duchenne Endocrine & Osteoporosis Care in an Ever-Changing Landscape," focused on understanding the impact of endocrine issues and poor bone health.

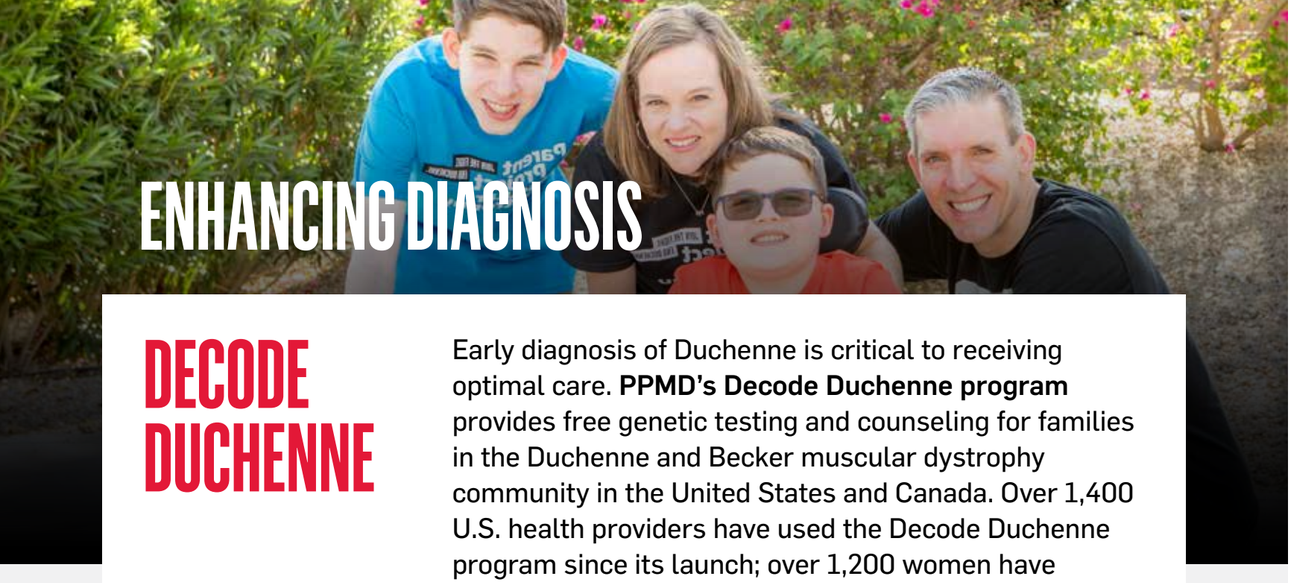
ENSURING OPTIMAL CARE

Every person living with Duchenne 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.

PPMD's Certified Duchenne Care Center (CDCC) program helps to ensure centers maintain the highest standards in clinical and sub-specialty services, rapidly apply new evidence-based knowledge, and comply with standards in clinical care that were established by the CDC Care Considerations.

PROGRAM IMPACT

Since its inception 10 years ago, PPMD's Certified Duchenne Care Center program has grown, encompassing 38 care centers across the country, working with 750 care providers with significant knowledge of Duchenne, and serving more than 4,700 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.



ENHANCING DIAGNOSIS

DECODE DUCHENNE

Early diagnosis of Duchenne is critical to receiving optimal care. **PPMD's Decode Duchenne program** provides free genetic testing and counseling for families in the Duchenne and Becker muscular dystrophy community in the United States and Canada. Over 1,400 U.S. health providers have used the Decode Duchenne program since its launch; over 1,200 women have received carrier testing, and over 1,650 participants have received diagnostic confirmation of Duchenne or Becker.

GENETIC TESTING

PPMD's partner laboratory, Revvity Omics, provides genetic testing to detect deletions, duplications, and smaller point variations in the Duchenne muscular dystrophy 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.

CARRIER TESTING

Decode Duchenne offers free carrier 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.

GENETIC COUNSELING

PPMD's genetic counselors are available to answer questions about genetic testing regardless of whether or not the participant or provider has used the Decode Duchenne program. Genetic counselors can discuss testing for other family members, answer questions about the inheritance of Duchenne and risks of recurrence, interpret genetic test results, and provide insight about which approved therapeutics and/or clinical trials may be appropriate given the genetic variant in the family.

IDENTIFYING SIGNS OF DUCHENNE

To help drive an earlier diagnosis of Duchenne, 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 Duchenne or Becker diagnosis can leave families feeling isolated, overwhelmed, and confused. PPMD reminds families that they are not alone and connects them to the latest news, resources, advancements in research, and emerging treatments so they have the information they need to make the best decisions about care.

NEWLY DIAGNOSED RESOURCES

PPMD offers a wide variety of newly diagnosed resources on our website, giving these families everything they need to learn more about Duchenne and Becker, adjust to the diagnosis, find support, and explore care options.

ESTABLISHING A CARE TEAM

PPMD connects families and individuals with multidisciplinary neuromuscular teams that have experience and expertise managing all aspects of living with Duchenne. These comprehensive teams allow each specialist to give input into the best and most appropriate care.

PPMD FOR YOU

PPMD supports everyone through each step of their Duchenne journey — from initial diagnosis to questions about genetic testing and clinical trials, and navigating the school system or independent living. Our expert staff helps families and individuals understand the complexities of Duchenne and access the best neuromuscular care and services.

T.H.I.N.K. EMERGENCY CARE BUNDLE

PPMD equips individuals living with Duchenne and their caregivers with the tools they need to communicate their needs to healthcare providers during an emergency situation. Our accessible emergency materials include essential information about Duchenne and important action items to remember through our T.H.I.N.K framework:

T **Take your equipment with you to the hospital.**

H **Hand your emergency information card to medical staff.**

I **Inform medical staff of important oxygen precautions.**

N **Notify your neuromuscular team.**

K **Keep important documents with you.**

COLLABORATING TO CLOSE GAPS IN CARE

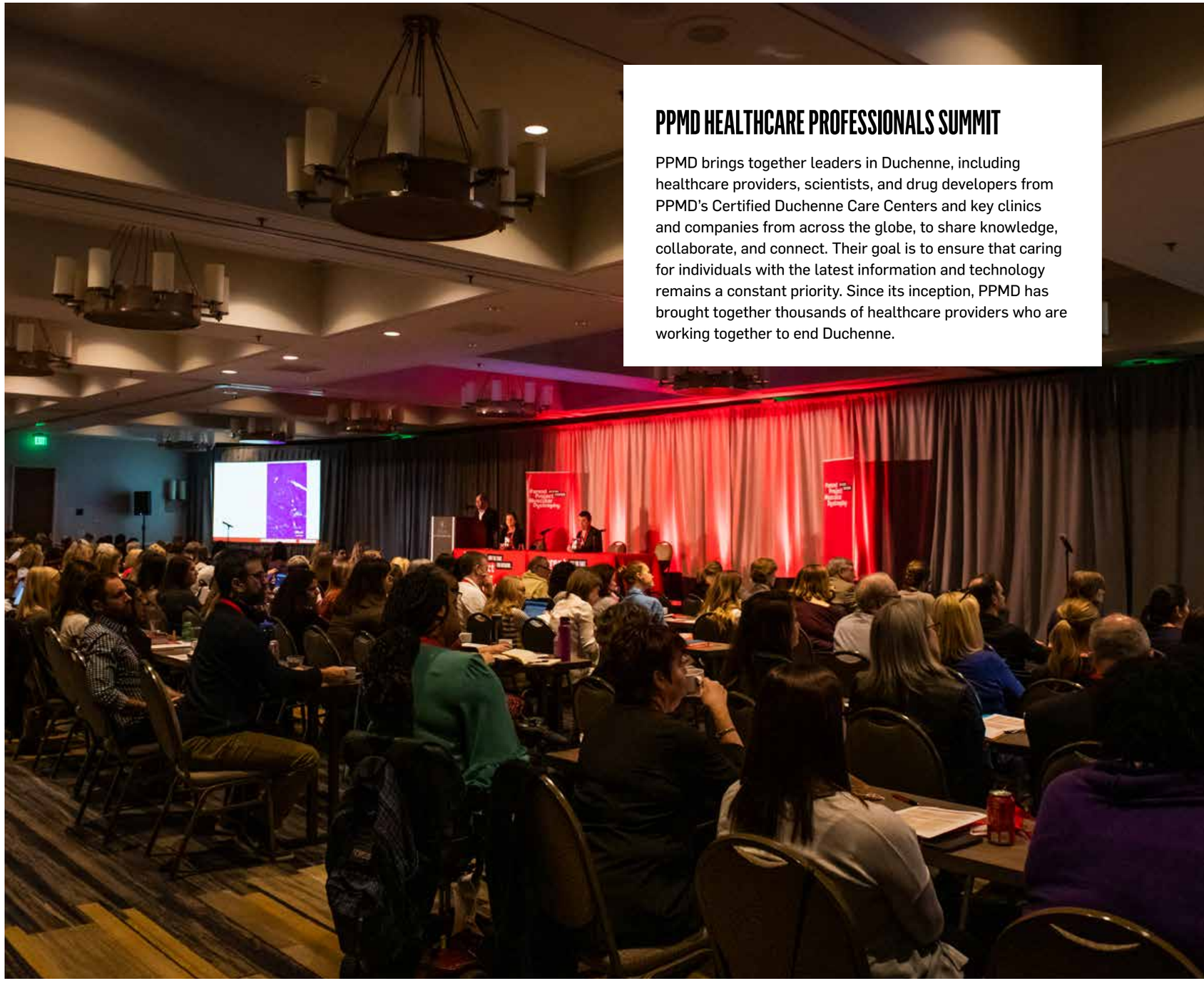
PPMD convenes researchers, industry partners, and experts in Duchenne to share learnings, navigate challenges, and explore solutions that push the Duchenne landscape forward and continually improve care for every person and every family with Duchenne and Becker.

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.

MODULE-BASED CONTINUING EDUCATION CURRICULUM

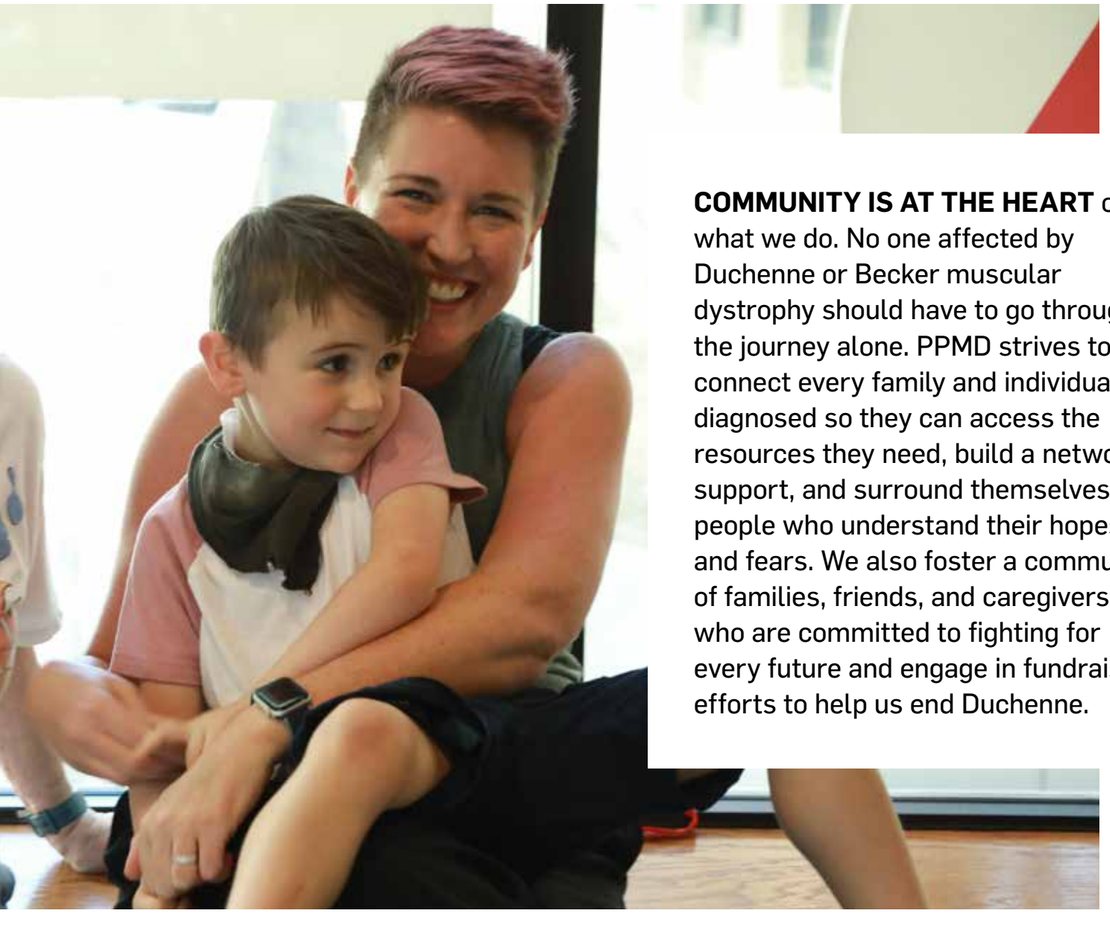
PPMD offers a Duchenne Professionals Masterclass which includes a comprehensive curriculum covering everything from the basics for new-to-Duchenne professionals through more advanced concepts for seasoned experts in the field. Additionally, the Duchenne Educators Masterclass is available to educators in the community to empower them to create and foster a safe, inclusive environment for learning.



PPMD HEALTHCARE PROFESSIONALS SUMMIT

PPMD brings together leaders in Duchenne, including healthcare providers, scientists, and drug developers from PPMD's Certified Duchenne Care Centers and key clinics and companies from across the globe, to share knowledge, collaborate, and connect. Their goal is to ensure that caring for individuals with the latest information and technology remains a constant priority. Since its inception, PPMD has brought together thousands of healthcare providers who are working together to end Duchenne.

Community FOR EVERY FUTURE



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 strives to connect every family and individual diagnosed so they can access the resources they need, build a network of support, and surround themselves with people who understand their hopes and fears. We also foster a community of families, friends, and caregivers who are committed to fighting for every future and engage in fundraising efforts to help us end Duchenne.

PPMD'S ANNUAL CONFERENCE

Each year, PPMD connects the Duchenne and Becker community through our **Annual Conference,** which is the largest, most comprehensive, annual international conference focused entirely on Duchenne and Becker. Families, caregivers, researchers, healthcare workers, industry partners, and people living with Duchenne and Becker gather to celebrate our progress over the last 30 years, share our hopes and fears, and address the community's most urgent needs.

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 35 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

Our goal is that no family ever feels alone in the early days of their diagnosis. From one-on-one meetings via our PPMD For You program, to quarterly virtual meet & greets, to our Newly Diagnosed Families track at our Annual Conference, we provide several touchpoints for new families to receive the resources they need to build a support network to rely on throughout their journey with Duchenne.

THE PPMD EFFECT

- **31** PPMD Connect Groups
- **50** PPMD Adult Advisory Committee Members
- **40+** Fundraising Events Annually
- **$45+ MILLION** Community Fundraising
- **15,000** Families Reached Through PPMD Gatherings

PPMD'S ADULT ADVISORY COMMITTEE (PAAC)

Our PAAC amplifies the voices of teens and adults living with Duchenne and Becker. Members of the PAAC hold monthly meetings to discuss urgent topics in the community, engage with Congress on important initiatives, and plan and implement Teen and Adult sessions at PPMD's Annual Conference. Through mentorship, outreach, education, and community events like virtual socials and game nights, the PAAC aims to elevate the lives of people living with Duchenne and Becker.

PPMD TOGETHER

PPMD Together regional meetings are about creating community and sharing experiences to shape a future where every individual affected by Duchenne and Becker can thrive. The PPMD Together meeting series offers the Duchenne and Becker community an opportunity to address the dynamic needs of our community by fostering collaboration between families, industry partners, care providers, and advocates.

PPMD LIGHTHOUSE

PPMD developed the virtual Lighthouse workshops to provide individuals and families affected by Duchenne and Becker with a safe harbor to build community, find connection, and center well-being as they discuss life and identity beyond diagnosis.

VIRTUAL SOCIALS

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

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.

FUELING THE FIGHT THROUGH *Community*

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 $45 million to advance therapies and improve care.

RACE TO END DUCHENNE

As PPMD's signature endurance fundraising program, friends and family take action to end Duchenne by racing in cities all over the country. Since 2005, over 6,000 participants have raised more than $15 million to accelerate treatments and improve care. In 2020, PPMD launched a virtual race series so more families can participate around the world.

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 $25 million towards advancing research in Duchenne.

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 involved over 115,000 coaches, and more than 1,700 high school and college football teams, with about 100 families participating each year. Now, we are expanding 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.

JOIN US

in the fight for every future

Get involved and help PPMD continue to fight for every future! From our Race to End Duchenne program to our DIY Fundraising efforts, there are lots of ways to lend a hand and raise funds and awareness. A monthly gift is an easy way to invest in continuous progress. **$0.83 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.

EndDuchenne.org

PPMD30

Parent Project Muscular Dystrophy 30

PARENTPROJECTMD.ORG

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Kerri McLaughlin — *Finance Manager*

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Parent Project Muscular Dystrophy 30

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STATEMENTS OF FINANCIAL POSITION

Years Ended DECEMBER 31, 2023 & 2022	2023	2022
ASSETS		
Current Assets		
Cash	4,804,466	3,194,727
Pledges & accounts receivable	1,565,673	1,782,425
Investment securities	2,396,078	2,349,399
Total financial assets	**8,766,217**	**7,326,551**
Employee advances	15,414	336
Merchandise inventory	65,513	87,750
Prepaid expenses	367,666	197,973
Total current assets	**9,214,810**	**7,612,610**
Property & equipment		
Office equipment	95,666	88,006
Office furniture	16,019	16,019
	111,685	104,025
Less accumulated depreciation	-91,324	-77,550
	20,361	26,475
Promissory notes receivable	252,548	—
Operating lease right-of-use assets	96,764	153,152
Other assets		
Investments at cost	850,479	850,479
Security deposit	5,938	5,938
	856,417	856,417
Total assets	**10,440,900**	**8,648,654**
LIABILITIES & NET ASSETS		
Liabilities		
Accounts payable and accrued expenses	435,449	463,474
Grant received with conditions	—	87,200
Research grants payable	102,502	62,917
Operating lease liabilities	96,764	154,825
Total Liabilities	**634,715**	**768,416**
Net assets		
Without donor restrictions	7,453,058	6,152,121
With donor restrictions	2,353,127	1,728,117
Total net assets	**9,806,185**	**7,880,238**
Total liabilities and net assets	**10,440,900**	**8,648,654**

Parent Project Muscular Dystrophy 30

PARENTPROJECTMD.ORG

STATEMENTS OF ACTIVITIES

Years Ended DECEMBER 31, 2023 & 2022	2023	2022
PUBLIC SUPPORT		
Contributions & grants	10,026,747	6,610,553
Conference income	587,511	483,304
Other meetings & conferences	20,000	—
Fees for service	603,458	315,171
Special events		
Gross income	2,775,933	2,923,010
Less direct expense	-272,298	-222,172
	2,503,635	2,700,838
	13,741,351	**10,109,866**
Investment income		
Interest & dividends	88,182	9,584
Gain on sale of donated securities	387	32,112
Unrealized gain (loss) on investments	157,348	-534,607
	245,917	-492,911
Other income		
Recovery of previously awarded grant	40,823	175,600
Other	10,342	—
Paycheck Protection Program	—	425,852
	51,165	601,452
	14,038,433	10,218,407
Assets released from restriction	—	—
Total income	**14,038,433**	**10,218,407**
FUNCTIONAL EXPENSES		
Program services		
Research	7,116,727	6,532,965
Education	1,933,388	2,168,132
Advocacy	1,137,597	910,780
	10,187,712	9,611,877
Supporting services		
Management & general	1,372,227	879,136
Fundraising	552,547	546,628
	1,924,774	1,425,764
Total expenses	**12,112,486**	**11,037,641**
Increase (decrease) in net assets	1,925,947	-819,234
Net assets — beginning of year	7,880,238	8,699,472
Net assets — end of year	**9,806,185**	**7,880,238**