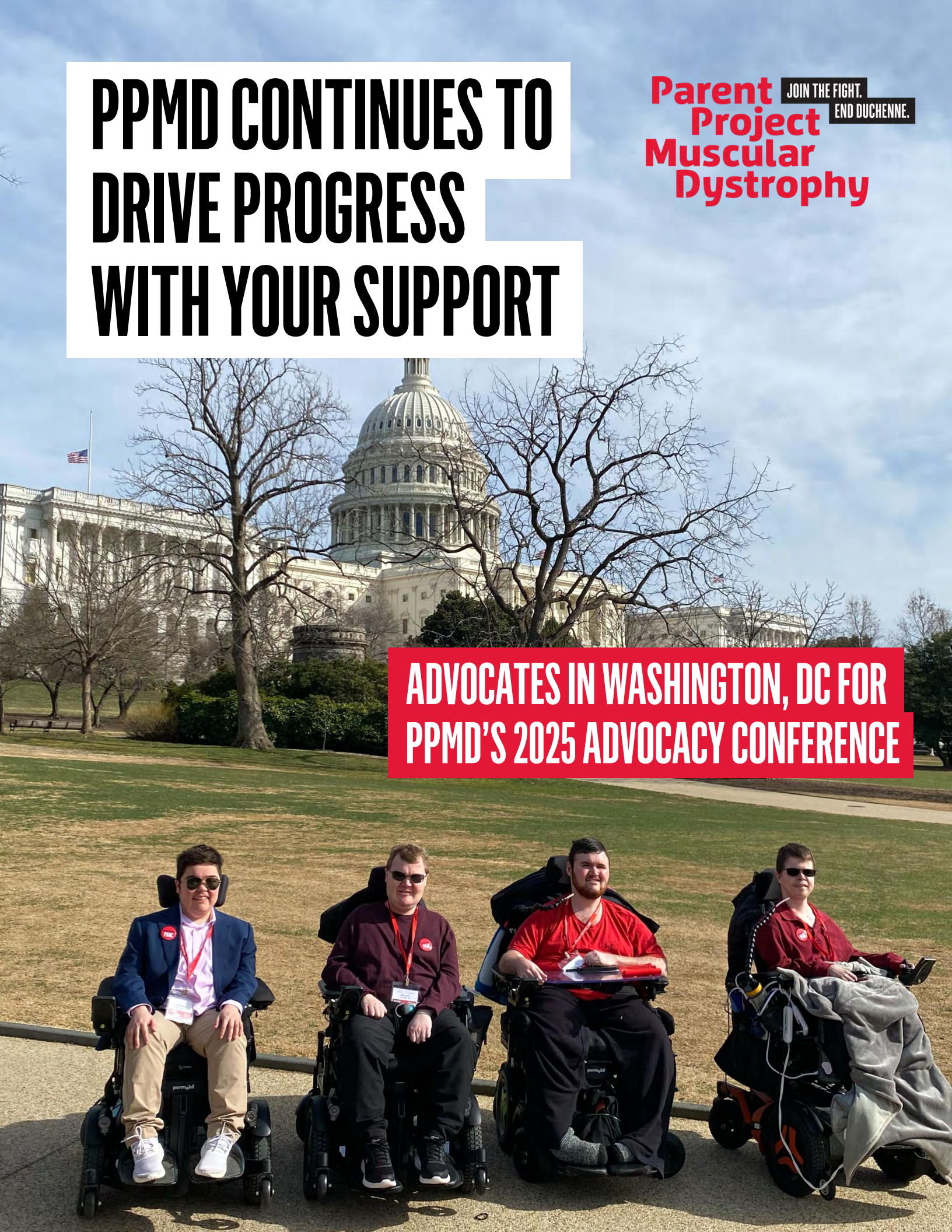
PPMD CONTINUES TO DRIVE PROGRESS WITH YOUR SUPPORT

Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.



ADVOCATES IN WASHINGTON, DC FOR PPMD'S 2025 ADVOCACY CONFERENCE

PPMD Continues to Drive Progress With Your Support

Overview

PPMD is committed to every future, working toward the day when every single family has options for, and access to, therapies and specialized care.

For over 30 years, that commitment has fueled our mission driven by the strength, generosity, and dedication of this remarkable community. In 2025, thanks to your support, families are receiving earlier diagnoses, care is evolving as the natural history of Duchenne changes, research is moving forward, and the bonds between families, advocates, and experts continue to grow. We are pleased to share the following updates to you on our work so far this year.

LEADERSHIP

In August, a new chapter for PPMD began with an expansion of our leadership team, welcoming Katherine Beaverson, MS, as our new Chief Executive Officer. Katherine joins us with over 15 years of experience in Duchenne and rare disease, beginning her career as a genetic counselor working directly with families. She brings a deep respect for clinical science, a heartfelt commitment to our mission, and a remarkable ability to connect across the research, care, and advocacy spaces. Pat Furlong will remain on as President and work in partnership with Katherine.

RESEARCH

Thanks to donor support of our Gene Therapy Initiative, PPMD continues to proactively address challenges in gene therapy research and treatments. In collaboration with CureDuchenne and MDA, PPMD co-funded a clinical trial exploring the use of the drug efgartigimod (Vyvgart) to reduce anti-AAV antibodies in Duchenne patients. This one-year study, led by Dr. Barry Byrne at the University of Florida, aims to determine if an immune-modulating therapy can enable more people to receive gene therapy safely, including those who currently have antibodies from prior infections or trials. By investing in forward-looking projects like this, we are tackling obstacles head-on so that gene therapy is as inclusive as it can be.

As part of PPMD's advocacy efforts the creation of the Wellstone Centers of Excellence has been a long-standing multicenter approach to advance research for Duchenne. In 2025 the University of Florida Myology Institute was again awarded one of the Wellstone awards, in collaboration with Northwestern University. PPMD has historically supplemented these awards in order to further their research capacity and has awarded supplemental funding to both institutions. PPMD's support to Northwestern University will support continued training of research and clinical fellows, cardiologist Dr. Fullenkamp who presented at PPMD's annual conference 2025 is a previous trainee from this Wellstone center. The University of Florida will focus their research efforts on enhancing our understanding of gene therapy through pre-clinical studies and muscle imaging. The goal is to better understand combination therapy along with gene therapy and elucidate the physiological changes of a Duchenne patient who has received a gene therapy.

PPMD's commitment to research goes beyond funding. We also generate and share knowledge to benefit the entire field. In the past year, PPMD and academic partners including Norah L. Crossnohere, PhD, from The Ohio State University College of Medicine, with Niki Armstrong, MS, CGC serving as the Principal Investigator, published a joint qualitative

study examining barriers to trial participation among Hispanic/Latinx families, published in Orphanet Journal of Rare Diseases, providing insights that will help ensure that clinical research is more inclusive, representative, and accessible to all.

Your support makes this work possible, and by supporting studies like this, you are helping break down barriers, strengthen equity in clinical trials, and bring us closer to a future where every family can benefit from new therapies. Your continued engagement means these insights will be transformed into action, expanding participation, improving trial design, and ultimately accelerating access to treatments for families living with Duchenne.

Thanks to your generosity, PPMD is moving forward with the Stability Study, a research partnership with The Ohio State University. This important project is focused on understanding what "stability" means to families living with Duchenne, what it looks like to slow disease progression, maintain abilities, and preserve quality of life. These insights will be shared with regulators, clinicians, and industry leaders so that future therapies are evaluated in ways that reflect what truly matters to our community. This study was shaped directly by families. During our 2024 Advocacy Conference Town Hall, parents and caregivers made it clear that stability should be recognized as a meaningful treatment benefit. Their input guided the questions we are asking and how the study was designed. Before year end, we will launch a Stability Study survey and anticipate sharing results in the first half of 2026. Together, we are making sure the path to new treatments reflects the real priorities of the Duchenne community.

PPMD issued two awards to researchers working on the Brain Involvement In Dystrophinopathies (BIND) project based in the EU. The focus of the BIND project has been to help elucidate the role of dystrophin in the brain and uncover the mechanisms by which dystrophin isoforms, or their absence plays in development.

DYSTROPHINOPATHY CLINICAL RESEARCH NETWORK (DCRN)

The Dystrophinopathy Clinical Research Network (DCRN) is a network of care centers and research institutions united to advance collaborative research and data sharing for Duchenne and Becker muscular dystrophy was announced late last year.

The vision of this network is that by fostering partnerships across disciplines, the DCRN will streamline real world data collection, ensuring that all families benefit sooner from clinical expertise and cutting-edge innovations. By leveraging existing clinical networks and technical infrastructure, the DCRN will revolutionize how we use sequential therapies to slow disease progression and redefine standards of care. Developing the DCRN is a complex multi-year initiative. From concept to reality, this clinical network would not be possible without the steadfast commitment of our community.

THE DUCHENNE REGISTRY

Thanks to your engagement, The Duchenne Registry remains the largest patient-reported outcomes registry for Duchenne, Becker and carrier females. Over 6,000 people from more than 125 countries have joined, contributing 17+ years of longitudinal data and helping with 175+ clinical studies and trial recruitments.

PPMD's Registry team has initiated three studies this year (described below) utilizing our data. The first study was presented at the Newborn Screening Symposium in early 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 also created a new group called Registry Champions, a small group of individuals who are actively engaged with the Registry and are helping to spread the word about the importance of participation, with the goal of growing engagement with the Registry. A highlight of this effort was a community webinar we hosted in May.

In 2025, we 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.

We continue to make progress on our Electronic Health Record (EHR) Study, with real world data from 170 patients flowing into the Duchenne Outcomes Research Interchange from nine CDCCs. The EHR Study represents the future of data sharing using a novel approach to aggregate data from consented patients in an automated way, thus reducing burden on clinic teams and patients themselves.

Now in its 12th year, Decode Duchenne continues to provide access to critical genetic testing for families living with Duchenne and Becker. More than 1,850 individuals have been tested through our diagnostic arm, and an additional 1,800 have been tested through our familial variant (carrier) arm. Our laboratory partner, Revvity, has expanded their testing for us to include neuromuscular panel testing for all individuals with negative diagnostic testing, and RNA sequencing for certain individuals with an unknown variant or a variant of uncertain significance. Behind every test is a family searching for answers. A genetic diagnosis can end years of uncertainty, open doors to treatment, and bring families renewed hope. Because of your generosity, Decode Duchenne continues to provide these life-changing answers, giving families clarity today and the promise of a brighter tomorrow.

ADVOCACY

2025 has been a historic year for Duchenne advocacy, one marked by both hard-fought victories and unprecedented challenges. Through it all, the power of our community's voice has never been more evident.

The importance of advocacy, and the power of our collective voice, was on full display at the 2025 Advocacy Conference in March, which brought more than 130 advocates from 30 states to Washington, D.C. Advocates met with their Members of Congress, sharing firsthand the urgency of Duchenne policy priorities with policymakers. These meetings played a pivotal role in securing FY2025 federal Duchenne funding and appropriations language, including an increase from $10 million to $12.5 million for our Department of Defense Congressionally Directed Medical Research Program (CDMRP) and $8 million in funding to the Centers for Disease Control & Prevention (CDC).

PPMD advanced advocacy on the access front, recognizing that new therapies are only meaningful if patients can actually receive them. Working in partnership with the Little Hercules Foundation, we closely tracked patterns of insurance denials and prior authorization delays that continue to create barriers for families. This evidence informed our engagement with both the Centers for Medicare & Medicaid Services (CMS) and private payers, where we pressed for consistent, equitable coverage of approved Duchenne therapies and adherence to standards of care.

In 2025, PPMD has placed a strong emphasis on building and strengthening its relationship with the Food and Drug Administration (FDA), particularly as the agency undergoes leadership changes under the new administration. Recognizing that regulatory decisions directly shape the pace and direction of therapy development, PPMD has sought to establish itself as a trusted partner and resource to FDA officials. Through formal comments, regular briefings, and direct dialogue, we have underscored the urgency of Duchenne while offering

the perspective of patients, families, and clinicians. Our goal is to collaborate with the agency to ensure that new guidance, review processes, and regulatory frameworks reflect the realities of the Duchenne community, supporting both innovation and patient safety. By leaning into this collaborative approach, PPMD is working to ensure that the FDA views the Duchenne community not just as stakeholders, but as essential partners in shaping the future of rare disease drug development.

We continue to collaborate closely with the CDC to advance critical Duchenne initiatives. Through the Muscular Dystrophy Surveillance, Tracking, and Research Network (MD STARnet), we are working to strengthen data collection, improve the accuracy of diagnosis and coding, and ensure that public health resources reflect the realities of living with Duchenne. These efforts provide the foundation for better standards of care, help track long-term outcomes, and inform policy decisions that shape access and services nationwide. By partnering with the CDC, PPMD is making sure that the voices and needs of the Duchenne community are represented at the highest levels of public health.

These efforts underscore a central truth: advocacy is not a once-a-year event but a year-round commitment. Whether through congressional outreach, regulatory engagement, or payer negotiations, PPMD and our community are driving progress on every front to ensure that innovation translates into real-world impact for every individual living with Duchenne.

NEWBORN SCREENING

For more than a decade, PPMD has worked tirelessly alongside our community towards newborn screening for Duchenne at federal and local levels. State by state, PPMD is fully committed to this effort, giving every child the opportunity to benefit from early diagnosis. Early diagnosis matters because it helps children begin early interventions and receive therapies at the right time.

This year, Texas, Florida, and Arizona brought historic progress in our quest to make

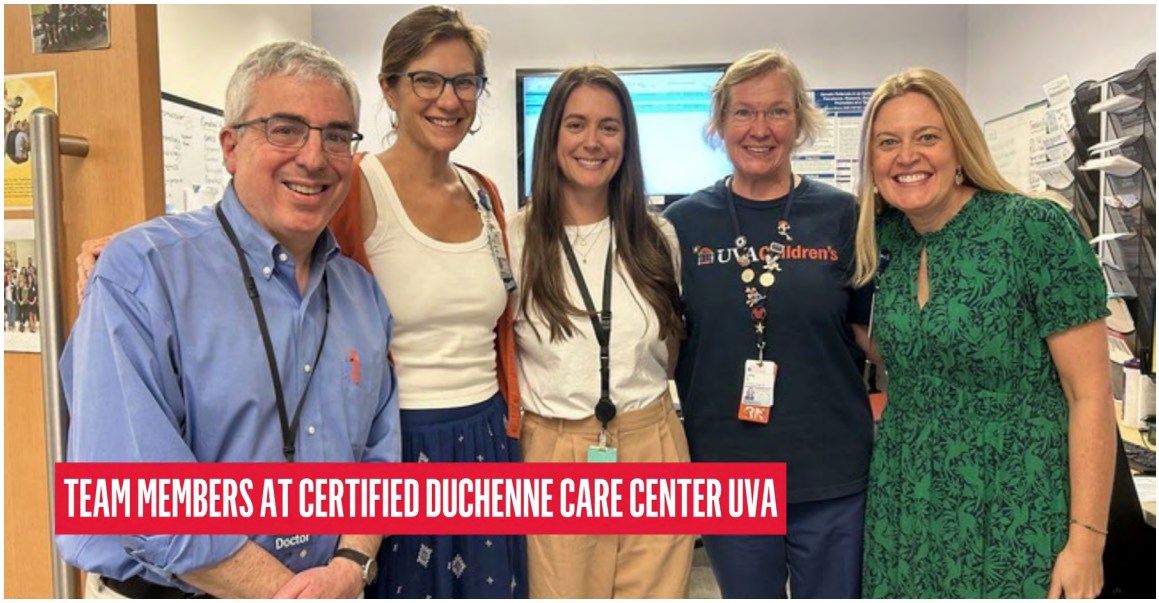
Duchenne newborn screening a reality across the nation. In May, Texas Governor Greg Abbott signed Senate Bill 1044 into law, officially committing to adding Duchenne muscular dystrophy to Texas's newborn screening panel. Just days later, Florida's Governor Ron DeSantis signed Florida's Duchenne newborn screening legislation (HB 1089/SB 524) into policy. Both states signed unfunded mandates, so advocacy will continue to ensure these programs start promptly. In June, Arizona's Governor Katie Hobbs signed the bipartisan state budget including the Duchenne newborn screening Senate Bill 1076. Ohio and Minnesota have fully implemented Duchenne screening, while Massachusetts, New York, and Illinois also have approved programs that are not yet active. Many other states remain in the planning or committee phase. Each advance means families receive answers at birth, children gain access to interventions sooner, and therapies can begin at the right time. Thanks to your support, these opportunities are being expanded to include more families every year.

Early Intervention Focus: In preparation for newborn screening in every state, PPMD has been proactively working to ensure that babies diagnosed with Duchenne have access to multidisciplinary care at diagnosis. In March, we hosted the Duchenne Early Intervention Symposium to present evidence about the impact of early treatment on outcomes in Duchenne. On December 1st, we will also host the Early Diagnosis and Care for Duchenne Masterclass, a collaboration with TREAT-NMD to convene pediatric specialists, physical therapists, genetic counselors, and researchers to discuss optimal care strategies for babies and toddlers with Duchenne. Last year, PPMD awarded $250,000 to Dr. Bo Hoon Lee of University of Rochester to support the initial development of Baby Duchenne, a clinical research network designed to develop natural history and guidance for care for babies identified through newborn screening. With this support, Dr. Lee created a framework on which to submit an application for additional funding through the Congressionally Directed Medical Research Programs, and this year she was awarded a grant to expand the program. With these resources, clinicians can be ready

on Day 1 with an informed plan for 0–3 year-old clinical care. By educating providers now, PPMD is laying the groundwork for a new generation of children with Duchenne who will benefit from earlier interventions and, we believe, improved outcomes over the long term.

Responding to Federal Changes: In April, an unexpected development temporarily clouded the path forward: the U.S. Department of Health and Human Services dissolved the federal Advisory Committee (ACHDNC) that was reviewing Duchenne's nomination to the national Recommended Uniform Screening Panel (RUSP). While this news was concerning – the ACHDNC had been the guiding body for newborn screening for over 20 years and was set to vote on recommending Duchenne to the RUSP in May – PPMD immediately pivoted to forge a new path forward. Our team sprang into collaboration with leading newborn screening experts, partners, and policymakers to explore ways to keep the nomination progressing. Through our combined efforts, the Health Resources and Services Administration (HRSA) agreed to allow the completion of the Evidence Review Report, developed by a third party to explore the possible impact of Duchenne newborn screening. We also convened members of the nomination's technical expert panel to present additional data at EveryLife Foundation's Newborn Screening Evidence Review workshop on September 12th. The Health Resources and Services Administration recently closed an open comment period for feedback regarding Duchenne newborn screening, and we anticipate a federal decision for adding Duchenne to the Recommended Uniform Screening Panel at any time. PPMD's stance is unwavering: every baby deserves early diagnosis and care. We have redoubled our state efforts, working to engage new allies, present powerful evidence to states, and maintain national momentum.

CARE



PPMD kicked off 2025 by bringing the global Duchenne care community together at our sixth annual Duchenne Healthcare Professionals Summit, convening over 360 experts – clinicians from 50+ centers, researchers, industry partners, and representatives from the FDA, NIH, CDC, and abroad – in one room to advance dystrophinopathy care. Attendees tackled

pressing topics including sequential therapies, lessons learned in gene therapy, early intervention through newborn screening, and the growing needs of adult patients.

At the conclusion of Summit 2025, we launched dystrophinopathy working groups to bring together experts across the field to address emerging challenges. At next year's Summit, these groups will reconvene to share progress and work toward consensus on modern Duchenne and Becker care to meet the current needs of patients and families.

PPMD's Certified Duchenne Care Center (CDCC) network continues to expand, bringing excellence in care to more families. In June, we announced the certification of Children's Hospital of Philadelphia (CHOP), marking our 39th CDCC. CHOP is also the first center to earn PPMD's new Clinical Research Designation. The Clinical Research Designation provides visibility into which CDCCs provide outstanding leadership in Duchenne clinical trials and research as well as world-class multidisciplinary care. By recognizing centers that integrate research with care, PPMD is bridging the gap between clinics and clinical trials. We look forward to designating additional CDCCs with research excellence in the coming years, as we continue to raise the bar for both care and clinical research across our network. PPMD also has scheduled to evaluate 1 more CDCC by the end of 2025. If the center passes the certification process it will become the 40th certified center. PPMD is continuously recertifying clinics, sharing resources, and expanding knowledge among our CDCC network to ensure that optimal care is provided.

This year, in partnership with the OPTIMIZE-DMD Consortium spearheaded by Drs. Leanne Ward (Children's Hospital of Eastern Ontario) and David Weber (CHOP, a CDCC), PPMD released an updated version of the PJ Nicholoff Steroid Protocol, shaped by a decade of experience from families and clinicians. The new guidance strengthens emergency stress

dosing, ensures safer tapering and transitions between steroids, and adds important updates on newer treatments like Agamree (vamorolone). It also lays out six critical concepts for care, from recognizing adrenal suppression to preventing crises and supporting safe transitions, so families and providers are better prepared in vulnerable moments. Because of this work, families can face emergencies with greater confidence and peace of mind, and care teams have the tools they need to keep children safe, including updated emergency care resources, vamorolone provider and family resource sheets, and more. None of this progress would be possible without the generosity of our supporters.

In April, PPMD and PPMD Italy co-hosted a Cardiopulmonary workshop in Rome. The workshop, which included a multi-disciplinary audience from across the US and Italy, aimed to both understand the current landscape of cardiac and pulmonary care against a global background, with a goal of working toward consensus recommendations for cardiopulmonary care in dystrophinopathy throughout the lifespan. A meeting report is in progress and will be shared with the community via publication in a peer-reviewed journal in the coming months.

PPMD was also happy to partner with key physical therapy leaders in the dystrophinopathy field in the creation of a two-day workshop for physical and occupational therapists to provide education on emerging evidence on the role of exercise in individuals living with both Duchenne and Becker muscular dystrophy. Led by physical therapists Claudia Senesac (University of Florida, retired), Donovan Lott (University of Florida), Tina Duong (Stanford University) and exercise physiologist Tanja Taivassalo (University of Florida), workshop participants learned not only about the latest research on exercise, but also how to create safe exercise plans for their patients. A workshop report is in preparation, and additional workstreams are in process as a direct result of this meeting.

This fall, PPMD hosted a workshop on the Brain and Behavior in Dystrophinopathy, a two-day meeting led by co-chairs Molly Colvin (Harvard University-Massachusetts General Hospital), Natalie Truba (Nationwide Children's Hospital, CDCC) and Francesco Muntoni (University

College London-Great Ormond Street Children's Hospital). This workshop delved into key areas in the care of individuals living with dystrophinopathy, including the role of steroids on behavior, new tools to facilitate increased access to behavior screening and treatment, the role of behavior in clinical trial participation, and deep discussion on medication- and non-medication-based interventions to support patients in their families.

As part of this meeting, PPMD also announced a new grant to Natalie Truba to continue her work alongside collaborator Molly Colvin to initiate validation studies of the BELS (Behavioral, Emotional, Learning, Social) Tool, which received early funding from PPMD for development and subsequent pilot testing at Arkansas Children's Hospital, a Certified Duchenne Care Center. The BELS aims to improve access to screening of behavioral and psychological symptoms, facilitating faster recognition of risk factors and symptoms and quicker referral to appropriate providers who can create meaningful plans of care.

CONFERENCE AND COMMUNITY

PPMD's Annual Conference, held in June in Las Vegas brought together over 1,200 families, researchers, clinicians, and industry partners from around the world for three days of informative and interactive sessions featuring the latest in research, clinical trials, approved and emerging therapies, care initiatives, quality of life issues, and more. The agenda was packed with informative and empowering sessions: we heard cutting-edge research updates straight from scientists, engaged in frank discussions about daily care and mental health, and participated in hands-on workshops. Teens and adults enjoyed a dedicated track led by our PAAC, sharing candid peer perspectives on navigating college, careers, and independence. Thirty-four families new to a Duchenne diagnosis connected with the community, finding hope and guidance for the road ahead. And of course, we made time to celebrate – from the opening reception to our closing dinner, the sense of ONE community was palpable. We reflected on how far we've come in 31 years and renewed our commitment to the

road ahead. PPMD's 2026 Annual Conference will be held June 25–27, 2026 in Orlando, at Disney's Coronado Springs Resort.

This year's Annual Conference also offered a meaningful moment for Lighthouse participants, as past and current members connected in person. Although Lighthouse sessions take place online, the relationships formed are deeply personal—and the chance to embrace, reflect, and support one another face-to-face underscored just how lasting those bonds have become. With approximately 50 participants in the spring, and the fall session now underway with nearly 70 participants, the series remains a vital source of comfort and community for families navigating grief within the Duchenne journey.

Our Race to End Duchenne program is also celebrating a special year – 2025 marks the 20th anniversary of our signature endurance fundraising program. We kicked off the celebration in January at the Walt Disney World Marathon Weekend, where an amazing 185 team members (runners, walkers and rollers) donned Race to End Duchenne shirts and hit the pavement together. Since then, our racers have participated in major events from coast to coast – including the Chevron Houston Marathon, the United Airlines NYC Half, the TD Five Boro Bike Tour and the Bank of America Chicago Marathon – all while raising awareness and dollars to advance our mission. We also added our first international marathon - the 2026 Tokyo Marathon - to our race calendar, an exciting expansion of the program. Thanks to the dedication of our Race to End Duchenne team members, every mile brings us closer to the finish line: a world without Duchenne.

PPMD's Adult Advisory Committee (PAAC) continues to thrive in 2025, empowering adults living with Duchenne and Becker through advocacy, education, and community engagement. At PPMD's Annual Conference in June, over 80 people with Duchenne and Becker participated in the Teen & Adult Track, led by the PAAC, which featured workshops on travel, technology, life planning, mental health, and relationships, alongside a vibrant resource fair.

A major highlight was the unveiling of a low-cost robotic arm developed in partnership with Virginia Tech to assist with everyday tasks—now funded for broader development thanks to PAAC feedback and support. Now more than 75 members strong, the PAAC continues to play an important role in representing the patient voice in much of PPMD's work.

Our PPMD Together meeting series continues to bring local communities together for conversation, connection and resources. Our Minnesota meeting in March was attended by 51members of the community; while our Charlotte meeting in September had 85 attendees. We are looking forward to a virtual meeting in November focused on establishing and maintaining friendships as well as meetings in Philadelphia and San Francisco in 2026. We look forward to these gatherings where families, clinicians, industry partners and advocates come together in a meaningful way.

The PPMD Connect program continues to thrive, providing local support and social opportunities in communities across the country. In addition to our geographic groups, we have seen wonderful growth in our specialized Connect groups for Siblings, Grandparents, Dads and Carriers, providing continuing opportunities for these groups to meet virtually and discuss topics important to their unique journeys. Our Seattle group, in partnership with the Akari Foundation and Seattle Children's Hospital, and with support from NS Pharma, held our first Connect event for Spanish-speaking families. Several families gathered for a barbecue and time together and left with Spanish-language resources and new connections to help support their journey with Duchenne and Becker.

Our Do-It-Yourself (DIY) fundraising program continues to grow and is a great way for families to take action and raise funds while bringing awareness of Duchenne and Becker to their own communities through fun and interactive events. Some highlights from the year so far include August Blues, a fishing tournament in Nantucket; Mitchell's Run Through Rockford, our longest-running DIY fundraising event honoring Mitchell Petersen, who passed away late last year; the Rev It Up 5K in Ohio; Singing Badly for a Good Cause, a

family-friendly karaoke event in Illinois; and the 20th annual Sam's Night in Dallas, which raised more than $1.5 million, becoming the highest fundraising event in 30+ year PPMD's history.

This year has shown us just how much progress is possible when we work together. From research breakthroughs to advances in care and advocacy, every success has been made possible because of you. Families are receiving answers sooner, children are starting therapies earlier, and the Duchenne community is stronger than ever. Yet there is still more to do. Many families are still waiting for options, and the road to treatments and cures requires continued investment and commitment. We are deeply grateful for your generosity, your voice, and your partnership. With your ongoing support, we can build on this momentum and move closer to the day when every child and every family has access to the care, road to therapies, and hope they deserve.

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