ABOUT PPMD
1994 - Questions and Missing Pieces

- What was the current state of research and care in Duchenne?
- What was the federal government doing?
- What data existed about the population?
- How do we move the needle toward treatments for Duchenne?

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SUCCESSES WITH ADVOCACY

MD-CARE Act 2001
Established:
- MD Centers of Excellence - to research MD
- MD STARnet tracking and surveillance - to understand the incidence, prevalence
- Muscular Dystrophy Coordinating Committee (MDCC)
- The Action Plan for Muscular Dystrophies

MD-CARE Act Reauthorization 2008
- Duchenne Care Considerations - develop & disseminate
- Enhanced clinical research in Duchenne
- Expanded MD-STARnet to collect data on teens and young adults with DBMD
- Added the National Heart, Lung and Blood Institute (NHLBI) to the MDCC

MD-CARE Act Amendments 2014
- Expanded research to focus on Endocrine, Pulmonary, Cardiac, and Transitions
- Additional federal agencies were added as members of the MDCC
- Expansion of CDC Duchenne Becker Care Considerations to include adults with Duchenne
- Update of MD Action Plan by MDCC

Since our advocacy efforts began in 2000, our community has helped to leverage over $800 million in federal funding into muscular dystrophy research, with over $500 million specifically for Duchenne.
Federal Agencies We Currently Focus On

For all agencies
We advocate to impact:
Focus
Funding
or
Flexibility
ADVOCACY

- Providing advocates the tools and information they need to advance legislation and regulatory policy efforts that will impact their lives and ensure Duchenne is a priority across all government agencies:
IMPROVEMENTS IN CARE

- Advocating for & participating in the development of Standards of Care
- Helping families find providers that meet our standards of care via Certified Duchenne Care Centers
- Connecting families with clinical trials, care, and research through DuchenneConnect, PPMD’s Duchenne Registry

Since our founding in 1994, the average lifespan of people with Duchenne has increased from late teens/early-20s to mid-20s/early-30s.
ADVANCES IN RESEARCH

PPMD has invested more than $50 million into Duchenne research, which has leveraged another $500 million in federal funding and billions in private investment.

- Since 2016, two therapies have been approved in Duchenne with additional therapies approaching the regulatory finish line.

- PPMD played a significant role in this process, convening thought leaders and community members to share their expertise and experiences with regulators and decision makers.

PPMD's leadership in the Duchenne community created an inflection point, resulting in over 40 pharmaceutical companies now in the Duchenne space—companies who have invested approximately $8 billion in the fight to end Duchenne.
How to advance these drugs through community involvement

• Continue advocating in Washington (funding and legislation)
• Collect data on through Duchenne Connect
• Tell patient stories to regulators
• Collect patient preference data about how families think and feel about emerging therapies
  • Weighing benefits and risks
  • New survey around gene therapy (access through Duchenne Connect)
Collaboration & Community

Thank you to our Foundations Partners

Thank you to our tour sponsors!

PPMD CONNECT
Michigan
Janell Lundy