JOIN THE FIGHT. END DUCHEENNE.
Mission Pillars

PPMD

Research  Advocacy  Care  Education
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
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:
  - Food and Drug Administration (FDA)
  - National Institutes of Health (NIH)
  - Centers for Disease Control and Prevention (CDC)
  - Department of Defense (DOD)
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.
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<th>Pre-Clinical</th>
<th>Phase 1</th>
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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
  • Thank you to everyone who completed the Gene Therapy survey (analysis expected in late spring)
PPMD Connect Program

- Established 2008 formally called FACES
- The official parent led outreach initiative of PPMD
- 24 regional groups around the country
- Focus on connecting, resource sharing, support, and advocacy (grassroots army!)
- New manager of program – Nicole Herring
- PPMD State Resource Effort
- Many work with clinics within their states
24 States specific groups established

- New Jersey
- Illinois
- Arizona
- South Texas
- Colorado/Wyoming
- North Texas
- Pacific North West
- Michigan
- Northern California
- Western New York
- Ohio
- Southern California

- Tennessee
- Florida
- Nebraska
- Virginia
- Northern Florida
- North Carolina
- Georgia
- Minnesota
- DMV - DC, Maryland, Northern VA
- Western Pennsylvania
- Long Island New York
State Specific Page Example

Massachusetts Resources

- Housing
  - Massachusetts Department of Health and Human Services
  - Massachusetts State Government Dept. of Health and Human Services webpage listing various sources for home modifications.
  - Website

- Financial Resources
  - Financial Resource list

- Equipment Resources
  - A list of organizations which offer opportunities for patients and families to obtain low cost, rental and donated equipment including recreational wheelchairs, assistive technology devices and power wheelchairs.
  - Resources links list

- Transportation Resources
  - Personal vehicle and public transportation information.
  - Transportation Resources list

- Accessibility & Recreation
  - An extensive list of Massachusetts specific and national camps and camp programs for people with disabilities, accessible recreation opportunities and Wish granting organizations.
  - Accessibility, Recreation and Camps Links List
Living Duchenne Resource Center

www.parentprojectmd.org/resources
Need

• Scattered information on resources
• Social media groups (buried detail)
• State specific needs
• Wiki-model
• Where do you start?
Education Section

- Education Matters
- The psychology of Duchenne
- Adaptive PE
- Behavior and Duchenne
- School Resources for class education
Access to approved therapies resource page

www.parentprojectmd.org/accessresources

Goals
Provide education and guidance on access process
Provide product specific resources for Patients, Clinicians, Payers
JOIN THE FIGHT.
END DUCHENNE.

Pat Furlong
President and CEO