About PPMD

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SVP Community Engagement
PPMD Founder and President, Pat Furlong

ONE TOUGH MOTHER

• Founded PPMD in 1994
• Sons, Christopher and Patrick
• MS in Nursing, ICU, Dialysis
• Refused to accept ‘there is nothing you can do’
PPMD’s mission is 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.

• **Comprehensive approach** – 4 Pillars
  – Research, Advocacy, Care and Education

Together with our amazing community, we attack Duchenne from every angle.
1994 QUESTIONS

• What was the current state of research and care in Duchenne?

• What was the federal government investment?

• What data existed on the Duchenne population?

• What are the gaps we need to fill in order to move the needle toward better care and treatments for Duchenne?

CRITICAL MISSING PIECES

- Standard of Care
- Federal Dollars
- Data on population
- Natural History
- Outcome Measures
- Advocacy
- Industry
- Biomarkers
- Trial Infrastructure
The Muscular Dystrophy Community Assistance, Research & Education (MD-CARE Act)

**MD-CARE Act 2001**
- Centers of Excellence
- MD STARnet tracking and surveillance
- MD Coordinating Committee – Action Plan for Muscular Dystrophies

**MDCA Reauthorized 2008 (MD-CARE Act 2)**
- Duchenne Care Considerations – Develop and Disseminate
- Added National Heart, Lung, and Blood Institute to MDCC
- Enhancement of clinical research
- Expansion of MD-STARnet

**MDCA Amended in 2014 (MD-CARE Act 3)**
- Expanded research to focus on Endocrine, Pulmonary and Cardiac, Transitions
- Additional federal agencies added as members of Coordinating committee
- Sharing of data from MD-STARnet
- Expansion of care considerations to include Duchenne adult population
- Update of MD Action Plan
LEVERAGING ADVOCACY

- 4 bills signed into law, reshaping the Duchenne landscape
- 12,000 messages & action alerts sent to Members of Congress
- Over $600 million in federal funding leveraged by PPMD community into research
- More than 2,000 meetings face-to-face with Members of Congress
ADVANCING RESEARCH

• Preclinical research
• Clinical research
• Trial infrastructure
• Outcome measures

• Biomarker development
• Natural history studies
• Expert meetings
• Repurposing drugs

$50 million+ invested by PPMD into Duchenne research & therapy development to date

Over 260 research grants awarded, supporting nearly every major therapeutic approach

~35 clinical trials in Duchenne at any given time

2 US drug approvals with additional promising therapies in development
EMA Granted Conditional Approval - 2014

FDA Granted Accelerated Approval - 2016

FDA Granted Full Approval - 2017

Parent Project Muscular Dystrophy

Duchenne Drug Development Pipeline 2019

* = will recruit/recruiting globally
*** = will recruit/recruiting EU only
**** = will recruit/recruiting Japan only
Therapeutic Strategies to Treating Duchenne

- Restoring or Replacing Dystrophin
- Combating Fibrosis
- Regulating Calcium Balance
- Reducing Inflammation
- Improving Muscle Growth & Protection
- Restoring Cellular Energy
- Improving Heart Function
DUCHENNE IMPACTS MORE THAN JUST MUSCLE
ADVANCING CARE

- 25 clinics awarded certification by PPMD across the US as of June 2019
- 700 care providers in PPMD network of certified clinics
- 20 years of identifying & addressing gaps in care through specialty workshops & consensus meetings
- 10 years added to average lifespan due to PPMD-led advances in care
ENGAGEMENT IN THE COMMUNITY

- Over 5000 patients & carriers across the globe have registered in The Duchenne Registry since launch.
- $36 million+ raised through Race to End Duchenne & family-led grassroots events since 1994.
- 14310 families reached in person, through 25 Annual Conferences, End Duchenne Tour stops, Roundtable discussions, & Advocacy Conferences.
- 26 PPMD Connect locations across the United States, providing local families outreach & mentoring.
Why WE are here...Drug Development Ecosystem

Government organizations
Patient groups
Universities
Companies
Regulations
Clinics
Economic factors
Federal government
Payers

Why WE are here...Drug Development Ecosystem
Thank You!