PPMD’s End Duchenne [Virtual] Tour: Atlanta, GA
Who is PPMD?

Pat Furlong
Founding President & CEO
1994 Questions

• What was the current state of research & care in Duchenne?
• What was the federal government investment in Duchenne?
• What data existed about the Duchenne population?
• What are the gaps we need to fill in order to move the needle toward better care & treatments for Duchenne?
Impact on Research

- $50 million+ invested by PPMD into Duchenne research & therapy to date
- Over 260 research grants awarded, supporting nearly every major therapeutic approach
- ~35 clinical trials in Duchenne at any given time
- 3 U.S. drug approvals with additional promising therapies in development

#EndDuchenne
The Drug Development Pipeline is full of potential treatments that are being tested. These include therapeutic approaches that restore or replace dystrophin and those that treat Duchenne symptoms. The goal? To test combinations of these therapies to create the best “cocktail” for each patient.
For over two decades, Parent Project Muscular Dystrophy (PPMD) has contributed to each stage of the drug development pipeline, awarding grants, filling in critical gaps, convening stakeholders, and redefining the clinical trial landscape.
Impact on 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

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How YOU Can Advocate

- Sign up to become an advocate to receive action alerts in your inbox at ParentProjectMD.org/AdvocacyAlerts
- PPMD can assist in organizing in district meetings with your local representatives
- Contribute to our preference studies and represent our community at meetings with federal agencies
- Adults living with Duchenne were the first patient representatives to serve on an FDA Advisory Committee for FDA decisions
- Attend PPMD’s Advocacy Conference
  - Held Q1 each year in Washington, DC - scheduled visits on Capitol Hill are arranged for all attendees to meet with the offices of their congressional members and tell their stories

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Impact on Care

- added to average lifespan due to PPMD-led advances
- awarded certification by PPMD across the US as of January 2020
- in PPMD network of certified clinics
- of identifying & addressing gaps in care through specialty workshops & consensus meetings

10 years
27 clinics
700 care providers
20 years
• In partnership with the CDC
• First published in January 2010, with updated guidelines published in 2018 in *Lancet Neurology*
• Diagnosis and management of Duchenne muscular dystrophy (3 parts):
  – Part 1: Diagnosis, neuromuscular, rehabilitation, endocrine, & gastrointestinal and nutritional management
  – Part 2: Respiratory, cardiac, bone health, & orthopedic management
  – Part 3: Primary care, emergency management, psychosocial care, & transitions of care across the lifespan
• Lead to the development of the Certified Duchenne Care Center program (2014)
  – Care Considerations = requirements for certification
  – Certification requirements were adapted for adult certification, with the first adult CDCC certified in 2019
  – Expanded program globally to include 2 international centers
• PPMD also lead the effort to an ICD-10 code approval specifically for Duchenne/Becker to
  – Goal: improve longitudinal surveillance of data
PPMD’S CERTIFIED DUCHENNE CARE CENTER PROGRAM

Plus, two Global Certified Duchenne Care Centers: Red Cross War Memorial Children’s Hospital (South Africa) and Motol University Hospital in (Czech Republic)

*As of January 2020
Engagement in the Community

- 26 PPMD Connect locations across the United States, providing local families outreach & mentoring
- Over 5,000 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
- 14,310 families reached in person, through 25 Annual Conferences, End Duchenne Tour stops, Roundtable discussions, & Advocacy Conferences

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Coach To Cure MD

- Coach To Cure MD, presented by Werner Ladder, is a partnership between the American Football Coaches Association (AFCA) and PPMD
- This year on September 26, 2020 coaches nationwide will wear a Coach to Cure MD patch and mention the program in interviews
- Know a team who would be interested? Interested in attending a game near you?
- CoachToCureMD.org

Georgia teams currently participating:
- Berry College
- Georgia State University

#EndDuchenne
PPMD’s 2020 Annual Conference

• Join over 700 families, healthcare professionals, and industry partners at PPMD’s annual conference to learn the latest progress in the fight to end Duchenne
• June 25 – 28 at the Phoenician in Scottsdale, AZ
• ParentProjectMD.org/Conference

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Other Ways to Get Involved

Find a local PPMD Connect group

Race to End Duchenne

Local DIY Events

#EndDuchenne
Comprehensive Duchenne Care
Duchenne is not just a disease of the muscles. It affects every system in the human body.
Care Guides

- Duchenne Family Guide
  - Partnered with MDA, Treat-NMD, and WDO
  - Includes the Duchenne Care Considerations in an “easier to digest” format

- New Diagnosis and Early Care Guide
  - Introduction to Duchenne/Becker and genetic testing, adjusting to the diagnosis and support, early care, and introduction to clinical trials

- Imperatives for Healthcare Providers
  - One-page fact sheets for healthcare providers unfamiliar with Duchenne
  - Versions for pediatric and adult patients
  - Also available in Spanish

- Education Matters
  - Comprehensive guides for parents and teachers
Emergency Care Information

• PPMD App
  – Includes Duchenne emergency information
• PPMD emergency information cards
  – Also available in Spanish
• PPMD large weatherproof emergency information cards for wheelchairs, scooters, and backpacks
• PJ Nicholoff Steroid Protocol
  – Guide for families and healthcare providers to navigate steroid stress dosing, tapering, and what to do in the case of a missed dose
• Online Resources
  – Emergency care and hospital checklists
  – Caring for the Flu
  – Fracture management

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