PPMD’s End Duchenne [Remote] Tour: Portland, OR
Who is PPMD?

Pat Furlong
Founder & CEO, PPMD
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?

Critical Missing Pieces:

- Data
- Federal Dollars
- Industry
- Outcome Measures
- Care Standards
- Advocacy
- Natural History

#EndDuchenne
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

- Signed into law, reshaping the Duchenne landscape
- Sent to Members of Congress
- Over $600 million in federal funding leveraged by PPMD community into research
- More than 2,000 face-to-face meetings with Members of Congress

#EndDuchenne
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 in Washington, DC
  – Will take place February 28 – March 2nd with scheduled visits on Capitol Hill are arranged for all attendees to meet with the offices of their congressional members and tell their stories

#EndDuchenne
Impact on Care

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

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Duchenne Care Considerations

- 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
- Led 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 led 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

27 CENTERS across the United States
700 CARE PROVIDERS caring for individuals with Duchenne
3,300 PATIENTS served at certified centers
79 CLINICAL TRIALS located at certified centers
+1,150 TRIAL PARTICIPANTS currently enrolled or in active recruitment

*As of January 2020

SEATTLE CHILDREN’S HOSPITAL
UCSF
UC DAVIS
UCLA
STANFORD CHILDREN’S HOSPITAL
PRIMARY CHILDREN’S HOSPITAL
CHILDREN’S HOSPITAL COLORADO
CHILDREN’S MERCY HOSPITAL
CHILDREN’S HEALTH & UT SOUTHWESTERN
UNIVERSITY OF IOWA CHILDREN’S HOSPITAL
AMERICAN FAMILY CHILDREN’S HOSPITAL
CHILDREN’S HOSPITAL WISCONSIN
AKRON CHILDREN’S HOSPITAL
LURIE CHILDREN’S HOSPITAL
UNIVERSITY OF ROCHESTER
NATIONWIDE CHILDREN’S HOSPITAL
YALE NEW HAVEN CHILDREN’S HOSPITAL
NEMOURS/ALFRED I. DUPONT HOSPITAL FOR CHILDREN
KENNEDY KRIEGER INSTITUTE
CHILDREN’S HOSPITAL OF THE KING’S DAUGHTERS
CHILDREN’S NATIONAL HOSPITAL
CHILDREN’S HOSPITAL OF RICHMOND AT VCU
DUKE UNIVERSITY MEDICAL CENTER
AMERICAN FAMILY CHILDREN’S HOSPITAL
UNIVERSITY OF ROCHESTER
NATIONWIDE CHILDREN’S HOSPITAL
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CHILDREN’S HOSPITAL OF RICHMOND AT VCU
DUKE UNIVERSITY MEDICAL CENTER

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 July 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

#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 23-27 at the Omni Orlando Resort at Champion’s Gate in Orlando, FL
- ParentProjectMD.org/Conference

#EndDuchenne
Other Ways to Get Involved

Find a local PPMD Connect group

Race to End Duchenne

Local DIY Events

#EndDuchenne