PPMD’s End Duchenne [Remote] Tour: Long Island, NY
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<tr>
<th>Time</th>
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| 10:00 am – 10:05 am | Welcome                                         | Ryan Fischer  
 *Parent Project Muscular Dystrophy* |
| 10:05 am – 10:20 am | Who is PPMD?                                     | Ryan Fischer  
 *Parent Project Muscular Dystrophy* |
| 10:20 am – 10:30 am | Get Involved                                     | Dianne Carroll  
 *Parent, PPMD Connect Coordinator* |
| 10:30 am – 10:40 am | Hope for Javier                                  | Jen Portnoy  
 *Parent, Founder* |
| 10:40 am – 11:10 pm | Genetics, The Duchenne Registry and Your Family  | Niki Armstrong, MS, CGC  
 *Parent Project Muscular Dystrophy* |
|                  | Understanding Your Mutation                     |                                                                              |
|                  | Decode Duchenne                                  |                                                                              |
|                  | The Duchenne Registry                            |                                                                              |
| 11:10 am – 12:30 pm | Duchenne Expert Panel: Research and Care         | Moderator: Ryan Fischer  
 *Parent Project Muscular Dystrophy* |
|                  | Cardiology Standards of Care                    | Peter Morelli, MD  
 *Stony Brook Children’s* |
|                  | Neuromuscular Standards of Care                 | Yaacov Anziska, MD  
 *SUNY Downstate* |
|                  | Physical Therapy                                | Christina Rao, PT  
 *Yale-New Haven Children’s Hospital* |
|                  | Care Coordination and Local Resources           | Dawn Dawson, CPNP/ANP  
 *Stony Brook Children’s* |
Who is PPMD?

Ryan Fischer
SVP, Community Engagement
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 in Duchenne at any given time
- ~35 clinical trials
- 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 face-to-face meetings 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
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

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

New York teams who have participated:
- Hamilton College
- Rensselaer Polytech Institute
- Morrisville State
- Hobart College
- The College at Brockport
- St. John Fischer College
- Pace University
- Marist College Football
- Buffalo State College
- Syracuse University Football
- Utica College

#EndDuchenne
PPMD’s 2020 Annual Conference

• PPMD will be hosting our annual conference virtually **July 22-25**
  – The PPMD team is committed to making this conference as complete and robust as our in-person meeting including panels about research, care & advocacy – even opportunities for virtual meet-ups with other families!
  – **For updates, please visit: ParentProjectMD.org/Conference**
• We will also be hosting a virtual newly diagnosed track starting the week of **June 22nd** with a virtual meet & greet with other families, Pat Furlong, and other PPMD staff members followed by a "Duchenne 101" webinar the week of **July 9th**
  – **To register for this track, please visit:**
    join.parentprojectmd.org/newlydiagnosedscholarship

#EndDuchenne
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

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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
  - COVID-19 Resource Center
  - Emergency care and hospital checklists
  - Fracture management

#EndDuchenne
Duchenne Expert Panel

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Please submit your questions through the chat box
Thank you!

ENDDUCHENNE.ORG