PPMD End Duchenne Tour
Parent Project Muscular Dystrophy

Overview

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Grassroots Advocacy
1994 - Questions and Missing Pieces

- What was the current state of research and care in Duchenne?
- What was the federal government investment in Duchenne?
- What data existed about the Duchenne population?
- How do we move the needle toward better care and treatments for Duchenne?

Care standards
Natural history data
Outcome measures

Federal investment & coordination
Companies on board
Mobilized grassroots community
Results 2001 - Present

Care
• Care considerations published and updated
• Certified Duchenne Care Centers

Research
• Wellstone Centers of Excellence
• Animal Studies
• Basic and Translational Research Grants

Data collection
• Outcome measures
• Natural History Studies
• MD-STARnet Surveillance
• ICD-10 Code

Federal Coordination and funding
• MD Coordinating Committee grows
• Action Plan for MD’s
• 500+ million in Duchenne Funding

Drug Development & Access
• 2 Approved Therapies
• 45+ Companies
• Pipeline Full of Hope
• Pre-competitive, collaborative consortium (PPMD Duchenne Drug Development Roundtable)
• Payer Engagement
ADVOCA CY

• 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.

ANNUAL ADVOCACY CONFERENCE
Federal Agencies We Currently Focus On

For all agencies
We advocate to impact:
Focus
Funding
or
Flexibility
PPMD Hill Days
Building
Congressional
Champions

Appropriations Bill &
Annual Report Language
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.
• Decreased heart function
• Cardiomyopathy
• Leads to heart failure

Duchenne affects more than just muscle

Duchenne Muscular Dystrophy Care Considerations

Duchenne muscular dystrophy (DMD) is a rare genetic (inherited) disease defined by muscle weakness that gets worse over time and ultimately affects the heart and lungs. People born with DMD will see many healthcare providers throughout their lives.

The updated care considerations for DMD are contained in a set of three articles published in *Lancet Neurology*. They offer the latest clinical considerations to improve care and quality of life for people living with DMD. Clinicians who specialize in treating people with DMD developed these care considerations based on current evidence for optimal care reported in the literature and their own clinical experiences.

As science and medicine are advancing, people with DMD are living longer; therefore, their care throughout life is evolving as well. These care considerations, funded by the Centers for Disease Control and Prevention (CDC), are intended to raise the standards of care, help clinicians provide the best possible care to people with DMD, and give families and caregivers the necessary information to manage their care.

Read the articles here:

- [Diagnosis and management of Duchenne muscular dystrophy. Part 1: Diagnosis, neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management](#)
- [Diagnosis and management of Duchenne muscular dystrophy. Part 2: Respiratory, cardiac, bone health, and orthopaedic management](#)
- [Diagnosis and management of Duchenne muscular dystrophy. Part 3: Primary care, emergency management, psychosocial care, and transitions of care across the lifespan](#)
An Introduction to the Updated 2018 Clinical Care Guidance on the Diagnosis and Management of Duchenne

Neurology Care, Diagnostics, and Emerging Therapies of the Patient with Duchenne

Rehabilitation Management of the Patient with Duchenne

Bone Health and Osteoporosis Management of the Patient with Duchenne

Obesity and Endocrine Management of the Patient with Duchenne

Nutritional and Gastrointestinal Management of the Patient with Duchenne

Respiratory Management of the Patient with Duchenne

Cardiac Management of the Patient with Duchenne

Orthopedic and Surgical Management of the Patient with Duchenne

Primary Care and Emergency Department Management of the Patient with Duchenne

Psychosocial Management of the Patient with Duchenne

A Transition Toolkit for Duchenne

Evaluating Implementation of the Updated Care Considerations for Duchenne
Duchenne Family Guide

• Collaborative effort between PPMD, WDO, TREAT NMD and MDA

• Being translated into multiple languages

• Available for download and printing on PPMD website

• Soon to be available in hard copy
Imperatives for DUCHENNE MD

• Collaborative effort between PPMD, WDO and TREAT - NMD
• 1 page snapshot of recommended care
• Helpful for providers and families
• Available on PPMD (English) and on the TREAT NMD site in multiple languages
• New Adult Imperatives Coming!!!
Certified Duchenne Care Center Program

– Operationalization of the CDC Care Guidelines
– Creating a network that will:

• Provide care in alignment with the Standards of the Certified Duchenne Care Center Program which are in agreement with the CDC Care Considerations
26 Certified Duchenne Care Centers!

- Seattle Children's Hospital
- Gillette Children's Hospital
- American Family Children's Hospital
- University of Rochester
- Children's Hospital Wisconsin
- Lurie Children's Hospital
- Massachusetts General Hospital
- Yale New Haven Children's Hospital
- Nationwide Children's Hospital
- Nemours/Aldred L. DuPont Hospital for Children
- Kennedy Krieger Institute
- Children's National Hospital
- Children's Hospital of the King's Daughters
- UVA
- Duke University Medical Center

- UCSF
- UC Davis
- Stanford Children's Hospital
- UCLA
- Children's Hospital L.A.
- Children's Hospital Colorado
- Children's Mercy Hospital
- University of Iowa Children's Hospital
- Cincinnati Children's Hospital

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Continuing to Improve the CDCCs

• Defining a dashboard and metrics
  – New ICD 10 code will enable patient/service identification
  – Eventually evaluate longitudinal follow up of patient outcomes
• Updating requirements for certification aligned with updated Care Considerations
• Requirements for Adult Certified Duchenne Care Centers
• Global efforts to standardize care
PPMD Care Resources

- PPMD Care Pages
  - By stage of disease
  - By system
- Emergency Card
- Wheelchair Card!
- Emergency checklists
- Mobile app
- FACES Coordinators
- PPMD
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.
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.
Access & Reimbursement
Our Path to Access?

Photo credit: Conrad Reynoldson
ACCESS & COVERAGE RESOURCES

With the first two FDA approvals of Duchenne therapies, the Duchenne community has entered in a new environment for access and reimbursement. With this brave new world at play, PPMD has been leading the effort to educate and guide the community through these unchartered waters.

OVERVIEW OF THE FUNDAMENTALS OF ACCESS

- What are the major differences with Medicaid vs private insurance?
- What is the process for appealing a denial, what are the layers of appeal?
- What are Medical vs. Drug Benefits?
- What is the role of a PBM’s or Pharmacy Benefit Managers?
- Types of Insurance Coverage?
- What are formularies?

These webinars are non-product specific, but can provide you with a basic understanding about the path to access.
PPMD Duchenne Newborn Screening Program

Duchenne NBS Steering Committee

- Care Considerations for Infants with DMD WG
- Outreach & Education to Patients & HCPs WG
- NBSTRN Integration and LPDR WG
- Laboratory Test Refinement WG
- Evidence Review WG
- Ethical & Legal Considerations WG

- PPMD Convened Meeting of Stakeholders to Discuss Model of Pilot: October 2017; Pilot launched October 2018
- Complete CK Assay Validation Studies: FDA approval of PKI kit expected Q1 2019

Contact Annie@parentprojectmd.org
Resource Categories

Tracking payer determinations by state & payer and integrating them into our website...
PPMD’s Connect Program

• PPMD’s Connect (formally FACES) is the official volunteer, parent-led outreach program of Parent Project Muscular Dystrophy.

• Serves as a regional PPMD point of contact for families affected by Duchenne and Becker.

• Goals of the Connect program:
  • **Family Mentoring:** To help local and regional families better understand the diagnosis of Duchenne or Becker muscular dystrophy and connect them with local resources.
  
  • **Grassroots Outreach:** To support national awareness and advocacy campaigns.
  
  • **Fundraising:** To support PPMD research, advocacy, care, and education programs.
How to Find Your Local Group

• **New Jersey**
  Facebook: PPMD’s Connect New Jersey
  ppmdconnectnewjersey@gmail.com

• **Western PA**
  Facebook: PPMD’s Connect Western PA
  ppmdconnectwesternpa@gmail.com

• **Virginia**
  Facebook: PPMD’s Connect Virginia
  ppmdconnectcentralvirginia@gmail.com

• **DMV** (DC, Maryland, Northern VA)
  Facebook: PPMD’s Connect DMV
  **Currently looking for a coordinator for this group, if interested please email connect@parentprojectmd.org.**
What Can YOU Do ?!

Advocate in Washington, DC!
March 1-3, 2020

PPMD’s Annual Conference
June 25-28, 2020
Scottsdale, AZ

Coach to Cure MD
September 28

Race to End Duchenne!