PPMD End Duchenne Tour
Parent Project Muscular Dystrophy

Overview

Pat Furlong
President, CEO
PPMD
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?
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
• 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:
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

Possible learning, behavior and psychosocial issues (ADD, OCD, speech, learning, behavior, sensory, psychosocial, anxiety, depression)

Weakened diaphragm
• Difficulties with airway clearance
• Need for assistive ventilation
• Risk of pneumonia

Osteoporosis, osteopenia
• Risk of fractures

Loss of skeletal muscle
• Weakness
• Decreased function
• Decreased ROM

Constipation
• GERD
• Stones
• Incontinence

Weakened diaphragm
• Difficulties with airway clearance
• Need for assistive ventilation
• Risk of pneumonia

Risk of fractures

Constipation
• GERD
• Stones
• Incontinence
Updated Duchenne Care Considerations
Published January 2018

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](#)

- All sections updated to reflect progress in care & research
- All sections updated to reflect care through the lifespan of Duchenne, not just pediatric care
- New sections within 2018 CDC Care Considerations:
  - Primary/Emergency Care
  - Endocrine Management
  - Transition of Care for Teens & Adults

PARENT PROJECT MUSCULAR DYSTROPHY | ENDDUCHENNE.ORG
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
• 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
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
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
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
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.
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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.

**DISCOVERY & PRECLINICAL**
- Exploratory research awards
- Validation & replication study services
- Updated Duchenne Care Consideration Guidelines & Family Guide
- Duchenne Newborn Screening Program
- The Duchenne Registry
- ChildMuscleWeakness.org
- An early diagnosis program
- AAP motor delay tool
- ICD-10 code refinement

**TRIAL READINESS/PHASE 1**
- Certified Duchenne Care Center program & Clinical Trial Awareness program
- Duchenne Specialty Care Workshops
- PPMD / C-Path Duchenne Regulatory Science Consortium
- Duchenne Drug Development Roundtable – engaging sponsors in pre-competitive space
- Partnering with federal agencies (MDCC, FDA, CDC, NIH, DoD, CMS, SSA)
- The Duchenne Registry trial readiness services
- Duchenne FDA Guidance for industry

**PHASE 2/3 & RECRUITMENT**
- Trial education and recruitment
- Duchenne community engagement
- Leading creation of forward thinking expert publications, i.e.: Putting Patients First: Patients are Waiting, & numerous patient & caregiver preference study publications.
- Advisory Committee & IND meeting support
- Leading passage of 5 federal bills, securing Duchenne-specific federal funding, & supporting rare disease legislation.

**REGULATORY APPROVAL**
- Clinical trial support
- Drug development research awards
- FDA & regulatory engagement
- The Duchenne Registry trial recruitment services
- Multichannel community outreach & education series
- Clinical trial participant education
- Expert consultation informing trial enrollment & design

**POST-MARKET & ACCESS**
- Pioneering access, coverage, & reimbursement strategy
- Decode Duchenne, free genetic testing
- Patient engagement initiatives
- Post-marketing strategy development
- Payer engagement
Finalized February 2018

“The newly finalized Guidance … was preceded by a pioneering effort from Parent Project Muscular Dystrophy who, in 2014, submitted their own independent proposed draft guidance that provided important scientific and patient input from the DMD community.

It helped inform the FDA’s development of both our own draft guidance and the final version issued today.”

- Commissioner Scott Gottlieb
**Goals:**

- To identify *current* policy, care, and clinical trial priorities among our Duchenne community members (by sub-population).

- To begin to identify measures of impact not currently captured in health economic models or value frameworks.

**The Audience:** Included federal agency & pharmaceutical industry partners

**More than 400 members of our Duchenne community participated**
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 uncharted 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
State Specific Resources –
22 PPMD Connect Groups & Growing
Resource Categories

Tracking payer determinations by state & payer and integrating them into our website...
PPMD School Advocacy Resources

Brain Pop Video
PPMD’s 4-minute animated video about Duchenne for kids of all ages.
https://www.youtube.com/watch?v=6wLnR7GJakY

Education Matters For Parents
Helpful tips on how to talk about Duchenne
Issues to be aware of at school
An Individualized Education Plan (IEP) overview
Sample scenarios and suggested ways to respond

Education Matters For Teachers
A practical overview of Duchenne
Classroom accommodations
Suggested inclusive school activities
What to be aware of regarding treatment/medications
Confidentiality/sensitivity concerns

School Presentation Materials
‘Chocolate Chip Cookie’ class presentation
http://www.parentprojectmd.org/site/PageServer?pagename=Understand_professionals_teachers
What Can YOU Do ?!

• Advocate!!                                March 3-5

• RUN!!!

• Coach to Cure MD!!

• Join a Connect Group!

F.A.C.E.S.

Families Advocating, Connecting, Educating, and Supporting

PARENT PROJECT MUSCULAR DYSTROPHY | ENDDUCHENNE.ORG
Race to End Duchenne

Rock ‘n’ Roll Las Vegas Marathon

November 16 & 17, 2019
Race to End Duchenne

• PPMD’s signature endurance program
• More than $12 million raised since it started in 2005
• Teams at major marathons such as TCS New York City Marathon, Bank of America Chicago Marathon & Chevron Houston Marathon
• Also participate in 5K, 10K, bike races, triathlons and more!
• Runners commit to raise funds to support PPMD’s mission
• Benefits include: private team gatherings during race weekend, official team gear, personal fundraising page and more
Rock ‘n’ Roll Las Vegas Marathon

5K
Saturday, Nov. 16
6 pm

10K
Sunday, Nov. 17
4 pm

Full & Half Marathons
Sunday, Nov. 17
4:30 pm

In exchange for raising funds to support PPMD’s mission, runners receive:

- Discounted race entry
- Personal fundraising page
- Invitations to our private team celebration event
- Official Race to End Duchenne team race shirt and t-shirt
- Dedicated staff person to answer questions and provide support.
PPMD’s Connect
Southern California

Rhiannon Hubbard & Polly Sundeen
Coordinators
PPMD’s Connect
Southern California

• 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 Us

Facebook:
www.facebook.com/groups/PPMDCConnectSoCal

Email:
ppmdconnectnebraska@gmail.com
Thank you!