PPMD End Duchenne Tour
Parent Project Muscular Dystrophy

Overview

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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
ADVOCACY

- 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.
Duchenne affects more than just muscle

- Decreased heart function
- Cardiomyopathy
- Leads to heart failure

- 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

- Decreased heart function
- Cardiomyopathy
- Leads to heart failure
Updated Duchenne Care Considerations

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
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
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
By April 2019 -
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.
<table>
<thead>
<tr>
<th>Drug/Method</th>
<th>Phase</th>
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<tr>
<td>Exondys 51 (Eteplirsen) [Sarepta]</td>
<td>Pre-clin</td>
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<tr>
<td>Emflaza [PTC Therapeutics]</td>
<td>Phase I</td>
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<td>Spironolactone &amp; Eplerenone [Ohio State University]</td>
<td>Phase I/I</td>
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<td>Translarna (Ataluren) [PTC Therapeutics]</td>
<td>Phase II</td>
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<td>Givinostat [Italfarmaco] *</td>
<td>Phase I/I</td>
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<td>Raxone (Idebenone) [Santhera] *</td>
<td>Phase III</td>
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<tr>
<td>SRP-4045/SRP-4053 [Sarepta] *</td>
<td>Phase III</td>
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<td>RG6206 [Roche] *</td>
<td>Phase III</td>
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<td>Edasalonexent (CAT-1004) [Catabasis]</td>
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<td>Vamorolone (VBP15) [Reveragen] *</td>
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<td>Ezutromid (SMT C1100) [Summit PLC] *</td>
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<td>Pamrevlumab (FG-3019) [Fibrogen]</td>
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<td>Epicatechin</td>
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<td>NS-065/NCNP-01 [NS Pharma]</td>
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<td>Follistatin Gene Transfer [Nationwide Children's]</td>
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<td>MNK-1411 Cosyntropin Acetate [Mallinckrodt] *</td>
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<td>Myoblast Transplantation [Chu De Quebec]</td>
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<td>Exon Skipping 53 [Daichi - Sankyo] ***</td>
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<td>Nationwide Micro-Dystrophin Gene Transfer</td>
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<td>WVE-210201 Exon 51 Skipping [WAVE]</td>
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<td>Nationwide GaIGT2 Gene Therapy [Nationwide]</td>
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<td>PF-06939926 Mini-Dystrophin Gene Therapy [Pfizer]</td>
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<tr>
<td>SGT-001 Micro-Dystrophin Gene Transfer [Solid]</td>
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<td>Rimeporide [EspeRare] **</td>
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<td>AT-300 [Akashi]</td>
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<td>Nationwide Exon 2 Skipping for Duplication 2...</td>
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<td>Tamoxifen** [University of Geneva]</td>
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<td>MA-0211/MTB-1 [Mitobridge/Astellas]</td>
<td>Phase III</td>
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BROADLY ENGAGING THE DEVELOPMENT PIPELINE

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
Access & Reimbursement
Our Path to Access?

Photo credit: Conrad Reynolds
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
State Specific Resources –
22 PPMD Connect Groups & Growing
Resource Categories

Tracking payer determinations by state & payer and integrating them into our website...
What Can YOU Do ?!

- Advocate!! Washington DC!!
- RUN!!!

Coach to Cure MD!!

- Join a Connect Group!