Advocacy – Now More Than Ever

JOIN THE FIGHT.
END DUCHENNE.

Annie Kennedy &
Ryan Fischer
Today –
Duchenne community is among the most empowered & effective patient advocacy communities in Washington
PPMD has played a vital role in every single victory in the fight to end Duchenne since 1994.

1994
PPMD founded and 1st Annual Conference

1997
PPMD invests in first Duchenne Muscular Dystrophy Research Center

1998
Standards become gold standard

1999
MD CARE Act signed into law

2000
1st Annual Advocacy Conference held

2001
MD CARE Act passed

2003
PPMD funds first Duchenne Drug Discovery Program

2006
PPMD and OSI partner community thought leaders to develop Standards of Care

2009
Standards of Care published

2012
PPMD helps lead the way for FDA to be passed by Congress, mandating regulatory flexibility for rare diseases

2014
PPMD leads a community effort to draft Guidance for the FDA around Duchenne
   PPMD certifies first clinic as part of Certified Duchenne Care Center Program

2015
PPMD funding creates infusion center for children
   companies focused on Duchenne

2016
1st approval in Duchenne – Exondys 51

2017
PPMD announces largest grant to date – $2.2 million – and launches Gene Therapy Initiative
   21st Century Cures signed into law

2018
- Expand Gene Therapy Initiative
- Certify additional Duchenne Care Centers (18 have been established)
- Launch Global Certified Duchenne Care Center Program with first international certification
- Expand Duchenne registry to more countries (Australian portal added this year)
- Continue leading efforts to obtain an ICD code for Duchenne
- Continue leading Newborn Screening effort to ensure early diagnosis
- PPMD End Duchenne Tour to visit 8 more states, both served/engaged and underserved populations

*Sigh* Duchenne world would be years behind where it is today without PPMD

DR. LEE SWEEDEY

“Passing the MD-CARE Act was a critical step...”
SEN. ROGER WICKER

“They’re doing everything.”
MINDY CAMERON, PARENT

“PPMD has been the spark that ignited the flame of determination in all of us...”
DR. BRENDA WONG

*Pat and PPMD provided a voice to the community...*
DR. ERIC HOFFMANN
One Connection

One Congressional Member

One Champion... at a Time.
The Muscular Dystrophy Community Assistance, Research & Education Act (MD-CARE Act)
The Muscular Dystrophy Community Assistance, Research & Education Act (MD-CARE Act)
The Muscular Dystrophy Community Assistance, Research & Education Act (MD-CARE Act)

**MD-CARE Act 2001**

Established:
- Centers of Excellence
- MD STARnet tracking and surveillance
- MD Coordinating Committee - MDCC to develop ‘MD Action Plan’

**MD-CARE Act Amendment 2008**

- Added the National Heart, Lung, and Blood Institute (NHLBI) to MDCC
- Enhancement of clinical research
- Expansion of MD-STARnet
- Authorized development of Duchenne Care Considerations – Develop and Disseminate

**Paul D Wellstone MD-CARE Act Amendment 2014**

- Expanded research to focus on Endocrine, Pulmonary, Cardiac & Transition into Adulthood
- Additional federal agencies added as members of Coordinating committee
- Sharing of data from MD-STARnet with community researchers
- Update of DBMD Care Considerations to include adults with Duchenne & reflect care advances since 2010
- Update of initial ‘MD Action Plan’ by MDCC
Federal Agencies We Currently Collaborate With
PPMD Hill Days
Building Congressional Champions

Appropriations Bill & Annual Report Language
Developing Relationships –
So that Congressional leaders view ALL Issues through the lens of our
Duchenne Community
Sometimes those friendships formed in your backyard go places…
Senator Blumenthal – viewing policies & issues through our Duchenne lens
PDUFA V – A Game-Changer For Patient Communities

In the spirit of Patient Focused Drug Development (developing ‘tools of engagement’). . . .

✓ *Putting Patients First* white paper
✓ PPMD Benefit-risk studies
✓ *Patients are Waiting* white paper
✓ Draft Guidance on Duchenne
✓ Registry data
✓ PROs
✓ Testimony from patient community & clinical experts
✓ Meaningful engagement with the FDA
The Patient Focused Impact Assessment Act (PFIA)

Passed in the Senate -- and then later became a key provision within 21CC
“The 21st Century Cures bill codifies patient focused drug development as a part of the FDA’s mission.”

- Janet Woodcock

in a listening session with Commissioner Califf on the day of the bill’s signing
The PPMD community has led the passage of 5 Congressional Bills!

Muscular Dystrophy Community Assistance Research & Education Act (MD-CARE Act) 2001

Paul D. Wellstone MD-CARE Amendments 2008

Paul D. Wellstone MD-CARE Amendments 2014

Patient Focused Impact Assessment (PFIA) Act (2016)

Better Empowerment Now to Enhance Framework and Improve Treatments Act of 2017 (BENEFIT Act)
Duchenne Community – Prior to 1st Duchenne Approvals

- An Engaged Patient Community
- Published Standards of Care
- Well-characterized disease
- Natural History Studies
- Community-Specific Benefit-Risk data
- Biomarkers
- Guidance for Industry
- Validated screening test

- Developed Outcome Measures
- Collaborative Pharmaceutical Industry (DDDR)
- International patient registry
- Federal Agency Engagement
- National Surveillance Program (epidemiology)
- Certified Duchenne Care Center Network
It’s About Access
FDA Guidance on Duchenne Muscular Dystrophy

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
2017 DDDR Small Meeting Series

Key focus was how to apply the ‘regulatory learnings’ of 2015 & 2016 back into our robust Duchenne pipeline

• **Clinical Trial Readiness – Patient, Families, and Treating Physicians** (February 1, 2017)
• **Optimizing Clinical Trial Design** (March 1, 2017)
• **Clinical Trial Readiness – Sites & Investigators** (April 5, 2017)
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.
Roadmap for Navigating Path to Access

**STAGE 1**
Prescription written by Doctor and submitted to Insurer

**STAGE 2**
Patient, Drug Company, and Prescribing Doctor work with Insurer to process prescription

**STAGE 3**
Drug is provided to Patient
Our Path to Access?

Photo credit: Conrad Reynoldson
COMPASS MEETING REPORT

Working Together to Map our Pathway Forward
• Expanded on FDA’s Patient Focused Drug Development (PFDD) Workshop framework

• Created a new, innovative model – in collaboration with FDA
• Goals:
  – To identify current policy, care, and clinical trial priorities among our Duchenne community members (by sub-population)
  – To renew engagements with existing federal & industry partners
  – To begin to identify measures of impact not currently captured in health economic models or value frameworks
• All federal partners invited
• Community Advisory Board
• White paper published in advance of the meeting to provide context of current Duchenne landscape
• Webcast, live polling throughout Compass Meeting
Thank you, Community Advisory Board!
Panels – Represented the Duchenne journey

More than 400 members of our Duchenne community participated as a part of the discussion that day.

The Audience – included federal agency & Duchenne pharmaceutical industry partners
Panel 1 – Duchenne, the Early Journey & Elementary Years

Themes addressed:
• Emerging symptoms & diagnostic odyssey
• Seeking care & making treatment decisions
• Clinical trial participation
• Accommodating Duchenne in family life
Panel 2 – Transition Time: The ‘Tween & Teen Years

Themes addressed:
• Emotional time in Duchenne journey
• Physical features of transition phase
• Expanding care needs
• Access barriers
• Narrowing clinical trial options
• Social & educational needs
• Access & advocacy
• Future aspirations
Panel 3 – Reflecting on Duchenne’s Impact on the Family Unit

Themes addressed:
• Collateral costs of Duchenne
• Marital strains
• Loss of family privacy
• Impacted health of other family members
• Sibling impacts
• Joys between sorrows
Panel 4 – Through the Adult Lens

Issues highlighted:
• Life after high school
• Unfailing support of their families
• Adaptation & resilience
• Symptom impacts & Evolving trade-offs for benefits & risks of therapies and activities
• Clinical trials & Compassionate Use
• Need for Non-ambulatory outcome measures
• Access barriers
• Eligibility for transplant as heart becomes more compromised
• Mental health care
• Pain control
• Family planning
• Webcast, live polling
<table>
<thead>
<tr>
<th>Research Participation</th>
<th>Online young (n=24)</th>
<th>Online teen (n=31)</th>
<th>Online adult (n=19)</th>
<th>PFDD Totals (n=70)</th>
<th>Total (n=144)</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Clinical trial for an investigational drug therapy</td>
<td>6</td>
<td>17</td>
<td>9</td>
<td>40</td>
<td>72</td>
<td>50%</td>
</tr>
<tr>
<td>Natural history study</td>
<td>7</td>
<td>6</td>
<td>6</td>
<td>46</td>
<td>65</td>
<td>45%</td>
</tr>
<tr>
<td>MRI imaging study</td>
<td>2</td>
<td>11</td>
<td>4</td>
<td>21</td>
<td>38</td>
<td>26.39%</td>
</tr>
<tr>
<td>Have never participated in any of the above</td>
<td>12</td>
<td>9</td>
<td>7</td>
<td>8</td>
<td>36</td>
<td>25%</td>
</tr>
<tr>
<td>Clinical trial for a robotic device</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>8</td>
<td>8</td>
<td>5.56%</td>
</tr>
<tr>
<td>Clinical trial for gene or cell therapy</td>
<td>0</td>
<td>1</td>
<td>0</td>
<td>6</td>
<td>7</td>
<td>4.86%</td>
</tr>
</tbody>
</table>
When asked which three activities would be most important to preserve or maintain “right now,” here is how responses stacked up across groups:

<table>
<thead>
<tr>
<th>Activity to preserve or maintain</th>
<th>10 or younger (42 respondents)</th>
<th>11–17 (60 respondents)</th>
<th>18 or older (45 respondents)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Walk for a longer time</td>
<td>74% (ranked 1st)</td>
<td>not asked</td>
<td>not asked</td>
</tr>
<tr>
<td>Walk up stairs</td>
<td>65% (ranked 2nd)</td>
<td>not asked</td>
<td>not asked</td>
</tr>
<tr>
<td>Rise from the floor</td>
<td>60% (ranked 3rd)</td>
<td>not asked</td>
<td>not asked</td>
</tr>
<tr>
<td>Feed self/bring hands to mouth</td>
<td>7% (ranked 9th)</td>
<td>57% (ranked 1st)</td>
<td>64% (ranked 1st)</td>
</tr>
<tr>
<td>Walk around the home</td>
<td>not asked</td>
<td>42% (ranked 2nd)</td>
<td>not asked</td>
</tr>
<tr>
<td>Stand up from sitting in a chair</td>
<td>12% (ranked 7th)</td>
<td>37% (ranked 3rd)</td>
<td>not asked</td>
</tr>
<tr>
<td>Reposition self at night</td>
<td>not asked</td>
<td>not asked</td>
<td>53% (ranked 2nd)</td>
</tr>
<tr>
<td>Use cell phone/controller</td>
<td>not asked</td>
<td>32% (ranked 4th)</td>
<td>31% (ranked 3rd)</td>
</tr>
</tbody>
</table>
When asked which three measures of health would be most important to preserve or maintain “right now” at the current age:

<table>
<thead>
<tr>
<th>Global measure to improve or preserve</th>
<th>10 or younger (42 respondents)</th>
<th>11–17 (60 respondents)</th>
<th>18 or older (45 respondents)</th>
</tr>
</thead>
<tbody>
<tr>
<td>More energy</td>
<td>76% (ranked 1st)</td>
<td>47% (ranked 3rd)</td>
<td>36% (ranked 4th)</td>
</tr>
<tr>
<td>Stronger heart</td>
<td>40% (ranked 2nd)</td>
<td>53% (ranked 1st)</td>
<td>78% (tied for 1st)</td>
</tr>
<tr>
<td>Stronger bones</td>
<td>36% (ranked 3rd)</td>
<td>45% (ranked 4th)</td>
<td>53% (ranked 3rd)</td>
</tr>
<tr>
<td>Better behavior</td>
<td>31% (ranked 4th)</td>
<td>10% (ranked 9th)</td>
<td>not asked</td>
</tr>
<tr>
<td>Stronger breathing</td>
<td>26% (ranked 6th)</td>
<td>48% (ranked 2nd)</td>
<td>78% (tied for 1st)</td>
</tr>
<tr>
<td>Healthy weight</td>
<td>19% (ranked 8th)</td>
<td>40% (ranked 5th)</td>
<td>13% (ranked 8th)</td>
</tr>
</tbody>
</table>

* Responses in 11-17 age group distributed across 5 different measures; speaks to many physical transitions within this time period
Ramifications of denials & delays related to durable medical equipment (DME):

- Functional decline including acceleration of disease progression
- Risk of serious complications and possibly premature death
- Increased caregiver time and burden
- Increased out-of-pocket and system costs
- Risks of injury or physical functioning when using inappropriate and/or out-dated equipment
“Three formerly complete strangers now have keys to our home—our son’s caregivers. The respite time is nice for my wife and these state-paid helping hands relieve us of the stuff that sometimes grinds your attitude—bathing, toileting, etc. I’ve been around this community long enough to know things aren’t going to get easier. I consider these three people gifts our family, but they’re so difficult to find and they’re so difficult to secure with a solid schedule.”

—Lance, father of Micah, age 15
"Duchenne truly impacts every single aspect of our families' lives: the houses we live in, the cars we drive, the way we participate in activities, the activities we choose to participate in. It impacts every single thing with one exception—how much we deeply love our boys."

—Lisa
“I’m doing my best to manage the effects of Duchenne currently, but keeping stable and even getting a bit of strength from a new drug would mean a lot living with a degenerative disease.”

—Colin W, adult with Duchenne
• Report being published today

• Action Item implementation begins this summer
Thank you!