



PPMD End Duchenne Tour

Parent Project Muscular Dystrophy *Overview*

Annie Kennedy
Senior Vice President
Legislation & Policy

Parent Project Muscular Dystrophy
JOIN THE FIGHT.
END DUCHENNE.



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?



Critical Missing Pieces

Care standards
Natural history data
Outcome measures
Federal investment & coordination
Companies
Mobilized grassroots community

Results 2001 - Present

Care

- Care considerations published
- Care much more standardized (clinics)

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**

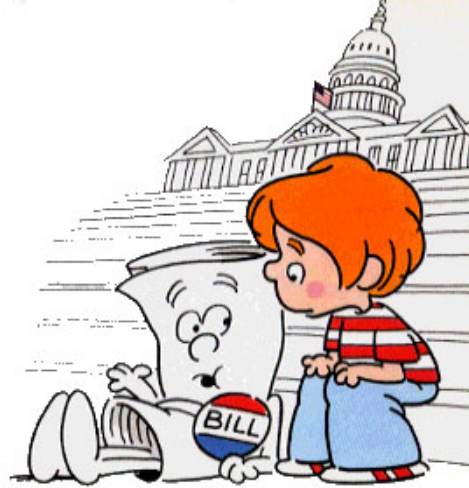


The Muscular Dystrophy Community Assistance, Research & Education Act (MD-CARE Act)

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
- 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

The PPMD community has led the passage of 5 Congressional Bills!

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

**Patient Focused Impact Assessment
(PFIA) Act (2016)
*(became a key provision of 21 CC)***

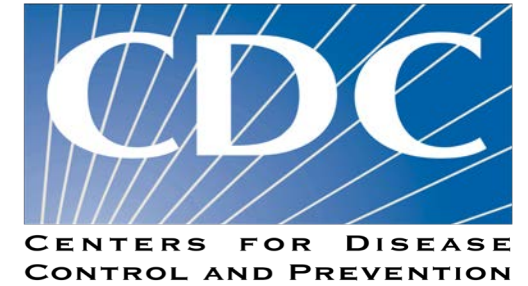
**Paul D. Wellstone MD-CARE
Amendments 2008**

**Paul D. Wellstone MD-CARE
Amendments 2014**

**Better Empowerment Now to Enhance
Framework and Improve Treatments
Act of 2017
(BENEFIT Act)**



Federal Agencies We Currently Collaborate With



National Institutes of Health





PPMD Hill Days

Building Congressional Champions



Appropriations Bill & Annual Report Language



ADVOCACY

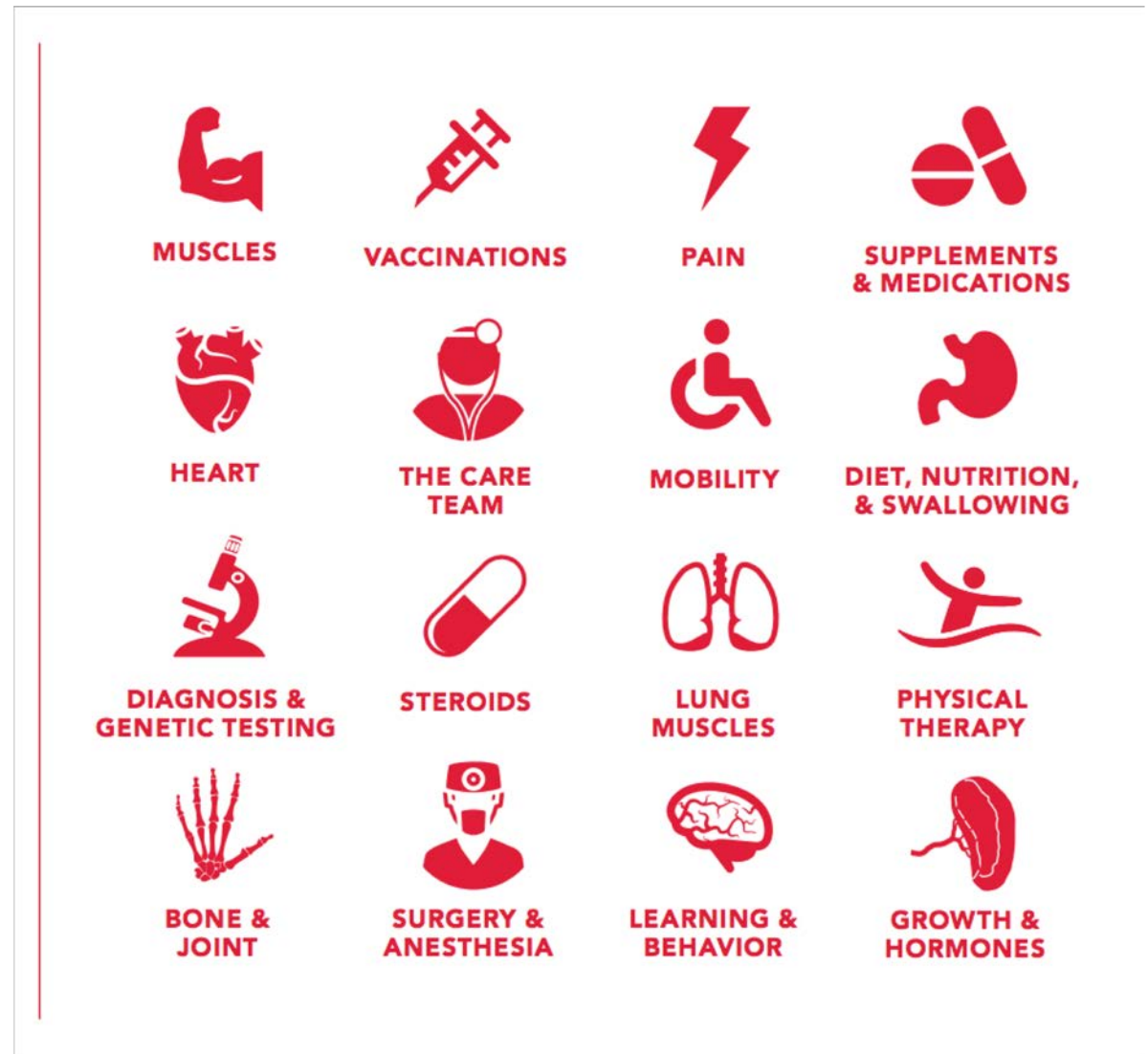
- **The only annual Advocacy Conference** focused on Duchenne
- Focused on **advancing MD-CARE Act** as well as regulatory policy
- **Prioritize impacting:**
 - Food and Drug Administration (FDA)
 - Center for Disease Control (CDC)
 - National Institutes of Health (NIH)
 - Department of Defense (DOD)

Save the Date!
ANNUAL ADVOCACY CONFERENCE
MARCH 3-5, 2019
Washington, DC ~ Mayflower Hotel



CARE

- Advocating and participating in the development of a **Standard of Care (recently updated)**
- Established the Duchenne **Certified Care Center Program**
- Focusing on **gaps that exist** in care sub-specialties



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

Care Considerations *Pediatrics* Supplement – Published October 2018

[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](#)

PEDIATRICS
OFFICIAL JOURNAL OF THE AMERICAN ACADEMY OF PEDIATRICS

October 2018, VOLUME 142 / ISSUE Supplement 2

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Supplement Article

- Title Page
Pediatrics Oct 2018, 142 (Supplement 2) 142S2; DOI: 10.1542/peds.2018-142S2
 PDF
- An Introduction to the Duchenne Muscular Dystrophy Care Considerations
Leanne M. Ward, David J. Birnkrant
Pediatrics Oct 2018, 142 (Supplement 2) S1-S4; DOI: 10.1542/peds.2018-0333B
Building on the 2018 DMD Care Considerations, in this article, we introduce companion articles that provide detailed guidance on managing patients with DMD.
 PDF
- Neurology Care, Diagnostics, and Emerging Therapies of the Patient With Duchenne Muscular Dystrophy

A Transition Toolkit for Duchenne Muscular Dystrophy: Supplementary Materials

Supplementary Material 1: Transition Readiness Assessment for Young Adults with Duchenne Muscular Dystrophy

Please fill out this form to help us see what you already know about your health, how to use health care and the areas you need to learn more about. If you need help completing this form, please ask your parent/caregiver. Then we'll review the form together to develop a plan.

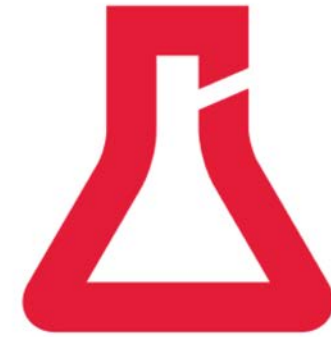
Date: _____	Participants in today's discussion:
Name: _____	_____
DOB: _____ Age: _____	_____

Transition Importance and Confidence										
On a scale of 0 to 10, circle the number that best describes how you feel right now										
How important is it to you to prepare for/change to an adult doctor before age 22?										
0 (not)	1	2	3	4	5	6	7	8	9	10 (very)
How confident do you feel about your ability to prepare for/change to an adult doctor?										
0 (not)	1	2	3	4	5	6	7	8	9	10 (very)

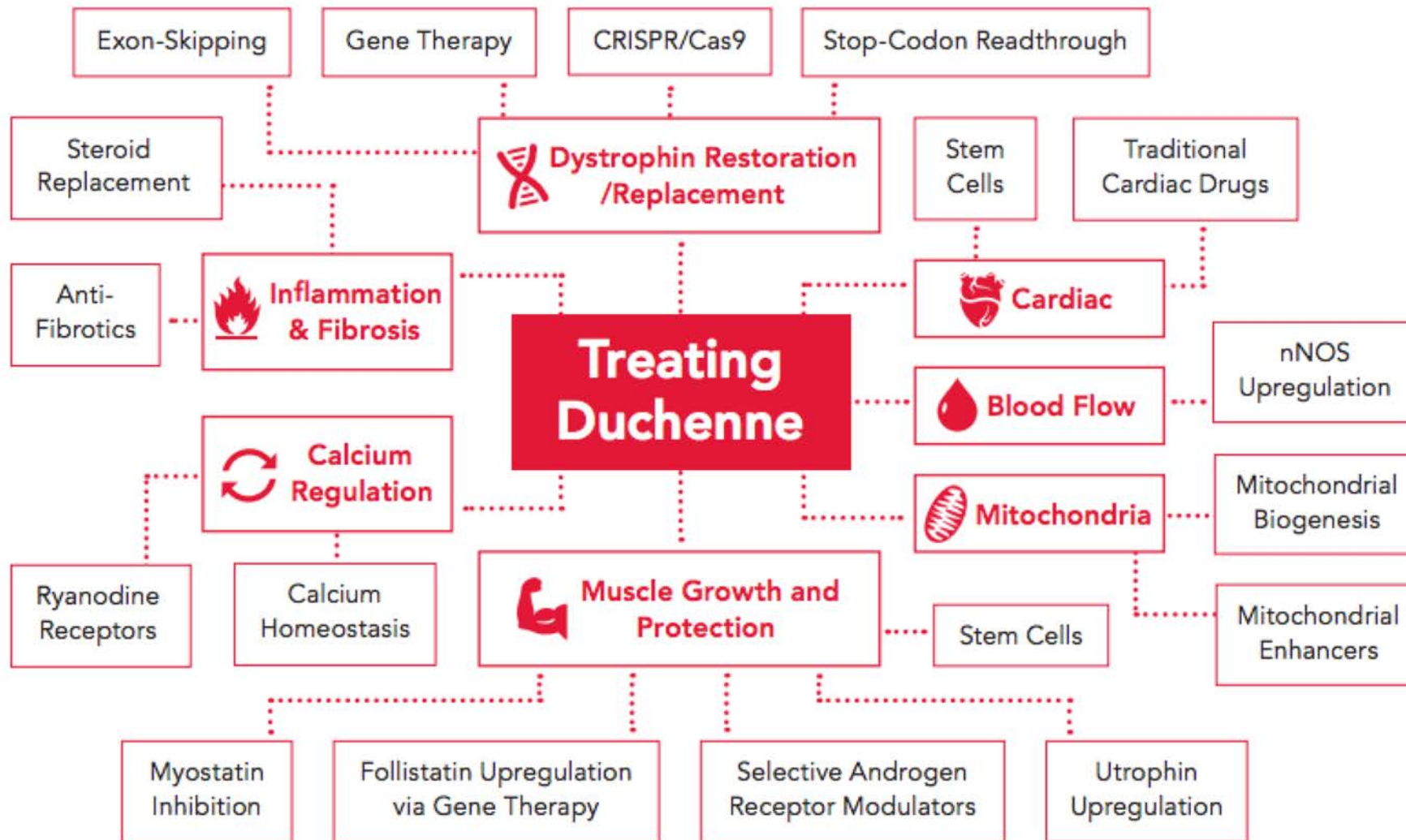
Publication pending; Shared with permission Pediatrics

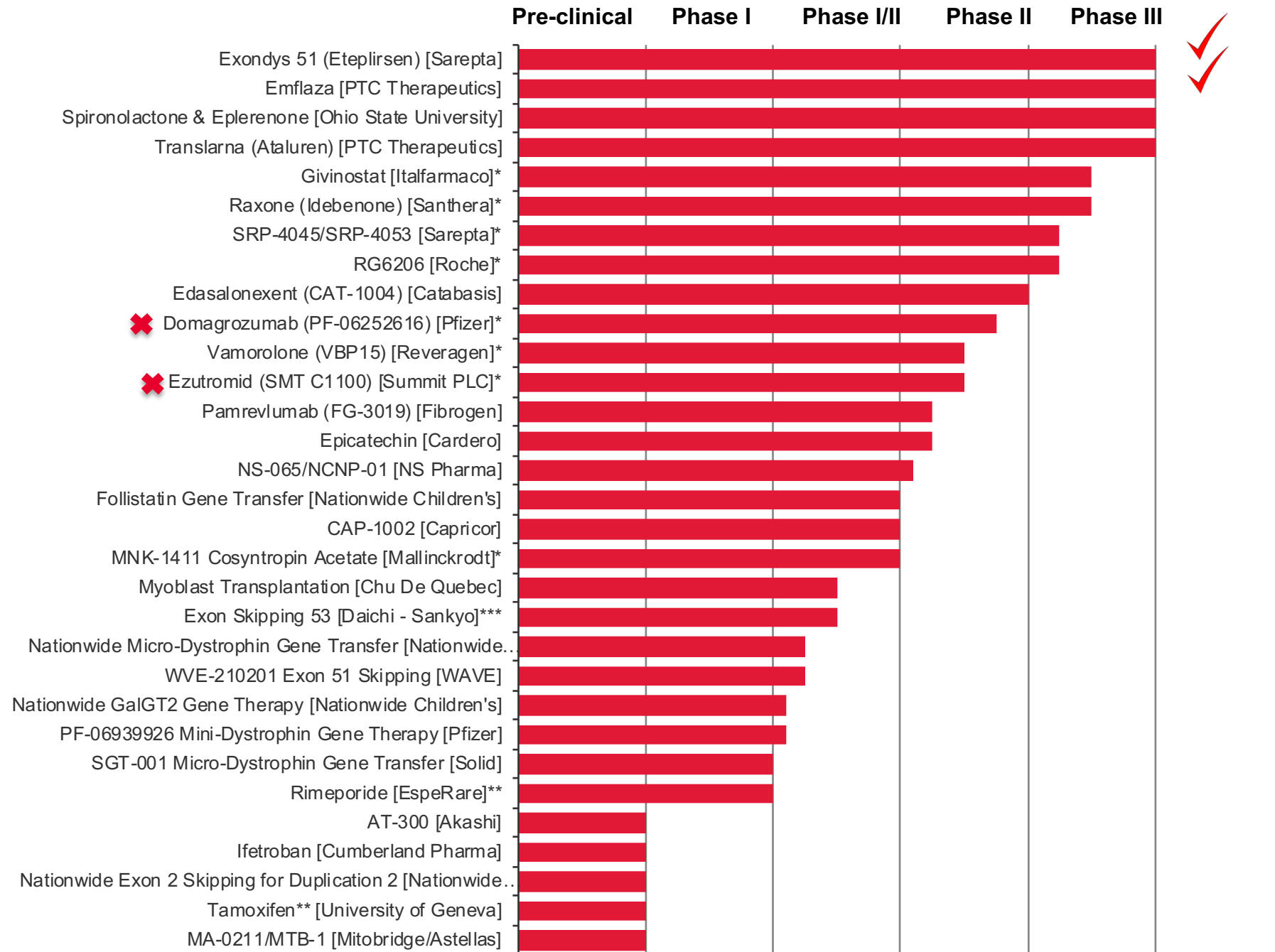
ADVANCES IN RESEARCH

- Invested **over 50 million** in research, leveraging **\$500 million** in federal dollars and billions in private dollars
- Have worked to **establish a research infrastructure** to enable drug development
- **2 approved therapies** with over **25 active treatment trials** and **over 40 companies** in the space



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.



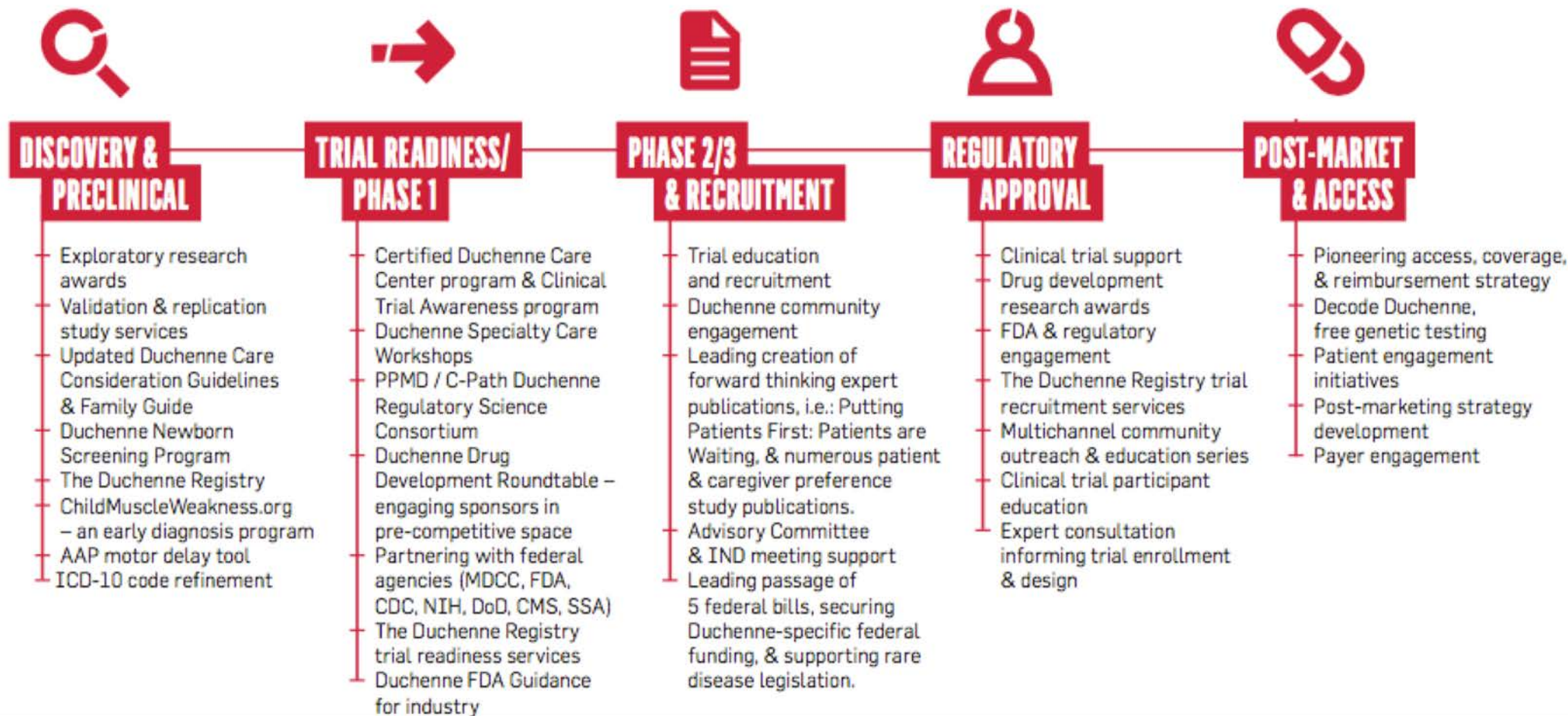




BROADLY ENGAGING THE DEVELOPMENT PIPELINE

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.

**Parent
Project
Muscular
Dystrophy**



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

Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment Guidance for Industry

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

February 2018
Clinical/Medical

**Parent
Project
Muscular
Dystrophy**

THE DUCHENNE PATIENT-FOCUSED COMPASS MEETING

WASHINGTON, DC - MARCH 5, 2018

- All federal partners invited
- White paper published prior to the meeting
- Webcast & live polling
- Compass Report published June 2018



National Institutes of Health



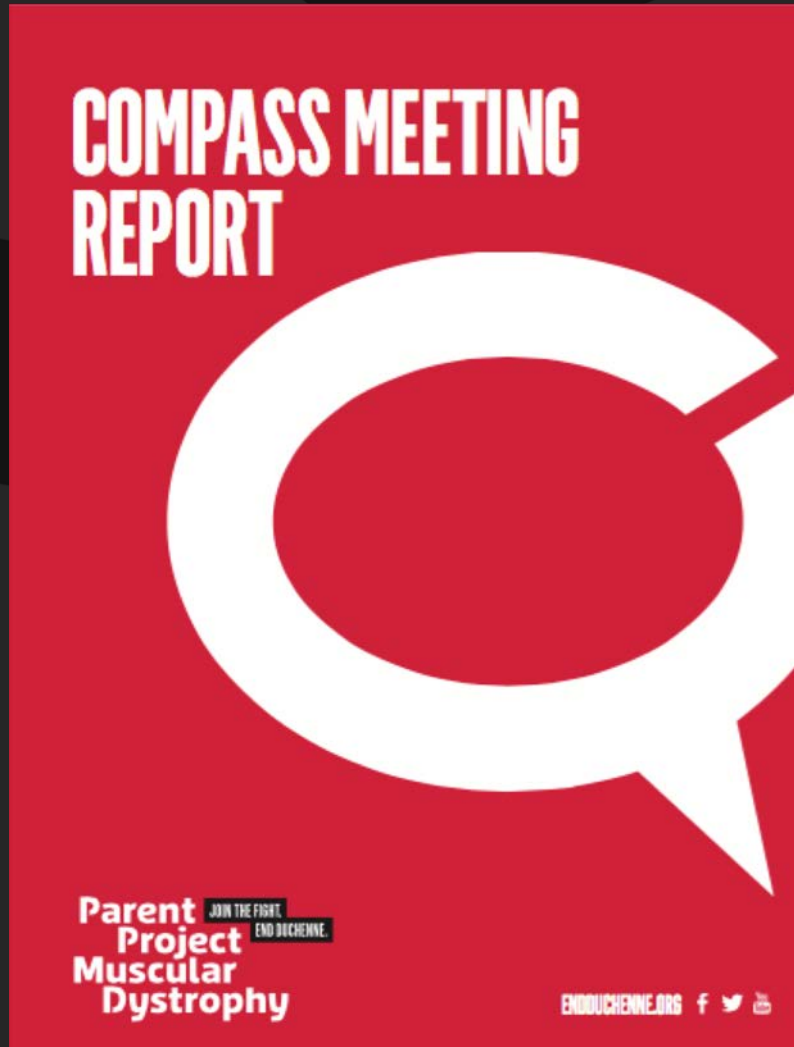
CENTERS FOR DISEASE CONTROL AND PREVENTION



CENTERS FOR MEDICARE & MEDICAID SERVICES



Duchenne Patient – Focused Compass Meeting (March 2018)



The Audience: Included federal agency & pharmaceutical industry partners

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.

More than 400 members of our Duchenne community participated

**Parent
Project
Muscular
Dystrophy**

It's About Access



Our Path to Access?



*Photo credit:
Conrad Reynoldson*

INSURANCE ACCESS & COVERAGE RESOURCES

[Home](#) > [Care](#) > [For Families](#) > [Insurance Access & Coverage Resources](#)

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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

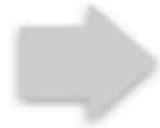
- What are the major differences with Medicaid vs private insurance?
- What is the process for appealing a denial, what are the layers of appeal?
- What are Medical vs. Drug Benefits?
- What is the role of a PBM's or Pharmacy Benefit Managers?
- Types of Insurance Coverage?
- What are formularies?

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

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

Stage 1 Resources:

Drug	Resources
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EMFLAZA	<ul style="list-style-type: none">• Emflaza is a corticosteroid that demonstrates anti-inflammatory and immunosuppressant effects. Emflaza is available in an immediate-release tablet formulation at multiple dosages (6 mg, 18 mg, 30 mg, 36 mg) as well as in an oral suspension formulation (22.75 mg/mL). Emflaza label and prescribing information• Please contact EmflazaCares to be connected with a case manager who can help you begin the process for access. EmflazaCares will contact your physician for the start form and the prescription.• Emflaza FAQs• Learn about the AssistanceFund
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EXONDYS 51	<ul style="list-style-type: none">• EXONDYS 51 is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (Duchenne) in patients who have a confirmed mutation of the Duchenne gene that is amenable to exon 51 skipping. FDA label document• Unsure if you are amenable to this therapy? Try our Deletion tool to perform a search on your mutation
------------	---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------

For U.S. Residents:

- For those amenable to exon 51 skipping please contact [SareptAssist](#) to be connected with a case manager who can help you begin the process for access. [SareptAssist](#) will contact your physician for the start form and the prescription.
- Learn about the [AssistanceFund](#)
- [Sample Letter of Medical Necessity \(for Clinicians/Doctors\)](#)
- [SareptAssist Patient Services Overview](#) [PPMD Webinar Recording]

If you live outside of the U.S.:

- Sarepta supports a Managed Access Program (Expanded Access Program) for those residing in numerous countries, including: Argentina, Brazil, Canada, Colombia, France, Germany, Greece, Iceland, Italy, Mexico, Spain, Turkey, and the United Kingdom.
- For more information about eligibility and access, visit [Sarepta's Managed Access Program](#).



STAGE 1

Prescription written by
Doctor and submitted to
Insurer

Includes both General & Product-Specific Resources and Innovative Access Efforts

STAGE 2

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

WHAT HAPPENS IF ACCESS IS DENIED BY MY INSURER?

It is at *this* stage a complication could occur.

If you receive a denial, there are steps you can take in order to **appeal** the decision. Your clinician will be a key advocate in this process. The denial letter should have specific information about the appeals process.

The clinician or doctor can request what is called a "**peer to peer**" where your clinician will interact directly with the insurer *one-on-one* to provide justification for access and coverage.

Things to Consider:

- Ask your clinician and the insurer about whether a "peer to peer" can occur and how to initiate.
- Write your own **Patient Appeal Letter** (sample below)
- Has your clinician submitted a **Letter of Medical Necessity**? (sample below)

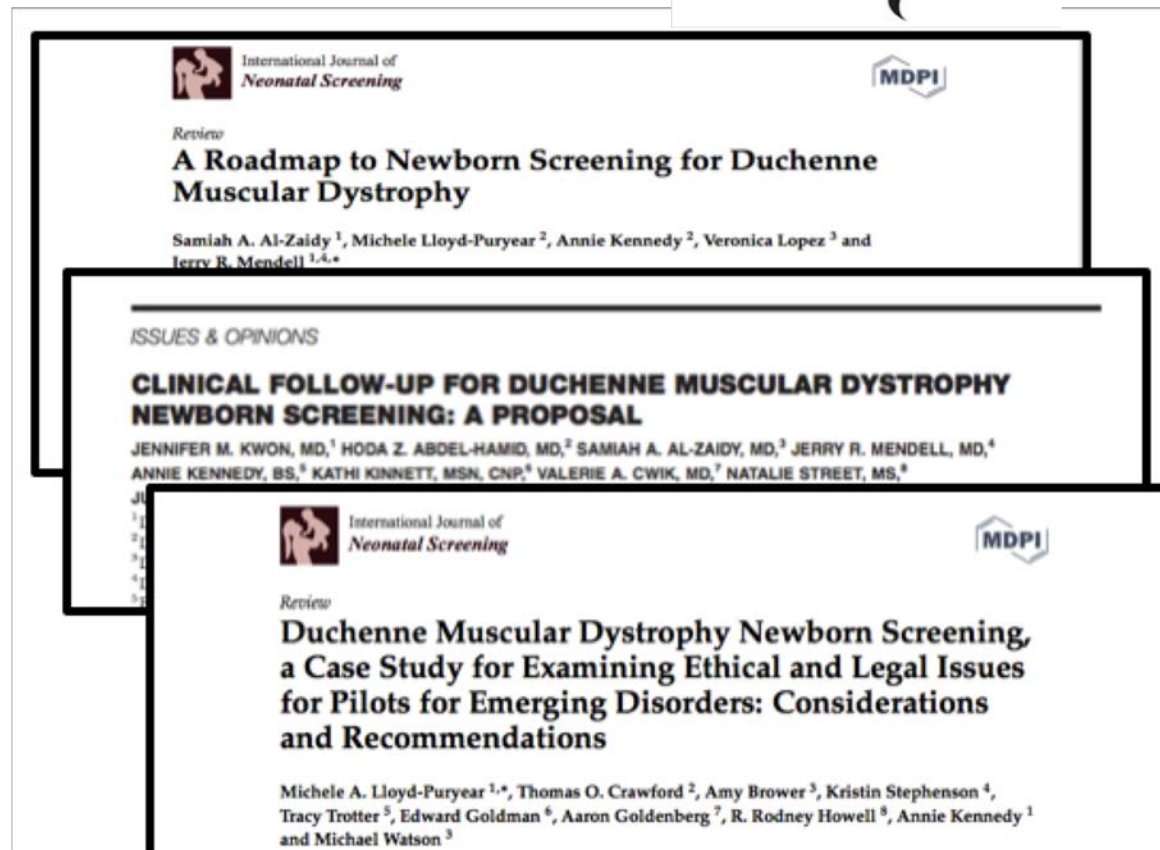
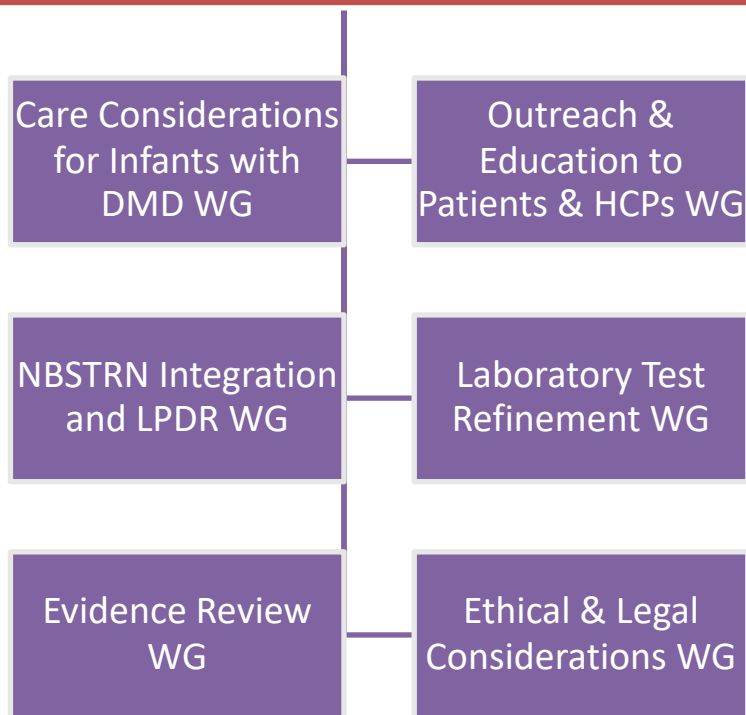
Tips for Advocating for Access:

- **Track all communications** with the drug company, health insurer, and clinician/doctor. Save all letters and emails and write down who you speak with and when. Request letters sent by your physician.
- **Consider contacting your state Consumer Assistance Program (CAP)** or Department of Insurance during an appeal process. Visit localhelp.healthcare.gov.

PPMD Duchenne Newborn Screening Program

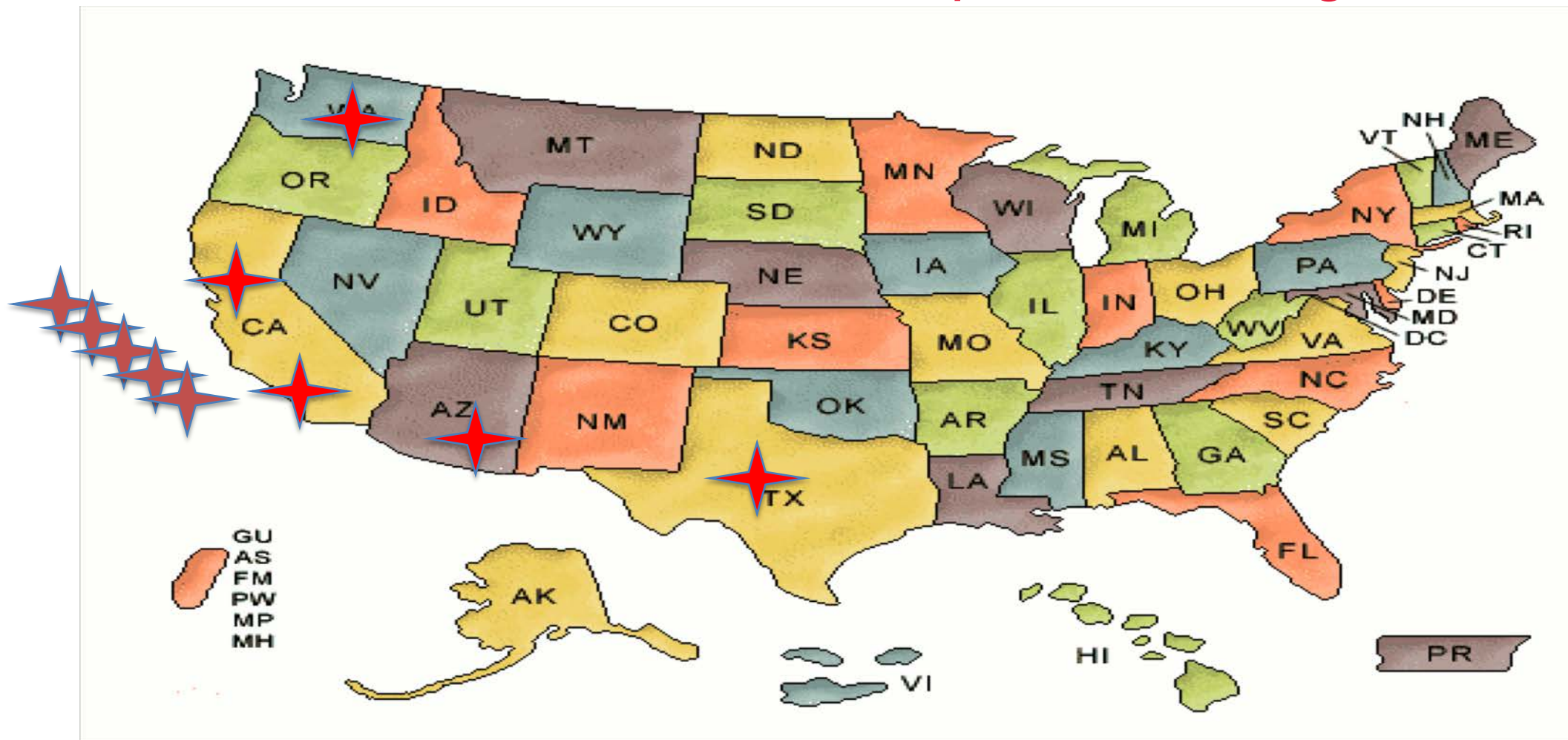


Duchenne NBS Steering Committee



- 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**

State Specific Resources – 22 PPMD Connect Groups & Growing



Resource Categories



Health & Wellness

- Bed & Accessories
- Home Care
- Orthotics
- Respiratory Equipment
- Toileting & Showering
- Wellness
- Emergency Preparedness Resources



Independent Living

- Employment
- Home Care



Insurance & Funding

- Funding Sources
- Insurance



PPMD Resource Fair Participants

- Connect Conference Resource Fair Participants



Accessibility

- Assistive Technology
- Automotive
- Clothing
- Housing and Home Modification
- Lifts & Standers
- Mobility
- Positioning & Transferring
- Service Animals



Education

- Primary and Secondary Education
- College
- Parent and Teacher Resources
- Assistive Technology
- Education Rights



Disability Rights

- Disability Rights
- Education Rights



Travel & Recreation

- Travel
- Recreation
- Video Gaming



State-Specific Resources



Advocacy

- Advocacy Groups
- Wish Foundations

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

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

A Parents Guide

A Teacher's Guide

Adaptive PE

Learning & Behavior in Duchenne

School Presentation Materials

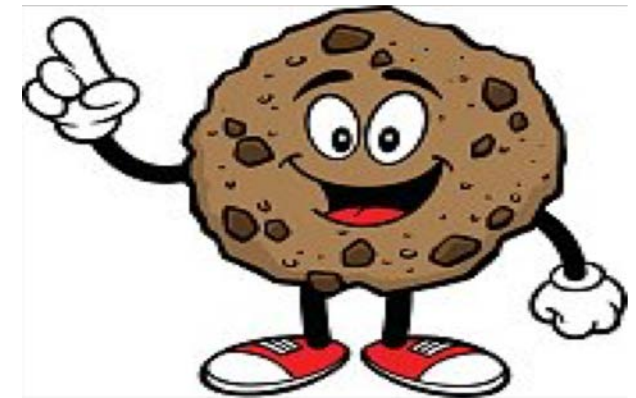
'Chocolate Chip Cookie' class presentation

IEP, 504 Plan, and School Accommodation Considerations

IEP vs 504 Plan side by side comparison

Tips & tools for creating IEPs and 504 for students with Duchenne & Becker

<https://www.parentprojectmd.org/care/for-families/classroom-resources-for-teaching-about-duchenne/>



PPMD School Webinars

Navigating School: An Ongoing Journey

<https://www.youtube.com/watch?v=M1py6M9ce0I>

From Where We Sit (Adults with Duchenne):

‘PCA’ – 3 Little Letters that Mean So Much

<https://www.youtube.com/watch?v=5IBEdb4IBIk>

Promoting Independence In Children with Duchenne:

Panel of Parents Whose Sons Live Away from Home Reflect on How they Navigated the Delicate Balance of Protecting Their Sons, While Fostering Independence

<https://www.youtube.com/watch?v=vtWbyt-j-IA>



PPMD Adult Advisory Committee

Nominations Being Accepted Through Early November

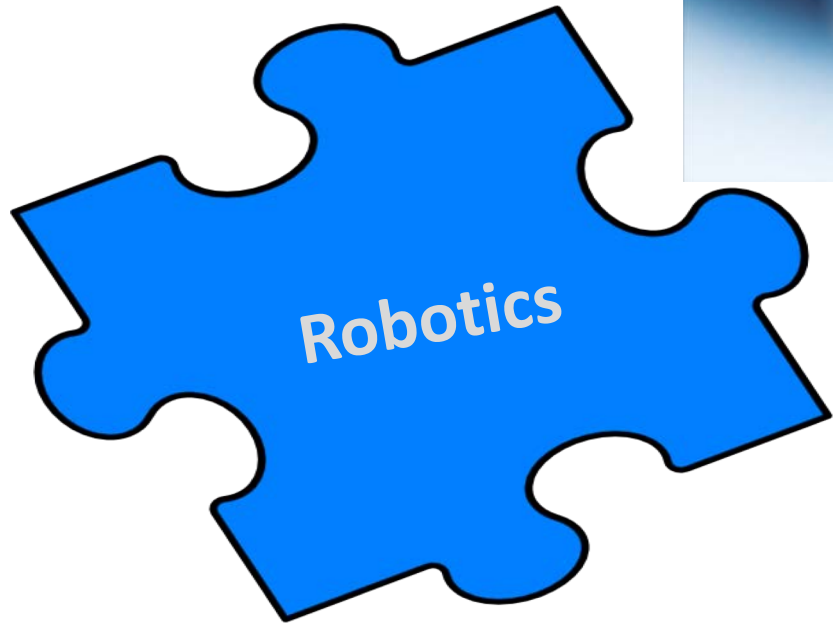
Transitions Initiative

PPMD's recent National leadership –

- MD CARE Act 2014
 - CDC MD STARnet – Transitions surveys & data elements
 - MDCC Action Plan 'Living With' section
 - Care Considerations, Lancet January 2018
 - Transitions Tool, *Pediatrics* supplement – published October 2018 Care Considerations Transitions section

PPMD Adult Advisory Committee (PAAC)

Transition to Care (T to C) federal coalition established –
PPMD among 4 advocacy partner leads, with CDC, NICHD
Coalition meeting convened, June 2018



Flextension

Innovator:
Arjen Bergsma & Micha Paalman

Community Member:
Justus Kuijer



X-Ar Arm

Innovators:
**Blake Mathie
& NJIT**

Community Member:
Zach Smith

Solid Suit

Innovators:
SRI, now Seismic

Community Partner:
Solid Biosciences & PPMD
community



PPMD conducted community survey of preferences related to robotics in 2015

Clear priorities among the highly valued activities include:

- standing from a seated position
- picking up an object from the floor
- repositioning oneself at night, and
- the ability to bring one's hands to mouth



Thank you!