

How to Engage with Federal Agencies

Centers for Disease Control and Prevention (CDC):



Annie Kennedy

Senior Vice President for Legislation and Public Policy
Parent Project Muscular Dystrophy

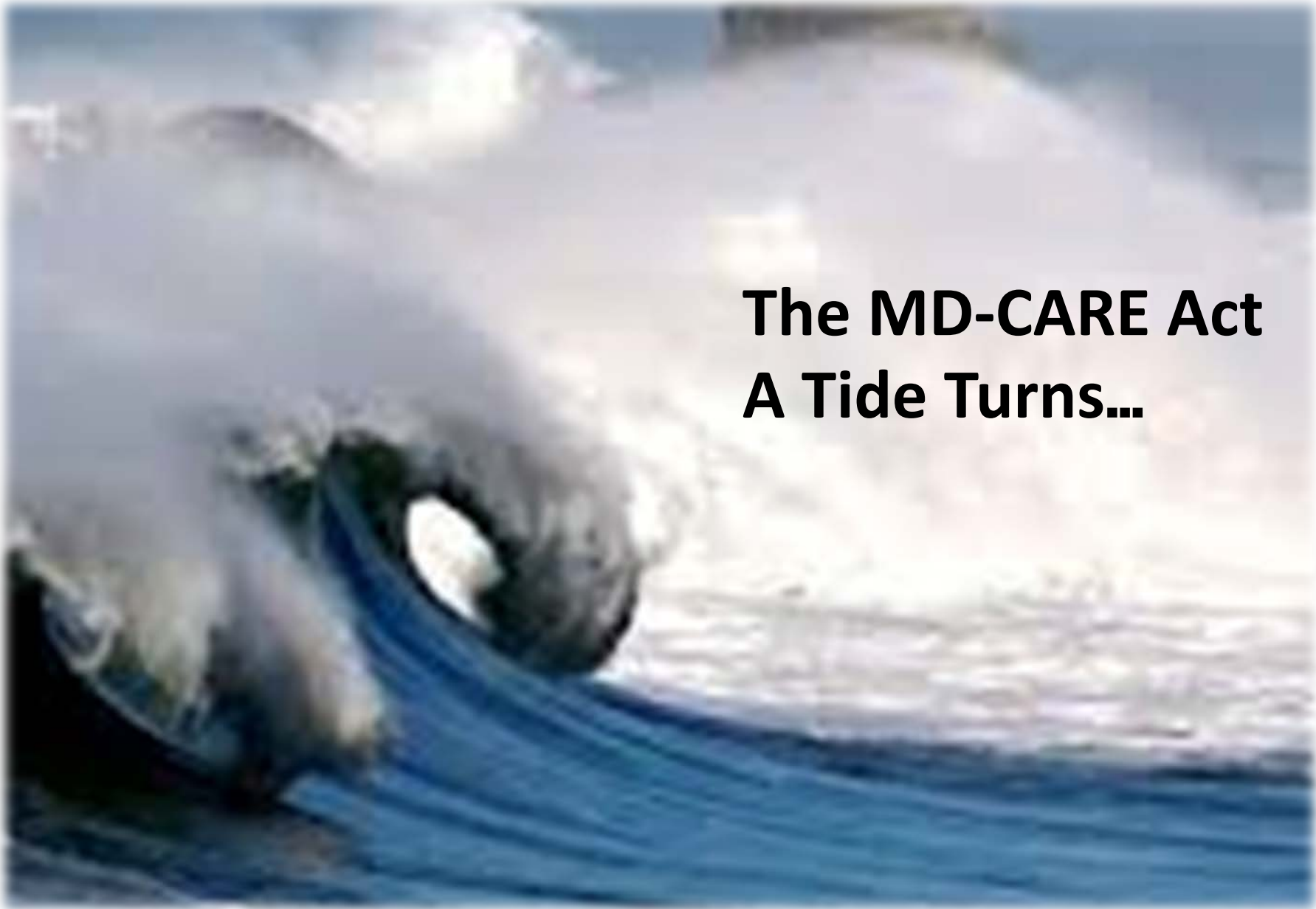
PPMD Working to *End Duchenne* Through Collaborations with Federal Partners



PPMD's Approaches

- **Building Relationships**
- **Facilitating Legislation**
- **Report Language/ Appropriations**
- **Utilizing Federal Agency Opportunities** (grants, meetings, Councils, etc)





**The MD-CARE Act
A Tide Turns...**

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

MD-CARE Act 2001

Centers of Excellence
MD STARnet tracking and surveillance
MD Coordinating Committee –
Action Plan for Muscular Dystrophies



MDCA Reauthorized 2008 – (MD-CARE Act 2)

Added the National Heart, Lung, and Blood Institute to MDCC
Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers
Enhancement of clinical research
Expansion of MD-STARnet
Duchenne Care Considerations – Develop and Disseminate

MDCA Amended in 2014 – (MD-CARE Act 3)

Expanded research to focus on Endocrine, Pulmonary and Cardiac, Transitions
Additional federal agencies added as members of Coordinating committee
Sharing of data from MD-STARnet
Expansion of care considerations to include Duchenne adult population & reflect updates in care
Update of MD Action Plan

Results of 3 iterations of MDCA

Care

Care considerations published
Care much more standardized

Research

Wellstone Centers of Excellence
Animal Studies
Basic and Translational Research grants

Federal Coordination and funding

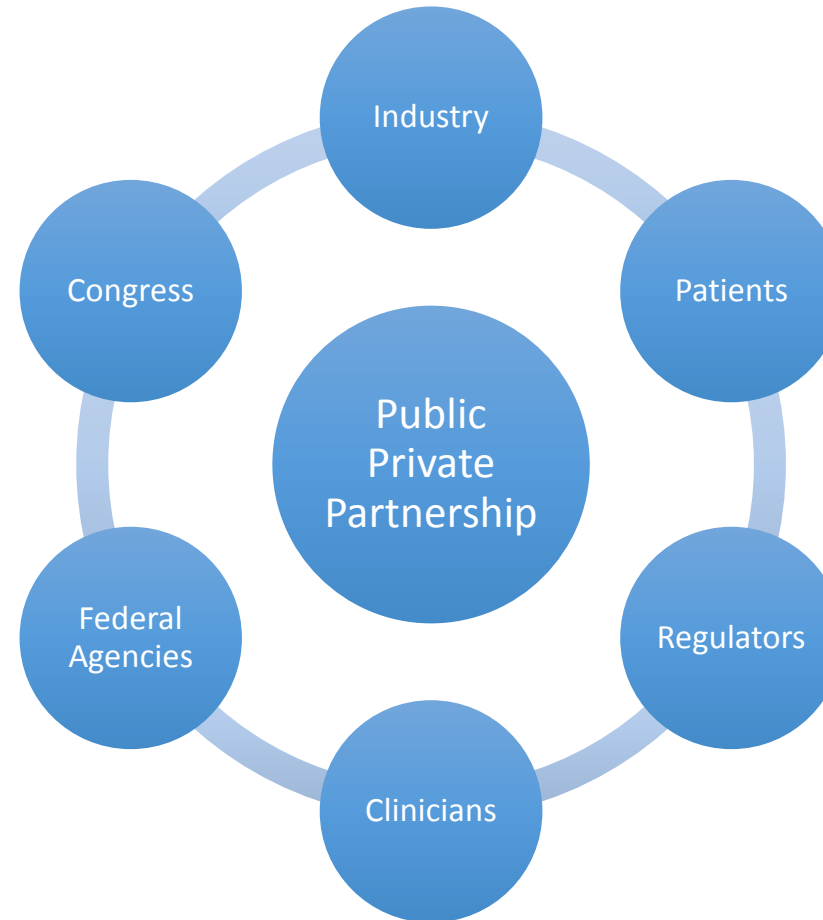
Coordinating Committee grows
Research Plan for MD's
500+million in Duchenne Funding

Data collection

Outcome measures
Natural History Studies
MD-STARnet Surveillance

Drug Development

45+ companies
Pipeline full of hope



Federal Agencies We Focus On



National Institutes
of Health



CENTERS FOR DISEASE
CONTROL AND PREVENTION



CDC as Key Collaborator

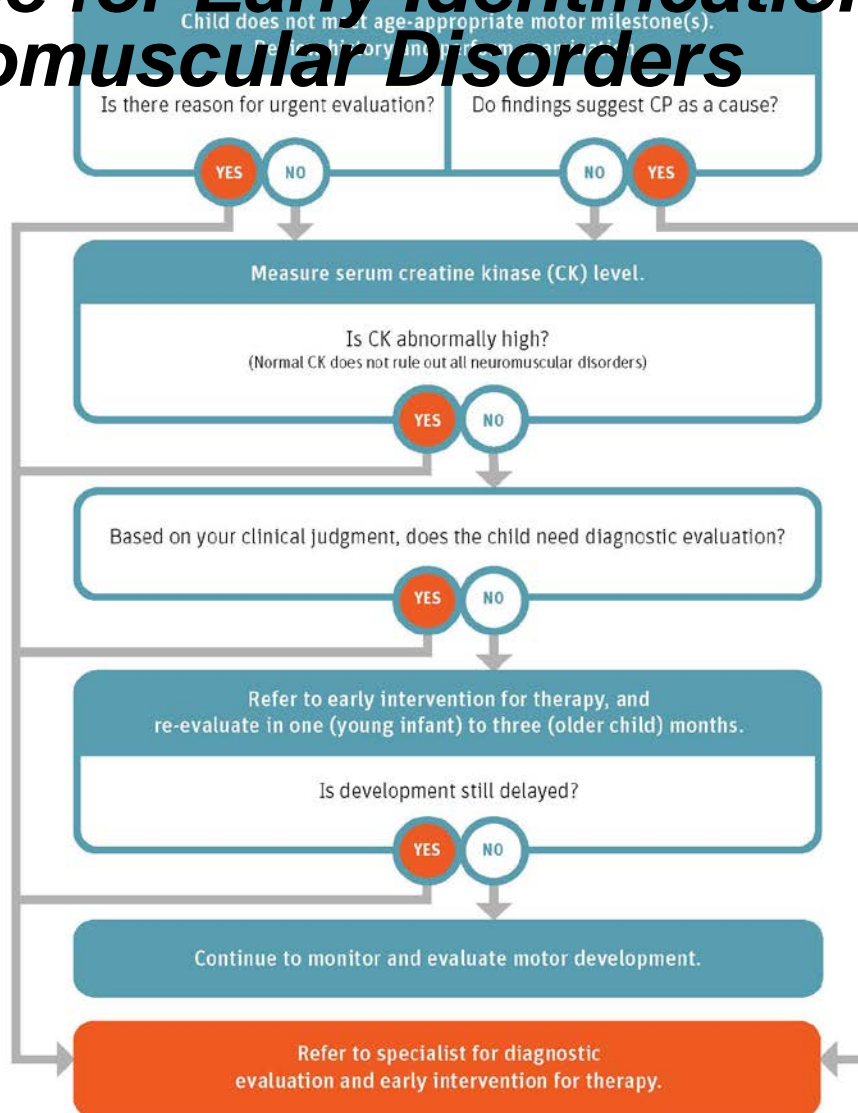
- **Member, Muscular Dystrophy Coordinating Committee**
- **MD STARnet surveillance**
- **DBMD Care Considerations (published in Lancet Neurology 2010, update under review)**
- **Early Identification of Childhood NMDs initiative**
- **Ohio Duchenne NBS pilot**
- **Member, National Duchenne NBS Steering Committee & relevant Workgroups (led by PPMD)**
- **Collaborator on national Duchenne Transitions initiatives**
- **Collaborator on data integration & health economics efforts**



The National Task Force for Early Identification of Childhood Neuromuscular Disorders

- Provider Tools
- Community Assessment of 'terms' used when expressing concerns to physicians
- Aligns with AAP Bright Futures
- Motor Delay Algorithm
- Clinical Pearls
- Videos
- Resources for Talking with Families
- Cooperative outreach campaign

www.ChildMuscleWeakness.org



**** Funded through a grant by CDC NCBDDD to PPMD,
led by Kathy Mathews, MD & Holly Peay, PhD**

The Muscular Dystrophy Surveillance Tracking and Research Network (MD STARnet)

- ❑ Population-based surveillance system
- ❑ Longitudinal surveillance for DBMD (2002-2011)
 - **Arizona, Colorado, Iowa, Western New York, Georgia, Hawaii**
- ❑ Cross-sectional pilot surveillance for all MD types (2011-2014)
 - Arizona, Colorado, Iowa, Western New York
- ❑ Longitudinal surveillance for all MDs (2014 - 2019)
 - Colorado, Iowa, Western New York, **South Carolina, North Carolina – Piedmont region, and Utah/Nevada**

**Average age of diagnosis was 5 years.
Unchanged in 20 years.**

Average delay of 2.5 years between detected onset of symptoms and definitive diagnosis.

Ciafaloni E, Fox DJ, Pandya S et al. Delayed diagnosis in Duchenne muscular dystrophy: data from the Muscular Dystrophy Surveillance, Tracking, and Research Network (MD STARnet). *J Pediatr.* 2009 Sep;155(3):380-5.

