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

Public Private Partnership

Congress  
Industry  
Patients  
Regulators  
Federal Agencies  
Clinicians
Federal Agencies We Focus On

- National Institutes of Health (NIH)
- Department of Defense (DoD)
- Centers for Disease Control and Prevention (CDC)
- Administration for Community Living (ACL)
- Food and Drug Administration (FDA)
- Social Security Administration (SSA)
- Health Resources and Services Administration (HRSA)
- Centers for Medicare & Medicaid Services (CMS)
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

** 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.

Thank You!