Creating a Pathway for Approvals & Access --

PPMD Advocacy

Annie Kennedy
Senior Vice President – Legislation & Policy
Creating infrastructure and context to inform decision-making
MD-CARE Act

Dramatically increased federal investment into MD research.

Established:
• national disease surveillance
• international care standards
• federal coordinating committee,
• collaborative research networks.
Building a Research & Regulatory Infrastructure for Duchenne Therapy Development

**Duchenne Drug Development Roundtable**

- Formal pre-competitive consortia of Duchenne industry partners
- Works to identify shared priorities, challenges, and opportunities for collaboration
Building a Research & Regulatory Infrastructure for Duchenne Therapy Development

PPMD led **Patient-Focused Drug Development** efforts

- *Putting Patients First* and *Patients Are Waiting* white papers
- **Patient preference** studies
- The **Duchenne Registry**
- PPMD led community effort to draft **Duchenne Guidance for Industry**; served as foundation for FDA’s Duchenne Guidance for Industry
Building a Research & Regulatory Infrastructure for Duchenne Therapy Development

PPMD led passage of **Patient-Focused Impact Assessment Act (PFIA)** in 2016

Ensured patient experience data is incorporated into regulatory review process
PPMD led passage of **BENEFIT Act** in U.S. Senate in 2017

Ensured patient experience data is incorporated into the FDA Benefit/Risk Framework
PPMD played critical role in key regulatory provisions included in 21st Century Cures Act and PDUFA VI, both signed into law in 2017.

Patient-Focused Impact Assessment Act (PFIA)

- Advancing Target Therapies provision
- PFDD provisions
- Expanded Access
- Data sharing
Building a Research & Regulatory Infrastructure for Duchenne Therapy Development

  - Surveillance, care standards, MDCC, Wellstones, research funding

- **Duchenne Drug Development Roundtable**
  - Patient-Focused Drug Development (PDUFA V)
  - Certified Duchenne Care Center Program
  - Duchenne Drug Development Roundtable

- Patient-Focused Impact Assessment Act (PFIA)
  - BENEFIT Act
  - FDA Guidances

- **PROs, Guidance, White papers, Patient Preference studies**

Key regulatory provisions in **21st Century Cures Act** and **PDUFA VI**
After Approval – There So Much More To This Pathway…
ICD-10 code

- Led nomination of specific DBMD ICD-10 code
- implemented in CMS addenda in October 2018
Building an Infrastructure to inform Decision-making within the Access Environment

Duchenne Patient-Focused Compass Meeting

- Externally-led Patient Focused Drug Development meeting, March 2018
- Included Patient Community, FDA (CDER & CBER), CMS, DOD, SSA, NIH, Dept of Ed, and Industry
• National Duchenne Newborn Screening program, initiated in 2015
• launched Duchenne Newborn Screening Pilot in October 2018
• Collaboration with AAP, CDC, ACMG, and New York State Department of Health
• NYS Pilot funded through pre-competitive consortia: Sarepta Therapeutics, PTC Therapeutics, Wave, Solid Biosciences, Perkin Elmer, Pfizer Inc, and PPMD
Building an Infrastructure to inform Decision-Making within the Access Environment

Payer Engagement & Access Navigation

- Engaging directly with Payers – Commercial & State Medicaid
- Facilitating engagement of payers & clinical/patient community
- CDCC Clinician Consensus Statement
- PPMD Access Resource Center
Building an Infrastructure to inform Decision-Making within the Access Environment

Establishing Value by Engaging with Valuators & Health Economists

- Working to build Valuation models that are more patient-centric
- ICER Engagement & framework for Duchenne product assessment
- HE data elements that truly reflect Duchenne community experience (caregiver/family spillover)
Convened meeting with payers, clinicians, methodologists, patient community representatives
May 2019
Report pending later this summer
Building a Research & Regulatory Infrastructure for Duchenne Therapy Development

- Surveillance, care standards, MDCC, Wellstones, research funding
- Certified Duchenne Care Center Program
- PROs, Guidance, white papers, Patient Preference studies
- ICD-10 code
- DDDR
- key regulatory provisions in 21st Century Cures Act and PDUFA VI
- Patient-Focused Impact Assessment Act (PFIA)
- BENEFIT Act
- National Duchenne Newborn Screening Program
- Duchenne Patient-Focused Compass Meeting
- Certified Duchenne Care Center Program
- Duchenne Registry
- Engagement with Valuators & Health Economists
- Payer Engagement & Access Navigation
- PROs, Guidance, white papers, Patient Preference studies
- Duchenne Outcomes Mtg
- Duchenne Patient-Focused Drug Development (PDUFA V)
- Approvals!

Parent Project Muscular Dystrophy | ENDDUCHENNE.ORG
**Discovery & Preclinical**
- Identify unmet medical needs
- Symptom priorities
- Understand burden of disease
- Identify target treat profile

**Development (Trials)**
- Inform endpoint development
- Inform which outcome measures to use in trial
- Inform development of PRO’s
- Ensure you understand preferences of target population of study (trial decision making)

**Regulatory**
- Understand: risk tolerance
- Tolerance for uncertainty
- Benefit preferences (trade offs)
- Understand meaningful benefit
- Understand preferences of sub-populations and subgroups

**Post-Market**
- Labeling considerations
- Value based resource allocation
- Payer determinations
- Disease burden

*Pathway image from Quintiles, May 2016*