Creating a Pathway for Approvals & Access --

PPMD Advocacy

Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.

Annie Kennedy

Senior Vice President – Legislation & Policy

Creating infrastructure and context to inform decision-making

Building a Research & Regulatory Infrastructure for Duchenne Therapy Development

MD-CARE Act
(2001, 2008, 2014)

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

Building a Research & Regulatory Infrastructure for Duchenne Therapy Development

PPMD led passage of **BENEFIT Act** in U.S. Senate in 2017

Ensured patient experience data is incorporated into the FDA Benefit/Risk Framework

Building a Research & Regulatory Infrastructure for Duchenne Therapy Development

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

MD-CARE Act
(2001, 2008, 2014)

Surveillance, care standards, MDCC, Wellstones, research funding

key regulatory provisions in
21st Century Cures Act and **PDUFA VI**

Patient-Focused Impact Assessment Act (PFIA)

BENEFIT Act

FDA Guidances

Duchenne Registry

Approvals!

Patient-Focused Drug Development (PDUFA V)

Certified Duchenne Care Center Program

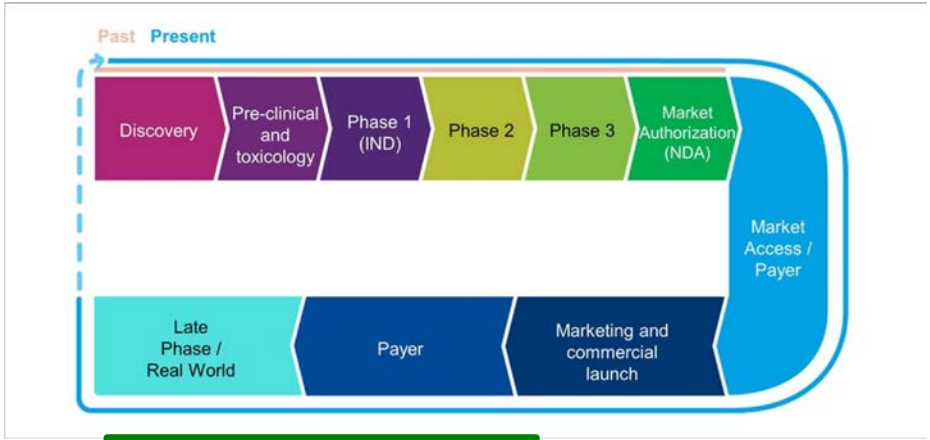
Duchenne Drug Development Roundtable

PROs, Guidance, White papers, Patient Preference studies

After Approval – There So Much More To This Pathway…

Quintiles, May 2016

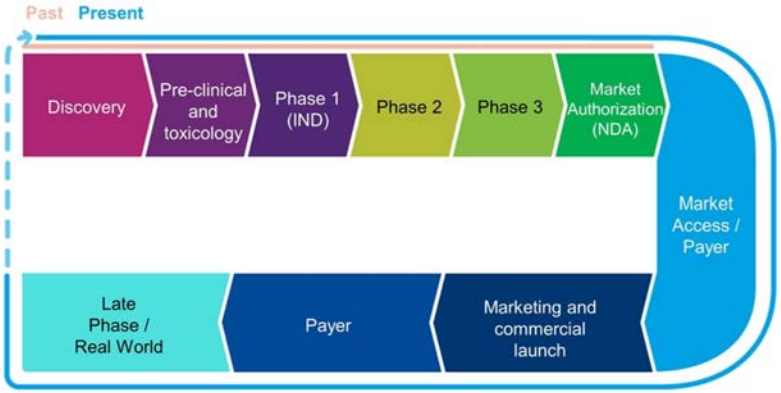
Building an Infrastructure to inform Decision- Making within the Access Environment



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

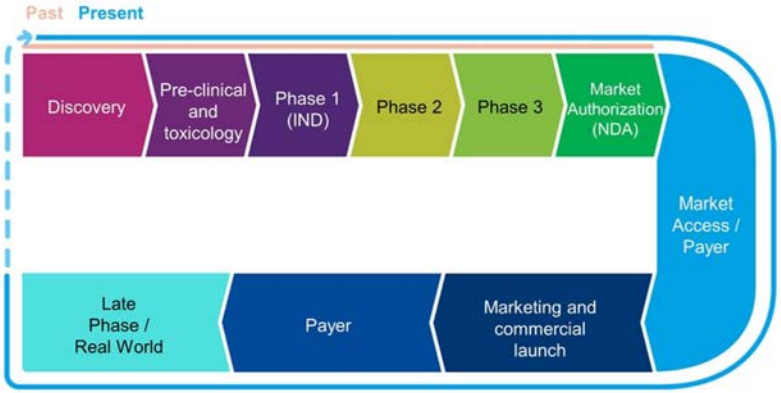
Building an Infrastructure to inform Decision- Making within the Access Environment

National Duchenne Newborn Screening Program

- 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

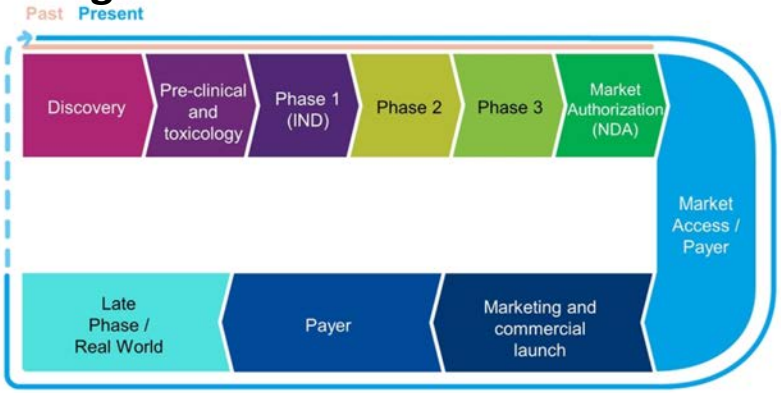
Building an Infrastructure to inform Decision- Making within the Access Environment

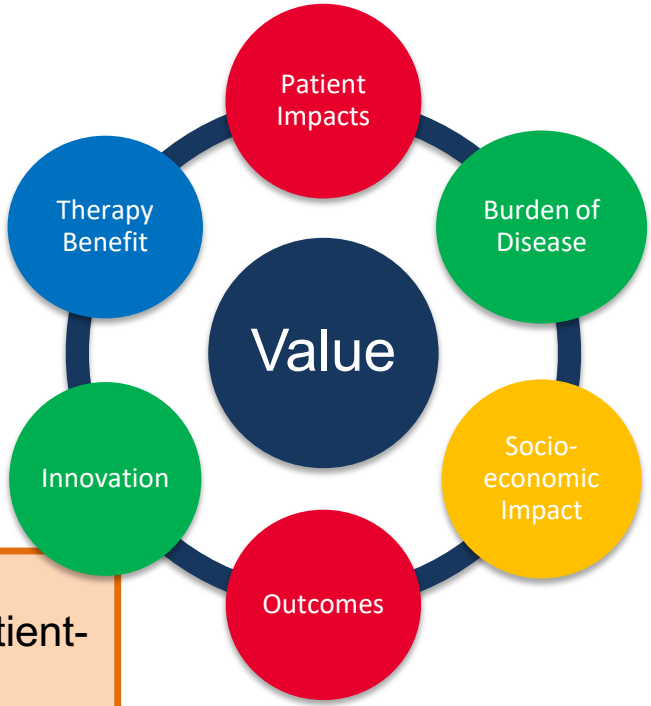


Payer Engagement & Access Navigation

- 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

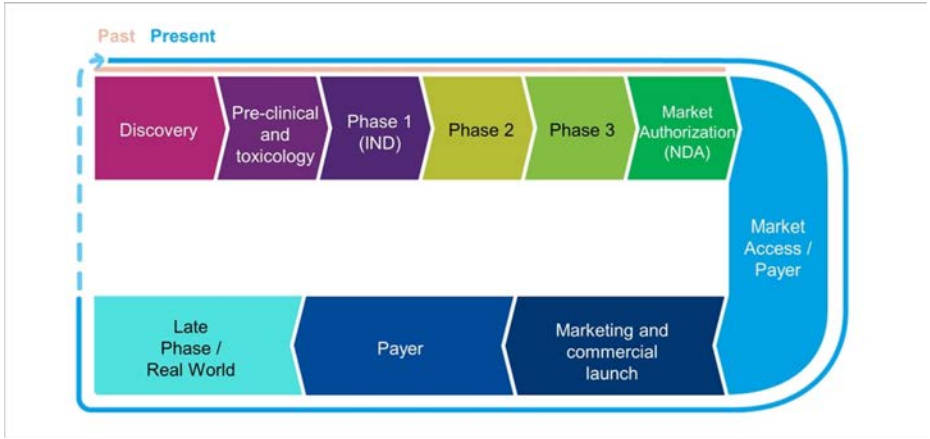




Establishing Value by Engaging with Valuators & Health Economists

- 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)

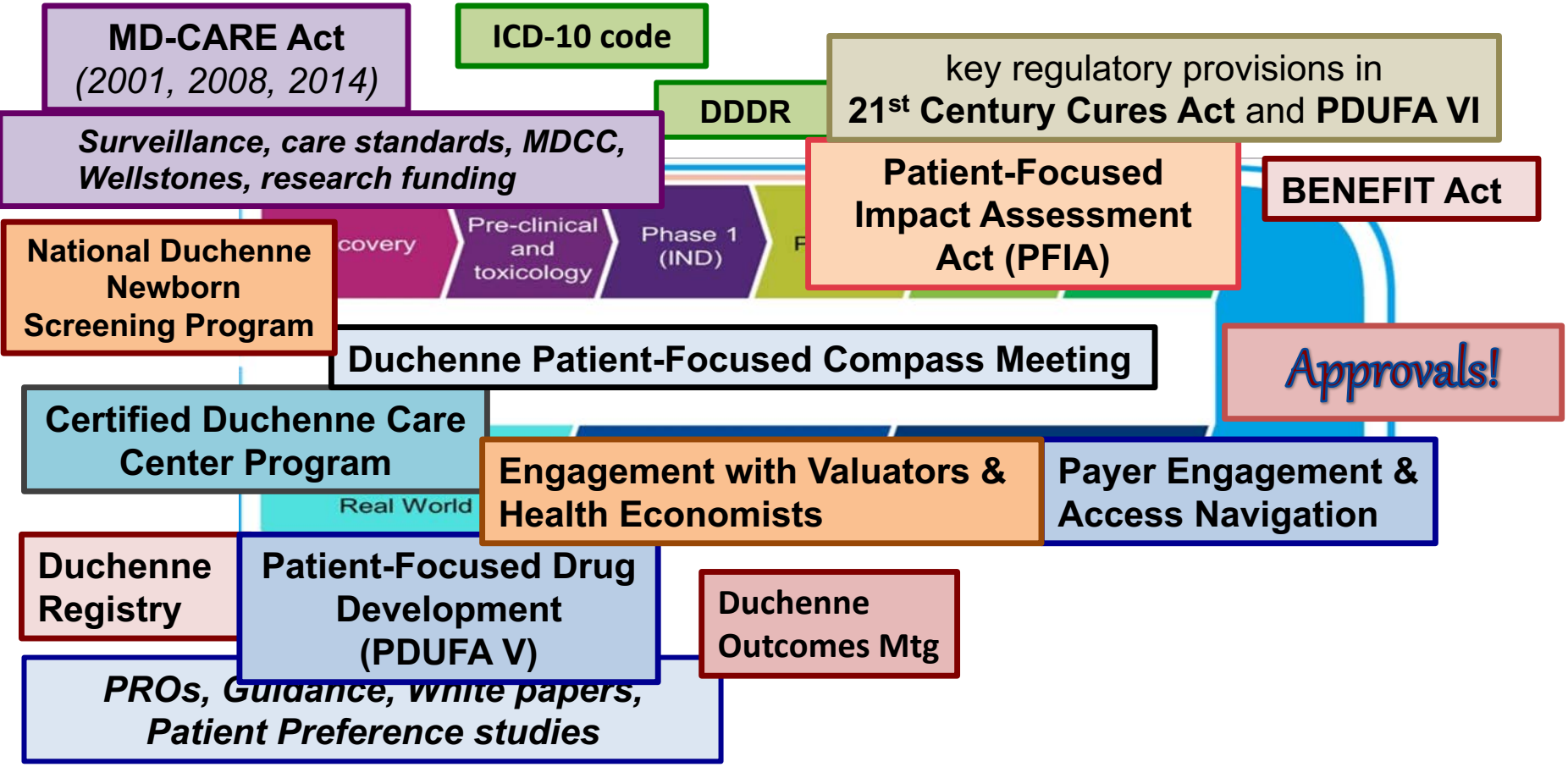
Building an Infrastructure to inform Decision- Making within the Access Environment



PPMD Duchenne Outcomes Meeting

- Convened meeting with payers, clinicians, methodologists, patient community representatives
- May 2019
- Report pending later this summer

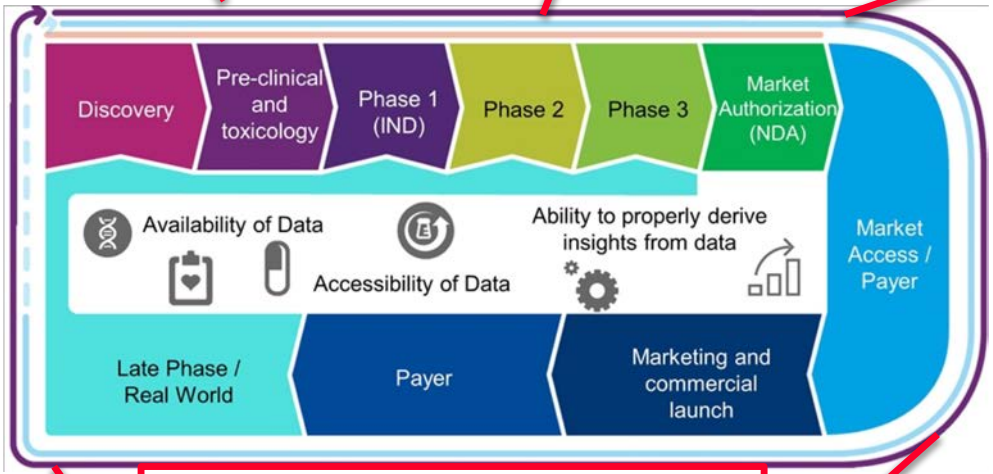
Building a Research & Regulatory Infrastructure for Duchenne Therapy Development



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


Researchers
Industry
Federal Agencies
Coalition Groups
Clinicians
Duchenne Patient community
Congress & Legislators
Health & Regulatory Policymakers
Payers, PBMs, Valuators, HEs
Adults with Duchenne/Becker
Siblings
Carriers & Manifesting Carriers