Progress & Opportunities towards in the Integration of Patient Experience Data within Regulatory Review

Parent Project Muscular Dystrophy

JOIN THE FIGHT.
END DUCHENNE.

Annie Kennedy
SVP – Legislation & Policy


SUPPORTING EVERY SINGLE diagnosis
AMPLIFYING EVERY SINGLE voice
CARING FOR EVERY SINGLE person
CONNECTING EVERY SINGLE family
INVESTING IN EVERY SINGLE therapy
Research
Advocacy
Parent Project Muscular Dystrophy
Care
Education
Grassroots Advocacy

PDUFA V –
A Game-Changer For Patient Communities

In the spirit of Patient Focused Drug Development (developing 'tools of engagement')….

- ✓*Putting Patients First* white paper
- ✓PPMD Patient-Preference studies (4;2)
- ✓*Patients are Waiting* white paper
- ✓Draft Guidance on Duchenne
- ✓*The DuchenneRegistry* data
- ✓Critical Path - DRSC
- ✓Testimony from patient community & clinical experts
- ✓Meaningful engagement with the FDA

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by a pioneering effort from Parent Project Muscular Dystrophy who, in 2014, submitted their own independent proposed draft guidance that provided important scientific and patient input from the DMD community.

It helped inform the FDA's development of both our own draft guidance and the final version issued today."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

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

DISCOVERY & PRECLINICAL

- Exploratory research awards
- Validation & replication study services
- Updated Duchenne Care Consideration Guidelines & Family Guide
- Duchenne Newborn Screening Program
- The Duchenne Registry
- ChildMuscleWeakness.org – an early diagnosis program
- AAP motor delay tool
- ICD-10 code refinement

TRIAL READINESS/ PHASE 1

- Certified Duchenne Care Center program & Clinical Trial Awareness program
- Duchenne Specialty Care Workshops
- PPMD / C-Path Duchenne Regulatory Science Consortium
- Duchenne Drug Development Roundtable – engaging sponsors in pre-competitive space
- Partnering with federal agencies (MDCC, FDA, CDC, NIH, DoD, CMS, SSA)
- The Duchenne Registry trial readiness services
- Duchenne FDA Guidance for industry

PHASE 2/3 & RECRUITMENT

- Trial education and recruitment
- Duchenne community engagement
- Leading creation of forward thinking expert publications, i.e.: Putting Patients First: Patients are Waiting, & numerous patient & caregiver preference study publications.
- Advisory Committee & IND meeting support
- Leading passage of 5 federal bills, securing Duchenne-specific federal funding, & supporting rare disease legislation.

REGULATORY APPROVAL

- Clinical trial support
- Drug development research awards
- FDA & regulatory engagement
- The Duchenne Registry trial recruitment services
- Multichannel community outreach & education series
- Clinical trial participant education
- Expert consultation informing trial enrollment & design

POST-MARKET & ACCESS

- Pioneering access, coverage, & reimbursement strategy
- Decode Duchenne, free genetic testing
- Patient engagement initiatives
- Post-marketing strategy development
- Payer engagement

Patient Focused Impact Assessment (PFIA) Act (2016)

Passed in the Senate -- and then later became a key provision within 21CC

Both bills focused on integrating patient experience data into regulatory review and creating formal processes for feedback.

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

PFIA's Path to Implementation

Hemophilia community

June 2015
PFIA S. 1597
Introduced in the Senate
End Duchenne Rally

Duchenne community

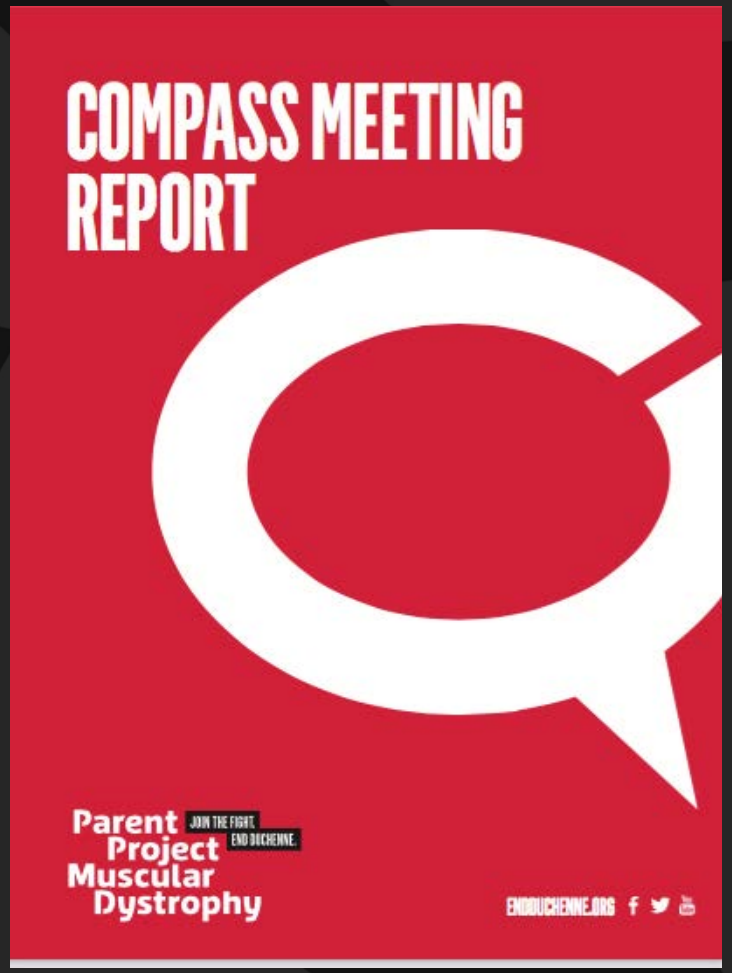
March 9, 2016
PFIA passes Senate!

December 2016
21st Century Cures becomes law – language from PFIA becomes Sec 3001

January 2018 –
'Patient Experience Data' section added to US FDA Drug Reviews!
FDA's review and approval of Genetech's hemophilia A treatment, Hemlibra

PFIA

- **Patient-experience data checklist within product review**
- **Guidance for engagement between industry & advocacy partners**



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

FDA Advisory Committee Meetings

Opportunities to Expand Upon the Integration of Patient Experience Data within Regulatory Review

- **The inclusion of additional guidance on the processes for iterative engagement with the FDA as patient experience data is developed and then integrated within a product's development.**

- **The need for formalized processes for ensuring that - when appropriate – relevant patient experience data is made available to reviewers and Advisory Committee members.**

- **An opportunity for dedicated time during the Advisory Committee meeting for the introduction & review of PED relevant to a specific product.**

Recent FDA Advisory Committee Agenda

9:00 AM	**Call to Order & Introduction of the Committee**
9:05 AM	Conflict of Interest Statements
9:10 AM	**FDA Introductory Statements**
9:30 AM	**Applicant Presentation**
10:45 AM	**Clarifying Questions**
11:00 AM	**Break**
11:15 AM	**FDA Presentations**
	FDA Efficacy Review
12:30 PM	**Clarifying Questions**
12:45 PM	**Lunch**
1:45 PM	**OPEN PUBLIC HEARING (90 minutes)**
3:15 PM	**Questions to the Committee/ Committee Discussion**
4:30 PM	**Adjournment**




Thank you!