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

JOIN THE FIGHT. END DUCHENNE.

Annie Kennedy
SVP – Legislation & Policy
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✓ Draft Guidance on Duchenne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 Duchenne Registry 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by a pioneering effort from Parent Project Muscular Dystrophy who, in 2014, submitted their own independent proposed draft guidance that provided important scientific and patient input from the DMD community.

*It helped inform the FDA’s development of both our own draft guidance and the final version issued today.*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

**BROADLY ENGAGING THE DEVELOPMENT PIPELINE**

**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
- 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/JC-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 for 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 and Duchenne, & numerous patient & caregiver preference study publications.
- Advisory Committee & INO 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
- Patient engagement initiatives
- Post-marketing strategy development
- Payer engagement

**POST-MARKET & ACCESS**
- Pioneering access, coverage, & reimbursement strategy
- Decode Duchenne, free genetic testing
- Duchenne Registry outreach & education series
- Clinical trial participant education
- Expert consultation
- Informing trial enrollment & design

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

Patient Focused Impact Assessment (PFIA) Act (2016)

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

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

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

March 9, 2016
PFIA passes Senate!

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

PFIA’s Path to Implementation

Hemophilia community
Duchenne community
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

<table>
<thead>
<tr>
<th>Time</th>
<th>Activity</th>
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<tbody>
<tr>
<td>9:00 AM</td>
<td>Call to Order &amp; Introduction of the Committee</td>
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<tr>
<td>9:05 AM</td>
<td>Conflict of Interest Statements</td>
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<tr>
<td>9:10 AM</td>
<td>FDA Introductory Statements</td>
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<tr>
<td>9:30 AM</td>
<td>Applicant Presentation</td>
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<tr>
<td>10:45 AM</td>
<td>Clarifying Questions</td>
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<tr>
<td>11:00 AM</td>
<td>Break</td>
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<tr>
<td>11:15 AM</td>
<td>FDA Presentations</td>
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<tr>
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<td>FDA Efficacy Review</td>
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<tr>
<td>12:30 PM</td>
<td>Clarifying Questions</td>
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<tr>
<td>12:45 PM</td>
<td>Lunch</td>
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<tr>
<td>1:45 PM</td>
<td>OPEN PUBLIC HEARING (90 minutes)</td>
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<tr>
<td>3:15 PM</td>
<td>Questions to the Committee/ Committee Discussion</td>
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<tr>
<td>4:30 PM</td>
<td>Adjournment</td>
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Thank you!