Patient Input in Medical Product Development

Andrea Furia-Helms, Director of Patient Affairs Staff, FDA
Annie Kennedy, Senior Vice President, Legislation & Public Policy, Parent Project Muscular Dystrophy
David R. Zook, Partner, Faegre Baker Daniels LLP
Moderated by **Eleanor Perfetto**, Executive Vice President, Strategic Initiatives, National Health Council

Patient Input in Medical Product Development

Eleanor Perfetto, PhD, MS

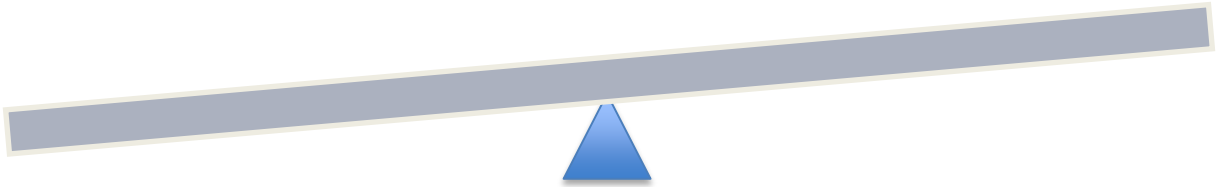
Executive Vice President, Strategic Initiatives,
National Health Council


American Heart Association
Learn and Live
American Diabetes Association
Cure • Care • Commitment
National Alopecia Areata Foundation
American Kidney Fund
FIRST
Foundation for Ichthyosis & Related Skin Types
Educate • Inspire • Connect
COPD FOUNDATION
ALPHA-1 FOUNDATION
NATIONAL PSORIASIS FOUNDATION
American Autoimmune Related Diseases Association, Inc.
Huntington's Disease Society of America
PKD FOUNDATION
Polycystic Kidney Disease
Tourette Association of America
Awareness. Research. Support.
NATIONAL Eczema ASSOCIATION
alzheimer's association
AMERICAN LIVER FOUNDATION
Your Liver. Your Life.
amputee coalition
THE MARFAN FOUNDATION
Know the signs. Fight for victory.
resolve
THE NATIONAL INFERTILITY ASSOCIATION
colorectal cancer alliance
aafa Asthma and Allergy Foundation of America
MGFA Myasthenia Gravis Foundation of America, Inc.
PUTTING PATIENTS FIRST
NATIONAL HEALTH COUNCIL
IDF Immune Deficiency Foundation
Arthritis Foundation
Champion of Yes
National Foundation for Ectodermal Dysplasias
American Cancer Society
Celiac Disease FOUNDATION
Sjögren's Syndrome Foundation
WomenHeart
The National Coalition for Women with Heart Disease
BEYOND CELIAC
awareness • advocacy • action
NATIONAL OSTEOPOROSIS FOUNDATION
ALS ASSOCIATION
nfed
Supporting you. Supporting each other.
NATIONAL FOUNDATION FOR ECTODERMAL DYSPLASIAS
Barth Syndrome Foundation
MS National Multiple Sclerosis Society
Prevent Blindness
Our Vision Is Visio
Spina Bifida Association of America
OSTEOGENESIS IMPERFECTA FOUNDATION
Unbreakable Spirit
American Foundation for Suicide Prevention
The LAM Foundation
A BREATH OF HOPE
NATIONAL HEMOPHILIA FOUNDATION
for all bleeding and clotting disorders
prevent cancer FOUNDATION
Hydrocephalus Association
Mended Hearts
THE MYOSITIS ASSOCIATION
National Blood Clot Alliance
Stop The Clot®
LUPUS FOUNDATION OF AMERICA
LUNG CANCER ALLIANCE
Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE
NORD
National Organization for Rare Disorders
CMTA
Charcot-Marie-Tooth Association

PFDD: Advantages and Challenges

Advantages

- Patient voice being heard
- Focus on patient-centered endpoints
- Better, patient-friendly trials
- Information patients can use



Challenges

- Companies haven't done business this way before
- New/revamped policies and procedure needed (e.g., contracting)
- Internal, cross-functional education needed (e.g., clinical, compliance, procurement)

U.S. FOOD & DRUG ADMINISTRATION

FDA Patient Affairs Staff

Enhancing Patient Engagement

Andrea Furia-Helms, MPH
Director, Patient Affairs Staff
Office of Clinical Policy and Programs
Office of the Commissioner

FDLI
Food and Drug Law Institute

May 3, 2019

Overview

II

Overview

III

Objectives

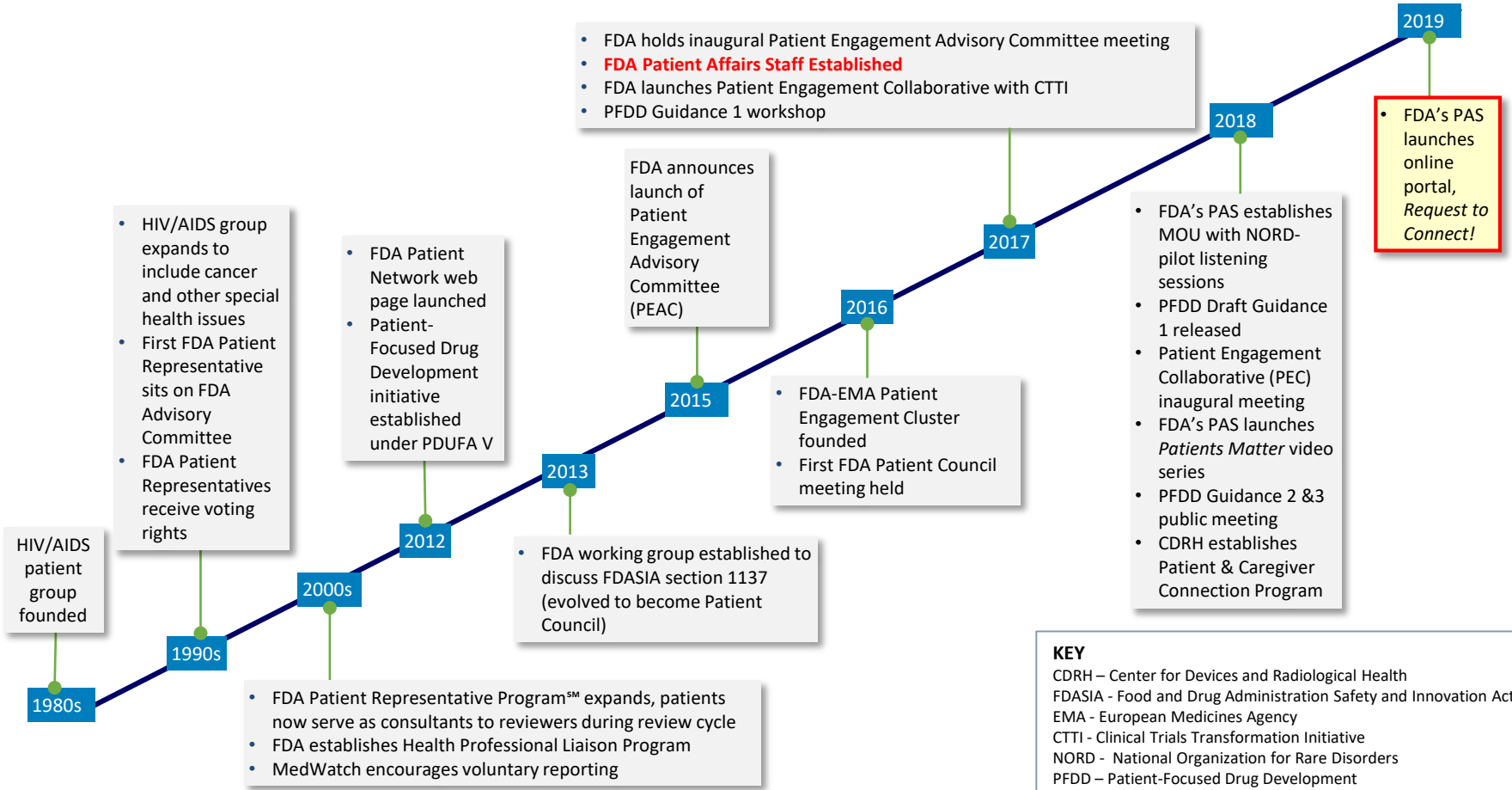
III

Programs

IV

Questions

Evolution of Patient Engagement at FDA



KEY

CDRH – Center for Devices and Radiological Health
FDASIA - Food and Drug Administration Safety and Innovation Act
EMA - European Medicines Agency
CTTI - Clinical Trials Transformation Initiative
NORD - National Organization for Rare Disorders
PFDD – Patient-Focused Drug Development
PAS – Patient Affairs Staff

Patient Affairs Staff

- Established late 2017 in FDA's Office of the Commissioner
- Reports into the Principal Deputy Commissioner
- Collaborates closely with the FDA medical product centers, other FDA offices and the patient community to enhance ongoing patient engagement efforts

"Early and iterative engagement can improve clinical and regulatory understanding of diseases and conditions, provide a common understanding of the most urgent patient needs, and inform drug development programs."

Scott Gottlieb, M.D.

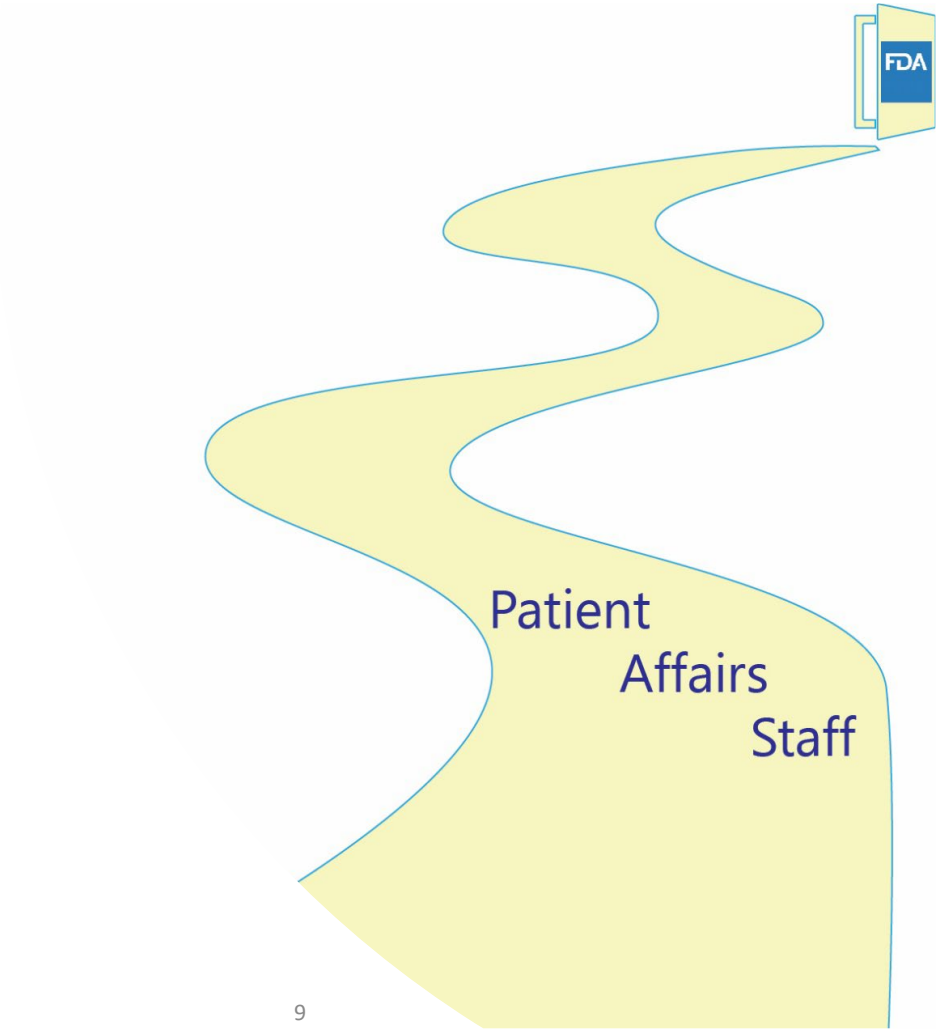
Patient Affairs Staff Purpose

Mission

Work with patients and their advocates to incorporate their perspectives into FDA's regulatory activities by facilitating dialogue and collaboration under FDASIA section 1137, FDARA and 21st Century Cures

Vision

An inviting, welcoming and meaningful experience for patient communities to engage with the FDA

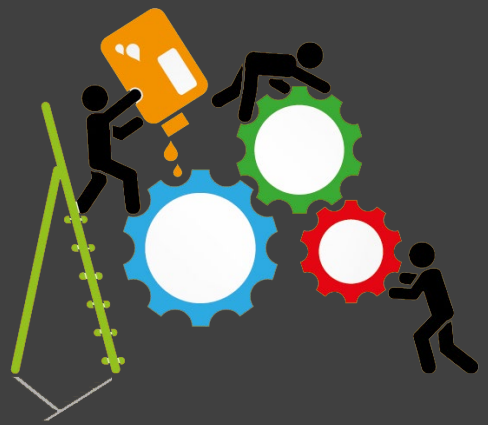


Patient Affairs Staff

- Engage with patients and advocates to hear and incorporate their perspectives in **cross-cutting programs and activities**
- Create and assist with **public-private collaborations and partnerships**
- Enhance FDA's **external communication platforms** with patients

Patient Affairs Staff Programs

FDA and EMA Patient Engagement Cluster



U.S. FOOD & DRUG ADMINISTRATION

EUROPEAN MEDICINES AGENCY

Mutual exchange on:

- Approaches for engaging and involving patient stakeholders
- High profile topics of mutual interest, especially those with potential high public interest
- Priorities and goals regarding future collaborations to enhance engagement

Patient Engagement Collaborative

I. Background and Purpose

The PEC will be an ongoing, collaborative forum in which the patient community and regulators will discuss an array of topics regarding increasing patient engagement in medical product development and regulatory discussions at FDA. The PEC will be a joint endeavor between the CTTI and FDA. The activities of the PEC may inform relevant FDA and CTTI activities. The PEC is not intended to advise or otherwise direct the activities of either organization, and membership will not constitute employment by either organization.

Federal Register Notices, Docket No. FDA-2017-N-6395

FDA Patient Listening Sessions

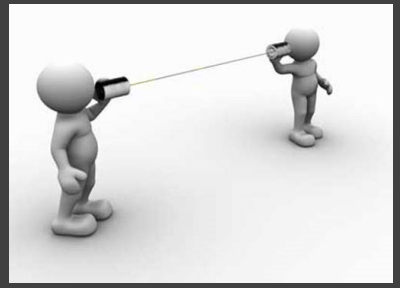
Rare Diseases Pilot

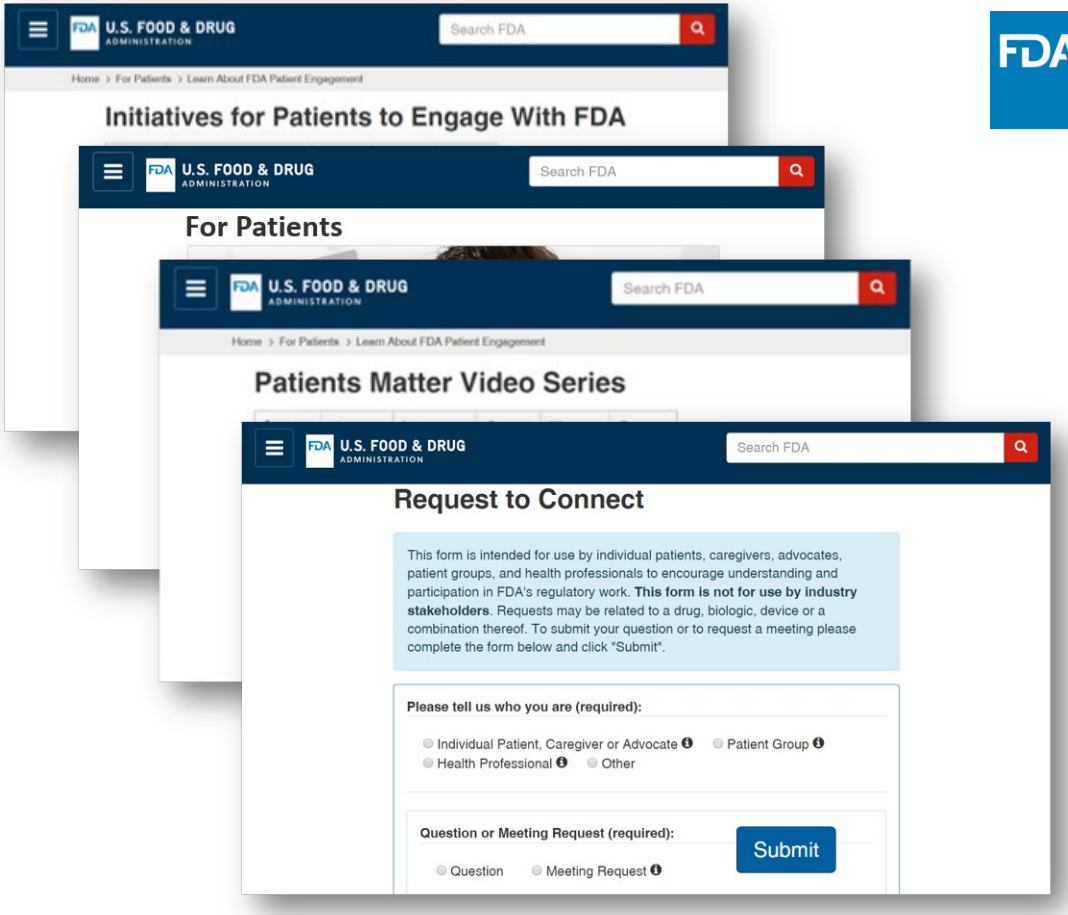
- Memorandum of Understanding with the National Organization for Rare Disorders (NORD)
- Inform regulatory decision-making
- Educate review staff about rare diseases
- Help patients and their advocates understand the FDA's mission and work
- Provide a starting point to inform early stage research & development
- Assess the value to expand to other therapeutic areas

Types:

- FDA-requested (specific set of questions to ask of a particular patient sub-population)
- Patient-requested (patient community wants to share their experiences and perspectives with the FDA)

Enhanced External Communication Tools





www.fda.gov/**RequestToConnect**

FDA Patient Representative Program

FDA Patient Representative ℠ consultants provide direct input to the Agency's decision-making process in over 300 diseases and conditions and participate on FDA Advisory Committees and in review division assignments.

Criteria for becoming an FDA Patient Representative:

Medical Product Center Patient Initiatives

Center for Drugs

- Professional Affairs and Stakeholder Engagement (PASE)
- FDA-led Patient-Focused Drug Development Meetings (PFDD)
- Externally-led Patient-Focused Drug Development Meetings
- PFDD Methodological Guidance Series

Center for Medical Devices

- Partner with Patients
- Patient Engagement Advisory Committee
- Patient and Care-Partner Connection Program

Center for Biologics

- Interactive Meetings with Patients
- CBER Workgroups:
- CBER Patient Engagement Workgroup
- CBER Rare Disease Coordinating Committee
- CBER Science of Patient Input (SPI) Team

FDA Patient Engagement Contacts

Office of the Commissioner

- FDA Patient Affairs Staff: PatientAffairs@fda.gov
- FDA Patient Representative Program: FDAPatientRepProgram@fda.hhs.gov

Center for Biologics

- CBER's Patient Engagement Initiatives: CBERPatientEngagement@fda.hhs.gov
- Office of Communication, Outreach and Development: OCOD@fda.hhs.gov

Center for Medical Devices

- Patient Engagement Meeting Requests: CDRH_PatientMeetings@fda.hhs.gov
- CDRH's Division of Industry and Consumer Education: DICE@fda.hhs.gov

Center for Drugs

- Patient Focused Drug Development: patientfocused@fda.hhs.gov
- CDER's Professional Affairs and Stakeholder Engagement: CDERPASE@fda.hhs.gov

When in doubt…contact Patient Affairs!

PatientAffairs@fda.gov

@FDAPatientInfo

301-796-8460

www.fda.gov/RequestToConnect

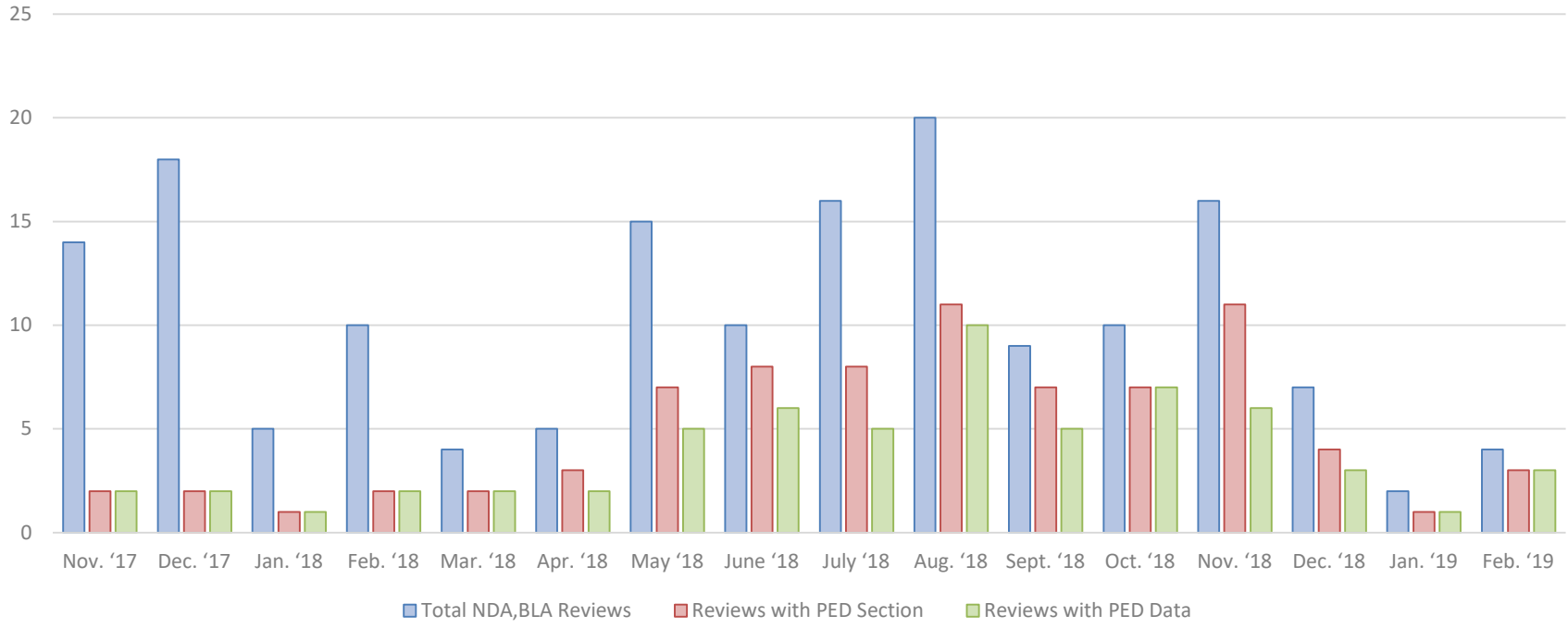
Dave Zook

Faegre Baker Daniels LLP

PFDD Meetings by Division

Division	Internal	External	Total
Neurology Products	3	7	10
Allergy, and Rheumatology Products	3	4	7
Anesthesia, Analgesia, and Addiction Products	4	1	5
Dermatology and Dental Products	2	2	4
Gastroenterology and Inborn Errors Products	2	2	4
Cardiovascular and Renal Products	1	2	3
Oncology Products 2	2	1	3
Hematology Products	2	1	3
Anti-Infective Products	2	0	2
Psychiatry Products	1	1	2
Anesthesiology, General Hospital, Respiratory, Infection Control and Dental Devices	0	1	1
Anti-Viral Products	1	0	1
Bone, Reproductive, and Urologic Products	1	0	1
Clinical Evaluation and Pharmacology / Toxicology	1	0	1
Metabolism and Endocrinology Products	1	0	1
Oncology Products 1	1	0	1
Transplant and Ophthalmology Products	1	0	1
Depends on location of ailment	0	1	1
Total	28	23	51

Patient Experience Data in NDA/BLA Approvals



Patient Input in Medical Product Development

Annie Kennedy

Senior Vice President, Legislation & Public Policy
Parent Project Muscular Dystrophy

PPMD has played a vital role in every single victory in the fight to end Duchenne since 1994.

2018

- Gene Therapy Initiative includes patient preference study and 3 clinical trials in boys with Duchenne
- Certify additional Duchenne Care Centers (24 have been established)
- Launch Global Certified Duchenne Care Center Program with first international certification
- Launch Duchenne Outcomes Research Interchange (DORI) as expansion of The Duchenne Registry
- Led establishment & implementation of ICD code for Duchenne
- Continued national Newborn Screening effort to ensure early diagnosis & launched Duchenne NBS Pilot in New York State
- PPMD End Duchenne Tour to visit 8 more states, both served/engaged and underserved populations

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