A Case Study in Creating Networking and Partnering Opportunities:
FDA Patient Affairs Staff and the National Organization for Rare Disorders (NORD)

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MEMBERS MEETING
September 25-26, 2019
Evolution of Patient Engagement at FDA

- **1980s**: HIV/AIDS patient group founded
- **1990s**: FDA Patient Representative Program™ expands, patients now serve as consultants to reviewers during review cycle
  - FDA establishes Health Professional Liaison Program
  - MedWatch encourages voluntary reporting
- **2000s**: FDA Patient Network web page launched
  - First FDA Patient Representative sits on FDA Advisory Committee
- **2012**: FDA Patient Representatives receive voting rights
  - First FDA Patient Network web page launched
- **2013**: Patient-Focused Drug Development initiative established under PDUFA V
- **2015**: FDA working group established to discuss FDASIA section 1137 (evolved to become Patient Council)
- **2016**: FDA-EMA Patient Engagement Cluster founded
  - First FDA Patient Council meeting held
- **2017**: FDA announces launch of Patient Engagement Advisory Committee (PEAC)
- **2018**: FDA Patient Affairs Staff Established
- **2019**: FDA holds inaugural Patient Engagement Advisory Committee meeting
  - FDA Patient Affairs Staff Established
  - FDA launches Patient Engagement Collaborative with CTTI
  - PFDD Guidance 1 workshop

**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
- FDA’s PAS establishes MOU with NORD-pilot listening sessions
  - PFDD Draft Guidance 1 released
  - Patient Engagement Collaborative (PEC) inaugural meeting
  - FDA’s PAS launches Patients Matter video series
  - PFDD Guidance 2 & 3 public meeting
  - CDRH establishes Patient & Caregiver Connection Program
  - FDA’s PAS launches online portal, Request to Connect!
Patient Affairs Staff (PAS) in the Office of the Commissioner leads patient engagement activities across the medical product Centers—to allow dialogue and collaboration between patients, their advocates, and the FDA.

Who we are

• Creating and assisting with public-private collaborations and partnerships
• Lead cross-cutting programs and activities that leverage best practices and enhance patient engagement.
• Enhancing FDA’s external communication platforms (e.g., Request to Connect, FDA’s For Patients webpage, social media, etc.)
Patient Listening Sessions

Rare Diseases

- Collaboration through an MOU 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

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)
What are Listening Sessions?

**ARE**

- Non-public, non-advisory discussions between FDA staff and patients, their caregivers, and/or their advocates
- 1 to 2 hour meetings
- Via phone, in person at FDA in Silver Spring, MD, or a mix of the two
- Meant to facilitate expeditious sharing of patient or advocate perspectives on:
  - Disease burden
  - Treatment burden
  - Impact on daily activities
  - Priorities to consider in medical product development programs

**Are NOT**

- Open to industry
- Avenues for the endorsement of specific medical products
- Able to guarantee representative or comprehensive perspectives on disease or treatment burden
- Meant to take the place of other patient input and engagement processes, e.g., the FDA Patient Representative Program, Patient-Focused Drug Development (PFDD) Meetings
Previously Conducted Listening Sessions

FDA-Requested Listening Sessions
May 13, 2019 - Sanfilippo Syndrome
February 20, 2019 - Celiac Disease
December 4, 2018 – Fabry Disease
October 23, 2018 – Gene Therapy as a Treatment Modality for Hemophilia

Patient-Requested Listening Sessions
September 17, 2019 - Osteogenesis Imperfecta
August 7th, 2019 - Osteoarthritis (OA)
June 13th, 2019 - Neurofibromatosis (NF)
May 29th, 2019 - Fibrodysplasia Ossificans Progressiva (FOP)
January 16, 2019 - Amyotrophic Lateral Sclerosis (ALS)
November 5, 2018 - Biliary Atresia, Progressive Familial Intrahepatic Cholestasis, Wilson's Disease
NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 280 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.
Listening Sessions Coordination

1. Disease is identified by FDA division

2. NORD connects with disease-specific organization
   1. If no organization
   2. If multiple organizations

3. NORD recruits interested individuals
   1. Demographic diversity; age, location, knowledge, gender,
   2. FDA Questions,
      1. i.e. knowledge of gene therapies
      2. disease severity
      3. role (patient v caregiver)

4. FDA reviews and selects

5. NORD connects, one-on-one with selected participants
Listening Sessions: Feedback

Review Division Staff
The listening session format provides the opportunity for review staff to directly connect with patients, refocus on the ultimate mission of what we do, and integrate the patient voice and experience in our guidance and decision making. It is extremely valuable and appreciated. The materials that we were provided with prior to the session were excellent.

I think the prep sessions were very helpful and helped the reviewers to develop focused questions, maximize the time with the patients. I think the reviewers were very good at keeping things moving while making sure everyone participant had an opportunity to provide input.
Patient/Caregiver Participants

It is very heartening for our community to see such interest and action taken to enhance the agency's understanding of what patients and families face with Sanfilippo!

I feel that all of us were able to explain our stories very clearly even in a short time. I understand that we have so much more to tell the FDA. But we were able to answer questions much longer than we intended, which I felt was amazing ... I want to make a difference, and I want to inspire the world...thank you for giving us your time and your full attention. I’d love to come back and share more of my story in person.
# Medical Product Center Patient Initiatives

## Center for Drugs Evaluation and Research (CDER)
- 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 and Radiological Health (CDRH)
- Partner with Patients
- Patient Engagement Advisory Committee
- Patient and Care-Partner Connection Program

## Center for Biologics Evaluation and Research (CBER)
- Interactive Meetings with Patients
- CBER Workgroups:
  - CBER Patient Engagement Workgroup
  - CBER Rare Disease Coordinating Committee
  - CBER Science of Patient Input (SPI) Team
Patient Engagement Across FDA

- FDA Patient Affairs Staff: PatientAffairs@fda.gov
- FDA Patient Representative Program: FDAPatientRepProgram@fda.hhs.gov
- Patient Engagement Meeting Requests: CDRH_PatientMeetings@fda.hhs.gov
- CDRH’s Division of Industry and Consumer Education: DICE@fda.hhs.gov
- CBER’s Patient Engagement Initiatives: CBERPatientEngagement@fda.hhs.gov
- Office of Communication, Outreach and Development: OCOD@fda.hhs.gov
- Professional Affairs and Stakeholder Engagement: CDERPASE@fda.hhs.gov
- CDER Division of Drug Information: DrugInfo@fda.hhs.gov
- Patient Focused Drug Development: patientfocused@fda.hhs.gov
External Communication Tools

www.fda.gov/RequestToConnect
NORD/FDA Promotional Opportunities

• 300 Member Organizations

• Communication channels:
  • Facebook Group
  • E-newsletter
  • Annual training and local networking

• Webinar Resources
  • FDA EL-PFDD
  • Online Portal
  • Listening Sessions
When in doubt...contact Patient Affairs!

PatientAffairs@fda.gov  301-796-8460
@FDAPatientInfo

www.fda.gov/RequestToConnect