Your Voice Matters: How to Engage with the FDA

For sound, stream audio through your speakers. If you are having trouble accessing sound, please send a message using the chat box in the lower left hand corner.

Alone we are rare. Together we are strong.
This webinar is being recorded.
Submit your questions using the chat function. It can be found at the **left hand side** of the window.
NORD, an independent nonprofit, is leading the fight to improve the lives of rare disease patients and families. We do this by supporting patients and organizations, accelerating research, providing education, disseminating information and driving public policy.
Starting a Not-for-Profit Organization: You can do it, we can help!

February 20, 2020 at 2:00pm ET
Register: https://bit.ly/2tB7DWH
Learn more and find local events:
https://rarediseases.org/rare-disease-day/
Notes & Updates

Learn more and register:
https://rarediseases.org/living-rare-forum/
Speakers

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

Michelle Tarver, MD, PhD  
Director, Patient Science & Engagement  
Office of Strategic Partnerships & Technology Innovation

Robyn Bent, RN, MS  
Director, Patient Focused Drug Development  
Center for Drug Evaluation and Research (CDER)

Diane Maloney, JD  
Associate Director for Policy  
Center for Biologics Evaluation and Research (CBER)
FDA Patient Affairs Staff

Your Voice Matters: How to engage with the FDA

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

January 23, 2020
The Importance of the Patient Voice

• Needs and priorities important to patients and caregivers
• Diverse opinions and experiences
• Risk tolerance and potential benefit
• Real world experience

Patients are at the heart of FDA’s work
Evolution of Patient Engagement at FDA

- **1980s**
  - HIV/AIDS patient group founded

- **1990s**
  - HIV/AIDS group expands to include cancer and other special health issues
  - First FDA Patient Representative sits on FDA Advisory Committee
  - FDA Patient Representatives receive voting rights

- **2000s**
  - 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

- **2012**
  - FDA Patient Network web page launched
  - Patient-Focused Drug Development initiative established under PDUFA V

- **2013**
  - FDA working group established to discuss FDASIA section 1137 (evolved to become Patient Council)

- **2014**
  - FDA-PAS establishes MOU with NORD

- **2015**
  - FDA announces launch of Patient Engagement Advisory Committee (PEAC)
  - FDA-EMA Patient Engagement Cluster founded
  - First FDA Patient Council meeting held

- **2016**
  - FDA-EMA Patient Engagement Collaborative (PEC) inaugural meeting

- **2017**
  - FDA-PAS expands, patients now serve as consultants to reviewers during review cycle

- **2018**
  - FDA’s PAS establishes MOU with NORD, expands to include cancer and other special health issues
  - MedWatch encourages voluntary reporting
  - FDA Patient Representatives receive voting rights
  - 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

- **2019**
  - FDA’s PAS launches online patient portal, PatientsAskFDA
  - FDA-PAS/NORD pilot listening sessions completed, Staff Manual Guide 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

**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 (PAS)

PAS is in the Office of the Commissioner lead patient engagement activities across the medical product Centers to allow dialogue and collaboration between patients, their advocates, and the FDA.

- Create and assist with public-private collaborations and partnerships
- Lead cross-cutting programs and activities to leverage best practices and enhance patient engagement
- Enhance FDA’s external communication platforms (e.g., PatientsAskFDA portal, FDA’s For Patients webpage, educational videos, social media, etc.)
PAS Programs and Activities

- Patient Engagement Collaborative
- FDA Rare Disease Patient Listening Sessions
- Enhancing communications
Patient Engagement Collaborative

- FDA & Clinical Trials Transformation Initiative (CTTI) established an external group of patient organization and individual representatives
- Modeled after the EMA’s Patients’ and Consumers’ Working Party (PCWP)
- **Purpose:** To discuss topics about enhancing patient engagement in medical product development and regulatory discussions at FDA
Patient Listening Sessions

Rare Diseases

- 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
- Pilot to assess the value & establish a process
FDA Rare Disease Patient Listening Sessions

Two Types:

1. **FDA-requested**: specific set of questions to ask of a particular patient sub-population

2. **Patient-requested**: patient community wants to share their experiences and perspectives with the FDA

**Request a Patient Listening Session**
www.fda.gov/PatientsAskFDA

**Patient Listening Sessions Webpage**
www.fda.gov/PatientListeningSessions
### What Are and Are Not Patient Listening Sessions

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<th>ARE</th>
<th>Are NOT</th>
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<tr>
<td>• Non-public, non-advisory discussions</td>
<td>• Open to industry</td>
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<tr>
<td>between FDA staff and <strong>patients, their</strong></td>
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<td>caregivers, and/or their advocates</td>
<td>Avenues for the endorsement of</td>
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<td>• 1 to 2 hour meetings</td>
<td><strong>specific medical products</strong></td>
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<td>• Via <strong>phone, in person</strong> at FDA in</td>
<td>Able to guarantee <strong>representative or</strong></td>
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<tr>
<td>Silver Spring, MD, or a <strong>mix of the</strong></td>
<td><strong>comprehensive perspectives</strong> on</td>
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<td>two</td>
<td>disease or treatment burden</td>
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<tr>
<td>• Meant to facilitate <strong>expeditious sharing</strong></td>
<td>• Meant to take the place of <strong>other</strong></td>
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<td>of patient or advocate perspectives on:</td>
<td><strong>patient input and engagement</strong></td>
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<tr>
<td>▪ Disease burden</td>
<td><strong>processes</strong>, e.g., the FDA Patient</td>
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<td>▪ Treatment burden</td>
<td>Representative Program, Patient-</td>
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<td>▪ Impact on daily activities</td>
<td>Focused Drug Development (PFDD)</td>
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<tr>
<td>▪ Priorities to consider in medical</td>
<td>Meetings</td>
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<td>product development programs</td>
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Patient Listening Sessions

Previously Conducted Listening Sessions

**FDA-Requested Listening Sessions**
December 2, 2019 - **Gastroparesis**
November 13, 2019 - **Childhood Cerebral Adrenal Leukodystrophy (CCALD)**
October 17, 2019 - **Sanfilippo Syndrome (Pediatric Types A & B)**
May 13, 2019 - **Sanfilippo Syndrome (Types A, B,C & D)**
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**
November 6, 2019 - **Cerebral Cavernous Malformation (CCM)**
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**
Listening Sessions: Feedback

Review Division Staff
The patients and caregivers who spoke did an amazing job conveying their experiences, concerns, and desires for treatments. They were remarkable. I truly appreciated hearing from them.

I appreciated the discussion regarding the mismatch in endpoints and hope that we will be able to use that information to inform future drug development.

The patients comments appeared to be in line with the type of information that the agency needs to help drug development for those patients.
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 definitely feel the FDA heard all of our perspectives during the Listening Session.
I thought the follow-up questions were excellent and explored our answers more deeply so as to really try and understand what we were trying to convey.

I felt the Listening Session was very valuable.
I hung up the phone and said to my husband immediately, “I think that went very well. They were trying so hard to understand what living with Gastroparesis really is like. They were really listening!”
Enhancing Communication with Patients
Submit Questions & Meeting Requests

www.fda.gov/PatientsAskFDA
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 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 Caregiver Connection

Center for Biologics Evaluation and Research (CBER)

• Interactive Meetings with Patients
• CBER Workgroups:
  o CBER Patient Engagement Workgroup
  o CBER Rare Disease Coordinating Committee
  o 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

Patient Engagement Initiatives: CDRH_PatientEngagement@fda.hhs.gov

CDRH’s Division of Industry and Consumer Education: DICE@fda.hhs.gov

CDER Division of Drug Information: DrugInfo@fda.hhs.gov

Center for Biologics

Office of Communication, Outreach and Development: OCOD@fda.hhs.gov

Offices of the Commissioner

Professional Affairs and Stakeholder Engagement: CDERPASE@fda.hhs.gov

CBER’s Patient Engagement Initiatives: CBERPatientEngagement@fda.hhs.gov

Patient Focused Drug Development: patientfocused@fda.hhs.gov
When in doubt...contact Patient Affairs!

PatientAffairs@fda.gov
301-796-8460

www.fda.gov/Patients

@FDAPatientInfo

www.fda.gov/PatientsAskFDA
Center for Devices & Radiological Health (CDRH)

Your Voice Matters: How to engage with FDA’s CDRH

Michelle Tarver, MD, PhD
Director, Patient Science & Engagement
Office of Strategic Partnerships & Technology Innovation
Center for Devices and Radiological Health

January 23, 2020
Patients are at the Heart of All We Do

CDRH Vision:
Patients in the U.S. have access to high-quality, safe, and effective medical devices of public health importance, first in the world.
Patients as Co-Pilots

• Patients are:
  – using devices themselves at home
  – more involved in shared decision-making and disease management with their healthcare professionals
  – communicating and connecting with each other through social media and other forums, sharing symptoms, side effects, advice, and providing support

• Patient groups are:
  – developing longitudinal disease registries
  – training patient advisors to improve clinical study design and conduct to be more patient-friendly and efficient
## Benefits of Hearing the Patient Voice

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<tr>
<td>🛠️</td>
<td>Inform device design or clinical trials</td>
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<td>Bring to light new considerations to inform CDRH’s thinking on current issues</td>
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<td>📝</td>
<td>Raise or confirm problems that may exist with specific devices</td>
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<td>🔧</td>
<td>Communicate treatment preferences</td>
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<td>Identify specific population’s approach to benefit-risk for a given treatment</td>
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Patient Input in Regulatory Efforts

Science of Patient Input

- intentional, meaningful interactions with patients that provide opportunities for mutual learning and effective collaborations across the total product life cycle

Patient-Reported Outcomes (PRO)

- any report of the status of a patient’s health condition that comes directly from the patient, without interpretation of the patient’s response by a clinician or anyone else

Patient Engagement

Patient Preferences (PPI)

- qualitative or quantitative assessments of the relative desirability or acceptability of attributes that differ among alternative diagnostic or therapeutic strategies

Patient Reported Outcomes (PRO)

- qualitative or quantitative assessments of the relative desirability or acceptability of attributes that differ among alternative diagnostic or therapeutic strategies

Science of Patient Input
Understanding the patients’ perspectives and proactively incorporating them into all our decisions and regulatory activities where appropriate.

- Consistent Regulatory Review
- Culture of Patient Engagement
- Optimized Research Roadmap
Patient Group Conversations

Patient Experiences with Weight-Loss Devices
Invitation Only Meeting

Have you used one of the following medical devices to help you lose weight?
• Intragastric Balloon (Orbera, Obalon, or ReShape)
• AspireAssist
• VBLOC Maestro

Would you like to come to a meeting at the Food and Drug Administration (FDA) and tell them about your experience?
FDA would like patient feedback on topics such as:
• Your quality of life
• Your treatment expectations versus results
• Your level of satisfaction with your results
• What you need to achieve your weight loss goals

This is your chance to have your voice heard by the FDA

ENTERAL NUTRITION CONSUMER FEEDBACK MEETING
By Invitation Only

The purpose of this meeting is for tube feeders and parents & caregivers of tube feeders to share their experience with tube feeding and tube feeding products with the Food and Drug Administration.

The meeting will cover:
• Patient needs for enteral feeding
• Including blended diets, medication delivery, tube feeding at school/work, concerns and challenges with tube feeding, venting/draining
• Patient demonstrations and videos on blended diet preparation
• An in-depth discussion of ENTERAL
• Including the transition, concerns, experiences, syringes and medication delivery

Lunch will be provided and accommodations made for tube feeding.

May 22nd, 2017 10am – 3:30pm
U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Please contact XXX to confirm attendance at XXXXX by May XX, 2017.

Hosted By:
FEEDING TUBE AWARENESS FOUNDATION
The Qley Foundation
Patient & Caregiver Connection*: Goals

To provide CDRH staff with access to patients & caregivers who are willing to share their individual experiences regarding:

- Medical devices used for diagnosis, treatment, or management of their disease
- Living with their specific disease
- Current issues or trends related to medical devices

Provides FDA timely access to aggregate patients’ voices

*FDA is not seeking, nor will patients or caregivers formally or informally provide group opinions, advice, or recommendations to CDRH.
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<td>Consortium</td>
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<td>NORD</td>
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<td>National Organization for Rare Disorders</td>
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<td>The TMJ Association, Ltd.</td>
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<td>JDRF</td>
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<td>IMPROVING LIVES, CURING TYPE 1 DIABETES</td>
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<td>Facing Our Risk of Cancer EMPOWERED</td>
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<td>The Michael J. Fox Foundation</td>
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<td>American Association of Kidney Patients</td>
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<td>COPD Foundation</td>
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<tr>
<td>National Alliance for Caregiving</td>
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CDRH Community Town Hall: Patient & Caregiver Connection

June 18, 2019
CDRH Patient Engagement Advisory Committee (PEAC)

PEAC role: To help ensure patients’ needs and experiences are considered in FDA’s work on medical devices

• PEAC goals:
  • to better understand and integrate patient perspectives into CDRH’s oversight
  • to improve communications with patients about benefits, risks, and clinical outcomes related to medical devices
  • to identify new approaches, unforeseen risks or barriers, and unintended consequences associated with medical devices

• PEAC members are diverse patients, caregivers, and patient advocates
  • Share perspectives and expertise on various issues
  • Advise and provide formal recommendations to FDA
  • Serve as a resource to CDRH as a body of experts in patient experience, needs, and the activities of the patient community
PEAC 2019 Recommendation—Communication Framework for Cybersecurity

• Medical devices are increasingly connected which can increase risk of potential cybersecurity threats
• Allow patients to be part of the boots-on-the-ground intelligence system
• Clarify actionable steps for patients when issuing cybersecurity safety communications
• Empower patients to maintain good cyber hygiene
• Engage with patients throughout the process

PEAC 2018 Recommendation—Support Data Sharing

- Patients are generating data on social media platforms, registries, and through wearables that could potentially inform regulatory efforts
- Shared a letter of support for the principles of openly sharing non-proprietary data collected by medical devices
- Encourage the empowerment of patients in the development and evaluation of medical devices & become active members in monitoring of devices
- Help enrich the understanding of benefits and risks of technology

U.S. Food and Drug Administration Supports Principles of Open Sharing of Data

Inspired by Patients, Driven by Science

CDRH DRAFT GUIDANCE ON PATIENT ENGAGEMENT
PEAC 2017 Recommendation—Framework for Patient Engagement in Clinical Trials

• Demystify barriers to engaging with patients as advisors in the design and conduct of clinical trials

• Encourage sponsors to involve patients as key opinion leaders in the process and empower them to contribute
Patient Engagement in Clinical Investigations

- 2017 Patient Engagement in Medical Device Clinical Trials
- 2018 PEAC Meeting Discussion Document Posted
- 2019 Draft Guidance Posted

https://www.fda.gov/advisory-committees/committees-and-meeting-materials/patient-engagement-advisory-committee
Draft Guidance Objectives

• Help sponsors understand how they can use patient engagement to elicit experience, perspectives, and other relevant information from patient advisors to improve the design and conduct of medical device clinical investigations

• Highlight the benefits of engaging with patient advisors early in the medical device development process

• Illustrate which patient engagement activities are generally not considered by FDA to constitute research or an activity subject to FDA’s regulations, including regulations regarding institutional review boards (IRBs)

• Address common questions and misconceptions about collecting and submitting to FDA patient engagement information regarding the design and conduct of a medical device clinical investigation
## Example of Roles for Patients in Medical Device Clinical Investigations

<table>
<thead>
<tr>
<th>Study/Research Participants</th>
<th>Patient Advisors</th>
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<tbody>
<tr>
<td>• Study/research participants are individuals who are or become a participant in research, either as a recipient of the test article or as a control, and may include healthy individuals.</td>
<td>• Patient advisors refers to individuals who have experience living with a disease or condition, and can serve in an advisory or consultative capacity to improve clinical investigation design and conduct, but who are not study/research participants themselves.</td>
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CDRH Encourages Patient Engagement Through Draft Guidance

Resources

FDA CDRH Websites:


Contacts for Medical Devices

• For Patient-Reported Outcome Questions: CDRH-PRO@fda.hhs.gov

• For Patient Preference Information Questions: CDRH-PPI@fda.hhs.gov

• For Patient Engagement Questions: CDRH_PatientEngagement@fda.hhs.gov

• If you are not sure: michelle.tarver@fda.hhs.gov
Thank You
CDER Patient Engagement

Robyn Bent, RN, MS
Director, Patient Focused Drug Development
FDA Center for Drug Evaluation and Research

January 23, 2020
Topics to Cover

1. Patient-Focused Drug Development (PFDD) Efforts
   • PFDD Meetings
   • Externally-Led PFDD Meetings
   • Methodologic Guidance Series
   • Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data

2. PASE Programs and Initiatives
   • Engagement and Targeted Outreach
Creating Opportunities for Dialogue

• Patients are uniquely positioned to inform understanding of the therapeutic context for drug development and evaluation
  – There is a need for more systematic ways of gathering patient perspectives on their condition and treatment options Development

• Patient-Focused Drug Development
  – FDA convened 24 meetings on specific disease areas in FY 2013-17
    – Meetings can help advance a systematic approach to gathering input
PFDD Meetings

• Meetings follow similar, but tailored design
  – Take into account current state of drug development, specific interests of FDA review division, needs of the patient population

• Discussion elicits patients' perspectives on their disease and on treatment approaches

• Input is generated in multiple ways:
  – Patient panel comments and facilitated discussion with in-person participants
  – Interactive webcast and phone line for remote participants
  – A federal docket allowing for more detailed comments
Sample PFDD Agenda

• Opening Remarks
• Setting the context
  – Overview of Disease
  – Overview of Discussion Format
• Discussion Topic 1-
  – Panel Discussion
  – Audience Discussion with Polling
• Discussion Topic 2 (with a short break)
  – Panel Discussion
  – Audience Discussion with Polling
• Open Public Comment
• Closing Remarks
Sample Discussion Overview

**Topic 1: Symptoms and daily impacts of condition that matter most to patients**

- How would you describe your condition?
- What are the most significant symptoms that you experience resulting from your condition?
- Are there specific activities that are important to you but that you cannot do at all or as fully as you would like because of your condition?
- How has your condition changed over time?

**Topic 2: Current Approaches to Treatment of condition**

- What are you currently doing to help treat your condition?
- How well does your current treatment regimen manage your condition?
- What are the most significant downsides to your current treatments, and how do they affect your daily life?
- What challenges or barriers to accessing or using medical treatments for your condition have you or do you encounter?
- What specific things would you look for in an ideal treatment for your condition?
Externally-Led PFDD: The Opportunity

- Patient organizations identify and organize patient-focused collaborations to generate public input on specific disease areas.

- Meetings provide an important opportunity to hear directly from patients, patient advocates, and caregivers about the symptoms that matter most to them, the impact the disease has on patients’ daily lives, and patients’ experiences with currently available treatments.
What we have Learned from PFDD Meetings

- Patients with a chronic serious disease are experts on what it’s like to live with their condition.

- The “chief complaints” heard in PFDD meetings often were not being factored explicitly into drug development plans, including measures planned for collection in trials.

- Patients want to be as active as possible in the work to develop and evaluate new treatments.
Update on PFDD Guidances and Public Workshops

PFDD Guidance 1: Collecting Comprehensive and Representative Input
- Workshop held on December 18, 2017
- Issued Draft Guidance in June 2018

PFDD Guidance 2: Methods to Identify What is Important to Patients
- Workshop held on October 15-16, 2018
- Issued Draft Guidance in September 2019

PFDD Guidance 3: Select, Develop or Modify Fit-for-Purpose Clinical Outcome Assessments
- Workshop held on October 15-16, 2018
- Discussion Document published

PFDD Guidance 4: Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making
- Workshop held on December 6, 2019

PFDD Guidance 5: Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data
- Workshop held on March 19, 2018
- Issued Draft Guidance in December 2018

Workshop on Enhancing Patient Input on Clinical Trials
- Workshop held on March 18, 2019
- Meeting summary report publicly available
PASE Programs

- Engagement and Targeted Outreach
- Network of Experts (NoE)
- Safe Use Initiative
- Drug Trial Snapshots
Sample Drug Trial Snapshot

Were there any differences in how well the drug worked in clinical trials among sex, race and age?

- **Sex:** ADAKVEO worked similarly in males and females.
- **Race:** The majority of patients were Black or African Americans. The number of patients in other races were limited; therefore, differences in how ADAKVEO worked among races could not be determined.
- **Age:** The majority of patients were adults between 18-63 years of age. There were not enough patients older than 65 years to determine whether there was any difference in how ADAKVEO worked between older and younger patients.

What are the possible side effects?

ADAKVEO may cause serious infusion-related reactions and platelet clumping.

Most common side effects of ADAKVEO include nausea, joint pain, back pain and fever.

Were there any differences in side effects among sex, race and age?

- **Sex:** The occurrence of side effects was similar between males and females.
- **Race:** The majority of patients were Black or African Americans. The number of patients in other races were limited; therefore, differences in how ADAKVEO worked among races could not be determined.
- **Age:** The majority of patients were adults between 18-63 years of age. There were not enough patients older than 65 years to determine whether there was any difference in side effects between older and younger patients.
How Can Stakeholders Contribute?

- Support research
- Develop patient registry
- Conduct natural history studies
- Collect patient experience data
- Coordinate stakeholder work
- Communicate, educate and outreach
- Convene meetings
- Contribute to guidance and policy development
Resources

CDER PFDD Website:  
https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

Drug Trial Snapshots:  
https://www.fda.gov/drugs/drug-approvals-and-databases/drug-trials-snapshots

CDER Professional Affairs and Stakeholder Engagement:  

ClinicalTrials.gov: https://clinicaltrials.gov/

NCATS Genetic and Rare Diseases Information Center:  
https://ncats.nih.gov/gard

NCATS Toolkit for Patient-Focused Therapy Development:  
https://ncats.nih.gov/toolkit
Thank you!
Your Voice Matters:
How to Engage with FDA CBER

Diane Maloney, J.D.
Associate Director for Policy
FDA Center for Biologics Evaluation and Research

NORD webinar:
January 23, 2020
Your Voice Matters:

CBER-regulated Products

- Allergenics
- Blood Products
- Human Tissues and Cellular Products
- Gene Therapies (including genome editing)
- Vaccines (preventative and therapeutic)
- Xenotransplantation Products
- Devices Related to Biologics
- Some other biologics (e.g., phage therapy)
CBER Works with Others at FDA on Patient Engagement

We work regularly with:

| CDER | CDRH | Patient Affairs Staff | Stakeholder Engagement Staff |

Routine cross-cutting agency meetings
CBER Patient Engagement Groups

- CBER Patient Engagement Workgroup
- CBER Rare Disease Coordinating Committee
- Science of Patient Input Initiative
Patient Engagement - Legislation

21st Century Cures

- Patient experience (PE) Statement
- PE report
- Guidance on developing and submitting draft guidance and PE data

Methodological Guidances

PDUFA VI

- FDA PFDD on-line repository
- Workshop and report: Enhancing patient perspectives in clinical trials
How CBER Works with Individual Patients and Patient Advocacy Groups

**Medical product and policy development**
- Patient Representative Program
- NORD Rare Disease Listening Sessions
- Public meetings, workshops, PFDDs, Advisory Committee meetings
  - Patient Engagement Advisory Committee (devices)
- Comments on guidance and rule making
- MedWatch reporting

**Advancing efforts to strengthen FDA-patient community relationship**
- Patient Engagement Collaborative
- CBER outreach (e.g., educational webinars, presentations at advocacy meetings)

**Other opportunities**
- Patient organizations: request a meeting: CBERPatientEngagement@fda.hhs.gov
- Expanded Access requests
Example of Listening Sessions

• Hemophilia and Gene Therapy
  – to understand patient/caregiver concerns, perceived risks and benefits, and expectations of gene therapy as a treatment modality for hemophilia
  – to understand perspectives of patients/caregivers who may be interested in using gene therapy as a treatment modality for hemophilia
Workshop: Facilitating End to End Development of Individualized Therapeutics

• Foster development of treatment of one individual (or a very small number) based on engineering a product aimed at the specific molecular mechanism underlying a patient’s illness

• Explore scientific & manufacturing challenges

• Explore challenges from patient perspective

• Workshop: March 3, 2020
Thank you!
Question and Answer Session
Resources

To learn more information and find a clinical trial, please visit:
clinicaltrials.gov
Questions?

Submit your questions in the chat box.

Our presenters will answer them in the order in which they came in and based on relevance to the discussion.
Thank you.

Alone we are rare. Together we are strong.

rarediseases.org