Engaging Patients in Medical Device Regulatory Efforts

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Patient Engagement Lead
Center for Devices & Radiological Health
Food and Drug Administration
December 3, 2020

FDLI
Food and Drug Law Institute

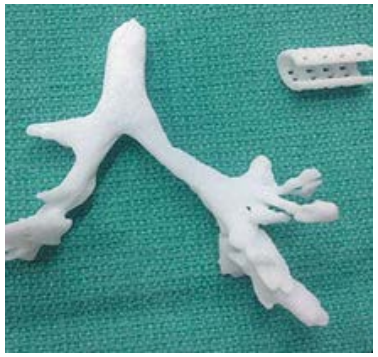
Polling Question #1

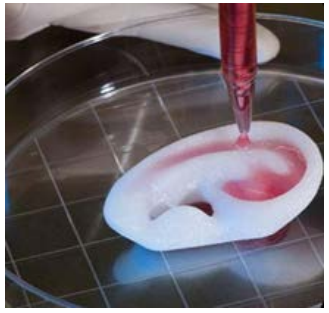
Question:

What is one way that you have engaged with CDRH?

Answer:

a. **Small group conversations**
b. **Public meetings**
c. **Advisory committee meetings**
d. **Written correspondence or comments**
e. **Other**
f. **I have never engaged with CDRH**





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.

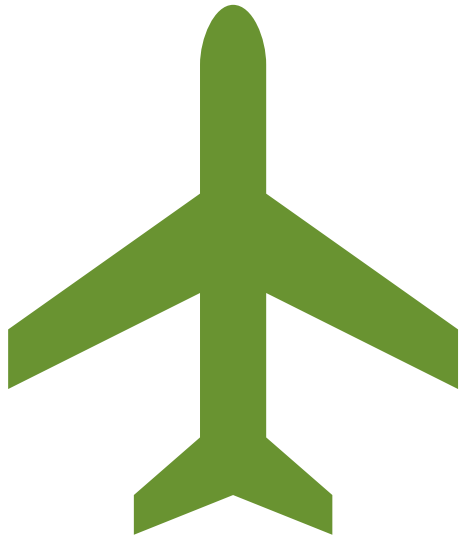
Patients are Co-Pilots in Their Care

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



Polling Question # 2

Question:

What is Patient Engagement?

Answer:

a. **Intentional interactions between FDA and patients**
b. **Provides opportunities for FDA and patients to learn from one another**
c. **Allows for effective collaborations between FDA and patients across the total product life cycle**
d. **All of the above**

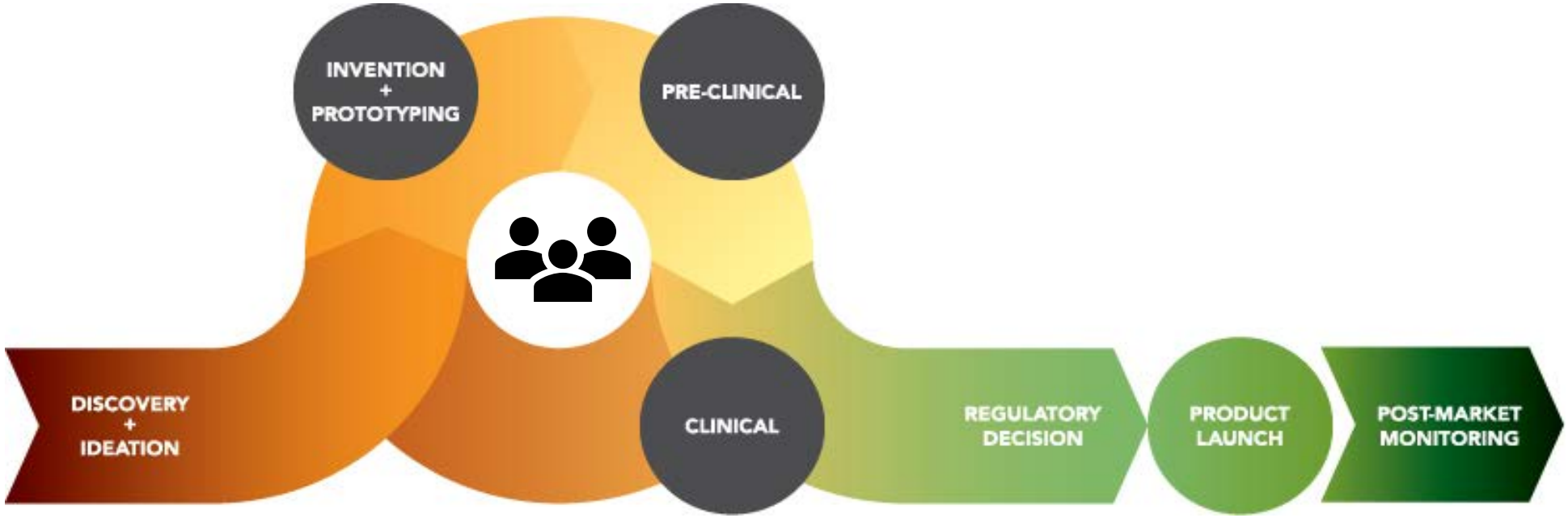
Defining Patient Engagement*

Patient engagement is defined as intentional, meaningful interactions with patients that provide opportunities for mutual learning and effective collaborations.

Source: https://www.fda.gov/about-fda/center-devices-and-radiological-health/cdrh-patient-engagement

*draft definition

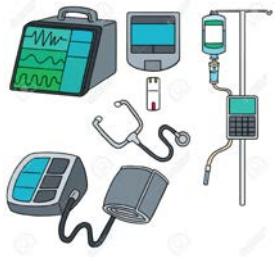
Patient Input Benefits All Phases of the Total Product Lifecycle (TPLC)



Inform device or clinical study design	Bring to light new considerations to inform thinking on current issues	Raise or confirm problems that may exist with specific devices	Communicate benefits and risks of medical devices	Identify specific population's views on benefit-risk for a given treatment

Patient & Caregiver Connection*: Goals

To enhance CDRH staff's ability to hear, understand and integrate **patients & caregiver perspectives in the course of their work** by those who are willing to share their individual experiences regarding:



Medical devices used for diagnosis, treatment, or management of their disease

Living with their specific condition

Current issues or trends related to medical devices

* The program is designed to broaden CDRH staff exposure to patients' viewpoints, but not to provide policy advice, recommendations, or opinions.

Current Partners of the Patient & Caregiver Connection

Patient Engagement at CDRH

Inspired by Patients, Driven by Science

U.S. FOOD & DRUG ADMINISTRATION

TMJ ASSOCIATION
PATIENT CONVERSATIONS WITH THE FDA

OFFICE OF HEALTH TECHNOLOGY (OHT-1)
JANUARY 27, 2020

FDA

Bringing Valuable Perspectives to FDA

FDA Workshop: Evolving Role of Artificial Intelligence in Radiological Imaging February 25-26, 2020

- Patients and a caregiver were invited to share their perspectives on this emerging technology
- Enriched the conversation with many developers commenting on how helpful it was to their product development

Assessing the Impact of COVID-19 on Medical Devices and Supplies

AAKP American Association of Kidney Patients

COVID-19 Supplies, Device, and Access Impact Survey–May 2020

In May 2020, members of the AAKP completed a survey to inform the U.S. Food and Drug Administration (FDA) about dialysis device-related concerns. Survey responses helped the FDA to assess possible challenges kidney patients were having accessing supplies, devices, and services. This information was used to help inform CDRH's response efforts to address device shortages.

Responding to COVID-19 Patient Community Questions

FDA

MDA Muscular Dystrophy Association

June 2020, Webinar

NORD National Organization for Rare Disorders

September 16, 2020 Webinar

CDRH Townhall Meeting: Engaging Patients Through the Total Product Life Cycle of a Digital Health Technology, September 10, 2020

- CDRH employees heard about the valuable input patients have provided across the total product life cycle of a digital health technology
- Patients shared their personal experience with using a digital health technology to manage their medical condition

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

Patient-focused and relevant recommendations

1-2 Homework assignments per year

1-2 PEAC Meetings per year

PEAC 2019 Recommendation—Communication Framework for Cybersecurity

- Communicating Cybersecurity Vulnerabilities to Patients: Considerations for a Framework posted for comments
  - Docket open until 12/21/2020
  - https://www.fda.gov/about-fda/cdrh-patient-engagement/communicating-cybersecurity-vulnerabilities-patients-considerations-framework
- Allow patients to be part of the boots-on-the-ground intelligence system
- Clarify actionable steps for patients when issuing cybersecurity safety communications
- Announced cyber hygiene miniseries for the public
  - https://www.fda.gov/medical-devices/digital-health-center-excellence/cybersecurity

PEAC 2020 Recommendation for Artificial Intelligence (AI) and Machine Learning (ML) in Medical Devices

- Demographic composition of datasets on which the software learns and is validated
- How is device information shared with patients
- Factors that impact patient trust in the technology

https://www.fda.gov/about-fda/cdrh-patient-engagement/cdrh-patient-engagement-advisory-committee

Polling Question #3

Question:

What are some ways that CDRH engages patients?

Answer:

a. Patient Engagement Advisory Committee Meetings (PEAC)

b. Public Workshops

c. Townhall meeting for staff

d. Patient Conversations

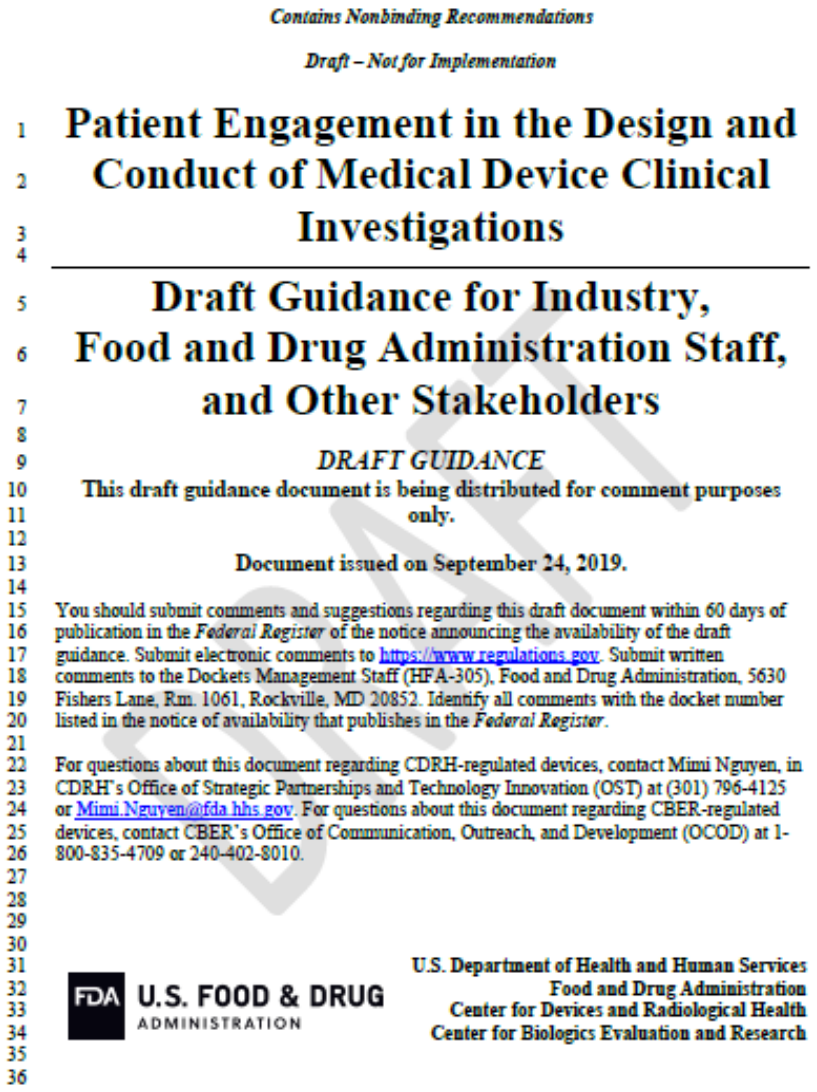
e. All of the above

CDRH DRAFT GUIDANCE ON Patient Engagement

Inspired by Patients, Driven by Science

CDRH Encourages Patient Engagement Through Draft Guidance

- https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-engagement-design-and-conduct-medical-device-clinical-investigations



Contains Nonbinding Recommendations

Draft – Not for Implementation

Patient Engagement in the Design and Conduct of Medical Device Clinical Investigations

Draft Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

DRAFT GUIDANCE

This draft guidance document is being distributed for comment purposes only.

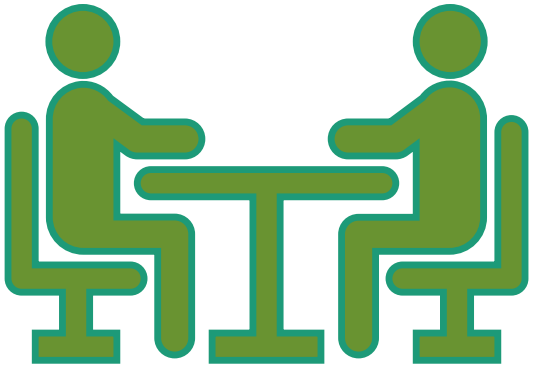
Document issued on September 24, 2019.

You should submit comments and suggestions regarding this draft document within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. Identify all comments with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions about this document regarding CDRH-regulated devices, contact Mimi Nguyen, in CDRH's Office of Strategic Partnerships and Technology Innovation (OST) at (301) 796-4125 or Mimi.Nguyen@fda.hhs.gov. For questions about this document regarding CBER-regulated devices, contact CBER's Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010.

FDA U.S. FOOD & DRUG ADMINISTRATION

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health
Center for Biologics Evaluation and Research



Draft Guidance Objectives

- Help sponsors understand how they can use patient engagement to elicit experience, perspectives, and other relevant information from patient advisors 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); and
- 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.

Polling Question # 4

Question:

What are some roles for patients in medical device clinical studies?

Answer:

a. As a test article recipient

b. As a “control” subject

c. As a consultant who can improve the clinical design and conduct

d. All of the above

Resources

FDA CDRH Websites:

Engagement CDRH: https://www.fda.gov/about-fda/center-devices-and-radiological-health/cdrh-patient-engagement

PEAC: *https://www.fda.gov/about-fda/cdrh-patient-engagement/cdrh-patient-engagement-advisory-committee*

Patient & Caregiver Connection: *https://www.fda.gov/about-fda/cdrh-patient-engagement/cdrh-patient-and-caregiver-connection*

Patient Preference: https://www.fda.gov/about-fda/cdrh-patient-engagement/patient-preference-information-ppi-medical-device-decision-making

Patient-Reported Outcomes: https://www.fda.gov/about-fda/cdrh-patient-engagement/patient-reported-outcomes-pros-medical-device-decision-making

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

U.S. FOOD & DRUG ADMINISTRATION

Engaging with FDA: Opportunities and Boundaries

Susan Chittooran, MSW
Patient Affairs Staff
Office of the Commissioner

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Disclaimer

The views and opinions expressed in the following PowerPoint slides are those of the individual presenters and should not be construed to represent FDA's view or policies

OUTLINE

1. Overview
2. Patient Affairs Staff patient initiatives
3. FDA Patient Engagement Activities
4. Resources

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Overview

Understanding patient engagement at FDA

The Importance of the Patient Voice

- Insights on issues, needs and priorities that are important to patients and caregivers
- Diverse opinions and experiences
- Insights on risk tolerance and potential benefit
- Real world experience

Patients are at the heart of FDA's work!

Evolution of Patient Engagement at the FDA

1988

- Office of AIDS Coordination established

1993

- Office of AIDS Coordination renamed to Office of AIDS and Special Health Issues (OASHI) and broadened to include patients with cancer and other serious and life-threatening diseases
- First FDA Patient Representative® served on an advisory committee

1996

- FDA Patient Representatives® received voting rights on advisory committees

2001

- FDA Patient Representative Program®
  role expanded to serve as consultants to scientific and regulatory reviewers

2008

- Patients and consumers encouraged to report medical product problems using FDA's existing MedWatch system

2012

- A section of the FDA website is created specifically For Patients
- Patient-Focused Drug Development (PFDD) initiative launched

2013

- Internal working group examines ways to increase patient involvement in FDA processes
- Consumer-friendly form introduced in FDA's MedWatch system to report medical product problems

2015

- Patient Preference Information (PPI) framework and guidance for medical device decision making
- Patient Engagement Advisory Committee (PEAC) meetings regarding medical devices

2016

- FDA and European Medicines Agency (EMA) Patient Engagement Cluster created
- First Patient Council (internal) meeting held

2017

- PAS established in the Office of the Commissioner
- Public Workshop on PFDD guidance

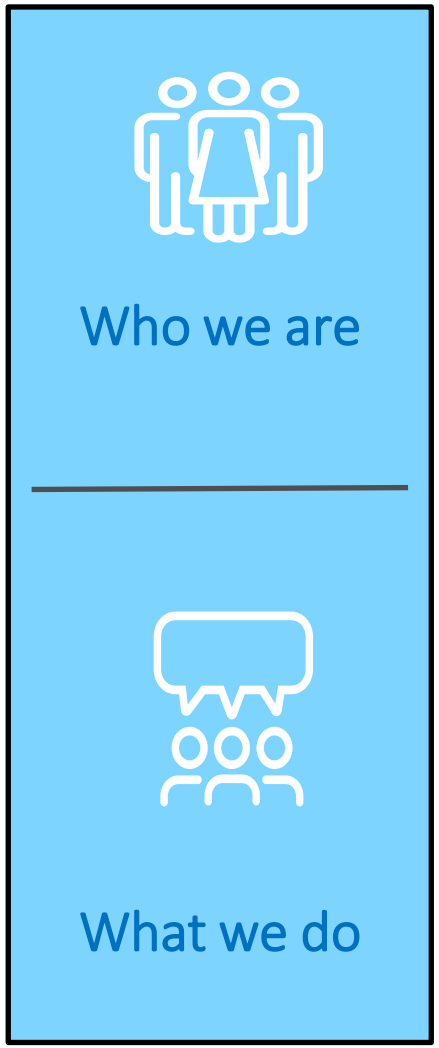
2018

- Memorandum of Understanding with National Organization For Rare Disorders (NORD) launched the Patient Listening Session pilot program
- Patient Engagement Collaborative (PEC) launched with Clinical Trials Transformation Initiative (CTTI)
- Center for Devices and Radiological Health (CDRH) Patient & Caregiver Connection (P&CC) program launched
- Public Workshops on PFDD guidances and drafts released

2019-2020

- Patient Affairs Staff (PAS) online webform, Patients Ask FDA
- PFDD Workshop on Guidance 4
- Draft PFDD Guidance 2 released
- COVID-19 Patient Resources Page Launched
- Final PFDD Guidance 1 released

Patient Affairs Staff



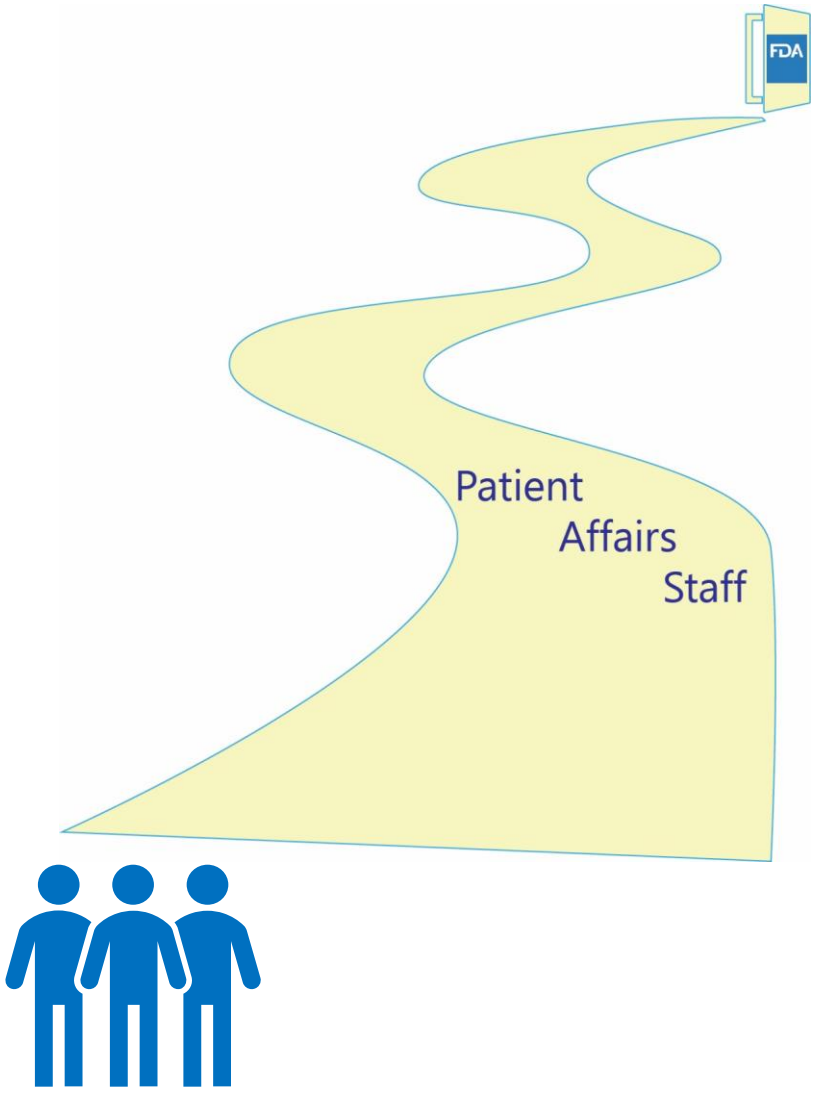
- Small team in the Office of the Commissioner dedicated to providing an inviting, welcoming and meaningful experience for patient communities to engage with the FDA

- Lead patient engagement activities across the medical product Centers through:
  - Cross-cutting programs and activities
  - Public-private collaborations and partnerships
  - Enhance external communication platforms

Patient Affairs initiatives

Cross-center patient activities

PAS Programs and Activities



Patient Listening Sessions

FDA/EMA Patient Engagement Cluster

Patient Engagement Collaborative

Enhancing Communications

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What is an FDA Listening Session?

- One of the many ways that patients can **share their experience** living with and managing a disease or condition
- Patients & caregivers can **talk directly with FDA** scientific staff
- A **resource** for FDA's medical product Centers to **quickly engage** with patients or their advocates

Patient Listening Sessions

NORD® National Organization for Rare Disorders

FDA U.S. FOOD & DRUG ADMINISTRATION

REAGAN-UDALL FOUNDATION for the Food and Drug Administration

Rare Diseases

- Memorandum of Understanding with the National Organization for Rare Disorders (NORD)
- Inform regulatory decision-making
- Educate review staff
- Help patients and their advocates understand the FDA's work
- Starting point to inform early stage R&D

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Patient Listening Sessions

Types of Listening Sessions

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

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

Listening Session Summaries can be found on the Patient Listening Sessions Webpage
www.fda.gov/PatientListeningSessions

Understanding Patient Listening Sessions

What do FDA Patient Listening Sessions look like?

ARE

- Non-public, non-advisory discussions between FDA staff and patients, their caregivers, and/or their advocates
- 1 to 1.5 hour meetings
- Via phone, in person at FDA or a mix of the two
- Cross-center meetings (2+ FDA centers)
- 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

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Polling Question #1

Which of the following statements is true in regard to FDA's Patient Listening Session initiative?

A. Industry can attend Patient Listening Sessions
B. Patient Listening Sessions are cross-center meetings (involve 2 or more FDA centers)
C. Listening Sessions are public meetings
D. Patient Listening Sessions are a good way to tell FDA why they should approve a investigational medical product (e.g. unapproved drug)

Patient Engagement Cluster

FDA U.S. FOOD & DRUG ADMINISTRATION

EUROPEAN MEDICINES AGENCY

Mutual exchange on:

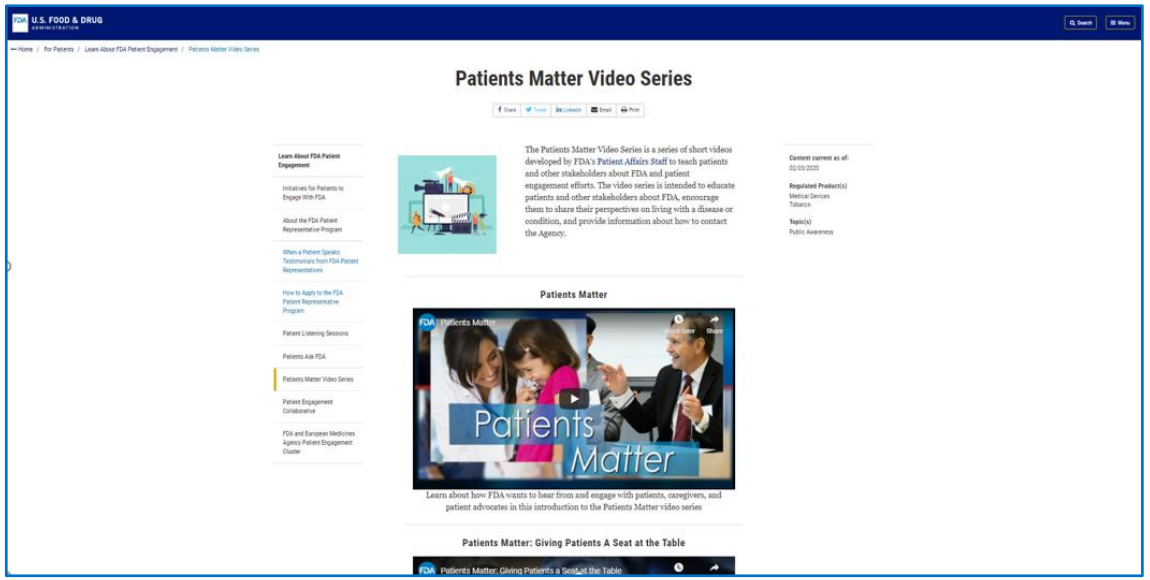
- Engaging and involving patient stakeholders
- High profile topics of mutual interest
- Collaborations to enhance engagement

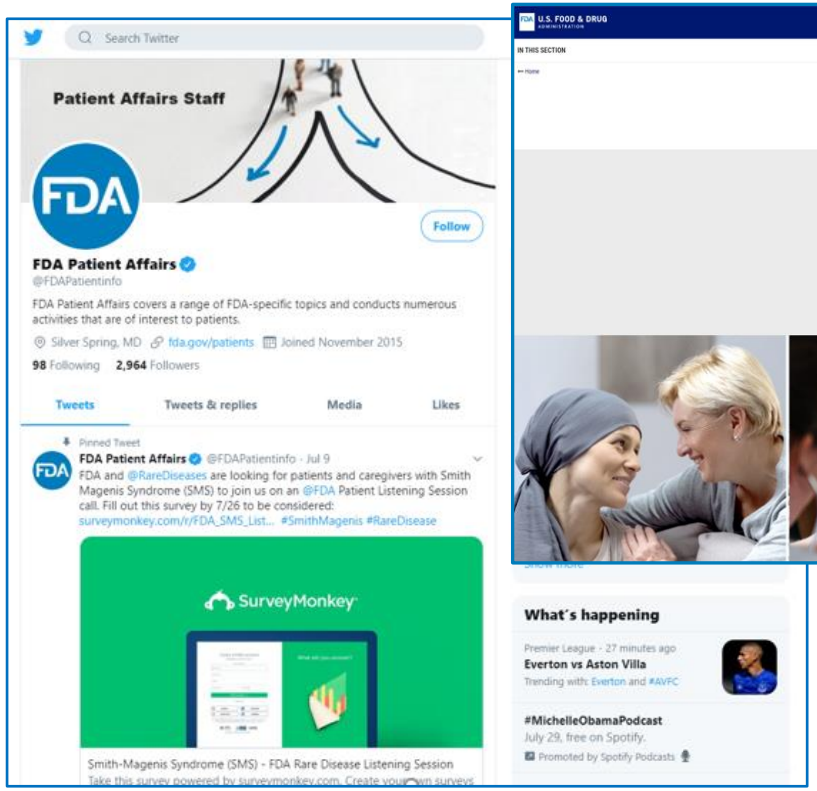
Publication:
Nature Reviews Drug Discovery 30 September 2019 -
Engaging patients in medicines regulation: a tale of two agencies
https://www.nature.com/articles/d41573-019-00164-y

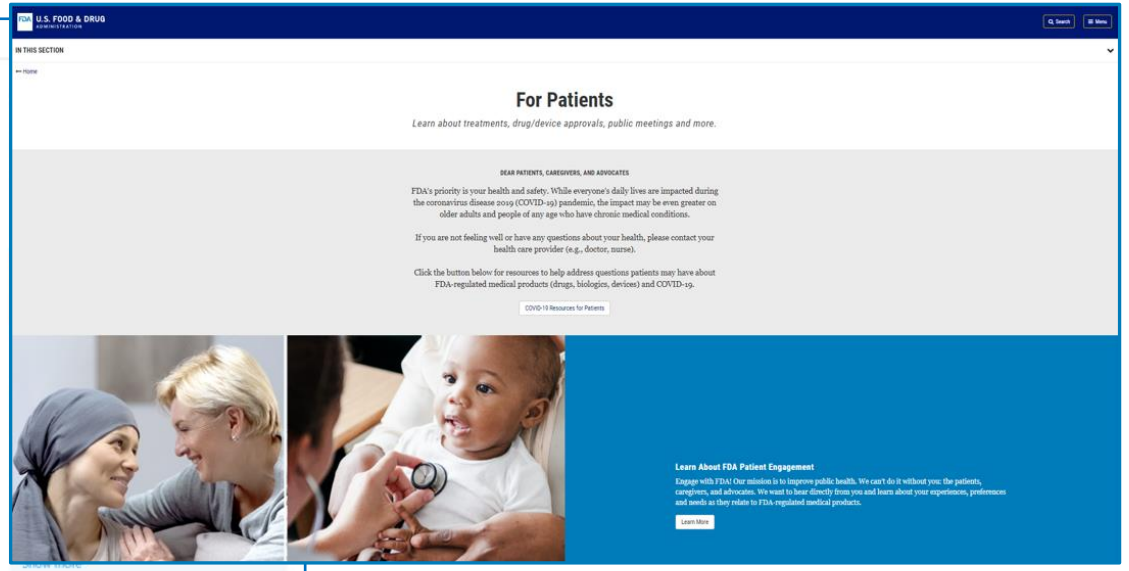
Patient Engagement Collaborative

- FDA & Clinical Trials Transformation Initiative (CTTI)
- EMA's Patients' and Consumers' Working Party (PCWP) model
- Purpose: Discussions about engaging patients in medical product development and regulatory discussions

Enhancing Communications







Recent examples of FDA's cross-center collaboration

- MDA webinar
- NORD rare disease webinar
- FDA's patient COVID page
- FDA Patient Council (internal FDA workgroup)
- 17 Patient Listening Sessions
- Cross-center patient meetings/calls

FDA Patient Initiatives

Patient activities in other offices/Centers

FDA Patient Representative Program SM

FDA Patient Representative®

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

Criteria for becoming an FDA Patient Representative:

FDA's Medical Product Center Patient Engagement Initiatives

Center for Drug Evaluation and Research (CDER)

- FDA-led Patient-Focused Drug Development Meetings (PFDD)
- Externally-led Patient-Focused Drug Development Meetings
- PFDD Methodological Guidance Series
- Critical Path Innovation Meetings (CPIM)

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

Center for Medical Devices and Radiological Health (CDRH)

- Patient Engagement Advisory Committee
- Patient and Care-Partner Connection Program
- Community Town Halls
- Public Health Symposiums
- Patient Group Conversations

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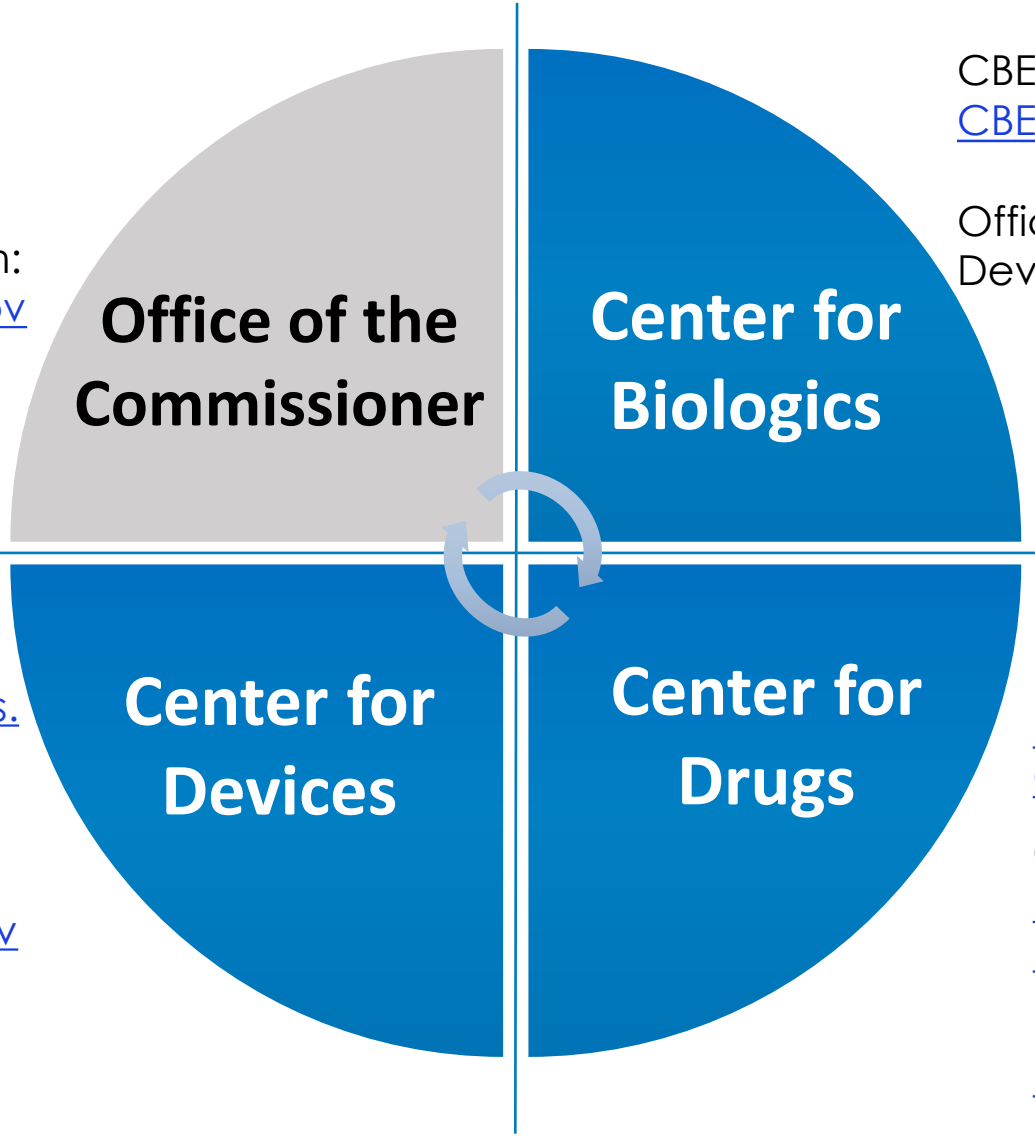
Resources

Tools and resources for engagement

Patient Engagement Across FDA

FDA Patient Affairs Staff:
PatientAffairs@fda.gov
https://www.fda.gov/PatientAffairs

FDA Patient Representative Program:
FDAPatientRepProgram@fda.hhs.gov
https://go.usa.gov/xfB4h



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

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

Patient Engagement Initiatives:
https://go.usa.gov/xfBdx
CDRH_PatientEngagement@fda.hhs.gov

Patient Engagement Meeting Requests:
CDRH_PatientMeetings@fda.hhs.gov

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

Professional Affairs and Stakeholder Engagement:
https://go.usa.gov/xfBpG
CDERPASE@fda.hhs.gov

CDER Division of Drug Information:
https://go.usa.gov/xfBpM
DrugInfo@fda.hhs.gov

Patient Focused Drug Development:
https://go.usa.gov/xfBph
patientfocused@fda.hhs.gov

Acronyms

Acronym	Full Name
AC or ADCOM	Advisory Committee
CBER	Center for Biologics Evaluation and Research
CDER	Center for Drug Evaluation and Research
CDRH	Center for Devices and Radiological Health
CTTI	Clinical Trials Transformation Initiative
FDA	U.S. Food and Drug Administration
NORD	National Organization for Rare Disorders
OC	Office of the Commissioner
PAS	Patient Affairs Staff (located in OC)
PASE	Professional Affairs and Stakeholder Engagement Staff (located in CDER)
PEAC	Patient Engagement Advisory Committee
PFDD	Patient Focused Drug Development
PPI	Patient Preference Initiative

Polling Question #2

You want to have a meeting with FDA. How do you go about submitting a meeting request?

A. Email the FDA Commissioner directly
B. Repeatedly contact FDA on social media (Twitter, Facebook)
C. Just show up at FDA's campus
D. Email everyone you know at FDA
E. Submit a request using the PatientAskFDA webform
F. I have no idea

Questions & Meeting Requests

U.S. Department of Health and Human Services

FDA U.S. FOOD & DRUG ADMINISTRATION

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Patients: Ask FDA

This form is for:

- Patients
- Caregivers
- Advocates
- Health Care Professionals

*This form **is not** for industry stakeholders.*

Please use this form to:

- Ask a question to FDA
  or
- Request a meeting with FDA

To report adverse events that you observe or suspect for human medical products please use the **MedWatch reporting form.*

This form is for requests about:

- Diseases or Health Conditions
- Drugs
- Devices
- Vaccines/Blood/Biologics

*For other requests please visit the **FDA contacts page**.*

Please tell us who you are (required):

Individual Patient, Caregiver or Advocate | Patient Group | Health Professional | Other

Question or Meeting Request (required):

Question | Meeting Request

What is your request about? (required):

a drug | a medical device | a vaccine, blood or biologic | disease or health condition | multiple or unknown

Is your request about a specific FDA program? (required):

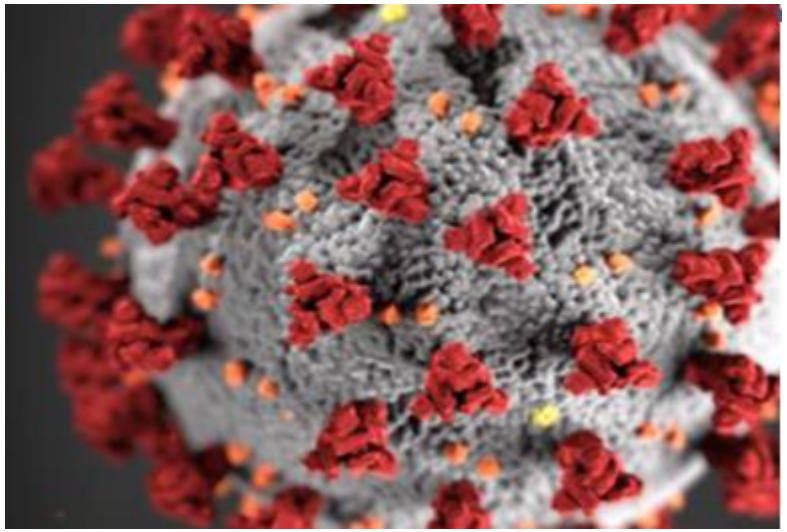
Yes | No, or I do not know

Name of Disease or Condition (if applicable):

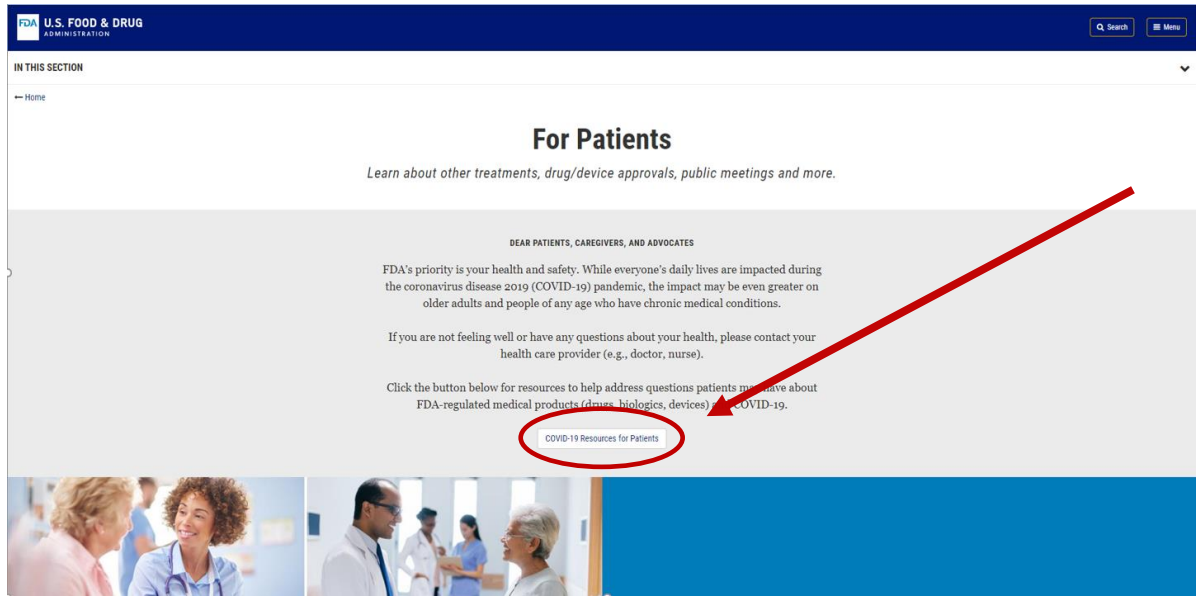
Enter Name of Disease or Condition (if applicable)

www.fda.gov/PatientsAskFDA

COVID-19
INFORMATION & UPDATES



www.fda.gov



www.fda.gov/patients

Patient Affairs Team

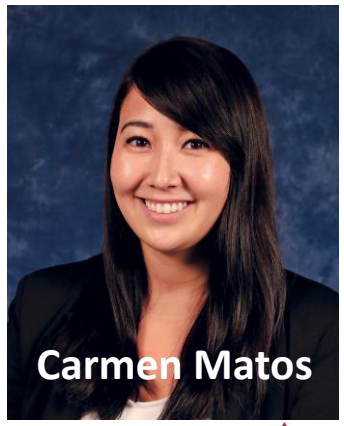
PatientAffairs@fda.gov

301-796-8460

www.fda.gov/Patients

@FDAPatientInfo

www.fda.gov/PatientsAskFDA



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U.S. FOOD & DRUG ADMINISTRATION

Center for Drug Evaluation and Research

Engaging with FDA: Opportunities and Boundaries

CDR Sadhna Khatri, PharmD, MPH, MS

Regulatory Officer

Professional Affairs and Stakeholder Engagement (PASE)

Office of Center Director

FDLI
Food and Drug Law Institute

Dec 3, 2020

CDER's Public Health Mission

CDER's mission is to:

- Promote and protect public health by assuring that safe and effective drugs are available to Americans

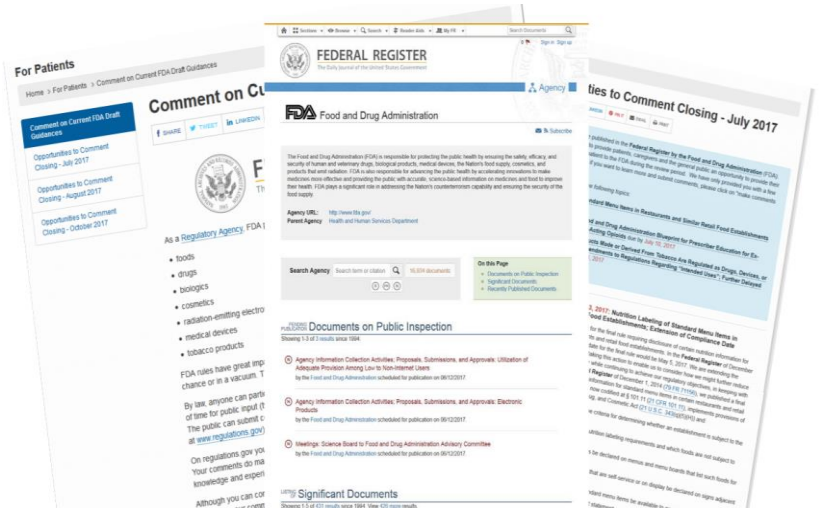
Ultimately, patients are the focus of all CDER activities and we need to engage with them

Opportunities for Engagement at CDER

- Patient-Focused Drug Development meetings (PFDD)
  - Focused on better understanding the disease and patient experience
- Advisory Committee Meetings
  - Open Public Hearing Portion
- Listening sessions, and meetings with Patients and Patient Organizations
  -Typically scheduled with the Review Division

Opportunities for Engagement at CDER (continued)

- Citizen Petitions
- Comments to the docket for Federal Register Notices
- Guidance development
- Emails, letters and phone calls



What is Patient-Focused Drug Development (PFDD)?

PFDD is a systematic approach to help ensure that **patients' experiences, perspectives, needs, and priorities** are captured and meaningfully incorporated into drug development and evaluation.[1]

[1] https://www.fda.gov/drugs/development-approval-process-drugs/patient-focused-drug-development-glossary

Patients are **experts** in their condition.

It is important to get patient input early in the drug development process.

- Patients' "chief complaints" may not be factored explicitly into medical product development plans, including measures of medical product benefit planned in clinical studies.
- The lessons learned from PFDD meetings range from experiences common across rare diseases to disease specific experiences that matter more most to patients.
  - Specific experiences that matter most to patients
  - Patient perspectives on meaningful treatment benefits
  - How patients want to be engaged in the drug development process

PFDD Meetings

CY2013
- Chronic Fatigue Syndrome/ Myalgic Encephalomyelitis
- HIV
- Lung Cancer
- Narcolepsy

CY2014
- Sickle Cell Disease
- Fibromyalgia
- Pulmonary Arterial Hypertension
- Inborn Errors of Metabolism
- Hemophilia A, B, and Other Heritable Bleeding Disorders*
- Idiopathic Pulmonary Fibrosis
- Female Sexual Dysfunction

CY2015
- Breast Cancer
- Chagas Disease
- Functional Gastrointestinal Disorders
- Parkinson's Disease and Huntington's Disease
- Alpha-1 Antitrypsin Deficiency*
- Non-tuberculous Mycobacterial Lung Infections

CY2016
- Psoriasis
- Neuropathic Pain Associated with Peripheral Neuropathy
- Patients Who Have Received an Organ Transplant

CY2017
- Sarcopenia
- Autism
- Alopecia Areata
- Hereditary Angioedema*

CY2018
- Opioid Use Disorder
- Chronic Pain

CY2019
- None

CY2020
- Systemic Sclerosis
- Stimulant Use Disorder

CY2021
- Vitiligo

** Meetings conducted by FDA's Center for Biologics Evaluation and Research*

Externally-led PFDD: The Opportunity

- FDA announced the **opportunity for Externally-led PFDD** meetings in **December 2015.**
- Since then, more than **30 externally-led PFDD meetings** have been hosted by patient organizations following the process outlined on FDA's Externally-led PFDD webpage

- **Considerations:**
  - Disease area that is **chronic, symptomatic**, or affects functioning and activities of daily living;
  - Disease area for which aspects of the disease are **not formally captured in clinical trials**;
  - Disease area for which there are **currently no therapies** or very few therapies, or the available therapies do not directly affect how a patient feels, functions, or survives;
  - Disease areas that have a **severe impact on identifiable subpopulations** (such as children or the elderly)

Meetings **Strengthen Understanding** of Disease and Treatment Burden

Patient input from meetings can support FDA staff:

- In conducting benefit-risk assessments for products under review, by informing the therapeutic context
- Advising drug sponsors on their development programs

It might also support drug development more broadly:

- Identify areas of unmet need in the patient population
- Identify or develop tools that assess benefit of potential therapies
- Raise awareness and channel engagement within the patient community

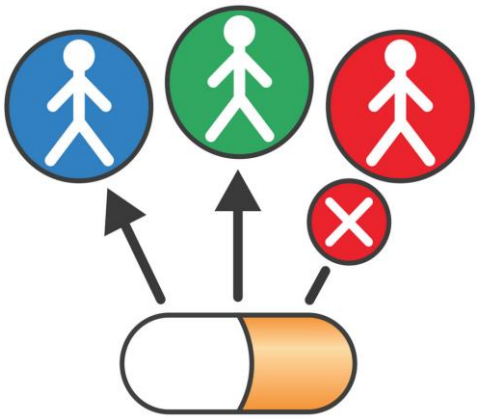
Meeting summary reports capturing patient experience data may be shared on FDA's website:

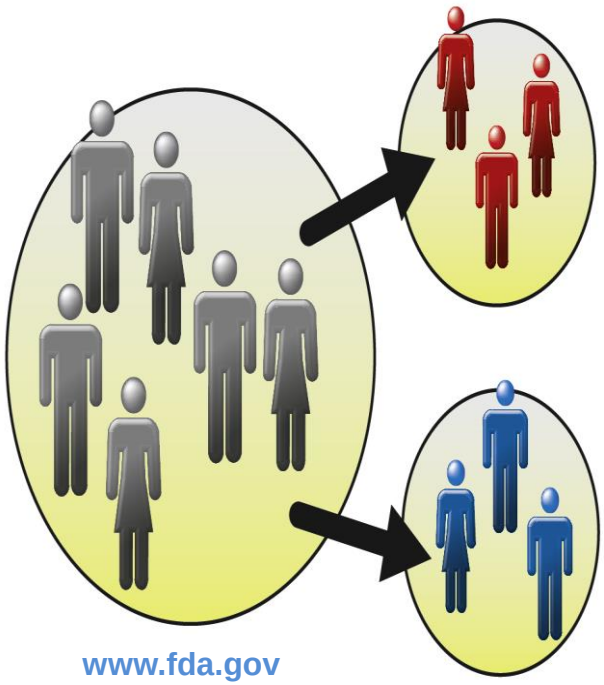
- FDA's *External Resources or Information Related to Patients' Experience* webpage provides links to certain publicly available external reports and resources

Why Patient Inputs are Valued

- Identify what matters/what is important to patients
- Benefit in development of clinical trials that are meaningful and realistic
- Raise FDA's Awareness

The Value Patient Engagement Adds





- Patient input can direct drug development in many ways:
  - helps with the understanding of diseases and their impact
  - helps identification of specific symptoms that are significant to patients
- Helps design better clinical trials

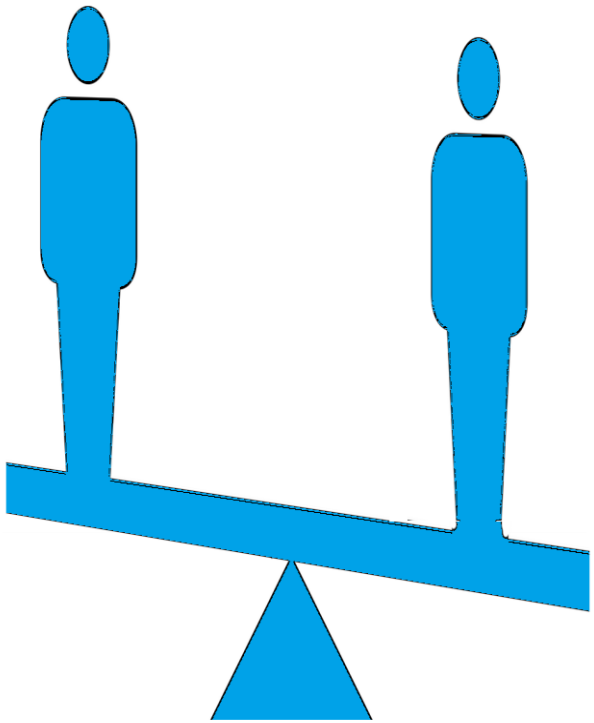
We want to hear from you…

Transparency, The Law, and Confidentiality
(What we can't do or say)

THE FDA CODE OF FEDERAL REGULATIONS

- FDA Code of Federal Regulations (CFR) is a huge sea of regulations that the FDA has created for regulating all products that come under its purview of regulation. The FDA codes of federal regulations are numbered and cover all products, processes and the activities that go into their creation.

Bias, Fairness, and Consistency

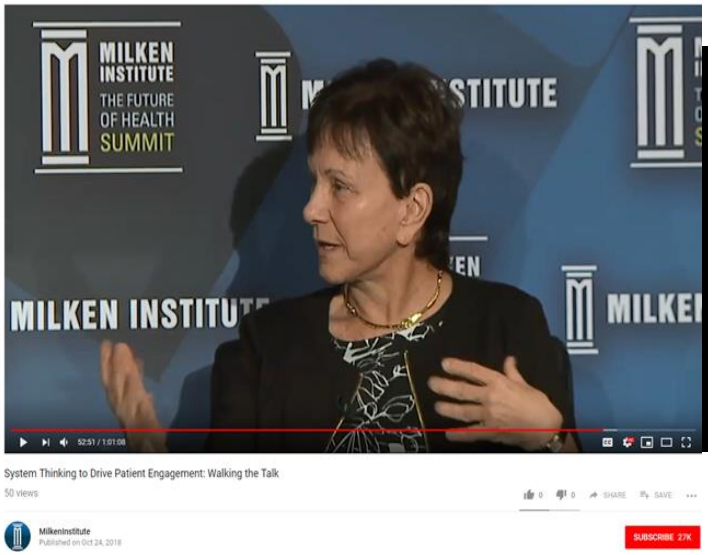


- Avoid bias to one company over another
- Focus on the specific scientific facts presented
- Meetings are granted free of bias
- Fairness, and consistency
- Open dialogue with patients and industry
- Points of view connected with sponsor support (financial for example) may have less credibility

Patient Inputs are always considered, But….We Can't Always Follow Them

- Statute
- Differences of opinion on interpretation of underlying facts
- Differences in views on practicality
- Conflict with laws or regulations creating legal risk
- Inconsistency with policy position or previous decisions
- Evolution of underlying data

Understanding the Patient's Perspective



PASE

- Conduit into the Center for stakeholders' concerns, viewpoints, and ideas
- Enhances stakeholders' awareness of the Center's current thinking
- Promotes collaborative actions regarding issues of mutual concern

"…FDA is working on developing a core set of measures…not just generally what's measured in lab values or hospital events, but ***actually understand patient experience on this drug*** *and how much disease is alleviated from their point of view…"*- Janet Woodcock

Future of Health Summit 2018
System Thinking to Drive Patient Engagement: Walking the Talk
https://www.milkeninstitute.org/videos/view/system-thinking-to-drive-patient-engagement-walking-the-talk?BackURL=%2Fvideos%2F

Example of Engagement with Depression and Bipolar Support Alliance (DBSA) DBSA Campaign Overview

Identify Unmet Need	Utilize Resources	Meaningful Output
• Current clinical trial endpoints focus on symptom control • Patients report of what is important to them-improvement in domains that support functionality	• Requested a meeting with CDER • PASE facilitated a Listening session with CDER's review division and DBSA	• Scientific Workshop: Convened all the stakeholders to explore patient defined wellness • Externally-led PFDD Meeting: format for patients to share what outcomes are important to them

Efforts to Capture the Patient's Voice

Public Workshops:

- Roadmap for Engaging with FDA's CDER: To help public and patient advocacy groups gain understanding of how to effectively engage with CDER
- Navigating CDER: To help public and patient advocacy groups gain understanding of how to engage with CDER
- Diabetes Outcome Measures: Forum for dialog on outcomes of direct relevance to diabetes patients living with the disease
- Rare Diseases: Strategies, tools and best practices for effective advocacy in rare diseases drug development

www.fda.gov

PASE's COVID-19 Stakeholders

PASE supports CDER's COVID-19 response efforts by facilitating communication and strategic engagement with external stakeholder groups. Some representative examples of engagements conducted are included below.

External Stakeholders

Patient Advocacy Groups

LUPUS FOUNDATION OF AMERICA

ASTMH AMERICAN SOCIETY OF TROPICAL MEDICINE & HYGIENE

Arthritis Foundation®

HCP Professional Organizations

AMERICAN COLLEGE OF CHEST PHYSICIANS

Society of Critical Care Medicine The Intensive Care Professionals

AAAAI AMERICAN ACADEMY OF ALLERGY ASTHMA & IMMUNOLOGY

American College of Allergy, Asthma & Immunology

Chain pharmacies, PBMs, etc.

APhA

ashp pharmacists advancing healthcare

NCPA NATIONAL COMMUNITY PHARMACISTS ASSOCIATION

American Association of Colleges of Pharmacy AACP

NACDS NATIONAL ASSOCIATION OF CHAIN DRUG STORES

Resources

- Request meeting with CDER to share perspectives, ideas, concerns (PASE)
- CDERPASE@fda.hhs.gov
- Patient Focused Drug Development (PFDD) Meeting
- patientfocused@fda.hhs.gov
- For any question on Drugs contact CDER's Division of Drug Information
- DrugInfo@fda.hhs.gov

Thank you

Contact Info: CDERPASE@fda.hhs.gov or Sadhna.Khatri@fda.hhs.gov