Patient Engagement & Regenerative Medicine: An FDA CBER Workshop for Patient Advocates

Thursday, May 6, 2021, 11:00 a.m.–3:00 p.m.

Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration
Workshop Agenda

11:05 a.m.  How FDA Is Advancing Regenerative Medicine

11:35 a.m.  Panel Discussion: The Value of the Patient Perspective in Regenerative Medicine Therapy Development

12:45 p.m.  Lunch

1:15 p.m.   FDA Panel Discussion: How to Engage with FDA When Opportunities Arise

2:30 p.m.   Q&A/Closing Remarks
Virtual Meeting Considerations

- The meeting will be recorded and available online.
- Use the Q&A box to submit questions throughout the presentation.
- For any technical difficulties, please use the chat function.
Session 1: How FDA CBER Is Advancing Regenerative Medicine

Speaker:
Rachael Anatol, PhD
Deputy Director
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration
“The Food and Drug Administration is responsible for protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices and by ensuring the safety of our nation’s food supply, cosmetics, and products that emit radiation.”
Organizational Structure
Mission

Ensure the **safety, purity, potency, and effectiveness** of biological products including **vaccines, allergenics, blood and blood products, cells, tissues, and gene therapies** for the prevention, diagnosis, and treatment of human diseases, conditions, or injury.

Through CBER’s mission, the Center also seeks to protect the public against the threats of emerging infectious diseases and bioterrorism.
Organizational Structure: CBER
The Office of Tissues and Advanced Therapies (OTAT) promotes the public health through collaborative, science-based regulation of medical products. This includes facilitating drug development and ensuring safety of individuals. OTAT’s regulatory decisions are data-driven, impartial, and compassionate.
Diversity of OTAT-Regulated Products

Gene Therapies (GT)

- Ex vivo genetically modified cells
- Non-viral vectors (e.g., plasmids)
- Replication-deficient viral vectors (e.g., adenovirus, adeno-associated virus, lentivirus)
- Replication-competent viral vectors (e.g., measles, adenovirus, vaccinia)
- Microbial vectors (e.g., Listeria, Salmonella)

Stem Cells/Stem Cell Derived

- Adult (e.g., hematopoietic, neural, cardiac, adipose, mesenchymal)
- Perinatal (e.g., placental, umbilical cord blood)
- Fetal (e.g., neural)
- Embryonic
- Induced pluripotent stem cells (iPSCs)
Diversity of OTAT-Regulated Products

Products for Xenotransplantation

- **Functionally mature/differentiated cells** (e.g., retinal pigment epithelial cells, pancreatic islets, chondrocytes, keratinocytes)
- **Therapeutic vaccines and other antigen-specific active immunotherapies**
- **Combination products**
  - Engineered tissues/organs
- **Devices**
- **Tissues**

- **Blood- and plasma-derived products**
  - Coagulation factors
  - Fibrin sealants
  - Fibrinogen
  - Thrombin
  - Plasminogen
  - Immune globulins
  - Antitoxins
  - Venom antisera for scorpions, snakes, and spiders
Regenerative Medicine Therapies (RMTs)

- OTAT regulates regenerative medicine therapies (RMTs).
- RMTs were defined in the 21st Century Cures Act: Title III, Section 3033, signed into law in 2016.
- RMTs include:
  - Cell therapies
  - Therapeutic tissue engineering products
  - Human cell and tissue products
  - FDA interpretation of Section 3033 of the 21st Century Cures Act adds: “Gene therapies, including genetically modified cells, that lead to a sustained effect on cells or tissues”
Example of RMT Products

Cellular therapy
Courtesy of NIH Image Gallery

iPSCs regenerated neurons
Courtesy of NIH Image Gallery

IPSCs regenerated muscle cells
Courtesy of NIH Image Gallery

Stem cells engineered to grow cartilage
Courtesy of NIH Image Gallery
Definition

**Autologous, allogeneic, or xenogeneic cells** that have been **propagated, expanded, selected, pharmacologically treated, or otherwise altered** in biological characteristics ex vivo to be administered to humans and applicable to the prevention, treatment, cure, diagnosis, or mitigation of disease or injuries.

**Examples**
- Stem cells and stem cell-derived products
- Pancreatic islets
- Chondrocytes
Tissue-Engineered Products

- FDA does not have a legal or regulatory definition for tissue-engineered products.
- Tissue-engineered products seek to restore, maintain, improve or replace damaged tissues and organs through the combination of scaffolds, cells and/or biologically active molecules.

Source: https://doi.org/10.1007/978-981-10-3701-6_14
Human Gene Therapy Products

- Human gene therapy products mediate their effects by transcription or translation of transferred genetic material or by specifically altering host (human) genetic sequences.

- Common gene therapy products:
  - Plasmids
  - Viral vectors
  - Bacterial vectors
  - Ex vivo genetically modified cells
  - Products incorporating genome editing
Gene Therapy – Ex Vivo and In Vivo Administration

**Ex vivo**

1. Extract cells (BM, PBMCs)
2. Use vectors to genetically modify cells
3. Introduce modified cells back to patient

**In vivo**

Direct delivery to patient using viral or non viral vector
Approved Cellular and Gene Therapy Products

- Abecma (idecabtagene vicleucel)
- Breyanzi (lisocabtagene maraleucel)
- Gintuit (allogeneic cultured keratinocytes and fibroblasts in bovine collagen)
- HPC (hematopoietic progenitor cells), Cord Blood
- Imlygic (talimogene laherparepvec)
- Kymriah (tisagenlecleucel)
- Laviv (azficel-T)
- Luxturna (voretigene neparvovec-rzyl)
- Maci (autologous cultured chondrocytes on a porcine collagen membrane)
- Provenge (sipuleucel-T)
- Tecartus (brexucabtagene autoleucel)
- Yescarta (axicabtagene ciloleucel)
- Zolgensma (onasemnogene abeparvovec)
Product Development Overview

- Preclinical Development
- Preclinical
- Phase 1
- Phase 2
- Phase 3
- BLA
- Marketing Application
- Post-marketing

IND submission
Conventional Clinical Development

Phase 1: Exploratory
- Designed to evaluate safety and side effects

Phase 2: Confirmatory
- Expanded safety; evaluates efficacy

Phase 3: Confirmatory
- Emphasis on efficacy, additional information on safety; expanded study
FDA’s Role in Regulating RMTs

- Regulate products over their entire lifecycle – during development and after approval
- Provide oversight of clinical trials to protect patient safety and patient rights
- Advance development by:
  - Publishing policy and guidance documents
  - Providing advice and education to product manufacturers
- Engage stakeholders to facilitate development of innovative products that meet patient needs
OTAT Patient Engagement

Goal: Learn directly from patients

- Impact of the disease and its treatment
  - Chief complaints (e.g., most bothersome signs/symptoms)
  - Burden of living with and managing a disease or condition
  - Impacts on activities of daily living and functioning

- Clinical trial considerations
  - Burden of participating in clinical studies
  - Risk tolerance

- Perspectives about current and potential treatments
  - Expectations of benefits
  - Tolerance for harms or risk
  - Preferences
  - Unmet medical needs
How Does OTAT Receive Patient Input?

- Advisory Committee Meetings
- Special Government Employee Consultants
- Public Meetings and Workshops
- Patient-Focused Drug Development Meetings
- FDA/NORD Rare Disease Listening Sessions
- Meetings with Patient Organizations
Enhancing Collaboration

- Encourage sponsors to invite patients/advocates to their formal meetings with OTAT
- Encourage patient groups to take on translational science activities
  - Approach FDA with ideas – CPIMs, DDT qualification, NH studies, etc.
- Encourage patient groups to work together
- Begin collaboration early
Contact Information

Regulatory Questions

- OTAT Main Line: 240-402-8190
- Email: OTATRPMS@fda.hhs.gov and Lori.Tull@fda.hhs.gov

Resources

- CBER website: [www.fda.gov/BiologicsBloodVaccines/default.htm](http://www.fda.gov/BiologicsBloodVaccines/default.htm)
- Phone: 1-800-835-4709 or 240-402-8010
- Consumer Affairs Branch: ocod@fda.hhs.gov
- Manufacturers Assistance and Technical Training Branch: industry.biologics@fda.hhs.gov
- Follow us on Twitter: [https://www.twitter.com/fdacber](https://www.twitter.com/fdacber)
Thank You!

Rachael Anatol, Ph.D.
Rachael.Strong@fda.hhs.gov
Session 2: The Value of the Patient Perspective in Regenerative Medicine Therapy Development

Moderator:
Anne Rowzee, PhD
Associate Director for Policy
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration
Session 2 Panelists

- **Xavier Liogier d’Ardhuy**, PhD, Chief of Translational Science, Loulou Foundation
- **Lei Xu**, MD, PhD, Chief of General Medicine Branch 2, Division of Clinical Evaluation and Pharmacology/Toxicology, OTAT, CBER, FDA
- **Elizabeth Hart**, MD, Branch Chief, General Medicine 1, OTAT, CBER, FDA
- **Debbie Drell**, Director of Membership, National Organization for Rare Disorders
- **Rob Adler**, Patient
Case study 1: Loulou Foundation meeting on observational study design

Xavier Liogier d’Ardhuy, PhD, Chief of Translational Science of the Loulou Foundation
Care for CDD Patients: the Big Picture

1. **Rare**: 1 in 42,000 births but vastly underdiagnosed.
   - ICD-10-CM only available since October 2020

2. **CDD children are unique**: phenotype differs from any other neurogenetic or other disorder.

3. **Two main devastating problems**
   - Severe epilepsy
   - Global and profound developmental delays

4. **Multiple other related issues**
   - Gastrointestinal, musculoskeletal, vision, autonomic
Epilepsy Therapies in CDD

- **Standard anti-seizure drugs**
  - Limited efficacy
  - Often multidrug/high dose – toxic to cognitive processing speed, attention, language, motor tone and control, mood….

- **Diets** – ketogenic, modified Atkins

- **Neuromodulation** – vagus nerve stimulation

- **Corpus callosotomy** – little evidence of efficacy
To develop the field before developing a therapy

This is often the patient community...

... but also Foundations and Academics

Build a pipeline
Build trial readiness
Build the registries
Build the tools
Build the science
To develop the field before developing a therapy

Medical classifications
- E.g.: ICD-10-CM

FDA interactions
- Externally-led Patient-Focused Drug Development meeting
- **Critical Path Innovation Meeting (CPIM)**: project-specific, regulatory dialogue opportunity, Dec. 2020
  - Clinical Outcome Assessments in the early phase of development
  - Natural history study designs and implementation
- **CPIM follow-up call**: interaction with CBER officers, Feb. 2021
CPIM questions to the FDA

• **Question #1:**
  a) Does the FDA have advice on the proposed study design, including the sample size, the proposed age ranges, and the number and frequency of study measures?

• **Question #2:**
  a) Does FDA agree with the use of developmentally appropriate but not age-appropriate outcome measures in CDD?
  b) Is FDA aware of additional outcome measures for neurodevelopmental disorders that we should consider for inclusion into the observational study?

• **Question #3:**
  a) What advice does FDA have that might help enable this observational study to eventually be used as a potential external comparator arm?
  b) What is an acceptable way to record “adverse events” in an observational study to make it acceptable as a control in future trials?
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• Debbie Drell, Director of Membership, National Organization for Rare Disorders

• Rob Adler, Patient
Patient Engagement at Public Meetings: NORD Case

Debbie Drell
Director of Membership
National Organization for Rare Disorders
FDA Patient Listening Sessions: Diverse, Powerful, Emotional

- Organized jointly by NORD and FDA
- Listening sessions are private, non-recorded
- Safe spaces to share
- Finding the right combination of patient and caregivers is critical to success: diversity of perspectives is invaluable
- How does FDA find participants and prepare them for the meeting?
- The GSD1A L.S. received the largest amount of speaker interest – 75!
As the pandemic drags on, trust in government — at all levels — falls
Percentage of Americans who trust in federal/state a great deal or fair amount to look out for the best interests of them and their families

Source: Axios/Ipsos Coronavirus Index, each wave includes ~1,000 U.S. Respondents 18+
Rare Disease Communities

7000 Rare Diseases

Nonprofits 17%

No Nonprofit 83%

Multiple in the same space
Enter NORD: What Does It Mean to Be 40 Years Old?

Switzerland * Sherlock Holmes * Shepherd

Convener * Detective * Sensitive Entry Point
Expand Your Reach = Diversity and Inclusion

NORD
National Organization for Rare Disorders

Members
Social Media

Members
Community

Physicians

Patients

rarediseases.org
Selecting and preparing participants to speak effectively at FDA events
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Session 2: The Value of the Patient Perspective in Regenerative Medicine Therapy Development

Q&A Discussion

Moderator:
Anne Rowzee, PhD
Session 3: FDA Panel Discussion on How to Engage with FDA When Opportunities Arise

Moderator:
Karen Jackler, MPH
Patient Engagement Program Manager
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration
Session 3 Panelists

- Andrea Furia-Helms, Director of the Office of Patient Affairs, Office of Clinical Policy and Programs, Office of the Commissioner, FDA
- Sadhna Khatri, PharmD, MPH, Associate Director, Professional Affairs and Stakeholder Engagement, Office of the Center Director, Center for Drug Evaluation and Research, FDA
- Michelle Tarver, MD, PhD, Deputy Director, Office of Strategic Partnerships and Technology Innovation, Program Director for Patient Science, Digital Health Center of Excellence, Center for Devices and Radiological Health, FDA
- Rea Blakey, Associate Director, External Outreach and Engagement, Oncology Center of Excellence, FDA
How Patient Affairs Involves Patients and Advocates

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

Patient Engagement & Regenerative Medicine: An FDA CBER Workshop for Patient Advocates
May 6, 2021
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!
<table>
<thead>
<tr>
<th>Year</th>
<th>Event</th>
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<tbody>
<tr>
<td>1988</td>
<td>Office of AIDS Coordination established</td>
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<tr>
<td>1993</td>
<td>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</td>
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<td>1996</td>
<td>First FDA Patient Representative® served on an advisory committee</td>
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<tr>
<td>2001</td>
<td>FDA Patient Representatives® received voting rights on advisory committees</td>
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<td>2008</td>
<td>Patients and consumers encouraged to report medical product problems using FDA’s existing MedWatch system</td>
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<td>2012</td>
<td>A section of the FDA website is created specifically for patients</td>
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<td>2013</td>
<td>Internal working group examines ways to increase patient involvement in FDA processes</td>
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<tr>
<td>2015</td>
<td>Patient Preference Information (PPI) framework and guidance for medical device decision making</td>
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<td>2016</td>
<td>FDA and European Medicines Agency (EMA) Patient Engagement Cluster created</td>
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<td>2017</td>
<td>First Patient Council (internal) meeting held</td>
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<td>2018</td>
<td>Memorandum of Understanding with National Organization For Rare Disorders (NORD) launched the FDA Patient Listening Session pilot program</td>
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<td>2019</td>
<td>Patient Affairs Staff (PAS) online webform, Patients Ask FDA</td>
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<tr>
<td>2020</td>
<td>COVID-19 Patient Resources Page Launched</td>
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<tr>
<td>2021</td>
<td>Office of Patient Affairs name changed from Patient Affairs Staff</td>
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Patient Affairs

Who we are

• 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

What we do

• 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 Programs & Initiatives

Cross-center patient activities
Patient Affairs Programs and Activities

- Patient Listening Sessions
- Patient Engagement Collaborative
- Enhancing Communications
Patient Listening Sessions

Rare Diseases

- Memorandum of Understanding with the National Organization for Rare Disorders (NORD)
- Supported by the Reagan Udall Foundation for FDA
- 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
Informing the Regulatory Process

Examples of issues that may be discussed during a Patient Listening Session

1. Patient Experience
   - Symptoms and aspects of a disease that patients and caregivers consider are most important to address
   - Disease burden and symptom progression
   - Activities/functions that are most important to preserve or restore
   - Experience with how current treatment regimens are working to manage symptoms

2. Treatment Options

3. Clinical Trials
Informing the Regulatory Process

Examples of issues that may be discussed during a Patient Listening Session

1. Patient Experience
   - Perspectives about priorities for potential treatments for a disease
   - Expectations of results from a potential treatment
   - Meaningful outcomes from new therapeutic interventions
   - Consideration of a potential medical product that decreased the severity of symptom(s), rather than completely removing or resolving it
   - Willingness to continue an investigational medical product before a patient started to feel relief from symptoms of a disease

2. Treatment Options

3. Clinical Trials
Informing the Regulatory Process

Examples of issues that may be discussed during a Patient Listening Session

1. Patient Experience

2. Treatment Options

3. Clinical Trials

- Considerations about participating in a clinical trial for an investigational therapy
- Barriers to participating in a clinical trials and natural history studies
- Understanding the benefit-risk trade-offs associated with severe or life-threatening side effects
- Designing clinical studies to be better tailored for patients and caregivers
- Differences in perceptions between an investigational drug vs. a gene therapy
- Determining meaningful clinical endpoints
• 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
Resources

Tools and resources for engagement
Patient Engagement Across FDA

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

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

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

Center for Biologics

Center for Drugs

Office of the Commissioner

Center for Devices

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

Office of Communication, Outreach and Development: OCOD@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
Questions & Meeting Requests

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.

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
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

¹https://www.fda.gov/drugs/development-approval-process-drugs/patient-focused-drug-development-glossary
Patients are experts on 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 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

CY 2013
- Chronic Fatigue Syndrome/Myalgic Encephalomyelitis
- HIV
- Lung Cancer
- Narcolepsy

CY 2014
- 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

CY 2015
- Breast Cancer
- Chagas Disease
- Functional Gastrointestinal Disorders
- Parkinson’s Disease and Huntington’s Disease
- Alpha-1 Antitrypsin Deficiency*
- Mycobacterial Lung Infections

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

CY 2016
- Psoriasis
- Neuropathic Pain Associated with Peripheral Neuropathy
- Patients Who Have Received an Organ Transplant

CY 2017
- Sarcopenia
- Autism
- Alopecia Areata
- Hereditary Angioedema

CY 2018
- Opioid Use Disorder
- Chronic Pain

CY 2019
- None

CY 2020
- Systemic Sclerosis
- Stimulant Use Disorder

CY 2021
- Vitiligo
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 or 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)
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
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.youtube.com/watch?v=rhypFNp4zwM&list=PLwJK8JzK8C_eS7zY1eq6CKx2Yz_8fwgBs&index=21
Example of Engagement with Depression and Bipolar Support Alliance (DBSA) Campaign Overview

**Identify Unmet Need**
- Current clinical trial endpoints focus on symptom control
- Patients report of what is important to them—improvement in domains that support functionality

**Utilize Resources**
- Requested a meeting with CDER
- PASE facilitated a Listening session with CDER’s review division and DBSA

**Meaningful Output**
- 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.
Resources

Request meeting with CDER to share perspectives, ideas, concerns (PASE)

CDERPASE@fda.hhs.gov

Patient Focused Drug Development (PFDD) Meeting

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

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
How Can Patients Engage with the FDA Center for Devices and Radiological Health (CDRH)

Michelle Tarver, MD, PhD
Deputy Director, Office of Strategic Partnerships and Technology Innovation
Program Director for Patient Science, Digital Health Center of Excellence
Center for Devices and Radiological Health
Patient Input & Engagement Useful Across Total Product Lifecycle (TPLC)
Regulatory Impact of PPI & PROs

24 Industry-sponsored regulatory PPI studies completed or in pipeline

Over 50% of PMAs, HDEs, and de Novos have PROs
Inspired by Patients, Driven by Science

CULTURE OF PATIENT ENGAGEMENT
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.
Patient & Caregiver Connection: Current Partners

<table>
<thead>
<tr>
<th>ICAN</th>
<th>Global Healthy Living Foundation</th>
<th>Spina Bifida Association</th>
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<tr>
<td>FORCE</td>
<td>GHLF creakyjoints</td>
<td>Faces &amp; Voices of Recovery</td>
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<td>TMJA</td>
<td>JDRF</td>
<td>COPD Foundation</td>
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<tr>
<td>National Leiomyosarcoma Foundation</td>
<td>AAKP</td>
<td>North American Spinal Cord Injury Consortium</td>
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<td>American Sleep Apnea Association</td>
<td>MDA</td>
<td>The Michael J. Fox Foundation</td>
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www.fda.gov
Gaining Insights from Patients on Their Disease and Devices Used to Treat It
Patients and a caregiver 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.
Impact of COVID-19 on 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.
Virtual CDRH Patient Engagement Townhall

FDA Welcomes Patients & Caregivers

Engaging Patients Through the Total Product Life Cycle of Digital Health Technology

SEPTEMBER 10, 2020
Patient Engagement Videos

FDA Devices: Our Commitment to Putting Patients First

www.fda.gov
CDRH Patient Engagement Advisory Committee (PEAC)

PEAC members are diverse patients, caregivers, and patient advocates

**GOAL:** To help ensure patients’ needs and experiences are considered in FDA’s work on medical devices and better understand and integrate patient perspectives into CDRH’s oversight

- 1-2 homework assignments per year
- 1-2 PEAC meetings per year
- Patient-focused and relevant recommendations
### Topics of PEAC Meetings

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<th>Topic</th>
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<td>1</td>
<td>Patient Engagement in Design, Conduct and Communication of Medical Device Clinical Trials</td>
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<tr>
<td>2</td>
<td>Patient-Generated Health Data &amp; Medical Device Safety Surveillance</td>
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<tr>
<td>3</td>
<td>Communicating Cybersecurity Vulnerabilities of Medical Devices</td>
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<tr>
<td>4</td>
<td>Artificial Intelligence &amp; Machine Learning</td>
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<tr>
<td>5</td>
<td><strong>STAY TUNED 2021</strong> Market Topic</td>
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</table>
Outcomes from PEAC 2017 on Clinical Trials

• Video of medical device clinical trials designed to encourage underrepresented populations to participate

https://www.youtube.com/watch?v=SApXnmZlgFE
CDRH Encourages Patient Engagement Through Draft Guidance


www.fda.gov
Outcomes from PEAC 2019 on Cybersecurity

National Cybersecurity Awareness Month

Video announcing launch of miniseries on cybersecurity hygiene
Inspired by Patients, Driven by Science

OPTIMIZING RESEARCH ROADMAP
Virtual Bootcamp:
Navigating the Journey from Digital Health Technologies to Meaningful Patient Outcomes

- 70-90 attendees each meeting from a range of small medical device and digital health companies as well as academia and patients
- Pre-recorded trainings on clinical outcome assessments, patient preference information, patient engagement, and digital health technologies (DHT)
- Virtual hands-on practice in small groups, based on two scenarios

Hands-on practice
- Practice 1: Patient Input to Support Your DHT Development
- Practice 2: Developing Your DHT to Be Used to Support Clinical Investigations
- Practice 3: Developing Your DHT to Be Used Outside of Clinical Investigations
Collaborative Patient Preference Studies
Addressing Preference-Sensitive Topics

Prostate Cancer  Chronic Pain  Adolescent Scoliosis  Heart Failure  End-Stage Kidney Disease

Uterine Fibroids
Breast Implants
Artificial Intelligence
Contraception
Peripheral Vascular Disease
FDA Patient-Centered Projects in Diverse Populations

- Patients (teens to seniors) with sleep apnea on positive airway pressure (PAP), collecting PPI and PGHD
- Stress urinary incontinence registry to collect PROs and mHealth
- Heart failure in pediatric patients PRO development
- PPI and PROs for mild to moderate glaucoma patients
- Heart failure in women and racial and ethnic groups qualitative interviews to augment PRO measure
- Virtual reality acceptability in under-represented populations qualitative evaluation
- Social media listening to understand how diverse populations talk about chronic pain
- Understanding under-represented populations attitudes towards COVID-19 diagnostic testing & providing data in registries
Other Research & Capacity Building Efforts

Science of Patient Input | Medical Device Collaboration | MDIC

Overview of Program:
CDRH Strategic Priority

COLLABORATIVE COMMUNITIES
What Is a Collaborative Community?

Collaborative communities are continuing forums where public- and private-sector members proactively work together to:

- Achieve common objectives and outcomes
- Solve shared challenges
- Leverage collective opportunities in an environment of trust, respect, empathy, and openness
Collaborative Communities with CDRH & Patient Participation

- Collaborative Community on Ophthalmic Imaging
- National Evaluation System for health Technology Coordinating Center (NESTcc) Collaborative Community
- International Liquid Biopsy Standardization Alliance (ILSA)
- Xavier Artificial Intelligence (AI) World Consortium
- Wound Care Collaborative Community

- Standardizing Laboratory Practices in Pharmacogenomics Initiative (STRIPE) Collaborative Community
- Case for Quality Collaborative Community
- Heart Valve Collaboratory (HVC)
- Pathology Innovation Collaborative Community (PICC)
- REducing SuiCide Rates Amongst IndividUals with DiabEtes (RESCUE) Collaborative Community
Patients & Medical Device Evaluation

Patient Engagement

Clinical Outcome Assessments

Patient Preference Information

Patient-Generated Health Data
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 Question CDRH_PatientEngagement@fda.hhs.gov
- For Collaborative Community Questions: CDRHCollabCommunities@fda.hhs.gov
Patient Engagement & Regenerative Medicine: An FDA CBER Workshop for Patient Advocates

May 6, 2021

Rea Blakey
Associate Director, External Outreach and Engagement

Oncology Center of Excellence
Oncology Center of Excellence

Center for Drug Evaluation and Research
  • CDER

Center for Biologics Evaluation and Research
  • CBER

Center for Devices and Radiological Health
  • CDRH
Create mutually beneficial and enduring relationships among communities, patients, patient/advocates, and OCE cancer product reviewers

- Facilitate access to cancer information for high-risk individuals, underserved/underrepresented populations, and the public

- Increase participation in clinical trials & improve the design of clinical trials

- Increase minority genomic database contributions to advance treatments

- Presenting National Black Family Cancer Awareness Week, June 17-23, 2021
Oncology Center of Excellence

Welcome!

“Straight Talk about Cancer, COVID-19 and Long-Hauler Syndrome”

Thursday, February 25, 2021
11 a.m. to Noon (CST)

Please stand by . . .
Our program will begin shortly.

U.S. Food and Drug Administration
Oncology Center of Excellence

For information regarding OCE’s
"PROJECT COMMUNITY: Empowering Patients Living with Cancer, Survivors, Advocates and Consumers," please visit:
https://www.fda.gov/about-fda/oncology-center-excellence/project-community
365 DAYS AND COUNTING: COVID’s Impact on the Oncology Community

Virtual Meeting - March 12th, 2021
Disparities in Cancer Prevention in the COVID-19 Era

John M. Carethers, Rajarshi Sengupta, Rea Blakey, Antoni Ribas, and Gypsyamber D'Souza

ABSTRACT

Screening for cancer is a proven and recommended approach to prevent deaths from cancer; screening can locate precursor lesions and/or cancer at early stages when it is potentially curable. Racial and ethnic minorities and other medically underserved populations exhibit lower uptake of cancer screening than nonminorities in the United States. The COVID-19 pandemic, which disproportionately affects minority communities, has curtailed preventive services including cancer screening to preserve personal protective equipment that was already behind becoming further behind as a result of the community ravaged from COVID-19. Fear of contracting COVID-19, limited access to safety-net clinics, and personal factors like financial, employment, and transportation issues are concerns that are intensified in medically underserved communities. Prolonged delays in cancer screening will increase cancer in the overall population from pre-COVID-19 trajectories, and elevate the cancer disparity in minority populations. Knowledge of the impact of the pandemic on cancer screening and continued surveillance of efforts to eliminate disparities is essential.

Disparities in Cancer Prevention in the COVID-19 Era

John M. Carethers, Rajarshi Sengupta, Rea Blakey, Antoni Ribas, and Gypsyamber D'Souza

Cancer Prev Res September 17 2020 DOI:10.1158/1940-6207.CAPR-20-0447
Breast Cancers + Colon Cancers = 1/6 Deaths

NCI
COVID’s Impact:

• The effect of COVID-19 on cancer screening and treatment for breast and colorectal cancer over the next decade suggests almost 10,000 excess deaths.

• That’s a ~1% increase in deaths from these tumor types during a period when we would expect to see almost 1,000,000 deaths from these two diseases types.
National Black Family Cancer Awareness Week: Engaging the Generations

#BlackFamCAN

“Conversation on Cancer”
National Panel Discussion
Thursday, June 17
2-3:30 p.m. ET

Social Media Campaign
Week June 17-23

Through 50th Anniversary
National Cancer Act of 1971
December 23, 2021
National Black Family Cancer Awareness Week: Engaging the Generations

Part of the "Conversation on Cancer"
Panel Discussion Series

Thursday, June 17
2-3:30 p.m. ET

Contact:
OCE-Engagement@fda.hhs.gov
National Black Family Cancer Awareness Week:
Engaging the Generations
June 17-23, 2021
#BLACKFAMCANCER
Enroll in cancer clinical trials
National Black Family Cancer Awareness Week: Engaging the Generations
June 17-23, 2021
#BLACKFAMCANCER
Discuss family cancer history
National Black Family Cancer Awareness Week: Engaging the Generations

June 17, 2021, at 2–3:30 p.m. ET
“Conversation on Cancer”
National Panel Discussion

June 17-23, 2021
Social Media Campaign
#BlackFamCAN

Contact: OCE-Engagement@fda.hhs.gov
www.fda.gov/OCE

OCE 2020 Annual Report

- Clinical Trial Guidance During COVID Meetings
  - Project Equity
  - Project Silver
  - Project Facilitate
  - Project Patient Voice
- Project Community/National Black Family Cancer Awareness Week
  - Conversations on Cancer public panel discussion series

✔ FDA.gov, search “OCE Stakeholder Meetings”
Session 3

CBER Patient Engagement Program and Cross-Center Collaboration

Karen Jackler, MPH
Patient Engagement Program Manager
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration
CBER patient engagement program and activities

Bringing it together
  - In-common mechanisms
  - Cross-center collaboration and information sharing

Examples

Other ways to engage with the FDA

My comments are an informal communication and represent my own best judgement. These comments do not bind or obligate FDA.
CBER Patient Engagement Activities

- CBER Patient Engagement Workgroup
- CBER Rare Disease Coordinating Committee
- Science of Patient Input Initiative
- Office of Communication, Outreach, and Development
How Can the Agency Receive Input?
In-Common Mechanisms

• Meetings with individual product centers
• Public meetings and workshops
• Citizen petition
• Docket comments via Regulations.gov
• During development as special government employee (SGE)
• Emails, letters, phone calls
Cross-Center Collaboration & Information Sharing

- NORD Listening Sessions
- Patient Engagement Collaborative

Patient Engagement Program

Patient-focused Drug Development (PFDD) PASE

PEAC Patient and Caregiver Connection

Project Community Conversations on Cancer
Cross-Center Collaboration & Information Sharing: Examples

- Listening sessions
  - Hunter syndrome/MPSII
  - Childhood cerebral adrenoleukodystrophy (CCALD)
  - Sanfilippo
  - GSD1
- CBER-planned PFDDs
  - Alpha-1 antitrypsin
  - Hemophilia and heritable bleeding disorders
  - Hereditary angioedema
- CDER/CBER technical advisors for Externally-led PFDD
- Invite cross-center attendance to center meetings with patient organizations
Beyond the PFDD or Listening Session: Other Ways to Engage with CBER

- Meeting reports
- Patient surveys/survey reports
- Natural history studies and patient registries
- White papers
- Case examples
Session 3: FDA Panel Discussion on How to Engage with FDA when Opportunities Arise

Q&A Discussion

Moderator:
Karen Jackler, MPH
Workshop Q&A

Tejashri Purohit-Sheth, MD, FACAAI, CQIA, CAPT, Medical, USPHS, Director of the Division of Clinical Evaluation & Pharmacology/Toxicology, OTAT, CBER, FDA

Sandra Retzky, DO, JD, MPH, Medical Officer, Division of Clinical Evaluation & Pharmacology/Toxicology, OTAT, CBER, FDA
Thank you for attending today’s event.

Stay in touch!

Follow CBER on Twitter (@FDACBER)

Visit our website: www.fda.gov/vaccines-blood-biologics