U.S. FOOD & DRUG ADMINISTRATION

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.

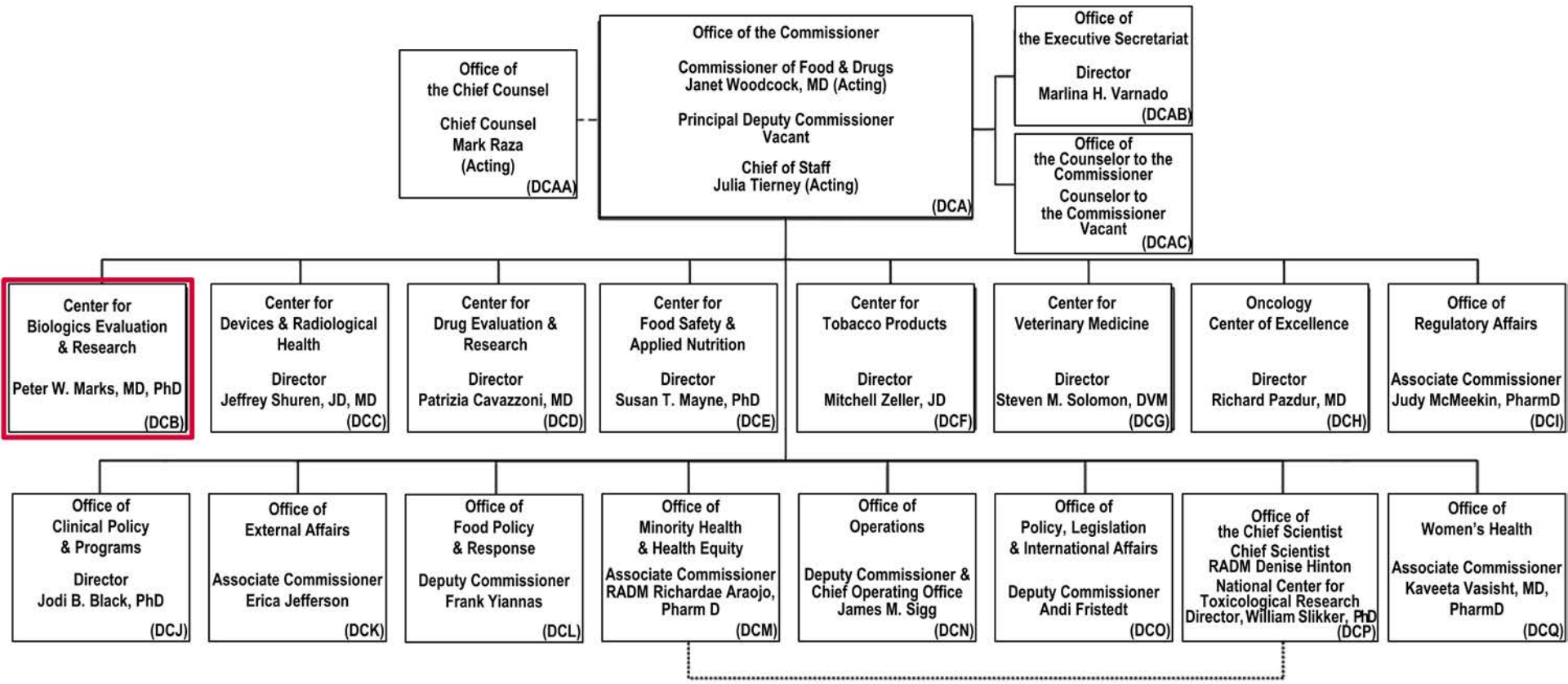
Session 1: How FDA CBER Is Advancing Regenerative Medicine

Speaker:
Rachael Anatol, PhD
Deputy Director
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

FDA Mission

"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



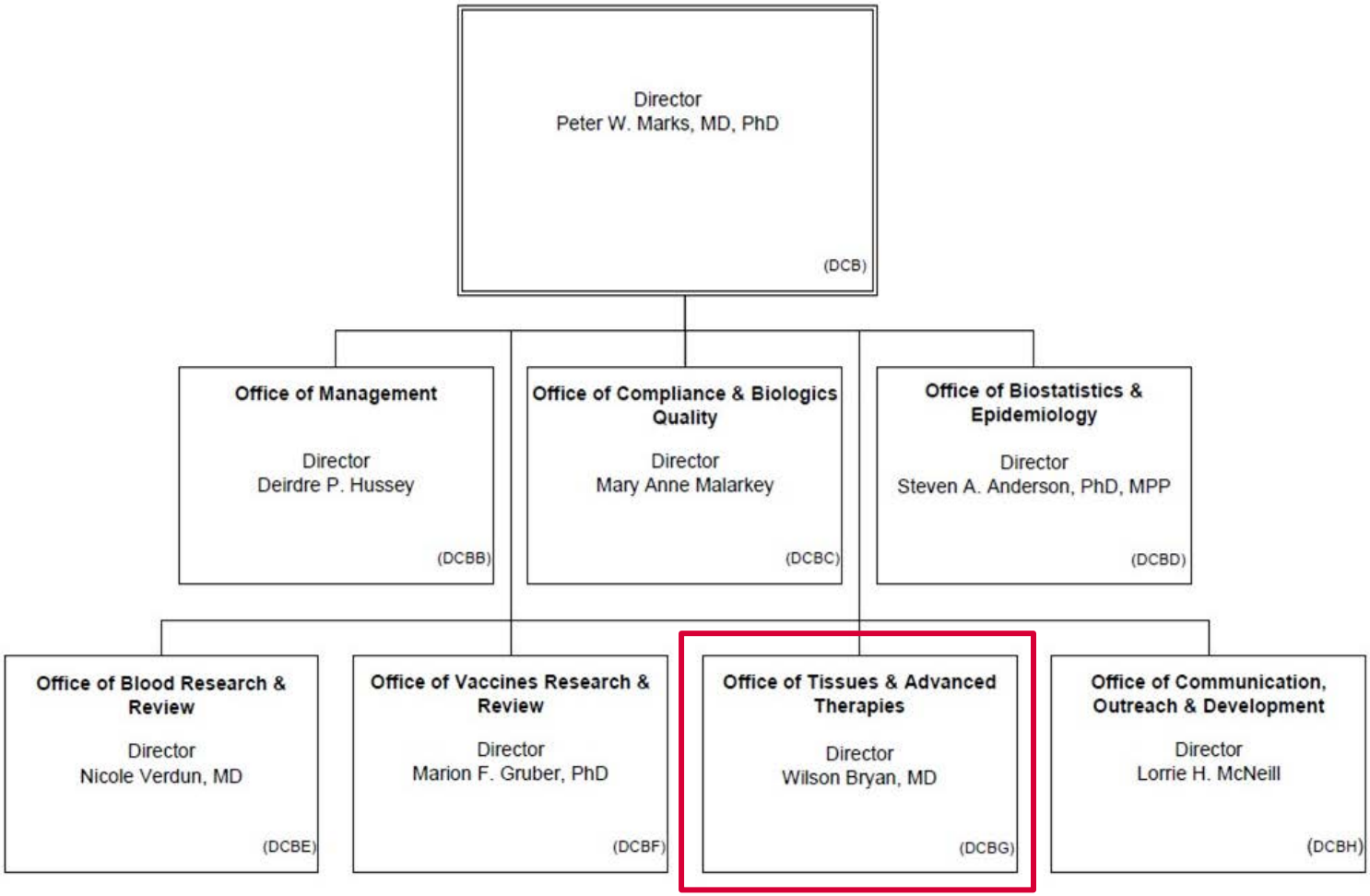
Center for Biologics Evaluation and Research (CBER)

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



OTAT Mission

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

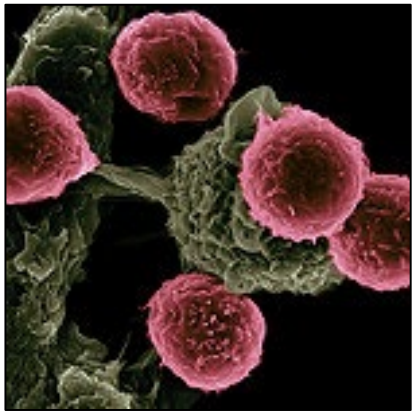
- **Functionally mature/differentiated cells** (e.g., retinal pigment epithelial cells, pancreatic islets, chondrocytes, keratinocytes)
- **Therapeutic vaccines and other antigen-specific active immunotherapies**
- **Combination products**
  - Engineered tissues/organs
- **Devices**
- **Tissues**

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

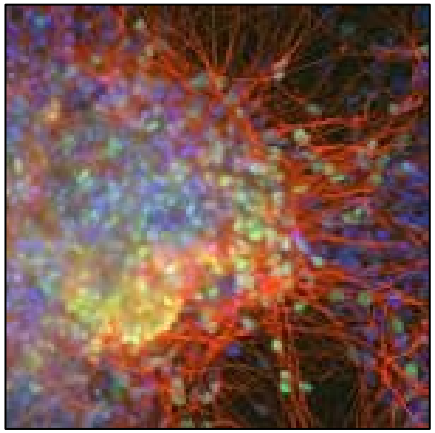
Regenerative Medicine Therapies (RMTs)

- 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”

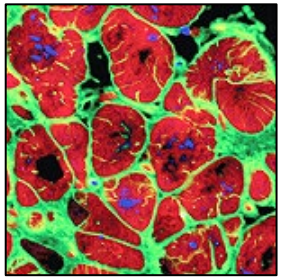
Example of RMT Products



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

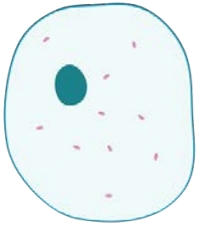
Cell Therapy Products

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.

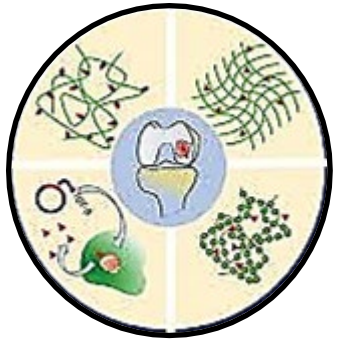
Examples

- 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.

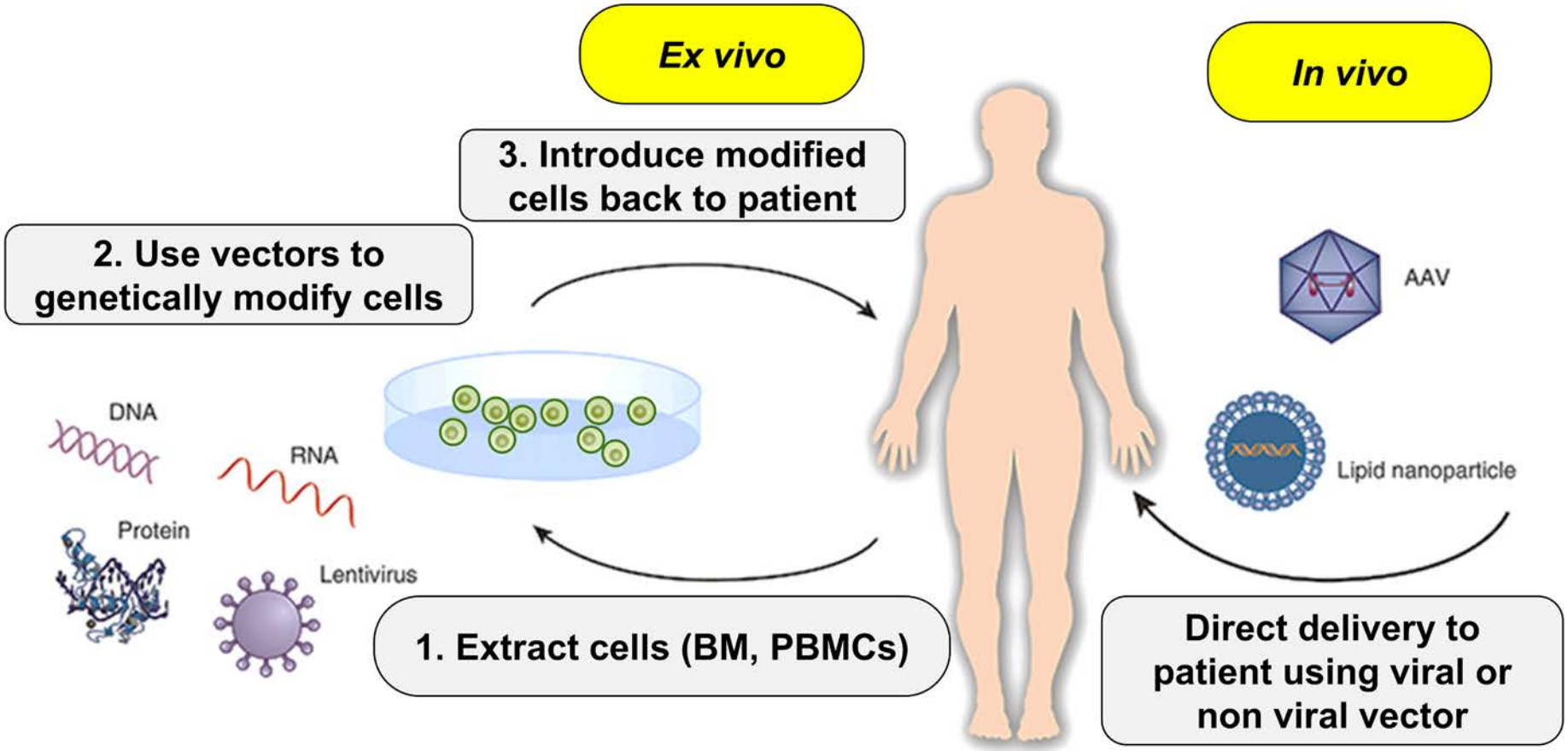


Source: https://doi.org/10.1007/978-981-10-3701-6_14

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

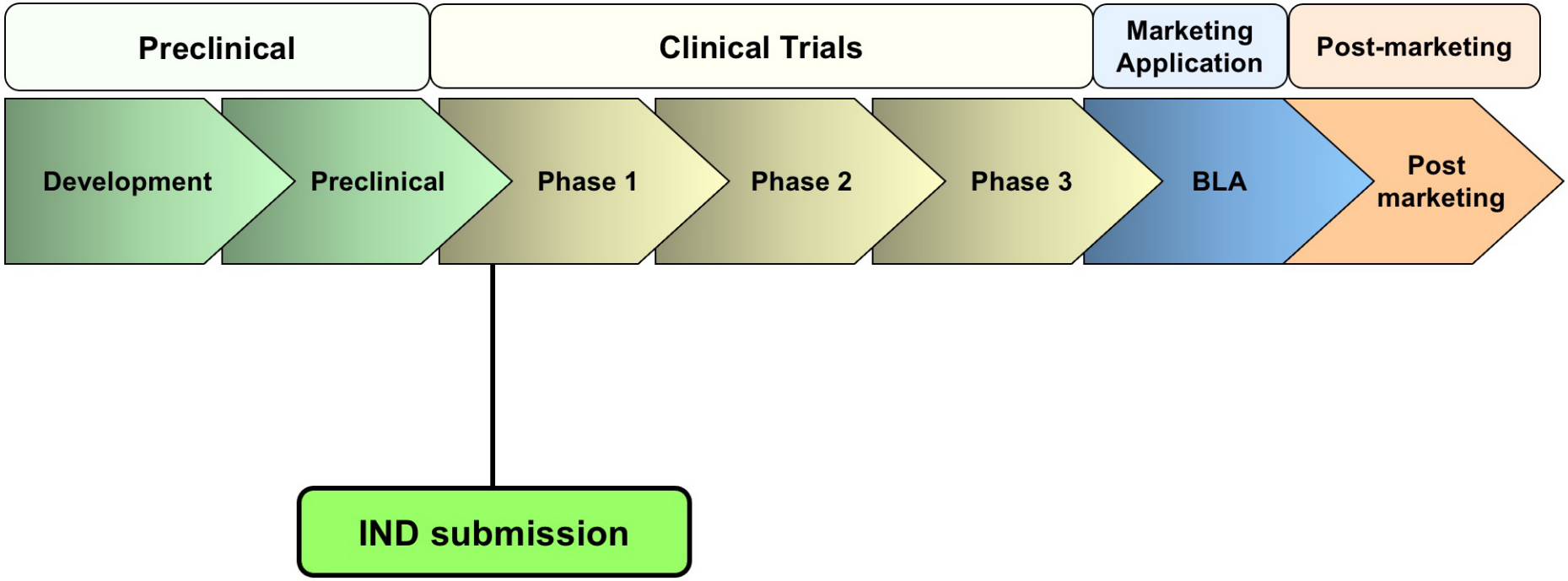
Gene Therapy – Ex Vivo and In Vivo Administration



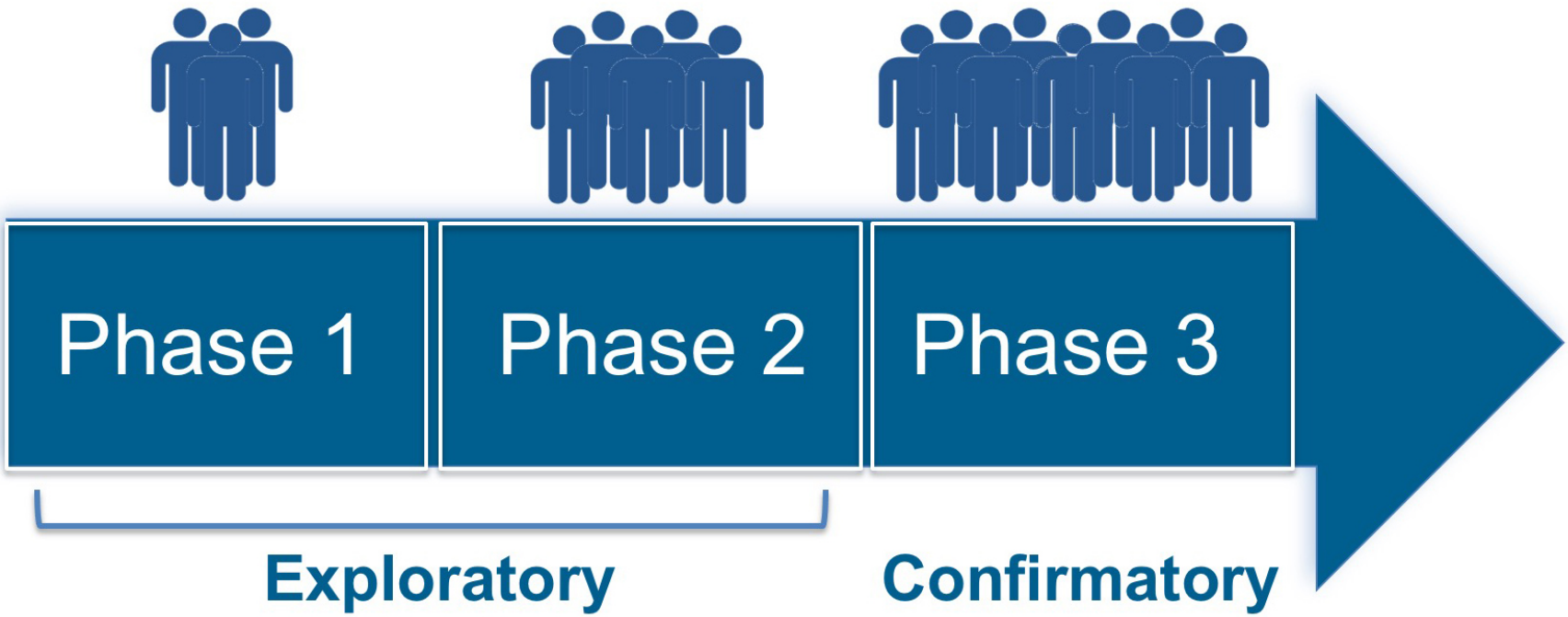
Approved Cellular and Gene Therapy Products

- **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



Conventional Clinical Development



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

- **OTAT Learn Webinar Series:** http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/ucm232821.htm
- **CBER website:** 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

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Thank You!

Rachael Anatol, Ph.D.
Rachael.Strong@fda.hhs.gov

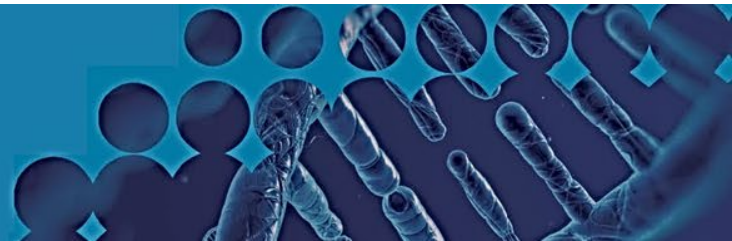
Session 2: The Value of the Patient Perspective in Regenerative Medicine Therapy Development

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

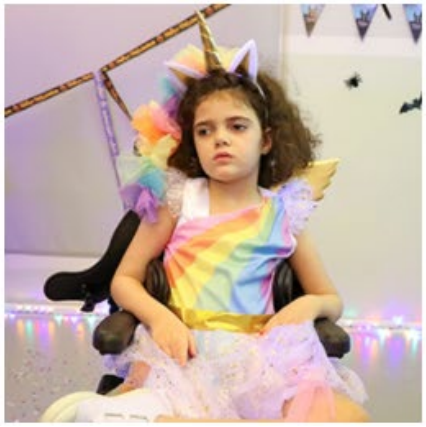


Case study 1: Loulou Foundation meeting on observational study design

Xavier Liogier d'Ardhuy, PhD, Chief of Translational Science of the Loulou Foundation

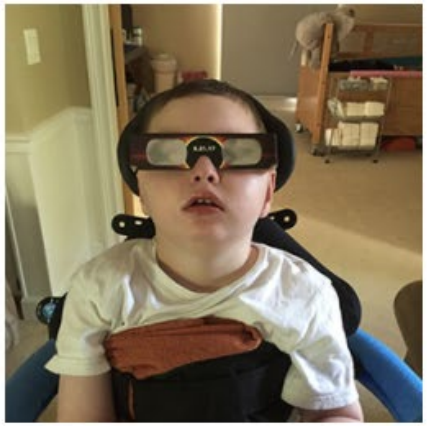
loulou FOUNDATION
Tackling CDKL5 Deficiency






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

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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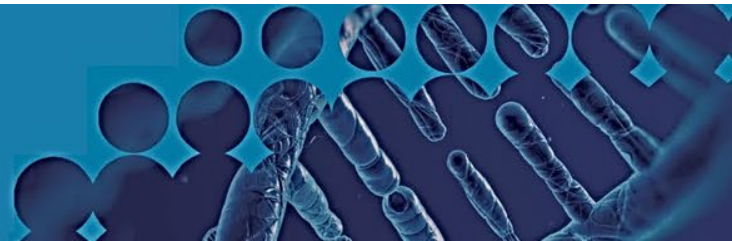

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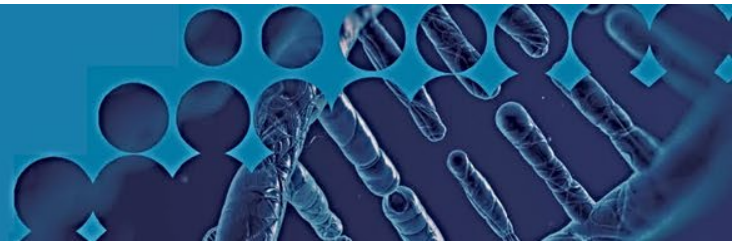


Patient Engagement at Public Meetings: NORD Case

Debbie Drell
Director of Membership
National Organization for Rare Disorders

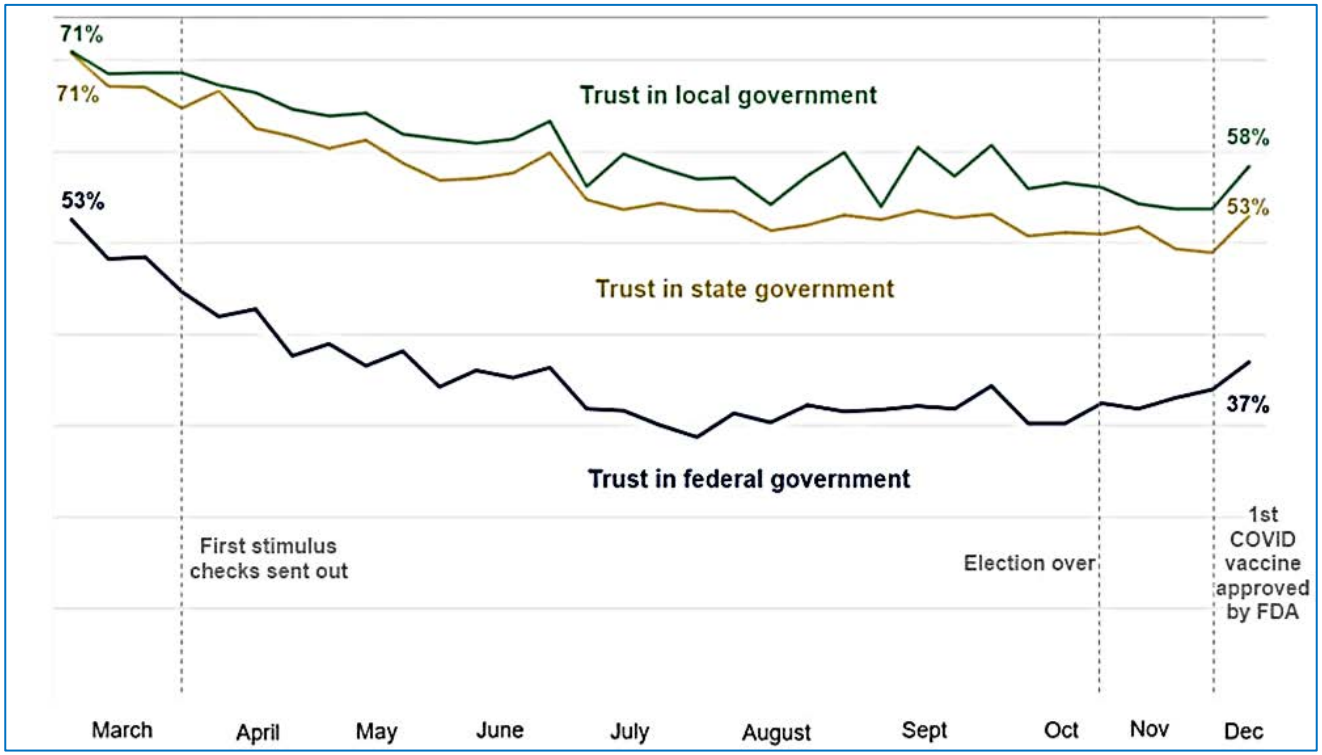
FDA Patient Listening Sessions: Diverse, Powerful, Emotional

- 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!

Trust in Government

As the pandemic drags on, trust in government — at all levels — falls

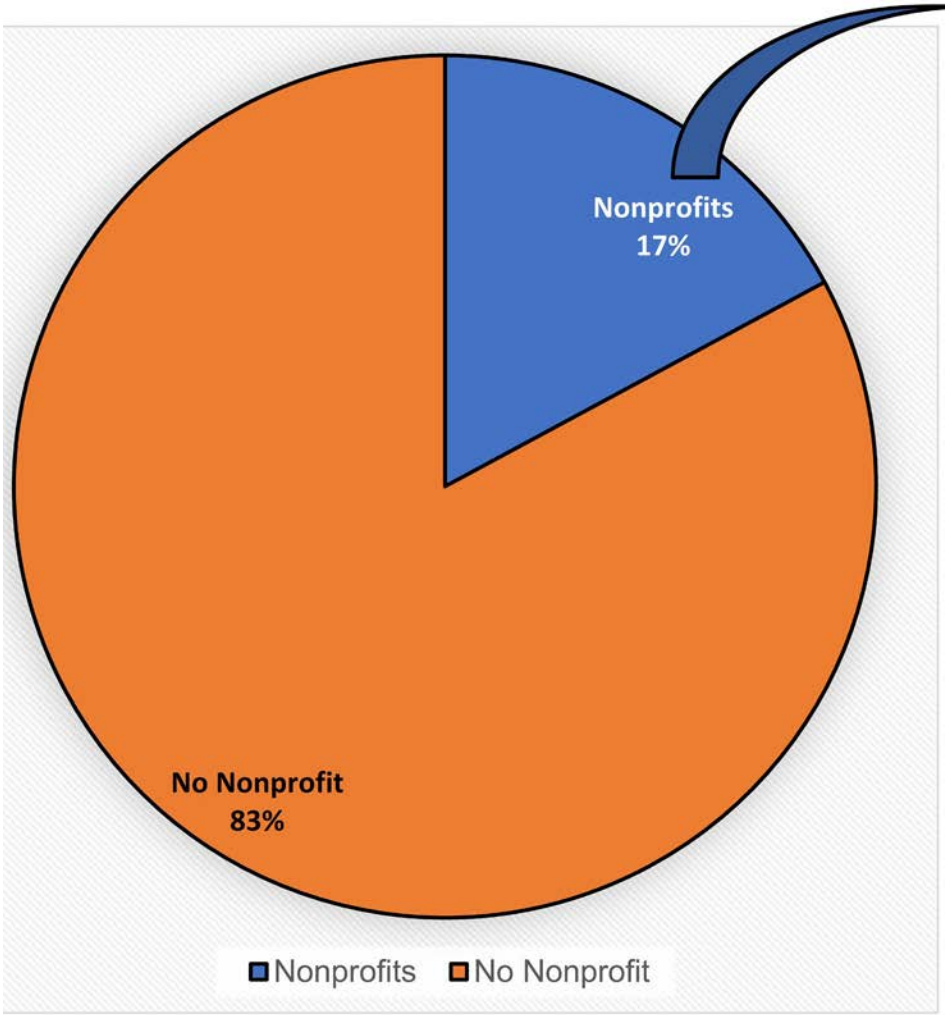
Percentage of Americans who trust in federal/state a great deal or fair amount to look out for the best interests of them and their families



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

Rare Disease Communities

7000 Rare Diseases



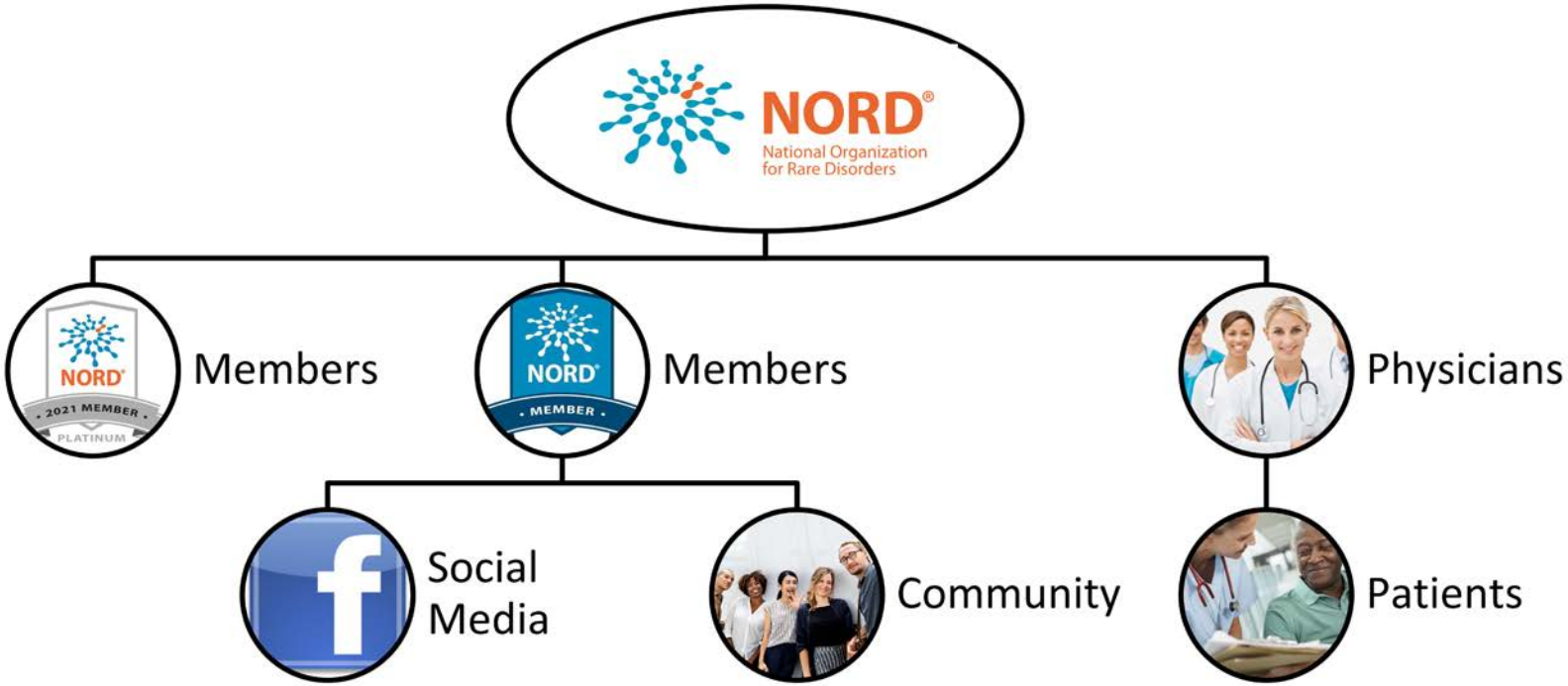
Multiple in the same space

Enter NORD: What Does It Mean to Be 40 Years Old?

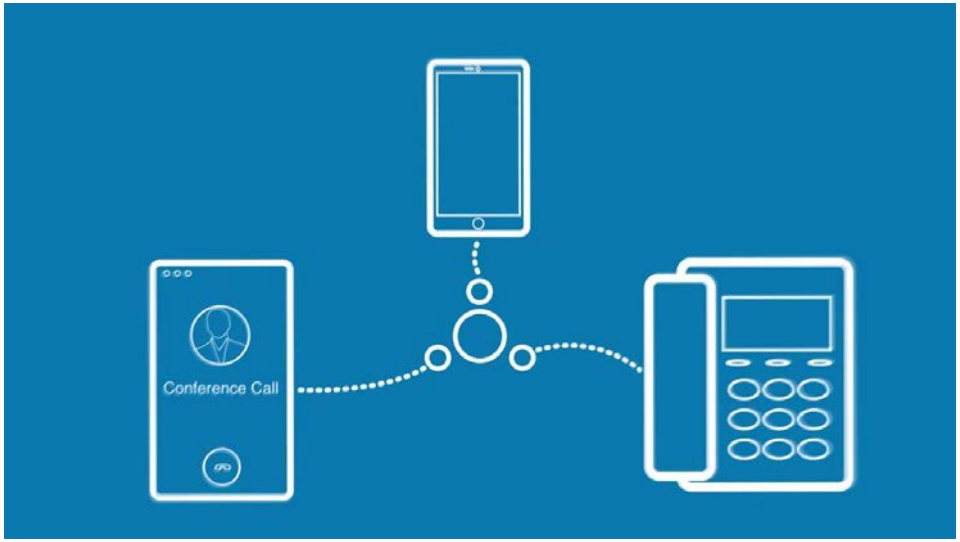
Switzerland * Sherlock Holmes * Shepherd

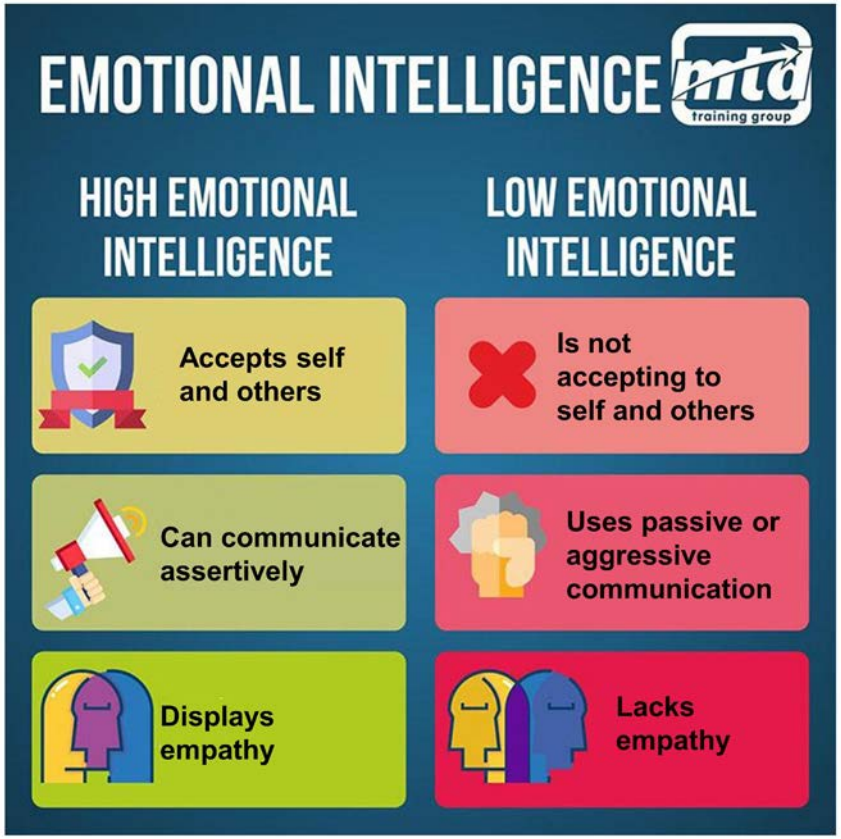
Convener * Detective * Sensitive Entry Point

Expand Your Reach = Diversity and Inclusion



Selecting and preparing participants to speak effectively at FDA events





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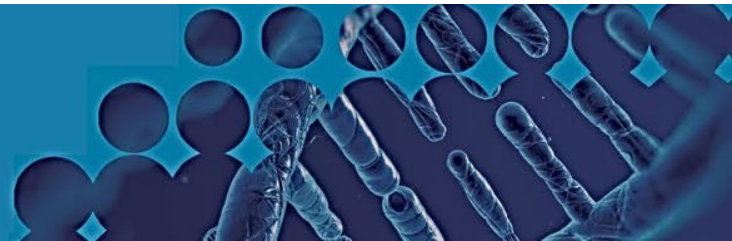

Session 2: The Value of the Patient Perspective in Regenerative Medicine Therapy Development

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

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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

1. Overview
2. Patient Affairs Programs & Activities
3. Resources

OUTLINE

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) announced in the Federal Register

2016

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

2017

- Office of Patient Affairs (formerly Patient Affairs Staff) established in the Office of the Commissioner
- Public Workshop on PFDD guidance
- Patient Engagement Advisory Committee (PEAC) meetings regarding medical devices

2018

- Memorandum of Understanding with National Organization For Rare Disorders (NORD) launched the FDA 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

- Patient Affairs Staff (PAS) online webform, Patients Ask FDA
- PFDD Workshop on Guidance 4
- Draft PFDD Guidance 2 released

2020

- COVID-19 Patient Resources Page Launched
- Final PFDD Guidance 1 released
- Muscular Dystrophy Association webinar on COVID-19
- FDA and NORD Listening Session on COVID-19 Impact on Rare Disease Communities

2021

- Office of Patient Affairs name changed from Patient Affairs Staff

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**

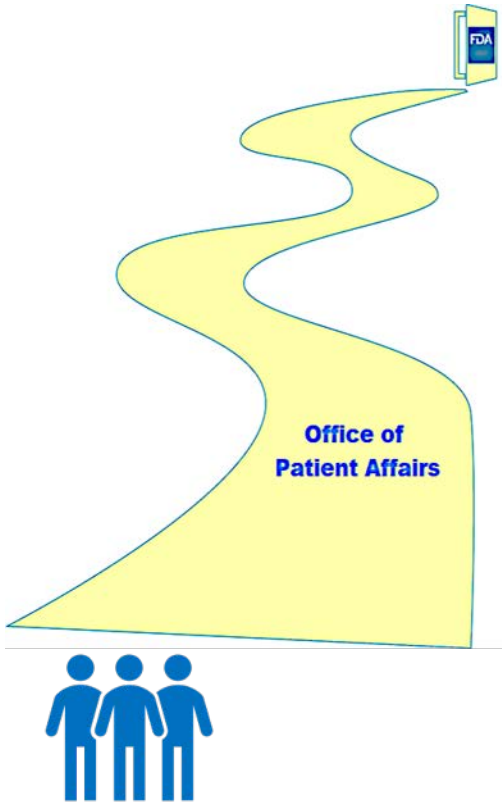
What we do

- **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

2. Treatment Options

3. Clinical Trials

- 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

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

- 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

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

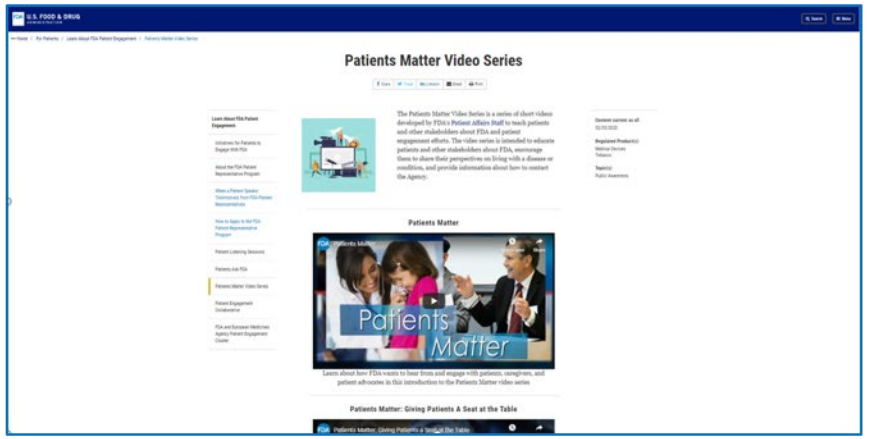
Patient Engagement Collaborative

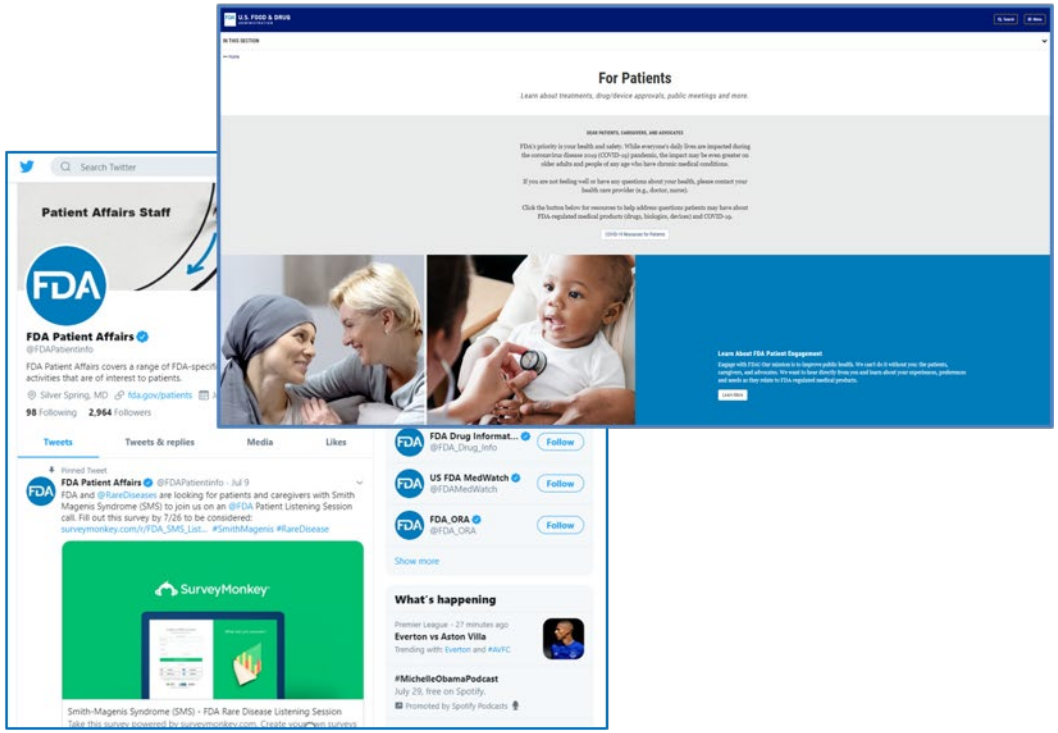
FDA U.S. FOOD & DRUG ADMINISTRATION

CLINICAL TRIALS TRANSFORMATION INITIATIVE

- 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





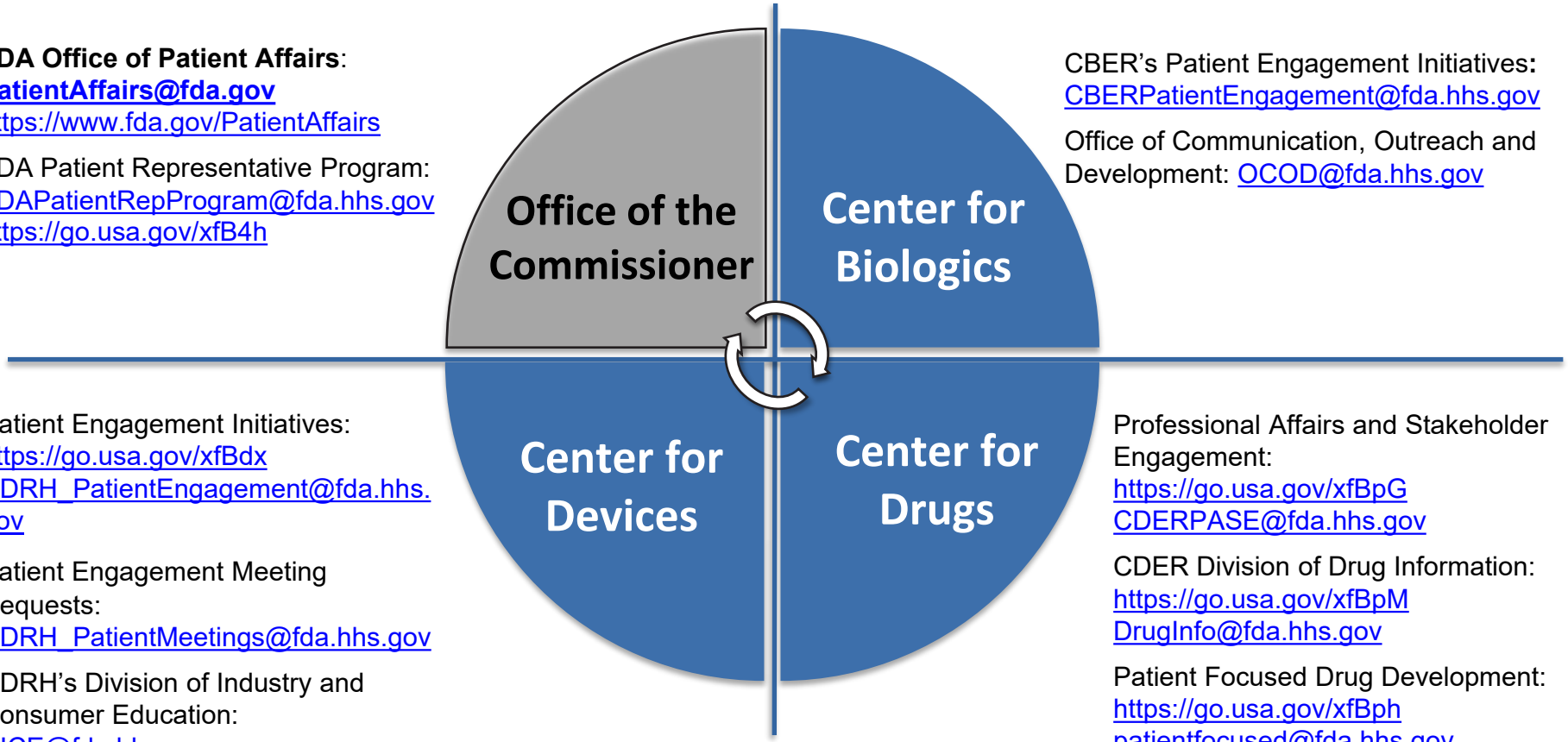
Resources

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



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

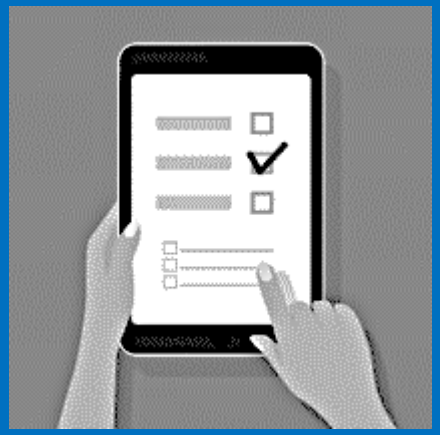
CDRH's Division of Industry and Consumer Education:
DICE@fda.hhs.gov

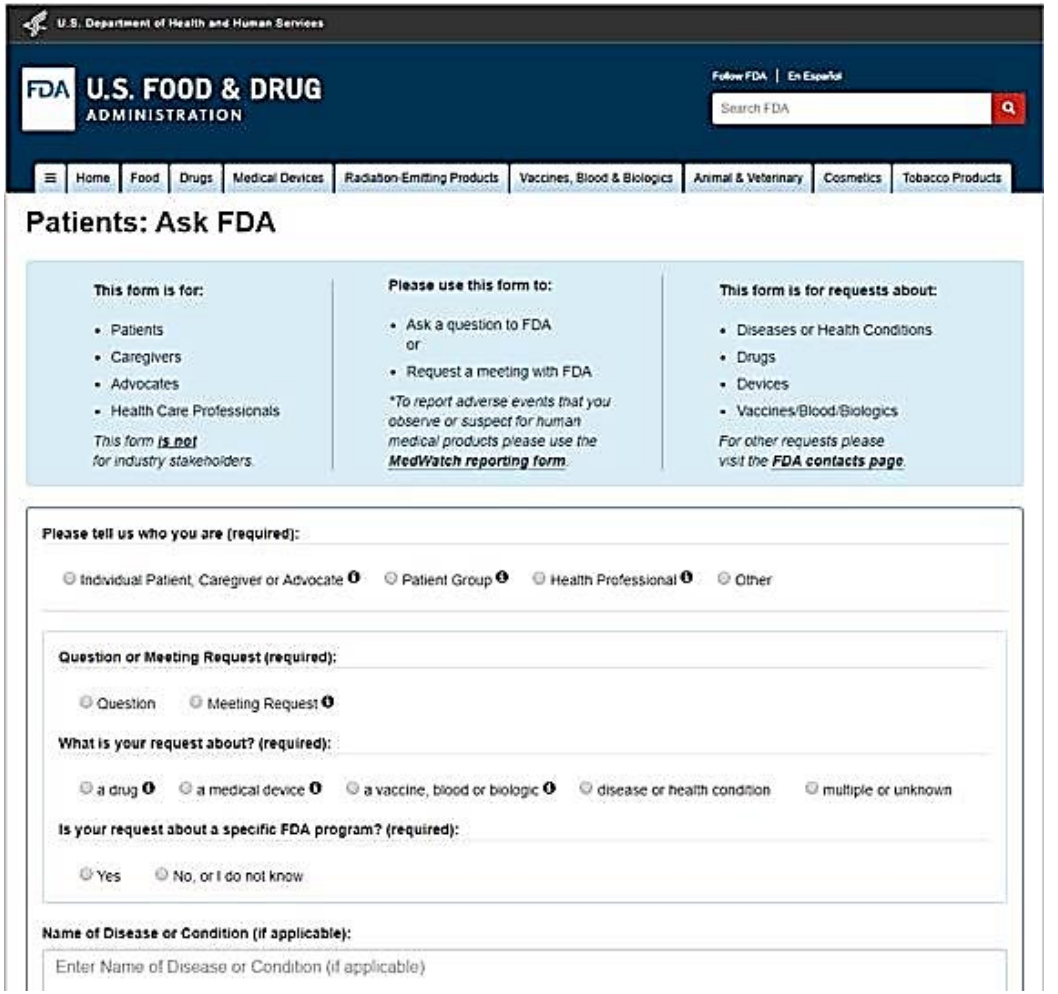
Professional Affairs and Stakeholder Engagement:
https://go.usa.gov/xfBpG
CDERPASE@fda.hhs.gov

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

Questions & Meeting Requests





www.fda.gov/PatientsAskFDA

Office of Patient Affairs

PatientAffairs@fda.gov

301-796-8460

www.fda.gov/Patients

@FDAPatientInfo

www.fda.gov/PatientsAskFDA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Carmen Matos


Ledet Muleta

Center for Drug Evaluation and Research

Engaging with FDA: Opportunities and Boundaries

CDR Sadhna Khatri, PharmD, MPH, MS

Supervisory Associate Director – Engagement Team

Professional Affairs and Stakeholder Engagement (PASE)

Office of Center Director

May 6, 2021

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 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

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

*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 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)

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

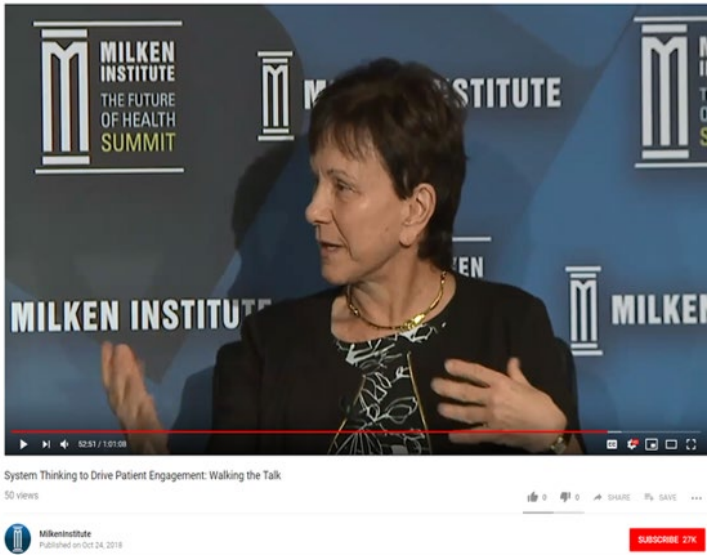
It might also support drug development more broadly:

- 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

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	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

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	*HCP Professional Organizations*	*Chain Pharmacies, PBMs, etc.*
LUPUS FOUNDATION OF AMERICA	AMERICAN COLLEGE OF CHEST PHYSICIANS	APhA
ASTMH AMERICAN SOCIETY OF TROPICAL MEDICINE & HYGIENE	Society of Critical Care Medicine The Intensive Care Professionals	ashp pharmacists advancing healthcare
Arthritis Foundation®	AMERICAN ACADEMY OF ALLERGY ASTHMA & IMMUNOLOGY	NCPA NATIONAL COMMUNITY PHARMACISTS ASSOCIATION
	American College of Allergy, Asthma & Immunology	NACDS NATIONAL ASSOCIATION OF CHAIN DRUG STORES
		American Association of Colleges of Pharmacy AACP Discover • Learn • Care: Improve Health

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

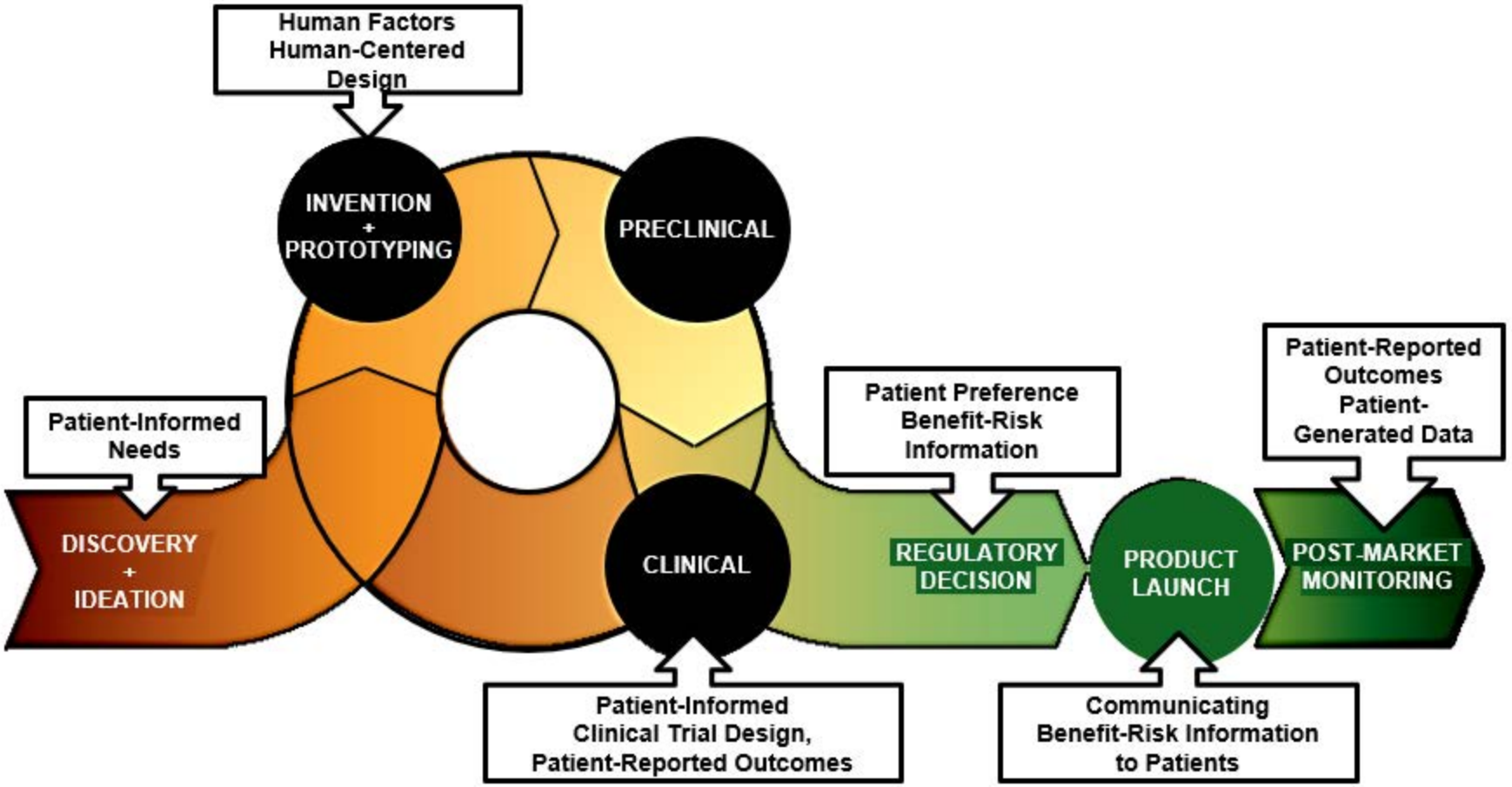
Thank you

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

FDA News Release

FDA approves first-of-kind device to treat obesity

SHARE TWEET + EMAIL

For Immediate Release

January 14, 2015

Release

Español

The U.S. Food and Drug Administration today approved the Maestro Rechargeable System for certain obese adults, the first weight loss treatment device that targets the nerve pathway between the brain and the stomach that controls feelings of hunger and fullness.

The Maestro Rechargeable System, the first FDA-approved obesity device since 2007, is approved to treat patients aged 18 and older who have not been able to lose weight with a weight loss program, and who have a body mass index of 35 to 45 with at least one other obesity-related condition, such as type 2 diabetes.

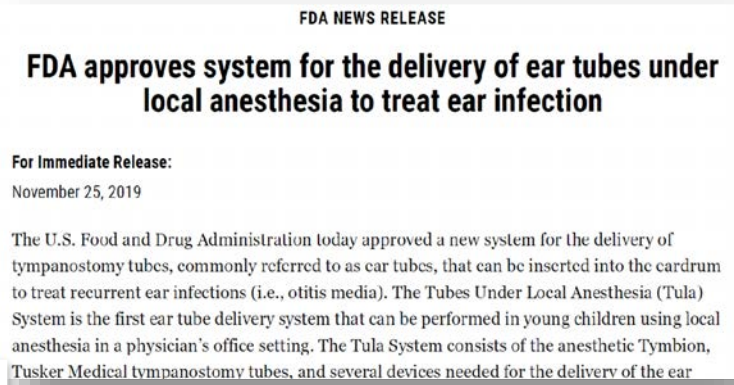
BMI, which measures body fat based on an individual's weight and height, is used to

24 Industry-sponsored regulatory PPI studies completed or in pipeline

NxStage Medical Announces FDA Clearance for Solo Home Hemodialysis Using NxStage® System One™

First clearance of its kind gives trained NxStage patients freedom to dialyze without a care partner

LAWRENCE, Mass., Aug. 28, 2017 /PRNewswire/ -- NxStage Medical, Inc. (Nasdaq: NXTM), a leading medical technology company focused on advancing renal care, today announced that the U.S. Food and Drug Administration (FDA) has cleared its System One for solo home hemodialysis, without a care partner, during waking hours.



FDA NEWS RELEASE

FDA approves system for the delivery of ear tubes under local anesthesia to treat ear infection

For Immediate Release:

November 25, 2019

The U.S. Food and Drug Administration today approved a new system for the delivery of tympanostomy tubes, commonly referred to as ear tubes, that can be inserted into the eardrum to treat recurrent ear infections (i.e., otitis media). The Tubes Under Local Anesthesia (Tula) System is the first ear tube delivery system that can be performed in young children using local anesthesia in a physician's office setting. The Tula System consists of the anesthetic Tymbion, Tusker Medical tympanostomy tubes, and several devices needed for the delivery of the ear

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

Gaining Insights from Patients on Their Disease and Devices Used to Treat It

TMJ ASSOCIATION
PATIENT CONVERSATIONS WITH THE FDA
OFFICE OF HEALTH TECHNOLOGY (OHT-1)
JANUARY 27, 2020

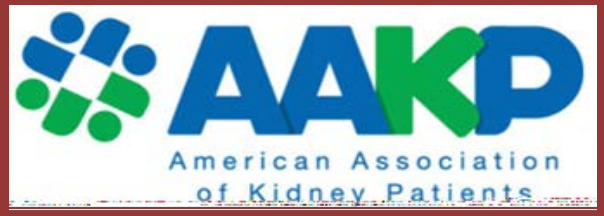
FDA Workshop: Evolving Role of Artificial Intelligence in Radiological Imaging February 25-26, 2020

- Patients and a caregiver invited to share their perspectives on this emerging it was to their product develop 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.



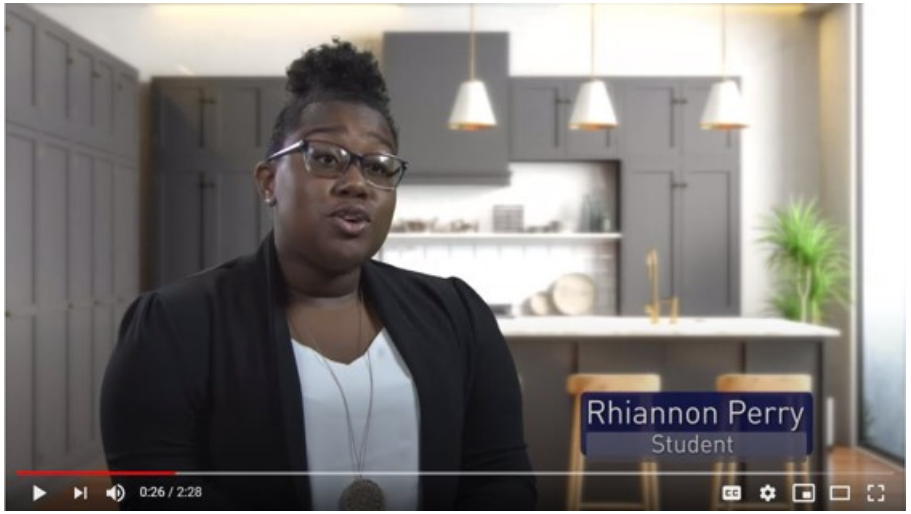
Virtual CDRH Patient Engagement Townhall

FDA Welcomes Patients & Caregivers

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

SEPTEMBER 10, 2020

Patient Engagement Videos



CDRH Patient Engagement Advisory Committee (PEAC)

PEAC members are diverse patients, caregivers, and patient advocates

<u>GOAL</u>: 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

1. Patient Engagement in Design, Conduct and Communication of Medical Device Clinical Trials

2. Patient-Generated Health Data & Medical Device Safety Surveillance

3. Communicating Cybersecurity Vulnerabilities of Medical Devices

4. Artificial Intelligence & Machine Learning

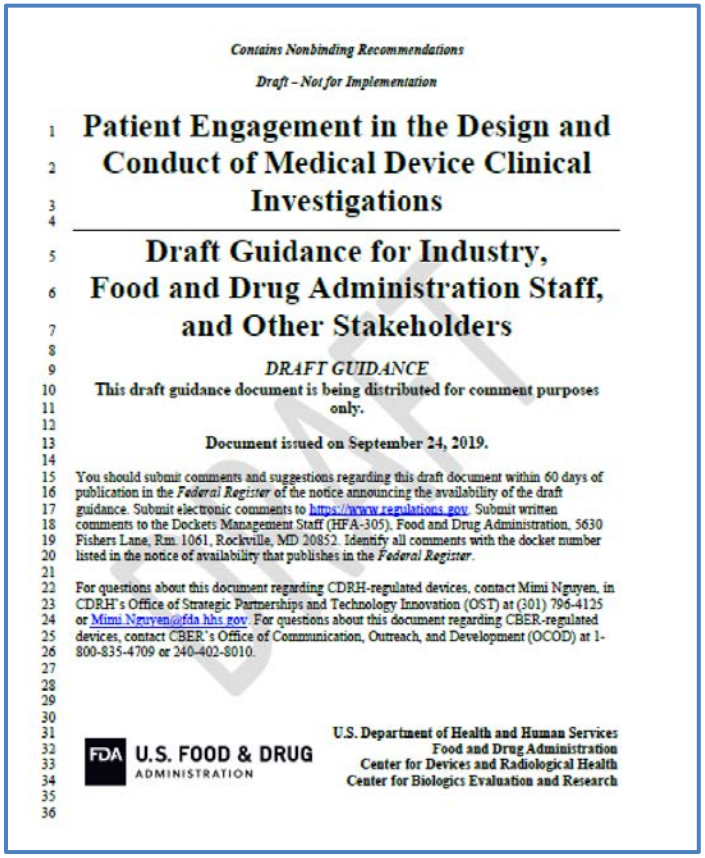
5. **STAY TUNED 2021**
Market Topic

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



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

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

Outcomes from PEAC 2019 on Cybersecurity

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

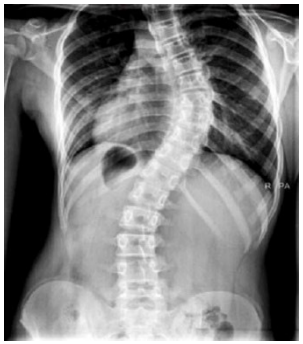
Collaborative Patient Preference Studies Addressing Preference-Sensitive Topics

Prostate Cancer

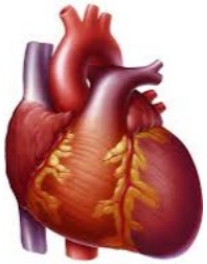
Uterine Fibroids

Chronic Pain

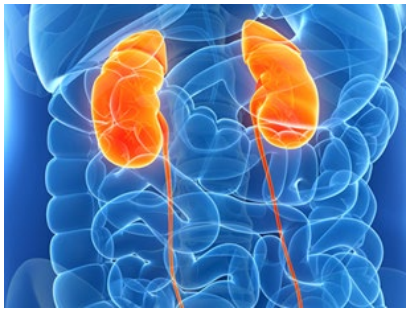
Adolescent Scoliosis



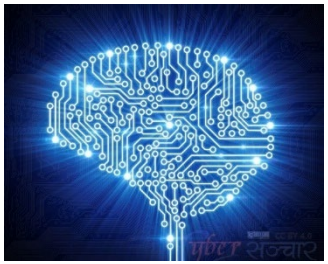
Heart Failure



End-Stage Kidney Disease

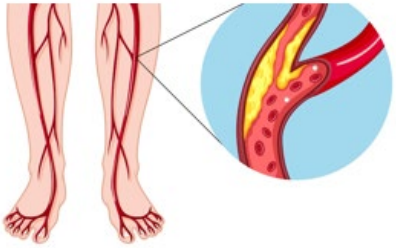


Breast Implants



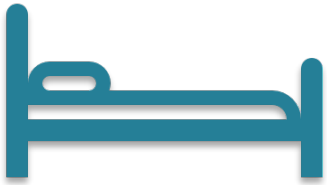
Artificial Intelligence

Contraception



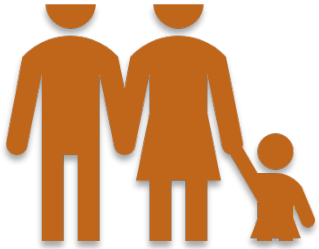
Peripheral Vascular Disease

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



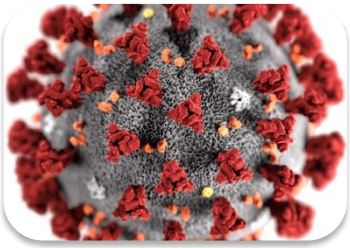
Heart failure in pediatric patients PRO development

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

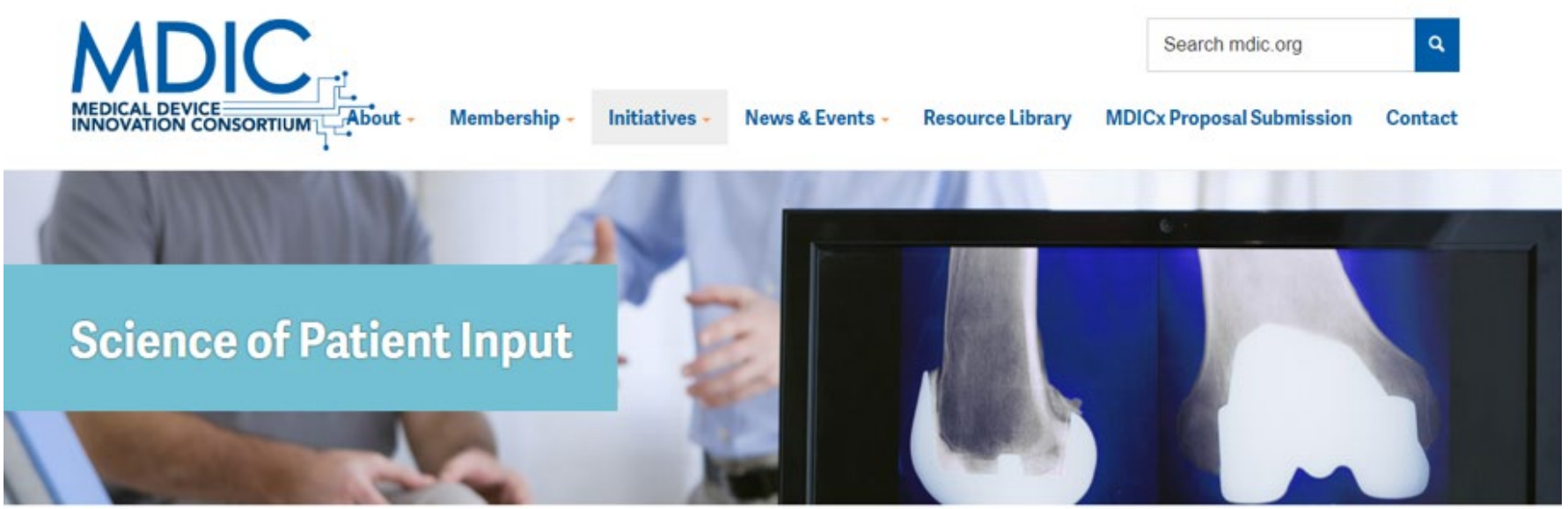
Social media listening to understand how diverse populations talk about chronic pain



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

Collaborative Communities: Addressing Health Care Challenges Together | FDA

Patients & Medical Device Evaluation

Patient Engagement

Clinical Outcome Assessments

Patient Preference Information

Patient-Generated Health Data

Resources

FDA CDRH Websites:

Patient Engagement : *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 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

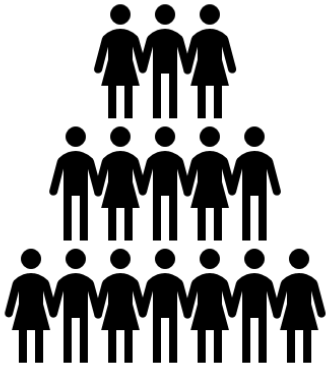
- **CDER**

Center for Biologics Evaluation and Research

- **CBER**

Center for Devices and Radiological Health

- **CDRH**



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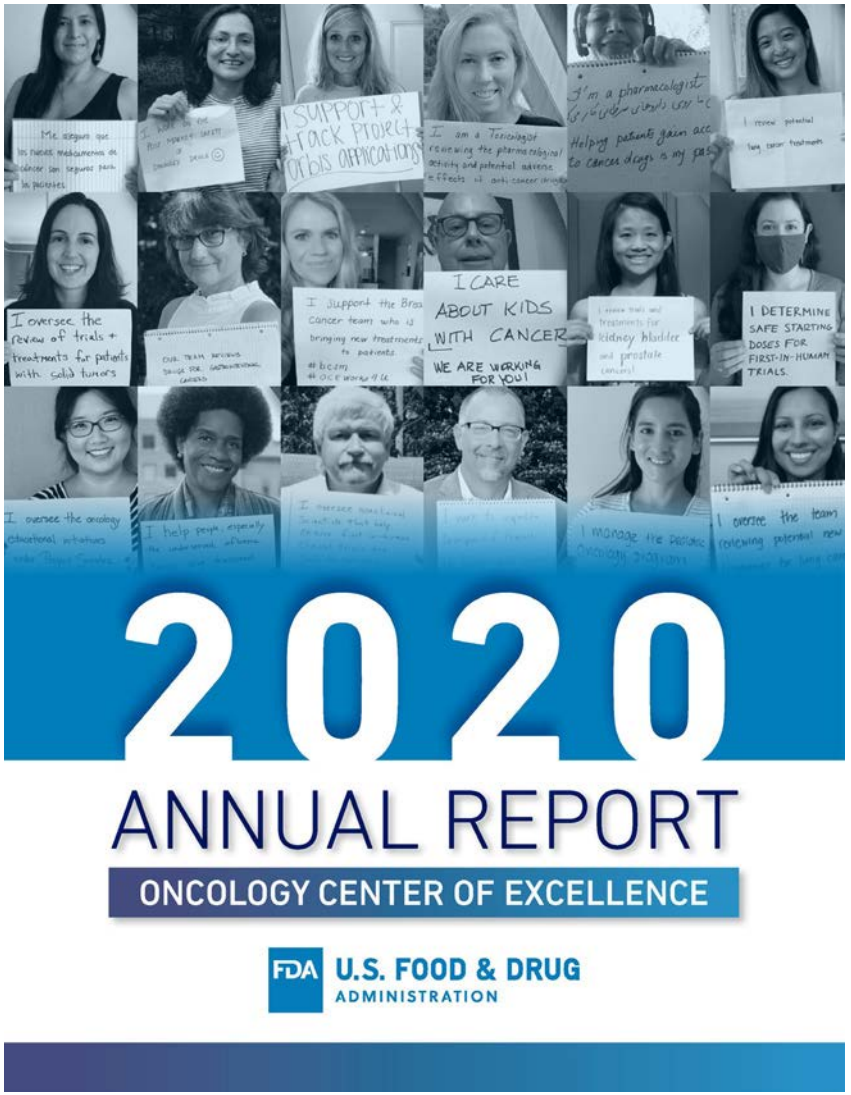

Encouraging Diverse Patient Voices

- Clinical Trial Guidance During COVID Meetings
- Project Equity
- Project Silver
- Project Facilitate
- Project Patient Voice

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PROJECT COMMUNITY

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**

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PROJECT COMMUNITY

PROJECT COMMUNITY

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FDA U.S. FOOD & DRUG ADMINISTRATION

CONVERSATIONS ON CANCER

Presented by the ONCOLOGY CENTER OF EXCELLENCE

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

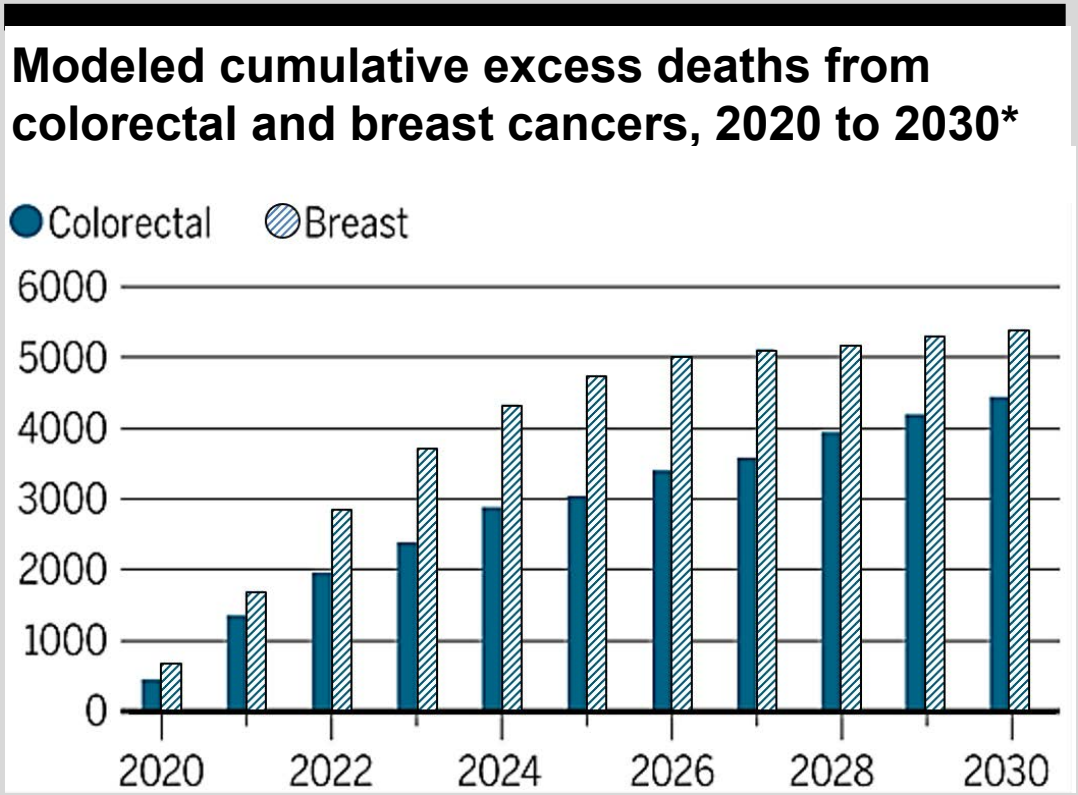
Cancer Prev Res September 17 2020 DOI:10.1158/1940-6207.CAPR-20-0447

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.



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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

Oncology Center of Excellence

National Black Family Cancer Awareness Week: Engaging the Generations

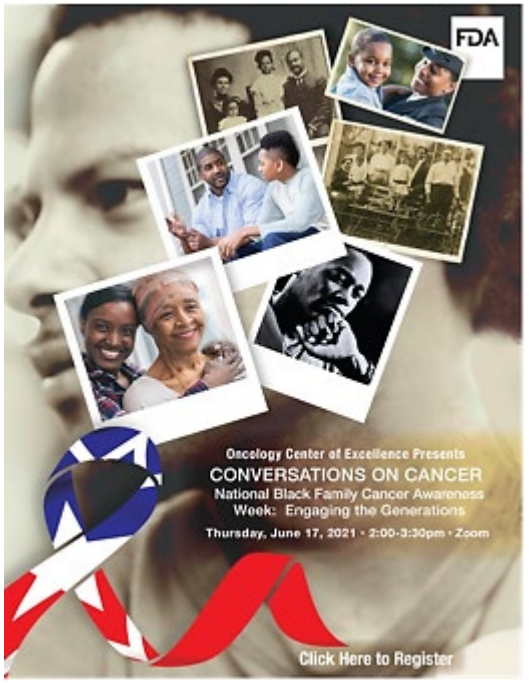
Part of the "Conversation on Cancer" Panel Discussion Series

Thursday, June 17

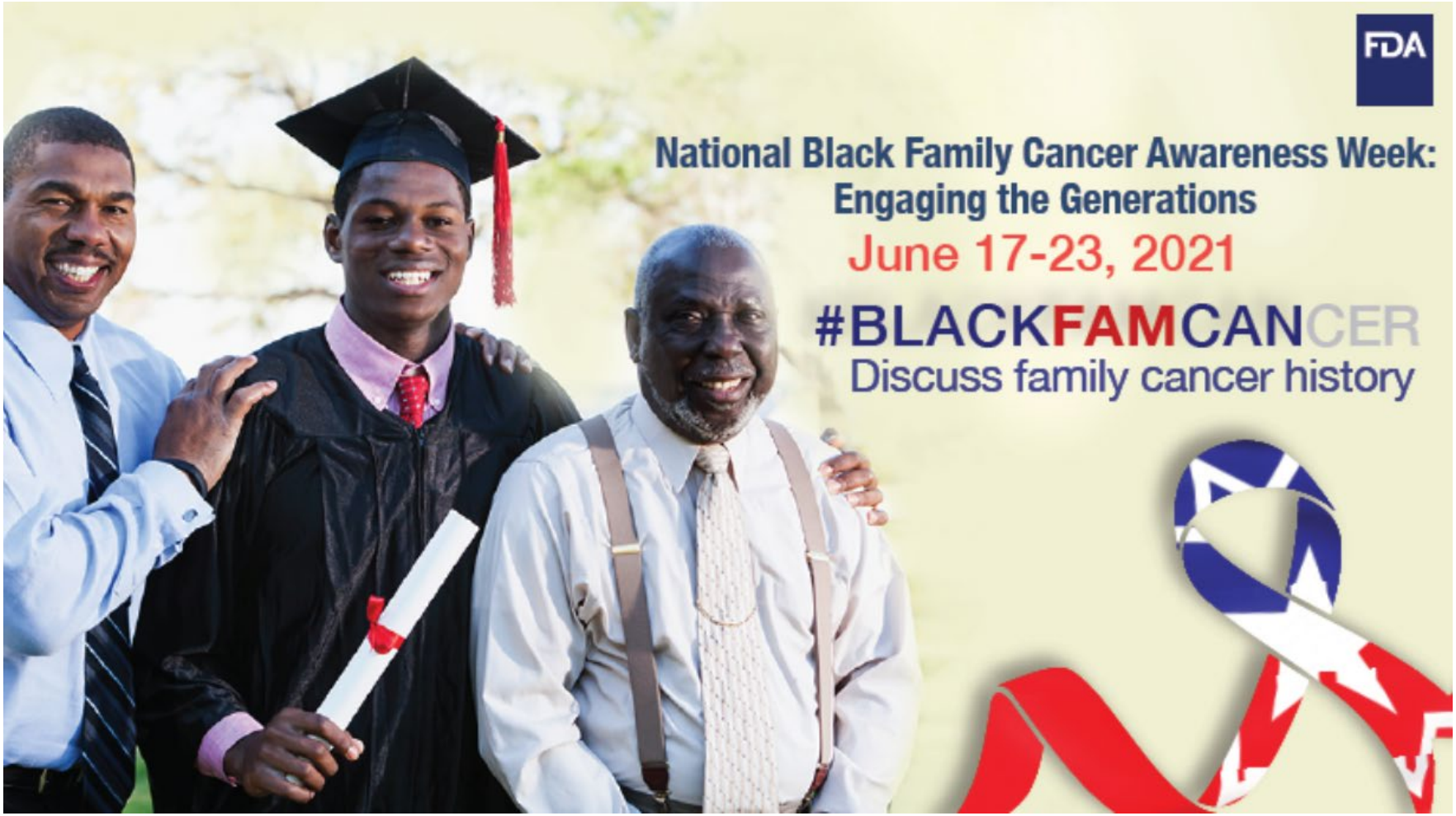
2-3:30 p.m. ET

Contact:

OCE-Engagement@fda.hhs.gov




FDA
National Black Family Cancer Awareness Week:
Engaging the Generations
June 17-23, 2021
#BLACKFAMCANCER
Enroll in cancer clinical trials


FDA
National Black Family Cancer Awareness Week:
Engaging the Generations
June 17-23, 2021
#BLACKFAMCANCER
Discuss family cancer history

FDA

Oncology Center of Excellence

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

Oncology Center of Excellence

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"**

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

Outline

- 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

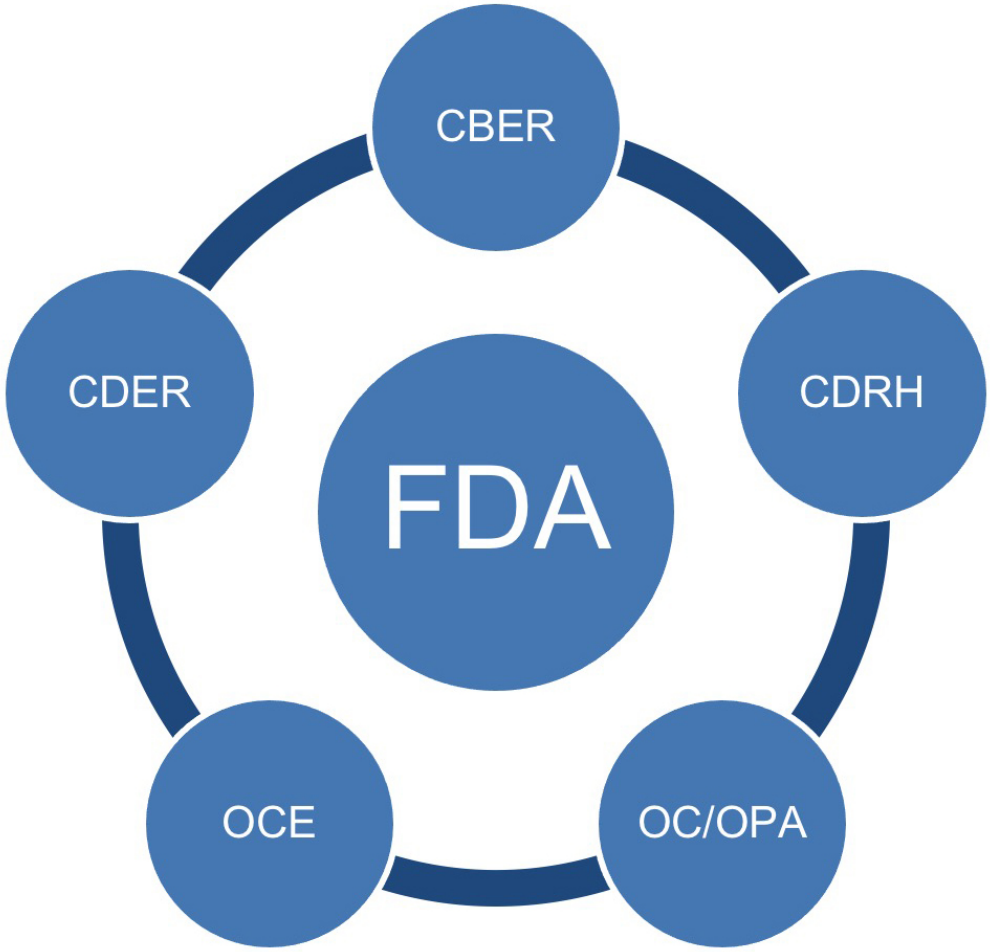
My comments are an informal communication and represent my own best judgement. These comments do not bind or obligate FDA.

CBER Patient Engagement Activities

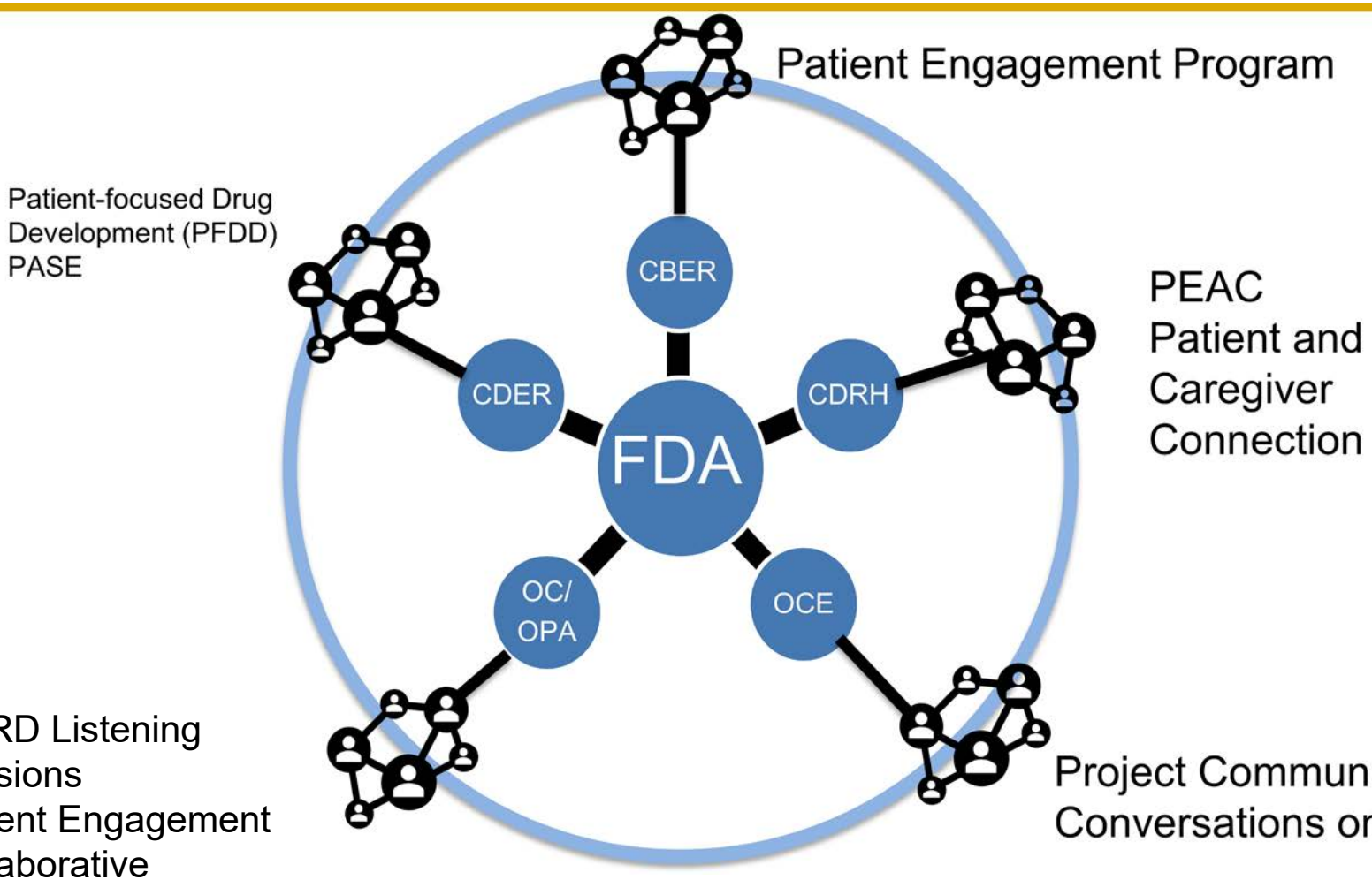
- CBER Patient Engagement Workgroup
- CBER Rare Disease Coordinating Committee
- Science of Patient Input Initiative
- Office of Communication, Outreach, and Development

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

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.

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