


# Welcome to Patient Engagement in Action: Insights from Patients & the FDA

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*November 21, 2019*

# **Patient Engagement in Action: Insights from Patients & the FDA**



# Disclaimer

- ▶ The views and opinions expressed in this presentation are those of the individual presenter and do not necessarily reflect the views of the Clinical Trials Transformation Initiative.
- ▶ The comments expressed today are those of the presenter only and do not necessarily represent the official positions or policies of the FDA.



# Introduction to CTTI

Pamela Tenaerts, MD, MBA

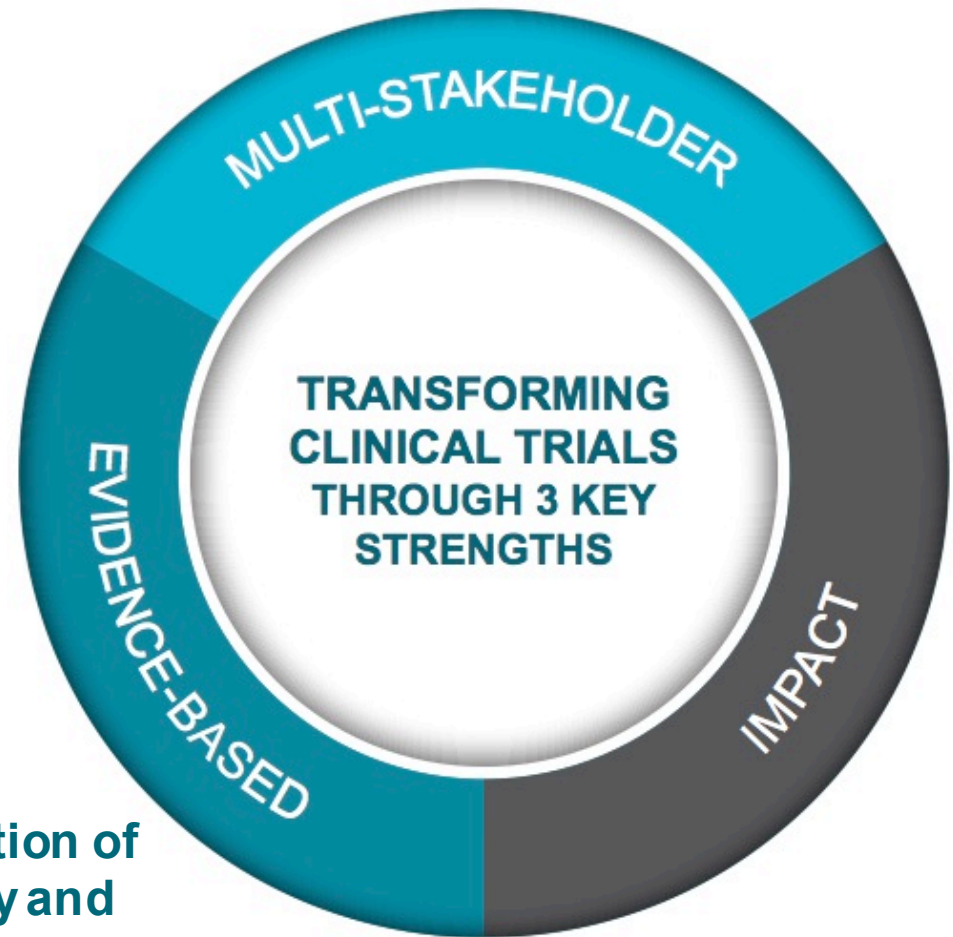
# CTTI Strengths



CLINICAL  
TRIALS  
**TRANSFORMATION**  
INITIATIVE

Public-private partnership  
Co-founded by Duke University & FDA  
Involves all stakeholders  
80+ members

**MISSION:** To develop and drive adoption of practices that will increase the quality and efficiency of clinical trials



# Multi-Stakeholder



# CTTI Membership



# CTTI Activities

## Quality

- ▶ Quality by Design
- ▶ Informing ICH E6 Renovation
- ▶ Diversity
- ▶ Analysis of ClinicalTrials.gov
- ▶ Recruitment
- ▶ Planning for Pregnancy Testing
- ▶ State of Clinical Trials Report
- ▶ Monitoring

## Patient Engagement

- ▶ **Patient Groups & Clinical Trials**
- ▶ **Patient Engagement Collaborative**

## Investigators & Sites

- ▶ Investigator Community
- ▶ Investigator Qualification
- ▶ Site Metrics

- ▶ Novel Endpoints
- ▶ Mobile Technologies
- ▶ Decentralized Clinical Trials
- ▶ Engaging Patients and Sites

- ▶ Real-World Data
- ▶ Registry Trials
- ▶ Master Protocols
- ▶ Antibacterial Drug Development
- ▶ Large Simple Trials
- ▶ Using FDA Sentinel for Trials

- ▶ Single IRB
- ▶ Data Monitoring Committees
- ▶ Informed Consent
- ▶ Safety Reporting

# Patient Engagement at CTTI

- Normed (no question) inclusion of patients as equal partners into every aspect of clinical trial (reform)
  - Full integration into the Steering Committee in 2015
  - Individual patients reimbursed for time on CTTI activities including projects
- Patient/caregivers on project teams have played critical role in shaping projects
- Co-founded Patient Engagement Collaborative with FDA
- Almost all CTTI recommendations to date include a recommendation to involve all stakeholders, particularly patients, in the process

# Today's Presenters

 Andrea Furia-Helms

- Director, Patient Affairs Staff, OC/FDA

 Michelle Tarver

- Director of Patient Science and Engagement, CDRH/FDA

 Robyn Bent

- Director, CDER PFDD Program, CDER/FDA

 Diane Maloney

- Associate Director for Policy, CBER/FDA

 Theresa Strong

- Director of Research Programs, Foundation for Prader-Willi Research

# FDA Patient Affairs Staff

*How FDA Involves Patients and Advocates*

**Andrea Furia-Helms, MPH**

Director, Patient Affairs Staff  
Office of Clinical Policy and Programs  
Office of the Commissioner



Patient Engagement in Action:  
Insights from Patients & the FDA  
November 21, 2019

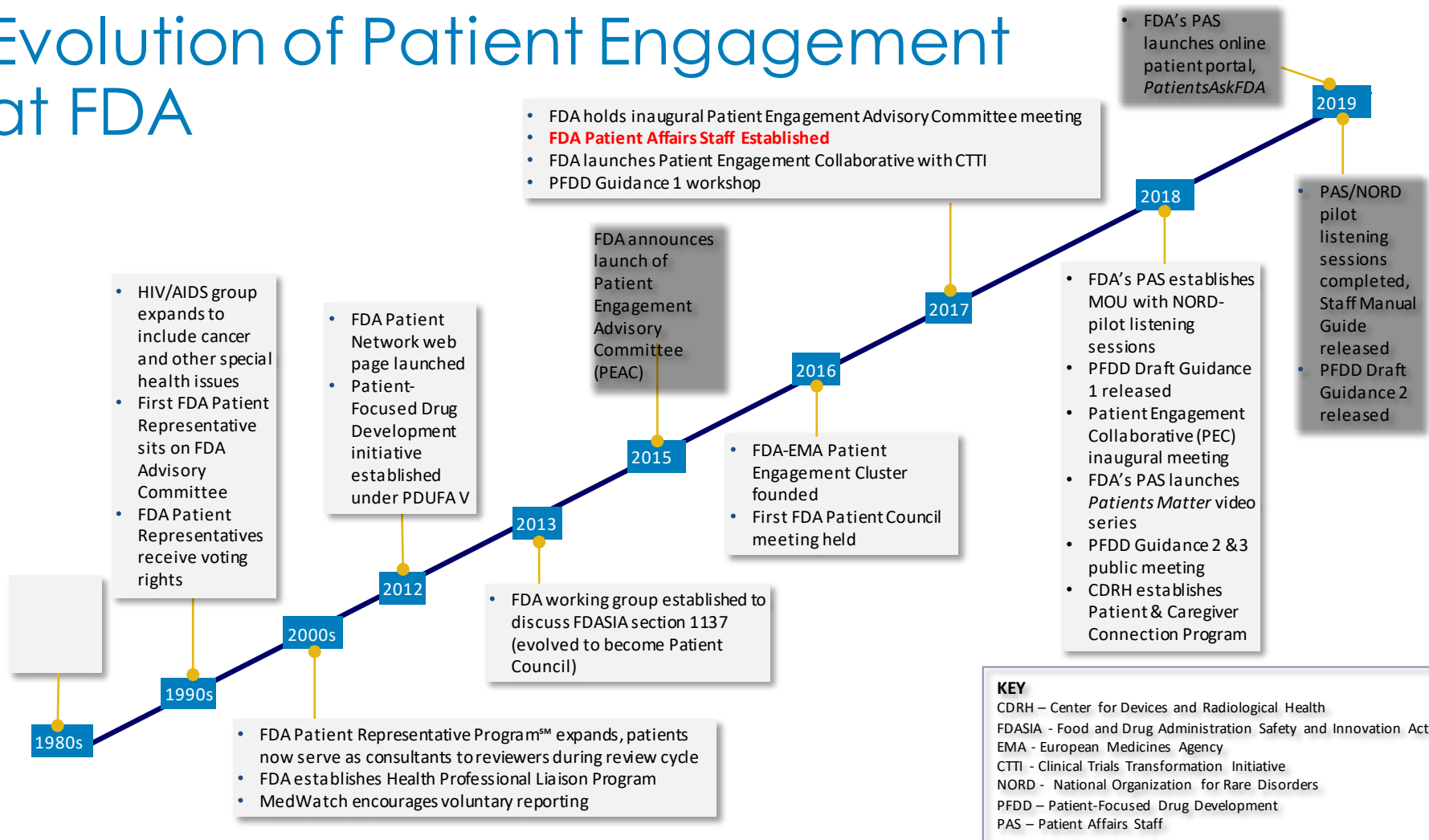
# The Importance of the Patient Voice



- 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 FDA



# Patient Affairs Staff (PAS)



Who we are

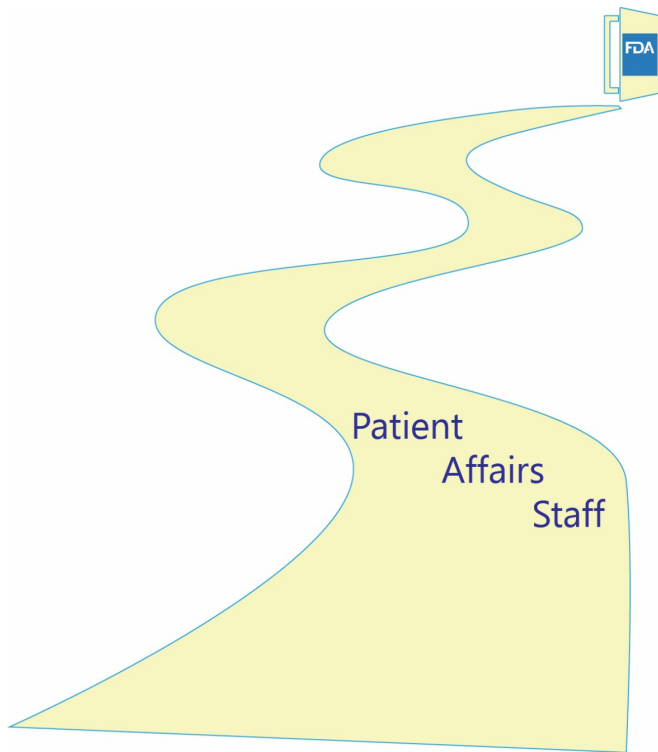


What we do

Patient Affairs Staff (PAS) in the **Office of the Commissioner** leads patient engagement activities **across the medical product Centers**—to allow dialogue and collaboration between patients, their advocates, and the FDA

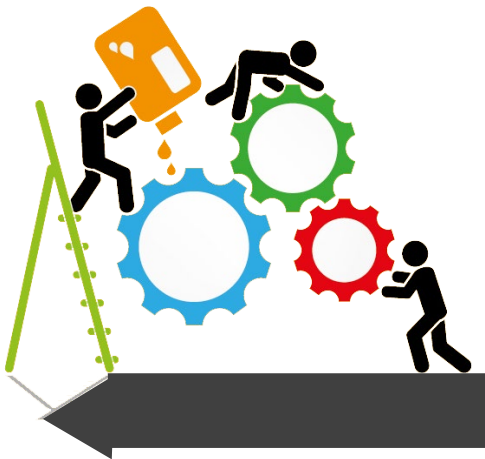
- Creating and assisting with **public-private collaborations and partnerships**
- Lead **cross-cutting programs and activities** that leverage best practices and enhance patient engagement.
- Enhancing FDA's **external communication platforms** (e.g., PatientsAskFDA, FDA's *For Patients* webpage, social media, etc.)

# PAS Programs and Activities



- FDA/EMA Patient Engagement Cluster
- Patient Engagement Collaborative
- FDA Rare Disease Patient Listening Sessions
- Enhancing communications

# FDA and EMA Patient Engagement Cluster



**U.S. FOOD & DRUG  
ADMINISTRATION**



**EUROPEAN MEDICINES AGENCY**

## Mutual exchange on:

- Approaches for engaging and involving patient stakeholders
- High profile topics of mutual interest, especially those with potential high public interest
- Priorities and goals for collaborations to enhance engagement

### Publication:

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



## Patient Engagement Collaborative (PEC)



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



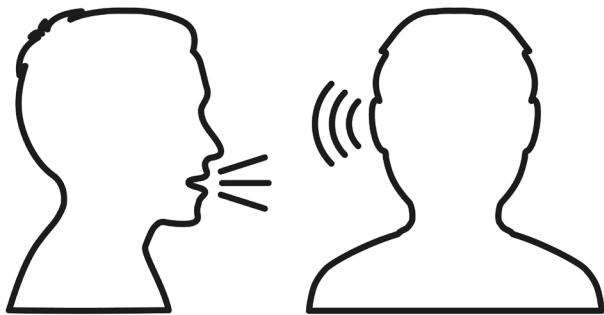
## Patient Listening Sessions



## Rare Diseases

- Memorandum of Understanding with the National Organization for Rare Disorders (NORD)
- Inform regulatory decision-making
- Educate review staff about rare diseases
- Help patients and their advocates understand the FDA's mission and work
- Provide a starting point to inform early stage research & development
- Pilot to assess the value & establish a process

# FDA Rare Disease Patient Listening Sessions



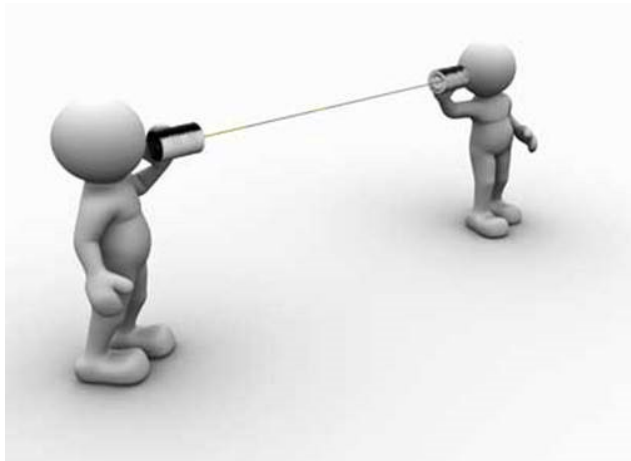
## Two Types:

1. **FDA-requested:** specific set of questions to ask of a particular patient sub-population
2. **Patient-requested:** patient community wants to share their experiences and perspectives with the FDA

**Request a Patient Listening Session**  
[www.fda.gov/PatientsAskFDA](http://www.fda.gov/PatientsAskFDA)

**Patient Listening Sessions Webpage**  
[www.fda.gov/PatientListeningSessions](http://www.fda.gov/PatientListeningSessions)

# Enhancing Communication with Patients



U.S. FOOD & DRUG ADMINISTRATION

Home > For Patients > Learn About FDA Patient Engagement

## Initiatives for Patients to Engage With FDA

SHARE TWEET LINKEDIN PIN IT EMAIL PRINT

| Initiative | FDA-led Patient-Focused Drug Development (PFDD) Meetings  | Externally-led PFDD Meetings   | NOORD MOU Pilot Listening Sessions   | Patient Engagement Collaborative (PEC) | Patient Engagement Advisory | Patient Representative Program (PRP) |
|------------|---|--|--|--|-----------------------------|--------------------------------------|
| Purpose    | Public meetings that systematically obtain the patient perspective on specific diseases and their | To allow patient organizations to identify and organize patient-focused collaborations to generate | Pilot listening sessions in rare diseases to inform FDA staff of disease and treatment | All diseases on request in a pilot     | On request in a pilot       | On request in a pilot                |

U.S. FOOD & DRUG ADMINISTRATION

Home > For Patients > Learn About FDA Patient Engagement

## For Patients

approvals, and learn what FDA is doing if new drugs to treat or prevent cancer.

U.S. FOOD & DRUG ADMINISTRATION

Home > For Patients > Learn About FDA Patient Engagement

## Patients Matter Video Series

SHARE TWEET LINKEDIN PIN IT EMAIL PRINT

The Patients Matter Video Series is a series of short videos developed by FDA's [Patient Affairs Staff](#) to teach patients and other stakeholders about FDA and patient engagement efforts. The video series is intended to educate patients and other stakeholders about FDA, encourage them to share their perspectives on living with a disease or condition, and provide information about how to contact the Agency.

# Submit Questions & Meeting Requests



U.S. Department of Health and Human Services

**FDA** U.S. FOOD & DRUG ADMINISTRATION

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Search FDA

Home Food Drugs Medical Devices Radiation-Emitting Products Vaccines, Blood & Biologics Animal & Veterinary Cosmetics Tobacco Products

## Patients: Ask FDA

|  |  |  |
|--|--|--|
| <p><b>This form is for:</b></p> <ul style="list-style-type: none"><li>• Patients</li><li>• Caregivers</li><li>• Advocates</li><li>• Health Care Professionals</li></ul> <p><i>This form <b>is not</b> for industry stakeholders.</i></p> | <p><b>Please use this form to:</b></p> <ul style="list-style-type: none"><li>• Ask a question to FDA or</li><li>• Request a meeting with FDA.</li></ul> <p><i>*To report adverse events that you observe or suspect for human medical products please use the <a href="#">MedWatch reporting form</a>.</i></p> | <p><b>This form is for requests about:</b></p> <ul style="list-style-type: none"><li>• Diseases or Health Conditions</li><li>• Drugs</li><li>• Devices</li><li>• Vaccines/Blood/Biologics</li></ul> <p><i>For other requests please visit the <a href="#">FDA contacts page</a>.</i></p> |
|--|--|--|

**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):**

[www.fda.gov/PatientsAskFDA](https://www.fda.gov/PatientsAskFDA)



# Medical Product Center Patient Initiatives

## Center for Drugs Evaluation and Research (CDER)

- Professional Affairs and Stakeholder Engagement (PASE)
- FDA-led Patient-Focused Drug Development Meetings (PFDD)
- Externally-led Patient-Focused Drug Development Meetings
- PFDD Methodological Guidance Series

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## Center for Medical Devices and Radiological Health (CDRH)

- Partner with Patients
- Patient Engagement Advisory Committee
- Patient and Care-Partner Connection Program

## Center for Biologics Evaluation and Research (CBER)

- Interactive Meetings with Patients
- CBER Workgroups:
  - CBER Patient Engagement Workgroup
  - CBER Rare Disease Coordinating Committee
  - CBER Science of Patient Input (SPI) Team



## When in doubt...contact Patient Affairs!



[PatientAffairs@fda.gov](mailto:PatientAffairs@fda.gov)



301-796-8460



[www.fda.gov/Patients](http://www.fda.gov/Patients)



@FDAPatientInfo

[www.fda.gov/PatientsAskFDA](http://www.fda.gov/PatientsAskFDA)



# **PATIENT ENGAGEMENT & MEDICAL DEVICES**

**Michelle Tarver, MD, PhD**

**Director, Patient Science & Engagement Program**

**Center for Devices and Radiological Health**

**Food and Drug Administration**

**November 21, 2019**

# Patients & Medical Device Evaluation



Patient  
Engagement



Clinical  
Outcome  
Assessments



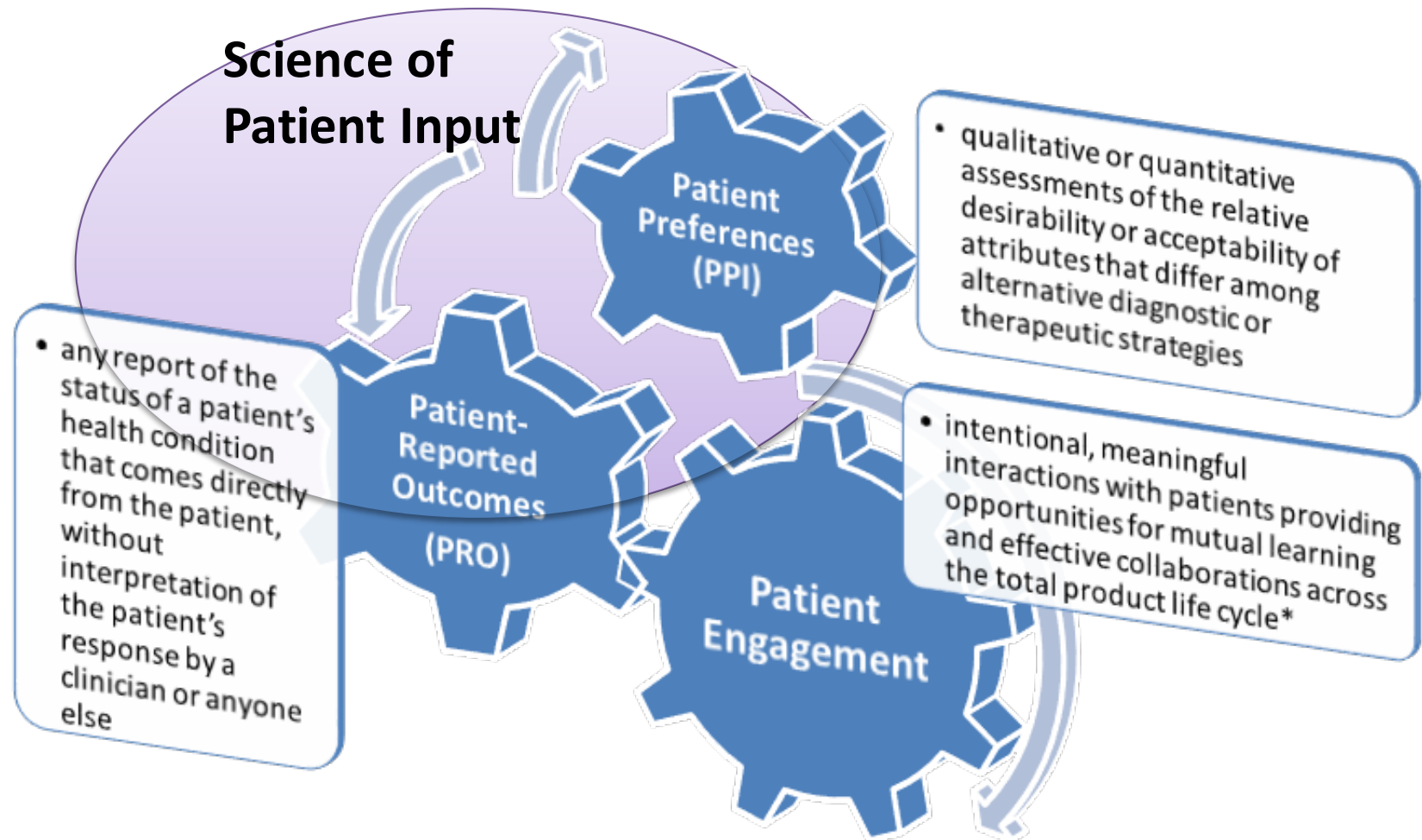
Patient  
Preference  
Information



Patient-  
Generated  
Health Data



# Patient Input in Regulatory Efforts



# CDRH Patient Science & Engagement Program

*Inspired by Patients, Driven by Science*

Understand the patients' perspectives and proactively incorporate them into all our decisions and regulatory activities where appropriate

Consistent  
Regulatory  
Review

Culture of  
Patient  
Engagement

Optimized  
Research  
Roadmap

# Patient Engagement at CDRH

*Inspired by Patients, Driven by Science*

# Patient Group Conversations

## Patient Experiences with Weight-Loss Devices

*Invitation Only Meeting*

Have you used one of the following medical devices to help you lose weight?

- Intragastric Balloon (Orbera, Obalon, or ReShape)
- AspireAssist
- VBLOC Maestro

Would you like to come to a meeting at the Food and Drug Administration (FDA) and tell them about your experience?

FDA would like patient feedback on topics such as:

- Your quality of life
- Your treatment expectations versus results
- Your level of satisfaction with your results
- What you need to achieve your weight loss goals

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



## ENTERAL NUTRITION CONSUMER FEEDBACK MEETING

**By Invitation Only**

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

The meeting will cover:

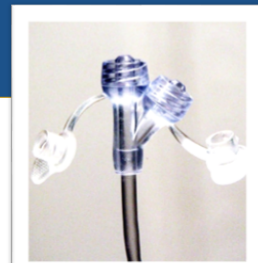
- Patient needs for enteral feeding
  - Including blended diets, medication delivery, tube feeding at school/work, concerns and challenges with tube feeding, venting/draining
- Patient demonstrations and videos on blended diet preparation
- An in-depth discussion of ENFit®
  - Including the transition,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

*Lunch will be provided and accommodations made for tube feeding*

**May 22<sup>nd</sup>, 2017 10am – 3:30pm**

**U.S. Food and Drug Administration**  
10903 New Hampshire Avenue  
Silver Spring, MD 20993

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



# 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

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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.

# CDRH Patient & Caregiver Connection Pilot Organizations

|   |   |  |  |  |
|---|---|--|--|--|
|    | <br>North American<br>Spinal Cord Injury<br>— Consortium — |    |  |   |
|    |   |    |   |  |
|    |    |    |   |  |
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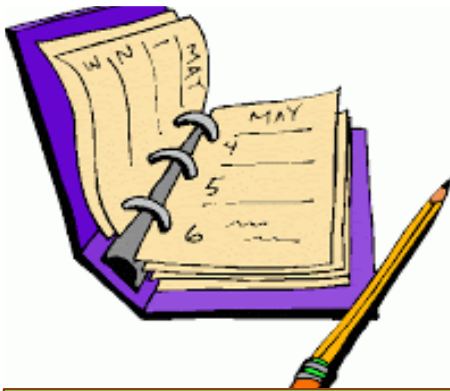
# CDRH Community Town Hall: Patient & Caregiver Connection

June 18, 2019

# 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

**SCIENTIFIC TOPICS INCLUDE:** unmet clinical needs, patient-reported outcomes, labeling, communication of risks and benefits, clinical trial design, registries, post-market monitoring, cybersecurity, and digital health

# PEAC 2019 Recommendation: Communication Framework for Cybersecurity



- Engage with patients throughout the process
- Allow patients to be part of the “boots-on-the-ground intelligence system”
- Clarify actionable steps for patients when issuing cybersecurity safety communications
- Empower patients to maintain good cyber hygiene

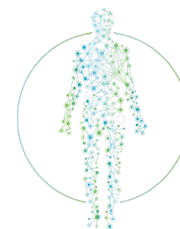


# PEAC 2018 Recommendation: Support Data Sharing



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

- Shared a letter of support for the principles of openly sharing non-proprietary data collected by medical devices
- Encourage the empowerment of patients in the development and evaluation of medical devices & become active members in monitoring of devices
- Help enrich the understanding of benefits and risks of technology



**Patient Safety**  
M O V E M E N T

<https://patientsafetymovement.org/news/u-s-food-and-drug-administration-supports-principles-of-open-sharing-of-data/>

# CDRH DRAFT GUIDANCE ON Patient Engagement

*Inspired by Patients, Driven by Science*

# PEAC 2017 Recommendation: Framework for Patient Engagement in Clinical Trials

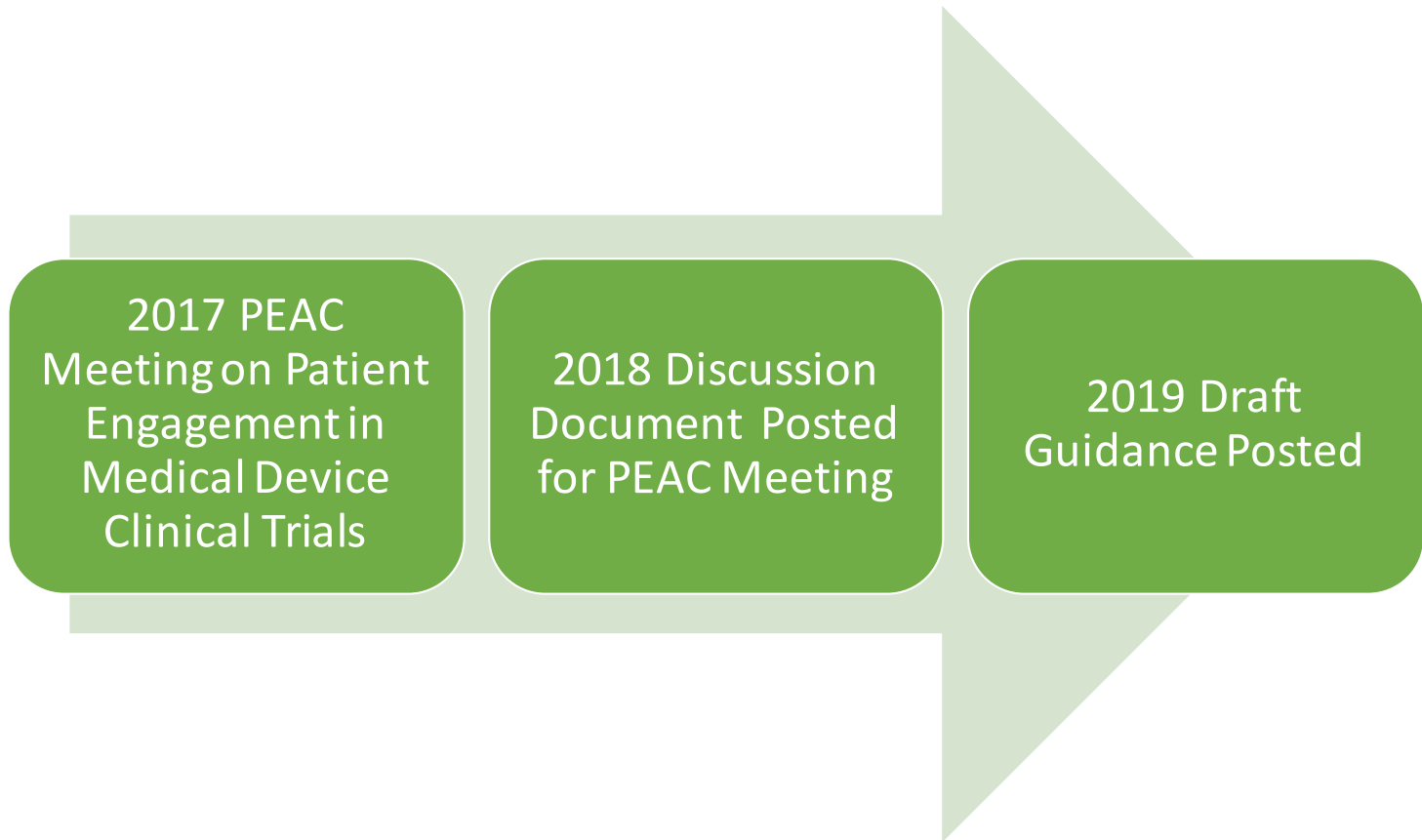
- *Demystify barriers to engaging with patients as advisors in the design and conduct of clinical trials*
- *Encourage sponsors to involve patients as key opinion leaders in the process and empower them to contribute*



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<https://www.fda.gov/medical-devices/guidance-documents-medical-devices-and-radiation-emitting-products/cdrh-fiscal-year-2019-fy-2019-proposed-guidance-development>

# Patient Engagement in Clinical Investigations



# Draft Guidance Objectives

- Help sponsors **understand how** they can use **patient engagement** to elicit experience, perspectives, and other relevant information from patient advisors **improve the design and conduct of medical device clinical investigations**
- **Highlight the benefits** of engaging with patient advisors early in the medical device development process
- Illustrate which **patient engagement activities** are generally **not** considered by FDA to **constitute research** or an activity subject to FDA's regulations, including regulations regarding institutional review boards (IRBs)
- **Address common questions and misconceptions** about collecting and submitting to FDA patient engagement information regarding the design and conduct of a medical device clinical investigation

# Roles for Patients in Medical Device Clinical Investigations

## Study/Research Participants

Individuals who are or become a participant in research, either as a recipient of the test article or as a control, and may include healthy individuals

## Patient Advisors

Individuals who have experience living with a disease or condition, and can serve in an advisory or consultative capacity to improve clinical investigation design and conduct, but who are not study/research participants themselves

# 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](mailto: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.

- Read the guidance at <https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-engagement-design-and-conduct-medical-device-clinical-investigations>
- FDA Docket: FDA-2019-D-3846 open until November 22, 2019 for public comments visit:
- <https://www.regulations.gov/docket?D=FDA-2019-D-3846>

## Resources



### CDRH Websites on Patient Engagement:

<https://www.fda.gov/about-fda/cdrh-patient-engagement/cdrh-patient-and-caregiver-connection>

<http://wcms-internet.fda.gov/about-fda/center-devices-and-radiological-health/cdrh-patient-engagement/latest>

### CDRH Websites on Patient Science:

Pre-Submissions: <https://go.usa.gov/xmVsh>

Patient Preference Information:

<https://go.usa.gov/xmVHG>

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

Patient-Reported Outcomes:

<https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-reported-outcome-measures-use-medical-product-development-support-labeling-claims>

<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](mailto:CDRH-PRO@fda.hhs.gov)

- For Patient Preference Information Questions:

[CDRH-PPI@fda.hhs.gov](mailto:CDRH-PPI@fda.hhs.gov)

- For Patient Engagement Questions:

[CDRH\\_PatientEngagement@fda.hhs.gov](mailto:CDRH_PatientEngagement@fda.hhs.gov)

- If you are not sure:

[michelle.tarver@fda.hhs.gov](mailto:michelle.tarver@fda.hhs.gov)



Thank You

---



*& Devices*

# FDA/CDER Patient Engagement

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

**CTTI**  
Patient Engagement Webinar  
Nov 21, 2019

# TOPICS TO COVER

## 1. Patient Focused Drug Development (PFDD) Efforts

- PFDD Meetings
- Externally Led PFDD Meetings
- Methodologic Guidance Series
- Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data

## 2. PASE Programs and Initiatives

- Engagement and Targeted Outreach

# Creating Opportunities for Dialogue

- Patients are uniquely positioned to inform understanding of the therapeutic context for drug development and evaluation
  - There is a need for more systematic ways of gathering patient perspectives on their condition and treatment options
- Patient-Focused Drug Development
  - FDA convened 24 meetings on specific disease areas in FY 2013-17
    - Meetings can help advance a systematic approach to gathering input

# PFDD Meetings

- Meetings follow similar, but tailored, design
  - Takes into account current state of drug development, specific interests of FDA review division, needs of the patient population
- Discussion elicits patients' perspectives on their disease and on treatment approaches
- Input is generated in multiple ways:
  - Patient panel comments and facilitated discussion with in-person participants
  - Interactive webcast and phone line for remote participants
  - A federal docket allowing for more detailed comments

# Externally-led PFDD: The Opportunity

- Patient organizations identify and organize **patient-focused collaborations** to generate public input on specific disease areas
- Meetings provide an important opportunity to **hear directly from patients**, patient advocates, and caregivers about the symptoms that matter most to them, the impact the disease has on patients' daily lives, and patients' experiences with currently available treatments.



# What we have learned from PFDD Meetings



- Patients with chronic serious disease are **experts** on what it's like to live with their condition
- The “chief complaints” heard in PFDD meetings often were not being factored explicitly into drug development plans, including measures planned for collection in trials
- Patients want to be as active as possible in the work to develop and evaluate new treatments

## Update on PFDD Guidances and Public Workshops

### PFDD Guidance 1: Collecting Comprehensive and Representative Input



- Workshop held on December 18, 2017
- Issued Draft Guidance in June 2018

### PFDD Guidance 2: Methods to Identify What is Important to Patients



- Workshop held on October 15-16, 2018
- Issued Draft Guidance in September 2019

### PFDD Guidance 3: Select, Develop or Modify Fit for Purpose Clinical Outcome Assessments



- Workshop held on October 15-16, 2018
- Discussion Document published

### PFDD Guidance 4: Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making



- **Upcoming workshop on December 6, 2019**

### PFDD Guidance 5: Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data



- Workshop held on March 19, 2018
- Issued Draft Guidance in December 2018

### Workshop on Enhancing Patient Input on Clinical Trials



- Workshop held on March 18, 2019
- Meeting summary report publicly available

# PASE Programs



PASE  
Programs  
and  
Initiatives

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

## How Can Stakeholders Contribute?

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



*Thank you!*

# Patient Engagement at CBER

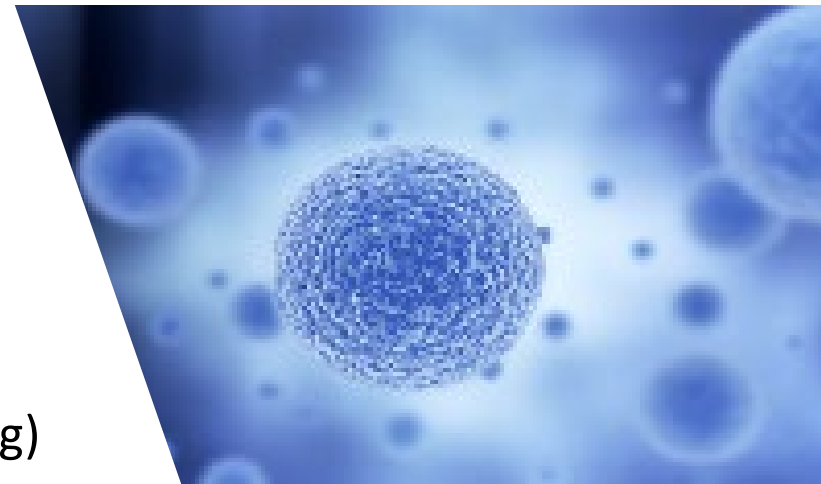
**Diane Maloney, J.D.**  
**Associate Director for Policy**  
**FDA Center for Biologics Evaluation and Research**

**CTTI webinar:**  
**Patient Engagement in Action: Insights from Patients & FDA**  
**November 21, 2019**

The views and opinions expressed  
in this presentation are mine and  
should not be attributed to or  
considered binding on FDA

## **We welcome patient input for all the products we regulate at the Center for Biologics Evaluation and Research**

- Allergenics
- Blood Products
- Human Tissues and Cellular Products
- Gene Therapies (including genome editing)
- Vaccines (preventative and therapeutic)
- Xenotransplantation Products
- Devices Related to Biologics



# CBER Works with Others in FDA on Patient Engagement



We work regularly with:

CDER

CDRH

Patient Affairs  
Staff

Stakeholder  
Engagement Staff

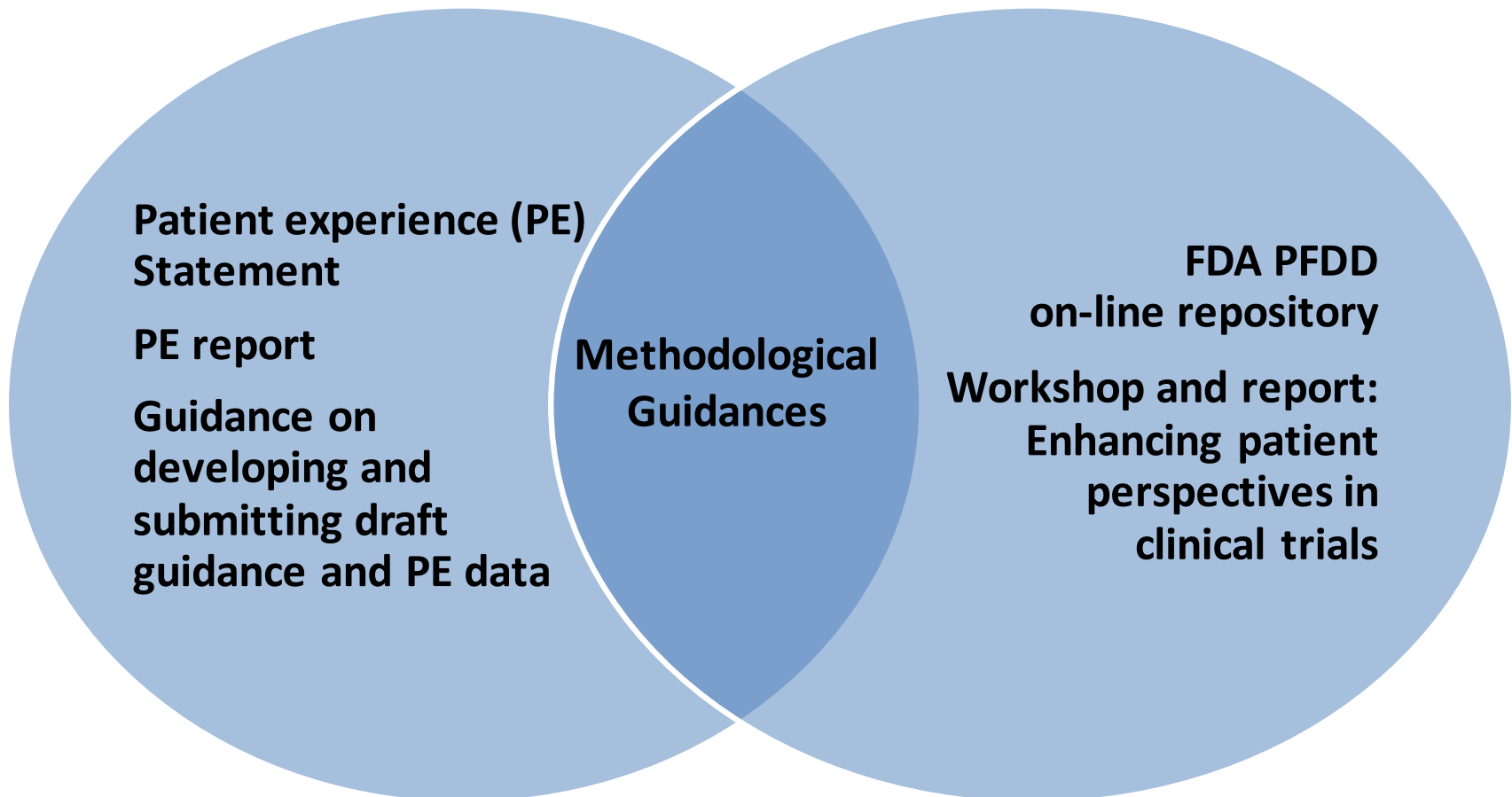


Regular cross-cutting agency meetings

# CBER Collaborations with CDER and CDRH

- Drugs and Biologics
  - Patient Focused Drug Development –FDA and externally led meetings
  - PFDD guidance documents
  - Patient experience data included in review
  - Outreach
- Medical Devices
  - Guidance documents (e.g., patient preference and patient engagement)
  - Outreach

# 21<sup>st</sup> Century Cures and PDUFA VI



# CBER Patient Engagement Groups



CBER Patient Engagement Workgroup

CBER Rare Disease Coordinating Committee

Science of Patient Input Initiative

# How CBER Works with Individual Patients and Patient Advocacy Groups

## Medical product and policy development

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

## Advancing efforts to strengthen FDA-patient community relationship

- Patient Engagement Collaborative

## Opportunities for one-on-one interaction or for addressing patient-specific needs:

- Patient organizations: request a meeting: [CBERPatientEngagement@fda.hhs.gov](mailto:CBERPatientEngagement@fda.hhs.gov)
- Expanded Access requests

# Upcoming topic: Uncommon diseases

- Some larger diseases becoming ‘smaller’
  - As we learn more about different genetic mutations underlying what otherwise appears to be one disease
- How to develop products for these diseases
  - Targeting different mutations occurring in one disease
- Patient input important
  - every patient counts
- More to come – e.g., workshops

*Thank you!*





# Patient Engagement in Action: The Patient Perspective

Theresa Strong, PhD

Director of Research Programs

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# Prader-Willi Syndrome

- Rare neurodevelopmental disorder that occurs spontaneously in ~1/15,000 births
- Due to loss of imprinted genes on chr 15q11-13
- Constellation of clinical symptoms: endocrine abnormalities, cognitive disability, behavioral/mental health challenges and abnormal hunger drive → morbid obesity
- Growth hormone therapy approved in 2000; does not improve hyperphagia
- Strict environmental control needed to prevent obesity
- Several new drugs in clinical trials



# Why is the patient perspective important?



- PWS is one of thousands of rare disorders
- Provides context and nuance to the 'laundry list' of associated symptoms and challenges
- Wide spectrum of challenges and severity in PWS – breadth of the patient community needs to be represented
- Natural history may be a moving target

# How has the PWS community brought the patient perspective to the FDA and medical product development?

- Generating natural history data: NIH RDCRN, Global PWS Registry
- Sharing Patient Experience Data: disease impact, unmet medical need, treatment priorities, risk tolerance
- International PWS Clinical Trials Consortium – Critical Path Innovation Meeting



- Working directly with sponsors

# Challenges and Lessons

- Resources are limited and there is much to do - define those critical gaps that patients are well-positioned to inform
- Learn basics of FDA mission and scope, as well as entry points for patient engagement
- Navigating FDA is a challenge, but the best thing to do is to jump in
- Start small, start early
- Ensure data stays with the patients/patient community
- Ongoing interaction is important to continue to build knowledge



# Patient Engagement Collaborative

Collaborating with FDA and CTTI to enhance patient engagement across medical product development / regulatory decision making process.

## Highlights:

- Assist FDA in being more welcoming, approachable
- Broaden reach and encourage more diversity in engagement
- Enhance the accessibility of information, so that patients effectively navigate, engage, and convey their perspective
- Educate stakeholders on the importance of patient engagement

# Looking ahead – optimizing effective patient engagement

- New resources to educate and support patients and patient groups as they engage with FDA
- New opportunities for engagement
- Continued work of FDA, CTTI, PEC, and patient advocacy groups to increase knowledge, transparency and effectiveness of incorporating patient perspectives
- Focus on reaching a broader patient population through multiple avenues



**Patient-Focused Drug Development: Collecting Comprehensive and Representative Input**  
Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

Learn more about Patient Listening Sessions:

[How does FDA benefit from Patient Listening Sessions?](#)

[How do I request a Patient Listening Session?](#)

[How do I prepare for a Patient Listening Session?](#)

[What happens after a Patient Listening Session?](#)

Patient-Led Listening Sessions are currently being scheduled in 2020.



## Audience Q&A

# Patient Engagement Across FDA

- **FDA Patient Affairs Staff:**  
[PatientAffairs@fda.gov](mailto:PatientAffairs@fda.gov)

- FDA Patient Representative Program:  
[FDAPatientRepProgram@fda.hhs.gov](mailto:FDAPatientRepProgram@fda.hhs.gov)

- Patient Engagement Meeting Requests:  
[CDRH\\_PatientMeetings@fda.hhs.gov](mailto:CDRH_PatientMeetings@fda.hhs.gov)

- CDRH's Division of Industry and Consumer Education:  
[DICE@fda.hhs.gov](mailto:DICE@fda.hhs.gov)

**Office of the  
Commissioner**

**Center for  
Biologics**

**Center for  
Devices**

**Center for  
Drugs**

- CBER's Patient Engagement Initiatives:  
[CBERPatientEngagement@fda.hhs.gov](mailto:CBERPatientEngagement@fda.hhs.gov)

Office of Communication, Outreach and Development:  
[OCOD@fda.hhs.gov](mailto:OCOD@fda.hhs.gov)

- Professional Affairs and Stakeholder Engagement:  
[CDERPASE@fda.hhs.gov](mailto:CDERPASE@fda.hhs.gov)
- CDER Division of Drug Information:  
[DrugInfo@fda.hhs.gov](mailto:DrugInfo@fda.hhs.gov)
- Patient Focused Drug Development:  
[patientfocused@fda.hhs.gov](mailto:patientfocused@fda.hhs.gov)

# THANK YOU.



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[www.ctti-clinicaltrials.org](http://www.ctti-clinicaltrials.org)