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

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Patient Engagement in Action: Insights from Patients & the FDA

November 21, 2019
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

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

- Clinical Investigators
- Patients, Caregivers & Patient Advocacy Groups
- Academia
- Trade & Professional Orgs
- IRBs
- Industry (pharma, bio, device, CRO, & tech)
- Government & Regulatory Agencies

Everyone is an equal partner at the table
CTTI Membership

*Version: August 06, 2019*
### CTTI Activities

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<th>Patient Engagement</th>
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### Mobile Clinical Trials

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<td>Decentralized Clinical Trials</td>
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<td>Engaging Patients and Sites</td>
<td>Antibacterial Drug Development</td>
<td>Safety Reporting</td>
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<td>Large Simple Trials</td>
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<td>Using FDA Sentinel for Trials</td>
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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
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**

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

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

- **2000s**
  - FDA working group established to discuss FDASIA section 1137 (evolved to become Patient Council)
  - FDA Patient Network web page launched
  - Patient-Focused Drug Development initiative established under PDUFA V

- **2012**
  - FDA Patient Representative Program℠ expands, patients now serve as consultants to reviewers during review cycle
  - FDA establishes Health Professional Liaison Program
  - MedWatch encourages voluntary reporting

- **2013**
  - First FDA Patient Council meeting held

- **2015**
  - FDA-EEMA Patient Engagement Cluster founded

- **2016**
  - FDA announces launch of Patient Engagement Advisory Committee (PEAC)

- **2017**
  - FDA-EEMA Patient Engagement Cluster inaugural meeting

- **2018**
  - FDA’s PAS establishes MOU with NORD-pilot listening sessions
  - PFDD Draft Guidance 1 released
  - Patient Engagement Collaborative (PEC) inaugural meeting
  - FDA’s PAS launches Patients Matter video series
  - PFDD Guidance 2 & 3 public meeting

- **2019**
  - FDA’s PAS establishes online patient portal, PatientsAskFDA
  - PAS/NORD pilot listening sessions completed, Staff Manual Guide released
  - PFDD Draft Guidance 2 released

**KEY**
- CDRH – Center for Devices and Radiological Health
- FDASIA - Food and Drug Administration Safety and Innovation Act
- EMA - European Medicines Agency
- CTTI - Clinical Trials Transformation Initiative
- NORD - National Organization for Rare Disorders
- PFDD – Patient-Focused Drug Development
- PAS – Patient Affairs Staff
Patient Affairs Staff (PAS) 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.

Who we are

- 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:
• 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
Submit Questions & Meeting Requests

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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<tr>
<th>Center for Medical Devices and Radiological Health (CDRH)</th>
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<tr>
<td>- Partner with Patients</td>
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<td>- Patient Engagement Advisory Committee</td>
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<td>- Patient and Care-Partner Connection Program</td>
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<th>Center for Biologics Evaluation and Research (CBER)</th>
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<td>- Interactive Meetings with Patients</td>
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<td>- CBER Workgroups:</td>
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<td>- CBER Patient Engagement Workgroup</td>
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<td>- CBER Rare Disease Coordinating Committee</td>
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<td>- CBER Science of Patient Input (SPI) Team</td>
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When in doubt...contact Patient Affairs!

PatientAffairs@fda.gov
301-796-8460

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

1. Patient Engagement
2. Clinical Outcome Assessments
3. Patient Preference Information
4. Patient-Generated Health Data
Science of Patient Input

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

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

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

*draft definition
**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.

<table>
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<tr>
<th>Consistent Regulatory Review</th>
<th>Culture of Patient Engagement</th>
<th>Optimized Research Roadmap</th>
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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/drainage
  - 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 22nd, 2017 10am – 3:30pm
U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Please contact XXX to confirm attendance at XXXXX by May XX, 2017.
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.
## CDRH Patient & Caregiver Connection Pilot Organizations

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<td>NORD</td>
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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

- 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

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

Patient Engagement in Clinical Investigations

2017 PEAC Meeting on Patient Engagement in Medical Device Clinical Trials

2018 Discussion Document Posted for PEAC Meeting

2019 Draft Guidance Posted

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

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

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

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

Resources

CDRH Websites on Patient Engagement:

CDRH Websites on Patient Science:
Pre-Submissions: https://go.usa.gov/xmVsh
Patient Preference Information:
https://go.usa.gov/xmVHG
Patient-Reported Outcomes:

Contacts for Medical Devices

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

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

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

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

- 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!
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
21st Century Cures and PDUFA VI

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

Methodological Guidances

FDA PFDD on-line repository
Workshop and report: Enhancing patient perspectives in clinical trials
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
- 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
Foundation for Prader-Willi Research
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
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
Audience Q&A
Patient Engagement Across FDA

- FDA Patient Affairs Staff: PatientAffairs@fda.gov
- FDA Patient Representative Program: FDAPatientRepProgram@fda.hhs.gov
- Patient Engagement Meeting Requests: CDRH_PatientMeetings@fda.hhs.gov
- CDRH’s Division of Industry and Consumer Education: DICE@fda.hhs.gov
- Office of the Commissioner
  - CBER’s Patient Engagement Initiatives: CBERPatientEngagement@fda.hhs.gov
  - Office of Communication, Outreach and Development: OCOD@fda.hhs.gov
- Center for Biologics
  - Professional Affairs and Stakeholder Engagement: CDERPASE@fda.hhs.gov
- Center for Devices
- Center for Drugs
  - CDER Division of Drug Information: DrugInfo@fda.hhs.gov
  - Patient Focused Drug Development: patientfocused@fda.hhs.gov
THANK YOU.

Pamela.tenaerts@duke.edu

www.ctti-clinicaltrials.org