How FDA Involves Patient Advocates

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Agenda

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Engage  Listen  Advocate

“Early and iterative engagement can improve clinical and regulatory understanding of diseases and conditions, provide a common understanding of the most urgent patient needs, and inform drug development programs.”

FDA Commissioner Scott Gottlieb, M.D.
Patient Affairs Staff (PAS)
Office of the Commissioner

• Established December 2017
• Works closely with the medical product centers and other offices in collaboration with patient communities to support and complement patient engagement efforts
• Reports to the Principal Deputy Commissioner for Food and Drugs
HIV/AIDS patient group founded 1988

1991

HIV/AIDS group expands to include cancer and other special health issues• First FDA Patient Representative sits on FDA Advisory Committee

1996

FDA Patient Representatives receive voting rights

2001

MedWatch encourages voluntary reporting

2006

FDA Patient Representative Program™ expands, patients now serve as consultants to reviewers during review cycle

2008

FDA announces launch of Patient Engagement Advisory Committee (PEAC)

2012

FDA Patient Network web page launched
Patient-Focused Drug Development initiative established under PDUFA V

2015

FDA holds inaugural Patient Engagement Advisory Committee meeting

2016

FDA-Patient Engagement Cluster founded
First FDA Patient Council meeting held

2017

FDA working group established to discuss FDASIA section 1137 (evolved to become Patient Council)

2018

FDA announces launch of Patient Engagement Advisory Committee (PEAC)

2013

FDA establishes Health Professional Liaison Program

2008

FDA Patient Representatives receive voting rights

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2018

FDA’s PAS establishes MOU with NORD- pilot listening sessions kick-off
PFDD Draft Guidance 1 released
1st Patient Engagement Collaborative (PEC) meeting
PFDD Guidance 2 & 3 public meeting

KEY
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
PAS Objectives

• Create and assist with public and private collaborations and partnerships to discuss regulatory topics of interest.
  ➢ We want to work with you!

• Provide leadership for cross-cutting programs and activities that can leverage best practices and enhance patient engagement.
  ➢ We work closely with the Centers to find ways to include your voice!

• Enhance FDA’s external communication platforms to expand public awareness and help patients, caregivers, and their advocates navigate FDA and the regulatory review process.
  ➢ We will listen to you so we can best meet your needs!
Objective 1:

PUBLIC & PRIVATE COLLABORATIONS AND PARTNERSHIPS
Patient Engagement Cluster

Mutual exchange on:

• Best practices to further enhance engagement activities, approaches, and ideas
• Approaches for engaging with and involving patient stakeholders
• High profile topics of mutual interest, especially those with potential high public interest
• Priorities and goals regarding future collaborations to enhance engagement
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
- Inaugural meeting – 29 August 2018
Objective 2:

CROSS-CUTTING PROGRAMS AND ACTIVITIES
Patient Listening Sessions
Rare Diseases Pilot

• Memorandum of Understanding with the National Organization for Rare Disorders (NORD)
• Enhance the incorporation of patient experience information into regulatory discussions
• Inform FDA review division staff what is important to patients (e.g., disease burden, risk tolerance, impacts on daily activities and QOL)
• Types:
  – FDA-requested (specific set of questions to ask of a particular patient sub-population)
  – Patient-requested (patient community wants to share their experiences and perspectives with the FDA)
• Assess the value to possibly expand to other therapeutic areas
What are Listening Sessions?

**ARE**

- Meant to facilitate *expeditious sharing of patient or advocate perspectives* on:
  - Disease burden
  - Treatment burden
  - Impact on daily activities
  - Quality of life
  - Priorities to consider in medical product development programs

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**Are NOT**

- **Public, advisory** discussions between FDA staff and *patients, their caregivers, or their advocates*
- Open to *industry*
- Avenues for the endorsement of *specific medical products*
- Able to guarantee *representative or comprehensive perspectives* on disease or treatment burden
- Meant to take the place of *other patient input and engagement processes*, e.g., the FDA Patient Representative Program, Patient-Focused Drug Development (PFDD) Meetings
Objective 3:

ENHANCING FDA COMMUNICATION WITH PATIENTS
A Central Entry Point

• A place for patients and patient advocacy stakeholders to start
• Developing a triage system to direct inquiries to appropriate center or office
  – Working closely with CBER, CDER, CDRH & other offices (e.g., OEA)
• Leveraging CDER’s External Stakeholder Meeting Request (ESMR) System
Patient Outreach and Education

• Managing a database of patient advocacy organizations
• Email communications to patient stakeholders
• Expanding our use of social media
• Educational video series

Contacts: PatientAffairs@FDA.gov

@FDAPatientInfo

Patients Matter
Improving FDA’s *For Patients* webpage

www.fda.gov/forpatients
Other FDA initiatives

CENTERS
Center Initiatives

Center for Drug Evaluation and Research

- 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

Center for Biologics Evaluation and Research

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

Center for Devices and Radiological Health

- Partner with Patients
- Patient Engagement Advisory Committee
- Patient and Care-Partner Connection Program (under development)
Contacts

Rare Disease Listening Sessions Pilot and Patient Engagement Collaborative (Patient Affairs Staff): patientaffairs@fda.hhs.gov

FDA Patient Representative Program: FDAPatientRepProgram@fda.hhs.gov

Patient Focused Drug Development: patientfocused@fda.hhs.gov

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

CDER’s Professional Affairs and Stakeholder Engagement: CDERPASE@fda.hhs.gov

CDRH’s Division of Industry and Consumer Education: DICE@fda.hhs.gov
When in doubt...

Contact PAS!

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Thank you!

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