U.S. FOOD & DRUG ADMINISTRATION

How FDA Involves Patient Advocates

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Partners in Progress: Cancer Patient Advocates and FDA Public Workshop II

How Patients Engage
November 27, 2018

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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."

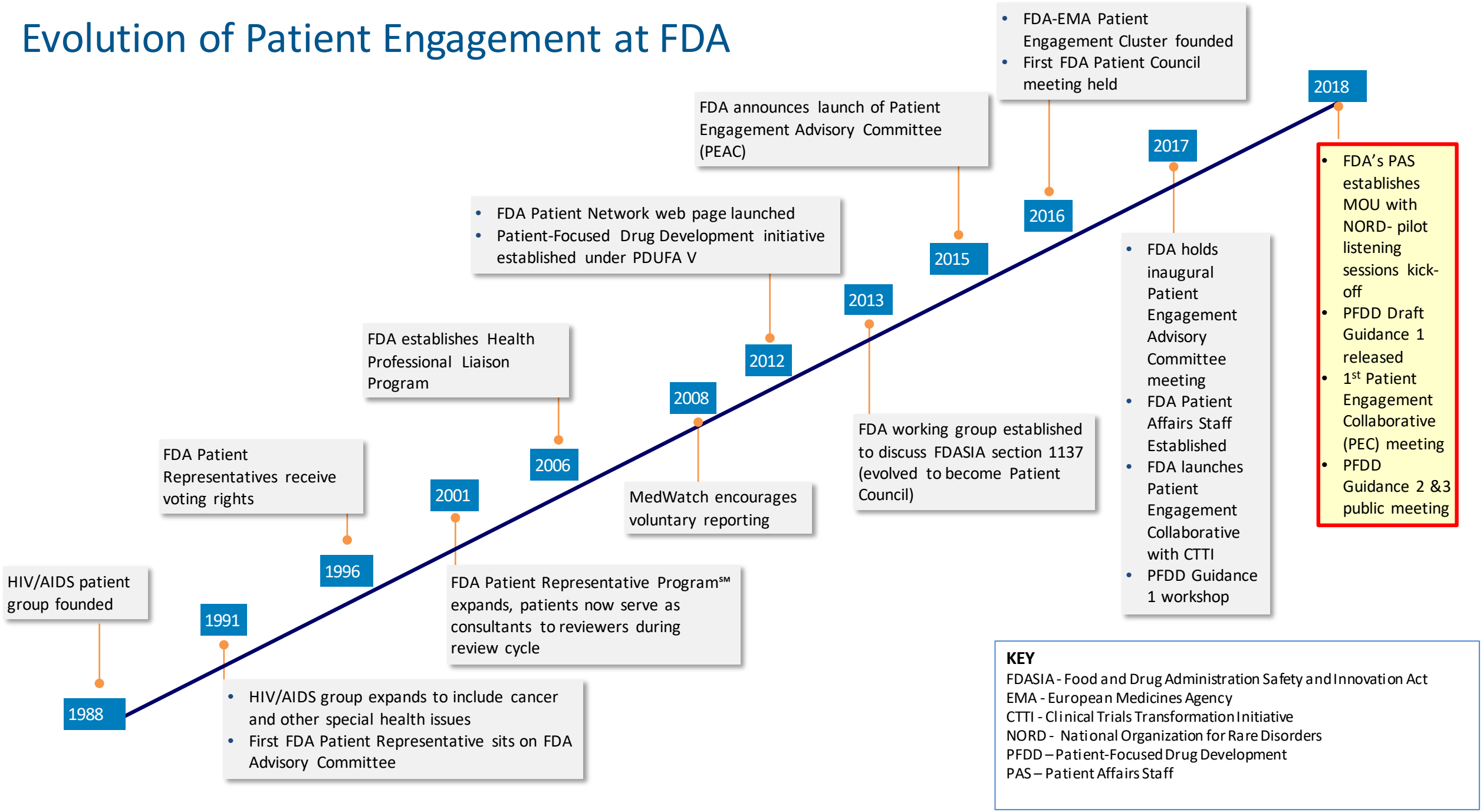
FDA Commissioner Scott Gottlieb, M.D.

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

Evolution of Patient Engagement at FDA

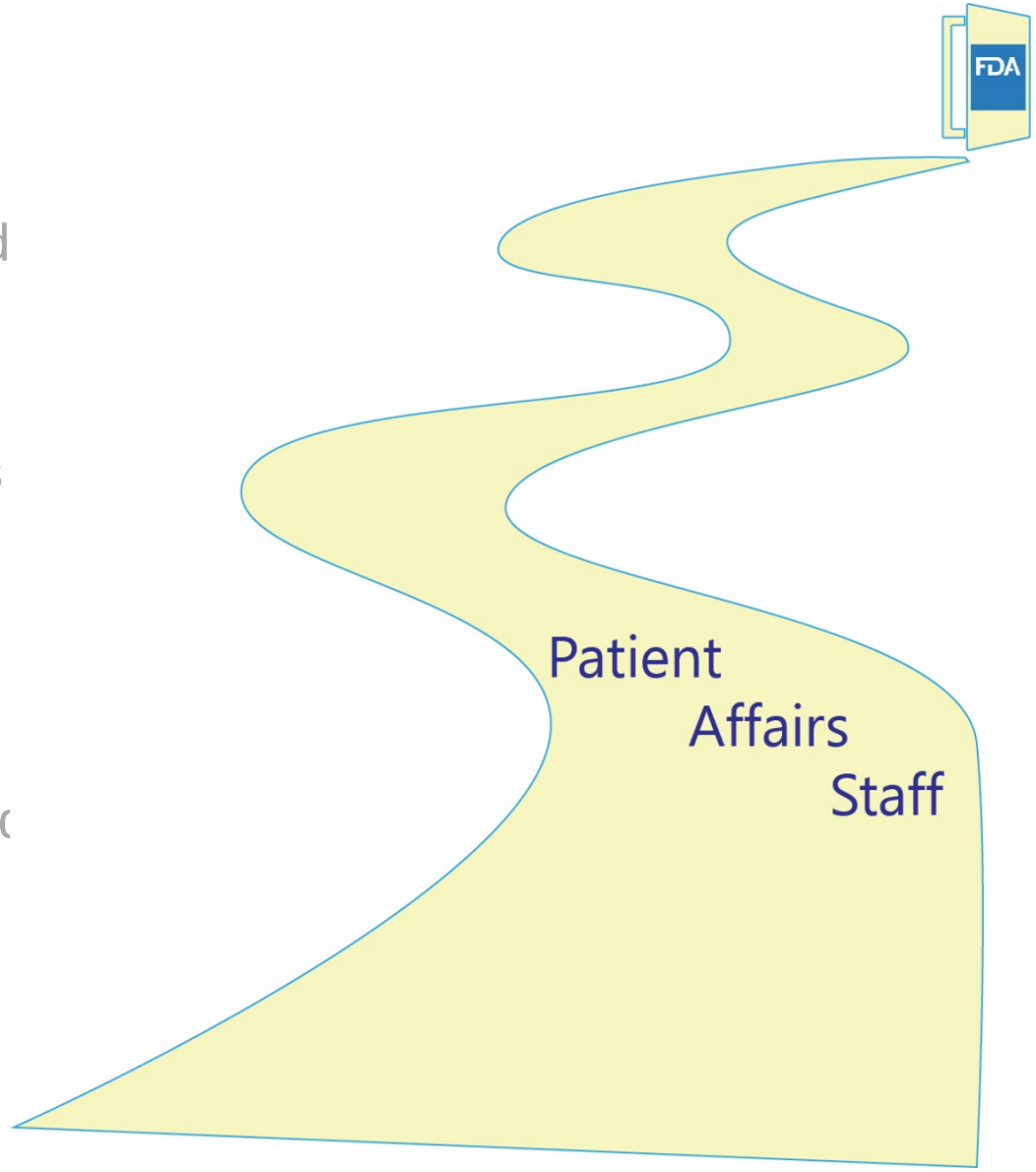


KEY

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

PEC Members 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

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

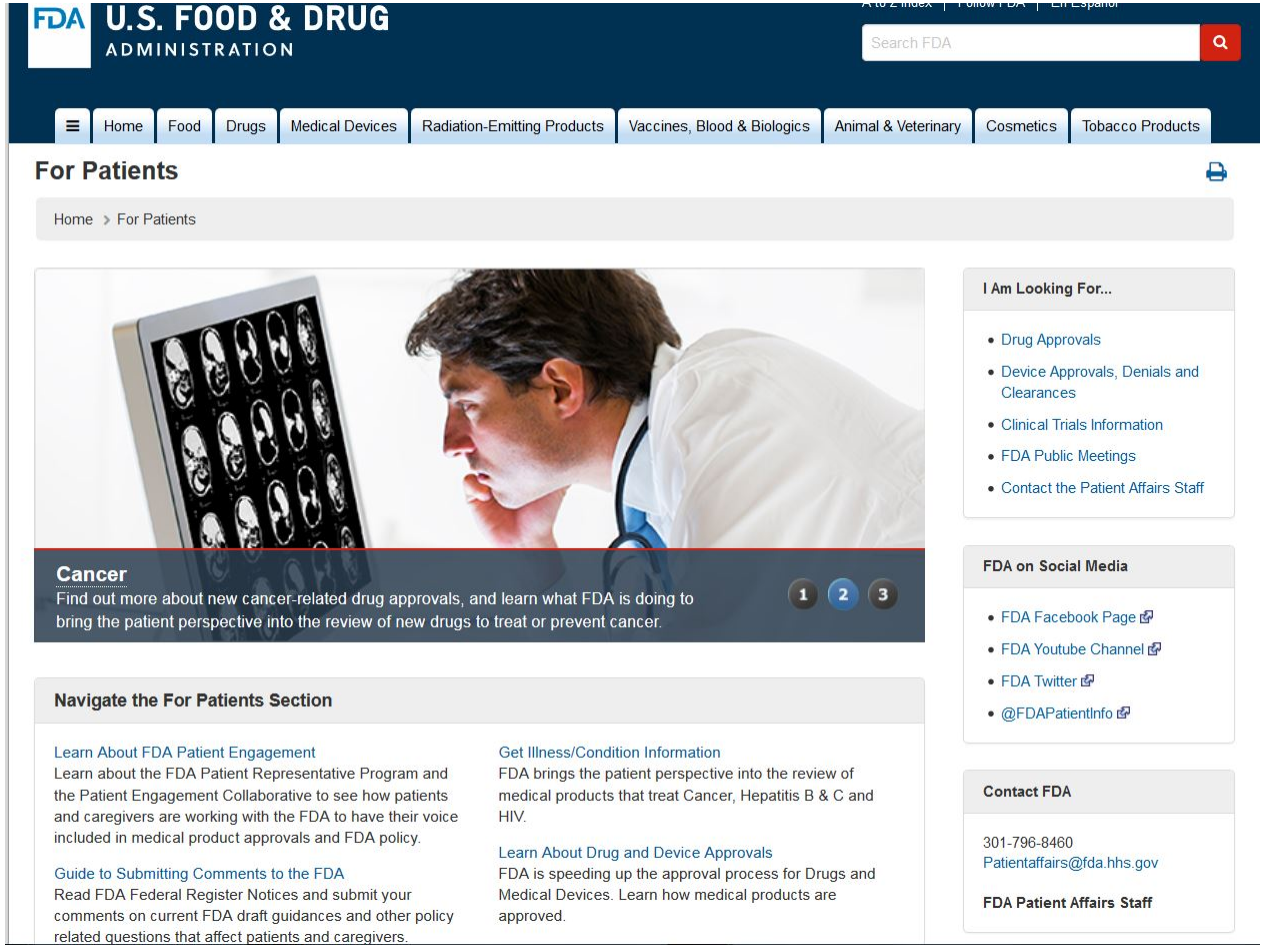
Contacts

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

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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

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When in doubt…

Contact PAS!

PatientAffairs@FDA.gov **@FDAPatientInfo** **301-796-8460**

Thank you!

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