U.S. FOOD & DRUG ADMINISTRATION

FDA's Patient Representative Program[SM]

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FDA Public Workshop: Partners in Progress 2018 - Cancer Patient Advocates and FDA

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FDA Patient Representative Program

Agenda

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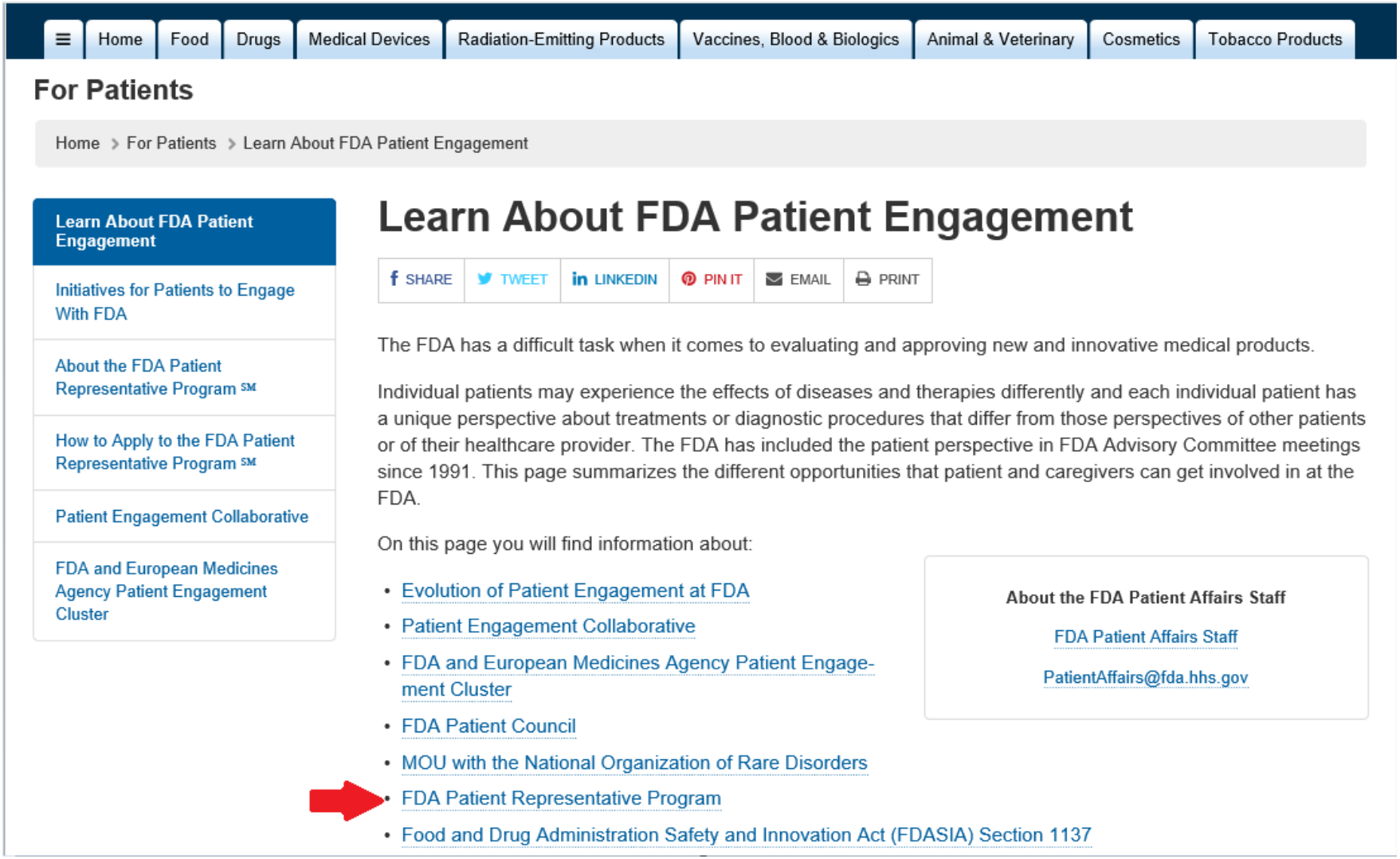
FDA Patient Representative Program



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For Patients

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- Initiatives for Patients to Engage With FDA
- About the FDA Patient Representative Program SM
- How to Apply to the FDA Patient Representative Program SM
- Patient Engagement Collaborative
- FDA and European Medicines Agency Patient Engagement Cluster

Learn About FDA Patient Engagement

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The FDA has a difficult task when it comes to evaluating and approving new and innovative medical products.

Individual patients may experience the effects of diseases and therapies differently and each individual patient has a unique perspective about treatments or diagnostic procedures that differ from those perspectives of other patients or of their healthcare provider. The FDA has included the patient perspective in FDA Advisory Committee meetings since 1991. This page summarizes the different opportunities that patient and caregivers can get involved in at the FDA.

On this page you will find information about:

- Evolution of Patient Engagement at FDA
- Patient Engagement Collaborative
- FDA and European Medicines Agency Patient Engagement Cluster
- FDA Patient Council
- MOU with the National Organization of Rare Disorders
- FDA Patient Representative Program
- Food and Drug Administration Safety and Innovation Act (FDASIA) Section 1137

About the FDA Patient Affairs Staff

FDA Patient Affairs Staff

PatientAffairs@fda.hhs.gov

https://www.fda.gov/ForPatients/PatientEngagement

FDA Patient Representative Program

How did it all start?

- Late 1980s: HIV/AIDS crisis.
- Patients wanted a more active role in FDA regulation of medical products.
- First Patient Representative Serves: Antiviral Drugs Advisory Committee, Feb. 13-14, 1991.
- 1991: FDA Patient Representative Program formed.

FDA Patient Representative Program

What is the FDA Patient Representative Program?

Mechanism for advocates (patients and caregivers) to provide formal input to the Agency's decision-making process as medical products (drugs, biologics, and medical devices) are regulated.

Special (or Regular) Government Employees

FDA Patient Representative Program

Two Primary Ways for Engaging with FDA

1. Serve on Advisory Committees (panel members)

2. Consult with Review Divisions (consultations)

FDA Patient Representative Program

Serve on Advisory Committees

- A panel of outside experts convened periodically to advise the FDA on safety and efficacy issues about regulated medical products.
- 31 Advisory Committees; Medical Devices Advisory Committee (18 panels)
- Committee members include:
  - *Chair*
  - *Medical Experts*
  - *Consumer Representative*
  - *Industry Representative*
  - *Patient Representative*

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Consult with Review Divisions

- Brings the patient voice earlier in the regulatory process.
- "Homework" assignments.
- Consult directly with scientific review staff and sponsors
- Closed meeting (telecon)

Experiences Represented

200 FDA Patient Representatives
300-500 diseases/conditions/device experiences

- AIDS/HIV
- Alzheimer's Disease
- Asthma
- Cancer (various)
- Cardiovascular disease
- Cerebral Palsy
- Crohn's disease
- Cystic Fibrosis
- Duchenne Muscular Dystrophy
- Diabetes
- Diabetes (insulin pumps)
- Fabry Disease
- Hepatitis B
- Hepatitis C
- Infantile Spasms
- Lung Transplantation
- Lupus
- Lysosomal Acid Lipase Deficiency
- Major Depressive Disorder
- Muscular Dystrophy
- Obesity/Weight Control
- Opioid Use
- Parkinson's Disease
- Pompe Disease
- Sickle Cell Disease
- Short Bowel Syndrome
- Temporomandibular joint disorder
- Transplantation

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How Do We Recruit?

- National Patient Advocacy Organizations
- Regional or Local Organizations
- Health Care Providers
- FDA Centers and Divisions
- Agency-Sponsored Meetings and Activities (work with PAS)
- Self-Nominations
- FDA Patient Representatives
- Web, Email, Social Media

Building Relationships With Rare Disease Communities

Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

Solve ME/CFS Initiative

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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What CRITERIA Do We Use?

- Personal experience with the disease or condition, either as a patient or primary caregiver.
- Patient community awareness: active with patient advocacy organizations.
- Knowledgeable about treatment options and research.
- Someone who is analytical and objective, doesn't need to be a scientist but should grasp scientific principles and understand issues, experienced with decision making based upon complex information.
- Good communications skills.
- Commitment to serve.
- Minimal or no financial or ethical conflicts of interest.

FDA Patient Representative Program

We Train and Prepare!!!

- Describe significance of program
- Describe FDA regulatory framework and decision-making process (FDA 101)
- Share experiences: internal and peer
- Describe scenarios for the meeting
- Provide online resources for patients
- Share agency activities

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Areas of Input

Provides FDA with insight on issues, problems, and/or questions pertinent to the viewpoint of patients and family members living with a specific serious or life-threatening disease.

Clinical Trial Design
Entry Criteria
Endpoints
Drug Toxicity Issues
Quality of Life Issues
Adverse Events

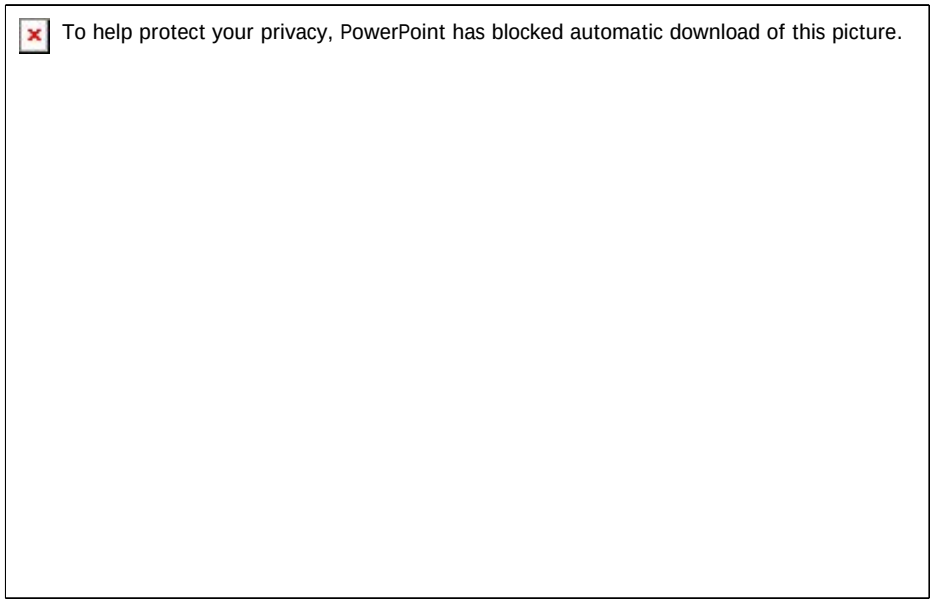
Study Recruitment (diversity, etc.)
Informed Consent
Expanded Access
Product Labeling
Risk/Benefit

FDA Patient Representative Program

As a result…

- Patients and caregivers having an active role on FDA Advisory Committees and in consultations with review divisions.
- Patient voice represented in important discussions about regulatory decision-making.
- Furthers an understanding and appreciation for FDA's role in medical product development, review and patient protection.
- Presence at the table.

FDA Patient Representative Program



THANK YOU!!

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https://www.fda.gov/ForPatients/PatientEngagement/