U.S. FOOD & DRUG ADMINISTRATION

FDA Patient Affairs Staff

How FDA Involves Patient Advocates

Susan Chittooran, MSW

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

#OCEPIP19

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Patient Affairs Staff

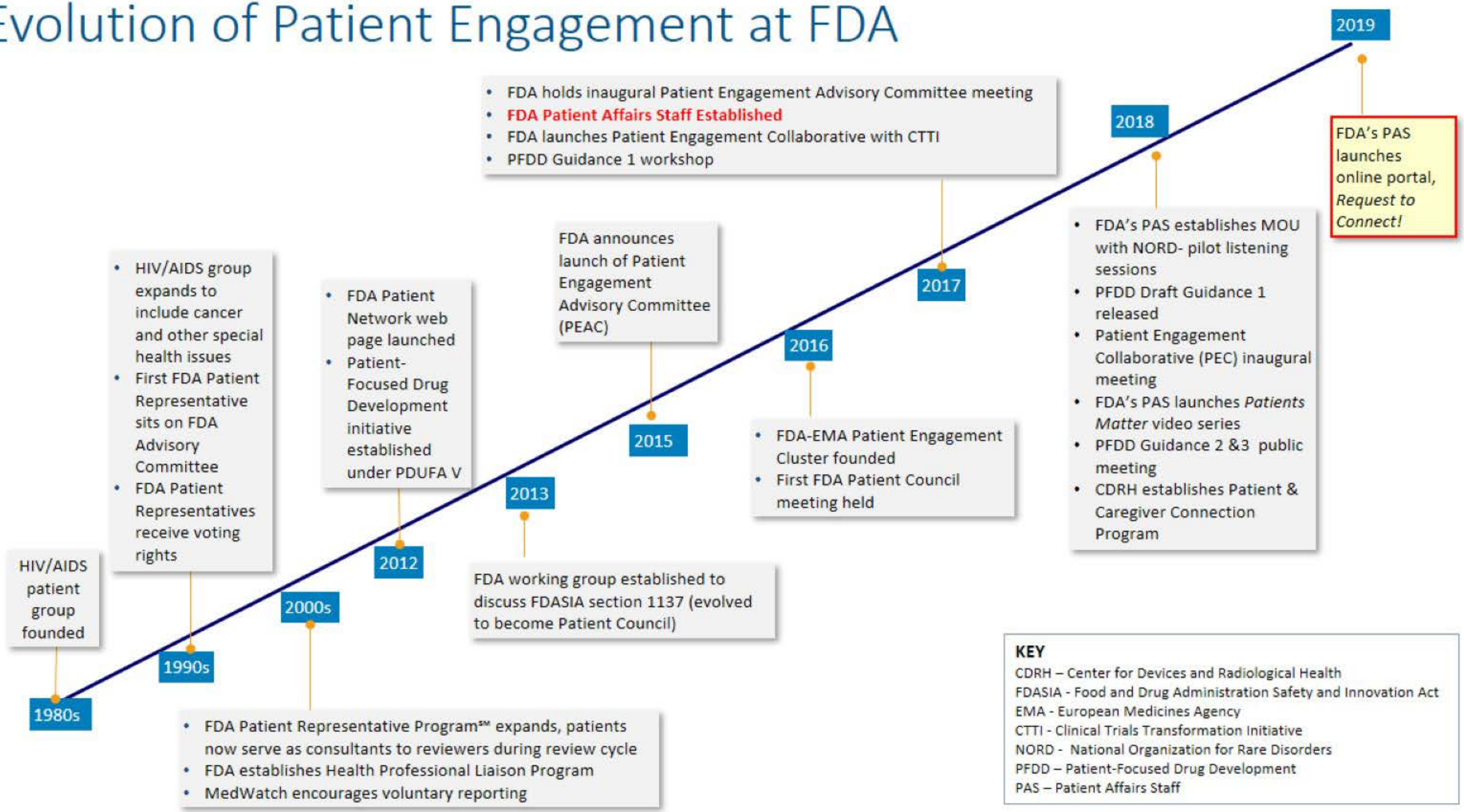
Who We Are & What We Do

The Importance of the Patient Voice

- Provide insight on issues, concerns, needs and priorities that are important to patients and caregivers
- Have diverse opinions and experiences
- Provide insights on risk tolerance and potential benefit
- Provide real world experience

Patients are at the heart of FDA's work

Evolution of Patient Engagement at FDA

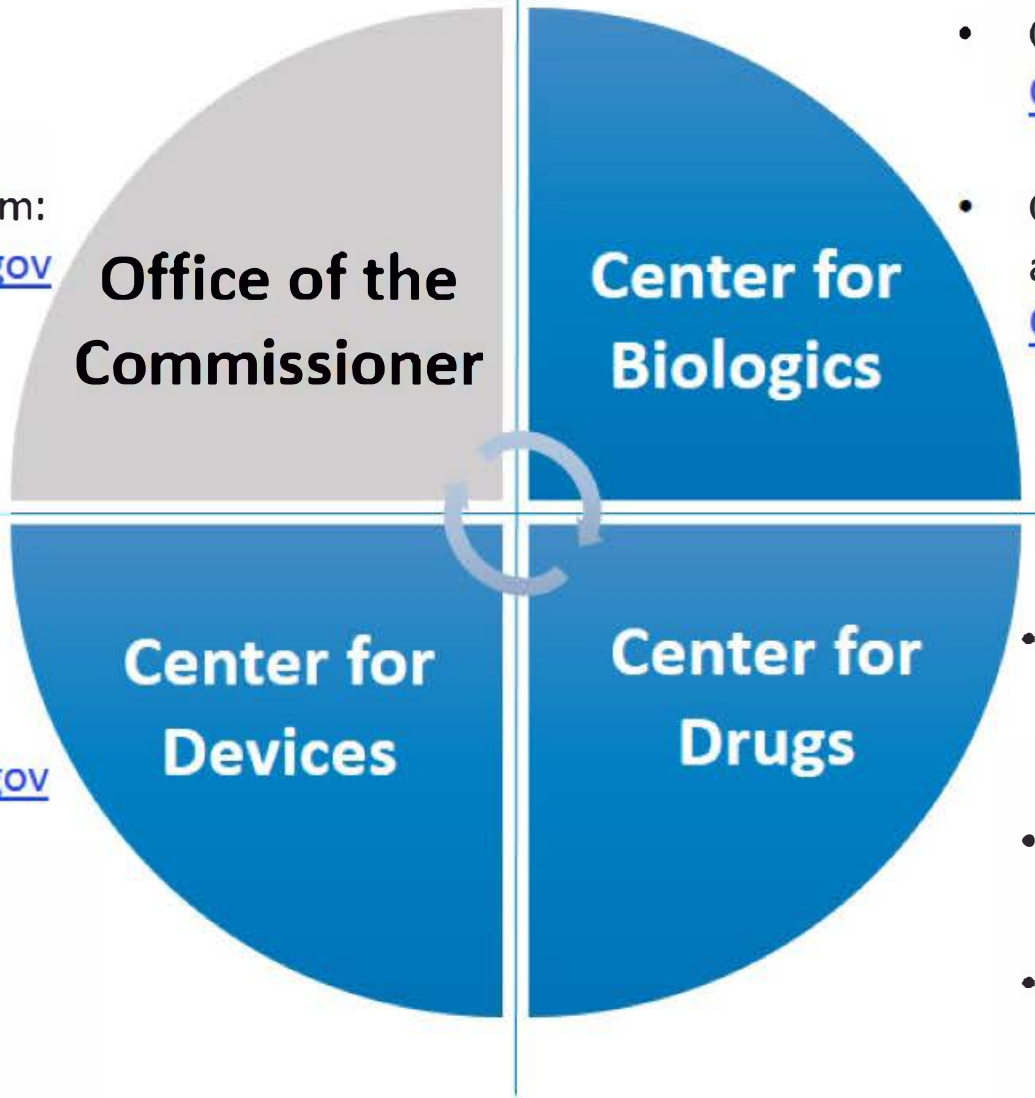


KEY

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 Engagement Across FDA

- **FDA Patient Affairs Staff:** PatientAffairs@fda.gov
- FDA Patient Representative Program: FDAPatientRepProgram@fda.hhs.gov



- CBER's Patient Engagement Initiatives: CBERPatientEngagement@fda.hhs.gov
- Office of Communication, Outreach and Development: OCOD@fda.hhs.gov

- Patient Engagement Meeting Requests: CDRH_PatientMeetings@fda.hhs.gov
- CDRH's Division of Industry and Consumer Education: DICE@fda.hhs.gov

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

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., FDA's *For Patients* webpage, social media, etc.)

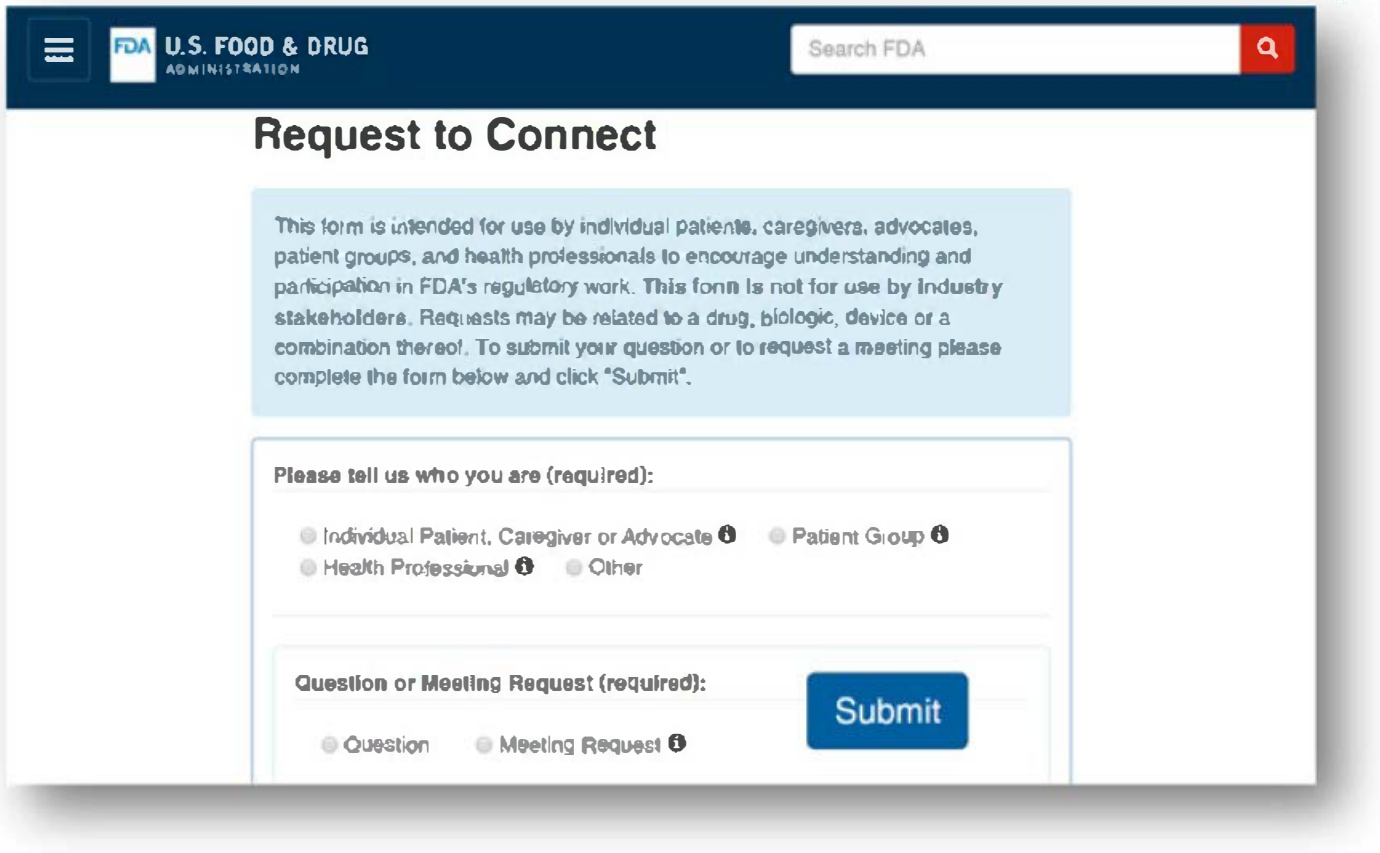
Patient Affairs Staff (PAS)

Our Programs and Initiatives

- FDA & EMA Patient Engagement Cluster
- Patient Engagement Collaborative
- FDA Rare Disease Listening Sessions
- FDA Patient Council

Enhancing Communication With Patients

Submit Questions & Meeting Requests



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FDA Rare Disease Listening Sessions

FDA Patient Listening Sessions

Rare Diseases Pilot

- 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
- Assess the value & establish a process document
- Provide a starting point to inform early stage research & development

Understanding FDA Rare Disease Listening Sessions

What are Listening Sessions?

ARE

- **Non-public, non-advisory** discussions between FDA staff and **patients, their caregivers, and/or their advocates**
- **1 to 2 hour** meetings
- Via **phone, in person** at FDA or a **mix of the two**
- Meant to facilitate **expeditious sharing of patient or advocate perspectives** on:
  - Disease burden
  - Treatment burden
  - Impact on daily activities
  - Priorities to consider in medical product development programs

Are NOT

- 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

FDA Rare Disease 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

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FDA Rare Disease Listening Sessions

Previously Conducted Listening Sessions

FDA Review Division-Requested Listening Sessions

May 13, 2019 - **Sanfilippo Syndrome**

February 20, 2019 - **Celiac Disease**

December 4, 2018 – **Fabry Disease**

October 23, 2018 – **Gene Therapy as a Treatment Modality for Hemophilia**

Patient-Requested Listening Sessions

September 17, 2019 – **Osteogenesis Imperfecta (OI)**

August 7, 2019 - **Osteoarthritis (OA)**

June 13, 2019 - **Neurofibromatosis (NF)**

May 29, 2019 - **Fibrodysplasia Ossificans Progressiva (FOP)**

January 16, 2019 - **Amyotrophic Lateral Sclerosis (ALS)**

November 5, 2018 - **Biliary Atresia, Progressive Familial Intrahepatic Cholestasis, Wilson's Disease**

FDA Patient Representative Program

FDA Patient Representative Program ℠

FDA Patient Representative℠ consultants provide direct input to the Agency's decision-making process in over 300 diseases and conditions and participate on FDA Advisory Committees and in review division assignments.

Criteria for becoming an FDA Patient Representative:

When a Patient Speaks Video

When a Patient Speaks Video Link

Link to Peg Ford Experience Ovarian Cancer video You Tube (Time 2 mins 20 seconds)

https://www.youtube.com/watch?v=UVan4qU27XA&feature=youtu.be.

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.

FDA Patient Representative Program

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

- AIDS/HIV
- Alzheimer's Disease
- Asthma
- Breast implants
- 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 Lypase Deficiency
- Major Depressive Disorder
- Muscular Dystrophy
- Nicotine Replacement Therapy
- Opioid Use
- Organ transplantation
- Parkinson's Disease
- Pompe Disease
- Sickle Cell Disease
- Short Bowel Syndrome
- Temporomandibular joint disorder

FDA Patient Representative Program

Two Primary Ways
FDA Patient Representatives Engage with FDA

1. Serve on FDA Advisory Committees
2. Consult with FDA Review Divisions

FDA Patient Representative Program

What CRITERIA Do We Use?

- Personal experience with the disease or condition (patient or caregiver).
- Advocacy experience: active with patient community and understands issues and concerns.
- Knowledgeable about treatment options and research.
- Analytical and objective: should grasp basic scientific principles and issues.
- Good communications skills: comfortable with public speaking.
- Commitment to serve: reliable
- Remain unbiased and unconflicted: activities and roles should not conflict with assignment—financial, appearance, ethical considerations.

When in doubt…contact Patient Affairs!

PatientAffairs@fda.gov

301-796-8460

www.fda.gov/Patients

@FDAPatientInfo

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