Patient Involvement at FDA

Andrea Furia-Helms, MPH
FDA Patient Representative Program
Office of Health and Constituent Affairs
Food and Drug Administration (FDA)

White Oak Campus

Silver Spring, Maryland

FDA's Mission and Regulatory Philosophy

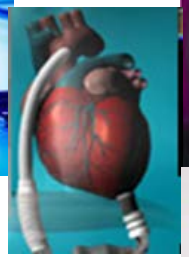
- *Protecting the public health* by assuring the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices
- *Advancing the public health* by helping to speed innovations that make medicines and foods more effective, safer, and more affordable; and helping the public get the accurate, science-based information they need to use medicines and foods to improve their health

FDA Product Centers

Medical (human)

- Center for Drug Evaluation and Research (CDER)
  - Drugs

- Center Devices and Radiological Health (CDRH)
  - Devices and electronic products that give off radiation

- Center for Biologics Evaluation and Research (CBER)
  - Vaccines, biologics and blood products


FDA Product Centers

Other

- Center for Food Safety and Applied Nutrition (CFSAN)
  - Food and cosmetics
- Center for Veterinary Medicine (CVM)
  - Animal and veterinary
- Center for Tobacco Products (CTP)
  - Tobacco products

Review Team

- Project Manager
- Medical Officer review all clinical studies
- Pharmacology/Toxicology Specialist
- Statistician
- Clinical Pharmacology/Biopharmaceutics Specialists
- Chemists/Biologists/Microbiologists

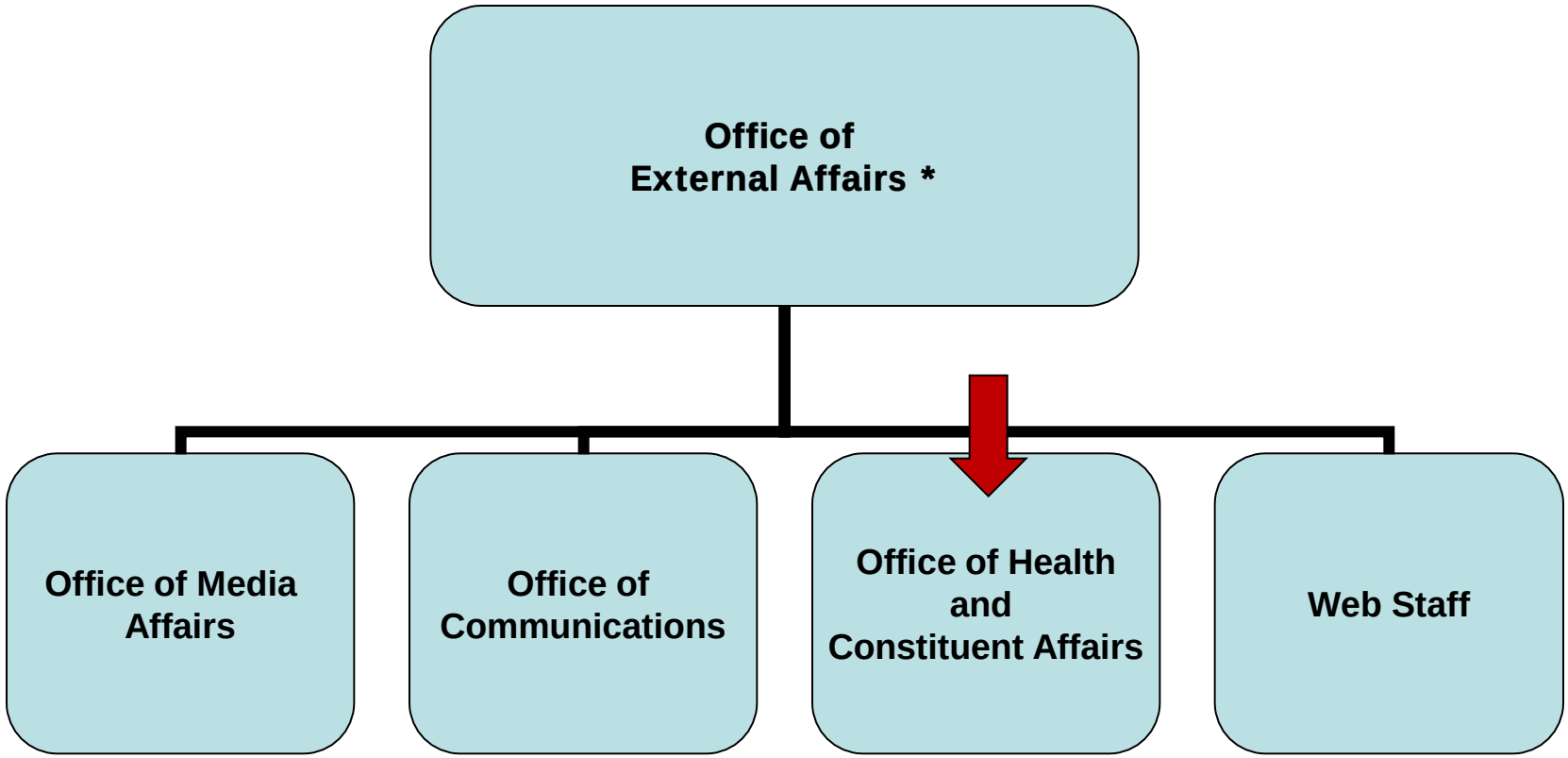
Review Team Responsibilities

- The review teams analyze new drug applications (NDAs) and biologic licensing applications (BLAs).
- During drug development, the teams review Investigational New Drug Applications (INDs).
- Review team members use their expertise to answer key questions:
  - Is it reasonably safe to study an investigational drug in humans and will proposed studies provide data needed to show safety and efficacy?
  - Is the drug safe and effective in its proposed use and do the benefits of the drug outweigh the risks?
  - Is the proposed labeling appropriate and if not what should it contain?
  - Are the methods used in manufacturing the drug and the controls adequate?

Office of the Commissioner

- Office of Chief Counsel
- Office of Chief Scientist
- Office of Legislation
- Office of Minority Health
- Office of Women's Health
- Office of Pediatric Therapeutics
- Office of Orphan Products
- Office of Policy and Planning
- Office of External Affairs (OEA)

OEA Organizational Structure



* Responsible for communications to the media, consumers, industry, patients, and health professionals

Office of External Affairs

Kathleen Quinn
Actg. Associate Commissioner

Office of Health and Constituent Affairs

Patient Liaison Program

Richard M. Klein
Program Director

LaKeecha (Keecha) Chenjo

Helene Clayton Jeter

Andrea Furia-Helms

Deborah Miller

Steve Morin

Salina Prasad

Heidi C. Marchand
Assistant Commissioner

Beth Fritsch
Deputy

Mary Hitch

Pat Kuntze

Kathy Duvall

Healthcare Professional Liaison Program

Anna Fine
Program Director

Stephanie Joseph

Cristina Klafhen

Teresa Rubio

Brenda Rose

Shannon Thor

Jay Wattenberg

Office of Health and Constituent Affairs (OHCA)

Patient Liaison Team

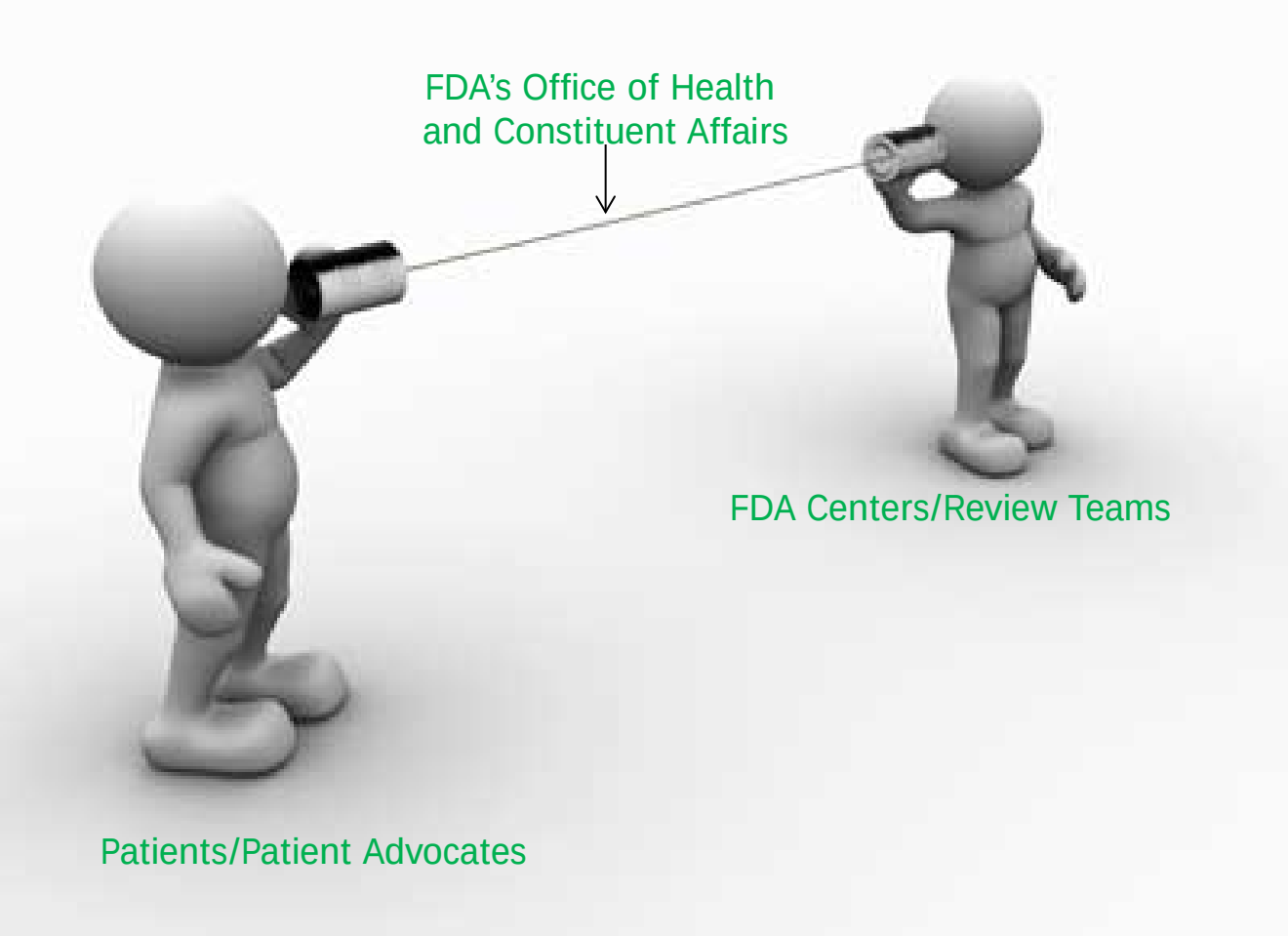
- Coordinates outreach and educational activities with patients, patient advocates and patient advocacy groups.

We listen.

We educate.

We advocate.

Making the Connection



OFFICE OF HEALTH AND CONSTITUENT AFFAIRS

PATIENT REPRESENTATIVE PROGRAM

Giving Voice to Patients

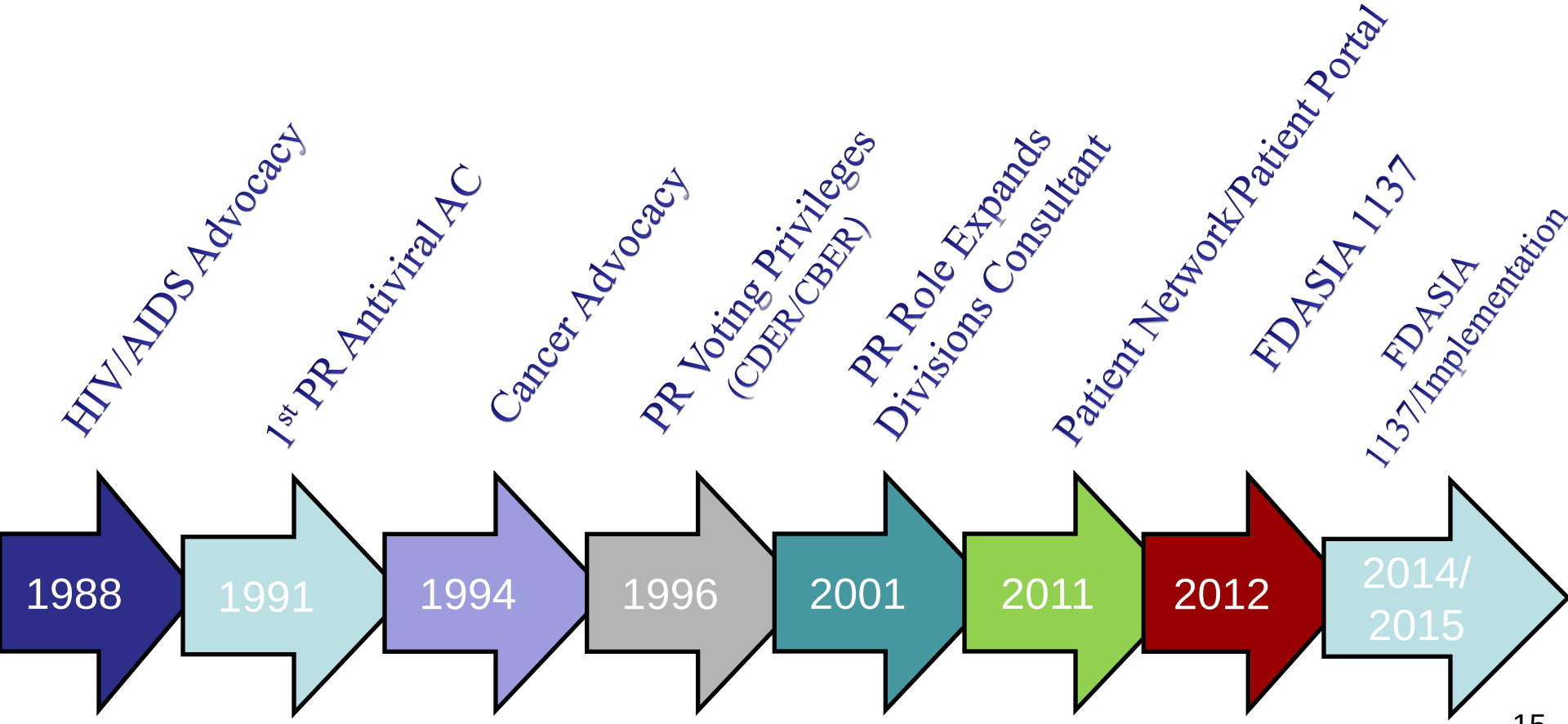
Patient Representative Program

Incorporates patient/community advocates' voices into advisory committee and division discussions

…and furthers an understanding and appreciation for FDA's role in medical product development, review and patient protection

2014 Patient Representative Workshop

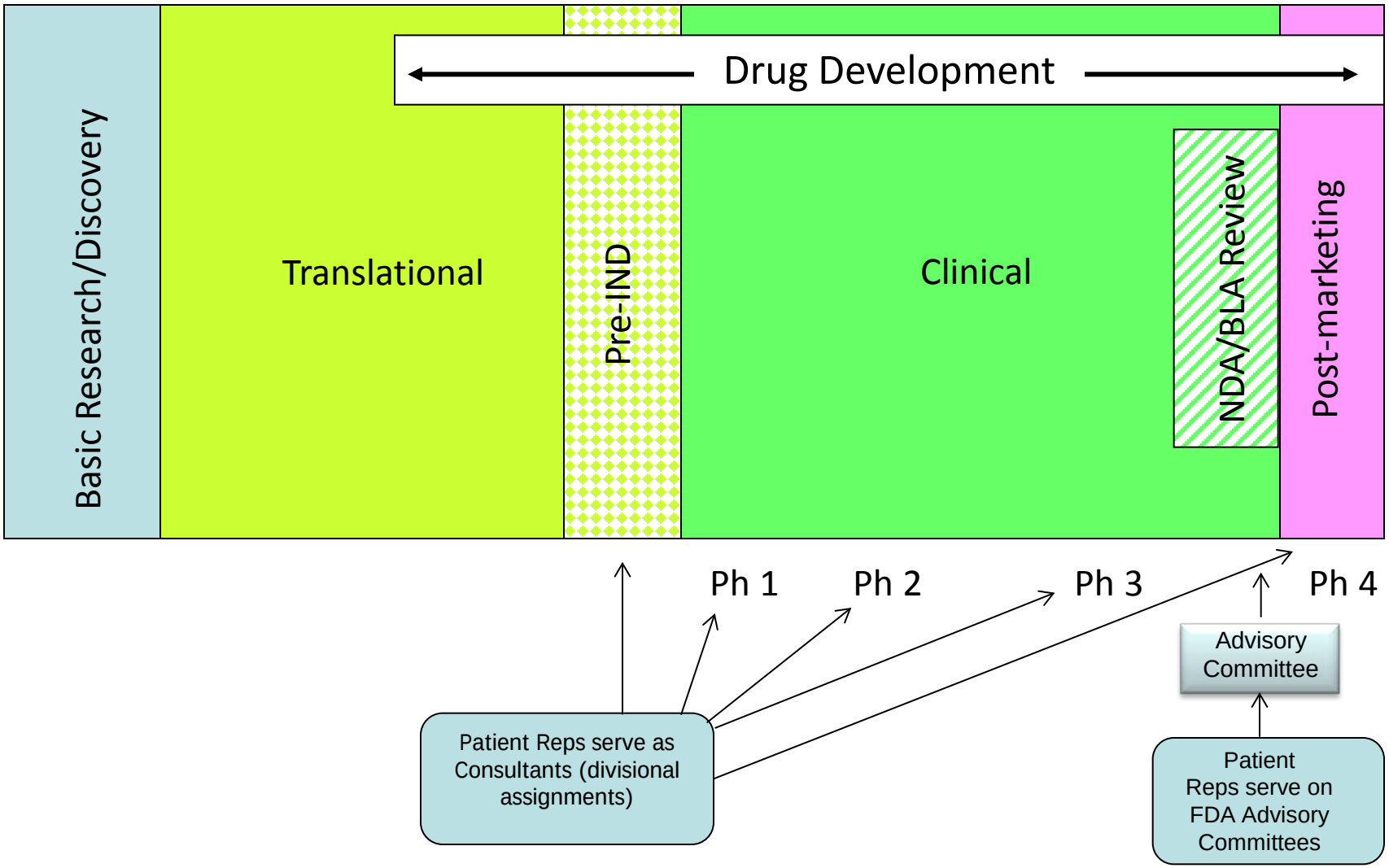
Patient Participation and Patient Rep Program Milestones



Requests for Patient Input

- Originate from review teams in medical product Centers (CDER, CBER, CDRH)
- Types of requests
  - Advisory Committee Meetings
  - Patient input in product development meetings
  - Consultation directly with review team
  - Listening sessions
  - Public workshops/meetings

Where Patient Reps Intersect



Who are Patient Representatives?

- Patients with a disease/condition
- Primary caregivers to patients (i.e., spouse, family member)
- Members of patient/community advocacy groups

Patients add value to FDA's decision making

Bring a diversity of opinion, viewpoint, and experience – patient advocates often think outside the box of a purely "scientific approach"

•Have a vested interest in conduct and outcome of trials leading to meaningful therapeutic options

•Provide "ground level" input that is based on personal and community experience – a *street sense*

•Help FDA understand how patients feel about risk tolerance to help FDA make better benefit/risk decisions.

Patient Representative Recruitment: Finding Candidates

- Advocacy organizations
  - Existing OHCA relationships
  - Online searches for new/virtual organizations
- Support groups (local and online)
- Online forums/blogs/interest groups
- Hospitals
- Academia/research institutions
- Researchers (principal investigators)
- Referrals from current Patient Representatives
- Other FDA meetings patients attend
- Open Public Hearing sessions at Advisory Committee meetings
- Attending conferences/events

Recruited as SGEs

(Special Government Employees)

- Rigorous Conflict of Interest screening
  - ❑ Investments
  - ❑ Employment
  - ❑ Officer positions in professional organizations
  - ❑ Consulting/advising
  - ❑ Contract/grants/CRADAS
  - ❑ Appearance of conflict
- Screened at initial recruitment and prior to product-specific assignment
  - ❑ Product at issue
  - ❑ Competing/Affected products

Criteria for Patient Representatives

- **Personal experience with the disease or condition**:
  - Patient
  - Primary caregiver (i.e., spouse, family member)
- **Have patient community awareness**:
  - Active in patient advocacy organizations or at least “plugged in”
  - Knowledgeable about treatment options and research in the disease area
  - Other advocacy activities
- **Someone who is analytical and objective**:
  - Doesn’t need to be a scientist, but should grasp scientific principles and understand the issues
  - Experience with decision making based on complex information
- **Good communication skills**:
  - Able to communicate thoughts and opinions to large group of scientific committee members.
- **Minimal or no conflicts of interest**

Patient Representative Recruitment: Next Steps

1) **Patient Rep Resume**
   - patient/caregiver experience
   - advocacy experience
   - ability to represent other patients
   - knowledge and skills related to disease area
   - alternate disease experience
   - special needs/accommodations
2) **Formal Phone Interview**
   - Questions based on criteria
   - Discuss conflict of interest (COI)
3) **SGE*/COI clearance through respective Center**

**Special Government Employee*

Snapshot of Current Patient Representatives

200 Reps | approx. 400 diseases/conditions

- AIDS/HIV
- Alzheimer's Disease
- Asthma
- Cancer (various)
- Cardiovascular disease
- Cerebral Palsy
- Crohn's disease
- Cystic Fibrosis
- Depression
- Diabetes
- Duchenne Muscular Dystrophy
- Fabry Disease
- Hepatitis B
- Hepatitis C
- Hypertension/Cardiovascular Disease
- Infantile Spasms
- Lung Transplantation
- Lupus
- Macular Degeneration
- Major Depressive Disorder
- Multiple Sclerosis
- Neuropathy
- Lysosomal Acid Lipase
- Obesity/Weight Control
- Parkinson's Disease
- Pompe Disease
- Polio
- Sickle Cell Disease
- Short Bowel Syndrome
- Temporomandibular joint (TMJ) disorder
- Urea Cycle Disorder

Training

- FDA 101 – Basic regulatory overview, interactive, usually conducted one-on-one by telephone
- Ongoing training
  - Regular webinars
  - Annual workshop
- Mentoring by senior patient representatives
- One-on-one support as needed

Quarterly newsletter keeps us in touch with representatives

U.S Food and Drug Administration

The Patient Representative Newsletter

Volume 3 | Issue 4 | October 2014

The Newsroom

Highlights from August 2014 Patient Representative Workshop

In August, nearly 30 newly recruited Patient Reps joined the FDA staff for our annual in-person training workshop designed to help teach participants about what's expected and involved in serving as official FDA "Special Government Employees." Participants, including a few experienced Patient Reps and other Federal agency representatives, were able to:

- Learn from experienced Patient Reps as they shared stories on how they have prepared for Advisory Committee meetings and consultations—the process from start to finish—and the many lessons learned.
- Engage face-to-face with OHCA staff and other FDA experts and learn more about how the regulatory process works, issues surrounding conflict of interest, the product lifecycle and pre-investigational new drug processes.
- Network with other newly recruited Patient Reps.

Our staff is excited to have you on board and look forward to working with each of you! Congratulations on your training!

If you'd like to access the presentations and speaker biographical sketches, please visit the event

Broadening Patient Input

FDASIA Section 1137

"Patient Provision"

- FDA Safety and Innovation Act (FDASIA) 2012
- Sec. 1137: Patient participation in medical product discussions
- Develop a systematic process to include patients earlier in medical product development through consultation with scientific review divisions

FDA Patient Network

Outgrowth of the Patient Program

Broadens opportunity for patient engagement

- Website
- Bi-weekly Email Newsletter
- Webinars & In-person Meeting's

and

- Expand understanding of FDA's role

Patient Network Newsletter

A bi-weekly newsletter containing FDA-related information on a variety of topics, including:

- new product approvals,
- significant labeling changes,
- safety warnings,
- proposed regulatory guidances
- opportunities to comment,
- and other information of interest to patients and patient advocates.

This bi-weekly newsletter provided by the Office of Health and Constituent Affairs at the Food and Drug Administration (FDA) is intended to inform you of FDA-related information on a variety of topics, including new product approvals,significant labeling changes, safety warnings, notices of upcoming public meetings, proposed regulatory guidances and opportunity to comment, and other information of interest to patients and patient advocates. Subscribe or update your subscriber preferences.

OFFICE OF HEALTH AND CONSTITUENT AFFAIRS
PATIENT NETWORK NEWS

Volume 5 | Number 4 | February 18 2015

Product Safety

FDA issues new draft documents related to compounding of human drugs

The FDA has issued five draft documents related to drug compounding and repackaging that will help entities comply with important public health provisions. The draft documents are applicable to pharmacies, federal facilities, outsourcing facilities and physicians.

The new category of outsourcing facilities was created under the Drug Quality and Security Act (DQSA), enacted by Congress in November 2013 in response to a deadly fungal meningitis outbreak that was linked to contaminated sterile compounded drug products. Drugs compounded in an outsourcing facility that meet certain conditions may be entitled to exemptions from certain provisions of the Federal Food, Drug, and Cosmetic Act (FD&C Act), including the new drug approval requirements and the requirement to label drug products with adequate directions for use. Outsourcing facilities are subject to current good manufacturing practice requirements and inspections by the FDA according to a risk-based schedule. More information

Telephone Inquiries & e-mails

- Respond to inquiries and requests
- Education
- Help patients navigate FDA
- Conduit to other parts of agency

Bisphosphonate / Hepatitis

Meetings

- Host meetings with patient advocacy groups
- Speak to patients at professional association meetings

OFFICE OF HEALTH AND CONSTITUENT AFFAIRS

PATIENT LIAISON PROGRAM

Connecting Patients with FDA

Keecha Chenjo
Patient Rep Program

Richard Klein
Director
HIV/Hepatitis

Helene Clayton Jeter
Cardio-Endocrine Program
Ophthalmic Issues

Andrea Furia Helms
Patient Rep Program
FDAISIA 1137 Coordination

Deb Miller
Cancer
Communications

Steve Morin
Patient Network
HIV/Hepatitis

Salina Prasad
Patient Rep Program
Neurological Conditions

Andrea Furia-Helms

FDA Patient Representative Program
Office of Health and Constituent Affairs
andrea.furia@fda.hhs.gov
http://www.fda.gov/ForPatients/
PatientRepProgram@fda.hhs.gov

U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993 USA
Telephone +011+1 (301) 798-8455
www.fda.hhs.gov