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

Office of External Affairs *

- Office of Media Affairs
- Office of Communications
- Office of Health and Constituent Affairs
- Web Staff

* 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

### Healthcare Professional Liaison Program

- Heidi C. Marchand  
  Assistant Commissioner
- Beth Fritsch  
  Deputy
- Mary Hitch
- Pat Kuntze
- Kathy Duvall

- Anna Fine  
  Program Director
- Stephanie Joseph
- Cristina Klaften
- 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

FDA’s Office of Health and Constituent Affairs

FDA Centers/Review Teams

Patients/Patient Advocates
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
Patient Reps serve as Consultants (divisional assignments)

Where Patient Reps Intersect

Basic Research/Discovery

Translational

Pre-IND

Drug Development

Clinical

Post-marketing

NDA/BLA Review

Ph 1

Ph 2

Ph 3

Ph 4

Advisory Committee

Patient Reps serve on FDA Advisory Committees

Patient Reps serve as Consultants (divisional assignments)
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

The Patient Representative Newsletter

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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 HCA 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 page.
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
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.
Meetings

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

Telephone Inquiries & e-mails

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

Bisphosphonate / Hepatitis
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

Expanded Access, Training, Presentations, Telephone Calls, Advocacy Intelligence, Problem Solving, Policy, Public Access
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