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Office of Health and Constituent Affairs
(OHCA)

Food and Drug Administration

April 24, 2013

Patient Representation at FDA
~ Patient Input ~

FDA input from patients and patient advocates

- Advisory Committee Meetings
  - Open public hearing
  - Written submission to advisory committees
- Public Policy Meetings
- Town Hall Meetings
- Written Comments
  - Federal Register Notices
  - Proposed Rules


TIME ISN'T
THE ONLY
THING THE
F D A IS
KILLING

PATIENT REPRESENTATIVES ON FDA ADVISORY COMMITTEES




Patient Representative Program

Growing since 1991

Incorporating patient/community advocate's voices into advisory committee discussions

…and furthering an understanding and appreciation for FDA's role in drug development and patient protection

Patient Representative Program

- Help to capture the unique perspective of patients and family members directly affected by a serious life-threatening disease
- Serve as SGEs (Special Government Employees) at advisory committee meetings to review products and policies related to serious and life-threatening diseases
- Participate in other FDA-related activities where the patient perspective is needed

Patients add value to FDA's decision making

- Bring a diversity of opinion, of 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 appreciate patient feelings about balancing efficacy and concern about risks, to help FDA make better risk/benefit decisions

A value judgment overlay on top of measureable, empirical clinical trial evidence

Who are patient representatives?

- Patients
- Patient Advocacy Group members
- Community Advocacy Groups members
- Family and/or caregivers
- Health Care Providers

Patient Representative Contributions

- Citing problems with trial design, including
  - adequacy of drug interaction data
  - trials that reflect real world use of product
  - enrollment issues
  - gender, age, or racial biases
- Symptom characterization/prioritization
- Patient concerns with adverse event acceptability
- Issues related to risk tolerance, drug safety
- Product labeling

Patient Representative Program

Managed in the Office of Health and Constituent Affairs

The Program's Activities:

- Recruitment & initial screening of New Patient Representatives
- Selection of Patient Representatives for:
  - Advisory Committees
  - Consultation with Review Division
- Conducts Training For Patient Representatives

More information: See FDA web site under

Information for Patients and Patient Advocates

Patient Representative Program Profile

Approximately 185 patient representatives
Approximately 105 diseases and conditions, including:

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

What do we look for in patient representatives?

- Bring a patient perspective to the process, grounded in personal experience
- Have patient community awareness
  - Active in patient advocacy organizations
  - Knowledgeable about treatment options and research in the disease area
- 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
  - Minimal or no conflicts of interest

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Criteria for Becoming a Patient Representative

- Personal experience with the disease or condition, either as a patient or as a primary caregiver
- Able to be objective while representing the concerns of patients with that disease, and willing to communicate their views
- Knowledgeable about treatment options for the disease and research in that area
- No obvious financial or ethical conflicts of interest for self or immediate family member

Becoming a Patient Representative

1) Resume
   - patient/caregiver experience
   - advocacy experience
   - ability to represent other patients
   - knowledge and skills related to disease area
   - alternate disease experience
   - work experience
   - education

2) Phone Interview

3) SGE (Special Government Employee) clearance

Challenges

- Conflict of Interest
  - Screened on entry to the program
  - Secondary, more specific screening when asked to serve
- Scheduling
  - Scheduling conflicts
  - May become too ill to attend/serve – have alternates
- Potential Impact on other advocacy activities
  - Conflict of interest issues might limit their activities

Training

Not all patients are prepared to participate out of the box -

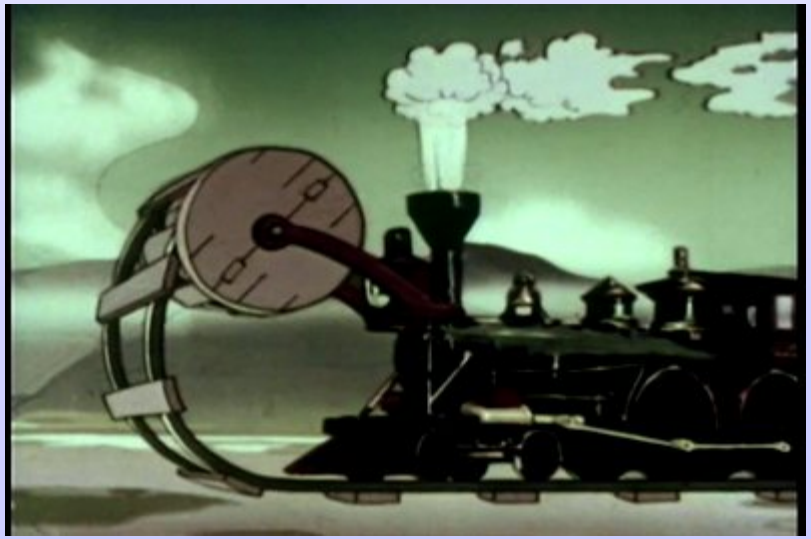
- Not fully aware/familiar with the regulatory framework and decision-making process
- May be intimidated by the scientific committee members
- Many unsure of their role, and its importance, or the value they bring to the discussion

Training

- FDA 101 – Basic regulatory overview, interactive, often conducted one-on-one by telephone
- Regular teleconference/webinar training modules
- Annual Patient Rep Workshop

What's Next?

Expanding program giving patients opportunity for input earlier in the product development process



Patient Representatives as Consultants

Patient Advocates may consult with FDA review divisions and participate in FDA/Sponsor meetings

Incorporate the patient perspective into the Clinical trial and post-approval phases of medical product development

FDASIA

- The Food & Drug Administration Safety & Innovation Act

Requires patient participation in medical product discussions

The Food & Drug Administration Safety & Innovation Act (FDASIA)

Section 1137

- ‘‘(a) IN GENERAL.—The Secretary shall develop and implement strategies to solicit the views of patients during the medical product development process and consider the perspectives of patients during regulatory discussions, including by –

  (1) fostering participation of a patient representative who may serve as a special government employee in appropriate agency meetings with medical product sponsors and investigators; and

  (2) exploring means to provide for identification of patient representatives who do not have any, or have minimal, financial interests in the medical products industry.

Implementation

The language is the first “official” codification of the FDA Patient Representative Program and other patient liaison activities

- Provides greater stability to the Program
- Provides for greater awareness of FDA’s patient liaison efforts
- May lead to additional resources for the program

– The ambiguous wording of the Section provides OHCA and Centers/divisions with flexibility in how best to move forward

Implementation

- Falls under the jurisdiction of OHCA
    - The FDA Patient Representative Program already exists and will be the backbone to provide greater patient input
    - Build upon recruitment, training and experience of our on-board patient representatives to participate earlier in the development process

Challenges

COI not always so clear cut

- Crisp rules for financial conflicts
- "*Appearance*" edges are not so sharp
  - The nature of patient advocacy sometimes conflicts with qualified patient representatives
    - Writing and blogging about new and ongoing trials or approvals
    - Serving as head of an advocacy group that has received funding from a pharmaceutical company
    - Working with pharmaceutical companies in a paid capacity, such as a community consultant or advisor

Challenges
Timing/Clearance

- SGE Appointment – from application through initial conflict-of-interest screening can take several months to complete
- Particular Matter clearance (once an SGE) – can take up to additional 6-8 weeks and has only a 3 month shelf life

Conflicts are not just related to the sponsor, but all potential competitor products as well

Challenges

Why timing is a problem?

- FDA has strict timeframes for meetings with sponsors and under UFA* requirements and internal guidances

For example

- Type A Meetings: sponsor would like to meet with FDA to discuss a path forward when trials is on clinical hold
    - 30 days from time of request
  - Type B Meetings: Pre-IND through Pre-NDA meetings
    - 60 days from time of request
  - Type C Meetings: Lower priority meetings
    - 75 days from time of request
- Usually takes longer to get patient consultant cleared

* User Fee Act

Evolving

Creating an agency-wide working group

- Define “appropriate agency meeting”
  - Definition will likely vary from Center-to-Center and Division-to-Division
- Identify log jams in clearance process
- Define the scope and nature of the relationship each division will have with a patient representative

Evolving

- OHCA developing a secondary pool of PRs
  - "Minimally conflicted" patients
    - Prospective screening with an eye on potential conflicts –
      - A blog or articles or op-eds, etc., that mention FDA or one of the many involved regulated products or business entities
    - Ensure PRs understand COI screening process, and their role in the fast turn-around
    - OHCA working closely with patient reps and FDA offices to ensure smooth flow of concepts – and paperwork

Patient Network

The best participation and input from patients comes from patients who understand the regulatory framework and processes.

Goal is to educate patient communities about FDA, respond to their questions, keep them apprised of opportunities to comment, and encourage appropriate participation in medical product development

Patient Network

- Scale up current OHCA educational and advocacy activities
- Institute a more proactive approach to patient engagement
- Primary FDA patient educational and advocacy resource

  for

  – Patients & Caregivers
  – Independent Patient Advocates
  – Patient Advocacy Organizations

Patient Network Components

- Multifaceted Website, including:
  - Ongoing FDA Initiatives
  - How Medical Products Get Approved
  - Clinical Trials Participation
  - Accessing Investigational Products using Expanded Access
  - Off-Label Use of Approved Drugs
  - FDA Advisory Committees
  - Interactivity (i.e. feedback mechanisms, live chats)
- Biweekly Email Newsletter
- Annual Meeting
- Listening Sessions and Other Briefings

U.S. Food and Drug Administration
Protecting and Promoting Public Health

www.fda.gov

U.S. Department of Health & Human Services

www.hhs.gov

Patient Network

Bringing Your Voice to Drug and Device Approval and Safety

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Understand Drug Development

Make Your Voice Heard

Become a Patient Rep

Find Clinical Trials

Share your unique perspective and serve on an advisory committee.

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Approved Drugs at FDA

Find a Clinical Trial

Ask the FDA

Get Informed

Learn about drug and device approval
See how they get to your pharmacy

Find out about off-label use
Read about off-label drug use

Get bi-monthly updates
Subscribe to *Patient Network News*

Listen to a Webinar
Learn more about drug safety

Talk to FDA

Make Your Voice Heard
Become a patient representative

Ask FDA
Submit your questions to FDA

Attend an FDA meeting
Participate in advisory committee meetings

Comment on a Regulation
Voice your opinion on FDA decision-making

Take Action

Join a clinical trial
Find one that may be right for you.

Find new treatment options
See about alternatives that may work for you

See new FDA approvals
View recently approved drugs

Subscribe to Patient Network News

Stay informed about product safety and new product approvals, and other important information for patients and patient advocates.

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Patient Network
Bringing Your Voice to Drug and Device Approval and Safety

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Get Involved

You can participate in FDA's important decisions about the regulation of medical products in many ways. You don't have to be an expert, and you don't need lots of time. Find out how you can make your voice heard and help ensure the effectiveness and safety of drugs, devices, and other medical products.

Become a Patient Representative

Add your voice to FDA processes to ensure input into important medical product development, review, and policy questions. Find out how.

Read More ▶

Calendar of Public Meetings

As a member of the public, you are welcome to attend the FDA's meetings. See what's coming up that may interest you.

Read More ▶

Ask the FDA

Ask your questions about newly approved or

Get Involved

- Become a Patient Representative
- Calendar of Public Meetings
- Ask the FDA
- Join a Live Chat
- Listen to Webinars With FDA Experts
- Comment on FDA Regulations
- Report Side Effects on MedWatch
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Report Side Effects on MedWatch

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Step 4 FDA Review

1 Discovery and Development | 2 Preclinical Research | 3 Clinical Research | 4 FDA Review | 5 Safety Monitoring

If a drug developer has evidence from its early tests and preclinical and clinical research that a drug is safe and effective for its intended use, the company can file an application to market the drug. The FDA review team thoroughly examines all submitted data on the drug and makes a decision to approve or not to approve it.

Find out how the FDA is Speeding Up the Approval Process.

New Drug Application

A New Drug Application (NDA) tells the full story of a drug. Its purpose is to demonstrate that a drug is safe and effective for its intended use in the population studied.

A drug developer must include everything about a drug—from preclinical data to Phase 3 trial data—in an NDA. Developers must include reports on all studies, data, and analyses. Along with clinical results, developers must include:

- Proposed labeling
- Safety updates
- Drug abuse information
- Patent information
- Any data from studies that may have been conducted outside the United States
- Institutional review board compliance information
- Directions for use

FDA Review

Learn How Drugs & Devices Get Approved

▼ The Drug Development Process
- Step 1: Discovery and Development
- Step 2: Preclinical Research
- Step 3: Clinical Research
- Step 4: FDA Review
- Step 5: FDA Post-Market Safety Monitoring

▶ The Device Development Process

Report Side Effects on MedWatch
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