Patients' Interactions in Pharmaceutical Regulatory Policies: The Experience of the U.S. FDA

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AIFA
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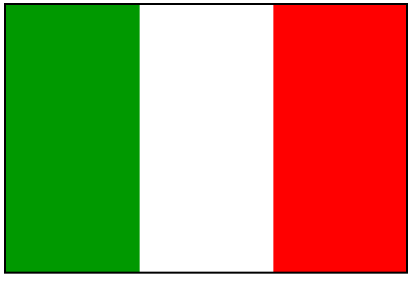


Patients and Consumers: Principles

We have many “stakeholders:, but we have only one “customer” - the patients of our country for whom we work and to whom we are responsible

Patients need to honestly perceive that they are the end and not the means to an end

They who must make informed decisions about products and their health

They who bear the ultimate benefits and the ultimate risks of the products we regulated

Patients and Consumers: Principles

Information, participation, transparency breed confidence and trust

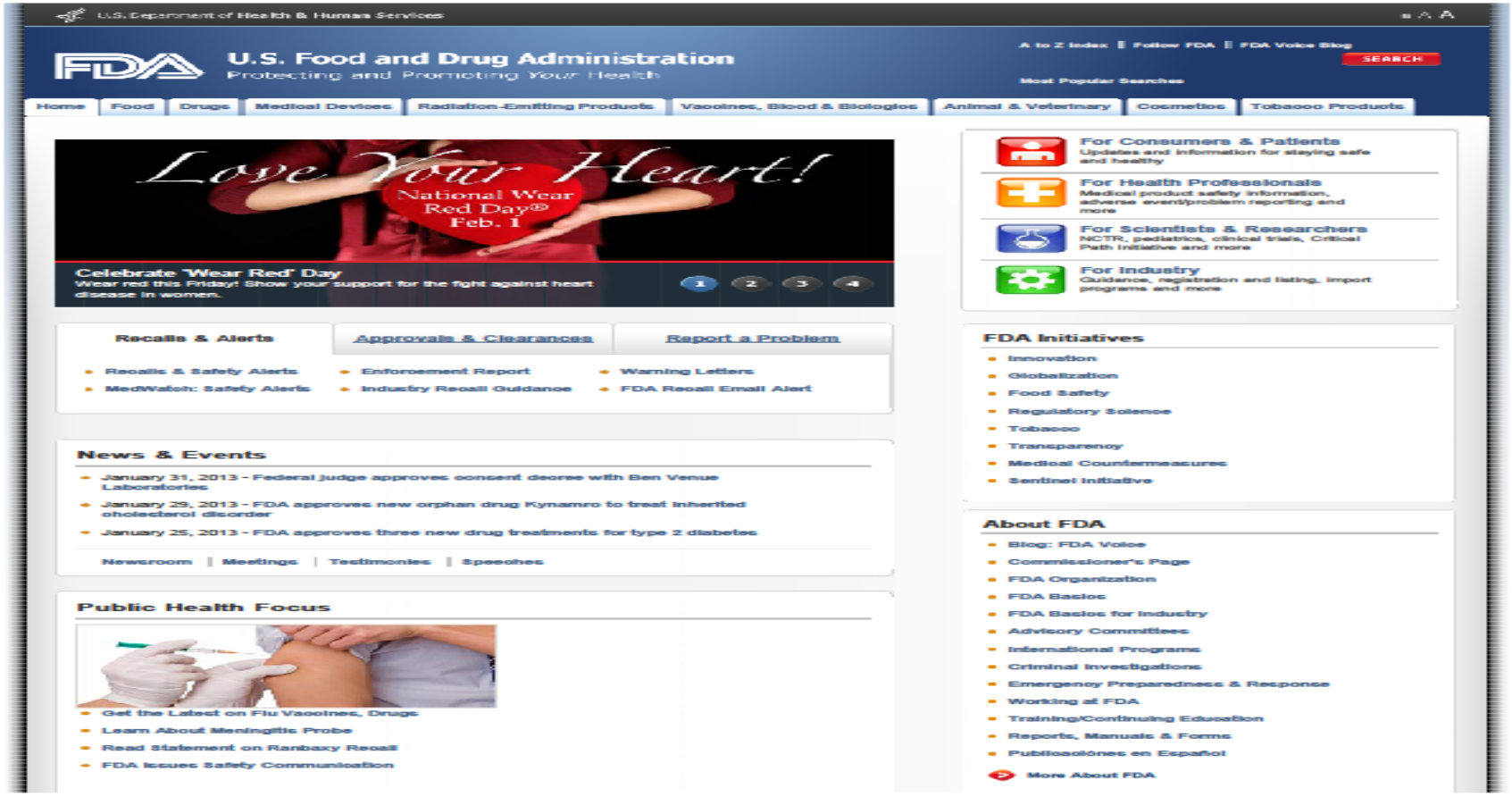
Silence, neglect, opacity breed fear and distrust

Patients and Consumers

Proactively Reaching Out to Patients and Consumers

Proactively Engaging Patients and Consumers in our regulatory processes and decision-making

U S Food and Drug Administration Home Page



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Proactive Outreach

Patient Network News

Twice monthly newsletter contain 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 opportunities to comments and other information of interest to patients and patient advocated

Proactive Outreach

Subscriptions

- Email Alerts
- RSS Feeds

Social Media

- Facebook
- Twitter
- Blogs

Multimedia

- Podcasts
- Videos
- Flickr

Content syndication

- Widgets
- Syndicated Content Feeds

Proactive Outreach

On Line Customer Feedback and Guidance:

American Consumer Satisfaction Index Survey

Developing Products for Rare Diseases & Conditions
- OOPD News, Events and Archives
- Archived OOPD News & Events

FDA Rare Disease Patient Advocacy Day

On March 1, 2012, the Food and Drug Administration (FDA) celebrated the fifth annual Rare Disease Day by hosting a "FDA Rare Disease Patient Advocacy Day" to engage and educate the rare disease community on regulatory processes related to rare diseases.

This meeting was held to enhance the awareness of the rare disease community as to FDA's roles and responsibilities in the development of products (drugs, biological products and devices) for the diagnosis, prevention, and/or treatment of rare diseases or conditions.

- For more information about Rare Disease Day, see *Rare Diseases Take Spotlight in Annual Event*.
- For more information about the History of FDA and Milestones in U.S. Food and Drug Law, see *About the FDA*

Meeting Evaluation: The FDA values your review of this event. We encourage participants to contact us with your comments at: orphan@fda.hhs.gov

Video Recording of March 01, 2012 event:

- Plenary Session (8:30am-10:35am)
- Plenary Session (10:55am-12:00pm)
- Keynote Address (1:30pm-2:00pm)
- Senior Townhall (4:30pm-5:00pm)

Recap of the Patient Advocacy Day Meeting:

- Wrap-Up of FDA Rare Disease Patient Advocacy Day March 1, 2012
- Patient Advocacy Day Pictures

Agenda:

- View event agenda

Presentations:

- View presentations and other resources

Sponsors:

The FDA Rare Disease Patient Advocacy Day is supported by the Food and Drug Administration (FDA), the National Institutes of Health (NIH), the National Organization for Rare Disorders (NORD), and the Genetic Alliance.

Page Last Updated: 07/18/2012
Note: If you need help accessing information in different file formats, see Instructions for Downloading Viewers and Players.

For Consumers

FDA Working with Patients to Explore Benefit/Risk: Opportunities & Challenges

http://www.blocentury.tv.com/player/1669511147001 Friday, May 18, 2012 at the FDA White Oak Campus, in Silver Spring, MD.

U.S. Food and Drug Administration Inaugural Patient Network Annual Meeting, hosted by FDA's Office of Special Health Issues (OSHI) in collaboration with the Center for Drug Evaluation and Research (CDER), the Center for Biologics Research and Evaluation (CBER), and the Center for Devices and Radiological Health (CDRH).

This meeting was held to allow FDA to gain a greater understanding of how patients define and perceive benefits and risks related to medical products. The FDA Patient Network hosted this one-day meeting to:

- review the drug and medical device regulatory processes;
- discuss where patient input is practical and most valuable; and,
- explore practical approaches to collecting meaningful patient input.

The meeting included a series of presentations, exercises, and panel discussions to facilitate a conversation with the patient community about these important topics. FDA asked patients and other interested parties to consider specific questions, posed in a 4/19/2012 Federal Register Notice, designed to frame discussion at the meeting.

Video Recording of the May 18, 2012 event:

Part 1

- Keynote Address Stephen Spielberg, M.D., Ph.D., Deputy Commissioner for Medical Products and Tobacco
- Drug Development: Laws, Regulations, Statutory & Regulatory Limitations Janet Woodcock, M.D., Director, CDER

Part 2

- Drugs and Biologics Development 101 Robert Yetter, Ph.D., Associate Director for Review Management, CBER
- Devices 101 Peper Long, Associate Director, External Relations, CDRH
- Drugs, Biologics and Devices Question and Answer Richard Klein, Director, Patient Liaison Program, OSHI (moderator)

Part 3

- Benefit-Risk Framework Patrick Frey, Director, Office of Planning and Analysis, CDER
- Patient-Focused Drug Development Theresa Mullin, Ph.D., Director, Office of Planning and Informatics, CDER
- Making Benefit-Risk Determinations Peper Long, Associate Director, External Relations, CDRH
- Patient Risk Tolerance Survey for Obesity Devices Martin Ho, M.S., Division of Biostatistics, Office of Surveillance and Biometrics, CDRH

Part 4

- Patient Risk Tolerance Survey for Obesity Devices Martin Ho, M.S., Division of Biostatistics, Office of Surveillance and Biometrics, CDRH (continued)
- Discussion of Federal Register Notice Questions
- Patient Perspectives Panel & Audience Discussion James Valentine, M.H.S., Project Manager, FDA Patient Network, OSHI (moderator)
- FDA Reaction Panel James Valentine, M.H.S., Project Manager, FDA Patient Network, OSHI (moderator)
- Closing Remarks Richard Klein, Director, Patient Liaison Program, OSHI

Original Federal Register Notice of Meeting, with framing questions

Photos of the Meeting

But not enough!

Only the front half of the circle

Not complete until the other half is equally as well populated with opportunities for interactions

Patients and Consumers

Proactively Reaching Out to Patients and Consumers

Proactively Engaging Patients and Consumers in our regulatory processes and decision-making

Engagement in Decision-making

Patient Representative Program

- **Members of our Public Advisory Committees**
- **Consultants to our Review Divisions during Drug Development (aka “scientific advice”)**

Patient-focused Drug Development Program

- **New program mandated under our most recent pharmaceutical law (FDASIA – 2012)**

Patient Representative Program

1991 – First Patient Representative on a Public Scientific Advisory Committee

(Anti-viral Drugs Advisory Committee – HIV/AIDS drugs)

2001 – First Patient Consultant Program

(Oncology)

Now combined into our Patient Representative Program

Special Government Employees for this purpose

Bound by COI / confidentiality as are our other advisory committee members

Patient Representative Program

Unique perspective of patients and family members directly affected, especially by serious and life-threatening diseases

Should have:

- **Personal experience with and/or be knowledgeable about the specific illness or condition**
- **Ability to articulate the perspective of patients**
- **Experience as a patient advocate**
- **Formal affiliation with a patient advocacy organisation**
- **Ability to identify issues through communication with patient constituencies**

Reimbursement

The FDA compensates patient representatives for time spent participating in the meeting and covers related travel expenses (such as food and lodging).

Training

If selected, the FDA staff offers training and support to Patient Representatives about policies, procedures, and regulation. Training includes:

- FDA 101 teleconference
- Monthly webinars
- Annual FDA Patient Representative workshops for first-year representatives
- Other one-on-one support as needed

How to Apply

Anyone may nominate—or self–nominate—a candidate to serve as a Patient Representative on FDA Advisory Committees. All nominations must include a resume or curriculum vitae.

Please mail, fax or email nomination to:

FDA Patient Representative Program
Office of Special Health Issues
Building 32, Room 5361
Silver Spring, MD 20993
Phone: (301) 796–8460
Fax: (301) 847–8623

The Office of Special Health Issues works with patients and their advocates to encourage and support their active participation in formulating of the FDA's regulatory policy. The staff is familiar with the concerns confronting patients and families dealing with a life-threatening illness.

www.fda.gov/oshi

Office of Special Health Issues
Food and Drug Administration

FDA Patient Representative Program

FDA Patient Representative Program

The FDA Patient Representative Program gives patients a role in reviewing and approving new drugs, biologics and medical devices.

In 1991, the first patient representative served on the Antiviral Drug Advisory Committee for HIV. The patient represenative was included in the advisory committee because patients' groups advocated for a larger role in the development and regulation of medical products.

Ten years later, the FDA created the Patient Consultant Program, which offered patients the chance to take part in the FDA's decision-making process at meetings of the FDA and product developers.

Since then, the FDA combined the two programs into a single FDA Patient Representative Program. Now, patient representatives can take part in various FDA-sponsored activities.

Patient Representative Responsibilities

A Patient Representative's responsibilities includes providing a patient's point of view during the process, asking questions, and offering comments. To do this, Patient Representatives participate in Advisory Commitee meetings or medical products (such as drugs, devices or biologics) development meetings. Sometimes, they participate in both.

Advisory Committees

Advisory Committees provide expert advice to the FDA to help FDA officials make sound decisions about new drugs, biologics, medical devices and other public health matters. Scientific experts, such as doctors, statisicians, consumser and industry represenatives sit on FDA Advisory Committees, in addition to patient representatives. Advisory Committees are open to members of the public and media.

Medical Product Development Meetings

Medical product development meetings are confidential discussions between FDA officials and a medical product product developer. Usually held in telephone conference calls, these meeting ensure the FDA and the developer hear the patients' perspective throughout the development process.

FDA Patient Representative Criteria

To be considered for the program, an applicant must be a legal U.S. resident at least 18 years of age and have:

- Personal experience with the disease either as a patient, or primary caregiver such as a family member or friend.
- Ability to be objective while representing the concerns of other patients
- Willingness to communicate their views
- Knowledge about treatment options for the disease and research in that area
- No financial or ethical conflicts of interests for self or close family member

Advisory Committee Members

Patient Members

Consumer Members

Why?

What can they contribute?

Clinical Trials Data

- **The trials data tell us:**
  - **What the demonstrated benefits are in the test population when used in certain way**
  - **What the known risks are in the test population when used in a certain way**
  - **They give was a benefit/risk profile in the test population when used in a certain way**
  - **This is the SCIENCE of medicines regulation**

Clinical Trials Data

- **The trials data do NOT tell us:**
  - **How to manage the known and unknown risks - or even if you can in a specific health care system**
  - **How to best communicate that risk - or even if it can be communicated in various communities**
  - **What specific communities' tolerances of the benefit/risk profile are**
  - **This is the ART/JUDGEMENT of medicines regulation**
  - **Generally IS community specific**
  - **In this area that patient and consumer members have often been pivotal in our decision-making**

Training for Members

Office of Special Health Issues / Center → Orientation

- **Mission and Structure of our agency**
- **Function and structure of the Advisory committee on which they will be serving**
- **Role and Responsibilities of the Patient Representative**
- **Conflict of Interest information pertinent to their designation as a special government employee**
- **Compensation for service, travel, and lodgings relative to committee meetings**
- **Observe other meetings**
- **Talk with those who have been patient representatives previously**

Development Consultants

Drug Development Patient Consultant Program

- Incorporates the perspective of patient advocates into the drug development process
- Allows patients an opportunity to participate in the drug development process with FDA and companies
- Started 2001 with 25 patients with various cancers
- Expanded now to other serious and life-threatening diseases

Development Consultants

- Patient consultants in the program have participated in over 50 company/FDA meetings providing the patient perspective on topics such as clinical trial design, endpoint determination, expanded access protocol development, and clinical trial patient recruitment strategies.
- The program now recruits patient consultants on an as needed basis.
- A Patient Consultant's term is not time-limited.

Development Consultants - Training

- A one and one-half day workshop that provides an overview of FDA, the drug review process, and the patient consultant's obligations under the conflict of interest and confidentiality regulations.
- A site visit to an FDA scientific review division hosted by OSHI.
- A monthly telephone lecture series building on the workshop.
- A patient consultant from the current team of trained patient consultants assigned to each newly recruited patient consultant as a mentor. The mentor is available for ongoing support and one on-one-training.

Development Consultants

- In order to provide consultation to both FDA and the drug company, it is important that the patient consultant have background information on the drug under review.
- Approximately three weeks before each meeting, the patient consultant is mailed the meeting package containing the meeting issues and questions. The patient consultant reviews the meeting package in preparation for the meeting. After reviewing the meeting package, the patient consultant's questions can be answered in a pre-meeting (via telephone) with FDA staff before the FDA and company meeting.
- *All aspects of this meeting, including the meeting itself, the meeting package, and even the existence of the product application with FDA are confidential.*

Patient-focused Drug Development

In FDASIA, FDA committed to a new initiative called Patient-Focused Drug Development

Goal: Obtaining the patient perspective on certain disease areas during the next five years. Assessment of a product's benefits and risks involves an analysis of the severity of the condition treated and the current treatment options available for the given disease. This information is a critical aspect of FDA's decision-making as it establishes the context in which the regulatory decision is made.

FDA believes that drug development and FDA's review process could benefit from a more systematic and expansive approach to obtaining the patient perspective on disease severity and current available options in a therapeutic area.

Patient-focused Drug Development

20 public meetings over five years (4/yr)

- **Each one dedicated to a specific disease (or group of closely related diseases)**

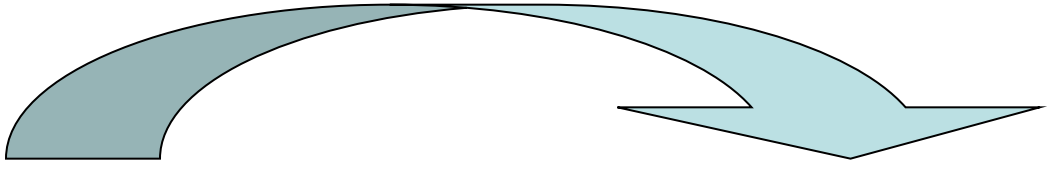
September 2012 – proposed long list of possible disease states

October and December 2012 – public meetings to discuss which to pick; and how to structure questions for patient input

Patient-focused Drug Development

Proposal on how FDA will incorporate patient perspectives on disease severity and unmet medical needs into our product development and market authorisation decision-making processes

Patient Involvement in Regulatory Decision-Making



OUTREACH **ENGAGEMENT**