



# FDA Efforts on Liaising with Patients

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## Outline

- Genesis of Patient Involvement with FDA
- Highlights of OSHI's (Office of Special Health Issues) History
- OSHI's Activities
- Patient Representative Program
- Lessons Learned from Including Patient Input
- Examples of Patient Representative's Input at Advisory Committee Meeting
- Advocates Perspectives

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## Before 1988...

FDA had a history and tradition of careful review, and a conservative approach to balancing potential risks and benefits of drugs.



Activists showed that they needed, and were willing, to take more chances with risks associated with new therapies...

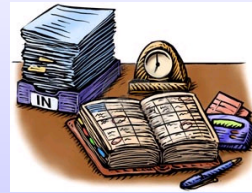
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## After 1988...

FDA responded by

- seeking ways to speed review time and access to promising therapies without jeopardizing patient safety or compromising scientific rigor, and
- creating OSHI (Office of Special Health Issues)



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## OSHI

... works with patients and their advocates to encourage and support their active participation in FDA decision-making and policy formulation.



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## Brief Highlights of OSHI's History

- 1988 Established Office for HIV/AIDS Advocates
- 1991 Initiated Patient Representative Program for HIV/AIDS
- 1994 Added other High Profile Special Health Issues
- 2001 Established Patient Consultant Program
- 2006 Established Health Professional Liaison Program
- 2008 Added MedWatch Program

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## OSHI Activities

- Represent patient and healthcare professional concerns
- Manage
  - **Patient Representative and Patient Consultant Programs**
  - Health Professional Liaison Program
  - MedWatch Program
- Coordinate outreach and educational activities with health professional organizations and patient advocacy groups
- Respond to e-mail and phone inquiries

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## OSHI Staff

- Nurses
- Pharmacists
- Clinicians
- Public Health Analysts
- Health Programs Liaisons
- Technology Experts

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## Patient Participation

- Advisory Committee Meetings
  - Open Public Hearing
  - Patient Representative
- Patient Consultant Program
- Public Meetings
- Public comment on proposed rules and policies
- Community Meetings, Workshops
- Informal input

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## Patient Participation

**Patient Representatives** – included in discussions as (voting) members on FDA Advisory Committees

**Patient Consultants** – work directly with FDA review divisions in clinical trial and post trial review phases

Both programs help capture the unique perspective of patients and family members directly affected by a serious disease

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## Who are patient representatives/consultants?

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

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## Patient Representative Program

FDA U.S. Food and Drug Administration A-Z Index Search

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Home > For Consumers > Consumer Information by Audience > For Patients and Patient Advocates

**Consumer Information by Audience**

- For Patients and Patient Advocates
- Patient Involvement

**Patient Representative Program**

- Overview
- Orientation
- Role of the Patient Representative
- Reimbursement
- How to Apply

**Overview**

The Patient Representative is responsible for providing the Food and Drug Administration (FDA) and the advisory committee the unique perspective of patients and family members directly affected by a serious or life-threatening disease. Among other things, the Patient Representative should have:

- Personal experience with and/or be knowledgeable about the specific illness or condition under discussion by the committee;
- The ability to articulate the perspective of patients;
- Experience as a patient advocate
- Formal affiliation with a patient advocacy organization;
- The ability to identify issues through communication with patient constituencies.

The Patient Representative must be well-informed about patient concerns and have the ability to understand and actively participate in advisory committee discussions. The Patient Representative serves on advisory committees when a product or therapy (drug, biologic or medical device) related to a serious or life-threatening disease is under review.

Patient Representatives serve on advisory committees that review products and therapies for the diagnosis and treatment of serious and life-threatening diseases including:

- Arthritis

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<http://www.fda.gov/ForConsumers/ByAudience/ForPatients/Advocates/PatientInvolvement/ucm123858.htm>



FDA includes Patient Representatives on advisory committee panels that review products and therapies related to serious and life-threatening diseases on a case-by-case basis.

These have included:

- AIDS/HIV
- Alzheimer's Disease
- Asthma/COPD
- Cancer
- Cardiovascular Disease
- Cerebral Palsy
- Chronic pain
- Crohn's Disease
- Depression
- Diabetes
- Fibromyalgia
- Hepatitis B
- Hepatitis C
- Irritable Bowel Syndrome
- Lung Transplantation
- Lupus
- Macular Degeneration
- Major Depressive Disorder
- MRSA
- Neuropathy
- Obesity/Weight Control
- Parkinson's Disease
- Polio
- Sickle Cell Disease
- Short Bowel Syndrome
- Temporomandibular joint (TMJ) Disorder



## Preparing Representatives for Their FDA Roles

- Initial one-on-one orientation and training by OSHI staff
- Ongoing training
  - Monthly teleconference/webinar training
    - Drug review processes
    - Understanding data sets
    - Clinical trial endpoints
    - Statistical analyses
  - Annual workshop
    - Role of advisory committees
    - Drug safety
    - Networking between patient representatives
    - Best practices from patient representative experiences
- Mentoring by senior patient representatives and consultants

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## FDA Benefits from Patient Representatives by...

- Involving patients from early stages of drug development when their input can have significant impact
- Helping patients develop the knowledge and skills to make meaningful contributions to product development based on their unique experience and perspective
- Integrating patient input in a meaningful way throughout the drug development life cycle

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## Patient Representative Contribution to Deliberations

- Trial Design
- Entry Criteria
- Endpoints
- Drug Toxicity Issues
- Quality of Life Issues
- Study Recruitment
- Informed Consent
- Expanded Access
- Product Labeling

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## What FDA Learned

- Be accessible
- Be responsive to change
- Be clear about our positions
- Listen
- Sometimes bend, sometimes stand strong

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## What Activists Learned

- FDA welcomes their involvement
- Build coalitions and send the best people
- Posturing is fine but realism brings results
- Lay the blame at the right place
- Drug industry needs oversight

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## What FDA and Activists Have Learned Together

- Share a common goal
- Both sides are essential to the process
- Neither side is always right or wrong
- Best public health when working together
- Keep talking even when we disagree
- We can work together

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## Value Provided to FDA

Provide FDA with insight on issues, problems, and/or questions pertinent to the viewpoint of patients and family members living with a specific serious or life-threatening disease.

- Product user perspectives – the human element
- Point of view on Risk/Benefit
- Quality of life – adverse events
- Patient input on labeling
- Contribution to better designed future clinical studies
- Inclusion of women and ethnic populations
- Community Ambassadors/Educators

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## Examples

### (1) Advisory Committee Meeting

- Inhaler device
- Device was large
- Patient Representative's perspective

### (2) Advisory Committee Meeting

- Inhaler device
- Risks were noted
- Patient Representative's perspective

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## An Advocate's Perspective...

Without question, having patients ask--or--demand to have an active voice in what scientist do and how they do it will, at times, be difficult, laborious, and even unpleasant. It is also undeniably right.

*AIDS, Activism, and the Politics of Health, Robert Wachter  
NEJM January 9, 1992*

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## An Advocate's Perspective...

- First, we should learn that new is not necessarily better...
- More is not necessarily better...
- We should learn to be realistic, but not cynical, when assessing motivations...
- Although the speed of drug development is important...evidence should not be sacrificed for speed.
- To have a realistic view of both the potentials and the drawbacks of new treatments...

*Musa Mayer  
Journal of Clinical Oncology, Vol 21, No 20 (October 15), 2003: pp 3881-3884.*

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## Thank you!

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