Patient Representative Program
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Patient Liaison Program
Food and Drug Administration

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Input from patients and patient advocates

- Advisory Committee Meetings
  - Open public hearing
  - Written submission to advisory committees
- Public Policy Meetings
- Written Comments
  - Federal Register Notices
    - Proposed Rules
    - Draft Guidances
Early AIDS Involvement

- First time patients sat at the table
- Ad hoc consultants
- Not official “advisors”
- Didn’t have access to complete backgrounders
- Didn’t vote
PATIENT REPRESENTATIVES ON FDA ADVISORY COMMITTEES
Patient Representative Program
Growing since 1991
Incorporating patient/community advocates’ voices into advisory committee discussions

…and furthering an understanding and appreciation for FDA’s role in medical product development and patient protection
What are the criteria for being considered for the Patient Representative program?

• Personal experience with the disease or condition as either a patient or primary care giver
• Patient community awareness; active in patient advocacy organizations, knowledgeable about treatment options and research, other advocacy activities
• Someone who is analytical and objective; doesn’t need to be a scientist but should grasp scientific principles and understand issues, experienced with decision making based upon complex information
• Minimal or no conflict of interest
Recruited as SGEs
(Special Government Employees)

• Recruited from advocacy organizations, through the FDA web site, and meetings

• 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
Conflict of Interest

Is a situation in which a person or organization is involved in multiple interests (financial, emotional, or otherwise), one of which could possibly corrupt the motivation of the individual or organization.

Conflict of Interest Rule

An employee is prohibited by criminal statute, Title 18 U.S.C. 208 (a) from participating personally and substantially in an official capacity in any particular matter in which, to his knowledge, he or any person whose interests are imputed to him under this statute has a financial interest, if the particular matter will have a direct and predictable effect on that interest.
Conflict of Interest Rule

Criminal statute Title 18 U.S.C. 208 (a)

- Imputed interest:
  - Spouse/minor children
  - Employer
  - Any organization in which you serve as an officer, director, trustee, or general partner
  - Any person or entity with whom you are negotiating (prospective employment)
  - Any organizational component you supervise
Patient Representatives

201 Reps | 120 diseases/conditions | ca 60 assignments/year

- 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

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

• Not fully aware/familiar with the regulatory framework and decision-making process
• Some 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
Patient Representatives

• Program continues to evolve
Impact
Grounded in experience

**OB/GYN -**
“As I listened, I heard the entire conversation focusing on a quick, 15-minute, in-office "snip and stitch procedure," "nothing to it."... I commented that there was far more to "the procedure" than the surgeons' clinical assessment, and that as a patient having had so much very painful vaginal surgery, I could attest to the fact that this "snip and stitch" might be 15 little minutes of their time, but, for the patient, it could mean two to three weeks of misery while the incision and stitches healed.”
- Barbara

**Cancer -**
“I recall asking the sponsor to explain how there could be a claim for clinical benefit when there was no survival advantage.”
- Karl

**Cancer –**
It’s a very painful reality that metastatic breast cancer is not curable. I don’t think that means, then, that we should just say, “here, try this” if there isn’t meaningful data to support it. In this study, as presented, there is missing data, there are inconsistencies, and I remain very uncomfortable about that. Considering all the toxicities that I and others have mentioned, I think that is too high a price to pay.
- Natalie
Impact

Abuse resistant opioids formulation -
I don’t think the tools are adequate to address diversion or the use of any reformulation to prevent any problems with children or pediatrics. That really wasn’t addressed today. If you’re planning to have something that is tamper-resistant, abuse resistant – then have only tamper resistant. Have only abuse resistant on the market. Because then if you have the original product still on the market, aren’t you still implicit in something then? You decide.
- Michael

Myelodysplastic syndrome (MDS) –
I personally have had over 700 units of blood. That’s a unit of blood every week. Any drug that reduces or eliminates the need for transfusions is life saving. I can’t tell you what that does to the quality of life – to be able to go six months or a year without a transfusion, spending 7 or 8 hours in a hospital each week when you have a disease that’s life threatening – and many patients only have 2 – 4 years. There is no cure. Making patients transfusion independent is the next best thing.
- Robert
Patient Consultant Program

- FDA Safety and Innovation Act (FDASIA) 2012
- Sec. 1137: Patient participation in medical product discussions
- Develop a systematic process to include patients earlier in drug development than the Advisory Committee stage through consultation with scientific review divisions
Divisional Assignments
for Patient Consultants

- Consult directly with scientific review staff
- Participate in sponsor meetings as consultants to FDA
- Ten divisional assignments during 2014
Patient Input

Basic Research/Discovery

Translational

Pre-IND

Clinical

NDA/BLA Review

Post-marketing

Drug Developers

Patient Engagement With Developer/Sponsor?

Patient Consultation?

Ph 1

Ph 2

Ph 3

Ph 4

Advisory Committee

Patient Representative
FDA Patient Network

Outgrowth of the Patient Program

Broadens opportunity for patient engagement

- Website
- Bi-weekly Email Newsletter
- Webinars & In-person Meeting’s
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
- opportunity to comment,
- and other information of interest to patients and patient advocates.
Encourage Input Through Public Comment
Opportunities for Input through Public Meetings
Because knowledge about the process is important to meaningful advocacy and engagement.
Webinars with FDA Experts
Drug and Device Development
Patient Network Meetings and Webinars

- Diabetes Live Chat – March 2014
- Pediatric Cancer Advocacy Outreach Meeting – November 2014
- FDA-Patient Dialogue on Unmet Needs in Diabetes – November 2014
Telephone Inquiries & e-mails

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

Meetings

- Host meetings with patient advocacy groups
- Speak to patients at professional association meetings (e.g., ASCO)