Patient Representative Program

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Patient Liaison Program
Food and Drug Administration

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Input from patients and patient advocates

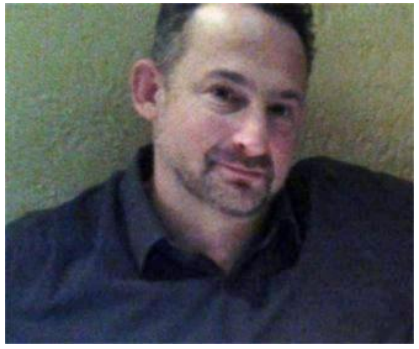
- Advisory Committee Meetings
  - Open public hearing
  - Written submission to advisory committees
- Public Policy Meetings
- Written Comments
  - Federal Register Notices
    - Proposed Rules
    - Draft Guidances

Early AIDS Involvement





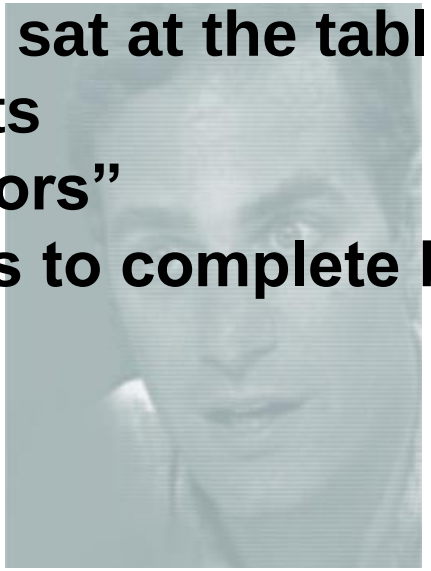





Early AIDS Involvement

- 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

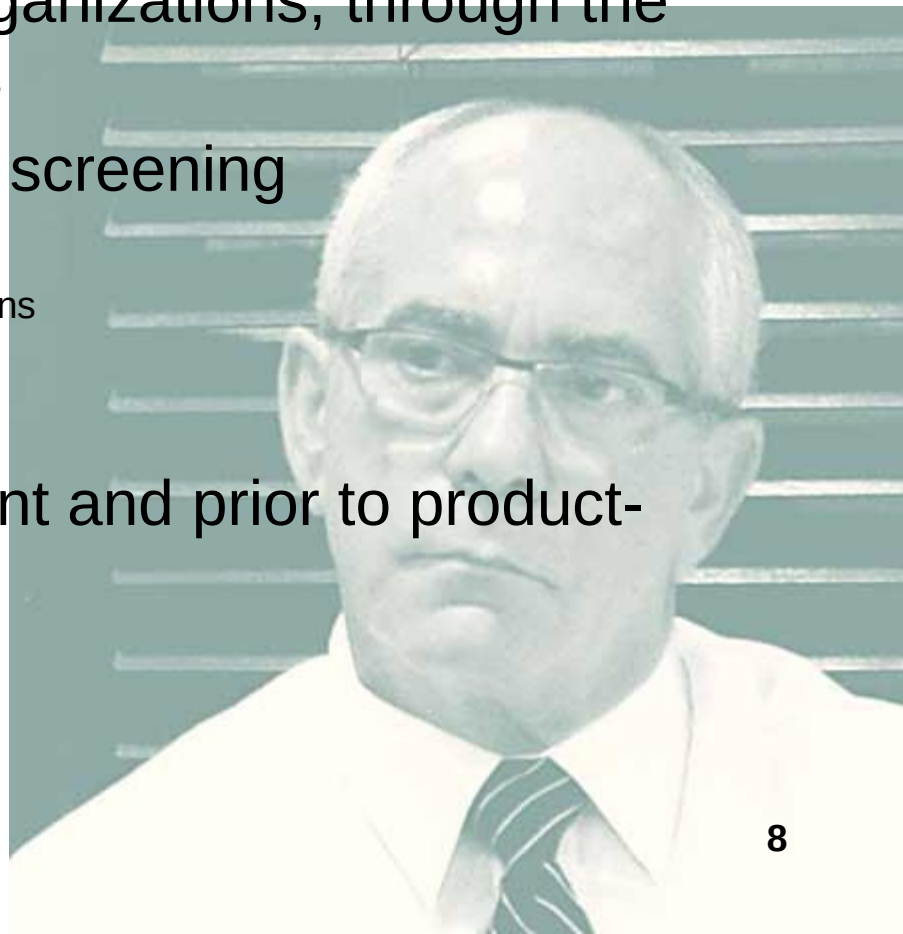
What are the criteria for being considered for the Patient Representative program?

- 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

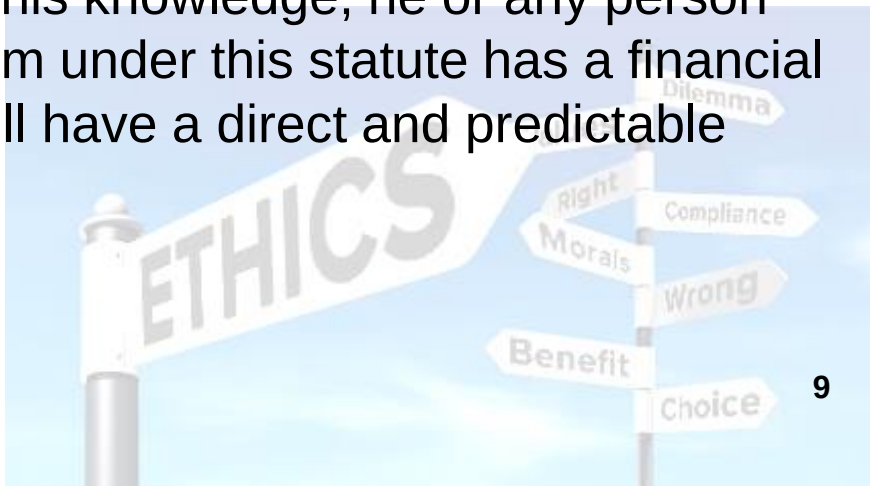


Conflict of Interest

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/Cadiovascular 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 -

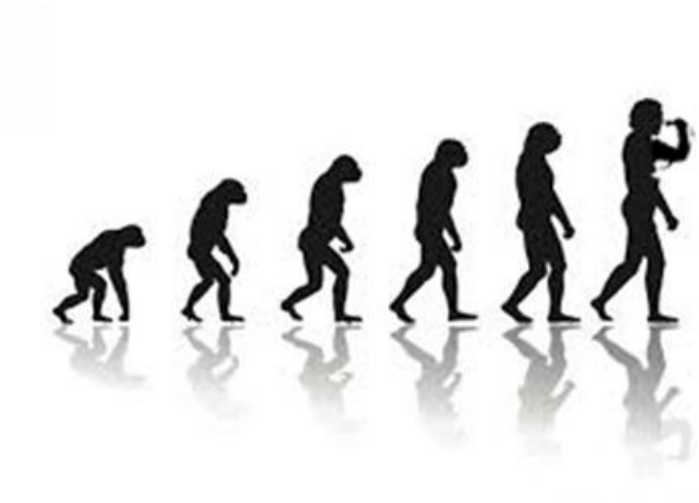
- Not fully aware/familiar with the regulatory framework and decision-making process
- Some may be intimidated by the scientific committee members
- Many unsure of their role, and its importance, or the value they bring to the discussion

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 -

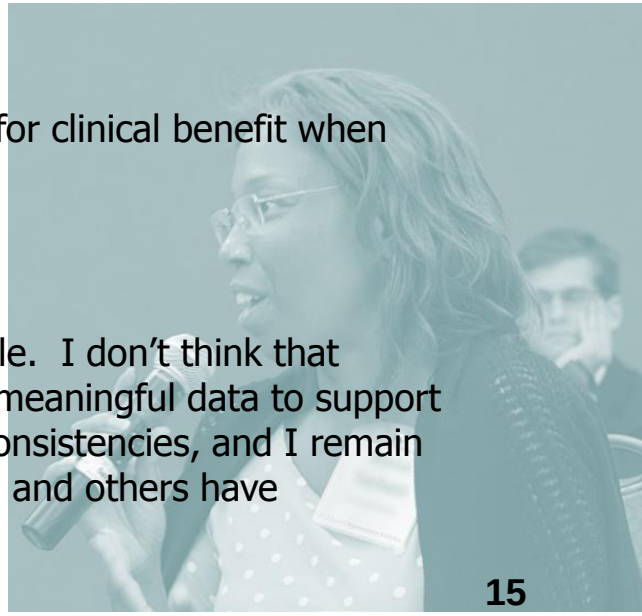
"I recall asking the sponsor to explain how there could be a claim for clinical benefit when there was no survival advantage."

-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.

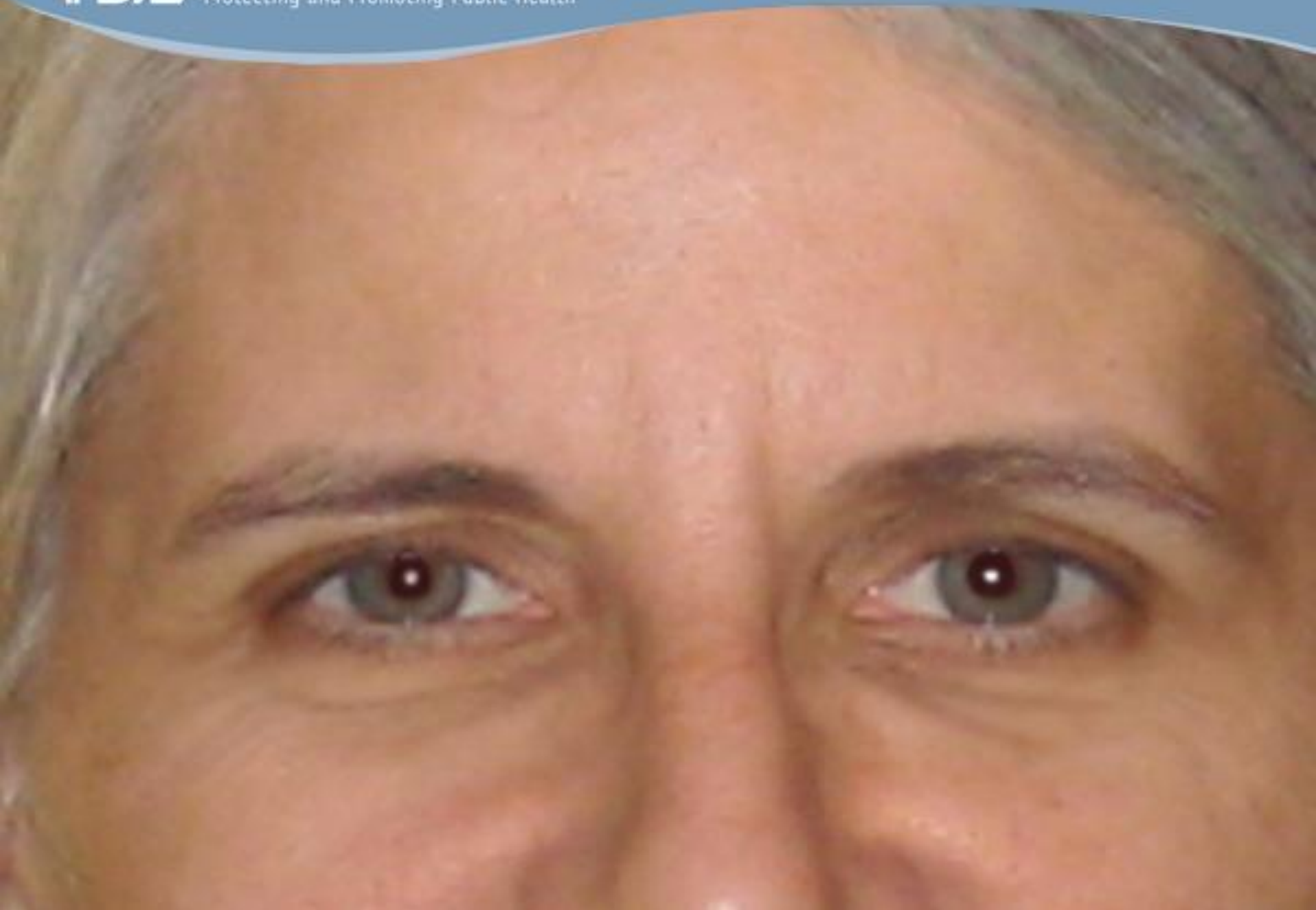
-Natalie



Impact

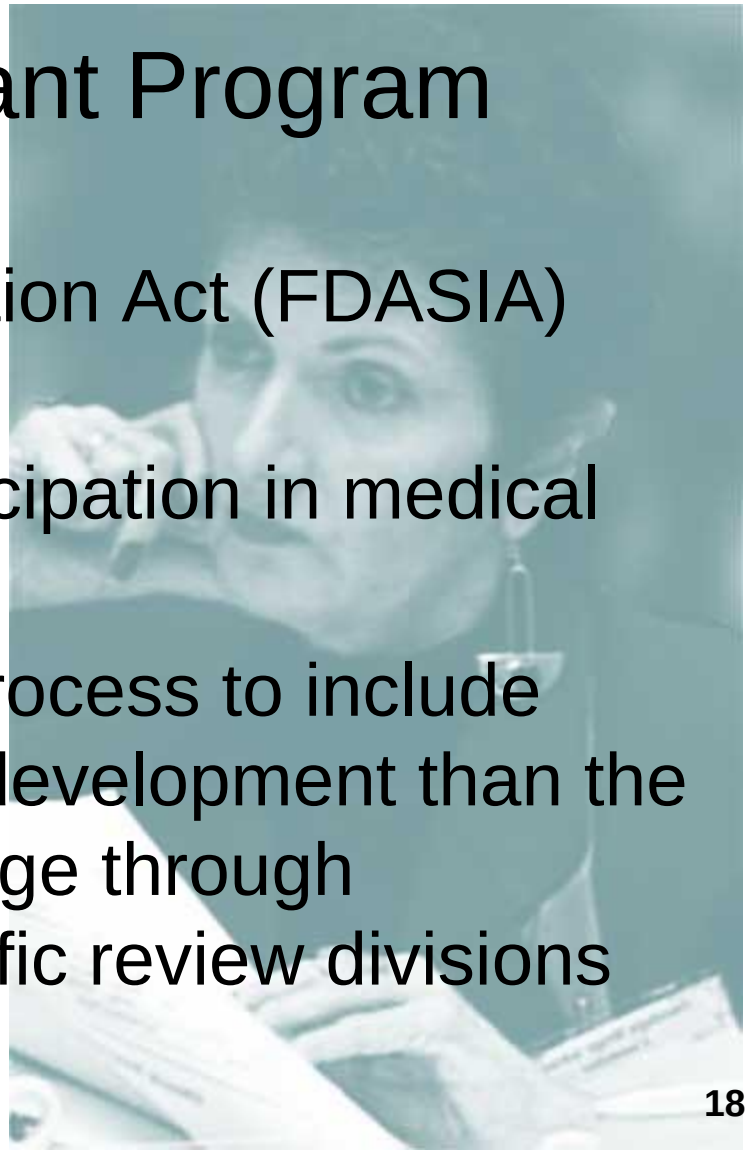
Abuse resistant opioids formulation -
I don't think the tools are adequate to address diversion or the use of any reformulation to prevent any problems with children or pediatrics. That really wasn't addressed today. If you're planning to have something that is tamper-resistant, abuse resistant – then 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

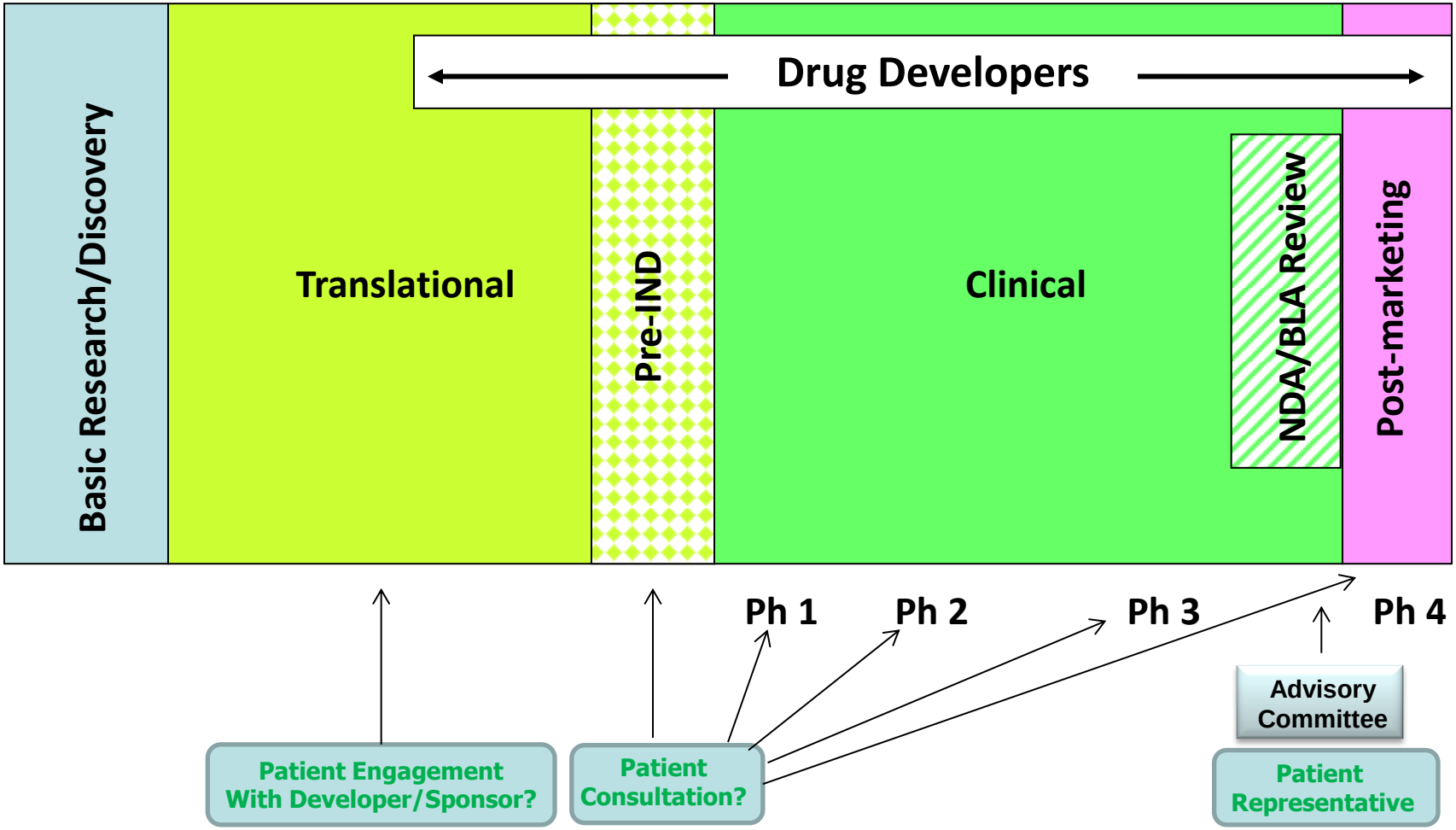
- FDA Safety and Innovation Act (FDASIA) 2012
- Sec. 1137: Patient participation in medical product discussions
- Develop a systematic process to include patients earlier in drug development than the Advisory Committee stage through consultation with scientific review divisions



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



FDA Patient Network

Outgrowth of the Patient Program

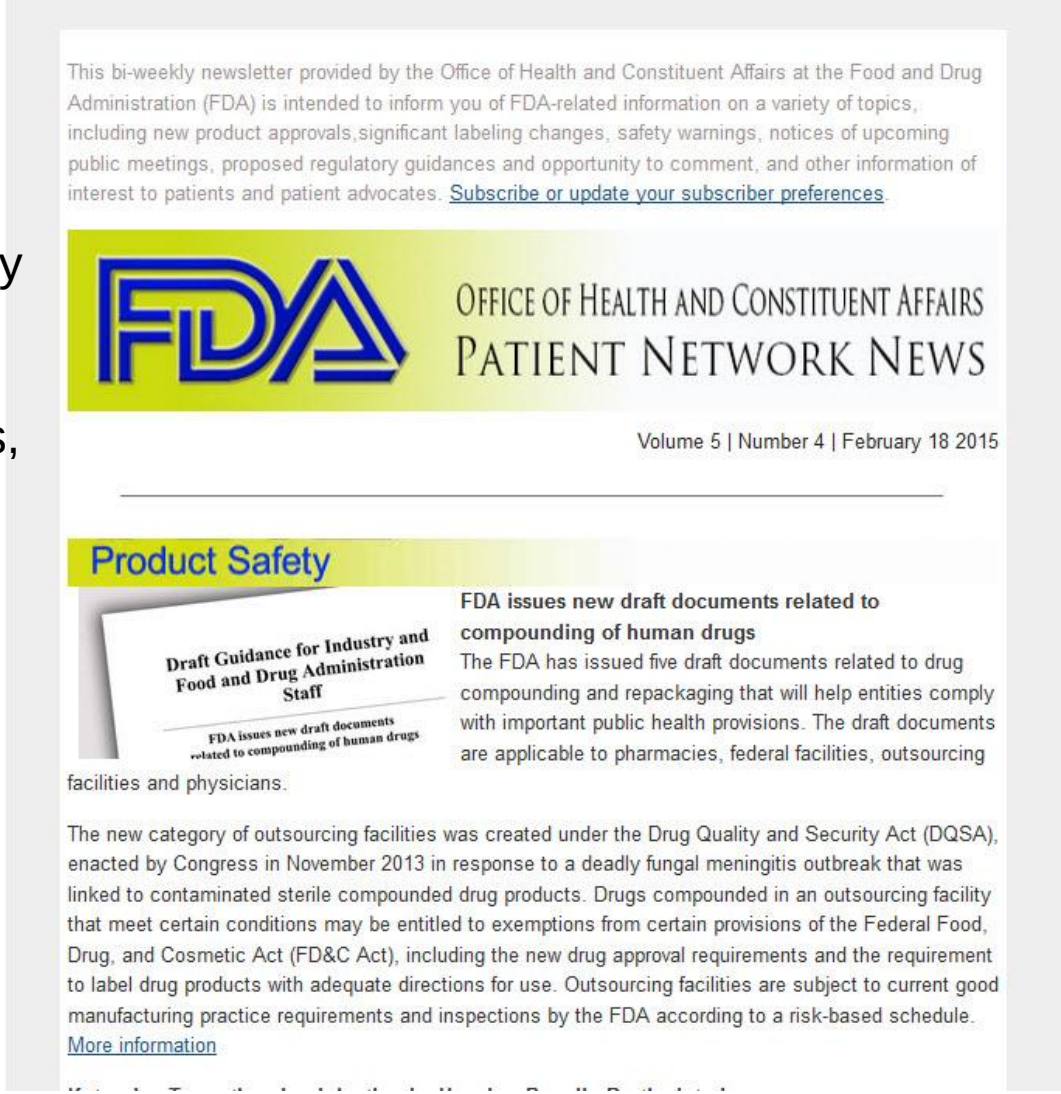
Broadens opportunity for patient engagement

- Website
- Bi-weekly Email Newsletter
- Webinars & In-person Meeting's

Patient Network Newsletter

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.



This bi-weekly newsletter provided by the Office of Health and Constituent Affairs at the Food and Drug Administration (FDA) is intended to inform you of 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 opportunity to comment, and other information of interest to patients and patient advocates. Subscribe or update your subscriber preferences

OFFICE OF HEALTH AND CONSTITUENT AFFAIRS
PATIENT NETWORK NEWS

Volume 5 | Number 4 | February 18 2015

Product Safety

FDA issues new draft documents related to compounding of human drugs

The FDA has issued five draft documents related to drug compounding and repackaging that will help entities comply with important public health provisions. The draft documents are applicable to pharmacies, federal facilities, outsourcing facilities and physicians.

The new category of outsourcing facilities was created under the Drug Quality and Security Act (DQSA), enacted by Congress in November 2013 in response to a deadly fungal meningitis outbreak that was linked to contaminated sterile compounded drug products. Drugs compounded in an outsourcing facility that meet certain conditions may be entitled to exemptions from certain provisions of the Federal Food, Drug, and Cosmetic Act (FD&C Act), including the new drug approval requirements and the requirement to label drug products with adequate directions for use. Outsourcing facilities are subject to current good manufacturing practice requirements and inspections by the FDA according to a risk-based schedule. More information

Encourage Input Through Public Comment

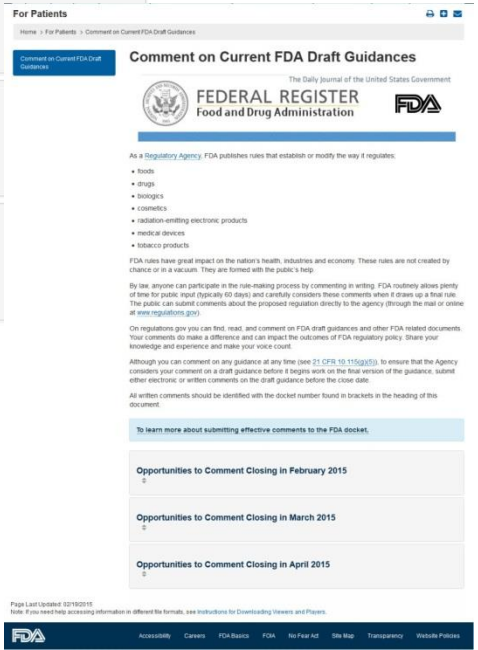
Opportunities to Comment Closing in February 2015

Request for comment by February 19, 2015: Clinical Trials Registration and Results Submission
This Notice of Proposed Rule making proposes requirements for submitting registration and summary results information, including adverse event information, for specified clinical trials of drugs (including biological products) and devices and for pediatric postmarket surveillances of a device to ClinicalTrials.gov, the clinical trial registry and results data bank operated by the National Library of Medicine (NLM). This proposed rule provides for the expanded registry and results data bank specified in Title VIII of the Food and Drug Administration Amendments Act of 2007 (FDAAA) to enhance patient enrollment, provide a mechanism to track subsequent progress of clinical trials, provide more complete results information, and enhance patient access to and understanding of the results of clinical trials. The proposed requirements would apply to the responsible party (meaning the sponsor or designated principal investigator) for certain clinical trials of drugs (including biological products) and devices that are regulated by the Food and Drug Administration (FDA) and for pediatric postmarket surveillances of a device that are ordered by FDA. To read the Federal Register Notice and to make comments electronically.

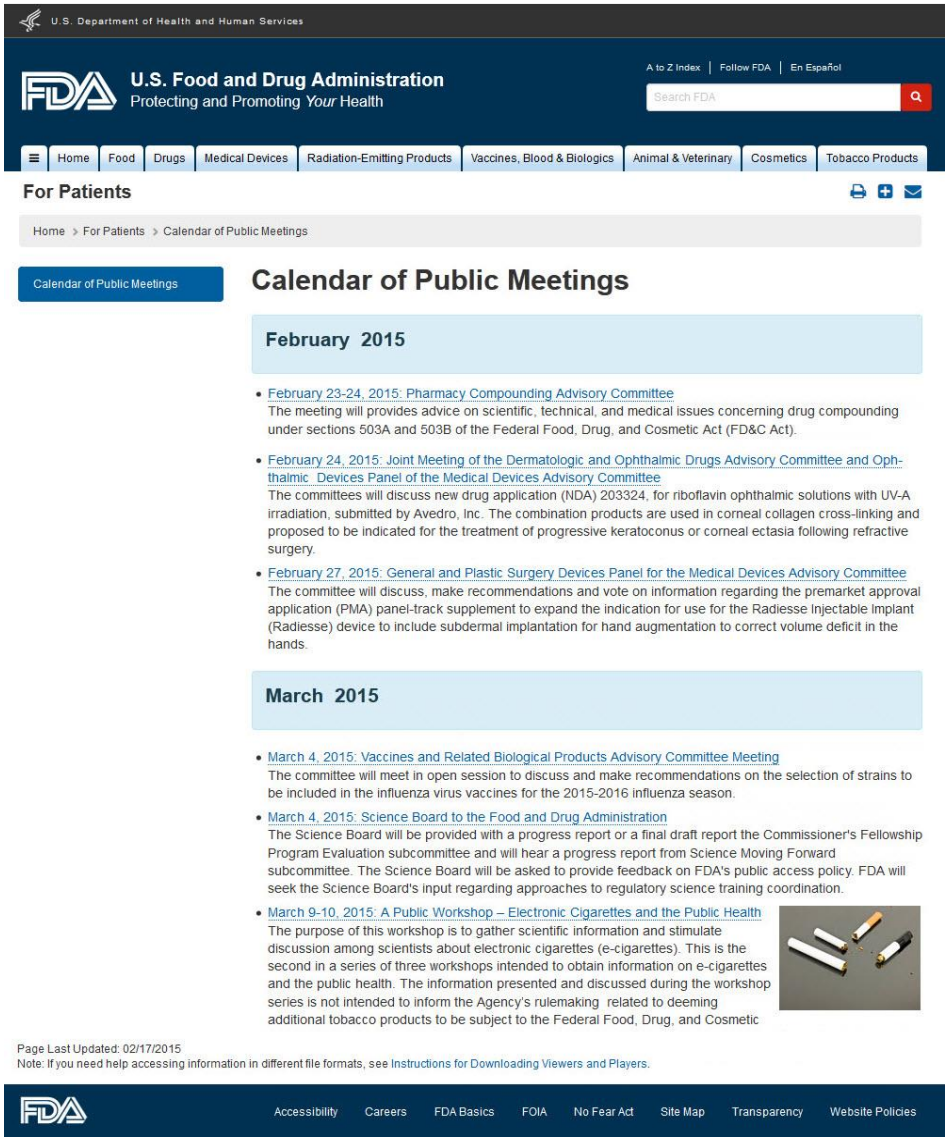
Opportunities to Comment Closing in March 2015

Opportunities to Comment Closing in April 2015

Opportunities to Comment Closing in May 2015



Opportunities for Input through Public Meetings



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For Patients

Home > For Patients > Calendar of Public Meetings

Calendar of Public Meetings

Calendar of Public Meetings

February 2015

- February 23-24, 2015: Pharmacy Compounding Advisory Committee
  The meeting will provides advice on scientific, technical, and medical issues concerning drug compounding under sections 503A and 503B of the Federal Food, Drug, and Cosmetic Act (FD&C Act).
- February 24, 2015: Joint Meeting of the Dermatologic and Ophthalmic Drugs Advisory Committee and Ophthalmic Devices Panel of the Medical Devices Advisory Committee
  The committees will discuss new drug application (NDA) 203324, for riboflavin ophthalmic solutions with UV-A irradiation, submitted by Avedro, Inc. The combination products are used in corneal collagen cross-linking and proposed to be indicated for the treatment of progressive keratoconus or corneal ectasia following refractive surgery.
- February 27, 2015: General and Plastic Surgery Devices Panel for the Medical Devices Advisory Committee
  The committee will discuss, make recommendations and vote on information regarding the premarket approval application (PMA) panel-track supplement to expand the indication for use for the Radiesse Injectable Implant (Radiesse) device to include subdermal implantation for hand augmentation to correct volume deficit in the hands.

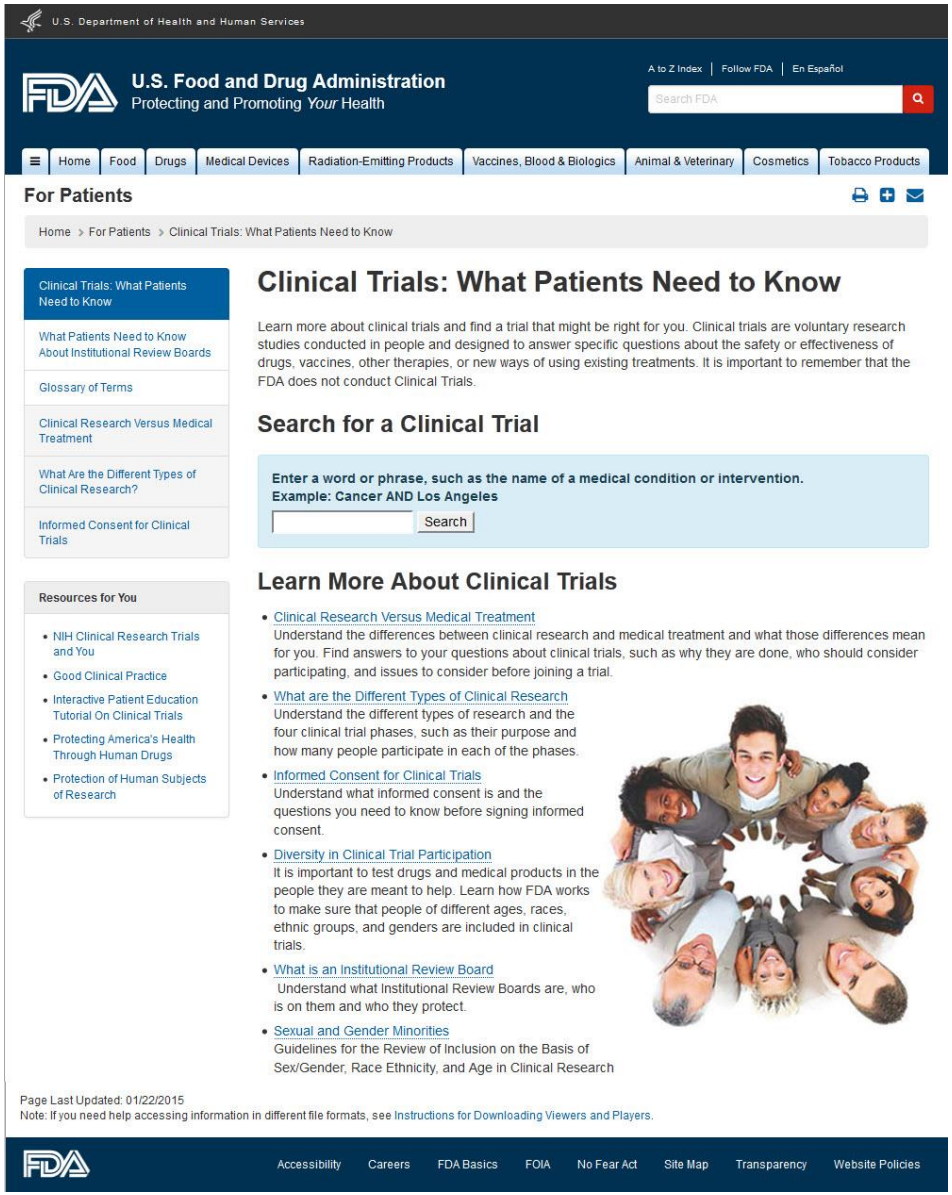
March 2015

- March 4, 2015: Vaccines and Related Biological Products Advisory Committee Meeting
  The committee will meet in open session to discuss and make recommendations on the selection of strains to be included in the influenza virus vaccines for the 2015-2016 influenza season.
- March 4, 2015: Science Board to the Food and Drug Administration
  The Science Board will be provided with a progress report or a final draft report the Commissioner's Fellowship Program Evaluation subcommittee and will hear a progress report from Science Moving Forward subcommittee. The Science Board will be asked to provide feedback on FDA's public access policy. FDA will seek the Science Board's input regarding approaches to regulatory science training coordination.
- March 9-10, 2015: A Public Workshop – Electronic Cigarettes and the Public Health
  The purpose of this workshop is to gather scientific information and stimulate discussion among scientists about electronic cigarettes (e-cigarettes). This is the second in a series of three workshops intended to obtain information on e-cigarettes and the public health. The information presented and discussed during the workshop series is not intended to inform the Agency's rulemaking related to deeming additional tobacco products to be subject to the Federal Food, Drug, and Cosmetic

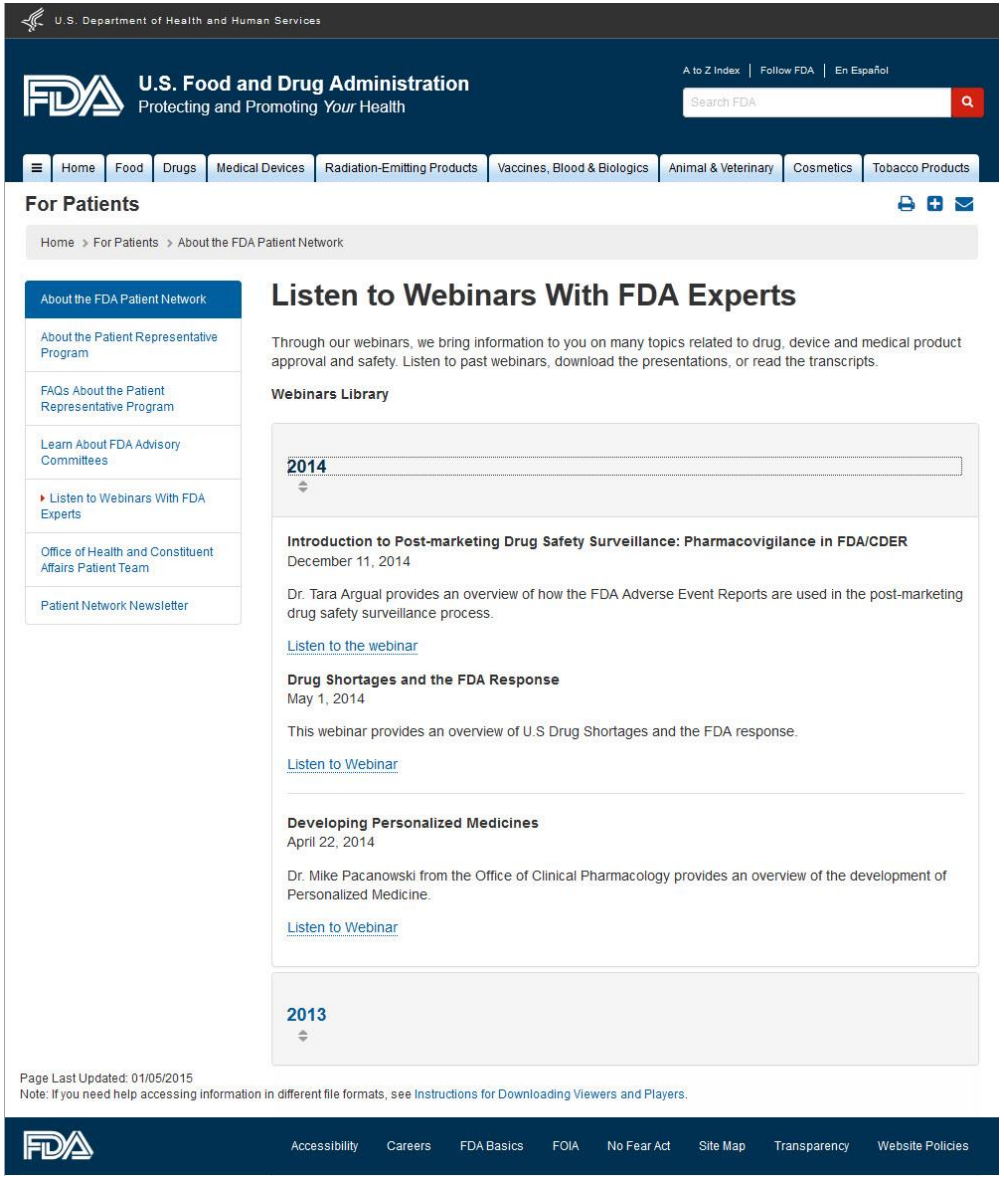
Page Last Updated: 02/17/2015
Note: If you need help accessing information in different file formats, see Instructions for Downloading Viewers and Players.

Accessibility | Careers | FDA Basics | FOIA | No Fear Act | Site Map | Transparency | Website Policies

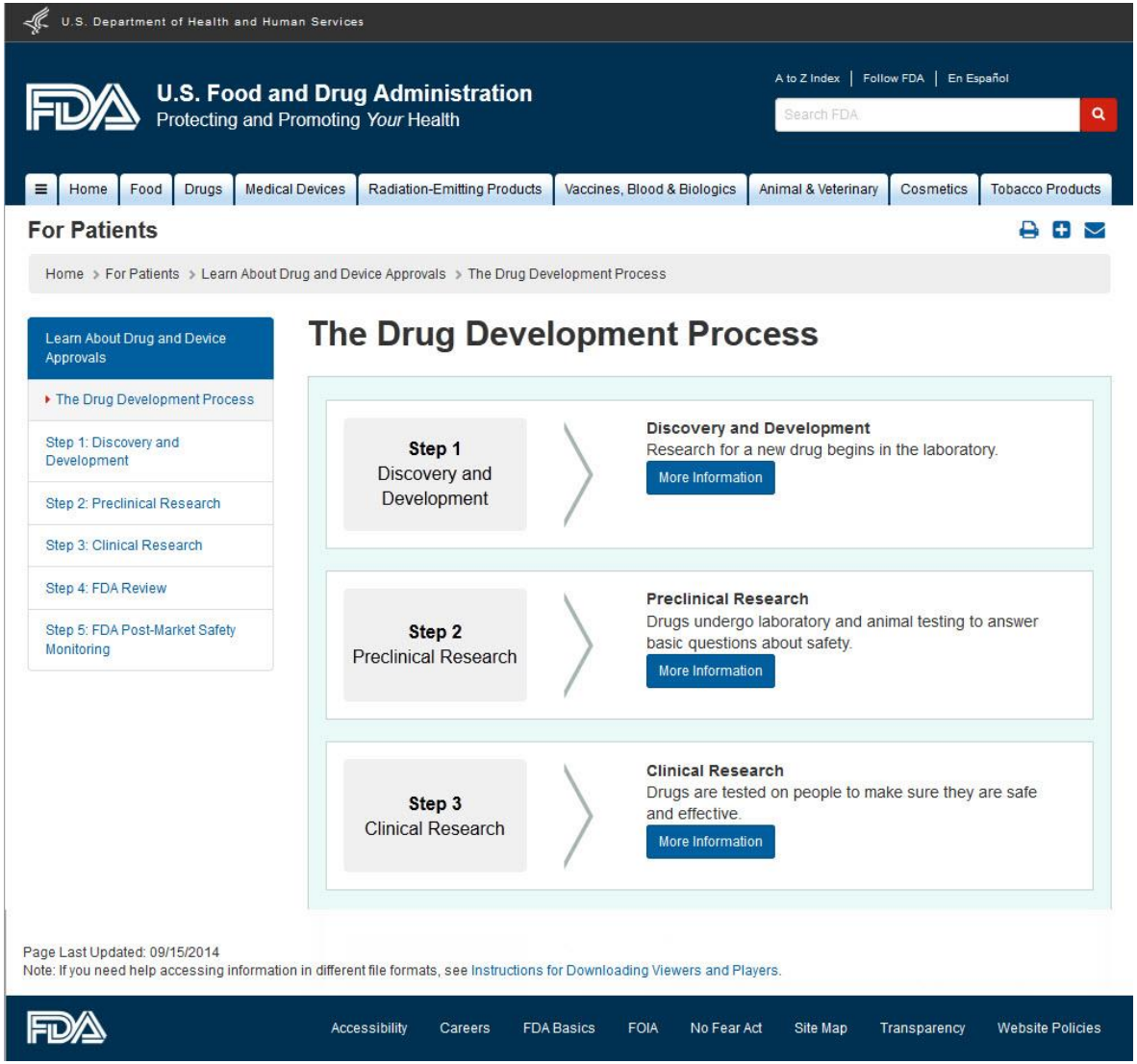
Because knowledge about the process is important to meaningful advocacy and engagement



Webinars with FDA Experts

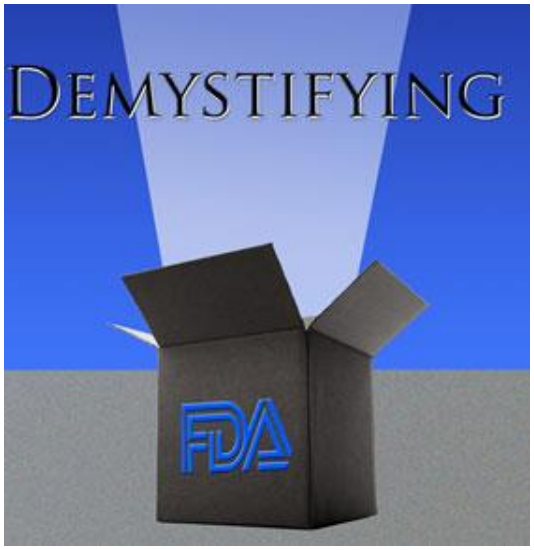


Drug and Device Development

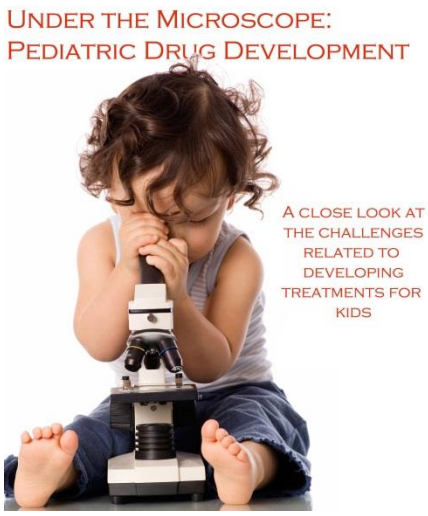


Patient Network Meetings and Webinars

May 2012



September 2013



September 2014

- **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

Bisphosphonate / Hepatitis

Meetings

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

Office of Health and Constituent Affairs

Patient Liaison Team

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