



## **Patient Representative Program**

Richard Klein

Office of Health and Constituent Affairs  
Patient Liaison Program  
Food and Drug Administration



**March 9, 2015**



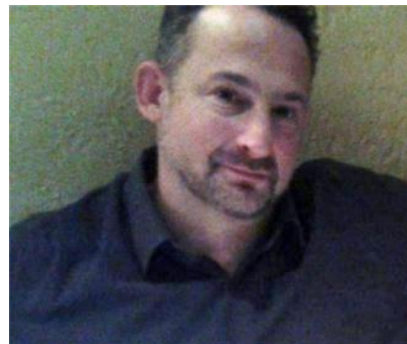
# Input from patients and patient advocates

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



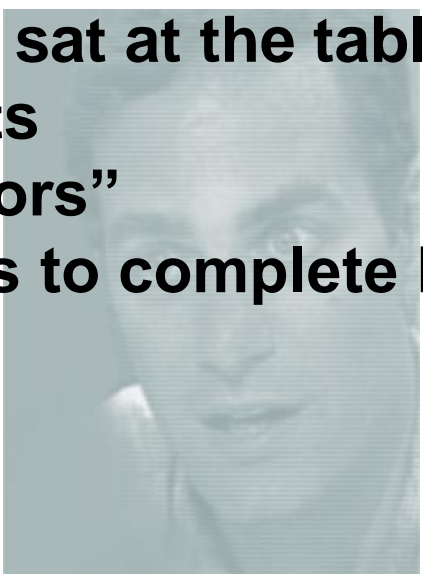


# Early AIDS Involvement



# 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?

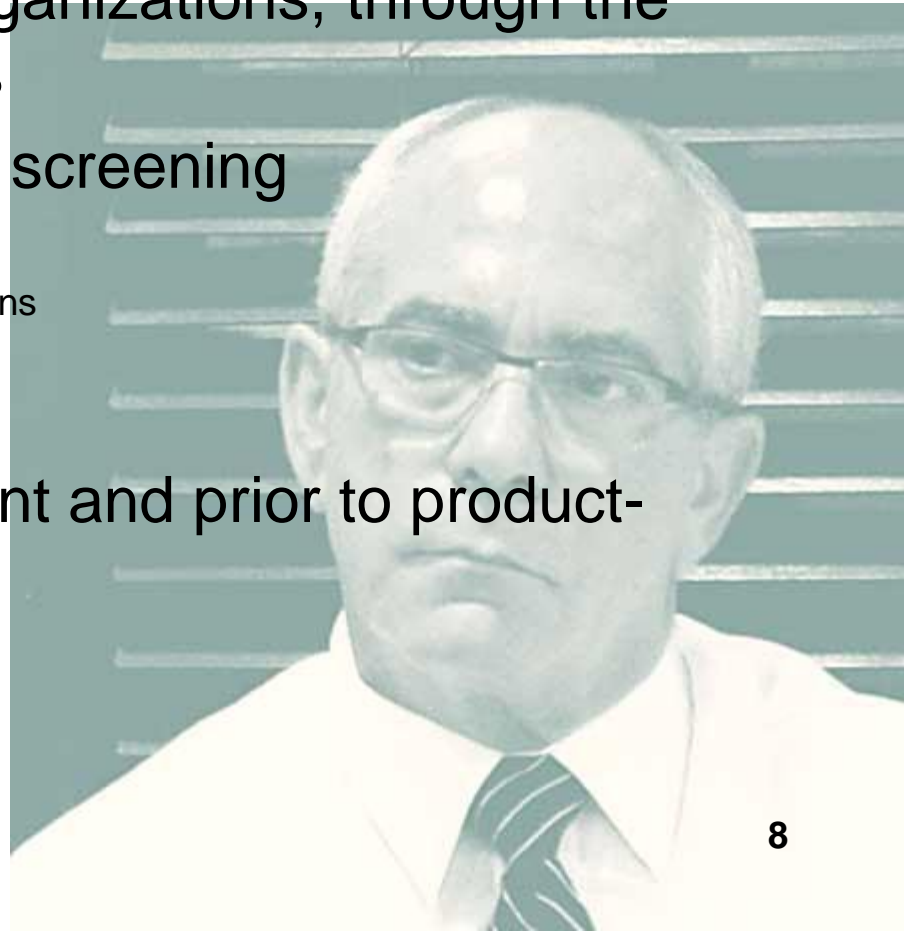
- Personal experience with the disease or condition as either a patient or primary care giver
- Patient community awareness; active in patient advocacy organizations, knowledgeable about treatment options and research, other advocacy activities
- Someone who is analytical and objective; doesn't need 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



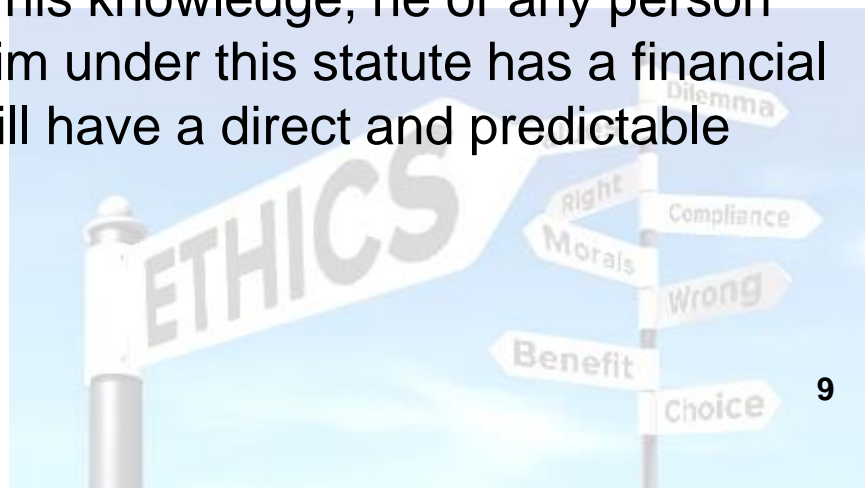


# Conflict of Interest


*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/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

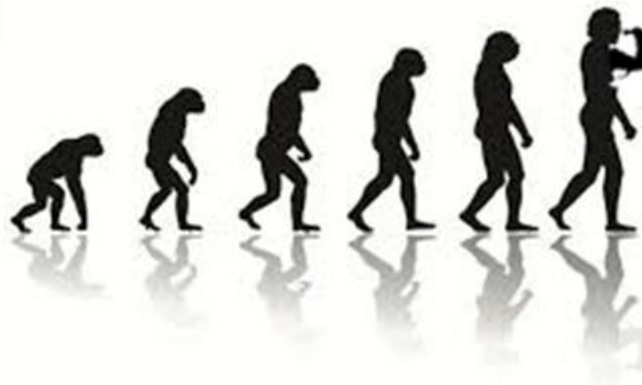
# Training

- 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 -*

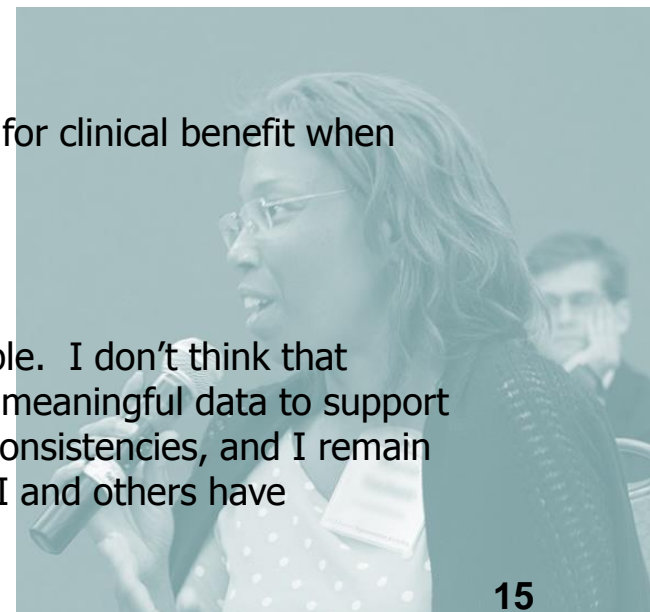
"I recall asking the sponsor to explain how there could be a claim for clinical benefit when there was no survival advantage."

-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 -*

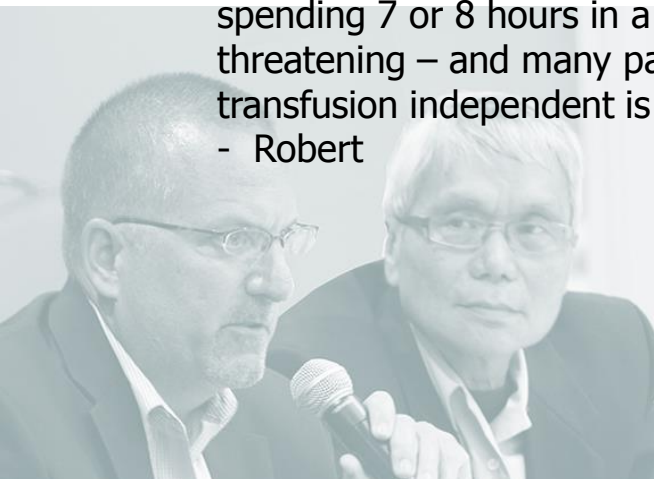
I don't think the tools are adequate to address diversion or the use of any reformulation to prevent any problems with children or pediatrics. That really wasn't addressed today. If you're planning to have something that is tamper-resistant, abuse resistant – then have only tamper resistant. Have only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







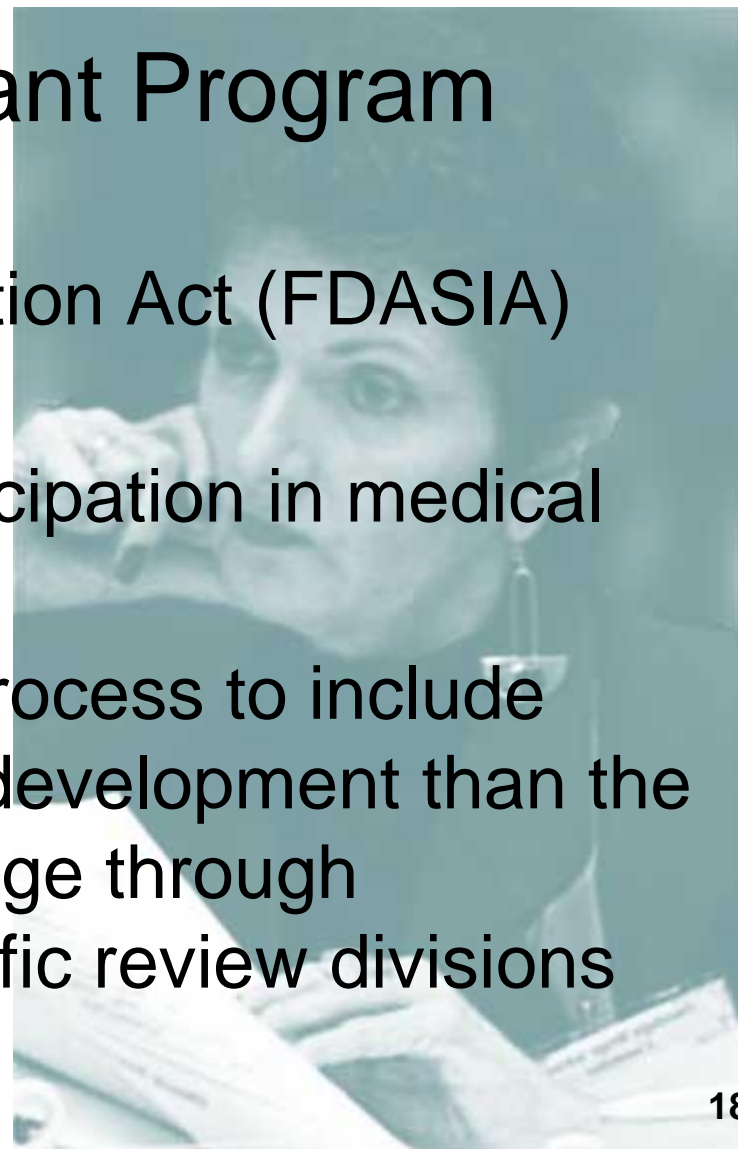
U.S. Food and Drug Administration  
Protecting and Promoting Public Health

[www.fda.gov](http://www.fda.gov)



# 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

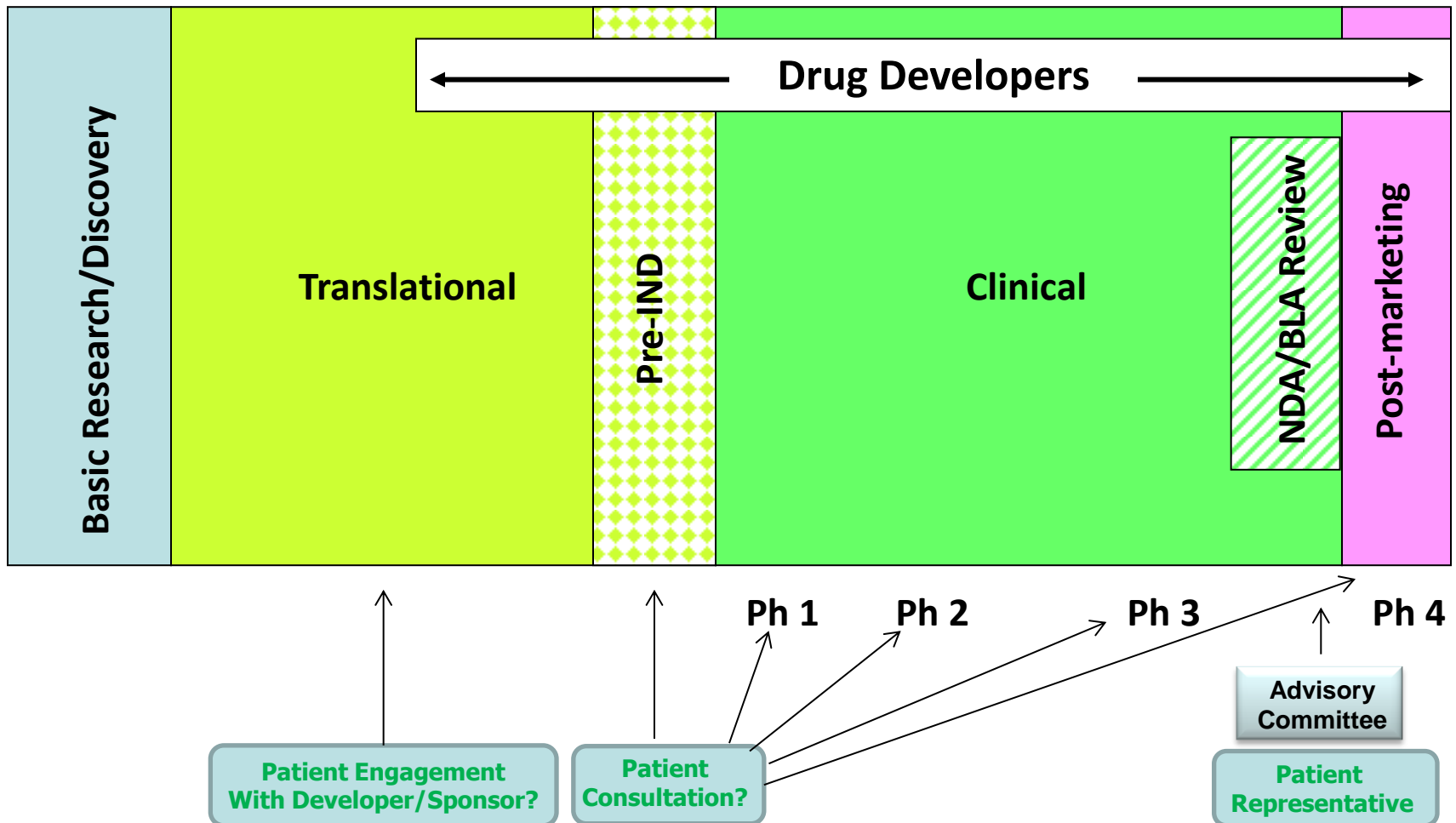


# Divisional Assignments for Patient Consultants

- 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.

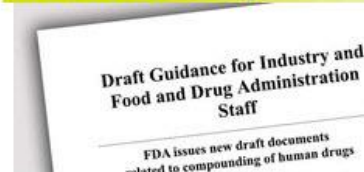
This bi-weekly newsletter provided by the Office of Health and Constituent Affairs at the Food and Drug Administration (FDA) is intended to inform you of FDA-related information on a variety of topics, including new product approvals, significant labeling changes, safety warnings, notices of upcoming 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

The screenshot shows the 'For Patients' section of the FDA website. It features a navigation bar with 'Home > For Patients > Comment on Current FDA Draft Guidances'. Below the navigation is a blue button labeled 'Comment on Current FDA Draft Guidances'. The main heading is 'Comment on Current FDA Draft Guidances' with the subtext 'The Daily Journal of the United States Government'. The Federal Register logo and 'Food and Drug Administration' are displayed. A list of topics is provided:
 

- foods
- drugs
- biologics
- cosmetics
- radiation-emitting electronic products
- medical devices
- tobacco products

 A paragraph explains that FDA rules have great impact on the nation's health, industries and economy. Below this, instructions are given for how to participate in the rule-making process by commenting in writing. A section titled 'On regulations you will find read and comment on FDA draft guidances and other FDA related documents' provides further details. At the bottom, there is a blue box with the text 'To learn more about submitting effective comments to the FDA docket,' followed by a list of 'Opportunities to Comment Closing in February 2015', 'Opportunities to Comment Closing in March 2015', and 'Opportunities to Comment Closing in April 2015'.



# Opportunities for Input through Public Meetings

U.S. Department of Health and Human Services

**U.S. Food and Drug Administration**  
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Home Food Drugs Medical Devices Radiation-Emitting Products Vaccines, Blood & Biologics Animal & Veterinary Cosmetics Tobacco Products

**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 provide 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

The screenshot shows the FDA website's 'Clinical Trials: What Patients Need to Know' page. At the top, there is a navigation bar with the FDA logo and the text 'U.S. Food and Drug Administration Protecting and Promoting Your Health'. Below this is a search bar and a menu with categories like Home, Food, Drugs, Medical Devices, etc. The main content area is titled 'Clinical Trials: What Patients Need to Know' and includes a sub-header 'For Patients'. The page features a sidebar with links to 'What Patients Need to Know About Institutional Review Boards', 'Glossary of Terms', 'Clinical Research Versus Medical Treatment', 'What Are the Different Types of Clinical Research?', and 'Informed Consent for Clinical Trials'. The main content area has a search box with the example 'Cancer AND Los Angeles' and a 'Search' button. Below the search box is a section titled 'Learn More About Clinical Trials' with a list of links to various resources. At the bottom right of the main content area, there is a photograph of a diverse group of people smiling and huddled together. The footer of the page includes the FDA logo and links for Accessibility, Careers, FDA Basics, FOIA, No Fear Act, Site Map, Transparency, and Website Policies.



# Webinars with FDA Experts

U.S. Department of Health and Human Services

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Home | Food | Drugs | Medical Devices | Radiation-Emitting Products | Vaccines, Blood & Biologics | Animal & Veterinary | Cosmetics | Tobacco Products

**For Patients**

Home > For Patients > About the FDA Patient Network

**About the FDA Patient Network**

- About the Patient Representative Program
- FAQs About the Patient Representative Program
- Learn About FDA Advisory Committees
- ▶ Listen to Webinars With FDA Experts
- Office of Health and Constituent Affairs Patient Team
- Patient Network Newsletter

## Listen to Webinars With FDA Experts

Through our webinars, we bring information to you on many topics related to drug, device and medical product approval and safety. Listen to past webinars, download the presentations, or read the transcripts.

**Webinars Library**

**2014**

**Introduction to Post-marketing Drug Safety Surveillance: Pharmacovigilance in FDA/CDER**  
December 11, 2014

Dr. Tara Argual provides an overview of how the FDA Adverse Event Reports are used in the post-marketing drug safety surveillance process.

[Listen to the webinar](#)

**Drug Shortages and the FDA Response**  
May 1, 2014

This webinar provides an overview of U.S Drug Shortages and the FDA response.

[Listen to Webinar](#)

**Developing Personalized Medicines**  
April 22, 2014

Dr. Mike Pacanowski from the Office of Clinical Pharmacology provides an overview of the development of Personalized Medicine.

[Listen to Webinar](#)

**2013**

Page Last Updated: 01/05/2015  
Note: If you need help accessing information in different file formats, see [Instructions for Downloading Viewers and Players](#).

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# Drug and Device Development

U.S. Department of Health and Human Services

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A to Z Index | Follow FDA | En Español

Search FDA

Home | Food | Drugs | Medical Devices | Radiation-Emitting Products | Vaccines, Blood & Biologics | Animal & Veterinary | Cosmetics | Tobacco Products

**For Patients**

Home > For Patients > Learn About Drug and Device Approvals > The Drug Development Process

**Learn About Drug and Device Approvals**

- 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 Drug Development Process

**Step 1**  
Discovery and Development

**Discovery and Development**  
Research for a new drug begins in the laboratory.

[More Information](#)

**Step 2**  
Preclinical Research

**Preclinical Research**  
Drugs undergo laboratory and animal testing to answer basic questions about safety.

[More Information](#)

**Step 3**  
Clinical Research

**Clinical Research**  
Drugs are tested on people to make sure they are safe and effective.

[More Information](#)

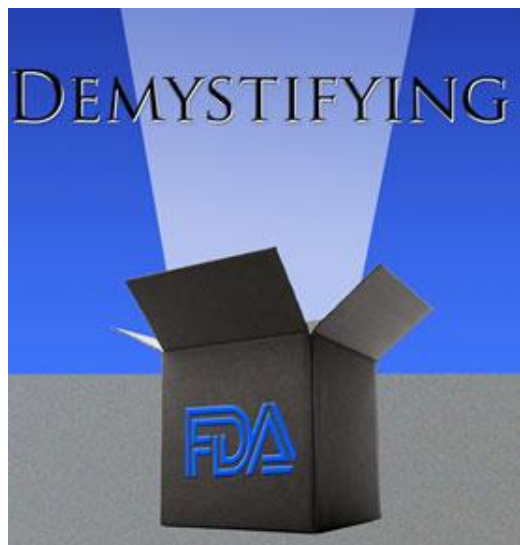
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**FDA** Accessibility | Careers | FDA Basics | FOIA | No Fear Act | Site Map | Transparency | Website Policies

# Patient Network Meetings and Webinars



May 2012



September 2013

UNDER THE MICROSCOPE:  
PEDIATRIC DRUG DEVELOPMENT



A CLOSE LOOK AT  
THE CHALLENGES  
RELATED TO  
DEVELOPING  
TREATMENTS FOR  
KIDS

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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[Richard.Klein@fda.hhs.gov](mailto:Richard.Klein@fda.hhs.gov)