

# How Patient Advocates Participate in Drug Development

**Patty Spears**

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2018

# Patient Engagement at the FDA

- ◆ The FDA has opportunities to engage patients
- ◆ All engagements are different, requiring different levels of expertise



**How can you become involved?**

# FDA Patient Engagement

- ◆ Go to [www.fda.gov/ForPatients/PatientEngagement/](http://www.fda.gov/ForPatients/PatientEngagement/)
- ◆ Many different ways to engage

## My involvement with the FDA

# Join the FDA Patient Representative Program

- ◆ Training!!
- ◆ Exposure to advocates of all diseases, not just cancer, learning from peers
- ◆ Ongoing education through webinars
- ◆ Request for patients with specific conditions throughout the year.

# Patient Focused Drug Development (PFDD) Initiative

- ◆ Hearing directly from patients about their specific experience.
- ◆ Scheduled throughout the year
- ◆ Help the FDA define what is important to patient to measure, how to measure and what amount of change is meaningful to patients.
- ◆ Patients do not need training to participate, all you need is your experience and ability to share with others

# Topic Specific Workshops

- ◆ Innovations in Breast Cancer Drug Development – Neoadjuvant Breast Cancer Workshop, sponsored by FDA, ASCO and AACR (2013)
- ◆ Innovations in Breast Cancer Drug Development – Next Generation Oncology Trials Breast Cancer Workshop sponsored by FDA, ASCO, AACR, BCRF (2014)
- ◆ These are more informational, but still require knowledge about the topic in order to contribute and bring the patient voice to the conversation

# Advisory Committees

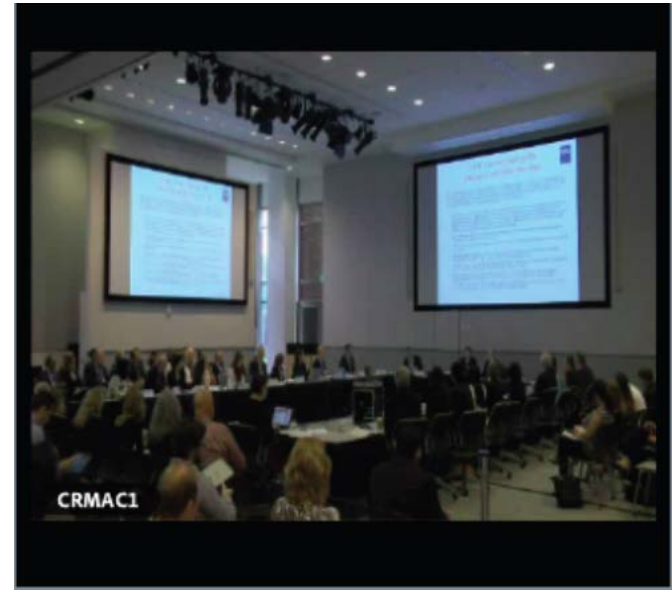
- ◆ Advisory Committees can require a higher level of experience or at least a level of commitment that a patient will learn what they need to know to contribute to the discussion and decisions being made.

**Learn more about the topic before you participate.**

# ODAC

## Oncologic Drug Advisory Committee

- ◆ You are part of a team that reviews a drug application.
- ◆ These are applications that the FDA is seeking additional advice on, not a clear approve/deny.
- ◆ This is not binding for the FDA but they take into account your votes and comments.





# ODAC – what to expect

- ◆ The data is sent to you to review. A lot of confidential materials.
  - ◆ Set aside dedicated time to review
- ◆ The data is presented at the meeting by the company, the FDA and the public (open comments)
  - ◆ You will have the opportunity to ask questions
- ◆ At the end, you are required to vote to recommend approval or not or abstain.
  - ◆ Voting is done on your microphone
  - ◆ After everyone votes, you say how you voted and why you voted that way.
- ◆ This is a big deal, it is streamed live and videotaped. Reporters can be there as well as a large in-person audience.

# Patient Reported Outcome Measurements PROMs

- ◆ PROMs are one part of Clinical Outcome Assessments (COAs)
- ◆ FDA is developing a series of 4 COA guidance documents
  - ◆ Guidance 1: Whom do you get input from, and why? How do you collect the information?
  - ◆ Guidance 2: What do you ask, and why? How do you ask non-leading questions that are well-understood by a wide range of patients and others?
  - ◆ Guidance 3: How do you decide what to measure in a clinical trial and select or develop fit-for-purpose clinical outcome assessments (COAs)?
  - ◆ Guidance 4: Once you have a COA measurement tool and a way to collect data using it, what is an appropriate clinical trial endpoint?

# PFDD

October 15-16, 2018

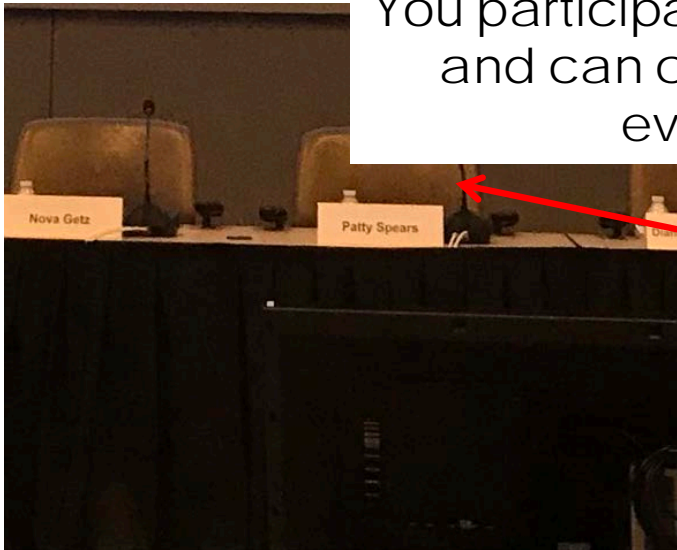
FDA U.S. FOOD & DRUG  
ADMINISTRATION

Public Workshop on Patient Focused Drug Development

Methods to Identify What is Important to Patients and  
Select, Develop or Modify Fit-for-Purpose Clinical  
Outcome Assessments

**Public Internet Access**  
Network: FDA-Public  
Password: publicaccess

You participate equally at the table  
and can comment along with  
everyone else!



# **GUIDANCE 2 DISCUSSION DOCUMENT OVERVIEW**

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**Methods to Identify What Is Important to Patients**

**Asking the Right Questions**

**Best Practices in How to Do  
Qualitative & Quantitative Research (Operationalization)**

# Send us your comments!

If you have examples, information, feedback or comments, please submit to the public docket for this workshop!

The docket will close on December 14, 2018.

How do you submit a comment?

- Please visit:

<https://www.regulations.gov/document?D=FDA-2018-N-2455-0001>

- Or search "Patient Focused Drug Development Workshop" on [www.regulations.gov](http://www.regulations.gov)

And **Click Comment Now!**

The screenshot shows the 'regulations.gov' website interface. At the top, there is a navigation bar with 'Home', 'Help', 'Resources', and 'Contact Us'. A search bar contains the text 'patient focused drug development workshop'. The main content area features a notice titled 'Patient-Focused Drug Development Guidance: Methods To Identify What Is Important to Patients and Select, Develop, or Modify Fit-for-Purpose Clinical Outcome Assessments; Public Workshop; Request for Comments'. Below the title, it states 'This Notice document was issued by the Food and Drug Administration (FDA)'. There is an 'Action' section with the text 'Notice of public workshop; request for comments.' and a 'Summary' section. A 'Date' section indicates the public workshop will be held on October 15 and 16, 2018. An 'Addresses' section provides the location: FDA's White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, the Great Room (Rm. 1503), Silver Spring, MD 20993. A 'Comment Now!' button is prominently displayed in the top right corner of the notice area, with a red arrow pointing to it from the text 'Click Comment Now!' in the slide's text. To the right of the main notice, there is a sidebar with a 'Document Information' section showing the ID 'FDA-2018-N-2455-0001', the date posted 'Aug 13, 2018', and the federal register number '2018-17272'. At the bottom of the sidebar, a 'Comments' section shows '1 Comments Received'.

# Final Thoughts

- ◆ Seek opportunities
- ◆ Do the work and do a good 'job' –be prepared
- ◆ Network! Network!
- ◆ Keep learning, challenge yourself.
- ◆ It is hard work, but it is worth it! You can make a difference!

# Questions



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