How Patient Advocates Participate in Drug Development

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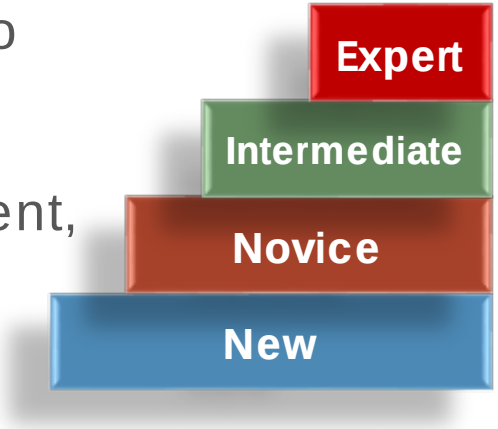
Patient Engagement at the FDA

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



A lot of experience and knowledge

More experience

Some training/experience

No experience

How can you become involved?

FDA Patient Engagement

- Go to 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 willlearn 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

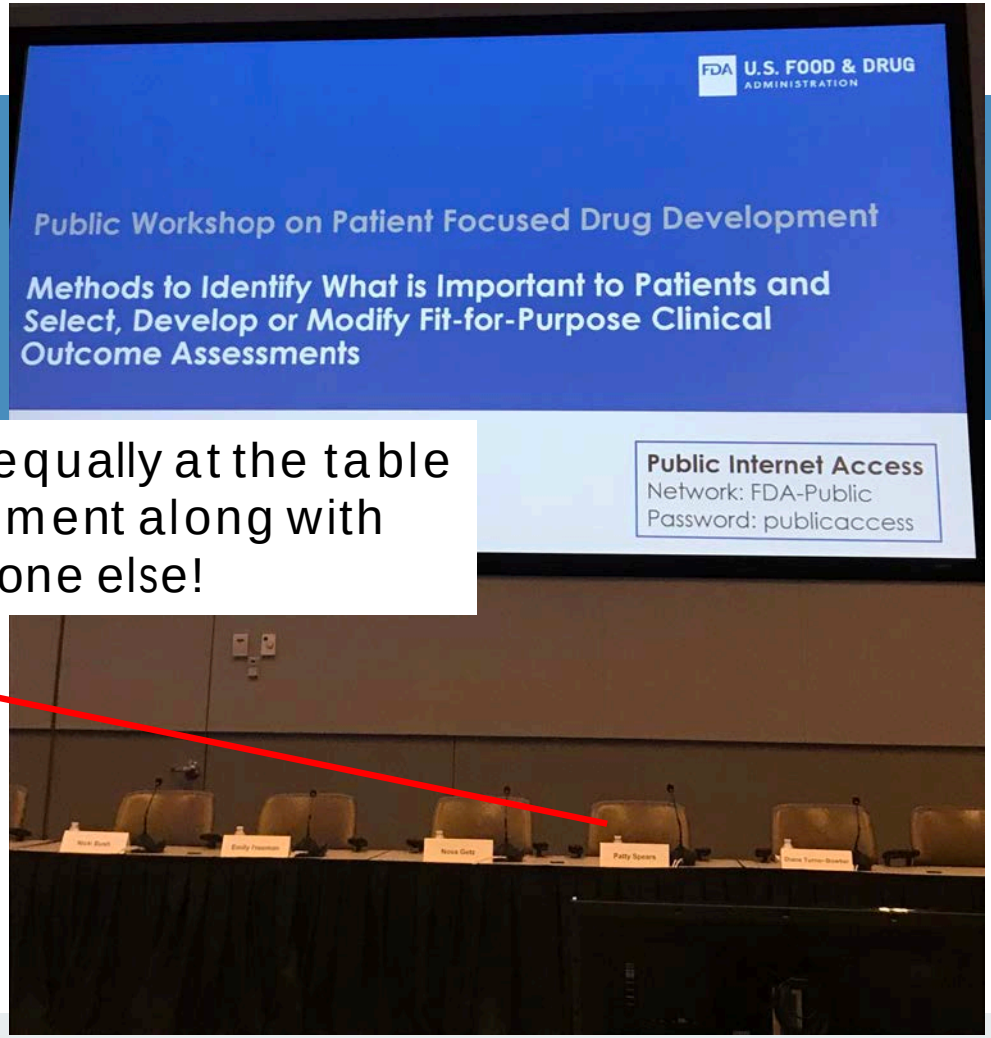
- The data is sent to you to review. A lot of confidential materials.
  - Set aside dedicated time to review
- The data is presented at the meeting by the company, the FDA and the public (open comments)
  - You will have the opportunity to ask questions
- At the end, you are required to vote to recommend 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

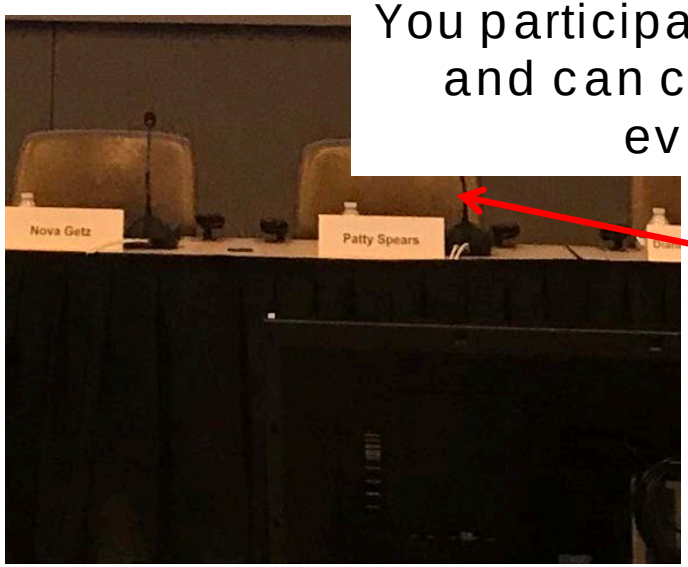
- PROMs are one part of Clinical Outcome Assessments (COAs)
- FDA is developing a series of 4 COA guidance documents
  - Guidance 1: Whom do you get input from, and why? How do you collect the information?
  - Guidance 2: What do you ask, and why? How do you ask non-leading questions that are well-understood 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



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



GUIDANCE 2 DISCUSSION DOCUMENT OVERVIEW

Methods to Identify What Is Important to Patients

Asking the Right Questions

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

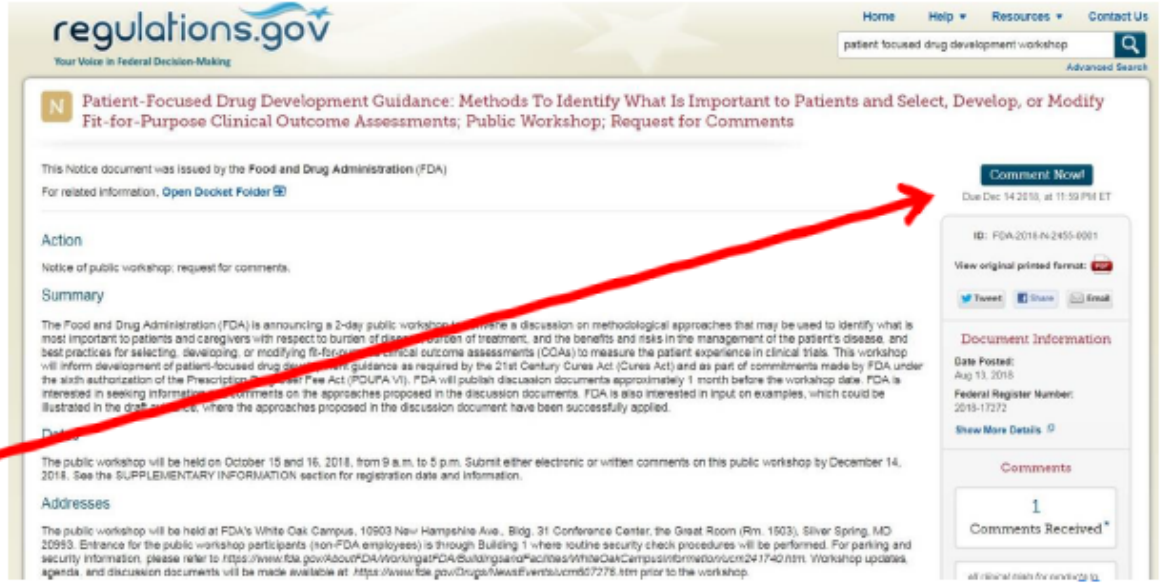
Send us your comments!

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
- And **Click Comment Now**!



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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