NATIONAL CENTER FOR HEALTH RESEARCH

The Voice For Prevention, Treatment And Policy

PCOR New Medical Products Project

Strengthening Patient Voices on Research Criteria

National Center for Health Research • 202 223-4000 • center4research.org

Background

The National Center for Health Research recruited patient and caregiver advocates to attend two events in June 2014 at the American Association for the Advancement of Science (AAAS) in Washington, D.C. On Thursday, June 12, we held a *Patient Advocacy Training Workshop* for 30 patients, caregivers, and their advocates to increase their understanding of and participation in FDA public meetings, public comments, and other opportunities to strengthen patient-centeredness of FDA research requirements. These participants are listed in Appendix A. Twenty-nine of the 30 Workshop participants and one additional invited patient advocate attended the Conference that we co-hosted with Harvard University and AAAS on Friday, June 13th, entitled "Evidence for New Medical Products: Implications for Patients and Health Policy."

This work was partially supported through a Patient-Centered Outcomes Research Institute (PCORI) Program Award.

The June 12 Workshop and June 13 Conference

The agendas for the *Patient Advocacy Training Workshop* and the *Evidence for New Medical Products Conference* are included in **Appendix B**.

The *Advocacy Workshop* was led by 10 patient and consumer advocates, who explained the scientific evidence and stakeholder perspectives that the Food and Drug Administration (FDA) considers when making decisions to approve or recall a medical product. With the participants, they explored the different ways patient advocates can make their voices heard by the FDA.

Patient advocate Diana Levine discusses her experience with a serious adverse reaction at the June 12 Workshop

The *Evidence for New Medical Products Conference* included five panels, offering a range of information and perspectives on topics such as weighing the risks and benefits of different research criteria for approval, the appropriate use of surrogate endpoints and biomarkers in research studies, and the role of post-market "big data" in the FDA approval process. Three patient advocates served as panel members, in addition to PCORI Director Joe Selby, and several other patient advocates asked questions and made comments during the Q & A sessions.

Patient Advocacy Workshop Objectives

Sally Greenberg of National Consumers League and Kim Witczak of WoodyMatters present on how patients can become engaged on FDA issues

The FDA approves drugs and devices for specific indications when the agency concludes that the benefits usually outweigh the risks. With that ratio in mind, the FDA approves numerous medical products despite evidence that many patients may not benefit and some patients may be seriously harmed. The FDA welcomes patient input on numerous aspects of their approval and regulatory process, but few patient advocates are aware of the opportunities or are able to take advantage of the chance to share their perspective when it matters most, such as at public meetings or in written comments that are an integral part of the FDA regulatory process. Many patient advocates do not fully understand FDA research criteria; for example, they might not realize that FDA approval does not guarantee that the benefits outweigh the risks for every patient, or that using the drug or device for non-approved indications may have substantial risks and no proven benefits. FDA terminology can be confusing; for example, FDA's "breakthrough" designation means that a new drug is considered promising, but that only preliminary studies have been completed and, therefore, the product is not yet proven safe or effective. Recent research has delineated the risks and benefits of different approval mechanisms, and this knowledge will help patients, caregivers and others to be more effective advocates as they become engaged with FDA opportunities for patient input.

The objectives of the Workshop were to explain these and other nuances of the FDA approval process, and to:

1) Teach patients/caregivers about available opportunities to provide a patient perspective on FDA research criteria for new medical products;
2) Train patients/caregivers to share their views at FDA public meetings and written comment opportunities; and
3) Assist patients in understanding the implications for patients of FDA approval criteria and sharing their views with the research and policy communities.

To determine whether or not we achieved our objectives, we administered a pretest/posttest questionnaire (see **Appendix C**) to assess changes in participants' knowledge, attitudes, and beliefs, and we asked participants to anonymously fill out a separate evaluation questionnaire (see **Appendix D)**.

Results of the Pretest/Posttest for *Patient Advocacy Workshop*

Impact on Participants' Knowledge

We evaluated the responses from three identical questionnaires to determine participants' knowledge and attitudes before the Workshop and how those changed after the Workshop and after the Conference. The questionnaires were handed out before the start of the June 12th Workshop, at the end of the June 12th Workshop approximately 6.5 hours later, and immediately following the Conference on June 13th. Of the 30 men and women who attended the Workshop as participants, 21 completed the pretest and the first posttest. The nine individuals who did not fill out the pretest were late arrivals or highly informed advocates who attended as presenters as well as participants.

The questionnaire consisted of 11 questions: 8 multiple choice questions that tested knowledge, 2 open-ended questions asking their opinions, and one multi-part rating question [the questions and answers are listed in Appendix C]. Participants included their first name on the questionnaire so that we could compare pretest and posttest scores. We performed several statistical analyses to analyze whether the changes in scores were statistically significant, including a paired t-test and several Chi-square tests.

Joyce Bichler of Breast Cancer Action and Desiree Walker, patient advocate

Using a paired t-test (since the scores were not independent), we compared the total number of correct responses for the first 8 questions for all participants who completed both the pretest and first posttest (after the *Patient Advocacy Workshop*). These scores are presented in Table 1. With possible scores ranging from 0 to 8, participants averaged 4.1 on the pretest and 6.0 on the posttest, which shows a highly significant increase in knowledge ($p<.0005$).

Table 1: Paired t-Test to Compare Correct Answers in Pretest and Posttest

t-Test: Paired Two Sample for Means		
	Pretest Range	*Posttest I Range*
Mean	4.14	6.00
Variance	2.73	2.30
Observations	21	21
t Statistic	4.812	
P(T<=t) two-tail	0.0005	
t Critical two-tail	2.09	
Mean Difference: (Posttest I)-(Pretest)	1.86	
Stand Deviation of Difference	1.77	
Stand Error of Difference	0.39	
Upper 95% CI of Difference	2.66	
Lower 95% CI of Difference	1.05	

We also compared answers to each of those 8 questions, to determine what information was learned as a result of the Workshop and the Conference. Unfortunately, some participants were not able to stay for the entire Conference, which ended later than scheduled. Since only 15 of the 30 participants completed the 2nd posttest questionnaire (which was distributed after the Conference) we did not use any inferential statistical analyses to compare those responses. However, when we looked at the responses to all three questionnaires, it was clear that most learning took place as a result of the Workshop, not the Conference.

On 7 of the 8 questions testing knowledge, the number of participants responding correctly increased, sometimes dramatically. The percentages are presented in Table 2.

Table 2: Correct Answers to Individual Questions in Pretest and Posttest

Question	Pretest (n=21)	Posttest I (n=21)
Q#1 Define FDA drug approval	57% (12/21)	90% (19/21)
Q#2 Drug vs. device standards	33% (7/21)	95% (20/21)
Q#3 Define double-blind clinical trials	90% (19/21)	100% (21/21)
Q#4 Define randomized, controlled trial	86% (18/21)	67% (14/21)
Q#5 Define biomarkers or surrogate endpoints	67% (14/21)	90% (19/21)
Q#6 Advantages of studying biomarkers	62% (13/21)	76% (16/21)
Q#7 Differences between pre- and post-market studies	10% (2/21)	48% (10/21)
Q#8 Define statistical significance	10% (2/21)	48% (10/21)

Comparisons in shaded boxes were statistically significant.

We conducted chi-square comparisons on individual questions for the same participants who completed both the pretest and posttest before and after the Workshop, as a way to better understand what was learned. **The answers to the first two questions (Q#1 and Q#2) and last two questions (Q#7 and Q#8) were significantly more likely to be correct after the Workshop ($p < .05$, and .01, respectively) .** Answers to three other questions (Q #3, 5 and 6) were slightly, but not significantly, more likely to be correct after the Workshop.

In contrast, there was a small but not significant decrease in correct responses to Q4, the definition of a randomized controlled trial. The few wrong answers showed confusion between randomized controlled clinical trials and randomized double blind clinical trials.

Lessons Learned

Patient advocate John James asks a question at the June 13 Conference

- Some of the greatest learning by the patient/caregiver advocates was on the issues that participants were not fully informed about prior to the Workshop and were most actively engaged in during the Workshop: the meaning of FDA drug approval **(Q#1),** and the difference between FDA standards for medical devices and prescription drugs **(Q#2**).
- There was also substantial learning on two multiple choice questions that included more than one correct answer: the differences between premarket and post-market studies **(Q#7)** and the meaning of statistical significance **(Q#8)**. For the former, the incorrect answers tended to include differences that could ideally differentiate the two but often do not, such as greater diversity in the clinical trials. Since so few participants understood what statistical significance meant prior to the Workshop, their understanding increased significantly despite a rather brief explanation of the term during the Workshop.
- Most of the participants understood what a double blind clinical trial (**Q#3**) and randomized clinical trial (**Q#4)** were prior to the Workshop, with more than 85% of the participants answering both these questions accurately before the Workshop.
- Most learning on the items measured was a result of the *Patient Advocacy Workshop*, not the Conference aimed at researchers. However, it was difficult to determine the impact of the Conference because half the Workshop participants did not complete the posttest that was distributed after the Conference. Since the Conference ran a little late, some participants left before it was over and others left immediately afterwards in order to catch their planes.

Impact on Participants' Attitudes and Beliefs (Q#9-10)

Two questions focused on attitudes rather than knowledge, to see how the Workshop and Conference might influence attitudes on specific issues and on participation in FDA opportunities for patients.

Direct to Consumer Advertising

Question # 9 asked whether the FDA should change the ways that companies are required to provide risk information on direct to consumer advertising, an issue that FDA is currently considering. This opinion question was intended as an "ice breaker" and a way to measure attitudes about a topic with which all participants had personal experience. We were interested to see if the Workshop would change those views, even though the topic was not specifically included in the training.

Q#9: The FDA requires companies to mention potential risks when those companies advertise their drugs on TV or magazines. Are those warnings helpful to patients? How would you improve them?

Nearly all participants felt that the warnings were helpful or had the potential to be helpful to patients if the warnings were improved. All agreed that the warnings required by the FDA need improvement. On the pretest, some felt that the warnings needed to be changed while others felt that the warnings would be fine if patients were trained to be better consumers or were able to review the risks with their doctor.

Since this topic was not included in the Workshop, responses changed little, although they tended to be more strongly worded in the Posttest.

Here are examples of responses from the Pretest:

> Participant A, Pretest: *The warnings for medications are helpful to patients, but its delivery is usually not as eye catching as the product. Similar to ads for beer and liquor, many consumers are not tuned to post-message info about warnings.*

> Participant B, Pretest: *Yes, some warnings will scare me away from using the new drugs with all the side effects. To improve the warnings, make the side effects in bigger script and more understandable for the patient. Doctors should go over the drug with patient.*

Here are examples of responses from the Posttest:

> Participant C, Posttest I: *Manufacturer may mention "moodiness" but neglect to state deep sadness - severe depression - uncontrollable crying - which is much more than "moodiness" indicates.*

> Participant D, Posttest I: *Any warnings for drugs used in media is not effective at all because most people do not pay attention to warnings. Warnings would curb marketing and this will not occur. Improved education and consumer awareness campaigns would help.*

Here are examples of responses from the Posttest II:

> Participant E, Post Test II: *I think these TV warnings are better than no warning at all. However, too often, TV ads make potentially dangerous drugs look more appealing, I don't think these ads belong on TV.*

> Participant D, Posttest II: *Though the warnings are somewhat helpful, they can sometimes cause patients to think that the listed risks are the only risks. They could be improved by requiring the prescribing physician to review them with the patient.*

Q#9 continued: How would you improve them?

- 15 respondents recommended changing the order of warnings given from most severe to least severe
- Other proposed improvements:
    - Increasing the font size of written warnings
    - Decreasing the speed at which warnings are spoken
    - Making warnings more understandable for the average patient
- Many participants stated that individuals do not pay enough attention to these warnings. They suggested more thorough communication on the part of the advertisement, such as listing the most severe side effects first or stating the risks both in print and through audio, would help improve patient understanding. Another suggestion was to require that physicians explain to patients the potential risks and side-effects of any medication they are prescribing.

FDA Approval: Too Fast or Too Slow?

A major policy issue being publicly debated today is whether the FDA approval process is too fast or too slow because of agency research criteria. Numerous patient advocacy organizations have criticized the FDA in recent years for taking too long to approve new drugs and devices, and FDA has responded to pressure to speed up their approval process by changing research criteria required for approval, for example reducing the number of clinical trials from two to one and substituting biomarker evidence for clinically significant outcomes. Question #10 asked participants their views on this important issue.

John James of Safe Patient Project and Kim Witczak of WoodyMatters participate in Workshop discussion

Q#10: Do you think the FDA approval process is too fast or too slow? If you have an opinion one way or the other, explain why you believe that to be true.

There was no strong consensus on this issue. In the pretest, 3 participants said they did not know enough about the approval process to comment, and others offered vague statements. When participants completed the posttest a few hours later, many of them were able to give thoughtful opinions on the FDA approval process. The answers of participants varied depending on their personal experiences with the FDA and various medical products. For example, patients who have had deaths in the family from cancer were more inclined to say that the FDA process is too slow. Participants who had family members harmed by a medical implant or drug complication were more inclined to say the FDA process was too fast.

Here are two examples of responses from the pretest:

Participant E, Pretest: *Too slow. People are dying waiting on the approval process.*

Participant F, Pretest: *The FDA approval process is not truly understood. There are many steps prior to FDA approval. There is also a lack of consistency in the process of approval for medical devices and drugs. It is difficult to truly gauge the process if it is unknown.*

Here are examples from the posttest I:

Participant G, Posttest I: *It varies depending on the intended treatment area. For some, either because of severity or lack of treatment options, it may be too slow. For others, where there are alternate options or lesser severity, it may be too fast.*

Participant H, Posttest I: *Too fast, there needs to be more post-market studies, and studies done on the effects of patients without a diagnosis or misdiagnosis can have.*

Here are examples from the posttest II:

Participant I, Posttest II: *Too slow. Too many people are dying because the medicine to cure them has not been approved but is available.*

Participant J, Posttest II: *The FDA's approval process for drugs and devices needs higher standards for pre-market studies and clinical trials. 510k devices require no evidence of clinical testing. Better safety testing is needed upfront and also post-market studies, all data needs to be made public.*

Advocates from Women Advocating for Reproductive Safety

Willingness to Participate in Future Advocacy (Q#11)

The survey asked participants to rank their willingness to participate in future advocacy, on a scale of 1-5, with 5 being "very likely to participate" and 1 being they would "never consider participating." Participants were presented with a number of different scenarios:

a. Speaking during the public comment period of an FDA meeting in the DC area
b. Speaking during the public comment period of an FDA meeting in a city near their homes
c. Writing a comment giving their perspective in response to an FDA "request for public comments"

d. Adding their name to a letter written by other patients or nonprofit organizations in response to an FDA "request for public comments"
e. Speak at a non-FDA event on FDA issues

On the pretest, answers ranged from 2 to 5, with participants most willing to participate in adding their name to a letter written by others (d) and least willing to participate in speaking during an FDA public comment period at a public FDA meeting in the Washington, DC area (a). However, 13 out of 21 participants reported, on each test, that they were very likely to consider participating in every suggested way (responded with a score of "5" on each question).

- Responses on the Pretest:
- Average willingness to participate: 4.5 [Range: 2.8-5]
- Responses on the Posttest I:
- Average willingness to participate: 4.8 [Range: 2.6-5]
- Responses on the Posttest II:
- Average willingness to participate: 4.5 [Range: 2.6-5]

By the end of the Conference, 5 participants who originally reported a moderate willingness to participate changed their likelihood of participation in all forums to a score of "5," indicating that they were very willing to participate.

Gregg Gonsalves of Yale University's Global Health Justice Partnership and Diana Levine, patient advocate

Participants' Evaluation of Workshop

The evaluation questionnaire (see Appendix C) was handed out at the end of the *Patient Advocacy Workshop* on June 12. The evaluation was filled out anonymously, with no space provided for the participants' names.

Twenty-three of the 30 participants completed their evaluations at the close of the Workshop.

Summary of Participants' Evaluation of Workshop

- All workshops and speakers were very highly rated
- Participants felt it was important to learn the ins and outs of the FDA and felt this goal was achieved
- Participants benefited from and appreciated the high level of interaction and lively discussion
- Enjoyed having "real world" examples from patient advocates such as Gregg Gonsalves and Kim Witczak, and watching videos of patients testifying at FDA public meetings
- 96% of participants reported they would like to continue to be involved with our Patient Advocacy efforts

Panel Evaluations (rated 1-5, with 1 being not at all helpful and 5 being very interesting or helpful)

1. **"Why is FDA important to patients?"** was facilitated by NCHR's Brandel France de Bravo. Participants liked having a basic overview of the FDA rules and regulations to start the day. They stated that Brandel's explanation was helpful in providing a context to understand the rest of the presentations.

   Ratings:
   - Average: 4.7
   - 5's: 74%
   - 4's: 26%

2. **"How does the FDA make decisions to approve, rescind, or recall a medical product?"** was a presentation and Q & A by NCHR's Diana Zuckerman.
   This was the highest rated presentation of the day, with an average rating of 4.9. Participants appreciated how knowledgeable Diana was about the material and how well she fielded questions and inspired a dialogue. Participants reported it was important for them to understand these crucial details of the FDA process. Several commented this session had so much good information that it could have been the whole day.

   Ratings:
   - Average: 4.9
   - 5's: 91%
   - 4's: 8%

3. **"Is FDA too fast or too slow?"** was facilitated by Gregg Gonsalves, co-director of the Yale Global Health Justice Partnership and AIDS patient activist.
   Participants found Gregg's presentation "fascinating," "powerful," and "stimulating." They liked learning about the FDA approval process through the "real world" example of the AIDS activists. It encouraged them to form coalitions with each other after seeing the power of another grassroots movement that ended up being so successful. The presentation gave many participants hope for achieving change for their respective advocacy groups.

   Ratings:
   - Average: 4.8
   - 5's: 83%
   - 4's: 9%
   - 3's: 4%

4. **"Opportunities for patients and patient advocates to provide FDA with the patient perspective"** was facilitated by NCHR's Paul Brown and Maura Duffy.
   Participants appreciated the opportunity to learn how to navigate the FDA and get a more thorough understanding of what to do when speaking at FDA public Advisory Committee meetings. They described the information as "helpful" and "useful."

   Ratings:
   - Average: 4.7
   - 5's: 70%
   - 4's: 30%

5. "**How can patients and patient advocates get the information and preparation they need to become engaged in FDA Decision Making?**" was facilitated by Kim Witczak, Director, WoodyMatters patient advocacy organization, and Sally Greenberg, Executive Director, National Consumers League.
   Participants liked the "passion" and "deep experience" of both women, who showed different perspectives about how patients can have their voices heard by the FDA on a range of issues. They stated the speakers were knowledgeable about their respective fields and liked the humor they displayed in their presentations.

   Ratings:
   - Average: 4.7
   - 5's: 74%
   - 4's: 17%
   - 3's: 4%

6. "**Playing the part**" facilitated by NCHR's Brandel France de Bravo, Anna Mazzucco, and Paul Brown.
   Several participants commented that it was helpful to watch videos seeing patients in action at FDA Advisory Committee meetings, and that it helped them realize what it would be like.

Ratings:

- Average: 4.4
- 5's: 61%
- 4's: 9%
- 3's: 17%
- 2's: 4%

General Questions

7. **Overall, how would you rate the speakers/moderators?**
   Ratings:
   - Average: 4.9
   - 5's: 87%
   - 4's: 13%

8. **Overall, how would you rate the Workshop?**
   Ratings:
   - Average: 4.9
   - 5's: 87%
   - 4's: 13%

9. **Would you like to continue to be involved in FDA Issues as a patient or caregiver advocate?**
   Ratings:
   - 96% responded YES
   - The one person who responded NO commented that their role at a patient advocacy organization "is not that of a patient advocate." All of the patients or patient advocates at the Workshop are committed to further collaboration with each other and the National Center for Health Research.

Our Overall Performance based on Participants' Anonymous Evaluations

What we did well:

- Gave details about the FDA ; clearly explained the agency and its approval process for new drugs and devices
- Answered questions with specific and appropriate detail; overall the instruction was very good
- Facilitated engaging discussions and Q&A sessions with the experts
- Encouraged collaboration between advocacy groups – many participants expressed interest in continued support from NCHR

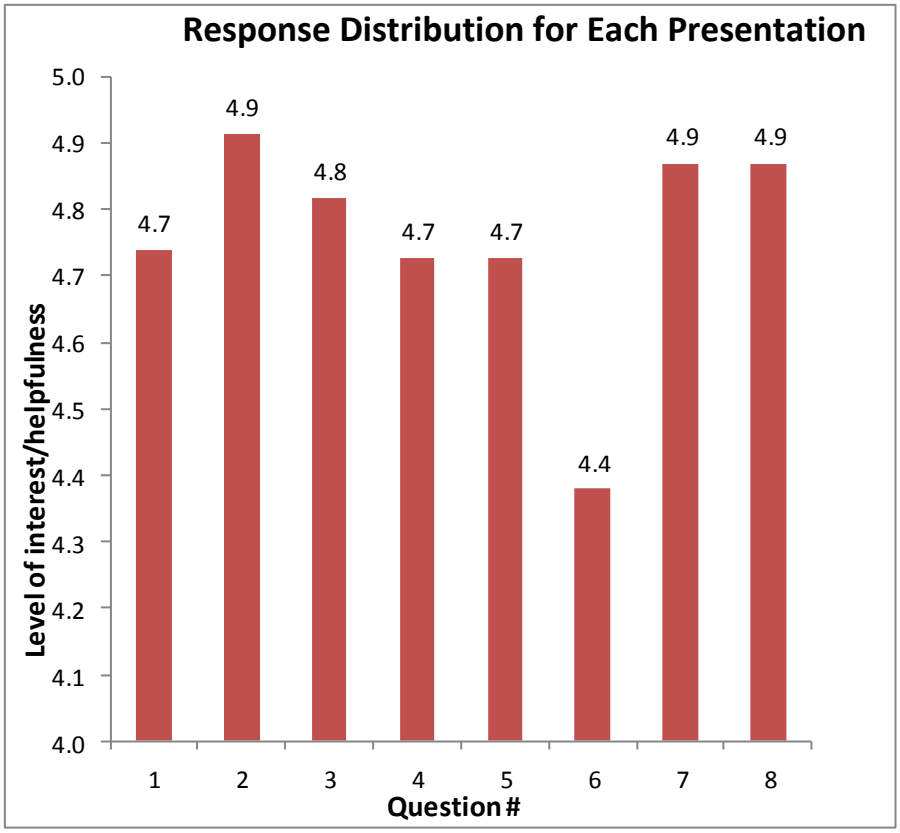
What we can improve next time:

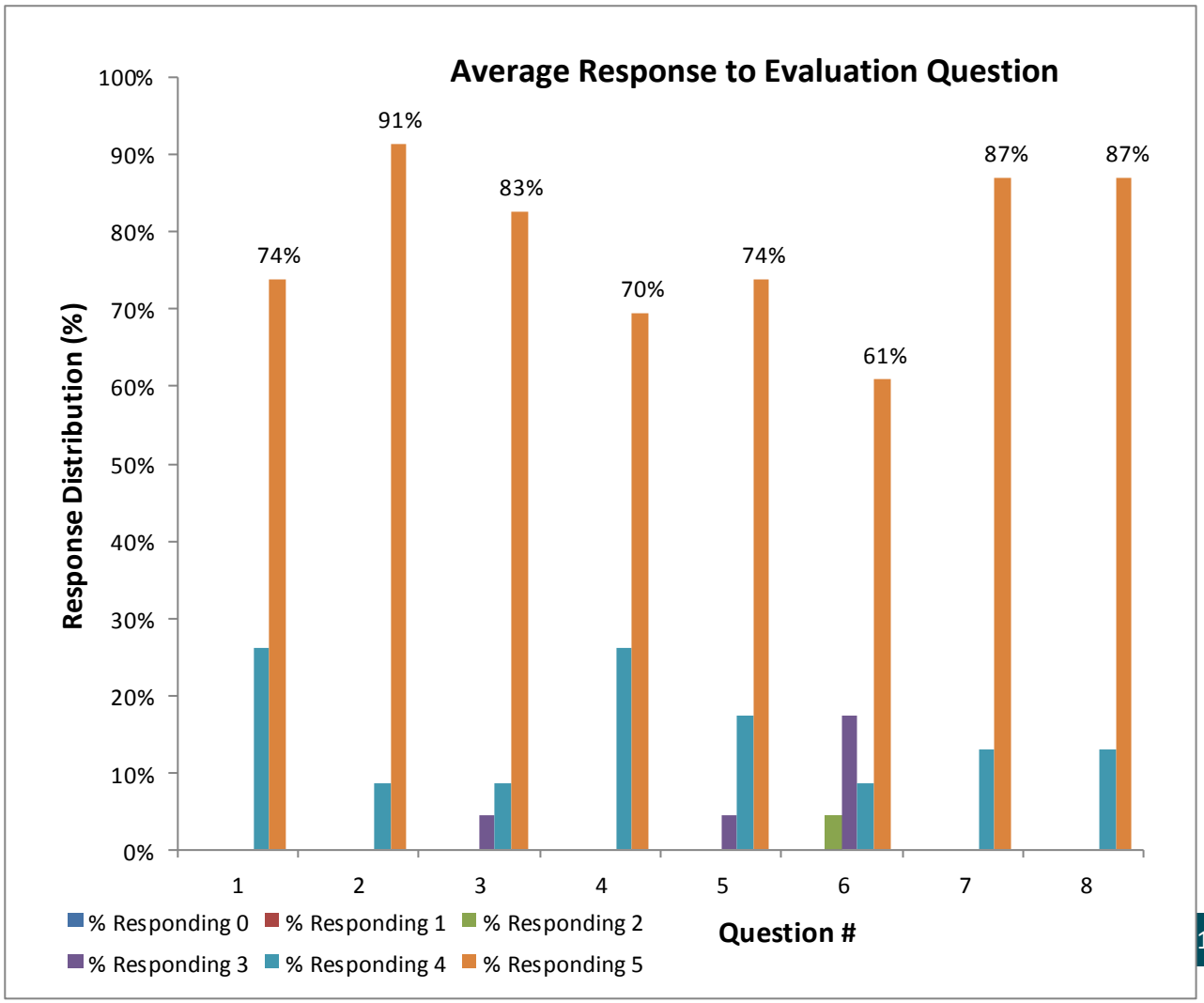
- Allow more time for presentations when participants were actively involved in the discussion and have numerous questions
- Use more video of real FDA testimonies, particularly examples of "good" patient advocates
- More food & drinks
- Bigger space

As can be seen on the graphs on the next page, across all the presentations, 78% of respondents rated the speakers, moderators, and Workshop as a 5 (very helpful/interesting), and 94% of all answers were 4's and 5's.

Question Key:

1. Why is FDA important to patients?
2. How does FDA make decisions to approve, rescind, recall?
3. Is the FDA too fast or too slow?
4. Opportunities to provide FDA with patient perspective
5. How can patients & patient advocates get info and prep to be involved in FDA decision-making?
6. Playing the part
7. How would you rate the speaker/moderators?
8. How would you rate the Workshop?





Trainers' Observations

The 30 participants were actively engaged throughout the Workshop, asking questions throughout and clearly wanting to share their views. The entire Workshop was energizing for the speakers and participants, for the experienced advocates as well as the new ones, and for those who had previously been engaged with FDA issues and those who never had. The only downside was that several of the staff and advocates that provided the training were unable to stick to the schedule because participants were hungry for additional information and in no hurry to get to the next topic.

Follow-Up from June 16-September 15, 2014

We contacted all participants via email in the weeks after the Conference to determine whether they would like to be part of an email listserv, sharing information and being kept informed of opportunities to engage on PCOR issues with the FDA. All but one said yes. In addition, several participants who had not previously been part of the Patient, Consumer, and Public Health Coalition have become members, and have participated in written public comments to the FDA, signing on those comments on behalf of patient organizations that they were affiliated with. As of September 15, eight of the organizations represented at the Workshop signed on to one or more written comments submitted to the FDA.

Two participants reached out to NCHR staff to ask for help in educating FDA staff about family members' experiences with an unsafe medical product. In both cases, NCHR helped them write to government officials and participated with them in conference calls.

The AIDS activist whom we met through the Workshop and who also attended the Conference has followed up with NCHR staff, arranging a meeting at the NCHR office with several other organizations representing HIV/AIDS patients, and also arranging a conference call with health policy staff at Yale.

The participants noted above, as well as numerous other Workshop participants, have also reached out to NCHR staff to express the need for additional training for themselves, as well as recommending that we offer the *Patient Advocacy Workshop* again to other patient and caregiver advocates. They offered their enthusiastic support and participation in future efforts.

Overall Conclusions

The *Patient Advocacy Workshop* provided an opportunity for advocates with a wide range of knowledge and perspectives to learn about the FDA approval process and ways in which they can make their voices heard by the FDA by engaging in opportunities for feedback. As demonstrated by the statistically significant increased knowledge about research standards for drug and device approval and participants' increased willingness to participate in future advocacy, the Workshop achieved all three objectives. Participants felt engaged and empowered, with 96% reporting that they would like to be involved in FDA issues as a patient or caregiver advocate. In the three months since the Workshop and Conference, the active engagement of most of the participants has persuaded us that additional workshops and patient engagement efforts on PCOR FDA issues would be welcomed in the patient advocacy community.

Appendix A: List of Workshop Participants

	Participant Name	Organization/Issue	Location
1	Joyce Bichler	Breast Cancer Action	San Francisco, CA
2	Gloria Black	National Consumer Voice for Quality Long-Term Care	Portland, Oregon
3	Brenda Bryant	Annie Appleseed (cancer survivors)	San Antonio, TX
4	Doris Champ	Annie Appleseed (cancer survivors)	North Little Rock, Arkansas
5	Wendy Dolin	Medication-Induced Suicide Education Foundation (MISSD)	Chicago, IL
6	Kathy Fee	Patient Advocate	Virginia Beach, VA
7	Angie Firmalino	Women Advocating for Reproductive Safety	Tannersville, NY
8	John Fratti	Patient Advocate	Hersey, PA
9	Mary Gabourel	Sisters Network Prince Georges County	Prince George, MD
10	Michelle Garcia	Women Advocating for Reproductive Safety	North Miami, Florida
11	Marge Ginsburg	Center for Healthcare Decisions	Sacramento, California
12	Gregg Gonsalves	Co-Director of Yale University's Global Health Justice Partnership	New Haven, CT
13	Sally Greenburg	National Consumers League	Washington, D.C.
14	Marian Hollingsworth	California Safe Patient Network	San Diego, CA
15	Kim Hudak	Women Advocating for Reproductive Safety	Cleveland, OH
16	John James	Safe Patient Project	Austin, TX
17	Sanford Jeames	American Cancer Society, US TOO International, Cancer Information Service	Austin, TX
18	Katie Kroner	Pulmonary Hypertension Association	Washington, D.C.

19	Karen Langhart	Patient/Family Advocate	Phoenix, AZ
20	Diana Levine	Patient Advocate	Marshfield, VT
21	Josephine Long	Sisters Network Prince Georges County	Prince Georges County, MD
22	Caitlin Morris	Families USA	Washington, DC
23	Daniela Nuñez	Consumers Union	Austin, TX
24	Sherrie Palm	Association for Pelvic Organ Prolapse Support (APOPS)	Milwaukee, WI
25	Amanda Rusmisell	Women Advocating for Reproductive Safety	Charlotte, NC
26	Liz Schulte	Northern Ohio Breast Cancer Coalition	Cleveland, OH
27	Elisabeth Vink	International Foundation for Functional Gastrointestinal Disorders	Milwaukee, WI
28	Desiree Walker	Patient safety advocate	New York, NY
29	Kim Witczak	WoodyMatters	Minneapolis, Minnesota
30	Maryann Wooden	Patient/Family Advocate	San Francisco, CA

Appendix B: Agendas for June 12 and June 13

Patient Advocacy Training Workshop (June 12, 2014)

11:00: Introductions: Each participant will briefly introduce themselves by name, organization (if relevant) and one sentence about the health issue(s) they want to work with the FDA on.

11:15: Training and Discussion Topic #1: Why is FDA important to patients? Brandel France de Bravo, National Center for Health Research, and Diana Levine and Michelle Garcia.

12:00: Lunch

12:30: Training and Discussion Topic #2: How does the FDA make decisions to approve, rescind, or recall a medical product?" Diana Zuckerman, National Center for Health Research.

1:30: Discussion Topic #3: Is FDA too fast or too slow? Presentation by Gregg Gonsalves, AIDS activist and co-director of the Yale Global Health Justice Partnership. Discussion led by Dr. Laurén Doamekpor, National Center for Health Research.

2:30: Training and Discussion Topic #4: What are the opportunities for patients and patient advocates to provide FDA with the patient perspective? Paul Brown and Maura Duffy, National Center for Health Research.

3:15: Break

4:00: Training and Discussion Topic #5: How can patients and patient advocates get the information and preparation they need to become engaged in FDA decision-making?" discussion led by Kim Witczak from Woody Matters and Sally Greenberg, National Consumers League.

5:00: Playing the Part—Videos and role-playing exercise of patients speaking at FDA events, led by by Brandel France de Bravo, Anna Mazzucco and Paul Brown.

Conference Agenda (June 13, 2014): "Evidence for New Medical Products: Implications for Patients and Health Policy"

8:00 am **Registration**
8:30 am **Welcome,** Remarks: Mark S. Frankel, American Association for the Advancement of Science
8:40 am **Keynote Addresses,** Introductions: Diana Zuckerman, National Center for Health Research
8:45 am **Remark**s: Congresswoman Rosa L. DeLauro (D-CT)
9:05 am **Remarks**: Margaret R. Hamburg (via live video), Food & Drug Administration

9:15 am **Panel I. Evidence-Based Policy: Opportunities and Challenges**
Moderator: Susan F. Wood, George Washington University
Speakers: Aaron S. Kesselheim, Brigham and Women's Hospital/Harvard Medical School
Robert Yarchoan, National Cancer Institute
Gregory D. Curfman, *New England Journal of Medicine*
Rita F. Redberg, *JAMA Internal Medicine*

10:45 am **Panel II. Speed vs. Safety: Implications for Public Health of Changing Standards for Getting Products to Market**
Moderator: Kim Witczak, Patient Safety Advocate
Speakers: Bernard Lo, Greenwall Foundation
Lisa M. Schwartz & Steven Woloshin, Dartmouth Medical School
Michael Rosenblatt, Merck 12:00 pm **Lunch**

1:00 pm **Panel III. Biomarkers, Surrogate Endpoints and Other Shortcuts that May or May Not Predict Health Outcomes**
Moderator: Thomas J. Moore, Institute for Safe Medication Practices
Speakers: Jerry Avorn, Brigham and Women's Hospital/Harvard Medical School
Joseph S. Ross, Yale School of Medicine
Diana Zuckerman, National Center for Health Research
Ann de Velasco, Women Heart of Miami

2:30 pm **Panel IV. Post-Market Surveillance and Comparative Effectiveness Research**
Moderator: Tianjing Li, Johns Hopkins University
Speakers: Robert Ball, Food & Drug Administration
Sebastian Schneeweiss, Brigham and Women's Hospital/Harvard Medical School
Patrick Ryan, Janssen Research and Development

3:45 pm **Break**

4:00 pm **Panel V. What Kinds of Data are Needed/Used? A Policy Roundtable**
Moderator: Curt D. Furberg, Wake Forest University
Speakers: C. Bernie Good, VA Pittsburgh Healthcare System
Louis Jacques, Georgetown University School of Medicine
Peter Lurie, Food & Drug Administration
Joseph V. Selby, Patient-Centered Outcomes Research Institute
Anne E. Trontell, Agency for Healthcare Research & Quality

5:20 pm **Wrap-Up and Adjourn**
Aaron S. Kesselheim, Brigham and Women's Hospital/Harvard Medical School

Appendix C: Pretest/Posttest Questionnaire

Q #1: FDA drug approval

When FDA approves a drug as safe and effective, what does that mean?

a) The drug does not seem to have any serious side effects.
b) **The drug has benefits that outweigh the risks when used in the intended manner.**
c) The drug can be taken by any adult and is considered safe.
d) Based on research on humans, the drug is proven effective for most patients.
e) The drug has been shown to work better than other available treatments for the same illness or disease.

Q #2: Drug vs. device standards

How are FDA standards for allowing medical devices to be sold different than for drugs?

a) **All drugs must be tested on people in clinical trials but most medical devices do not have to be tested on people.**
b) Standards for implanted medical devices are generally more stringent than for drugs.
c) Standards for medical devices are the same as those for drugs.
d) The standards for FDA approval of drugs vary depending on how risky the drug is, whereas the standards for medical devices are the same for all devices.

Q #3: Double-blind clinical trials

What is a double blind clinical trial?

a) Patients in the study do not know the purpose of the study.
b) The researchers do not know which patients are receiving the drug or medical device being tested but the patients know.
c) **Neither researchers nor patients know which patients are receiving the medical product being tested and which are getting a placebo (sugar pill or "pretend treatment).**
d) The patients are given a drug but not told what the expected risks or benefits are.

Q #4: Randomized, controlled trial

What is a randomized controlled clinical trial?

a) A study of whether patients' health improves after taking a new drug whose manufacture is controlled by the FDA.
b) A study where patients randomly choose whether they receive the medical product being tested or a placebo (no treatment).
c) **A study comparing the health of patients who were randomly assigned to receive a new medical product or not.**
d) The "gold standard" for research on human health and required by FDA for drugs and devices.

Q #5: Biomarkers or surrogate endpoints

What is a biomarker or surrogate endpoint?

a) A research finding that lets researchers know it is time to stop the study.
b) Another name for a drug that is being tested.
c) **An outcome that is not an important measure of health but is believed to predict an important measure of health.**
d) A genetic test given during a clinical trial.

Q #6: Biomarker advantages
For patients, what is the advantage of studies based on surrogate endpoints compared to patient health?

a) **The clinical trial may be completed more quickly.**
b) The drug will be safer and more effective.
c) The drug will be covered by most insurance companies.
d) The studies will be larger.

Q #7: Pre- and post-market studies
Below is a list of statements comparing pre-market and post-market studies. Please review the statements and check all that are true. More than one answer may be selected.

a) Pre-market studies are paid for by the manufacturer but post-market studies are paid for by the FDA.
b) **Companies have less incentive to complete post-market studies.**
c) Post-market studies always include a more diverse group of patients from the "real world" not just those carefully selected for study.
d) Post-market studies are usually better designed and more carefully conducted.
e) **Post-market studies are often longer than pre-market studies.**

Q #8: Finding significantly better results
A researcher says that a new medical product is significantly better than a placebo. What can you conclude from this statement? More than one answer may be selected.

a) The new drug is at least 15% better than the placebo.
b) **The drug is probably better than no treatment at all.**
c) The drug is better than older drugs already for sale.
d) The drug probably helps patients live longer.
e) **There is at least a 95% likelihood that the drug is better than placebo and less than a 5% likelihood that the better results occurred "by chance."**

Q #9: Open Ended Reponses

The FDA requires companies to mention potential risks when companies advertise their drugs on TV or magazines/ Are those warnings helpful to patients? How would you improve them?

Q #10: Open Ended Reponses
Do you think the FDA approval process is too fast or too slow? If you have an opinion one way or the other, explain why you believe that to be true.

Q #11: Participation Questions
Below are some examples of opportunities for informing the FDA about the perspective of patients and patient advocates like you. Please rate each of them on a scale of 1 to 5 by circling the number that applies to you, with 5 meaning you are very likely to consider doing this and 1 meaning you would never consider.

a. **Speak for a few minutes during the public comment period of an FDA meeting in the DC area about what you as a patient think is an important benefit or risk for a specific drug or medical device.**
b. **Speak for a few minutes during the public comment period of an FDA meeting in a city near you about what you as a patient think is an important benefit or risk for a specific drug or medical device.**
c. **Write a comment giving your perspective in response to an FDA "request for public comments" on a topic of importance to you as a patient.**
d. **Add your name as one of the people signing a letter written by other patients or nonprofit organization or coalition in response to an FDA "request for public comments" on a topic of importance to you as a patient.**
e. **Speak at a non-FDA event of FDA issues (such as a Congressional hearing, Congressional briefing, or a meeting with FDA officials) on a topic of importance to you as a patient.**

Appendix D: Evaluation of Patient Workshop

Please rate on a scale of 1 to 5 (**with 1 being not at all helpful or interesting, and 5 being very helpful or very interesting**) today's sessions. Circle the number that corresponds with your opinion of the sessions (including the discussion and presentations) and provide comments about what you found most or least helpful, and what you would have liked to learn more about.

1. First session after introductions: "Why is FDA important to patients?" moderated by Brandel France de Bravo from National Center for Health Research.

   1 2 3 4 5 (**1 not helpful or interesting and 5 very helpful/ interesting)**

   Comments?

2. After lunch: "How does the FDA make decisions to approve, rescind, or recall a medical product?" Diana Zuckerman from the National Center for Health Research and following discussion.

   1 2 3 4 5 (**1 not helpful or interesting and 5 very helpful/ interesting)**

   Comments?

3. "Is FDA too fast or too slow?" presentation by Gregg Gonsalves, co-director of the Yale Global Health Justice Partnership, and discussion facilitated by Laurén Doamekpor.

   1 2 3 4 5 (**1 not helpful or interesting and 5 very helpful/ interesting)**

   Comments?

4. "Opportunities for patients and patient advocates to provide FDA with the patient perspective," Paul Brown and Maura Duffy from the National Center for Health Research, and the following discussion.

   1 2 3 4 5 (**1 not helpful or interesting and 5 very helpful/ interesting)**

Comments?

5. “How can patients and patient advocates get the information and preparation they need to become engaged in FDA decision-making?” discussion lead by Kim Witczak from WoodyMatters and Sally Greenberg from National Consumers League.

   1 2 3 4 5 (**1 not helpful or interesting and 5 very helpful/ interesting)**

   Comments?

6. “Playing the part,” role-playing exercise with Brandel France de Bravo, Anna Mazzucco and Paul Brown.

   1 2 3 4 5 (**1 not helpful or interesting and 5 very helpful/ interesting)**

   Comments?

7. Overall, how would you rate the speakers/moderators? 1 2 3 4 5

8. Overall, how would you rate the Workshop? 1 2 3 4 5

9. Would you like to continue to be involved in FDA issues as a patient or caregiver advocate?

   YES NO

10. Do you have any other comments or suggestions you would like to make?