Building a Patient Network to Express Patients' Views on Research Criteria for New Medical Products

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Background

The National Center for Health Research (NCHR) recruited patient partners to attend an Introductory Training Workshop on November 13-14, 2015 in Washington, D.C. This two-day workshop was held for 31 patients, caregivers, and their advocates to learn more about the types of research that can determine the safety and effectiveness of drugs and medical devices. The goal was to increase their understanding of research issues so that they could be better informed participants at public meetings, more likely to express their perspective in written or oral public comments, and more likely to participate in other opportunities that would strengthen patient-centered perspectives that influence medical research conducted or used by federal agencies, university researchers, and nonprofit organizations.

This workshop is the second of four introductory workshops designed to build a network of patient partners that can work together in many different ways to improve PCOR. The first workshop was held in June 2014, supported in part by a previous PCORI engagement award. An advanced workshop will be held in June 2016 and will offer additional training to network members that participated in one of the first two workshops.

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

Patient Training Workshop Objectives

We are training patient partners who will provide leadership for a Patient Network of informed members. Members will have the knowledge and skills to provide their perspectives to federal agencies such as FDA, NIH, and CDC, as well as university researchers and nonprofit groups. The goal is to help improve the design of clinical trials and other research to ensure inclusion of outcomes that matter most to patients.

The objectives of the Workshop were to:

1) Train patient partners in understanding the types of clinical trials and other research to determine the effects of prevention and treatment, the importance of appropriate endpoints and subgroup analysis, patient-centered outcomes and comparative effectiveness research;
2) Train patient partners in how the FDA works and the regulatory approval process for medical products;
3) Train patients/caregivers to share their views at public meetings via oral and written comments, and to learn about opportunities to serve on advisory committees;
4) Train patient partners to be knowledgeable assets to researchers and agencies that seek patient engagement that will affect the prevention and treatment strategies.

The agenda for the *Patient Training Workshop* is included in **Appendix A**.

The *Patient Training Workshop* was led by five members of the NCHR staff, four patient and consumer advocates from other nonprofit organizations, two FDA staff, one PCORI staff member, one university faculty member, and five patient partners who discussed their personal experiences as presenters in panel discussions. Throughout the workshop, patient partners enthusiastically asked many questions and shared their opinions based on their personal experiences. When we broke into small groups, they also role played providing their views on treatment research priorities.

To determine whether or not we achieved our objectives, we administered a pretest/posttest questionnaire (see **Appendix B**) to assess changes in participants' knowledge, attitudes, and beliefs. In addition, we asked participants to anonymously fill out an evaluation questionnaire of the workshop in general and each presentation (see **Appendix C)**.

Desirée Walker, a breast cancer survivor and patient advocate, kicked things off with a motivational talk on how she became a patient advocate.

What did Patients Learn?

Identical questionnaires were distributed before the start of the workshop on November 13th and before the final (role-playing) activity on November 14th to determine participants' knowledge and attitudes before the Workshop and how those changed after the Workshop. All 31 men and women who attended the Workshop as participants completed the pretest and posttest.

The questionnaire consisted of 12 questions: 10 multiple choice questions that tested knowledge and 2 open-ended questions asking their opinions. Two of the multiple choice questions had more than one correct answer, so each of the five potential answers were individually scored as correct or incorrect. Total possible scores ranged from 0 to 18. Participants included their first name on the questionnaire so that we could compare pretest and posttest scores. Participants averaged 11.7 on the pretest and 14.5 on the posttest, which indicates a highly significant increase in knowledge (paired t-test: 6.97, $p<.0001$).

The scores are presented below in Table 1. The questions and answers are listed in Appendix B.

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

t-Test: Paired Two Sample for Means (N=31)		
	Pretest	*Posttest*
Mean	11.7	14.5
Standard Deviation	2.7	1.6
t Statistic	6.97	
P-Value (two-tailed)	Less than 0.0001	
Mean Difference: (Posttest I)-(Pretest)	-2.71	
Standard Error of Difference	0.389	
Upper 95% CI of Difference	-3.50	
Lower 95% CI of Difference	-1.92	

On 15 of the 18 questions testing knowledge, the number of participants responding correctly increased, sometimes dramatically. For half the 18 questions, 90% or more of the participants had the correct answer in the post-test, compared to only 1 question with that many correct responses in the pretest. The percentages are presented below in Table 2.

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

Question	Pretest (n=31)	Posttest (n=31)
Q#1 Criteria for FDA drug approval	77% (24/31)	58% (18/31)
Q#2 Drug vs. device standards	45% (14/31)	84% (26/31)
Q#3 Define p-value	61% (19/31)	97% (30/31)
Q#4 Define double-blind clinical trials	94% (29/31)	100% (31/31)
Q#5 Define randomized, controlled trial	48% (15/31)	48% (15/31)
Q#6 Define biomarkers or surrogate endpoints	77% (24/31)	90% (28/31)
Q#7 Advantages of studying biomarkers	45% (14/31)	81% (25/31)
Q#8 What is a subgroup	52% (16/31)	94% (29/31)
Q#9 Differences between pre- and post-market studies	A. 74%	71%
	B. 74%	97%
	C. 35%	58%
	D. 84%	94%
	E. 55%	61%
Q#10 Define statistical significance	A. 81%	90%
	B. 58%	61%
	C. 87%	97%
	D. 81%	97%
	E. 45%	68%

Comparisons in shaded boxes were statistically significant.

We conducted chi-square comparisons on individual questions, as a way to better understand what was learned. **The answers to six of the questions were significantly more likely to be correct after the Workshop, four of them at the p <.01 level or higher (Q#2, Q#3, Q#7, and Q#8) and two more at the p<.05 level (Q#9 option B and Q# 10 option D).** Answers to Q#4, Q#6, and most answers to Q#9, and Q#10 were more likely to be correct after the workshop, but the increases were not statistically significant. None of the decreases in correct answers were statistically significant. Overall, the results

show the greatest improvement in participants' understanding of clinical trial design, use of biomarkers and surrogate endpoints, the differences between premarket and post-market studies, and the meaning of statistical significance.

In contrast, the answer to Q#1, asking for the criteria for FDA approval, was non-significantly less likely to be correct after the workshop compared to before. In the posttest, a common incorrect response was "Based on research on humans, the drug is proven effective for most patients." In the next workshop, we will need to better clarify that FDA approval does not mean that a drug is effective for most patients, but instead that the benefits outweigh the risks for most patients. Many participants also remained confused about the nuances regarding different types of controlled clinical trials (Q#5). Although the percentage of correct responses were identical for Q#5 before and after the Workshop, some participants responded correctly in the pretest but incorrectly in the post-test, as well as vice versa. Their responses showed confusion between randomized controlled clinical trials and randomized double blind clinical trials.

Lessons Learned

- The greatest learning by the patient partners was on the issues that approximately 40-60% did not know prior to the Workshop: the difference between FDA standards for medical devices and prescription drugs **(Q#2**), the definition of p-value **(Q#3)**, understanding the advantages of biomarkers **(Q#7),** and what a subgroup analysis is **(Q#8).**
- More than 90% of the participants correctly defined a double blind clinical trial (**Q#4**) prior to the Workshop. However, participants became confused when we tried to explain to them about different types of controlled clinical trials **(Q#5)**. We will improve our training regarding the nuances of different types of controlled clinical trials.
- We will also improve our training on the criteria used for FDA approval **(Q#1)**, to avoid any confusion.
- There was also substantial learning regarding the differences between premarket and post-market studies **(Q#9)** and the meaning of statistical significance **(Q#10)**. 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 patient population being studied. We significantly improved knowledge about how new medical products might appear to benefit patients by being "significantly better than placebo" without actually helping patients live longer.

Impact on Participants' Attitudes and Beliefs (Q#11-12)

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

As shown below, Question # 11 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#11: 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?

Most participants said that the warnings were not helpful; many of them reported they were too wordy and sounded like a laundry list of side effects. Some participants had strong feelings on this topic, and said that all direct-to-consumer advertising should be banned. In contrast, a few participants said the ads were helpful because they enabled patients to be better informed about potential risks of drugs and devices.

Since this topic was not included in the Workshop, it was interesting to see how attitudes changed as a result of other knowledge gained during the Workshop. Most participants had the same views before and after the workshop. However, after the workshop, many participants said that the warnings should include efficacy of the drug or device, or what subgroups the drug had been tested on. Participants also reported that the warnings should include the percentage of common adverse events, rather than just a long list of possible side effects. This shows application of the knowledge that was presented at the workshop.

Here are a few examples of responses:

> Participant A, Pretest: *They are not particularly helpful because they have become "noise" that viewers/readers tune out. Potential risks should be standardized somehow and presented as infographic when possible. Short/clear. "Red light/green light", not small and like an auctioneer.*
>
> Participant A, Posttest: *They are too general to be truly helpful. Would need to be broken out (by **subgroup analysis**) to be meaningful. Also standardize language.*
>
> Participant B, Pretest: *Yes, and no. It is helpful to know all the side effects but it can also be frightening.*
>
> Participant B, Posttest: *Yes they are helpful in making an informed decision to use the medication or not. However it does not give you the percentages nor does it state if it is helpful for certain subgroups.*

Q#11 continued: How would you improve them?

Overall, many participants said that the warnings could be improved by highlighting the most common side effects, and stating those first. They reported that giving more specifics about the probability of side effects would also improve these warnings. Many participants stated that individuals do not pay enough attention to these warnings. They also stated that images shown of healthy happy individuals in beautiful settings can be distracting while discussing serious side effects.

- 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
    - Warnings should be given by doctors or the FDA, rather than pharmaceutical companies
- 8 respondents said that direct-to-consumer advertising should be banned entirely instead of attempting to improve them.

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 – which is a simpler way of saying that the standards are too high or too low. Numerous patient advocacy organizations have criticized the FDA in recent years for taking too long to approve new drugs and devices. The 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 #12 asked participants for their views on this important issue.

Q#12: 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 consensus on this issue. Many participants in the pretest said that they did not know enough about the issue to comment. When participants completed the posttest the next day, many of these same participants 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 question 12 showing an increase in knowledge of the subject:

Participant C, Pretest: *Not sure.*

Participant C, Posttest: *Both. Some drugs/devices need to be reviewed more thoroughly. Classification system may need to be reviewed for devices to help with decision making. Some drugs, the type of clinical trial must be better structured.*

Participant D, Pretest: *I am not familiar with the approval process-however it appears to be too slow as I have heard from friends who were candidates for drugs but the FDA had not approved them*

Participant D, Posttest: *The process is too fast as it appears to be focused on getting products to market. However, some processes appear to be too slow, I would rather see a quality process that truly examines how safe a drug or device is-Patient advocacy is a major component.*

Participant E, Pretest: *I don't know what it is now, but I am concerned about the push for faster approvals*

Participant E, Posttest: *I think the process is too fast and not enough safety/health info is analyzed to make a decision for all people the drug is intended to help.*

Willingness to Participate in Future PCOR Opportunities Pertaining to FDA Issues (Q#13)

The posttest survey asked participants to rank their willingness to participate in future opportunities to share their patient perspectives on a national level, 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

All participants expressed a desire to participate in one or more of these scenarios, with almost all participants responding with a 4 or 5 on multiple parts of the question. In the two months since the workshop, several participants have already signed up for FDA meetings, and signed on to letters or public comments that NCHR staff drafted in response to FDA's requests for comments.

Participants' Evaluation of Workshop

The Workshop evaluation pertaining to Friday Nov 13 was handed out on Saturday morning, November 14, before starting for the day. The evaluation for the activities of Nov 14 was handed out at the conclusion of the workshop, and included questions about the workshop as a whole and likelihood of participating in future events. The evaluation was filled out anonymously.

All 31 participants completed the evaluation for the first day of the workshop, and twenty-six of the 31 participants filled out the evaluation for the second day at the close of the workshop.

The evaluation questionnaires are in Appendix C.

Summary of Participants' Evaluation of Workshop

- The workshop and speakers as a whole were very highly rated
- Participants reported it was important to learn the ins and outs of the FDA and said this goal was achieved
- Participants benefited from and appreciated the high level of interaction and lively discussion
- Participants reported that they enjoyed having "real world" examples from patient advocates such as Desirée Walker and Tim Horn, watching videos of patients testifying at FDA public meetings, and the role playing exercise to practice the skills they learned

Nov. 13 Panel Evaluations (rated 1-5, with 1 being "not at all helpful" and 5 being "very interesting or helpful")

1. **"How I went from advocating for myself to advocating for others, to advocating on a national level"** facilitated by Desirée Walker, a breast cancer survivor who served as a consultant to the Workshop.

   Participants found Desirée to be an "inspiration," and a "great speaker." They thought the content of her talk was motivating, and that it was great to hear from someone who transitioned from being a patient to a strong advocate.

   Ratings: Average: 4.8, with 84% rating it 5

2. **"How does the FDA make decisions to approve, rescind, or recall a medical product?"** Presentation and Q & A by NCHR's Diana Zuckerman.

   Participants found Diana's talk very "interesting" and "informative." Many participants were not aware of the process for drug and device approvals and commented that the new information was "shocking!" and "eye-opening." Others mentioned how interesting she made the material, and that they were riveted the whole time. Several commented that this session had so much potent information that they wished they could have heard more.

   Ratings: Average: 4.8, with 77% rating it 5

3. **"Consumers Union and the Safe Patient Project"** presented by Victoria Burack, Health Policy Analyst at Consumers Union and two Workshop participants from Safe Patient Project.

   Participants were grateful for the opportunity to learn more about these organizations and said that they were given, "great resources for opportunities to advocate."

   Ratings: Average: 4.3, with 52% rating it 5

4. **"Engaging patients and stakeholders in research: the PCORI perspective"** facilitated by PCORI's Michelle Johnston-Fleece.

   Many participants were not familiar with PCORI and found it "enlightening" to learn more about what PCORI does. They appreciated the "thorough" and "informative" presentation, and reported it would be a "great resource."

   Ratings: Average: 4.4, with 55% rating it 5

5. "**Patient panel discussion about reaching out to the FDA or researchers**" featuring three workshop participants, with Diana Zuckerman as moderator.

   Participants loved hearing personal stories from fellow survivors. They said it was "powerful," "heartfelt" and "moving."

   Ratings: Average: 4.5, with 65% rating it 5

6. "**How can you become a Patient Representative?**" facilitated by Andrea C. Furia-Helms and Salina Prasad, both of the FDA.

   Some participants appreciated learning about the patient representative program, and hope to find a way to participate. However, other participants didn't feel this program would apply to them and were not sure how to get involved in a meaningful way.

   Ratings: Average: 4.0, with 32% rating it 5

7. **"Insider's View of Patient Advocacy and the FDA"** facilitated by Dr. Susan Wood, professor at GWU and former Director of FDA's Office for Women's Health.

   Participants said Susan was "excellent," "very interesting" and "very informative." They reported she was, "so knowledgeable and a force to really affect patient advocacy." Many wished they could have had more time with her.

   Ratings: Average: 4.6, with 71% rating it 5

Nov. 14th Panel Evaluations (rated 1-5, with 1 being "not at all helpful" and 5 being "very interesting or helpful")

1. "**Research 101 for Patient Advocates"** facilitated by NCHR's Margaret Dayhoff-Brannigan.

   Participants said this talk helped them to appreciate the importance of understanding the basics of research and statistics. They reported it was, "very informative" and "so useful."

   Ratings: Average: 4.5, with 58% rating it 5

2. "**The importance of subgroup analysis"** facilitated by NCHR's Laurén Doamekpor.

   Participants found it "shocking" and hard to understand why subgroup analysis isn't performed more often in clinical trials. Many said it was important to brainstorm effective ways to get this issue on the forefront.

   Ratings: Average: 4.7, with 73% rating it 5

3. "**The importance of patient advocates"** presented by Tim Horn, HIV Project Director from Treatment Action Group, and Dr. Susan Molchan from National Physicians Alliance.

   Participants enjoyed hearing a success story of patient advocacy from Tim Horn, but said it was a bit too specific to his disease and would have liked more general information. Participants were excited to learn about the National Physicians Alliance, and found Susan's talk to be "informative."

   Ratings: Average: 4.5, with 58% rating it 5

4. "**Opportunities for patient engagement at the FDA and NIH"** facilitated by Paul Brown and Dr. Tracy Rupp, both of NCHR.

   Participants said this talk was very "interesting" and "helpful," including providing them with "much needed info to navigate the system and explanations."

   Ratings: Average: 4.6, with 67% rating it 5

5. "**Videos of Patient Advocates and Role Playing exercise"** led by Margaret Dayhoff-Brannigan, Laurén Doamekpor, Paul Brown, and Tracy Rupp from the NCHR. After watching short videos, patient partners broke into small groups to role-play presentations on their own issues.

   This was a very popular session among most participants. They appreciated hearing what staff felt made a good presentation to the FDA. They enjoyed breaking down into small groups, and found it "very helpful" to get a chance to practice and receive feedback on testimony.

   Ratings: Average: 4.7, with 80% rating it 5

General Questions

6. Overall, how would you rate the speakers/moderators? Average: 4.7, with 75% rating them a 5

7. Overall, how would you rate the Workshop? Average: 4.7, with 71% rating it 5

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

92% responded YES (of the 24 that responded)

Only two people responded No. One person said no because they are afraid they "wouldn't speak up enough" and that they are "too easily intimidated," while the other person said they wouldn't like to now "because I don't have a device or medication issue." 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

Examples of comments from participants:

Participant A: *"After this workshop, I feel that I am on my way to being better informed about scientific studies and I have some ideas about which organizations and individuals to contact and work with in our advocacy efforts."*

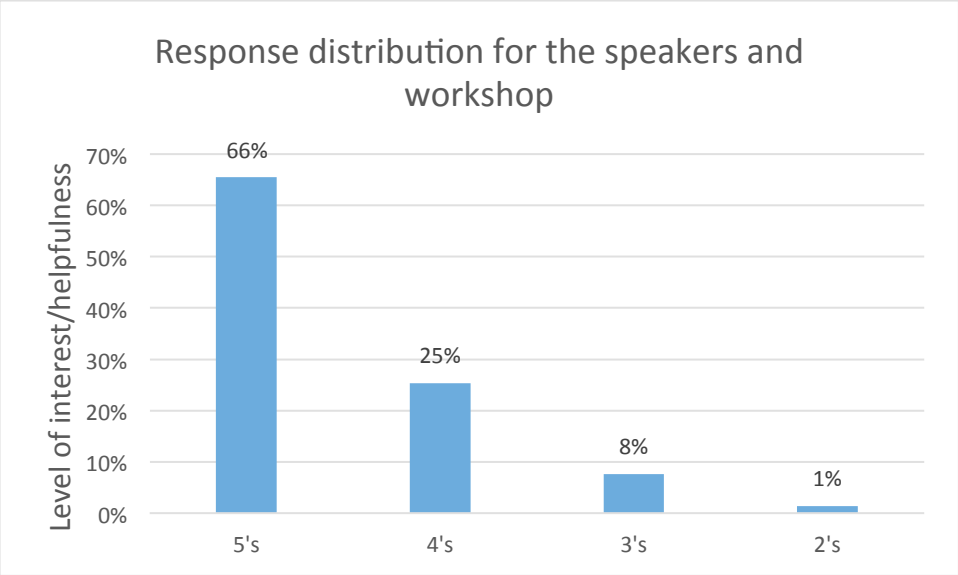
Participant B: *"This was a great workshop: Inspiring for me as a beginning advocate."*

Participant C: *"Thank you for inviting me to participate in this workshop. This was enormously beneficial to the advocacy work I'm involved with, and will help me advise others. 10 out of 10!"*

What we can improve next time:

- Make sure that everyone gets a chance to ask questions (some participants dominated the Q & A, which left less time for others). Improve the microphone usage to prevent it from being distracting and disrupting the flow.
- Encourage participants to ask fewer questions specific to personal issues, and more that the general group would benefit from.
- Provide bios and photos of each of the participants so it would be easier to get to know everyone.
- More in-depth information "about what we can actually do and how to do it."
- Provide more specific examples of where advocacy has made a difference and what else patients can do.

As can be seen on the graph below, across all the presentations, 66% of respondents rated the speakers, moderators, and Workshop as a 5 (very helpful/interesting), and 91% of all answers were 4's and 5's.



Trainers' Observations

The 31 participants were actively engaged throughout the Workshop, asking questions 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 or NIH issues and those who never had. There was a tendency by some participants to ask questions that were specific only to their personal experiences and area of interest, rather than relevant to the whole group. Also, the conversation at times was dominated by a few outspoken members of the group.

Follow-Up from November 14, 2015-February 15, 2016

We contacted all participants via email in the weeks after the Workshop to invite them to join our new Patient Network, starting with a list serv for patient advocates who have participated in our workshops. The list serv makes it easy to share information and help advocates be informed of opportunities to engage on PCOR issues with the FDA or other agencies. All but one of the November 2015 Workshop participants have joined the list serv. We have also invited the participants from our June 2014 participants (prior PCORI Engagement award) to join the Patient Network. We now have a total of 45 patient partners from the two workshops in our network.

We have launched a website for the Patient Network (www.USAPatientNetwork.org), and have created a Facebook page (Facebook.com/USAPatientNetwork) and twitter page (Twitter.com/Patient_Network) to help patient partners better connect over shared interests. We are developing a toolbox for the website to provide links and tutorials to help patient partners become more engaged. We have also sent out our first monthly newsletter to the Patient Network, and will archive copies of the newsletter on our website.

In addition, three participants expressed interest in having the organizations that they work with join the Patient, Consumer, and Public Health Coalition. Their organizations have become members, and have participated in written public comments to the FDA, signing on those comments on behalf of their affiliated patient organizations.

One participant reached out to NCHR staff to ask for help in understanding new FDA classifications for an unsafe medical product. NCHR staff responded by phone and email to explain the issues and provide guidance.

Two participants have reached out to NCHR staff concerning upcoming FDA advisory committee meetings, for help and advice concerning their testimony. NCHR helped by phone and email to understand the issues and provide guidance.

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 Training Workshop again to other patient and caregiver advocates. They offered their enthusiastic support and participation in future efforts.

Overall Conclusions

The *Patient Training Workshop* provided an opportunity for patients and family members 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, NIH, and other agencies. 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 said they felt engaged and empowered, with 92% reporting that they would like to be involved in opportunities to provide patient perspectives at public meetings or in written comments. In the three months since the Workshop and Conference, the active engagement of most of the participants has clearly shown that additional workshops and patient engagement efforts on national issues would be welcomed in the patient partner community.

Appendix A: Agendas for Nov 13 and Nov 14

Patient Training Workshop

FRIDAY, NOVEMBER 13

9:30 am – Breakfast

10:00 am – Welcome, introductions, and survey
Diana Zuckerman, PhD, NCHR President
Each participant will briefly introduce themselves by name, organization (if relevant) and one sentence about what they hope to learn.

10:30 am – How I went from advocating for myself to advocating for others, to advocating on a national level
Desirée Walker, Breast Cancer Survivor
Presentation and Q & A

11:00 am – How does the FDA make decisions to approve, rescind, or recall a medical product?
Diana Zuckerman, PhD, NCHR President
Training and Discussion

12:30 pm - Lunch

1:30 pm – How does Consumer Reports evaluate the quality of medical products and how are patients involved?
Victoria Burack, Health Policy Analyst at Consumers Union
Two participants from Safe Patient Project
Presentation and Q & A

2:00 pm – Engaging patients & stakeholders in research: the PCORI perspective
Michelle Johnston-Fleece, MPH, Engagement Officer at PCORI
Presentation and Q & A

2:30 pm – Patient Panel: How we reached out to the FDA and to researchers
Moderator: Diana Zuckerman, PhD
Patient Panel: Three workshop participants

3:30 pm – How can you become a patient representative at the FDA?
Andrea C. Furia-Helms and Salina Prasad, FDA Patient Representative Program
Presentation and Q & A

4:00 pm – Inside the FDA and how you can get your voice heard
Susan Wood, PhD, Professor at the George Washington University
Presentation and Q & A

SATURDAY, NOVEMBER 14

9:30 am – Breakfast

10:00 am – Goals for the day and Research 101 for patient advocates
Margaret Dayhoff-Brannigan, PhD, NCHR Patient Network Project Manager
Training and Discussion

11:00 am – The importance of subgroup analysis for gender, race/ethnicity and age
Laurén Doamekpor, PhD, MPH, NCHR Public Health Analyst
Training and Discussion

11:45 am – The importance of patient advocates
Tim Horn, MPH, HIV Project Director of the Treatment Action Group
Susan Molchan, MD, National Physicians Alliance
Presentation and Q & A

12:30 pm – Lunch

1:30 pm – Opportunities for patient engagement at the FDA and NIH
Paul Brown, BA, NCHR Government Relations Manager
Tracy Rupp, PharmD, MPH, RD, NCHR Senior Fellow
Training and Discussion

2:15 pm – Final survey

2:30 pm – Videos of patient advocates and role playing exercise
Facilitated by: Margaret Dayhoff-Brannigan, Diana Zuckerman, Paul Brown, Tracy Rupp, Desirée Walker, and Laurén Doamekpor

3:40 pm – Final words of wisdom
Diana Zuckerman, PhD, NCHR President

4:00 pm – Adjourn

Appendix B: Pretest/Posttest Questionnaire

1. 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) Based on research on humans, the drug is proven effective for most patients.
d) The drug has been shown to work better than other available treatments for the same illness or disease.

2. 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 in clinical trials.
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.

3. Why is a p-value important?
a) It tells us if a medical product will be approved by the FDA.
b) It tells us how important a medical product is.
c) It tells us the probability that a result is true or just happened by chance.
d) It tells us if a study needs more data.

4. 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.

5. What is a randomized controlled clinical trial?
a) A study of whether patients' health improves after taking a new drug.
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 take a new medical product or not.
d) The "gold standard" for research on human health and required by FDA for drugs and devices.

6. What is a biomarker or surrogate endpoint?
a) A measurement that lets researchers know the study has ended.
b) Another name for a drug that is being tested.
c) A measurement that is believed to predict survival or improved health but isn't itself a measure of survival or improved health.

d) A genetic test given during a clinical trial.

7. For patients, what is the advantage of studies that measure a surrogate endpoint compared to one that measures 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.

8. What is subgroup analysis?

a) A type of statistical analysis that evaluates how different types of people respond to a medical product.

b) A type of statistical analysis that is used to decide whether a medical product will be approved by the FDA.

c) A type of statistical analysis to determine how much money a medical product will make annually.

d) A type of statistical analysis to choose who will be included in a clinical trial.

9. 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 or make the results public.

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 more scientific than pre-market studies.

e) Post-market studies are often longer than pre-market studies.

10. 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."

11. Please answer in 2-3 sentences

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?

12. Please answer in 1-2 sentences

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.

13: Here are some examples of opportunities for informing the FDA about the perspective of 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.

1 2 3 4 5 a. Speak for a few minutes during the public comment period of an FDA or NIH 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.

1 2 3 4 5 b. Speak for a few minutes during the public comment period of an FDA or NIH 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.

1 2 3 4 5 c. Write a comment giving your perspective in response to an agency's "request for public comments" on a topic of importance to you as a patient.

1 2 3 4 5 d. Add your name as one of the people signing a letter written by other patients or nonprofit organization in response to an agency's "request for public comments" on a topic of importance to you as a patient.

1 2 3 4 5 e. Speak at Congressional hearing, Congressional briefing, or a meeting with FDA or NIH officials on a topic of importance to you as a patient.

Appendix C: Evaluation of Patient Workshop

FRIDAY NOVEMBER 13, 2015

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: How I went from advocating for myself to advocating for others, to advocating on a national level, Desirée Walker, Breast Cancer Survivor. Rate talk and Q & A together.

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

   Comments?

2. How does the FDA make decisions to approve, rescind, or recall a medical product? Dr. Diana Zuckerman from the National Center for Health Research. Rate talk and discussion together.

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

   Comments?

3. First session after lunch: Victoria Burack, Health Policy Analyst at Consumers Union and 2 participants from Safe Patient Project (Rex Johnson and Linda Radach). Rate talk and Q & A together.

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

   Comments?

4. Engaging patients and stakeholders in research: the PCORI perspective. Michelle Johnston-Fleece from PCORI. Rate talk and Q & A together.

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

Comments?

5. Patient panel discussion about reaching out to the FDA or researchers. Featuring Jeremy, Katherine and Kim, with Diana Zuckerman as moderator.

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

Comments?

6. How can you become a Patient Representative? Andrea C. Furia-Helms and Salina Prasad from the FDA. Rate presentation and Q & A together.

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

Comments?

7. Insiders View of Patient Advocacy and the FDA, Dr. Susan Wood professor at GWU. Rate talk and Q & A together.

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

Comments?

SATURDAY NOVEMBER 14, 2015

8. Research 101 for Patient Advocates, Margaret Dayhoff-Brannigan from the National Center for Health Research. Rate presentation and Q & A together.

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

Comments?

9. The importance of subgroup analysis, Laurén Doamekpor, National Center for Health Research. Rate presentation and Q & A together.

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

Comments?

10. The importance of patient advocates, Discussion by Tim Horn, HIV Project Director from Treatment Action Group, and Dr. Susan Molchan from National Physicians Alliance. Rate presentation and Q & A together.

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

Comments?

11. Opportunities for patient engagement at the FDA and NIH, Paul Brown and Dr. Tracy Rupp, from the National Center for Health Research. Rate presentation and Q & A together.

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

Comments?

12. Viewing Videos of Patient Advocates and Role Playing exercise, led by Margaret Dayhoff-Brannigan, Laurén Doamekpor, Paul Brown, and Tracy Rupp from the National Center for Health Research. Rate presentation and Q & A together.

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

Comments?

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

14. Overall, how would you rate the workshop (both days)? 1 2 3 4 5

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

YES NO

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