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

Strengthening Patient Voices on Research Criteria

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Background

The National Center for Health Research (NCHR) held four two-day Patient Training Workshops in Washington, D.C. from 2015-17. Three workshops were introductory level and each were attended by approximately 30 patient partners. Twenty-six patient partners attended the Advanced Workshop, which included participants who attended either a 1-day Introductory Workshop we held in 2014 or the 2-day Introductory Workshop that we held in 2015.

The Workshops were designed to increase patient partners’ understanding of comparative effectiveness research (CER) that determines the safety and effectiveness of medical products. The goal was to provide patient partners with training that would increase their knowledge and confidence to provide patients’ perspectives regarding clinical research design and results. The Workshops provided information for patient partners to help them serve as reviewers on research grants and to meaningfully engage with university researchers and health agencies such as the Food and Drug Administration (FDA) and the National Institutes of Health (NIH).

The 101 participants are listed in Appendix A.

Two-day Introductory Patient Training Workshops were held in November 2015, October 2016 and June 2017. The Advanced Patient Training Workshop was held in June 2016.

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1 The 2014 Workshop was partially supported by a 2014 PCORI Engagement Award, while the 2015-2017 Workshops were partially supported by PCORI Engagement Award #1379.
These workshops were designed to build a network of trained patient partners that can become valuable assets to government, nonprofit, and university researchers to improve patient centered outcomes research (PCOR). The Advanced Patient Training Workshop was held to establish potential leaders in the network to help members improve their understanding of clinical research and the role the research and federal agencies play in making new medical products available. Each of the four workshops trained approximately 30 patient partners.

Almost all of the 101 patients who were trained created the USA Patient Network, an independent network of patient advocates working to ensure that medical products are safe and effective for all. The USA Patient Network has a website (www.USAPatientNetwork.org), Facebook page, Twitter Account, and list serv, so that Members can continue to learn and share information.

The four workshops and related activities were partially supported through a Patient-Centered Outcomes Research Institute (PCORI) Eugene Washington Engagement Award (#1379-NCHR).

**Patient Training Workshop Objectives**

**The major objectives of the workshops were:**

1) Teach patient partners the basics of clinical trial design and analysis, the standards for health outcomes used in the FDA approval processes, the importance of patient-centered outcomes, and the benefits of diversity in clinical trials and subgroup analysis;

2) Train patient partners to share their views at public meetings via oral and written comments;

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2 Two of the patient partners died during the two years of the project, and a small number did not join the USA Patient Network. However, several friends and family members of trained patient partners have joined the USA Patient Network.
3) Teach patient partners about opportunities to serve on advisory committees and as research proposal reviewers;
4) Provide patient partners with information about engagement opportunities that will enable them to share their perspectives regarding patient-centered outcome research, and give them the knowledge and confidence to seek those opportunities.

The agendas for the Patient Training Workshops are included in Appendix A.

**Curriculum**

The workshops were led by staff of the National Center for Health Research (NCHR), patient partners who served as consultants to the project, and guest speakers. Keynote speakers for each workshop were patient partners. They included Desiree Walker (breast cancer survivor), the keynote speaker for two workshops; Kim Witczak (Woody Matters founder); and Jeremy Lew (an unaffiliated patient advocate).

Patient Partners comprised all the speakers at one Patient Panel at each Workshop, role played the parts of Advisory Committee members and public speakers at the Mock FDA Advisory Committee meeting, and were active participants throughout the Workshops. In some cases, patient partners who were trained at our previous workshops returned to participate in the Patient Panel and Mock FDA Committee meeting as well.

Guest speakers included Tom Burton (Pulitzer Prize winning journalist for the Wall Street Journal who writes about medical products); Victoria Burack (Health Policy Analyst at Consumers Union); Tim Horn (HIV Project Director of Treatment Action Group); Michelle Johnston-Fleece (PCORI Engagement Officer), Shivonne Laird (PCORI Program Officer); Susan Molchan (Board member of the National Physician’s Alliance); Salina Prasad and Andrea Furia-Helms (FDA patient representative program); and Susan Wood (former Associate Commissioner for Women’s Health at FDA and associate professor at George Washington University School of Public Health).

**The curriculum included:**

1) Explaining the reasons for different types of clinical trials;
2) The importance of including women, men, people of color, and people under and over 65 in clinical trials;
3) FDA approval criteria for drugs and medical devices, and the role of patient partners in that process;
4) How patients can find out about clinical trials;
5) Options for desperate patients who are not eligible for existing clinical trials.

Short presentations were combined with Question and Answer sessions (Q&A’s) and small group breakout sessions to discuss examples of the concepts presented.

What did Patients Learn?

To determine if we achieved our objectives, we administered a pretest and posttest questionnaire. The questionnaires were used to assess changes in participants’ knowledge, attitudes, and beliefs.

Identical questionnaires were distributed to workshop participants before the start of the workshops and before the discussion at the end of the workshops to determine participants’ knowledge before the workshop and how it changed after attending the workshop. Almost all workshop participants completed the pretest and posttest. Only those that completed both questionnaires were evaluated.

The questionnaires from each workshop assessed participants on the same content. However, some questions in later questionnaires were modified based on feedback from earlier workshops. Appendix C is the questionnaire from the first workshop in November 2015 and Appendix D includes the questions from the second and third Introductory Workshop as well as a table showing which questions were comparable to those from the 2015 questionnaire. Several questions from the original questionnaire were excluded from data analysis because they were not comparable to questions in the revised questionnaire.
A sample size of 85 was used in data analysis for questions 1, 2, 3.2, 3.3, 4, and 6 from the Introductory workshops. Responses for questions 3.1, 5, 7.1, 7.2, and 7.3 were analyzed for only 54 participants, because there were no comparable questions on the original questionnaire. For the Advanced Questionnaire, 26 patient partners completed both a before and after questionnaire.

The introductory workshop questionnaires also included 2 open-ended questions on opinions on drug advertisements and speed of the FDA approval process.

There were a total of 8 multiple-choice questions, which included a maximum of 12 correct responses, since two questions had multiple parts; total scores ranged from 0 to 12. Participants included their first name on the questionnaire so that we could compare pretest and posttest scores. For the three Introductory Workshops, average scores increased from 7.6 to 9.6, indicating a significant increase in knowledge following the workshops (paired t-test: \( t=4.79 \), \( p=0.0006 \)) (Table 1).

**Table 1: Paired t-Test to Compare Correct Answers in Pretest and Posttest in the Introductory Workshops**

<table>
<thead>
<tr>
<th></th>
<th>Pretest (n=81)</th>
<th>Posttest (n=81)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mean</td>
<td>7.6</td>
<td>9.6</td>
</tr>
<tr>
<td>Standard Deviation</td>
<td>0.6</td>
<td>0.3</td>
</tr>
<tr>
<td>t Statistic</td>
<td>0.6</td>
<td>0.3</td>
</tr>
<tr>
<td>P-Value (two-tailed)</td>
<td>4.79</td>
<td>0.0006</td>
</tr>
</tbody>
</table>

For the Introductory Workshop, all 12 individual questions that were scored indicated an increase in knowledge, sometimes dramatically. As shown in Table 2, for 6 of the questions, there was a significant increase, at a significance level of 0.05, in posttest versus pretest scores following the Introductory Workshops.
### Table 2: Correct Answers to Individual Questions in Pretest and Posttest in the Introductory Workshops

<table>
<thead>
<tr>
<th>Question</th>
<th>Pretest</th>
<th>Posttest</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Q1</strong> Criteria for FDA drug approval (n=85)</td>
<td>74%</td>
<td>79%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q2</strong> Drug vs. device standards (n=85)</td>
<td>59%</td>
<td>93%</td>
<td>&lt;0.0001</td>
</tr>
<tr>
<td><strong>Q3</strong> Matching clinical trial types</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3.1) Uncontrolled clinical trial (n=54)</td>
<td>54%</td>
<td>69%</td>
<td>NS</td>
</tr>
<tr>
<td>3.2) Randomized controlled trial (n=85)</td>
<td>64%</td>
<td>65%</td>
<td>NS</td>
</tr>
<tr>
<td>3.3) Randomized double blind trial (n=85)</td>
<td>82%</td>
<td>98%</td>
<td>0.0081</td>
</tr>
<tr>
<td><strong>Q4</strong> Define biomarkers or surrogate endpoints (n=85)</td>
<td>75%</td>
<td>86%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q5</strong> Importance of subgroup analysis (n=54)</td>
<td>98%</td>
<td>100%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q6</strong> Advantages of studying biomarkers (n=85)</td>
<td>56%</td>
<td>80%</td>
<td>0.0008</td>
</tr>
<tr>
<td><strong>Q7</strong> Differences between pre- and post-market studies (n=54)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>2) 36% 60% 0.013</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3) 35% 62% 0.0052</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Q9</strong> Meaning of statistical significance (n=54)</td>
<td>95%</td>
<td>100%</td>
<td>NS</td>
</tr>
</tbody>
</table>

Comparisons in shaded boxes were statistically significant.

The Advanced Workshop questionnaire consisted of 10 multiple-choice, matching, and true/false questions, some of which had several parts, which tested knowledge on clinical trial design and standards used in the FDA approval process. The posttest included an open-ended question regarding patient participation in clinical trials. (Appendix E).
The multiple-choice questions had either one or several correct answers. Total possible correct scores ranged from 0 to 17. Average scores increased from 10.1 to 12.1 following the Advanced Workshop, indicating a significant increase in knowledge among the participants ($t=4.02, p=.001$) (Table 3).

For the Advanced Workshop, 14 of the 17 individual question parts showed an increase in scores. However, due to the small sample size, only two of these increases were statistically significant (Table 4).

**Table 3: Paired t-Test to Compare Correct Answers in Pretest and Posttest in the Advanced Workshop (N=26)**

<table>
<thead>
<tr>
<th></th>
<th>Pretest</th>
<th>Posttest</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mean</td>
<td>10.1</td>
<td>12.1</td>
</tr>
<tr>
<td>Standard Deviation</td>
<td>1.2</td>
<td>1.0</td>
</tr>
<tr>
<td>t Statistic</td>
<td></td>
<td>4.02</td>
</tr>
<tr>
<td>P-Value (two-tailed)</td>
<td></td>
<td>0.001</td>
</tr>
</tbody>
</table>
Table 4: Correct Answers in Advanced Workshop Pretest and Posttest

<table>
<thead>
<tr>
<th>Question</th>
<th>Pretest (n=26)</th>
<th>Posttest (n=26)</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Q1: Criteria for FDA drug approval</strong></td>
<td>62%</td>
<td>81%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q2: Drug vs. device standards</strong></td>
<td>62%</td>
<td>88%</td>
<td>0.03</td>
</tr>
<tr>
<td><strong>Q3: Matching clinical trial types</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3.1) Uncontrolled clinical trial</td>
<td></td>
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<td>3.2) Randomized double blind clinical trial</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>3.3) Randomized controlled clinical trial</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Q4: Define biomarkers or surrogate endpoints</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Q5: Why is subgroup analysis important?</strong></td>
<td>92%</td>
<td>100%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q6: Advantages of studying biomarkers</strong></td>
<td>8%</td>
<td>16%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q7: Differences between pre- and post-market studies</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>A)</td>
<td>36%</td>
<td>40%</td>
<td>NS</td>
</tr>
<tr>
<td>B)</td>
<td>24%</td>
<td>36%</td>
<td>NS</td>
</tr>
<tr>
<td>C)</td>
<td>52%</td>
<td>48%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q8: Advisory Committees</strong></td>
<td>88%</td>
<td>76%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q9: Define statistical significance</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>A)</td>
<td>84%</td>
<td>92%</td>
<td>NS</td>
</tr>
<tr>
<td>B)</td>
<td>96%</td>
<td>92%</td>
<td>NS</td>
</tr>
<tr>
<td>C)</td>
<td>60%</td>
<td>76%</td>
<td>NS</td>
</tr>
<tr>
<td>D)</td>
<td>40%</td>
<td>56%</td>
<td>NS</td>
</tr>
<tr>
<td><strong>Q10: Expanded access</strong></td>
<td></td>
<td></td>
<td>0.01</td>
</tr>
</tbody>
</table>

**Summary of Results**

**Introductory Patient Training Workshops**

- The percentage of correct answers increased from 63% before the Workshop to 80% afterwards.
- The greatest increase in knowledge was regarding the differences between FDA approval standards for drugs and medical devices (Q2). Correct responses increased from 59% before the Workshop to 93% afterwards.
- Prior to the Introductory Workshop, participants knew the least about the differences between pre- and post-market studies (Q7). The percentage of patients who correctly answered that question increased by nearly 20%.
Advanced Patient Training Workshop

- Most of the participants in the Advanced Workshop had previously attended one of our Introductory Workshops, so they already knew the importance of diversity in clinical trials and subgroup analyses (92%). Before the Advanced Workshop started, more than 80% correctly defined a randomized double blind clinical trial and understood the role of advisory committees.
- There was less room for improvement and the number of participants was small, resulting in statistically significant improvement on only 2 questions.
- The greatest learning was on expanded access (Q10), with correct responses increasing by 36%, and on understanding of the differences between FDA standards for drugs and medical devices (Q2), with correct responses increasing by 26%.

Open-Ended Questions

Direct to Consumer Advertising (Q9)

We included an open-ended question in the Introductory Workshop pretest and posttest questionnaires as an ice-breaker and to assess participants’ views on TV ads for prescription drugs.

Q9: 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?

Most participants said that the warnings were not helpful. Some participants stated that these ads should be banned. However, a few participants said the warnings were helpful and allowed patients to make an informed decision about the risks and benefits of drugs before taking them.

After the workshop, most participants did not change their views on this topic, but their responses tended to reflect what they had learned during the Workshop.

Here are examples of suggestions to improve warnings from the Pretest:
- Participant A, Pretest: Yes, they are helpful to patients. It allows the patient to be a part of the decision whether the drug is right for them. I would like the risks to be a larger part of the ad, but since the object is to sell the drug, this is not acceptable.
Participant B, Pretest: I believe mentioning risk for drugs advertised on TV is very helpful in educating someone about the possible dangerous side effects of a drug. I would improve the advertisements by adding more air time for more ads & saturate the public about a dangerous drug.

Here are examples of suggestions to improve warnings from the Posttest:

Participant A, Posttest: Yes, It helps patients to question whether the drug might be right for them. I would like to see how the risks affect the different categories of patients studied.

Participant B, Posttest: Require the company to list the percentage of patients with side effects. Require benefit to be specified – “average 26 minutes of more sleep a night.”

FDA Approval Too Fast or Too Slow? (Q10)

In the Introductory Workshop pretest and posttest questionnaires, we also asked patients whether drugs are approved too quickly or too slowly.

As expected, participants expressed differing views on this issue, often pointing out that the process can be too slow for those waiting on treatments for life threatening diseases but too fast for medical products that are not adequately tested. Some participants reported that they did not have enough information to answer this question. Many participants demonstrated their understanding of important issues, such as subgroup analysis.

Q10: Do you think the FDA approval process is too fast or too slow? If you have an opinion one way or another, explain why you believe that to be true.

Here are some examples of responses from the Pretest:

Participant A, Pretest: “I don’t feel like I personally have the knowledge to answer, but from what I do know (especially with how the government works) I’d lean to the side of too slow.”

Participant B, Pretest: “Depends on the population – for the general population the FDA approval process is probably ok. However for patients with rare disorders, the
process may not be fast enough to help patients – these are the patients who would benefit most from surrogate endpoints and biomarkers. They are also the patients who are more willing to accept a higher level of risk since their disorders are fatal.”

Here are some examples of responses from the Posttest:

**Participant A, Posttest:** “I think it should take however long is needed to get quality, telling data. Sample size, subgroups, etc. all need to be taken into account.”

**Participant B, Posttest:** “Depends on the product and the disease/patient population. For patients who are desperate with life threatening diseases (patients who may be willing to accept more risks) the current approval process may be too slow but FDA cannot ignore patient safety or the safety of a medication in order to approve a medication faster. Delicate balance between safety (benefits) or new meds for life threatening medical conditions and the risks of these meds. Sometimes the natural progression of a life-threatening disorder may be better than a drug approved too quickly.”

**Questions to Ask a Doctor Concerning Clinical Trial Participation? (Q10)**

In the Advanced Workshop posttest, we asked participants what questions are important for a patient considering participating in a clinical trial to ask their doctor.

Participants expressed somewhat similar views on the topic. Overall, they were concerned about the risks of the drug, how extensively the drug had been tested, and how patients would be compensated. Participants expressed comprehensive understanding of the clinical trial process including different phases, qualifications for studies, and potential of financial cost to participants.

Q10: Do you think the FDA approval process is too fast or too slow? If you have an opinion one way or another, explain why you believe that to be true.

Here are some examples of responses:

**Participant A, Posttest:** “What is the cost of the study (time, travel, etc), who’s responsibility to pay for additional medical costs, and what is the study protocol”
Participant B, Posttest: “Will the patient be taken care of if harmed (medically or financially), is this a phase 1, 2, or 3 clinical trial, and will travel be covered”

Participants’ Evaluation of Workshop

We asked participants to anonymously fill out evaluation questionnaires of each speaker/panel and of the workshop in general (see Appendix F). Participants rated the first day of the workshop at the beginning of the second day. They rated the second day at the conclusion of the workshop. The last evaluation included questions about the workshop overall and whether participants were interested in joining the USA Patient Network. The evaluations were scored on a rating scale from 1 to 5, with 1 being “not at all helpful” and 5 being “very interesting or helpful.”

Summary of Participants’ Evaluation of Workshop

- The workshop and speakers/panels in general were rated very highly. The overall evaluations had an average score of 4.8 on a scale from 1 to 5, with 1 being “not at all helpful” and 5 being “very interesting or helpful.”
- Participants reported that it was important to learn how the FDA works and stated that they learned valuable information from this workshop that they wanted to share with other patient partners.
- Participants benefited from the small group activities and appreciated the high level of interaction and lively discussion with other participants and NCHR staff.
- Participants reported that they thoroughly enjoyed participating in the “Mock FDA Advisory Committee Meeting” and that it was an excellent learning tool that allowed them to understand issues that may arise at a real meeting. This activity allowed participants to act as “committee members” for a reenactment of the real life meeting for the approval of Belsomra (a sleeping pill that FDA subsequently approved and is now widely advertised on TV).
- Ratings for individual speakers/panels are presented in Table 5
### Table 5: Presentation and Workshop Average Ratings

<table>
<thead>
<tr>
<th>Workshop Session</th>
<th>Average</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Friday</strong></td>
<td></td>
</tr>
<tr>
<td>1. Advocacy Matters</td>
<td>4.8</td>
</tr>
<tr>
<td>2. FDA Standards: What You Need to Know as an Advocate</td>
<td>4.8</td>
</tr>
<tr>
<td>3. Introduction to Clinical Trials</td>
<td>4.6</td>
</tr>
<tr>
<td>4. The Importance of Sex, Race/Ethnicity &amp; Age When Treatments are Studied</td>
<td>4.7</td>
</tr>
<tr>
<td>5. FDA Opportunities for Patients: Q &amp; A</td>
<td>4.8</td>
</tr>
<tr>
<td><strong>Saturday</strong></td>
<td></td>
</tr>
<tr>
<td>1. Opportunities for Patient Engagement at the FDA and NIH</td>
<td>4.7</td>
</tr>
<tr>
<td>2. Patient Panel: How Advocates Have Reached Out to FDA and Researchers</td>
<td>4.6</td>
</tr>
<tr>
<td>3. Mock FDA Advisory Committee Meeting</td>
<td>4.8</td>
</tr>
<tr>
<td>4. How You Can Get Your Voice Heard at the FDA</td>
<td>4.7</td>
</tr>
<tr>
<td>5. Discussion: Vision for the USA Patient Network</td>
<td>4.6</td>
</tr>
<tr>
<td><strong>Overall</strong></td>
<td></td>
</tr>
<tr>
<td>Speakers/Moderators as a whole?</td>
<td>4.8</td>
</tr>
<tr>
<td>Workshop as a whole?</td>
<td>4.8</td>
</tr>
</tbody>
</table>

### Panel Evaluations

1. **“Advocacy Matters”** Patient Keynote (Desirée Walker, Kim Witczak, or Jeremy Lew)

   Participants found Desirée to be very inspiring and motivating. Many of the participants reported her talk was “powerful, knowledgeable, and helpful.”

   Participants found Kim to be very inspiring and shared that her story was compelling.

   Participants found Jeremy's story to be empowering. Respondents noted that his engaging presentation generated useful reflection about patient advocacy and off-label use.

   **Average Rating: 4.8**
2. “What you Need to Know About FDA Standards” by Diana Zuckerman, PhD, President of the National Center for Health Research

Participants found Diana’s talk to be very informative and eye opening. They reported they were amazed by the information shared and the lack of safety and effectiveness criteria for many medical devices.

**Average Rating:** 4.8

3. “Introduction to Clinical Trials: What You Need to Know as an Advocate” by Margaret Dayhoff-Brannigan, PhD, Miara Jeffress, PhD, or Megan Polanin, PhD, National Center for Health Research

Participants thoroughly enjoyed the presentation and the accompanying visuals. Participants said they liked being involved in the activity and reported it was a great way to reinforce the information. Participants said that “seeing the evidence, good evidence, in making future treatment decisions was necessary, interesting, and well presented.”

**Average Rating:** 4.6

4. “The Importance of Subgroup Analysis for SexRace/Ethnicity and Age” by Lauren Doamekpor, PhD, Miara Jeffress, PhD, or Stephanie Fox-Rawlings, PhD, National Center for Health Research

Participants reported this was an important topic for discussion. Many participants enjoyed breaking into small groups to discuss subgroup analysis. Participants said this talk was “fascinating” and appreciated the different perspective on how to look at study results that they were previously unaware of.

**Average Rating:** 4.7

5. “FDA Opportunities for Patient Engagement” by Andrea Furia-Helms and Salina Prasad, FDA Patient Representative Program and Patient Liaison Team

Participants were grateful for the opportunity to get first hand insight from FDA representatives.

**Average Rating:** We changed the format to Q&A after the first workshop (which scored 4.5) and the average score increased to 4.8.
6. “Opportunities for Patient Engagement at the FDA and NIH” by Paul Brown or Jack Mitchell, National Center for Health Research

Participants found Paul's talk to be very helpful and provided valuable information. Many participants said that he needed more time.

Participants said Jack's presentation was “excellent and well-informed.” Many respondents requested follow-up materials to pursue opportunities at FDA and NIH.

**Average Rating: 4.7**

7. “Patient Panel: How Advocates Have Reached out to FDA and Researchers” by Katherine Leon, Rachel Brummert, Angela Lynch; or Mary Jackson, Jasmaine McClain, Cal Pierce, and Penelope Burau

Participants loved hearing directly from other patient advocates and found their stories to be encouraging, inspiring and motivating.

**Average Rating: 4.6**

8. “Mock FDA Advisory Committee Meeting Starring All of Us” Moderated by Diana Zuckerman, PhD, President of National Center for Health Research

At least 5-10 patient partners role played in the Mock Advisory Committee meeting. Whether or not they role played, workshop participants reported that they really enjoyed having the opportunity to witness what an FDA Advisory Committee Meeting can be like. Many said it was very helpful, provided real insight into the process, and was a great way to learn.

Many participants loved this session and said that it could be a stand-alone training.

**Average Rating: 4.8**


This panel was included in one of the Workshops. Participants really appreciated hearing from Susan Wood and loved her presentation style and how she guided the presentation based off of questions.

**Average Rating: 4.7**
10. “Discussion: Vision for the USA Patient Network”

Participants said they were excited to discuss the plans and goals for the Network moving forward.

Average Rating: 4.6

General Questions

11. Overall, how would you rate the speakers/moderators? Average rating: 4.8

12. Overall, how would you rate the Workshop? Average rating: 4.8

13. Would you like to continue to be involved in the USA Patient Network?

100% responded YES. All patient partners stated their commitment to staying involved in the USA Patient Network as well as further collaboration with each other and the National Center for Health Research.

Our Overall Performance Based on Participants’ Anonymous Evaluations

What We Did Well

- Provided detailed explanations on the FDA approval processes for new drugs and devices.
- Answered questions with specific and appropriate detail.
- Facilitated interactive and engaging small group activities, 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: “Words cannot express how helpful these two days were, the NCHR and fellow attendees, excellent.”

Participant B: “Appreciate the time and effort to put this together-- one voice is important but a set of collaborative voices is powerful, I think this was a wonderful opportunity --2 days jam packed with helpful information.”
Participant C: “Thank you for your leadership, vision, clarifying info, and network of support.”

Suggestions for Improvement from Patient Partners (some of which were suggested in early workshops and incorporated into later workshops)

- Provide handouts on the FDA approval process and how to become involved with the FDA.
- Provide more training on what methods work best for patients to get their voices heard, and how they can be effective communicators.
- Provide more detailed 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.

Trainers’ Observations

The participants were actively engaged throughout the workshops, asking questions and sharing their views. Participants displayed an eagerness to learn as much as possible to better prepare them for advocacy work focused on patient centered outcomes research. All four workshops seemed to empower and energize participants. The workshops provided an environment to brainstorm about ways to work together or as individuals to share their perspectives as patients regarding clinical trial design and outcome criteria to ensure safer, more effective treatments. Participants were very motivated to actively participate in the future and expressed interest in continuing to work with the National Center for Health Research and as members of the USA Patient Network.

Follow Up and Advocacy Activities

We invited the new patient partners to join our listserv and mailing list for the monthly newsletter. We also posted all of the materials from the workshop on the USA Patient Network website under the tools section (www.USAPatientNetwork.org). The USA Patient Network consists of patients, caregivers, and their friends and family members that are united by a common goal: to make sure that medical treatments are as safe and effective as possible.
Network website includes webinars, based on presentations and Q&A sessions from the workshops. Many of the webinars are in the Members Only section of the website.

Following the workshops, The USA Patient Network members have become involved in patient engagement opportunities at the FDA. They have been advocating for several different issues, such as better informed consent based on patient-centered outcome research. The USA Patient Network members sent an email letter to Salina Prasad and Andrea Furia-Helms, from the FDA Patient Representative Program, to reiterate our request that the FDA make patient safety a priority when the FDA requires data for approval decisions.

Since the workshops, participants have engaged in advocacy work at the federal level. A few examples of advocacy activities are below:

- Several members testified at the FDA Unapproved Uses of Approved Medical Products Advisory Committee meeting on November 9-10, 2016. Participant Jonathan Furman spoke at the FDA meeting on behalf of the USA Patient Network about the need for informed consent regarding risks of off-label prescriptions.

- On January 6, 2017 Patient Network members provided comments to the FDA on establishing the Patient and Care-Partner Connection Program.

- On June 12, 2017 the USA Patient Network wrote a letter to FDA in support of establishing a new Office of Patient Affairs.

- On June 15, 2017 USA Patient Network members Jamee Cook, Chandra DeAlessandro, and Raylene Hollrah met with Congresswoman Rosa DeLauro to discuss the lack of safety data on implants and the need for the FDA to require better research. These same USA Patient Network members also met with FDA representatives to discuss better information for patients about risks of implants.

**Overall Conclusions**

The Patient Training Workshops provided an opportunity for patient partners with a wide range of knowledge and perspectives to come together and learn more about comparative effectiveness and the research criteria for medical product approval. Participants also learned how they can have their voices heard by the FDA, NIH, and other agencies. The project achieved all of the planned objectives and trained patient partners to be valuable assets to researchers at federal agencies and medical centers to improve patient centered outcomes research. This was shown by the statistically significant increase in knowledge about research standards for drug and device approval and growing confidence that patient partners expressed. The active engagement of participants following the workshops indicates that the Workshops energized and empowered the patient partners.
Appendix A: Agendas

Introductory 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
   Rex Johnson and Linda Radach, 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

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
**Advanced Patient Training Workshop**

**FRIDAY, JUNE 3**

12:00  **Lunch, Introductions, Rules for the Workshop, and Getting Started**  
*Diana Zuckerman, PhD, NCHR President*  
After filling out the questionnaire, each participant will briefly introduce themselves by name, organization (if relevant) and one sentence about what they hope to learn.

**Direct to Consumer Drug Ad Videos and Discussion:** What do the ads *say* about risks and benefits vs. what do they *imply* about risks and benefits?

1:30  **What you Need to Know About FDA Standards: For Advanced Advocates**  
*Diana Zuckerman, PhD, NCHR President*  
*DTC ads vs. FDA pivotal studies*  
Training and Discussion

3:00  **Break**

3:15  **Advocacy Matters**  
*Desirée Walker, Breast Cancer Survivor*  
Presentation and Q & A

3:50  **Understanding The Evidence For Good Treatments**  
*Margaret Dayhoff-Branigan, PhD*  
Training and Small Group Discussion on different types of clinical trials, why the number of patients matter, etc.

4:30  **FDA Opportunities for Patient Engagement Q & A**  
*Andrea Furia-Helms and Salina Prasad, FDA Patient Representative Program and Patient Liaison Team*

5:00  **Patients and Reporters (Q & A)**  
*Tom Burton, Wall Street Journal*

5:30  **Happy Hour**

6:30  **Dinner at 14k Restaurant at Hamilton Crowne Plaza Hotel**

**SATURDAY, JUNE 4**

9:00  **Breakfast**

9:30  **Envisioning Yourself as a Patient Advocate Leader**
10:00 Will this treatment work for me?
Miara Jeffress, PhD,
Presentation and Discussion of Results by sex, age, and race

11:00 Mock FDA Advisory Committee Meeting Starring All of Us!
Short presentations by staff and participants portraying FDA and Company
Sponsor, with all participants contributing to Advisory Committee discussion,
Public Comment period
Moderated by Diana Zuckerman, PhD, NCHR President

12:30 Lunch

1:00 The Reality and Fantasy of Participation in Clinical Trials
Tracy Rupp, PharmD
Diana Zuckerman on Expanded Access and “Right to Try” Laws

2:15 Final Survey

2:30 Discussion: Vision for the USA Patient Network?
Moderated by Diana Zuckerman and Kim Witczak

3:15 Adjourn
Introductory Patient Training Workshop

FRIDAY, OCTOBER 14

9:00 am  Breakfast (yogurt, bagels, pastry, fruit, granola bars, coffee, tea, juice)

9:20  Welcome, Introductions, Survey, Rules for the Day, and TV Ad Discussion
Diana Zuckerman, PhD, NCHR President
Each participant will briefly introduce themselves by name, organization (if relevant), and one sentence about what their main interest is as a patient or patient advocate.
Our survey will help us know what you already know.
TV ad will provide a context: What do you want to know about a medical treatment?

10:30  Advocacy Matters
Kim Witczak, Woody Matters
Presentation and Q & A

11:00  What You Need to Know About FDA Standards
Diana Zuckerman, PhD, NCHR President
Training and Q & A

12:30 pm  Lunch

1:30  Introduction to Clinical Trials: What You Need to Know as an Advocate
Miara Jeffress, PhD
Presentation, Q & A, and Small Group Discussion

2:30  How You Can Get Your Voice Heard at the FDA
Susan Wood, PhD, Former Associate Commissioner for Women’s Health at FDA
Presentation and Q & A

3:15  Break

3:30  Patient Panel: How Advocates Have Reached out to the FDA and to Researchers
Moderator: Diana Zuckerman, PhD
Patient Panel: Katherine Leon, Angela Lynch

4:30  FDA Opportunities for Patients: Q & A
Andrea Furia-Helms and Salina Prasad, FDA Patient Representative Program and Patient Liaison Team
**SATURDAY, OCTOBER 15**

9:00 am  **Breakfast**

9:30  **The Importance of Sex, Race/Ethnicity, and Age when Treatments are Studied**  
*Stephanie Fox-Rawlings, PhD*  
Presentation and Small Group Discussion

10:30  **Opportunities for Patient Engagement at the FDA and NIH**  
*Paul Brown, NCHR Government Relations Manager*  
Presentation

11:00  **Mock FDA Advisory Committee Meeting Starring All of Us!**  
Short presentations by staff and participants portraying FDA and Drug Company, with Workshop participants role playing as Advisory Committee members, Public Comment speakers  
*Moderated by “Mock Committee Chair” Dr. Diana Zuckerman, PhD, NCHR President*

12:30 pm  **Lunch**

1:30  **Discussion: Vision & Action Plan for USA Patient Network**  
Planning Session/Discussion on the goals of the Patient Network and what you would like to see as our goals and how to achieve them

2:30  **Survey: What You Learned and What you Liked**

3:00  **Next Steps and Words of Wisdom**  
*Diana Zuckerman, PhD, NCHR President*  
Q & A and Discussion

3:45  **Adjourn**
**Introductory Patient Training Workshop**

**FRIDAY, JUNE 2**

9:00 am  **Breakfast** (yogurt, bagels, pastry, fruit, granola bars, coffee, tea, juice)

9:20  **Welcome, Introductions, Survey, Rules for the Day, and TV Ad Discussion**

 _Diana Zuckerman, PhD, NCHR President_

Each participant will briefly introduce themselves by name, organization (if relevant), and one sentence about what their main interest is as a patient or patient advocate.

Our survey will help us know what you already know.

TV ad will provide a context: What do you want to know about a medical treatment?

10:30  **Advocacy Matters**

 _Jeremy Lew, spinal surgery patient_

Presentation and Q&A

11:00  **What You Need to Know About FDA Standards**

 _Diana Zuckerman, PhD, NCHR President_

Training and Q & A

12:30 pm  **Lunch**

1:30  **Patient-Centered Outcomes Research Institute: Putting Patients First**

 _Shivonne Laird, PhD, PCORI_

Brief Remarks

1:45  **Introduction to Clinical Trials: What You Need to Know as an Advocate**

 _Megan Polanin, PhD_

Presentation, Q & A, and Small Group Discussion

2:45  **Patient Panel: From the Personal Level to Helping Make Bigger Changes**

Moderator: Diana Zuckerman, PhD

Patient Panel: Cal Pierce, Jasmaine McClain, Mary Jackson, Penelope Burau

3:45  **Break**

4:00  **The Importance of Sex, Race/Ethnicity, and Age when Treatments are Studied**

 _Stephanie Fox-Rawlings, PhD_

Presentation and Small Group Discussion
5:00  Q & A

5:30  Happy Hour

6:30  Dinner at 14k Restaurant at Hamilton Crowne Plaza

SATURDAY, JUNE 3

9:00 am  Breakfast

9:30  Opportunities for Patient Engagement at the FDA and NIH
      Jack Mitchell, NCHR Government Relations Director
      Presentation and Q & A

10:20  FDA Opportunities for Patients: Q & A
       Andrea Furia-Helms and Salina Prasad Miller, FDA Patient Representative
       Program and Patient Liaison Team

11:00  Mock FDA Advisory Committee Meeting Starring All of Us!
       Short presentations by staff and participants portraying FDA and Drug Company, with Workshop participants and NCHR staff role-playing as Advisory Committee members and Public Comment speakers
       Moderated by “Mock Committee Chair,” Dr. Diana Zuckerman, PhD, NCHR President

12:30 pm  Lunch

1:30  Discussion: Vision & Action Plan for USA Patient Network
      Planning session/discussion on the goals of the USA Patient Network and what you would like to see as our goals and how to achieve them

2:30  Survey: What You Learned and What you Liked

3:00  Next Steps and Words of Wisdom
      Diana Zuckerman, PhD, NCHR President
      Q & A and Discussion

3:45  Adjourn
# Appendix B: List of Workshop Participants

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<td>Rick</td>
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Appendix C: Original (2015) 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 receive 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.
Appendix D: Revised Pretest/Posttest Questionnaire

1. When FDA approves a drug as safe and effective, what does that mean?
   a) The drug has been shown to work better than other available treatments for the same illness or disease.
   b) The drug is proven effective for most patients.
   c) The drug has benefits that outweigh the risks for most patients for the approved use.
   d) The drug does not have any serious side effects.

2. How are FDA standards for allowing medical devices to be sold different than for drugs?
   a) Standards for approving medical devices are the same as those for drugs.
   b) 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.
   c) Standards for approving medical implants are usually more stringent than for drugs.

3. Please identify the type of clinical trial in each description. The options are on the right.
   e__ 1) A researcher studies the effect of a new drug on one group of patients. There is no comparison group.
   a__ 2) Patients are assigned randomly to a treatment or control group, and neither the researchers nor patients know which group.
   c__ 3) A study compares the health of patients who were randomly assigned to take a new medical product to those who were not assigned to the new treatment. The doctor and patients know which treatment they are getting.

   a) Randomized double blind clinical trial
   b) Randomized single blind clinical trial
   c) Randomized controlled clinical trial
   d) Controlled clinical trial
   e) Uncontrolled clinical trial

4. What is a biomarker or surrogate endpoint?
   a) A genetic test given during a clinical trial.
   b) Another name for a drug that is being tested.
   c) A measurement that lets researchers know the study has ended.
   d) A measurement that is believed to predict improved health but isn’t itself a measure of improved health.
5. 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 covered by most insurance companies.
   c) The drug will be safer and more effective.

6. Why is subgroup analysis important?
   a) It evaluates how different types of people (sex, age, race, etc) respond to a medical product.
   b) It tells you if a medical product will be approved by the FDA.
   c) It determines how much money a medical product will make annually.

7. Please identify which of these statements are true of pre- or post-market studies on drugs and devices submitted to the FDA.

   Answer with: Pre, Post, both or neither

   _______ a) These studies are usually paid for by the manufacturer
   Neither b) These studies are usually supposed to last at least 2 years
   Neither c) FDA requires these studies to show a clear benefit for women and men of different ages
   Neither d) These studies must always compare a new treatment to an older treatment

8. A researcher says that a new medical product is significantly better than a placebo at the $p < 0.05$ level. What can you conclude from this statement?
   a) The new drug is better than older drugs already for sale.
   b) The new drug probably helps patients live longer.
   c) There is less than a 5% likelihood that the results showing the drug's benefits occurred “by chance.”

   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?

10. 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.

The Introductory Workshop questionnaire consisted of 8 multiple-choice questions, some of which had multiple parts that tested knowledge on clinical trial design and standards
used in the FDA approval process. The question numbering in data analysis descriptions corresponds to the question numbers from the revised questionnaires.
Appendix E: Advanced Workshop Questionnaire

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

- e) The drug has been shown to work better than other available treatments for the same illness or disease.
- f) The drug is proven effective for most patients.
- g) The drug has benefits that outweigh the risks for most patients for the approved use.
- h) The drug does not have any serious side effects.

2. How are FDA standards for medical devices different than for drugs?

- d) Standards for approving medical devices are the same as those for drugs.
- e) 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.
- f) Standards for approving medical implants are usually more stringent than for drugs.
- g) 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. Please identify the type of clinical trial in each description. The options are on the right.

- e 1) A researcher studies the effect of a new drug on one group of patients. There is no comparison group.
- a 2) Patients are assigned randomly to a treatment or control group, and neither the researchers nor patients know which group.
- c 3) A study compares the health of patients who were randomly assigned to take a new medical product to those who were not assigned to the new treatment. The doctor and patients know which treatment they are getting.

a) Randomized double blind clinical trial
b) Randomized single blind clinical trial
c) Randomized controlled clinical trial
d) Controlled clinical trial
e) Uncontrolled clinical trial

4. What is a biomarker or surrogate endpoint?

- e) A genetic test given during a clinical trial.
- f) Another name for a drug that is being tested in a clinical trial.
- g) A measurement that lets researchers know the study has ended.
h) A measurement that is believed to predict improved health but doesn’t measure improved health.

5. Why is subgroup analysis important?
   d) It evaluates how different types of people (sex, age, race, etc) respond to a medical product.
   e) It tells you if a medical product will be approved by the FDA.
   f) It determines how much money a medical product will cost patients.
   g) It can be used to choose what outcomes will be studied in a clinical trial.

6. For patients, what is the advantage of studies that measure a surrogate endpoint compared to one that measures patient health?
   d) The clinical trial may be completed more quickly.
   e) The drug will be covered by most insurance companies.
   f) The drug will be safer and more effective.

7. Please identify which of these statements are true of pre- or post-market studies on drugs and devices submitted to the FDA.

Answer with: Pre, Post, both or neither

Neither 1) These studies are usually paid for by the manufacturer

Neither 2) These studies usually include patients with no other diseases.

Both 3) FDA requires these studies to show a clear benefit for diverse patients by separately analyzing safety and effectiveness for women, men, older and younger adults, and major racial groups.

8. False True or FALSE: FDA must follow the recommendations of their advisory committees.

9. A researcher says that a new medical product is significantly better than a placebo at the p < 0.05 level. What can you conclude from this statement?
   d) The new drug is better than older drugs.
   e) The new drug probably helps patients live longer.
   f) There is less than a 5% likelihood that the results occurred “by chance”.
   g) The new drug is probably better than no treatment at all.
Appendix F: Evaluation of Patient Workshops

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. “What You Need to Know About FDA Standards” by Diana Zuckerman, PhD, President of the National Center for Health Research

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

Comments?


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

Comments?

3. “Introduction to Clinical Trials: What You Need to Know as an Advocate” by Margaret Dayhoff-Brannigan, PhD, Miara Jeffress, PhD, and Megan Polanin, PhD, National Center for Health Research

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

Comments?

4. “The Importance of Subgroup Analysis for Gender/Race/Ethnicity and Age” by Lauren Doamekpor, PhD, Miara Jeffress, PhD, and Stephanie Fox-Rawlings, PhD, National Center for Health Research

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

Comments?
5. “FDA Opportunities for Patient Engagement Q&A” by Andrea Furia-Helms and Salina Prasad, FDA Patient Representative Program and Patient Liaison Team

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

6. “Opportunities for Patient Engagement at the FDA and NIH” by Paul Brown and Jack Mitchell, National Center for Health Research

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

7. “How Advocates Have Reached out to FDA and Researchers” by Katherine Leon, Rachel Brummert, Angela Lynch; or Mary Jackson, Jasmaine McClain, Cal Pierce, and Penelope Burau

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

8. “Mock FDA Advisory Committee Meeting Starring All of Us” Moderated by Diana Zuckerman

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

9. “How You Can Get Your Voice Heard at the FDA” by Susan Wood, PhD, from the George Washington University

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

10. “Discussion: Vision for the USA Patient Network”

1 2 3 4 5  (1 not helpful or interesting and 5 very helpful/interesting)
Comments?
11. Overall, how would you rate the speakers/moderators? 1 2 4 5

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

13. Would you like to continue to be involved in the USA Patient Network?
   YES  NO

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