Workshop Executive Summary:
Understanding Patients’ Preferences: Stimulating Medical Device Development in Kidney Disease

August 12-13, 2015
Baltimore, Maryland
Disclaimer

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Kidney Health Initiative Overview

KHI Mission

To advance scientific understanding of kidney health and patient safety implications of new and existing medical products and to foster development of therapies for diseases that affect the kidney by creating a collaborative environment in which FDA and the greater nephrology community can interact to optimize evaluation of drugs, devices, biologics, and food products.

About KHI

The Kidney Health Initiative (KHI) is a partnership established by the American Society of Nephrology (ASN) and the U.S. Food and Drug Administration (FDA) to improve kidney health and patient safety as well as optimize evaluation of drugs, devices, biologics, and food products. Since its inception, KHI has steadily increased its membership to its current state of over 75 member organizations.

More than 20 million Americans have kidney disease and more than 600,000 Americans have end stage renal disease. ASN is dedicated to improving care for the millions of people in the United States, and around the world, affected by kidney disease. However, to improve care for patients with kidney disease, more innovation is required. In particular, during the past decade, too few new therapies have appeared on the market in the United States.

With this problem in mind, the leadership of ASN met with representatives from the FDA on World Kidney Day 2012 to discuss ways to strengthen the relationship between the FDA and the kidney community. By September 2012, then FDA Commissioner Margaret A. Hamburg, MD, and then ASN President Ronald J. Falk, MD, FASN, signed a Memorandum of Understanding establishing KHI.

KHI is a collaborative environment for all stakeholders in the kidney community to help foster development of optimum therapies for diseases that affect the kidney. KHI members may include:

- Patient organizations
- Health professional organizations
- Research institutions
- Foundations
- Pharmaceutical and biotechnology companies
- Device manufacturers
- Dialysis organizations
- U.S. and international government agencies

To learn more about the Kidney Health Initiative, please visit www.kidneyhealthinitiative.org.
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Executive Summary

Approximately 110 members of the kidney community gathered in Baltimore, Maryland for a one and a half day workshop to discuss the concept and use of patient preferences in medical device development. Participants in the discussions included patients on hemodialysis (HD) - both in-center and home, patients on peritoneal dialysis (PD) and patients who have received a kidney transplant (See Appendix A). Attendees also included family members, care partners, physicians, nurses, researchers, device manufacturer employees, non-profit and foundation members, and government representatives, including the U.S. Food and Drug Administration (FDA) and the National Institutes of Health (NIH). This workshop provided a forum for kidney patients to voice their concerns and preferences about their treatments in order for patient feedback to be included in various phases of future medical device development.

Throughout the one and a half day workshop, there were 14 presentations encompassing topics such as the importance of patient preferences and how they are measured, patient assistance in the development of new medical devices, patient involvement in clinical trials, and the patients’ roles in improving devices already on the market. Five panel discussions allowed attendees to pose questions to the speakers. Meeting participants also attended three breakout sessions to explore the concepts, ideas, and challenges outlined by the speakers.

Highlights

• Patients were eager to communicate their preferences, and many shared innovative ideas for device development.

• Patients gained perspective on the importance of being proactive to improve their care. This includes communicating effectively with their healthcare providers (HCPs), being active and inquiring participants in clinical trials, and communicating their questions or concerns to the FDA.

• Patients noted that it would also be important for payers, such as the Centers for Medicare and Medicaid Services (CMS), to understand the patients’ perspective.

• Participants were enthusiastic about continuing to be a part of the discussion, after the workshop, and recommended the use of an online forum where patients, researchers, FDA and industry can engage.
Recommendation

Based on the various discussions during the workshop and follow-up conference calls, the workgroup has identified the following potential post-workshop opportunities for KHI:

- Review this meeting summary to determine:
  - Additional activities as suggested by the attendees,
  - How the summary can support the development of 1-2 white papers, and
  - If the meeting summary can be packaged and sent to various constituent groups, posted online, or shared with the broader community.
- Develop a brief overview using the speakers’ slides for attendees (and others) to share the highlights of the meeting.
- Develop 1-2 white papers that focus on 1) the patient engagement exercise and lessons learned and 2) the content that was shared and strategies for moving the concept of incorporating patient preferences into medical device development forward.
- Request members of the KHI Patient and Family Partnership Council or attendees to submit articles on their experience to various organizations such as AAKP, NKF, PatientsLikeMe®, ASN, etc.
- Incorporate comments from the excellent evaluations into the post-meeting slide set, or other articles and newsletters.
- Assess industry’s perspective on the workshop and determine what additional activities can support their efforts to include patients’ preference in medical device development.
- Explore opportunities for patient centered billing and reimbursement.
- Understand HIPPA restrictions, both perceived and real, to determine if there is an impact on how HCPs, researchers and industry include patients in the device decision making processes.

Additional opportunities have been identified and will be discussed with the KHI Patient and Family Partnership Council. Such opportunities include assessing an online patient community, partnering with the dialysis organizations to support product development, and notifying CMS of the workshop and attendees interest in its participation in future discussions.
Purpose of the Project

The Center for Devices and Radiological Health (CDRH) at FDA has emphasized the importance of incorporating patient preferences into product development programs and into regulatory decision making, but the relevant stakeholders (including industry, patients, and regulators) have an imperfect understanding of best practices for creating the tools to capture and analyze data related to patient preferences. KHI convened a workshop where regulators, the device industry, and patient advocacy groups came together to discuss the barriers and solutions to developing the necessary tools to facilitate the incorporation of patient preferences into product development and regulatory decision making for medical products in kidney disease (including – but not necessarily limited to – dialysis, vascular access, and transplantation).

Prior to the workshop, the KHI workgroup responsible for hosting the event developed and launched a short animated video to engage patients and encourage them to attend educational webinars held in April 2015. The video featured CeCe (pictured right), a kidney patient looking to provide her input and become more involved in the regulatory and design process for medical devices in her healthcare. The video has been viewed over 1,750 times, resulting in more than 160 patients expressing their interest in attending the webinars (and 245 total registrants). View the video on the KHI website.

The webinars educated patients on the product lifecycle, the role of the FDA/CDRH, the CDRH Patient Preferences Initiative, and increased patients’ interest and knowledge before participating in KHI’s workshop. The webinar and slides can be viewed on the KHI website.

The workgroup has developed a short slide deck that summarizes the workshop and asks that attendees and interested parties share this information with their community (fellow patients at local dialysis clinics, patient groups, or community groups). View the downloadable and printable workshop on the KHI Website.
Understanding Patients’ Preferences:  
Stimulating Medical Device Development in Kidney Disease  
Wednesday, August 12 - Thursday, August 13, 2015

Faculty List:

1. John F. Bridges, PhD, John Hopkins Bloomberg School of Public Health
2. Terri Bonadio, BSHA, RN, CNN, Fresenius Medical Care
3. Robert Califf, MD, Deputy Commissioner for Medical Products and Tobacco, U.S. Food and Drug Administration (FDA)
4. Celeste Castillo Lee, Patient Advocate for the Vasculitis Foundation, KHI Board of Directors Member, Chair of KHI Patient and Family Partnership Council
5. Dolph Chianchiano, JD, MPA, National Kidney Foundation, KHI Workgroup co-chair, KHI Board of Directors Member
6. Paul T. Conway, Kidney Transplant Recipient, President of the American Association of Kidney Patients
7. Benjamin Fisher, PhD, Center for Devices and Radiological Health (CDRH), FDA
8. Jennifer Flythe, MD, MPH, FASN, University of North Carolina, Chapel Hill
9. Gema Gonzalez*, PhD, Center for Devices and Radiological Health (CDRH), FDA
10. Frank Hurst, MD, FASN, Medical Officer in the Center for Devices and Radiological Health (CDRH), U.S. Food and Drug Administration, KHI Workgroup Chair
11. Dalia Jacob, MD, Baxter Healthcare, Inc.
12. Terry Litchfield*, Liaison for the American Association of Kidney Patients and Dialysis Patient Citizens
13. Steve L. Morin, RN, BSN, Office of Health and Constituent Affairs, FDA
14. Carolyn Neuland, PhD, Center for Devices and Radiological Health (CDRH), FDA, KHI Board of Directors Member
15. Kathryn O’Callaghan, BSE, Center for Devices and Radiological Health (CDRH), FDA
16. Sally Okun, RN, MMHS, PatientsLikeMe®
17. Holly L. Peay, PhD, CGC, RTI International and Duchenne Connect Network, a program of Parent Project Muscular Dystrophy
18. Sam Pederson*, Liaison for the American Association of Kidney Patients, KHI Board of Directors Member
19. Lillian Pryor*, MSN, RN, CNN, Fresenius Medical Care, Liaison for the American Nephrology Nursing Association
20. David Pudwill*, PhD, Center for Devices and Radiological Health (CDRH), FDA
22. Anindita Saha, BSE, Center for Devices and Radiological Health (CDRH), FDA
23. Doug Silverstein*, MD, Center for Devices and Radiological Health (CDRH), FDA
24. Francesca Tentori, MD, Arbor Research Collaborative for Health
25. Melissa Threlkeld, MHA, FACHE
26. Linda Upchurch, MBA, MHA, NxStage Medical, Inc.
27. F.P. Wieringa, PhD, Delegated by the Dutch Kidney Foundation and Dutch Kidney Patient Association

*Indicates that the individual participated as a breakout session facilitator.
Summary of Presentations
Welcome & Opening Remarks
Frank Hurst, MD, FASN, Center for Devices and Radiological Health (CDRH), FDA

Dr. Hurst welcomed the attendees, thanked everyone for attending, and recognized those individuals who were involved in the development of this workshop. He introduced the Kidney Health Initiative (KHI) and outlined its mission statement: To collaborate among members of the nephrology community, to advance scientific understanding of kidney health, and to foster development of therapies for diseases that affect the kidney.

Patients and Their Government: Shared Expectations and Responsibilities Determine High Impact Engagement
Paul T. Conway, President of the American Association of Kidney Patients, and kidney transplant recipient

Mr. Conway, a patient and advocate for kidney disease, stated that the key challenge in the kidney disease community is that disease knowledge has improved, but treatment of chronic kidney disease (CKD) or end-stage renal disease (ESRD) has not improved. In addition, the costs to cover disability and unemployment benefits for kidney patients (and consequently taxpayers) is mounting, and these rising costs have led to concerns about the capability of the government to keep pace with healthcare innovation and meet patient demand. The forces that are driving healthcare innovation include applied medical and pharmaceutical research, new information technology, data analytics, remote medical monitoring devices, changes in laws and regulations, the promise of a more responsive government, and the social media impact of patients and care partners. With a range of forces driving healthcare innovation, there are many healthcare stakeholders, including the President of the United States, Congress, federal regulatory agencies, state governments, professional medical and research organizations, inventors, investors, manufacturers, think tanks and nonprofits, the media, patients, and care partners.

Mr. Conway urged the meeting attendees not to underestimate the power of social media, as it is a means by which patients’ voices can be heard, and this public voice cannot be ignored by policymakers. Mr. Conway encouraged patients to advocate for healthcare innovation, telling them to not think their insight is insignificant or that they will be ignored. Mr. Conway urged everyone to take advantage of opportunities through KHI, the FDA, and other patient advocacy organizations.
Opening Remarks
Robert Califf, MD, Deputy Commissioner for Medical Products and Tobacco, FDA

Dr. Califf discussed how the FDA faces a difficult balance between fostering innovation and protecting patients. In the past, health care professionals led patient care. Today, patients have more access to medical information and take a more active role in their personal healthcare. Patients are now strong advocates for driving new developments in medicine to treat their diseases. Dr. Califf used the multiple myeloma community as an example how patient engagement drives innovation. The patients and researchers are involved in a joint effort to develop new treatment options.

Panel Discussion
Paul T. Conway; Robert Califf, MD
Moderators: Dolph Chianchiano, JD, MPA, National Kidney Foundation, KHI Board of Directors Member; Frank Hurst, MD, FASN

The opening presentations sparked questions from the attendees with panel members providing the following responses:

The panel discussed how patient advocacy organizations should take the lead in developing interactive forums to reach as many patients as possible. Social media was described as an effective way to allow less vocal people to voice their opinions and gather patient preferences.

When providing greater insight on how multiple myeloma created a research network with patient involvement, it was noted that an obvious lack of consistency in therapy recommendations and an inspired patient caretaker helped drive the development of the network. In the current dialysis population, patients are seen regularly, but open, frank communication with HCPs may not be occurring. Patients and care partners need to demand improvements in treatment options, and not be complacent with their care.

The panel discussed how corporate policy can be changed and noted that while the FDA has little influence on what pharmaceutical and medical device corporations are developing; KHI and consortiums such as this patient preference forum has the potential to motivate companies to innovate and develop new treatment options.

Other general comments from the audience and panel:
- Patients with kidney disease have trouble being advocates because they often feel so sick and drained, and they feel as though they are victims of their disease.
- CMS should be involved in this initiative.
- Patients cannot forget that their nephrologists are there for them and that they have a right to an open and frank conversation about their treatments.
FDA CDRH Patient Preference Initiative
Kathryn O’Callaghan, Center for Devices and Radiological Health (CDRH), FDA

Ms. O’Callaghan reported that patient advocacy groups have sparked the evolution of patient involvement in healthcare decisions; patients are more informed and taking charge of their own treatments. The goal of the Patient Preference Initiative is to develop a systematic way of eliciting, measuring, and incorporating patient preference information throughout the total product life cycle, thus driving patient-centric device development. Patient perspectives and input are important during the device discovery process, trial design, the compilation of benefit-risk information for regulatory decisions, and the development of communications to patients regarding specific products approved by the FDA. As an example, patient preference was measured in an obesity study that helped identify favorable benefit-risk profiles for patients considering different treatment options. In addition, the FDA has developed a Patient Preference draft guidance (published in May 2015) to help guide patient involvement in the regulatory decision making process as well as other areas of the total product life cycle. Patient-reported outcomes (PROs) in device studies are also an important consideration and the use of PROs may increase as a result of patient-centric regulatory efforts. CDRH is committed to integrating patient voice into regulatory decision-making. Below is a summary of this presentation:

- Patients are at the heart of everything we do.
- Patients have unique perspectives about the value of benefits and impact of risks of medical devices
- CDRH is committed to integrating the patient voice into regulatory decision-making
- We have made significant progress in laying the groundwork for patient preferences in kidney disease.
- Patients, researchers, industry, FDA, and others have a role to play in driving more patient-centric device development, evaluation, and delivery.

The Science of Measuring Patient Preferences
John F. Bridges, PhD, Johns Hopkins Bloomberg School of Public Health

Dr. Bridges stated that regulators, clinicians, and patients routinely make decisions that trade safety for a desired clinical benefit. His research uses quantitative methods to systematically evaluate these benefit-risk trade-offs for new or existing medical interventions. These approaches are especially useful in situations in which there may be disagreements on benefit-risk profiles (e.g., when patients see certain outcomes as entailing a bigger risk than clinicians do or vice versa). He highlighted some relevant examples from his research and noted some of the specific methods that could be used to survey patients in order to quantitatively assess patient preference including discrete-choice experiments or best-worst scaling.

Patients’ Preferences in Dialysis: What Do We Know?
Francesca Tentori, MD, Arbor Research Collaborative for Health

Dr. Tentori discussed the challenges and importance of assessing patients’ preferences in dialysis. There is an association between quality of life and mortality, and how a patient feels is the most important predictor of therapeutic outcome. Even when patient input is obtained in the current landscape, annual questionnaires are burdensome, and are often only completed by patients who are feeling well enough and are willing to invest the time. Thus, there is a need for more frequent and patient-friendly assessment of quality of life. What HCPs view as important for dialysis patients may not be the same as what patients deem important. Understanding these differences will help improve patient treatment.
Innovation Forum

Moderators: Celeste Castillo Lee, Patient Advocate for the Vasculitis Foundation, KHI Board of Directors Member, and Chair of KHI Patient and Family Partnership Council
Melissa Threlkeld, MHA, FACHE, CKD Patient, Austin, Texas

During the Innovation Forum, several members of industry used examples of existing products or products in development to present on how patient preferences could be used to enhance their product development process.

- **NxStage Medical Inc.** Presented Nx2me™, which is an iPad app used by patients to connect with nurses through a web-based portal. Through this app, nurses monitor patient well-being and intervene quickly, if necessary. NxStage also presented their home nocturnal HD system, and dual lumen needle that is designed to allow for single-needle dialysis.

- **W.L. Gore & Associates**, the makers of Gore-tex®, is developing an improved vascular graft that has a prolonged life, requires fewer repeat interventions, and results in fewer disruptions in dialysis schedules.

- **Debiotech** has developed a new home PD system. This system has many features such as a smaller size and weight, and a video conference feature that allows patients to communicate with nurses. Debiotech is also developing a home HD system that will incorporate patient feedback into the final design.

- **AWAK Technologies** is developing a wearable PD system that will allow patients to be mobile while undergoing automated PD.

- **Humacyte Inc.** is developing a human-derived graft that has the potential to lower risk of infection and clotting.

- **Blood Purification Technologies, Inc.** is developing a wearable artificial kidney that will more closely mimic native kidney function compared with regular intermittent hemodialysis. Be sure to check out their Facebook page.

- **The Kidney Project** is developing an implantable renal assist device that would function as an artificial kidney. Be sure to check out their Facebook page.
Engaging Patients in the Development of Medical Devices
Linda Upchurch, MBA, MHA

Ms. Upchurch stated that patient advocacy does not mean patients advocating only for policy changes, it also refers to patients speaking up for what they want and need in the health care setting. HCPs also need to ask patients what they find important. Engagement in the kidney community helps patients to know that they are not alone in their situation, and when patients exchange information they learn about other treatment options that may better fit their lifestyle.

Determining Acceptable Benefits and Risks of Emerging Therapies: A Patient Community’s Approach
Holly L. Peay, PhD, CGC, RTI International and Duchenne Connect Network, a program of Parent Project Muscular Dystrophy

Dr. Peay described a pilot study conducted by the Parent Project Muscular Dystrophy group to determine what patients and care partners consider to be a meaningful benefit of treatment and to determine how much risk patients are willing to accept to obtain that benefit. They found that patients are willing to tolerate a good amount of risk to receive even just a moderate benefit. Based on this research, the disease community developed a draft guidance, in partnership with the FDA, on Duchenne Muscular Dystrophy. Dr. Peay stated that measuring patient preferences in this manner helps drive the FDA to improve their benefit-risk framework. However, these quantitative approaches do not replace patient testimony, and so patients were encouraged to get their voices heard in any way possible.

First Things First! Prioritizing Patient Preferences
F.P. Wieringa, PhD, Delegated by the Dutch Kidney Foundation and Dutch Kidney Patient Association

Dr. Wieringa provided a brief description of the Dutch Kidney Foundation and the Dutch Kidney Patient Association and how these organizations are encouraging efforts to incorporate patient preference in treatments. Dr. Wieringa discussed how the Dutch Kidney Foundation has created an interactive app to manage the dietary needs of kidney patients. The Dutch Kidney Foundation’s NeoKidney Development’s joint venture with Debiotech of Switzerland and AWAK of Singapore was also highlighted as a way of incorporating patient input into the device design process. Their agreement to complete a functional model in 2015 will deliver a compact dialysis machine that patients can use themselves directly at home.

Panel Discussion
Linda C. Upchurch, MBA, MHA; Holly Peay, PhD, CGC; F.P. Wieringa, PhD

Great interest was generated among attendees in learning how the muscular dystrophy group got
started and how they were able to get patients involved. Dr. Peay described how the organization wanted to stay on top of the new FDA patient-focused drug development initiative, and that as a less common disease state, they knew that they would have to push for FDA attention. The patient advocate community played a large role in getting patients involved to take the Parent Project Muscular Dystrophy survey.

There was some discussion on the need to educate the public about kidney donation through increased advocacy efforts. Advocacy efforts to prolong the life of a donated kidney and the timeframe for the recipient to receive the donated kidney are also needed.

The role of technology in medicine was discussed, including the use of apps that get patients more involved in their treatment and assist in the monitoring of their diet. However, it was noted that not all kidney patients are technology savvy, and that there should also be efforts to include those patients.

**Breakout Discussions**

The workshop featured breakout sessions that facilitated small group discussion. The individuals that served as facilitators during the breakout discussions are listed with the workshop’s presenters in Appendix B.

**In ESRD care, what are some gaps that new devices may help?**

Patients and care partners had many innovative ideas for new devices and changes to current devices that may improve ESRD care. Suggestions included:

- Patient ability to track phosphorus, potassium, and albumin levels at home and more frequently than just at their monthly appointments.
- A water-proof bag that could cover an access port to allow patients to shower.
- Automation and wireless connectivity that:
  - Allows dialysis machines to automatically transmit data to dialysis centers (e.g., blood pressure)
  - Includes an anemia management function
  - Allows automation of dry weight measurement for home
  - Includes visible gauge denoting how much time remaining on PD cycler
- Improved safety functions that include:
  - Built-in battery backups in the event of a power outage
  - Mechanical blood return in the event of power outage (the current “manual rinse back” feature is not user-friendly)
  - Alarms that sound for any type of leak, including an alarm on the needle
- An app to help troubleshoot alarms.
- Remote monitoring for patients on home HD.
- Improved portability (e.g., reduced size, TSA-compliant case, and retractable cord for PD).
- Single needle options for home HD therapy.
How can device developers obtain input from patients and care partners for design issues?

The breakout groups agreed that a multifaceted approach is needed to obtain input from patients and care partners on device design. It was noted that a large proportion of patients with ESRD do not have Internet access. Therefore, more traditional approaches to obtain input should continue to include written surveys by mail or oral surveys by telephone, in-person focus groups, and face-to-face discussions in the clinic. It was also pointed out that many patients are not aware of the variety of new technologies and treatment options available to them. Suggestions for improved awareness included:

- Encouraging kidney organizations to provide more patient education and resources about newer technologies.
- Providing education for patients to prompt them to be actively involved in their treatment plans (e.g., providing talking points to help patients understand what treatment option is best for them).
- Creating a clearinghouse for kidney-related resources, including newer technological advances, ongoing clinical trials, and education for all stakeholders (i.e., the government, HCPs, patients, associations, industry, dialysis centers).
- Removing or adjusting compliance restrictions or regulations that impede patients from engaging with industry or research organizations.
- Surveying patients to ascertain how new or improved products could minimize suffering.
- Expanding physician education regarding home HD.
- Providing a resource for patients to use to find HCPs who may be more open to newer treatment options.

It was noted that there should be a process for dialysis facilities to meet with dialysis patients to continually discuss how to improve patient experience, patient education, and patient involvement in the design of innovative devices. Finally, patients agreed that there needs to be more patient-focused insurance reimbursement, which may provide better access to treatment modalities.

The camaraderie overall was really heartwarming. I not only met people who are coping with the same issues that I have, but have formed an additional network of support to help me cope with what I am going through... This was truly an amazing conference and I am looking forward to the next one. Thank you.

M. Howard, Patient

What function or components of medical devices are important to patients and care partners?

Patients desired better overall delivery of care and access to technology. Patients perceive that they are denied access to treatment options because their nephrologist or dialysis center does not support a specific treatment modality. From patients’ perspective, the use of home HD is commonly denied for this reason, and many agreed that large dialysis centers should include home HD as an option. Patients agreed that the reimbursement model needs to change so patients receive the best care available. Further, patients voiced the need for alternative insurance reimbursement strategies for new technologies or treatment modalities as soon as they are approved for use.
Patients want technology that allows them to monitor and manage their own care, with the hopes of reducing office visits. Patients believe that if they have the ability to monitor their laboratory values more frequently than just at their monthly office visits, they will be more in charge of their treatment, allowing for improvement in their own care. Patients also want improved communication with their care team and better “customer service” for office visits (e.g., not 15-minute visits because of insurance limits).

Session 2: How can patients and care partners help ensure the success of future clinical trials?

Moderators: Jennifer Flythe, MD, MPH, FASN, University of North Carolina, Chapel Hill
Celeste Castillo Lee, Ann Arbor, Michigan

Clinical Trials in Kidney Diseases: Where are all the Patients?

Jennifer Flythe, MD, MPH, FASN

Dr. Flythe stated it is more difficult to enroll patients with kidney disease in clinical trials in kidney studies compared with other disease states, in part because patients with stage 3 or stage 4 kidney disease are not always aware that they have kidney disease if they are not exhibiting any symptoms. She also noted that patients may be more interested in participating in studies that are addressing specific questions and outcomes that are more relevant to patients. Because of this, it would seem that we should be encouraging patients to be involved when designing the study. There are also ways in which active engagement with the patient populace can help improve the recruitment of patients into clinical trials, which are summarized below:
Patient Informed Clinical Trials
Sally Okun, RN, MMHS, PatientsLikeMe®

Ms. Okun spoke about PatientsLikeMe® and described it as a patient-powered social research network that brings patients who have similar disease states together to share information and experiences. She noted that in other disease areas, the PatientsLikeMe® network has been successfully used to identify and notify patients who are eligible to participate in clinical trials, to offer surveys to patients, and to provide a forum for developing potential patient reported outcomes (PROs).

Patient-Reported Outcomes (PROs) Instruments in Dialysis: Are We Measuring the Right Outcomes?
Jennifer Flythe, MD, MPH, FASN

Dr. Flythe described a patient-reported outcome (PRO) as “any report on the status of a patient’s health condition that comes directly from the patient, without interpretation of the patient’s response by a clinician or anyone else.”

PROs are used in research as endpoints to monitor symptoms and patient satisfaction. To properly evaluate a patient’s quality of life, many questions have to be asked because of differences in the interpretation of questions and definition of quality of life among patients. Patients need to be involved in developing the quality-of-life questions, and reevaluating questions later on to ensure that the proper outcomes will be measured. Dr. Flythe’s background presentations on PROs is summarized below:

Summary:
- PROs measure the status of a health condition directly from the patient
- PROs must be developed systematically with input from patients and undergo validity and reliability testing
- Health-related quality of life is a common PRO for dialysis clinical trials
- “Are current PRO measures adequate? Are we measuring the right information?”

Patient and Care Partner Engagement in Clinical Research

Celeste Castillo Lee

Ms. Lee, herself a hemodialysis patient, discussed organizations that foster the engagement of patients in clinical research. She is part of a program called the Patient Family Centered Care program at the University of Michigan, which creates partnerships between patients and families and faculty and staff to help improve patient/family experience, staff/faculty satisfaction, safety, clinical outcomes, and facility design and operational efficiency. Another group, the Patient Centered Outcomes Research Institute (PCORI) was created to fund research to provide the evidence needed to help patients and their care partners make informed decisions. PCORnet, developed through the Patient Centered Outcomes Research Institute, is a national patient-centered clinical research network that helps involve patients in research. Ms. Lee shared the lessons learned from engaging patients in clinical research:

Lessons Learned

- Patients can and want to be involved in the study pipeline
- Patients and Researchers
  - Often speak different languages
  - May need translators/facilitators until they learn to speak a common language
  - Negotiate a new type of relationship
- Both researchers and patients
  - Get better with practice
  - Need advice
  - May benefit from mentorship

Panel Discussion

Jennifer Flythe, MD, MPH, FASN; Sally Okun, RN, MMHS; Celeste Castillo Lee

It was noted that patients may need to change their point of reference on quality of life as their disease progresses (e.g., from CKD to ESRD, from dialysis to transplant). Because there may be multiple treatment modality transitions in the life of a kidney patient, it is important to perform longitudinal evaluations of quality-of-life assessments, which could be perhaps completed through programs such as PatientsLikeMe®. Patient advocacy groups and other organizations (KHI, AAKP, NKF, and PatientsLikeMe®) should collaborate in an effort to get more patients involved in the planning and implementation of clinical trials.

Breakout Discussions

Why do individuals choose to participate or not to participate in clinical trials?

One of the major barriers to participation in clinical trials is lack of awareness. Other concerns for participating in clinical trials included:

- Privacy issues
- Too many follow-up visits and loss of income when attending follow-up visits
- Fear of receiving a placebo
- Study recruiters lacking credibility and not presenting the trials to the patients in a way that make it seem desirable to participate (e.g., unable to answer patients’ questions)
- Use of outcome measures that are less important to patients
- Lack of feedback provided to patients after participating in trials
How can we increase patient involvement in clinical trials?

Patients want to know that their involvement in a trial was worth their time and that they made a difference. If patients were informed of the immediate and long-term benefits of participating in a study, they might be more likely to participate. Patients also want to know more about the benefits and risks of participating in a trial. If patients were asked by their healthcare professional (HCP) to participate, and the HCP could then explain the benefits and risks to them, they would be more likely to get involved than if they see a flyer in their HCP’s office. In addition, patients are not always aware of clinical trials. The ESRD Networks or patient advocacy groups could distribute information about clinical trials that are recruiting patients.

Patients find the extensive follow-up for clinical trials to be very demanding. They would like follow-up visits to be easier to schedule, particularly when they have to miss work for a visit. They would be more likely to participate in a trial if they had some sort of compensation (e.g., gas cards, paid parking, or monetary compensation) for follow-up visits. A compensation strategy would help ensure data is collected and keep patients from dropping out of a trial. Many kidney patients are on social security, rely on food stamps, or have no or limited income; therefore, it is difficult for them to participate in clinical trials without some form of compensation or benefit.

What study factors are important to patients and care partners?

Patients and care partners want to have all the information they need about a study before they participate. They want to understand what the time commitment will be, and they want to know that their time commitment is somehow appreciated, possibly with an incentive. Patients want to fully understand the risks and benefits involved in participating in a trial. They want to know that their private information will stay confidential. Finally, they want feedback on the study results.

What are some ways patients and care partners can help in the design of a clinical trial?

Patients are more than willing to provide input if they are asked. Patients suggested getting patient feedback at multiple stages in a trial, so either the trial could be adjusted or the feedback could be used to design better studies in the future. Patients want to make recommendations on primary and secondary endpoints, as well as provide input on benefits and risks of treatment options.

Are current patient-reported outcomes (PROs) adequate? Are we measuring the right information during a trial?

Current research studies may not measure what matters most to patients. There should be more of a focus on PROs related to cognitive decline, memory loss, and general well-being, as cognitive decline and memory loss are major issues for patients with kidney disease. Patients do not feel like they are properly educated on the full spectrum of symptoms of kidney disease. Patients also want to be asked about their well-being, in addition to the usual clinical parameters, which is a more accurate indicator of quality of life.

Patients stated that preferences can and should be assessed during clinical trials, and suggested that in addition to structured surveys, enrolled patients should have an opportunity to provide information on preferences not covered by a survey or to explain why they answered a survey question the way they did. Patients also stated they want to know the survey results and that information needs to be shared.
Patients also suggested that study participants be asked what risks they are willing to take to gain the benefit of an investigational drug/device at enrollment and throughout the study.

**Session 3: How can patients and care partners help with the decision to make a new device available as well as improve a device once it is on the market?**

*Moderators: Carolyn Y. Neuland, PhD, Center for Devices and Radiological Health, FDA, KHI Board of Directors Member; Terri Bonadio, BSHA, RN, CNN, Fresenius Medical Care*

**The Patient Voice and the FDA**

*Steve L. Morin, RN, Office of Health and Constituent Affairs, FDA*

Mr. Morin described the FDA patient network as a centralized resource where patients learn about the drug/device development process, attend webinars, sign-up to receive a patient network newsletter about FDA happenings, and find information about patient representative programs that incorporate patient voices into advisory committee discussion. This network is a great way for patients to learn more about the drug/device development process and learn how to become involved.

**FDA Regulatory Decision Making: Obesity Study as a Potential Tool**

*Anindita Saha, Center for Devices and Radiological Health, FDA*

Ms. Saha stated that the FDA conducted a pilot study to explore how patient preferences could be incorporated into the regulatory decision-making process(1). This study included patients with obesity who were considering different treatment options. The study helped to quantify the maximum risk they would be willing to take as well as the minimum clinical benefit that would be meaningful to them. The data collected in this study helped the FDA to draft a guidance on patient preference, with the goal of providing recommendations for including patient preferences information in future clinical trials and regulatory decisions. She concluded that it is important for patients to be involved in the regulatory decision-making process by talking directly to the FDA or participating in patient advocacy groups.

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"Excellent information, very helpful to the patients. Please do it again!"

K. Florence, Care Partner
Patient Impact on Benefit-Risk
Dalia Jacob, MD, Baxter Healthcare, Inc.

The benefit-risk ratio is the ratio of benefit of an action or therapy to its potential risks. This ratio can be improved by increasing the benefit or decreasing the risk of a certain therapy. Dr. Jacob stated that benefit-risk profiles are dynamic and need to be reviewed throughout the life cycle of a product. Mechanisms to identify and minimize risk include:

- Clinical trials
- Patient education
- Post-marketing surveillance
- Improved product design
- Clear drug/device labeling
- Risk Evaluation and Mitigation Strategies (REMS)
- Risk Management Plans (RMPs)

Panel Discussion
Steve L. Morin, RN; Anindita Saha; Dalia Jacob, MD

Patients were interested in learning more about the FDA patient network and how the FDA is using information gathered through the network. Patient voice is included when the FDA is voting on approval of drugs through patient representative programs and advisory committee discussions. Patients are also involved in the review of investigational new drugs and participate in patient representative conferences. It was noted that patients and providers do not always read the labeling for medical products, and that there is a need to ensure that everyone knows how to use medical products in order to maximize safety and effectiveness. While all physicians may not understand the intricacies of each device as described in the product labeling, someone on each patient’s healthcare team should be properly trained. Physicians are responsible for understanding the benefits and risks of treatment options, but may need to rely on other team members to assist patients with the specific details of device use.

Breakout Discussions

How can patients and care partners participate in the FDA process to “approve” a new medical device? What problems could arise?

Patients and care partners who participate in the device approval process need to realize that their opinions on device design do matter. Many patients do not know that the FDA has patient representatives or advocacy groups, nor are they aware of who to call at the FDA if they have concerns or opinions about a device. The FDA needs to establish more awareness of its patient-focused programs. Patients have recommended that the FDA create a patient portal that includes all the information a patient may need regarding treatment options, benefit vs risk analyses, available devices, and reporting of adverse events. The FDA needs to develop ways to communicate to patients using nontechnical language that is not perceived as “talking down” to patients.
What is the benefit vs risk and how do patients and care partners think about these topics?

Benefit vs risk analyses help foster the creation of a unique care plan that considers a patient’s values, home life, and goals. The analyses consider the patient’s support system and how something unconventional might be a viable resource. Patients find benefit vs risk analyses to be very subjective and vary depending on patient characteristics or even on the patient’s current situation. Thus, it is important to frequently evaluate a patients’ willingness to take a risk for a certain treatment benefit.

Using the idea of benefit/risk and a medical device label, how will patients determine what medical device is right for them?

Patients have discussions with their HCPs and other patients to determine what medical device is right for them. Often, they use trial and error to find the best device for their needs and preferences. Patients would like to see dialysis centers provide information about all device types, even if they are not provided at that center, so that patients can make educated decisions. The group recognized that the centers may not support this idea as patients may choose a treatment option that is not available at the center. Patients believe that patient-centered insurance reimbursement would eliminate this difficulty, and possibly reduce overall healthcare costs, increase efficiency, and perhaps even promote improved HCP-patient engagement.
Closing Remarks
Celeste Castillo Lee

The purpose of this workshop was to begin a dialogue with patients about medical device development and to educate patients on how their preferences could be included. The participants were very engaged and contributed to a lively discussion. This meeting summary highlights many of the ideas and suggestions from patients. In addition to the ideas communicated in the breakout sections, following are some additional “takeaways”:

- We should explore ways to involve CMS in a similar discussion, so the agency has a better understanding about the patients’ perspective and can consider patient-centered billing and reimbursement.
- At this and future workshops, there needs to be more discussion on transplants, including drugs for transplants, keeping transplants viable longer, and education for potential organ donors.
- Perceived HIPAA (or privacy) implications are keeping HCPs, researchers, and industry from being more involved with patients.
- Communication is extremely important: between HCPs, patients, and the FDA. We need to figure out ways to streamline this communication.
- Education = empowerment.
- Patient involvement in clinical trials is extremely important to ensure that patient values are expressed and incorporated in studies.
- There needs to be an online forum developed for engaging the kidney community (patients, researchers, FDA, industry) to carry this conversation forward.

The workshop achieved the goals of the organizing committee and then some! The challenge will be to build on the interest that was demonstrated for increased communication and collaboration between patients and family members, industry, and federal agencies to stimulate innovation.
D. Chianchiano, JD, MPA, KHI Workgroup Co-Chair, KHI Board of Directors Member

Additional Resources
Additional resources identified throughout the workshop and subsequent discussions can be found in Appendix C.
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Appendices
Appendix A

Understanding Patients’ Preferences: Stimulating Medical Device Development in Kidney Disease
Wednesday, August 12 - Thursday, August 13, 2015

Attendees by Stakeholder Group:

Care partner/Family member: 11
Government: 13
Industry: 11
Nephrologist: 5
Non-profit/Foundation: 6
Nurse: 4
Patient: 49
Scientist: 5
Other: 5

Total Number of Attendees: 109

Attendees by Geographic Location:

Midwest: 14
Northeast: 15
South: 44
West: 12
International: 1
N/A: 23

Total Number of Attendees: 109
Breakdown of Patient Attendees:

CKD (Pre-ESRD): 5
Hemodialysis: 12
Home Hemodialysis: 8
Peritoneal Dialysis: 8
Rare Kidney Disease: 4
Transplant: 12

Total Number of Patient Attendees: 49
Appendix C

Understanding Patients’ Preferences: 
Stimulating Medical Device Development in Kidney Disease 
Wednesday, August 12 - Thursday, August 13, 2015

List of Additional Resources:
The workshop focused on how patients’ preferences can be measured and used when developing new medical devices. The following links were highlighted as additional resources at the workshop.

1. FDA Patient Network
2. FDA Brings Patients Into the Process
3. FDA: Opportunity to Comment on Current FDA Draft Guidances
4. FDA Sponsored Public Meetings
5. National Institute of Diabetes and Digestive and Kidney Diseases
6. American Association of Kidney Patients
7. National Kidney Foundation
8. Vasculitis Patient Powered Research Network
9. PatientsLikeMe®
10. Medical Device Innovation Consortium’s Patient Centered Benefit-Risk Assessment
11. PCORnet, the National Patient-Centered Clinical Research Network
12. “Patient Uprising: With technology as a tool, more people are taking the lead when it comes to their own health care”, Kendall Morgan, American Way Magazine (May 2015)
13. FDA Draft Guidance - Patient Preference Information