Integrating Patient Preferences into Regulatory Decision Making to Advance Innovation in Kidney Disease

August 5, 2020
Welcome

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Health Services Advisory Group
ESRD Network #17
The National Forum of ESRD Networks
National Kidney Foundation
Public Policy Committee Chair
Kidney Advocacy Committee Chair
• Understand how the FDA uses patient preference information (PPI) in regulatory decision making

• Learn about KHI’s PPI project
  • How KHI members can help
What is patient preference information (PPI)?

- Qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions.

- PPI captures the value that patients place on aspects of the medical device in a way that accounts for differing perspectives on benefits and risks that come with using that device or treating the condition.

\[ PPI \neq PROM \text{ (patient-reported outcome measure)} \]
• **Patient preference studies**
  
  • Research methods that produce estimates of the relative desirability or acceptability of different aspects (“attributes”) of a new device to patients
  
  • Example attributes:
    
    • **Potential Device Benefits**
      • Fewer blood pressure medications
      • Fewer dietary restrictions
      • More dialysis-free time (more freedom)
    
    • **Potential Device Risks**
      • Higher risk of infection
      • Mechanical complications of the device
FDA-KHI PPI Project: Integrating Patient Preferences into Regulatory Decision Making to Advance Innovation in Kidney Disease

- **Overall objective:** Develop a sustainable strategy for collecting patient preference information from a representative sample of dialysis patients to drive patient-centered innovation in dialysis devices.

- **Specific objectives:** Develop a pilot patient preference survey that will serve as a prototype of similar surveys to be administered in the future.
  - Survey development and administration
  - Infrastructure development (sustainable process for administration)
• **KHI Project Plans (3-years)**
  
  • Highly collaborative with FDA
  
  • **Project leadership:**
    
    • **KHI:** Jenny Flythe, Ray Harris, Melissa West
    
    • **FDA:** Michelle Tarver, Murray Sheldon, Carolyn Neuland, Frank Hurst, Ani Saha, others
    
    • **Nephrologist advisors:** Kerri Cavanaugh and Mark Unruh
    
    • **Patient advisors:** Derek Forfang, Dave White, Caroline Wilkie
    
    • **Research partners:** RTI International and MIT
  
  • **Scope:** PPI re: wearable dialysis devices (HD or PD)
    
    • Selected because more “near-term” and immediately useful to the FDA
HOW FDA USES PATIENT PREFERENCE INFORMATION (PPI) IN REGULATORY DECISIONS

MICHELLE TARVER, MD, PHD
Where is Patient Input Useful in the TPLC?

- Human-Centered Design
- Human Factors
- Patient-Informed Needs
- Patient-Reported Outcomes
- Benefit-Risk Information
- Communicating Benefit-Risk Information to Patients
- Patient-Centered Outcomes
Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approval and De Novo Classifications

Guidance for Industry and Food and Drug Administration Staff


Factors to Consider in the Evaluation of Benefit-Risk in Medical Devices: A Framework for Industry, Regulatory Agencies, and Other Stakeholders

Guidance for Industry and Food and Drug Administration Staff

Document issued on December 27, 2016.

Factors to Consider When Making Benefit-Risk Determinations for Medical Device Investigational Device Exemptions

Guidance for Investigational Device Exemption Sponsors, Sponsor-Investigators and Food and Drug Administration Staff


Consideration of Uncertainty in Making Benefit-Risk Determinations in Medical Device Premarket Approvals, De Novo Classifications, and Humanitarian Device Exemptions

Guidance for Industry and Food and Drug Administration Staff


For questions about this document, contact the Office of Device Evaluation at 301-796-5510.
Patient-Focused Guidance Documents

Guidance for Industry
Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)
December 2015
Clinical Laboratory


Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

This document will be in effect as of October 23, 2016.
The draft of this document was issued on May 18, 2015.

For questions about this document regarding CDER-regulated devices, contact the Office of the Center Director (CDER) at 301-796-7000 or Amadini Tanya at 301-796-2757.

For questions about this document regarding CBER-regulated devices, contact the Office of Communications, Outreach, and Development (OCOD) at 301-443-8358 or 301-443-1430.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research
Center for Biologics Evaluation and Research
Center for Devices and Radiological Health
Regulatory Impact Patient Preference Information

23 Industry-sponsored regulatory PPI studies completed or in pipeline
Advance research and regulatory science towards outcomes that are most important to patients

1) To inform clinical trial design

2) To inform FDA decision-making

Patient Preference Information Fills a Knowledge Gap

Product development
- Identify unmet medical need
- Understand what matters most to patients about their disease or treatment

Clinical trial design
- Inform endpoint selection
- Inform performance goal
- Inform sample size

Benefit-risk assessment
- Align priorities with what matters most to patients about their disease or treatment
- Understand patient perspective on benefit-risk tradeoffs
A New Collaboration to Move Clinical Trials from Generic alpha of 0.05 to Therapy-Specific Patient-Values

A new approach to designing and interpreting clinical trials

Developing and testing a method to incorporate patient perspectives on benefit & risk as an explicit means to set significance levels in clinical trial design

1. Identify the outcomes important to patients, family members, and caregivers

2. Design and conduct a patient preference assessment study

3. Design methods for clinical trials approval based on explicit patient input

4. Assess medical device stakeholder acceptance of clinical trial designs based on patient preference

http://mdic.org/pcor
Collaborations are Integral to Our Work

- Kidney Health Initiative
- Medical Device Innovation Consortium
- Academic Center
- Centers of Excellence in Regulatory Science & Innovation
- ISPOR, SMDM, ISOQOL
- Network of Experts
- CBER, CDER, and PAS
- Health Professional Organization
- Patient Organization
- MDEpiNet
- CDRH Patient Science & Engagement

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Kidney Health Initiative

Medical Device Innovation Consortium
Innovation from Patients for Patients
PROJECT PLAN

Carol A. Mansfield, PhD
• **KHI Project Steps**

1. Develop survey
   - Determine most important attributes and risks to study
     - Review of wearable devices under-development
     - Prior KHI patient priorities survey
     - Published data on patient priorities
     - New qualitative research (patient interviews)
     - Develop and pre-test survey with patients

2. Administer survey and analyze results

3. Plan for future iterations (develop infrastructure to support future collection of PPI)

4. Disseminate/ community-share
• **Develop survey**
  • Identify the key set of device features and risks to include in the survey
    • Understand the devices that will be coming up for FDA approval – features and risks
    • Understand patients’ priorities and the details that are important to their decision
  • Recently completed
    • Review of wearable devices under-development
    • Prior KHI patient priorities survey
    • Published data on patient priorities
    • New qualitative research (patient interviews)
• **Next step: Develop survey instrument**
  • Survey will contain brief text describing key features of devices and 2-3 risks
  • Respondents will be asked to choose between getting dialysis with a wearable device that has some risks and getting dialysis in a traditional dialysis center that has lower risks
  • Risks will be varied to understand the point at which the risks of a wearable device outweigh the benefits of the device to that individual relative to in-center dialysis

• **Pre-test survey with patients**
  • One-on-one interviews to go through the survey instrument in detail
PROJECT OVERVIEW

• **Administer survey**
  • Survey will be programmed online
  • Respondents will take the survey on a computer or tablet

• **Analyze results**
  • The data allow us to estimate the maximum level of risk respondents would accept to get the benefits of the wearable device, which we call maximum acceptable risk
  • We will estimate how the acceptable level of risk varies by people’s characteristics and experience
  • MIT will use the estimates of maximum acceptable risk in a model that could help inform the design of clinical trials for the wearable devices, specifically the level of uncertainty in the results that might be acceptable
PATIENT PERSPECTIVE

Derek Forfang
SUMMARY & WRAP-UP
• Project status
  • Background survey development work [complete]
  • Patient interviews [complete]
  • Survey question development [in progress]

• Survey implementation
  • Goals:
    • To reach diverse and representative populations
    • Build capacity for future surveys
  • Will rely on community support and partnership to achieve these goals
Virtual Upcoming CDRH Meetings

• **September 29**—Using Patient Preference Information in Medical Device Regulatory Decisions: Benefit-Risk and Beyond”
  - [https://www.ispor.org/conferences-education/conferences/upcoming-conferences/ispor-fda-summit-2020/about/registration-information](https://www.ispor.org/conferences-education/conferences/upcoming-conferences/ispor-fda-summit-2020/about/registration-information)

• **September 30**—Patient-Reported Outcomes (PROs) and Medical Device Investigations: From Conception to Implementation

• **October 22**—SAVE THE DATE—PEAC Meeting on Digital Health Topic
Monthly KHI Member Town Hall

Wednesday, September 2, 2020 4:00PM EDT