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

August 5, 2020

KHI KIDNEY HEALTH INITIATIVE

Welcome

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Co-chair KHI PPI Project
KHI Board Member
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CDRH
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RTI Health Solutions

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KHI PFPC
Health Services Advisory Group
ESRD Network #17
The National Forum of ESRD Networks
National Kidney Foundation
Public Policy Committee Chair
Kidney Advocacy Committee Chair

KHI KIDNEY HEALTH INITIATIVE

OBJECTIVES

- 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 ≠ PROM (patient-reported outcome measure)

https://www.fda.gov/about-fda/cdrh-patient-engagement/patient-preference-information-ppi-medical-device-decision-making

- **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

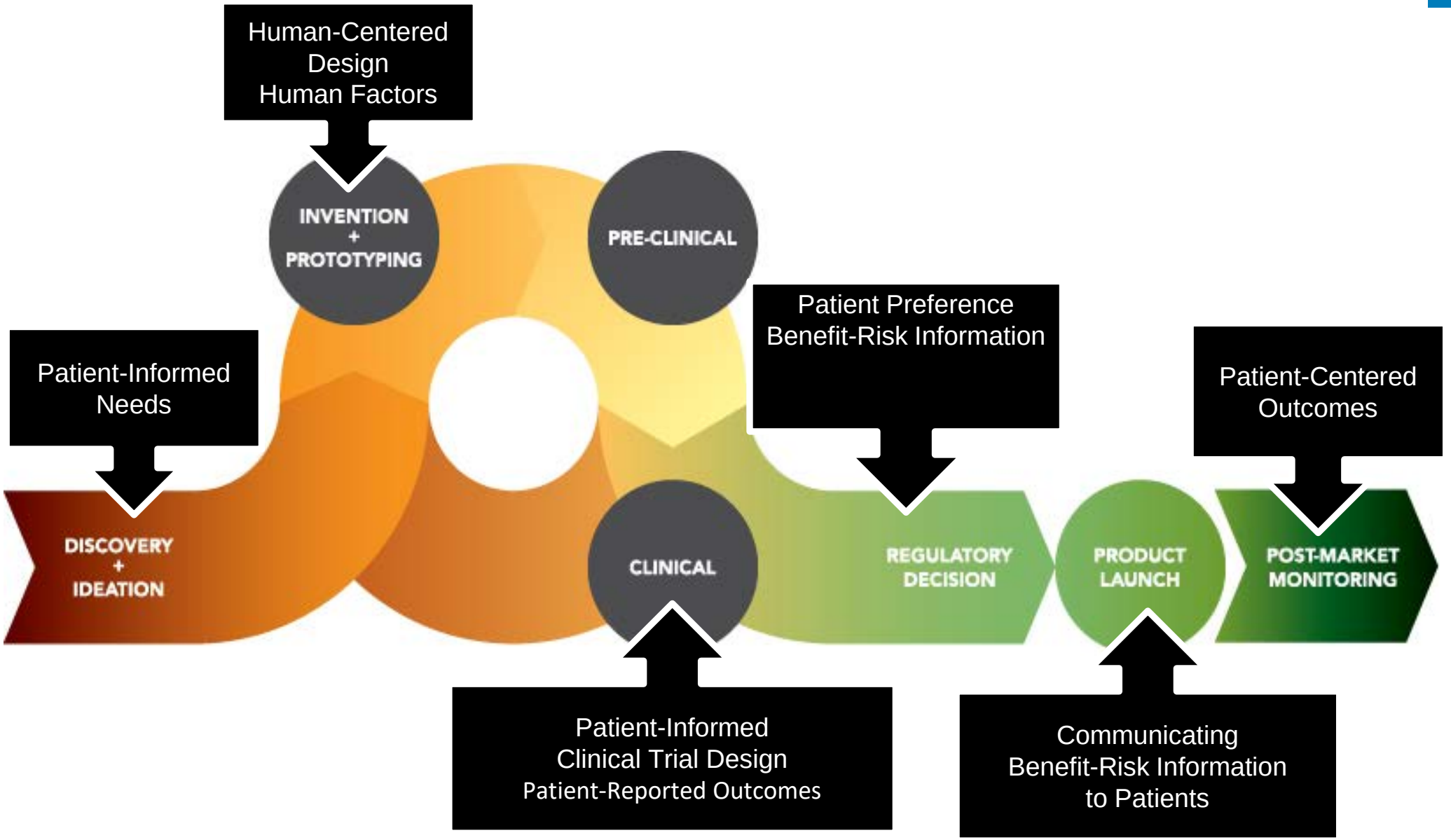
- **Specific objectives:** Develop a pilot patient preference survey that will serve as a prototype of similar surveys to be administered in the future
  - Survey development and administration
  - Infrastructure development (sustainable process for administration)

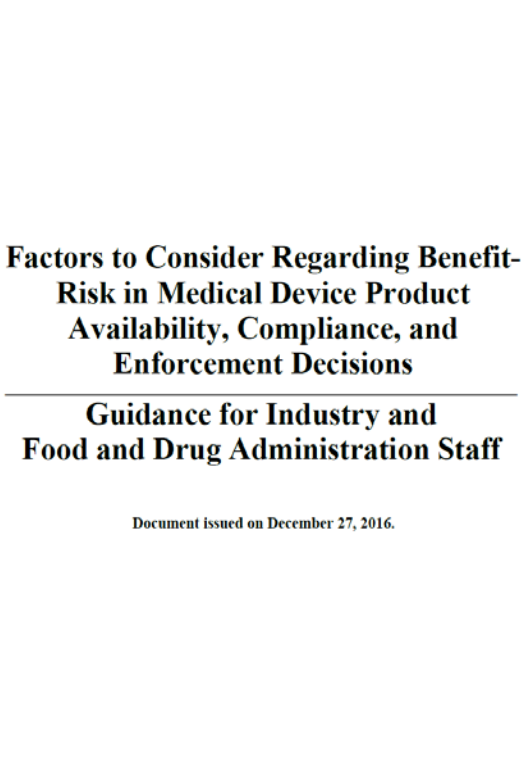
- # KHI Project Plans (3-years)
  - Highly collaborative with FDA
  - **Project leadership**:
    - **KHI:** Jenny Flythe, Ray Harris, Melissa West
    - **FDA:** Michelle Tarver, Murray Sheldon, Carolyn Neuland, Frank Hurst, Ani Saha, others
    - **Nephrologist advisors:** Kerri Cavanaugh and Mark 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

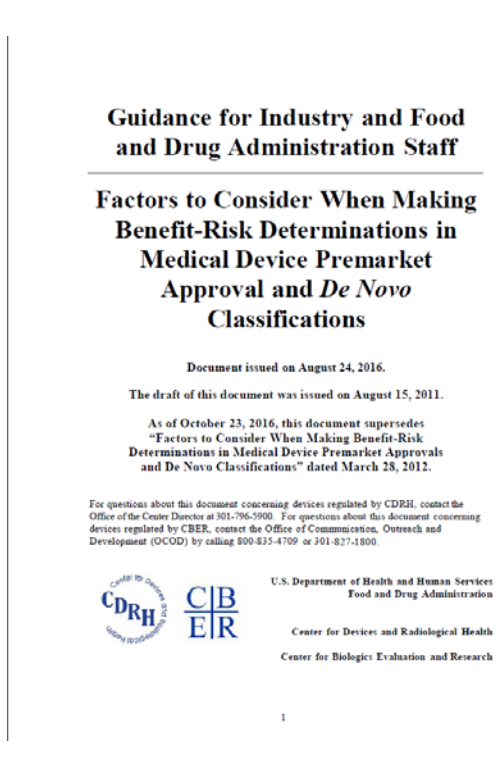
HOW FDA USES PATIENT PREFERENCE INFORMATION (PPI) IN REGULATORY DECISIONS

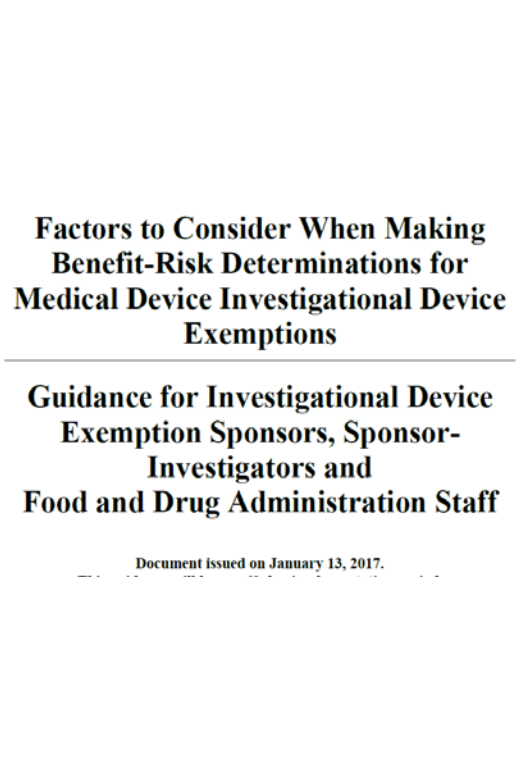
MICHELLE TARVER, MD, PHD

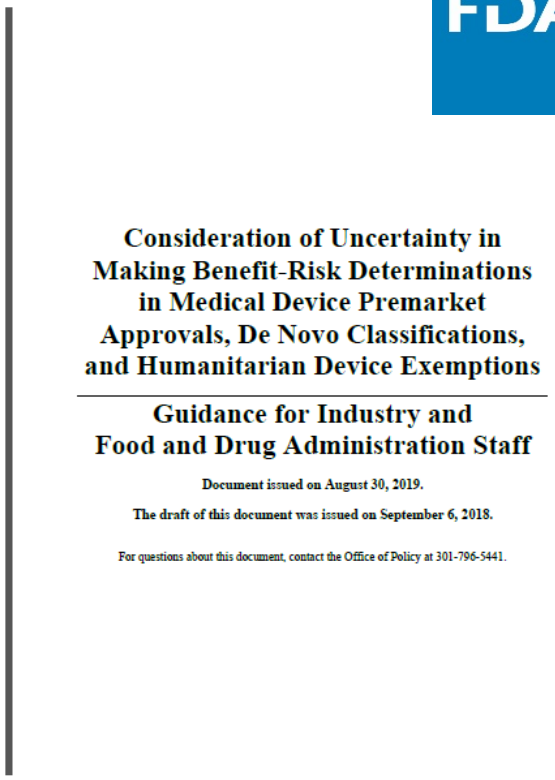
Where is Patient Input Useful in the TPLC?











Device Benefit-Risk Frameworks

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 2009
Clinical/Medical

Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and *De Novo* Requests, and Inclusion in Decision Summaries and Device Labeling

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

Document issued on August 24, 2016.
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 CDRH-regulated devices, contact the Office of the Center Director (CDRH) at 301-796-5900 or Anindita Saha at 301-796-2537 (Anindita.Saha@fda.hhs.gov)..

For questions about this document regarding CBER-regulated devices, contact the Office of Communication, Outreach, and Development (OCOD) at 1-800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration

Center for Devices and Radiological Health

Center for Biologics Evaluation and Research

Regulatory Impact Patient Preference Information

FDA News Release

FDA approves first-of-kind device to treat obesity

SHARE TWEET + EMAIL

For Immediate Release

January 14, 2015

Release

Español

The U.S. Food and Drug Administration today approved the Maestro Rechargeable System for certain obese adults, the first weight loss treatment device that targets the nerve pathway between the brain and the stomach that controls feelings of hunger and fullness.

The Maestro Rechargeable System, the first FDA-approved obesity device since 2007, is approved to treat patients aged 18 and older who have not been able to lose weight with a weight loss program, and who have a body mass index of 35 to 45 with at least one other obesity-related condition, such as type 2 diabetes.

BMI, which measures body fat based on an individual's weight and height, is used to

23 Industry-sponsored regulatory PPI studies completed or in pipeline

NxSTAGE

Aug 28, 2017

Previous Release

NxStage Medical Announces FDA Clearance for Solo Home Hemodialysis Using NxStage® System One™

First clearance of its kind gives trained NxStage patients freedom to dialyze without a care partner

LAWRENCE, Mass., Aug. 28, 2017 /PRNewswire/ -- NxStage Medical, Inc. (Nasdaq: NXTM), a leading medical technology company focused on advancing renal care, today announced that the U.S. Food and Drug Administration (FDA) has cleared its System One for solo home hemodialysis, without a care partner, during waking hours.

tusker smith&nephew

FDA NEWS RELEASE

FDA approves system for the delivery of ear tubes under local anesthesia to treat ear infection

For Immediate Release:

November 25, 2019

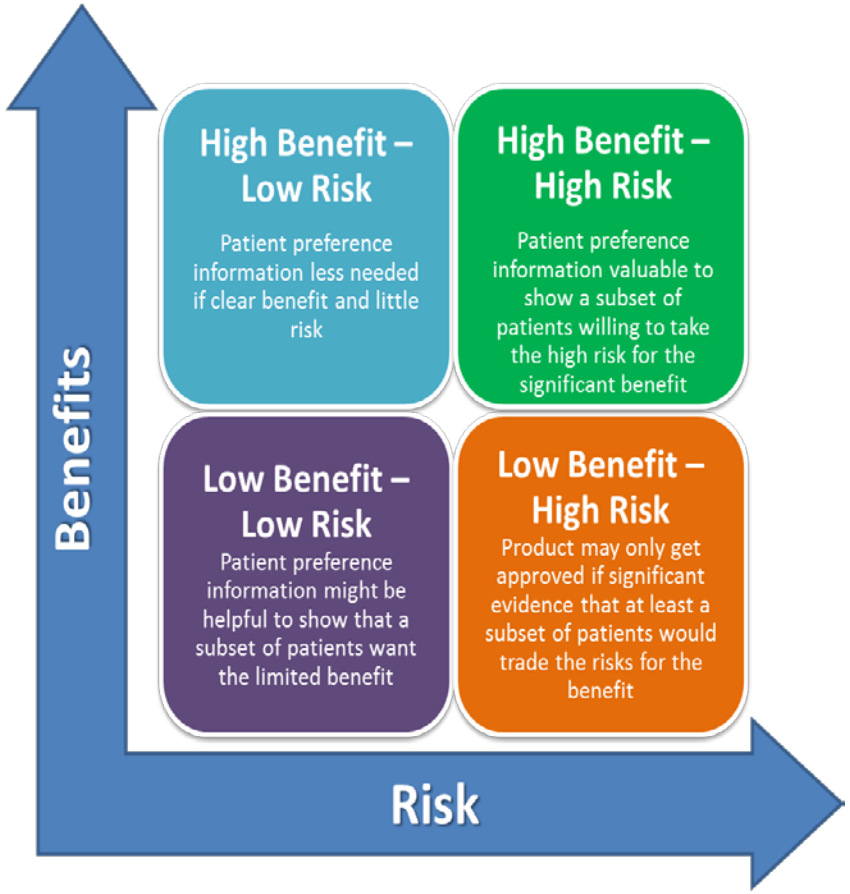
The U.S. Food and Drug Administration today approved a new system for the delivery of tympanostomy tubes, commonly referred to as ear tubes, that can be inserted into the eardrum to treat recurrent ear infections (i.e., otitis media). The Tubes Under Local Anesthesia (Tula) System is the first ear tube delivery system that can be performed in young children using local anesthesia in a physician's office setting. The Tula System consists of the anesthetic Tymbion, Tusker Medical tympanostomy tubes, and several devices needed for the delivery of the ear

Regulatory Impact

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



Ho M, Saha A, McCleary KK, et al. A Framework for Incorporating Patient Preferences Regarding Benefits and Risks into Regulatory Assessment of Medical Technologies. *Value Health.* 2016;19(6):746-750.

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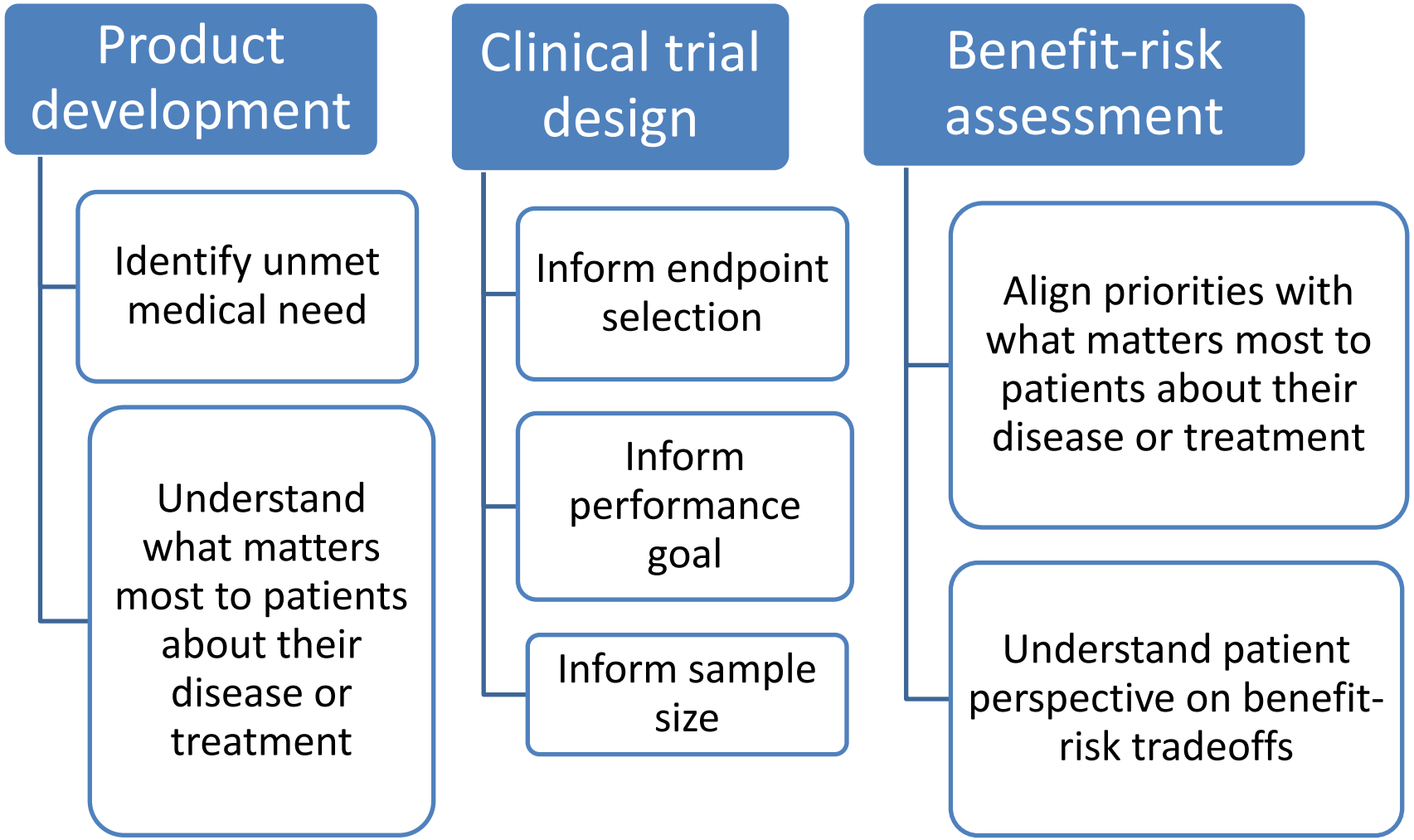
Patient Preference Information Fills a Knowledge Gap

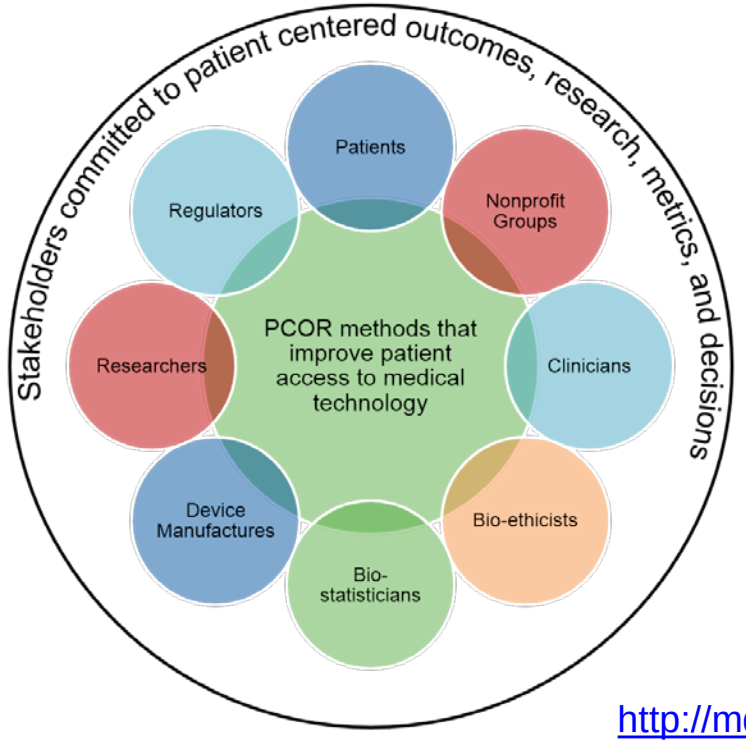


EXAMPLE

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

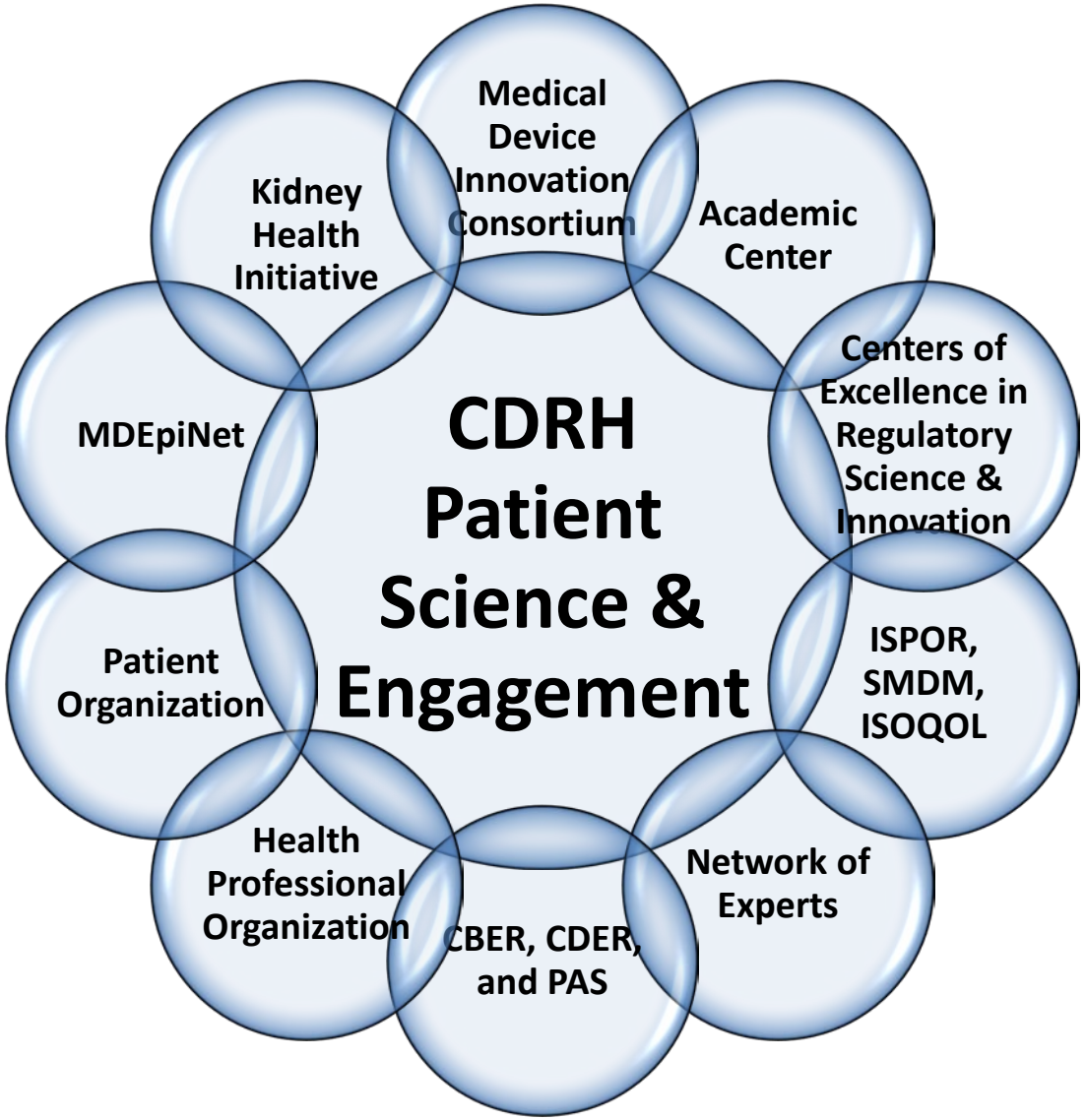


1. Identify the outcomes important to patients, family members, and caregivers
2. Design and conduct a patient preference assessment study
3. Design methods for clinical trials approval based on explicit patient input
4. Assess medical device stakeholder acceptance of clinical trial designs based on patient preference

MDIC MEDICAL DEVICE INNOVATION CONSORTIUM
Align › Achieve › Accelerate

http://mdic.org/pcor

Collaborations are Integral to Our Work



Innovation from Patients for Patients

PROJECT PLAN

Carol A. Mansfield, PhD

PROJECT OVERVIEW

- **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

PROJECT OVERVIEW

- **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)

PROJECT OVERVIEW

- **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

SUMMARY

- 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

Q & A

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
- **September 30--**Patient-Reported Outcomes (PROs) and Medical Device Investigations: From Conception to Implementation
  - https://www.fda.gov/medical-devices/workshops-conferences-medical-devices/virtual-public-meeting-patient-reported-outcomes-pros-and-medical-device-investigations-conception
- **October 22**—SAVE THE DATE—PEAC Meeting on Digital Health Topic

SAVE THE DATE!

Monthly KHI Member Town Hall

Wednesday, September 2, 2020 4:00PM EDT

KHI KIDNEY HEALTH INITIATIVE