Patient Preference Studies

Heather Benz, Ph.D.
External Expertise and Partnerships
Center for Devices and Radiological Health
US Food and Drug Administration

IVD Roundtable Meeting
November 29, 2017

Patients are at the Heart of What We Do

Center for Devices and Radiological Health Vision:
Patients in the U.S. have access to high-quality, safe, and effective medical devices of public health importance first in the world

Patient Preference

- Patient preferences are defined as 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
- Relevant preferences of care-partners (e.g., parents) and health care professionals may also be considered

Guidance: 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

What PPI Can Provide and How It Can Be Used

- PPI data can provide valuable information about:
  - Which benefits and risks are most important to affected patients
  - What benefit-risk tradeoffs are acceptable from the patient perspective
  - How do these patients think about these tradeoffs
  - Are there clinically-relevant subgroups of patients that would accept a particular benefit-risk profile and/or choose one treatment option over other alternatives

- Potential Uses of PPI:
  - Inform endpoints or effect size
  - Inform subgroup considerations
  - Labeling changes / expanded indications

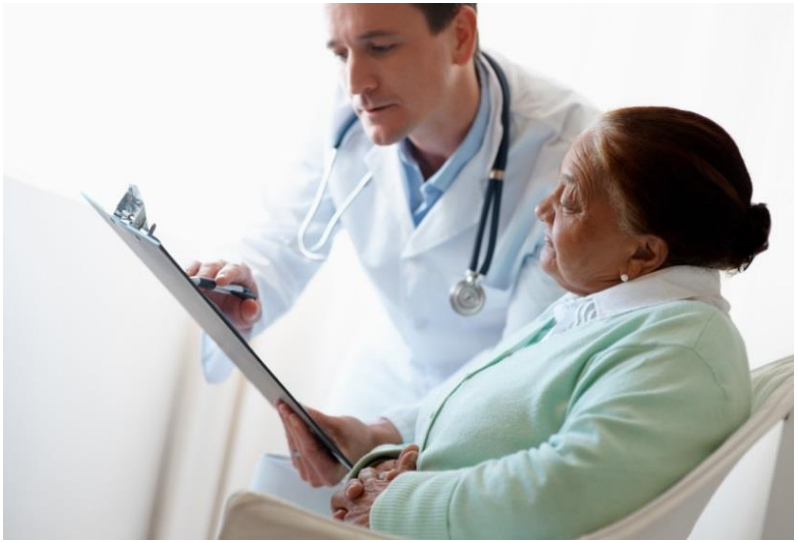
Recommended Qualities of Patient Preference Studies

Well-designed and conducted patient preference studies can provide valid scientific evidence regarding patients' risk tolerance and perspective on benefit. This may inform FDA's evaluation of a device's benefit-risk profile during the PMA, HDE application, and *de novo* request review processes.

A. All about Patients
   - Patient Centeredness
   - Sample Representativeness
   - Capturing Heterogeneous Patient Preferences
   - Comprehension by Study Participants

B. Good Study Design
   - Established Good Research Practices
   - Effective Benefit-Risk Communication
   - Minimal Cognitive Bias
   - Relevance

C. Good Study Conduct and Analysis
   - Study Conduct
   - Logical Soundness
   - Robustness of Analysis of Results



Regulatory Impact

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

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.

Examples: Patient Preferences in Diagnostics

- Positive predictive value and screening compliance
- Point of care testing
- Direct to consumer testing
- Prenatal testing
- Cancer screening

Submission of PPI to FDA

FDA encourages sponsors and other stakeholders to have early interactions with the relevant FDA review division if considering collecting and submitting PPI.

- Request an informational pre-submission meeting to discuss plans for designing or submitting a patient preference study
- Request participation from Martin.Ho@fda.hhs.gov and Anindita.Saha@fda.hhs.gov

Thank You

Heather Benz, Ph.D.
heather.benz@fda.hhs.gov


FDA
U.S. FOOD & DRUG
ADMINISTRATION