U.S. FOOD & DRUG ADMINISTRATION

NUMBERS OR NOISE:
INTERPRETING INTERNAL VALIDITY TESTS OF STATED-PREFERENCE DATA

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INTERPRETING INTERNAL VALIDITY TESTS OF STATED-PREFERENCE DATA

Moderator:

- **Kathryn O'Callaghan**, Assistant Director of Strategic Programs
  Center for Devices and Radiological Health
  U.S. Food and Drug Administration

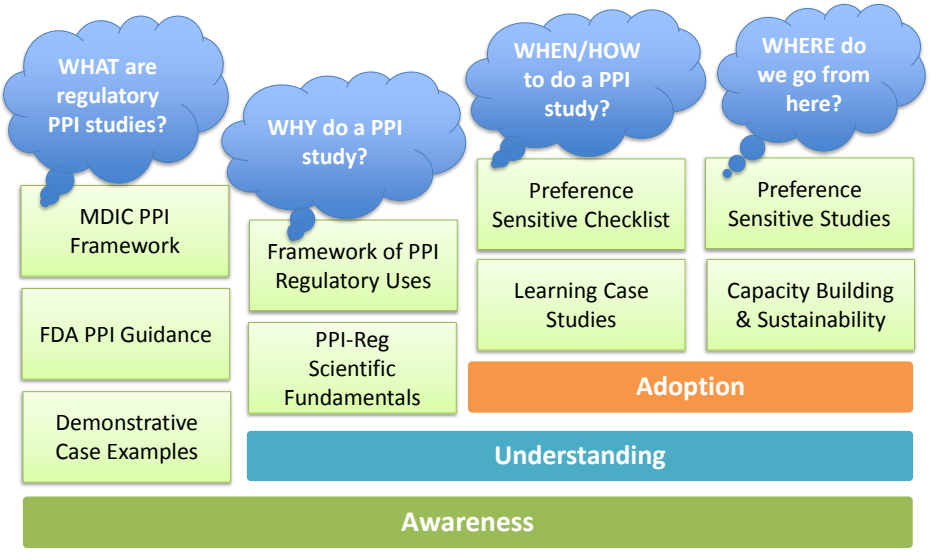
Panelists:

- **F. Reed Johnson**, PhD, Professor, Depts. of Population Health Sciences and Medicine
  Preference Evaluation Research Group
  Duke Clinical Research Institute

- **Kevin Marsh,** PhD, Executive Director, Patient-Centered Research
  Evidera Inc.

- **Jui-Chen Yang**, MEM, Research Economist
  Preference Evaluation Research Group
  Duke Clinical Research Institute

Dec. 2017 CERSI-FDA Workshop:
Advancing Use of PPI as Scientific Evidence for Medical Product Evaluation

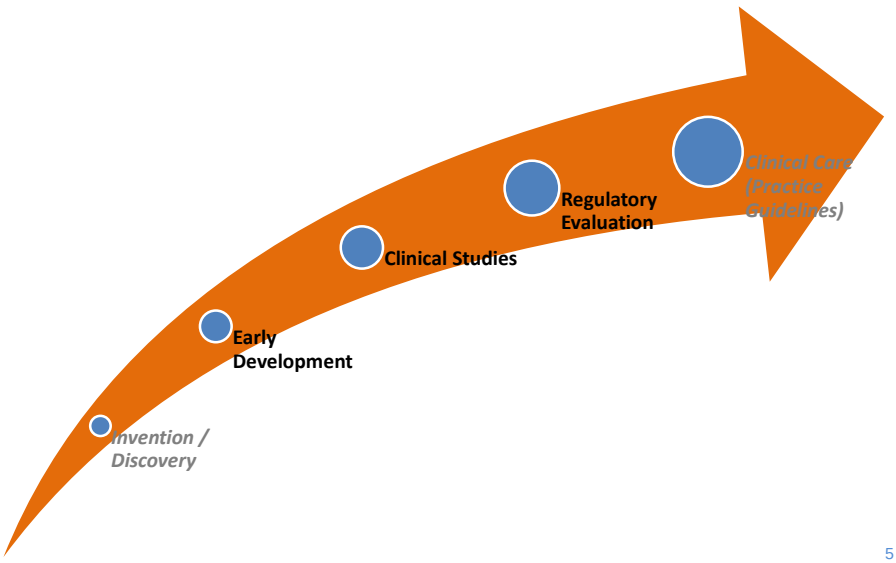


Begin with the End in Mind: How will this information be used?

Framework for Potential Uses of PPI in Medical Product Development

Development	Clinical Trial Design	Pre-Market Benefit-Risk Assessment	Post-Market
1. Identify unmet medical need 2. Understand what matters most to patients about their disease or treatment	1. Inform endpoint selection 2. Inform performance goal or effect size	1. Analysis of condition 2. Current treatment options 3. Patient perspective on benefit-risk tradeoffs 4. Population subgroup considerations	1. Inform interpretation of new data affecting benefit-risk assessment 2. Inform studies of new / expanded use populations 3. Communicate benefit-risk information to patients

Strength of Evidence Needed Depends on Context of Use



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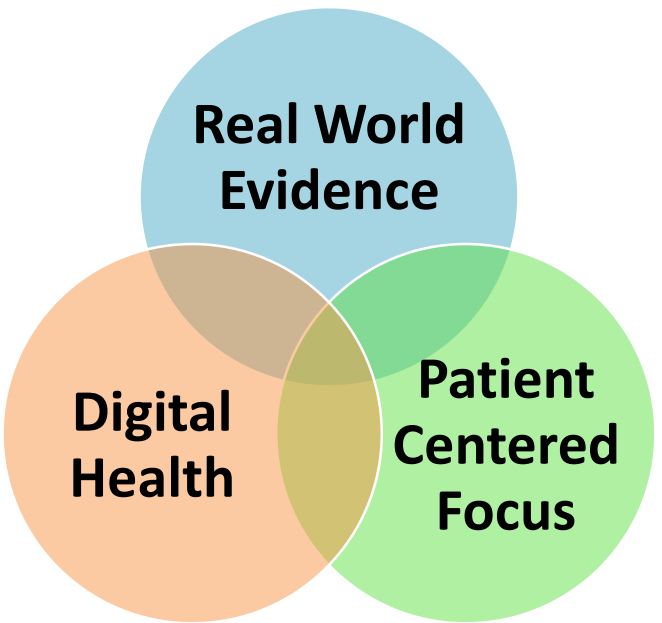

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Shared Goal

Improve patient health by better understanding patient needs, experiences and preferences

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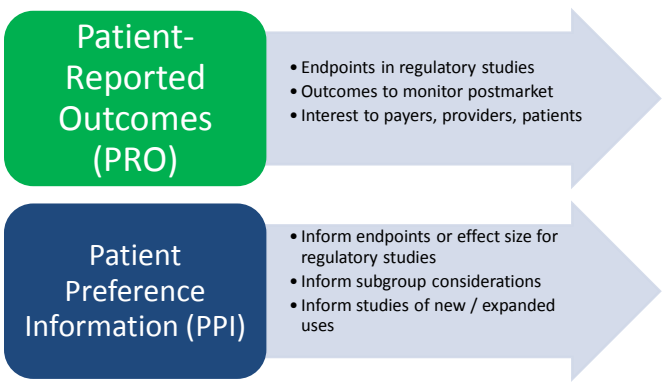
Value of Information from SPI Studies



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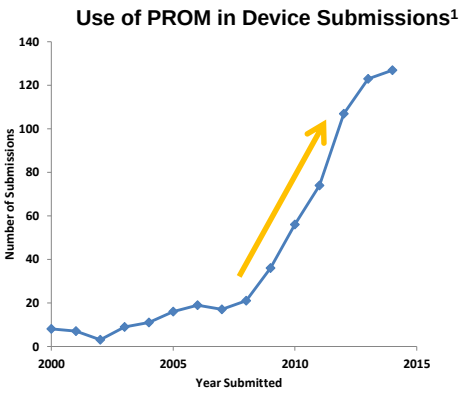
Significant Increase in Patient Perspective Studies

- **>500% increase** submissions with PROs
  (2009-2015)
- **>75% of clinical protocols** include PROs
  (FY17 pivotal study approvals)

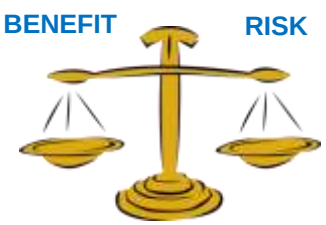


[1]*Submitted to CDRH as of FY2015*

How Patient Preferences Contribute to Regulatory Decisions for Medical Devices

Posted on **September 25, 2017** by **FDA Voice**

By: Jeffrey Shuren, M.D., J.D., Anindita Saha and Martin Ho, M.S.



- **Weight loss**
  - Patient-informed trial design
  - PMA approval
- **At home dialysis**
  - Patient risk tolerance
  - Expanded indication for solo at home use
- **Diabetes care**
  - Risk management for pediatric population
- **Ongoing studies**
  - Neurology
  - Oncology
  - Ophthalmics
  - Prosthetics
  - Women's health
  - Urology
  - Pediatrics

AUDIENCE PARTICIPATION

Thank You

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