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

Moderator:
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Panelists:
• F. Reed Johnson, PhD, Professor, Depts. of Population Health Sciences and Medicine
  Preference Evaluation Research Group
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• 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

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

- Invention / Discovery
- Early Development
- Clinical Studies
- Regulatory Evaluation
- Clinical Care (Practice Guidelines)

Real World Evidence

Digital Health

Patient Centered Focus
Shared Goal
Improve patient health by better understanding patient needs, experiences and preferences

Value of Information from SPI Studies

- **Patient-Reported Outcomes (PRO)**
  - Endpoints in regulatory studies
  - Outcomes to monitor postmarket
  - Interest to payers, providers, patients

- **Patient Preference Information (PPI)**
  - Inform endpoints or effect size for regulatory studies
  - Inform subgroup considerations
  - Inform studies of new / expanded uses
Significant Increase in Patient Perspective Studies

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

Use of PROM in Device Submissions

1Submitted to CDRH as of FY2015

How Patient Preferences Contribute to Regulatory Decisions for Medical Devices

- 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

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AUDIENCE PARTICIPATION

Thank You

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