Day 1 Wrap Up

FDA-CERSI Patient Preference Workshop
December 7-8, 2017

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Patients are at the Heart of What We Do
# Framework of PPI Uses 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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<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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<td>2. Understand what matters most to patients about their disease or treatment</td>
<td>2. Inform performance goal</td>
<td>2. Current treatment options</td>
<td>2. Communicate benefit-risk information to patients</td>
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<td>3. Patient perspective on benefit-risk tradeoffs</td>
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PPI: Science Meets Regulatory Needs
Relevance to diverse stakeholders

Patient
Development of patient-centric endpoints for clinical trials
Incorporation of the patient voice

Provider
Patient-oriented education to empower and help patients become a partner in their treatment decisions

Pharma
Design clinical trials that are meaningful to the lung cancer patient, leading to increased patient recruitment

Regulatory
Lay informational groundwork for patient-centered regulatory process
Preference Sensitive Areas

What makes a topic preference sensitive?

For which conditions might decision-making be enhanced by data from a patient preference study?

When does a patient preference study add value?

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Capacity Building
Toolkit for Appropriate Adoption of PPI for Regulatory Uses

WHAT are regulatory PPI studies?
- MDIC PPI Framework
- FDA PPI Guidance
- Demonstrative Case Examples

WHY do a PPI study?
- Framework of PPI Regulatory Uses
- PPI-Reg Scientific Fundamentals

WHEN/HOW to do a PPI study?
- Preference Sensitive Checklist
- Learning Case Studies

WHERE do we go from here?
- Preference Sensitive Studies
- Capacity Building & Sustainability

Adoption

Understanding

Awareness

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“The FDA’s work requires us to establish objective, consistent criteria on which we base our decisions. But ultimately, the criteria we use to judge benefit and risk turn on the parameters that matter to patients.

“Involving the end-user – the patient – in identifying health priorities and outcomes desired from health interventions is critically important.

“The bottom line is this: When assessing whether valid scientific evidence shows that a device’s probable benefit outweighs its likely risks, the FDA can also consider rigorous, systematically gathered patient preference information as a part of the totality of the evidence from clinical and nonclinical testing.”

- FDA Commissioner Scott Gottlieb, Oct 11, 2017
**Shared Goal**

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

- **Art of Patient Engagement**
- **Science of Patient Input**
- **Patient-Centric Health Care**
Thank You

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