Defining Good Evidence to Inform Decisionmaking and High Value Healthcare Services – An Industry Perspective

Kathy Buto
VP Health Policy
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Why defining good evidence matters to industry

• Can reduce uncertainty about acceptance of results of lengthy & costly studies on new treatments
• Can clarify how decisionmakers will assess the clinical value of treatments
• Will influence the nature of and investment in evidence development, both pre-approval and “real world”
• Will allow companies to distinguish significant innovation from more incremental innovation
PCORI’s Role in Defining Good Evidence

• Convening
• Transparency
• Methodological rigor
• Clarity around role of RCTs, observational studies, registries
• Developing translational tools for evidence
• Generating consumer understanding and insight in the use of evidence
• Trustbuilding among stakeholders
Two Issues

• Harmonization of Standards
• Communicating Findings
Harmonization of Standards

- Both regulatory agencies, e.g., FDA, and payers, e.g., CMS, are requiring comparative effectiveness studies
- But regulatory approvals require RCTs, with smaller numbers of patients
- While payers and reimbursement authorities want studies to assess benefits & risks in “real world” use
- Further, public & private payers may require different studies
- **Question**: Can harmonization of standards advance the conduct of CE studies?
Is Harmonization between Registration & Payer Studies a Good Idea?

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<th>PROs</th>
<th>CONs</th>
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<td>Reduces likelihood of duplicative studies and added cost of development</td>
<td>Could add requirements – increase, not streamline the total number of studies needed for registration</td>
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<td>Could lead to more predictable adoption &amp; diffusion</td>
<td>Could slow adoption &amp; diffusion</td>
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<td>Could improve post-marketing assessment (not just safety signals)</td>
<td>Could create confusion among patients, physicians if post-marketing assessments are not in context</td>
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<td>Could clarify how to disseminate findings from real world studies in a regulatory framework</td>
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<td>Opportunity to develop “hybrid” design</td>
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<td>(enroll broad population, randomize, plan registration analysis in a subset &amp; broader in remaining)</td>
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Communication of Findings

- Clear communication of results is key to appropriate use in context of individual care
- Challenge providing clear and current information to physicians and appropriate tools
- Challenge disseminating results to consumers
- Concern that results will be used selectively, to justify barriers to access
- Concern that CE research will focus on short-term results rather than long-term or societal benefits
- Challenge reconciling regulatory restrictions on dissemination for off label uses vs. findings from real world studies generated for payers
Focus in Communication

- Tailored to appropriate audiences
- Inclusive of appropriate limitations & potential for generalizability
- Useful in real world settings and actionable
- Timely, balanced, objective
Industry Focus

• Ensure structure and processes of PCORI will continue to be inclusive, transparent
• Seek clear evidence standards – knowing the rules will enable better clinical development programs
• Pursue approaches that harmonize study requirements and approaches to disseminating findings between regulators and payers, to extent feasible
• Actively participate in communicating findings and advancing use of evidence-based practice
• Address other issues, e.g., role of personalized medicine, assessment of value over time