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

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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?

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

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 generalizeability
- 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