The relevance of using patient preference elicitation methods in healthcare

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Industrial Age Medicine
‘Mass production’

Information Age Health Care
‘Precision medicine and patient engagement’
Institute of Medicine: 10 Rules for Redesigning Health Care

1. Care based on continuous healing relationships
   - Care whenever it’s needed, not just face-to-face
2. Customization based on patient needs and values
3. The patient as the source of control
4. Shared knowledge and free flow of information
5. Evidence-based decision making


Food and Drug Administration Center for Devices and Radiological Health, 2012

• When assessing such data in a Pre Market Approval (PMA) application or de novo petition, FDA realizes that some patients are willing to take on a very high risk to achieve a small benefit, whereas others are more risk averse. Therefore, FDA would consider evidence relating to patients’ perspective of what constitutes a meaningful benefit when determining if the device is effective, as some set of patients may value a benefit more than others. It should also be noted that if, for a certain device, the probable risks outweigh the probable benefits for all reasonable patients, FDA would consider use of such a device to be inherently unreasonable.
Structured Approach to Benefit-Risk Assessment in Drug Regulatory Decision-Making

PDUFA V Plan (FY 2013-2017)
Draft of February 2013
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BACKGROUND ON FDA’S FRAMEWORK FOR DRUG REGULATORY DECISION-MAKING

1 Drug Regulatory Decision-Making—At the Intersection of Law, Science, Medicine, Policy, and Judgment

Regulatory decisions that FDA makes in both the pre-market and post-market drug review process are based on the Agency’s assessment of the benefits and risks of the product under review. This assessment is informed by science, medicine, policy, and judgment, in accordance with applicable legal and regulatory standards.

FDA’s work in structured benefit-risk assessment coincided with efforts elsewhere in industry and at other regulatory agencies, such as the European Medicines Agency (EMA). These approaches are quite similar in their most basic forms: defining the context in which the decision is being made, identifying the important relevant information and data regarding benefit and risk, assessing that information with respect to its bearing on the decision, drawing conclusions from the information based on expert judgment, and communicating the decision and its rationale. FDA’s structured approach to benefit-risk assessment is described in more detail in the sections that follow.

European Medicines Agency (EMA) Roadmap 2015:
Public awareness of transparency and openness

• The Agency strives to make its opinions on the balance of benefits and risks as consistent and transparent as possible. A three-year project on benefit-risk methodology was begun in early 2009, aiming to identify decision-making models that can be used in the Agency's work.
Basis for Conjoint Analysis

Resources are scarce = Opportunity cost

Choices have to be made

- Choices reveal information about preferences
- Risks and benefits need to be compared
- Something is only of value if we are willing to give something up for it

Growth in Conjoint Analysis

- Patient experience increasingly recognized as important
- QALYs dominate at policy level
- Widely used within RCTs

Source: Bridges JFP et al, The Patient, 2005
Conjoint Analysis in Health Definitions

• Broadly defined, conjoint analysis is
  – any stated-preference method in which
  – respondents are asked to rate, rank, or choose from among
  – a set of profiles and in which
  – profiles are constructed of sets of attributes of
  – varying levels and
  – responses reveal the implicit decision weights respondents attach to each level of each attribute included in the study

Conjoint analysis = consider jointly

Alternative Conjoint-Analysis Approaches

• Choice-format conjoint analysis (discrete-choice experiments)
  – Respondents choose among a set of alternatives
• Graded-Pairs
  – Rate two alternatives
    • Strongly prefer A to B, Somewhat prefer A to B, A and B are about the same
• Best-Worst Scaling
  – Choose best and worst alternative from a set of 3 or more alternatives
• Contingent Valuation “willingness-to-pay”
  – I would do this - Yes or No
Basis for BWS vs DCE

- BWS initially proposed in place of category rating scales to address
  - Lack of discrimination between items
  - Unclear interpretation of rating scale (what is the meaning of 3 out of 10?)
- DCEs introduced by Louviere in 1980s because of grounding in utility theory (choices), but less information available through DCE than traditional CA
- Initial motivation of BWS to improve the efficiency of data collection from each respondent

Flynn T and Marley AJ, CenSoC, 2012