Science of Patient Input (SPI) Webinar Series

From Stories to Evidence: Quantitative patient-preference information to inform product-development and regulatory reviews

MDIC's Science of Patient Input Project is pleased to present this three-part webinar series designed to shed light on how to convert the patient experience into usable evidence for regulatory and non-regulatory applications.

Who should watch?

- Medical device and medical product developers interested in incorporating patient preferences at various stages of the total product life cycle.
- Patient groups interested in initiating patient preference studies
- Regulators interested in understanding patient preference data

After each webinar, MDIC will provide a brief recap and links to additional resources.

If your company would like more information on getting started in this area, please visit our resource page at mdic.org/spi/resources/ or contact SPI Program Director, Stephanie Christopher at schristopher@mdic.org.

Part Two:

The Basics of Discrete Choice Experiments to Elicit Patient Preferences:

From Research Question to Experimental Design

Originally Recorded on March 15, 2018

Speakers:

Shelby Reed, *Professor, Duke School of Medicine*

F. Reed Johnson, *Professor, Duke School of Medicine*

Juan Marcos Gonzalez**,** *Assistant Professor, Duke School of Medicine*

Watch the webinar and download the slides here: http://mdic.org/mdicx/#StoriesEvidencepart2

Summary:

A discrete choice experiment (DCE), also known as choice-based conjoint analysis, is a choice-based method to elicit preferences. In a DCE, respondents indicate choices among hypothetical alternatives that consist of a combination of features (attributes). The statistical analysis of the pattern of choices indicates the relative importance of the features.

The International Society for Pharmacoeconomics and Outcomes Research **(ISPOR) Checklist for Stated-Preference Applications in Medicine** is a useful resource for DCE design and analysis. The checklist outlines a 10-step process for developing preference studies, from defining the research question to presenting the study (Figure 1). (*View the checklist here*)

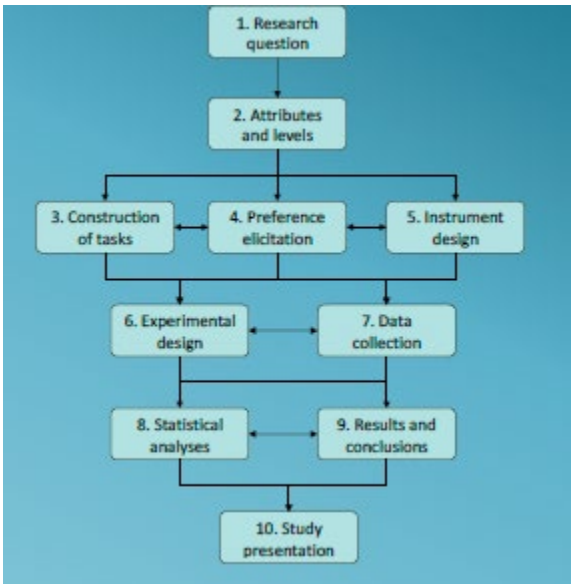

Figure 1. Checklist from the ISPOR Good Research Practices for Conjoint Analysis Task Force. https://www.ncbi.nlm.nih.gov/pubmed/21669364

In this webinar, experts from the Duke University School of Medicine reviewed and discussed ISPOR checklist steps one through six, which relate to instrument development:

1. **Research Question**
2. **Attributes and Levels**
3. **Construction of Tasks**
4. **Preference Elicitation**
5. **Instrument Design**
6. **Experimental Design**

Note: Steps 7-8 are covered in the third webinar in this series.

Developing the Research Question

The research question can vary in purpose, for example, "*What is the relative importance of less pain versus heart-attack risk?*" or "*What is the money-equivalent value (WTP) of an effective treatment-resistant depression?*" Regardless of the purpose, there are several basic considerations for developing a research question:

- **Study perspective –** Who (researchers, patient populations) is going to be involved? What is to be accomplished with the research? Why is it important?
- **Decision-making context –** Is the decision preference-sensitive?
- **Tractability –** Can the question be answered with available methods?
- **Feasibility –** Can the question be answered with available time, resources, and expertise?

Once the research question is formulated, a formal hypothesis and the implied evidence needed to test the hypothesis should be specified.

Watch the discussion and see examples of sample hypotheses and implied evidence, starting at 13 min

Identifying and Choosing Attributes and Levels

Once the research question, hypothesis, and type of evidence needed are specified, researchers must build an instrument to obtain those data. Instrument development begins by considering attributes and levels that are relevant to the research question and evidence.

Attributes are defined as generic features and levels are defined as variations within each feature. For example, in a preference survey about modes of transportation, attributes could be vehicle type and color. The corresponding levels for these attributes could be (car/bus) and (green/red), respectively.

Selected attributes must be **clinically relevant**, **outside of respondents' control**, and **vary independently**. When choosing levels for the attributes, the **range**, **number**, and **labeling** of the levels are important. The ranges of levels should be wide enough to encourage tradeoffs and include values that are observed or expected in clinical evidence.

Watch the discussion of attribute and level selection and see a sample attribute/level table, starting at 22 min

Constructing Choice Questions/Tasks

When constructing choice questions, researchers must consider the **number**, **type**, and **complexity** of the questions. **Patients' prior knowledge and experience** also play a role in the amount of information presented in the questions, as well as the wording (labeling).

Watch the discussion, starting at 31 min

Beyond the Choice Questions: Additional Components of the Survey Instrument

The survey instrument includes more than the choice questions It is appropriate to present the actual choice questions to the respondents only after providing enough background, including **consent**, **attribute descriptions**, a **risk tutorial**, and **practice choice questions** and **comprehension tests**.

Watch the discussion of survey instrument components and examples, starting at 35 min

Barriers to Valid Preference Data

Criticisms of stated preference studies and data from these studies tend to center around the following topics:

- **Risk and numeracy**
- **Participants' preparation for choice questions**
- **Hypothetical bias**
- **Survey complexity**

However, several checks can be built into the study to address these criticisms:

- **Risk tutorials –** Help patients better understand the concept of risk in by including a written summary of risk and risk icon arrays – which visually display probabilistic information – while emphasizing both positive and negative outcomes (Figure 2). After the information is presented, use follow-up questions to check for comprehension and reinforce the approach, if necessary.

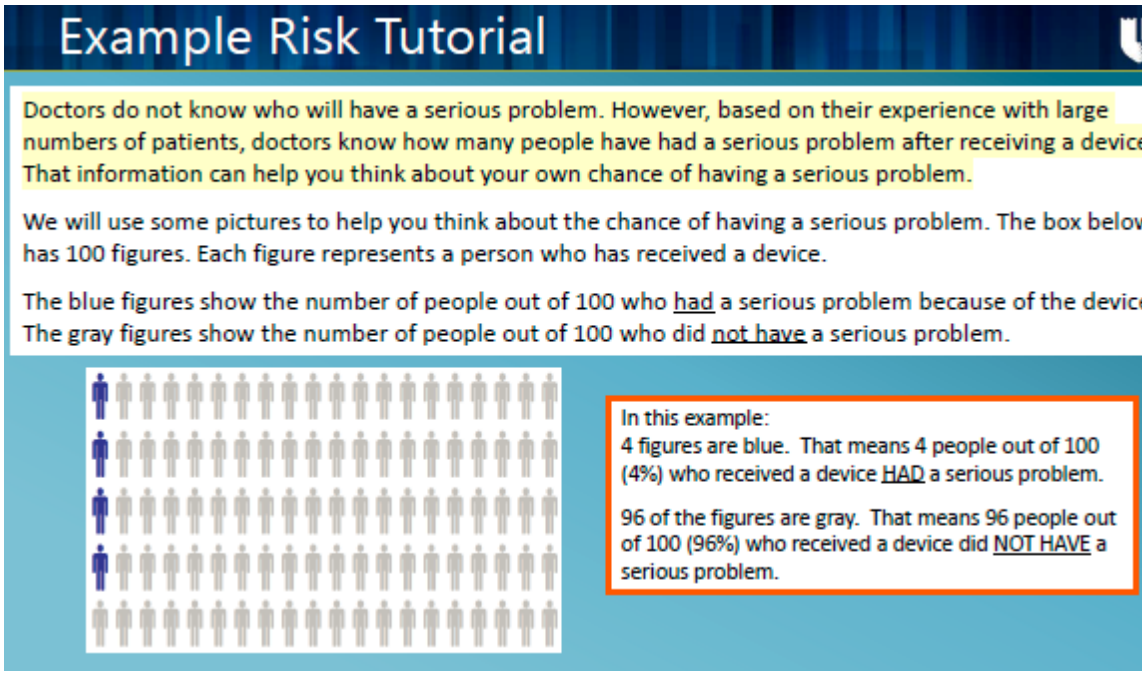

Figure 2. Example Risk Tutorial. The stick figure diagram is called a risk icon array and is used to visually depict risk.

- **Deliberate selection of decision context/decision frame –** Choose a frame that is clinically relevant and representative of a situation that the patient would encounter.
- **Practice questions –** Give patients an opportunity to practice thinking about tradeoffs using the survey question format and in the chosen decision frame. Increase the complexity of the practice questions until they resemble the choice questions from which the data will be collected.
- **"Cheap talk" to address hypothetical bias –** Cheap talk is a technique from economic game theory in which the negotiator attempts to influence results without giving any concessions. Including cheap talk that shares with respondents the challenge researchers face in working with hypothetical scenarios and asking for respondents' help can reduce hypothetical bias. The cheap talk may look something like the sample text below:

  > *"To make our study a success, we need your help with a problem we have in studies like this. Because our participants do not actually have to live with the results of the treatment they select, they often do not think carefully about what they would do if they really had to choose.*
  >
  > *If you do not pay attention to the information shown in each question as you would in real life, we will not get a true measure of how important various treatment benefits and risks actually are to people like you."*
  >
  > *We need your thoughtful answers to help us understand how you feel about possible medical devices."*

- **Face-to-Face Pretest interviews** – Conduct interviews with 10-12 respondents similar to the target population using a "think-aloud" protocol in which patients read and respond to the

survey and respond with whatever comes to mind as they work through it. Ask probing questions such as, “Why did you choose A?” or “How could we make that clearer?” to refine the instrument and fix problems as they are encountered.

Watch the discussion, starting at 40 min

Experiment and Instrument Design

It is not possible to design a realistic survey that evaluates all possible choices and questions related to the attributes in a study. Software algorithms can construct experimental designs that achieve the desired precision in the fewest number of questions, but the conflicting objectives of obtaining a reliable subject response and collecting granular data force require a practical approach to design beyond the limits of software.

Watch the discussion about practical design considerations, starting at 55 min

Audience Q&A:

The webinar panelists and officials from FDA answered audience questions. Listen to the Q&A, starting at 1 hr 4 min.

- *Are there examples where preferences about willingness to pay have been used by private or public payers to make coverage decisions?*
- *How do we identify attributes when a medical product is hypothetical or in early development stages?*
- *At what stage of product development is it best to conduct these studies?*
- *What is the relationship between attributes and levels in the preference study and the clinical endpoints in the study protocol?*

Related Links:
MDIC PCOR Project Workshop: Using Parkinson’s Patient Preferences to Re-Define Statistical Significance Levels in Clinical Trials

MDIC Patient-Centered Benefit-Risk (PCBR) Framework

MDIC Science of Patient Input (SPI) Project Page

Other Webinars in this Series

• Part 1: *Quantitative Patient Preference Studies Across the Total Product Life Cycle*

• Part 3: *The Basics of Discrete Choice Experiments to Elicit Patient Preferences: Results and Analysis*