REMS Assessments: Social Science Methodologies to Assess Knowledge Goals

Panel 1: Surveys to assess knowledge

Overview

- Panel 1's charge
- Language of the requirement to assess knowledge
- Summary of our observations
  - Common characteristics of surveys submitted
  - Observations leading to specific concerns
- Clarifying questions

Panel 1's charge

- GOAL: To provide input regarding specific, feasible strategies to improve surveys that are submitted to assess the educational components of REMS
- Specific discussion items:
  - Survey endpoints
  - Threshold for success
  - Survey participant recruitment (sampling frame)
  - Sample size
  - Question design
  - Process issues

Example of requirement for knowledge assessment

- Goals
  - To inform healthcare providers about the risks of…associated with [DRUG].
  - To inform patients or caregivers about the serious risks associated with [DRUG] treatment.
- Language from an Approval Letter

  "Evaluations of healthcare providers' understanding and patients' understanding of the risks of [DRUG]"

REMS Assessments Received

- First assessment received in September 2008
- 144 Assessments received as of December 2011
  - 55 Medication Guide-only
  - 33 Medication Guide/Communication Plan
  - 56 Other (mostly assessments of REMS with ETASU)

Common characteristics of REMS surveys submitted

- Pre-specified statistical analysis or hypothesis testing rarely included
- Some, but not most, surveys are piloted or pre-tested
- Convenience samples, e.g. contract with a large pharmacy chain and randomly sample x number of patients who received the drug on the list provided
  - The most common healthcare provider (HCP) recruitment strategy is to send letters or telephone HCP identified by pharmacies or audit services
  - The most common patient recruitment strategies are to use a pharmacy/network to identify patients and invite them by mail and ask HCP to identify patients on drug for drugs not dispensed in a pharmacy

Common characteristics of REMS surveys submitted

- Data collected include:
  - Knowledge of risks
  - Demographics
  - Time on drug
  - Medication Guide (MG) metrics (whether patient recalled receiving MG, source of MG, whether patient read MG, whether someone offered to explain MG)

Common characteristics of REMS surveys submitted

- Results reported as number answering correctly divided by number of respondents to question as a percentage (knowledge rate)
  - Some surveys have used a composite scoring system. Usually, these systems group related questions (for instance related to a single risk) and require the respondent to answer all questions (usually 3-4) correctly to be defined as a correct response.

Threshold for success

- Defined as the proportion of respondents responding correctly which, if met or exceeded, would support a conclusion that the desired knowledge was adequate in the population.
- The minority of surveys specify a threshold knowledge rate. Of those that do, 80% is usually selected.

Survey populations may not be representative

- 2010 study of survey methods:
  - Limited demographic information from REMS surveys were compared to patient and prescriber information from the SDI Physician Drug and Diagnosis Audit (PDDA).
  - Differences were assessed.

Smith, D., Willy, M., Karwoski, C., Winterstein, A. (2011, August). Generalizability of Risk Evaluation and Mitigation Strategies Effectiveness Evaluations. Poster presented at 27th International Conference on Pharmacoepidemiology & Therapeutic Risk Management, Chicago, Illinois.

Survey populations may not be representative

- Results:
  - N=7 patient surveys; N=2 HCP surveys
  - Five patient surveys and two HCP surveys showed differences between the certain survey population descriptors and PDDA data; gender, age, race (patients); specialty (prescribers).
- Limitations
  - Short history of FDAAA and REMS resulted in a very small number of assessments available for analysis
  - Many metrics of interest to assess generalizability such as income, education, and health literacy were not available for analysis.
  - Demographic patterns from SDI PDDA for products with low use may not be generalizable to REMS survey results.
  - Many REMS assessments were missing or poorly specified demographic categories of interest

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Sample size

- We have seen some very small sample sizes, sometimes in the single digits.
  - Examples of low sample size in patient surveys: 8, 20, 22, 30
  - Examples of low sample size in HCP surveys: 4, 9, 33
- These are usually associated with a drug with a small prescriber base and small patient population.
- Occasionally, the survey methodology specified a target number which was not met.
- Sample size rationale, when provided, is based on precision of knowledge rate estimate. Common assumptions: margin of error of 6%, confidence level of 95%, knowledge rate of 50%

Question design

- Surveys use the True/False/I don't know question forms for the vast majority of questions.
- The other question form is multiple choice, both "choose one" or "choose all applicable answers."
- A small number of surveys have used one or more clinical vignettes as part of the question.

Other concerns with surveys for REMS

- Level of difficulty of questions and lag time between reading of materials and survey potentially confounds results.
- Not all surveys are pre-tested.
- Because HCP and patients obtain information from different sources, the contribution of the REMS materials cannot be determined.

Questions for me?