



Challenges in the Evaluation of RiskMAPs

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Topic Areas

- Impact, purpose, common goals
- Measurement challenges
- Performance targets
- Timing
- Expertise and resources
- Use and sharing of results

Impact of RiskMAPs

Impact of RiskMAPs felt by many

- Patients taking the drug
- Physicians
- Pharmacists
- Pharmaceutical and biologics industries

But also.....

Impact of RiskMAPs

Impact of RiskMAPs also felt by

- Health care institutions seeking quality health care
- Government and private interests paying for drugs and their associated health care savings or costs
- Federal and State governments regulating drugs or health care professionals and settings



Common Goals in Evaluation

To help RiskMAPs achieve medication use that is

- Safe
- Effective
- Efficient
- High quality



Types of Measurements

Safe and effective

- Benefits attained > harms incurred

Efficiency

- Appropriate access maintained
- Minimal added costs and burdens on health care participants and operations

Quality (applied to appropriate medication use)

Health services

- increase likelihood of desired health outcomes
- are consistent with current knowledge

Candidate Measures

Safety and effectiveness of therapy

- Health outcomes or closest surrogate
- Unintended consequences

Efficiency

- Barriers encountered, costs
- Process & operations QA
- FMEA or root cause analyses

Quality

- Safety, effectiveness, efficiency, QA as above
- Equity, appropriateness or satisfaction of therapeutic choices

Performance Targets

Achieving health outcomes or close surrogates

- Zero or low numbers/rate of adverse event(s)

Adequacy of process implementation

- Processes in support of health outcomes, e.g. pregnancy testing, informed consent

Quality or satisfaction with decision-making

- Of patients, HCPs, others? What instrument(s)?

Success of knowledge transfer

- To patients, HCP, others? How best assess?



Scientific Challenges of Observational Data

Best measurements involve:

Outcomes that validate to an accepted standard

- High sensitivity and specificity

Sample populations

- Without selection bias (representative)
- Sized to provide sufficient power/precision in results

Minimal error

- Confounding
- Misclassification bias

Example of Scientific Challenges with Surveys

Surveys for measurement can

- augment typical health care data systems
- customize data collection
- diversify input to include multiple sources

However, surveys are often limited by

- framing effects and other survey design issues
- non-representative participants
- self-reports especially of sensitive information

Scientific Challenges in Setting 'Metrics'

Determining a reference group or comparator

- General population or those with disease/condition
- Untreated or receiving alternative treatments
- Pre/post comparisons before and after RiskMAP

Determining target(s)

- Are goals of 0 or 100% realistic?
- Is incremental progress acceptable? How much and how fast is it required?
- Who/how to determine what is acceptable?

Timing of Evaluations

- At the beginning as part of design
 - Formative assessments to assess existing knowledge, health care system processes, and likelihood of assimilating into clinical practice
- Periodically after implementation for quality assurance of operations and processes
 - May identify critical or redundant component tools
- Periodically to assess overall RiskMAP performance
 - Progress towards or achievement of target



Timing of Evaluations

How often is “periodically?”

Who/how to determine the best periodicity?



Evaluation Expertise & Resources

- Industry
- Government
- Academic or private entities funded by the above
- Aims
 - Avoid real or apparent conflicts
 - Transparency





Use and Sharing of Evaluation Results

- Informs and improves RiskMAPs
- Allows sharing of “lessons learned”
- Fosters best practices
 - improve effectiveness, consistency
 - lessen customizations
- Promotes knowledge transfer
 - FDA Advisory Committees, Websites
 - Scientific publications
 - Professional guidelines, other opportunities?