Discussion Panel on Patient-Stakeholder Engagement

Tuesday, September 18th, 2012
12:00 – 1:00 pm

Dr. Nazleen Bharmal, Moderator

Clinical Instructor of Medicine, General Internal Medicine

Dr. Paul G. Shekelle (via teleconference)

Director, Southern California Evidence-Based Practice Center, RAND Corporation

Director, Quality Assessment and Quality Improvement Program, RAND Health

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Physician and Faculty at the University of North Carolina (UNC)

Sarah Graham Kenan Professor in the Departments of Medicine and Social Medicine at UNC-Chapel Hill

Agenda

Introduction -Dr. Bharmal (10 minutes)

- Speakers
- Topic

Identifying and Prioritizing Research Gaps with Stakeholder Engagement- Dr. Carey (10 minutes)

- Discussion (10 minutes)

Experience Involving Stakeholders in Evidence-Based Processes - Dr. Shekelle (10 minutes)

- Discussion (10 minutes)

Discussion: Ways to move forward and how CTSI can support for stronger proposals with state of the art stakeholder engagement components, moderated by Dr. Bharmal (15 minutes)

Background Materials

- Selby, JV; Beal, AC; Frank, L. "The Patient-Centered Outcomes Research Institute (PCORI) National Priorities for Research and Initial Research Agenda", *JAMA.* April 18, 2012 Vol. 307. No. 15 1583-4.
- Concannon, et al. "A New Taxonomy for Stakeholder Engagement in Patient-Centered Outcomes Research" *JGIM.* 985-991.

CTS RRT Rapid Response Team

Identifying and Prioritizing Research Gaps with Stakeholder Engagement

Tim Carey, M.D., M.P.H.
Amica Yon, Pharm.D.
Chris Beadles, M.D.
Roberta Wines, M.P.H.

Importance:

Why We Need to Identify and Prioritize Research Gaps from Systematic Reviews

- Systematic reviews are the standard for evaluating the current state of scientific knowledge regarding a specific clinical or policy question.
- Identification and prioritization of research gaps has the potential to lead to more rapid generation of subsequent research, informed by input from stakeholders
- Audiences including researchers, funders, clinicians, advocates, and patients could use information about prioritized research gaps to understand areas of uncertainty and more quickly initiate studies.

Existing Methods to Identify and Prioritize Research Gaps

- Identification of research gaps from and within systematic reviews is common, but often very general.
  - Criteria used to date have been variable and often unclear.
- Prioritization of research gaps arising out of systematic reviews is not common at present.
- Only half of the systematic reviews in major journals discussed future research needs at all, one-fifth described study designs that would address research gaps.
- Text devoted to future research generally less than a paragraph.

Existing Methods to Identify and Prioritize Research Gaps

- A scan of reports published within the past two years by the Drug Effectiveness Review Project (N = 4), NIH Consensus Conferences (N=5), and the Cochrane Collaboration (N = 19) showed no standardized methods for identifying or prioritizing research gaps.
  - Cochrane Collaboration reviews generally included ‘implications for future research’ but the discussions were often nonspecific.
- Global Evidence Mapping (GEM) describes gap analysis as part of planning for future research after a systematic review is completed with stakeholder engagement.
- The James Lind Alliance (UK) supports the development of partnerships of clinicians, patients, and advocacy groups in the prioritization of areas of uncertainty in clinical medicine.

Existing Methods to Identify and Prioritize Research Gaps

Agency for Healthcare Research and Quality Future Research Needs

- AHRQ piloted 8 Future Research Needs (FRNs) Projects in 2010 to extract research gaps from a systematic review, transforming them into prioritized research questions with aided by diverse stakeholder groups.
- AHRQ EPCs have published multiple FRN methods papers to date.
- 7 steps common to AHRQ FRN projects.

Stakeholder Engagement

- Advisory *vs.* determinative
- Providers, patients and caregivers, advocates, funders, researchers, regulators, policymakers, manufacturers
  - Complicated issues regarding roles of advocacy groups vs role of patients
  - How to identify patients?
- Training needed
  - How much, by whom and how tailored?
- Conflict of interest/competing interest issues

Identification of Research Gaps

- "Topic or area for which missing or inadequate information limits the ability of reviewers to reach a conclusion for a given question."
- Utility of an analytic framework illustrating the relationship of gaps to the key questions and analytic framework of the review.
- Stakeholders may identify gaps not identified by the reviewers.
  - But…they need to be in the scope of the key questions.
- Gaps derived from GRADE
  - Insufficient or imprecise information
  - Biased information
  - Inconsistency or unknown consistency
  - Not the right information (wrong population or wrong outcome)

Priority Ranking

- Reviews may generate many gaps, need for prioritization
- Some organizations use broad internet data gathering
  - Will the participants understand all of the issues?
- Multiple methods currently used
  - Ranking 1-xx
  - Likert scale 1-7
  - Multi-voting, multiple (but limited) votes per choice
  - Pair-wise comparisons
  - Delphi methods
  - Consensus conference

Transformation of Research Gaps into Needs

- Gaps are generally in the form of a declarative sentence.
- Needs are questions similar to research questions in a grant proposal.
- Most organizations use PICOTS framework: **P**opulation, **I**ntervention, **C**omparator, **O**utcome, **T**imeframe, **S**etting.
- Methods questions may be important, but may not be a fit for PICOTS.

Dissemination and Implementation Issues

- Will the gaps and prioritization resonate with funders, advocacy groups and policymakers?
  - Need to work with them to identify the best formats and content for efficient communication of results
  - US environment is heterogeneous, with multiple federal agencies, PCORI, other foundations
  - Funders may use the priorities, but not acknowledge doing so.
- What are the best ways to communicate with the public and funders?
- What is the role of peer-reviewed articles?

Example: AHRQ Future research needs on ADHD

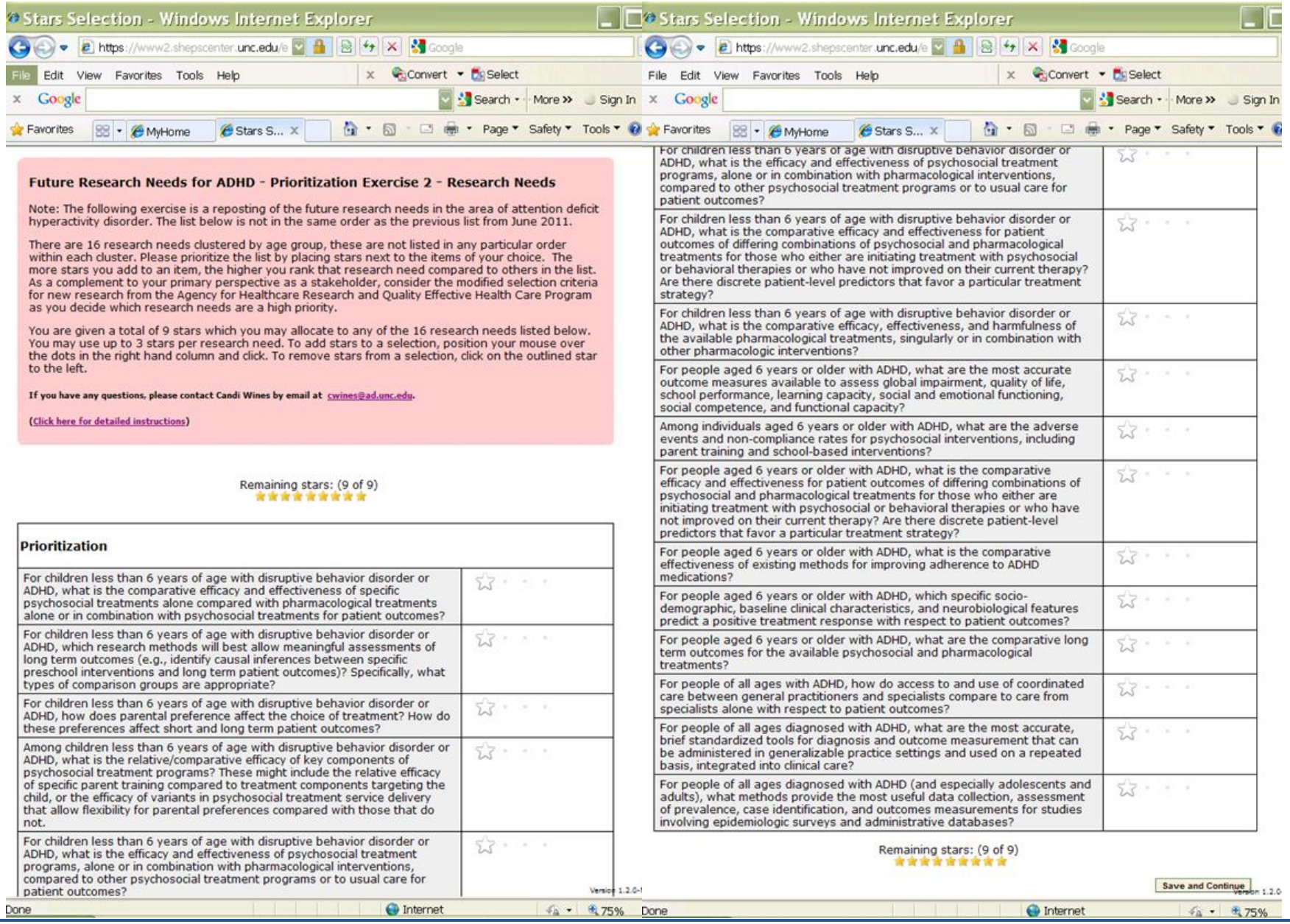
Key Questions from Comparative Effectiveness Review

KQ1	Among children less than 6 years of age with Attention Deficit Hyperactivity Disorder or Disruptive Behavior Disorder, what are the effectiveness and adverse event outcomes following treatment?
KQ2	Among people 6 years of age or older with Attention Deficit Hyperactivity Disorder, what are the effectiveness and adverse event outcomes following 12 months or more of any combination of follow-up or treatment, including, but not limited to, 12 months or more of continuous treatment?
KQ3	How do A) underlying prevalence of Attention Deficit Hyperactivity Disorder, and B) rates of diagnosis (clinical identification) and treatment for Attention Deficit Hyperactivity Disorder vary by geography, time period, provider type, and sociodemographic characteristics?

20 research gaps from the review mapped to the key questions, presented to a group of 12 stakeholders, including funders, advocates, clinicians, regulators, researchers, and policymakers.

After stakeholder input, 29 research gaps. 8 gaps emerged as the top future research needs after two rounds of prioritization using an online prioritization tool.

The next two slides show the presentation of one gap from identification to study design.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Future Research Needs for ADHD - Prioritization Exercise 2 - Research Needs
Note: The following exercise is a reposting of the future research needs in the area of attention deficit hyperactivity disorder. The list below is not in the same order as the previous list from June 2011.
There are 16 research needs clustered by age group, these are not listed in any particular order within each cluster. Please prioritize the list by placing stars next to the items of your choice. The more stars you add to an item, the higher you rank that research need compared to others in the list. As a complement to your primary perspective as a stakeholder, consider the modified selection criteria for new research from the Agency for Healthcare Research and Quality Effective Health Care Program as you decide which research needs are a high priority.
You are given a total of 9 stars which you may allocate to any of the 16 research needs listed below. You may use up to 3 stars per research need. To add stars to a selection, position your mouse over the dots in the right hand column and click. To remove stars from a selection, click on the outlined star to the left.
If you have any questions, please contact Candi Wines by email at cwines@ad.unc.edu.
(Click here for detailed instructions)
Remaining stars: (9 of 9)
Prioritization
For children less than 6 years of age with disruptive behavior disorder or ADHD, what is the comparative efficacy and effectiveness of specific psychosocial treatments alone compared with pharmacological treatments alone or in combination with psychosocial treatments for patient outcomes?
For children less than 6 years of age with disruptive behavior disorder or ADHD, which research methods will best allow meaningful assessments of long term outcomes (e.g., identify causal inferences between specific preschool interventions and long term patient outcomes)? Specifically, what types of comparison groups are appropriate?
For children less than 6 years of age with disruptive behavior disorder or ADHD, how does parental preference affect the choice of treatment? How do these preferences affect short and long term patient outcomes?
Among children less than 6 years of age with disruptive behavior disorder or ADHD, what is the relative/comparative efficacy of key components of psychosocial treatment programs? These might include the relative efficacy of specific parent training compared to treatment components targeting the child, or the efficacy of variants in psychosocial treatment service delivery that allow flexibility for parental preferences compared with those that do not.
For children less than 6 years of age with disruptive behavior disorder or ADHD, what is the efficacy and effectiveness of psychosocial treatment programs, alone or in combination with pharmacological interventions, compared to other psychosocial treatment programs or to usual care for patient outcomes?
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For children less than 6 years of age with disruptive behavior disorder or ADHD, what is the efficacy and effectiveness of psychosocial treatment programs, alone or in combination with pharmacological interventions, compared to other psychosocial treatment programs or to usual care for patient outcomes?
For children less than 6 years of age with disruptive behavior disorder or ADHD, what is the comparative efficacy and effectiveness for patient outcomes of differing combinations of psychosocial and pharmacological treatments for those who either are initiating treatment with psychosocial or behavioral therapies or who have not improved on their current therapy? Are there discrete patient-level predictors that favor a particular treatment strategy?
For children less than 6 years of age with disruptive behavior disorder or ADHD, what is the comparative efficacy, effectiveness, and harmfulness of the available pharmacological treatments, singularly or in combination with other pharmacologic interventions?
For people aged 6 years or older with ADHD, what are the most accurate outcome measures available to assess global impairment, quality of life, school performance, learning capacity, social and emotional functioning, social competence, and functional capacity?
Among individuals aged 6 years or older with ADHD, what are the adverse events and non-compliance rates for psychosocial interventions, including parent training and school-based interventions?
For people aged 6 years or older with ADHD, what is the comparative efficacy and effectiveness for patient outcomes of differing combinations of psychosocial and pharmacological treatments for those who either are initiating treatment with psychosocial or behavioral therapies or who have not improved on their current therapy? Are there discrete patient-level predictors that favor a particular treatment strategy?
For people aged 6 years or older with ADHD, what is the comparative effectiveness of existing methods for improving adherence to ADHD medications?
For people aged 6 years or older with ADHD, which specific socio-demographic, baseline clinical characteristics, and neurobiological features predict a positive treatment response with respect to patient outcomes?
For people aged 6 years or older with ADHD, what are the comparative long term outcomes for the available psychosocial and pharmacological treatments?
For people of all ages with ADHD, how do access to and use of coordinated care between general practitioners and specialists compare to care from specialists alone with respect to patient outcomes?
For people of all ages diagnosed with ADHD, what are the most accurate, brief standardized tools for diagnosis and outcome measurement that can be administered in generalizable practice settings and used on a repeated basis, integrated into clinical care?
For people of all ages diagnosed with ADHD (and especially adolescents and adults), what methods provide the most useful data collection, assessment of prevalence, case identification, and outcomes measurements for studies involving epidemiologic surveys and administrative databases?
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Example: AHRQ FRN on ADHD

Identify Research Gap:

For children less than 6 years of age with disruptive behavior disorder or ADHD, limited data are available about the efficacy and effectiveness of psychosocial treatment programs (e.g., parent training and summer behavior treatment programs), alone or in combination with pharmacological interventions, compared with other psychosocial treatment programs, alone or in combination with pharmacological interventions. (KQ 1)

After One Round of Prioritization Apply PICOTS and Develop Research Question:

P	I	C	O	T/S
Age < 6 years Diagnosed with ADHD or at risk for ADHD or diagnosed with disruptive behavior disorder (including ODD and CD by DSM)	Psychosocial interventions alone (including parent training and school-based interventions)	Pharmacological treatments, alone or in combination with psychosocial treatments	Outcomes for children and parents*	6 Months/ 1Year Private clinic, community clinic

Research Question: For children less than 6 years of age with disruptive behavior disorder or ADHD, what is the comparative efficacy and effectiveness of specific psychosocial treatments alone compared with pharmacological treatments alone or in combination with psychosocial treatments for patient outcomes?

Example: AHRQ ADHD FRN

After Second Round of Prioritization Develop Study Design Considerations:

Randomized controlled trials

Randomized trials could be designed to test various components in a 2x2 matrix of psychosocial treatment variants (parent training, school-based intervention, combination, or pharmacological).

- ***Advantages of study design for producing a valid result***
  Allows isolation of causal inferences related to the intervention being tested. Multiple-armed trials would allow testing of several hypotheses regarding relative efficacy of singular or combination treatment components.
- ***Ability to recruit/availability of data***
  Common condition in this age group with uncertainty regarding treatment choice; all arms receive some treatment.
- ***Resource use, size, and duration***
  Large sample size (*N* = 840; *n* = 210 per treatment arm) needed. Key outcomes such as school achievement will require follow-up of several years.
- ***Ethical, legal, and social issues***
  Vulnerable population, careful informed consent will need to occur.

State of the Science

- Multiple groups are currently conducting work in this area
- Sufficient common aspects to serve as a consensus
  - Criteria for gaps identification
  - Broad aspects of stakeholder panel composition
  - Need to train stakeholders in PCOR
  - Explicit prioritization method - but multiple methods currently used
  - Decisions regarding study design considerations
- We can use existing methods now while refining the approaches

Next Steps and Recommendations

1. Evaluate different stakeholder panel sizes and compositions in prioritization.
2. Evaluate the reliability of stakeholder prioritization through replication studies.
3. Test different methods of prioritization to assess for transparency, reproducibility and efficiency.
4. Clarify role of gap identification and prioritization with other methods such as VOI.
5. Identify best practices for training stakeholders, including patients and caregivers
6. Collaborate with other patient-centered outcome research programs in refining this area.

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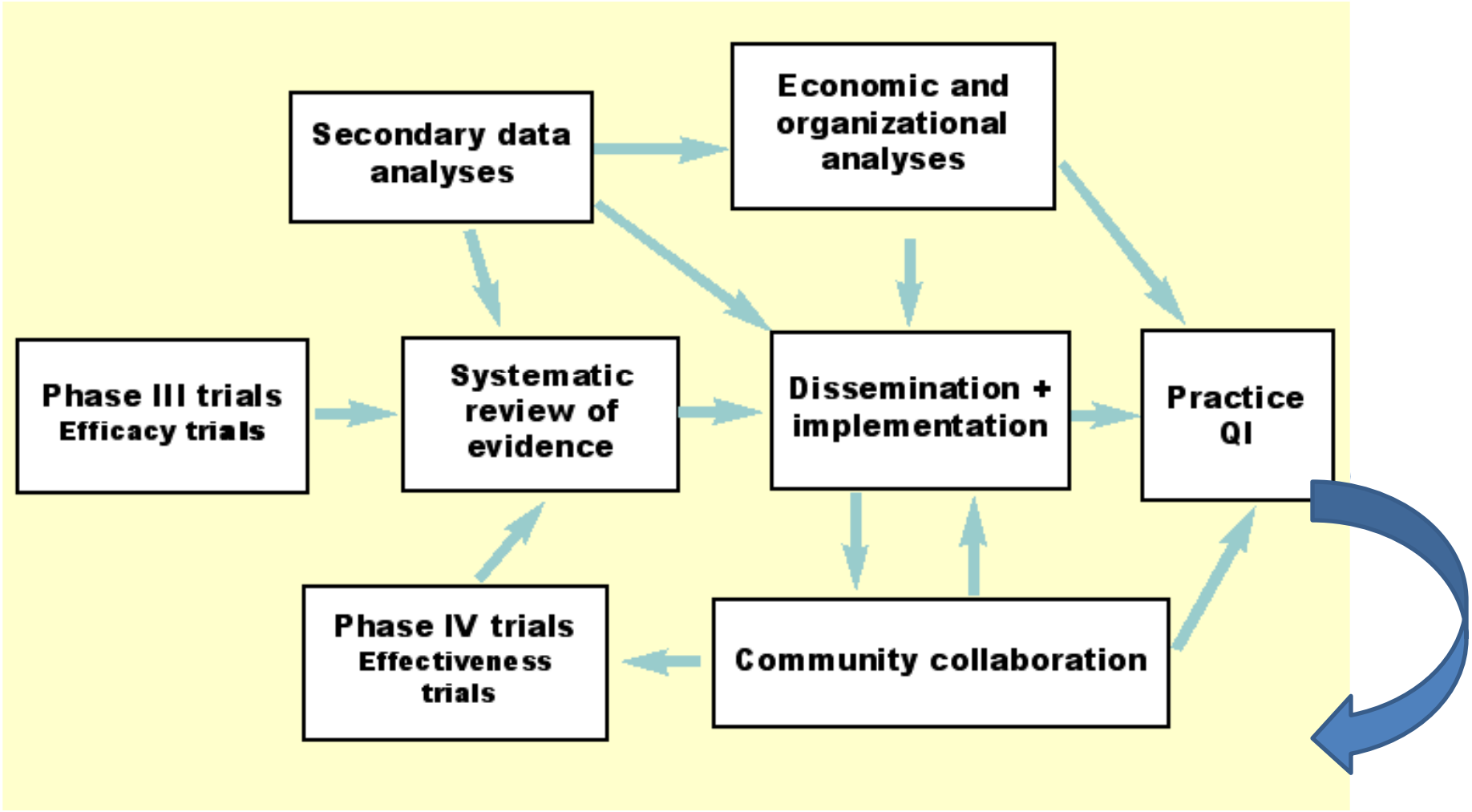
AHRQ: Stephanie Chang, Elisabeth Kato

OHSU and Duke EPC's: Jeanne-Marie Guise, Gillian Sanders-Schmidler

Schematic for comparative effectiveness research



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Experience Involving Stakeholders in Evidence-Based Processes

CTSI RRT Rapid Response Team

EXPERIENCE INVOLVING STAKEHOLDERS IN EVIDENCE-BASED PROCESSES

National or International

- Oregon Medicaid experiment from 1990's
- Patient involvement in AHCPR guideline panels
- Patient involvement in UK National Institute of Clinical Excellence
- Stakeholder involvement in the AHRQ Effective Health Care Program
- G-I-N Public (www.g-i-n.net/activities/gin-public)

EXPERIENCE INVOLVING STAKEHOLDERS IN EVIDENCE-BASED PROCESSES CONT.

Local

Patient involvement in developing quality indicators for multiple sclerosis (Barbara Vickey, Eric Cheng)

Patient involvement in developing quality indicators for urinary incontinence (Jennifer Anger)

Stakeholder involvement in practice re-design for well child visits (Paul Chung, Tumaini Coker)