

REQUEST FOR INFORMATION—Input on a Draft Translation Table Framework

BACKGROUND AND PURPOSE

The Patient-Centered Outcomes Research Institute (PCORI) is an independent, non-profit research organization created by the Patient Protection and Affordable Care Act of 2010 (PPACA). The mission of PCORI is to help people make informed health care decisions – and improve health care delivery and outcomes – by producing and promoting high-integrity, evidence-based information derived from research guided by patients, caregivers and the broader health care community. Research commissioned by PCORI aims to be responsive to the values and interests of patients and provide patients and those who care for them with reliable, evidence-based information for the health care choices they face.

The Methodology Committee of PCORI was established to develop and improve the science and methods of comparative clinical effectiveness research. In particular, the legislation calls on the Methodology Committee to develop “a translation table that is designed to provide guidance and act as a reference for the Board to determine research methods that are most likely to address each specific comparative clinical effectiveness research question,” and to produce a report by May 2012 outlining the progress on the development of this translation table.

The Methodology Committee has developed a preliminary translation framework that will inform the development of the translation table. The purpose of this Request for Information (RFI) is to invite input on the translation framework components and to engage stakeholder communities in the development of this translation tool. Responses to this RFI will be considered for inclusion in the May 2012 report. Details on the information requested and response submission instructions are provided in later sections of this document.

INTRODUCTION TO THE PROPOSED TRANSLATION FRAMEWORK AND TOOL

Box 1 defines patient-centered outcomes research, translation table, translation framework, and translation tool within the context of this RFI. **Figure 1** diagrams the proposed structure of the translation tool. **Box 2** describes the background and elements of a patient-centered research question. **Box 3** describes the proposed translation framework components.

Box 1: Definition of Patient-Centered Outcomes Research, Translation Table, Translation Framework, and Translation Tool.

Patient-Centered Outcomes Research (PCOR): PCOR aims to help people make informed health care decisions and allows their voice to be heard in assessing the value of health care options. PCOR:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, or health delivery system interventions to inform decision-making, highlighting comparisons and outcomes that matter to people;
- Is inclusive of an individual's preferences, autonomy and needs, focusing on outcomes that people notice and care about such as survival, function, symptoms, and health-related quality of life;
- Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination; and
- Investigates (or may investigate) optimizing outcomes while addressing burden to individuals, resources, and other stakeholder perspectives.

Translation Table (according to PPACA): “The translation table will provide guidance and act as a reference for the PCORI Board to determine research methods that are most likely to address specific comparative clinical effectiveness research questions.”

Translation Framework: The *translation framework* provides the theoretical underpinning and organizing structure for the *translation table and translation tool* that will help users identify the range of appropriate research designs and analytic approaches to answer specific patient-centered research questions. It is defined by a set of *framework components* (see Box 3) that can be used to guide the user in making choices in study design and analytic methods based on current scientific knowledge.

Translation Tool: Although the legislative mandate is to create a translation table, the usefulness of a tabular format may be limited. A *translation tool* is a dynamic implementation of the *translation framework* to help users apply it to specific research questions. Such a tool might take the form of decision trees, weighting or optimization algorithms, or other formats. More than one design recommendation may result, along with designs that are deemed unacceptable.

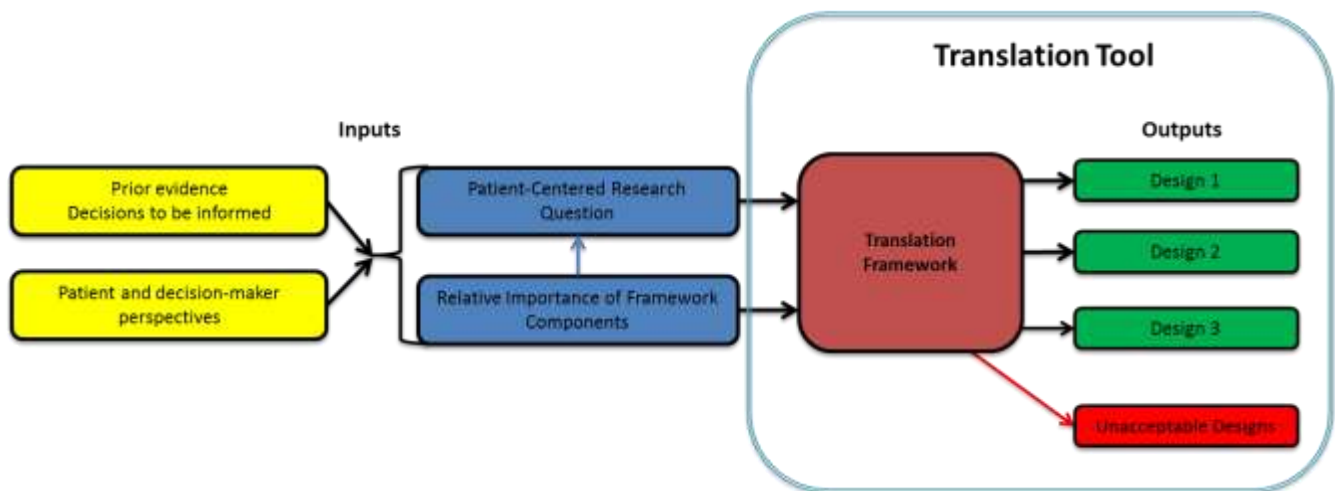
Proposed Structure of the Translation Tool

The translation tool will be the instrument by which the translation framework is operationalized. The patient perspective, patient-centered research question, and the relative importance of framework dimensions are key inputs to the translation tool.

The steps preceding the development of the research question establish how much and what kind of evidence is needed. These include a summary of prior studies, including what is known, unknown and why, and the decision the study in question is supposed to inform. Multiple perspectives can affect the choice of design; perspectives include those of patients, clinicians, researchers and policy makers. These perspectives affect choice of study design through both the choice of the patient-centered research question (see Box 2) and the relative importance patients and decisions-makers place on the various framework components (see Box 3).

Based on the question and relative importance of the components of the translation framework, the translation tool provides an output outlining acceptable and unacceptable designs. Output could be a ranked list of potential designs, a list of recommended and not recommended designs, or a table of potential designs with a description of the trade-offs involved with each design.

Figure 1: Proposed Structure of the Translation Tool.



Defining a Patient-Centered Research Question

A key step in designing patient-centered research is defining a research question that addresses questions that are important to patients and persons caring for them. PCOR should answer questions like:

1. “Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”
2. “What are my options and what are the benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can the health care system improve my chances of achieving the outcomes I prefer?”

For example, patients seek research that involves populations with similar characteristics to themselves, compares the actual intervention and comparator they are considering, assesses outcomes important to them and their caregivers, and uses a time frame that captures relevant benefits and harms in a setting (e.g. hospital or clinic) typical of what they might find in their community.

Box 2. Defining a Patient-Centered Research Question.

Background

- Prior evidence: Empirical studies of intervention-outcome and studies related to mechanism
- Decision(s) that the research is intended to inform

A patient-centered research question specifies the following elements:

- Population of patients / research participants
- Intervention(s) relevant to patients in target population
- Comparator(s) relevant to patients in target population
- Outcomes meaningful to patients in target population
- Timing: outcomes and length of follow-up
- Setting and providers

Proposed Translation Framework Components

Every design has characteristics that must be balanced when developing a study to address a particular research question. For example, in order for the results to be obtained faster or to maximize external validity, one might conduct a study with less internal validity than an RCT. A study design without a comparator, based on information in a device registry, might be acceptable for

assessing device failure rates, but not to assess device effectiveness. A question about the effectiveness of a moderately toxic cancer therapy might put highest priority on minimizing bias and maximizing precision, which may require an RCT. Often, logistical issues can be more important than scientific ones, e.g. if only a limited number of patients are available to study, or if the data in existing datasets is not well suited for the question. The elements that should be assessed to inform choices and tradeoffs that will be made in selecting research designs and methodologies are captured by these proposed *translation framework components*.

Box 3. Proposed Translation Framework Components.

Intrinsic translation framework components

- Internal validity (bias)
- External validity (generalizability, or applicability to non-study settings and populations)
- Precision
- Heterogeneity in risk or benefit (e.g. subgroup or “personalized” evidence)
- Ethical dimensions of the study (including considerations of risk-benefit balance and study burden for study participants)

Extrinsic translation framework components

- Time urgency (e.g., rapidly changing technology, policy or public health needs)
- Logistical constraints (e.g., feasibility of collecting information from participants, number of participants available, study complexity)
- Data availability, quality and completeness

Prioritization of framework components is a key input into the translation framework and may affect the question itself. Policy makers, clinicians, researchers and patients may prioritize framework components differently. Hence, input is requested on how differing perspectives might be incorporated into the choice of study design.

The proposed framework components listed above may not apply perfectly to the wide range of research methods and questions comprising patient-centered outcomes research. The application of the translation framework to particular research domains may require customization or modification of the framework.

INFORMATION REQUESTED

We seek suggestions for the improvement of the draft framework components, thoughts on alternate components or frameworks, discussion of how these components apply to different research domains, and comments on the limitations of the proposed framework.

To fully explain and illustrate input and comments submitted, we encourage respondents to provide specific examples or case studies based on patient-centered research questions. Case studies should briefly describe the research question of interest and demonstrate application of the translation framework. The decisions about the design and methods used in patient-centered outcomes research require consideration of the tradeoffs in applying each translation framework component. Authors of case studies should discuss these decisions/tradeoffs from the perspective of at least one potential decision-maker or stakeholder.

Below is a partial list of broad research domains from which such examples or case studies might be drawn.

- 1) Studies on the safety and/or effectiveness of medications
- 2) Studies on the safety and/or effectiveness of vaccines
- 3) Studies on the safety and/or effectiveness of devices
- 4) Studies on the safety and/or effectiveness of behavioral therapies
- 5) Studies comparing two different treatment modalities, e.g. medication vs. device, medication vs. behavioral therapy, surgery vs. physical therapy etc.
- 6) Studies on the safety and/or effectiveness of surgical interventions
- 7) Studies on the safety and/or effectiveness of diagnostic tests
- 8) Studies on the safety and/or effectiveness of imaging strategies
- 9) Studies on the effectiveness of health promotion programs
- 10) Studies on the effectiveness of delivery system interventions
- 11) Modeling the effectiveness of therapies for populations beyond primary evidence

We are not seeking case studies on how to develop patient-centered research questions but rather, discussion of the tradeoffs between designs one would make based on preferences on the relative importance of framework components. For example, for a research question involving use of a new medical device, one would discuss the different designs that would be most appropriate/inappropriate based on prior evidence in this area and the relative importance of framework components (e.g., time urgency, precision, subgroup effects) to the decision-maker.

Case studies based on patient-centered research questions and supported by references and other documentation have the greatest chance of being used in the report.

Responses to this RFI will be considered for inclusion in the May 2012 report. Authors of case studies chosen for inclusion in the published report or of suggestions that result in qualitative modification of the framework will be publicly acknowledged.

HOW TO SUBMIT A RESPONSE

Responses to this RFI must be submitted electronically to <http://www.pcori.org/provide-input/translation-table/provide-information/> no later than **February 17, 2012 at 5:00 PM ET**.

All responses must include the names of its author(s), organization affiliation(s), contact email address(es), phone number(s) and conflict of interest declaration to be considered for inclusion in the May 2012 Methodology Report. Authors may be contacted for additional information.

INQUIRIES

Questions about this RFI should be submitted electronically to RMWG@pcori.org.

ABOUT PCORI POLICIES

PCORI intends to acknowledge consenting authors of detailed examples and case studies that are incorporated, in whole or in part, into the Methodology Report. PCORI will contact authors selected for acknowledgment in advance of publication.

Conflict of Interest – PCORI requires disclosure of any potential conflicts of interest, to be considered for acknowledgement in the published report.

DISCLAIMER

Response to this RFI is voluntary. Authors are responsible for obtaining any necessary permission to submit a response to this request. PCORI does not intend to make any awards for funding based on responses to this RFI or to otherwise pay for the preparation of any information submitted or for PCORI's use of such information. PCORI reserves the right to use information provided by respondents for any purpose deemed necessary and legally appropriate. Any individual(s) or organization(s) responding to this request should ensure that its response is complete and sufficiently detailed.