Response: Methods for Involving Patients in Topic Generation for PCOR

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Presentation goal

1. Consider how the proposed patient-oriented topic generation process can work in the US
2. Consider how to engage other constituencies
3. Consider who in the US is doing similar or related work to the proposed process modeled on UK's James Lind Alliance (JLA)
4. Discuss the acceptability and generalizability of the paper's recommendations

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Sources of my perspective—

KAISER PERMANENTE

EVIDENCE-BASED PRACTICE CENTERS

AHRQ
Agency for Healthcare Research and Quality
Advancing Excellence in Health Care www.ahrq.gov

The Center for Health Research

U.S. Preventive Services Task Force

CTSA Clinical & Translational Science Awards

CESR
Kaiser Permanente Center for Effectiveness & Safety Research

Effective Health Care Program
Helping You Make Better Treatment Choices

Oregon EPC

pcori
PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Overall comments

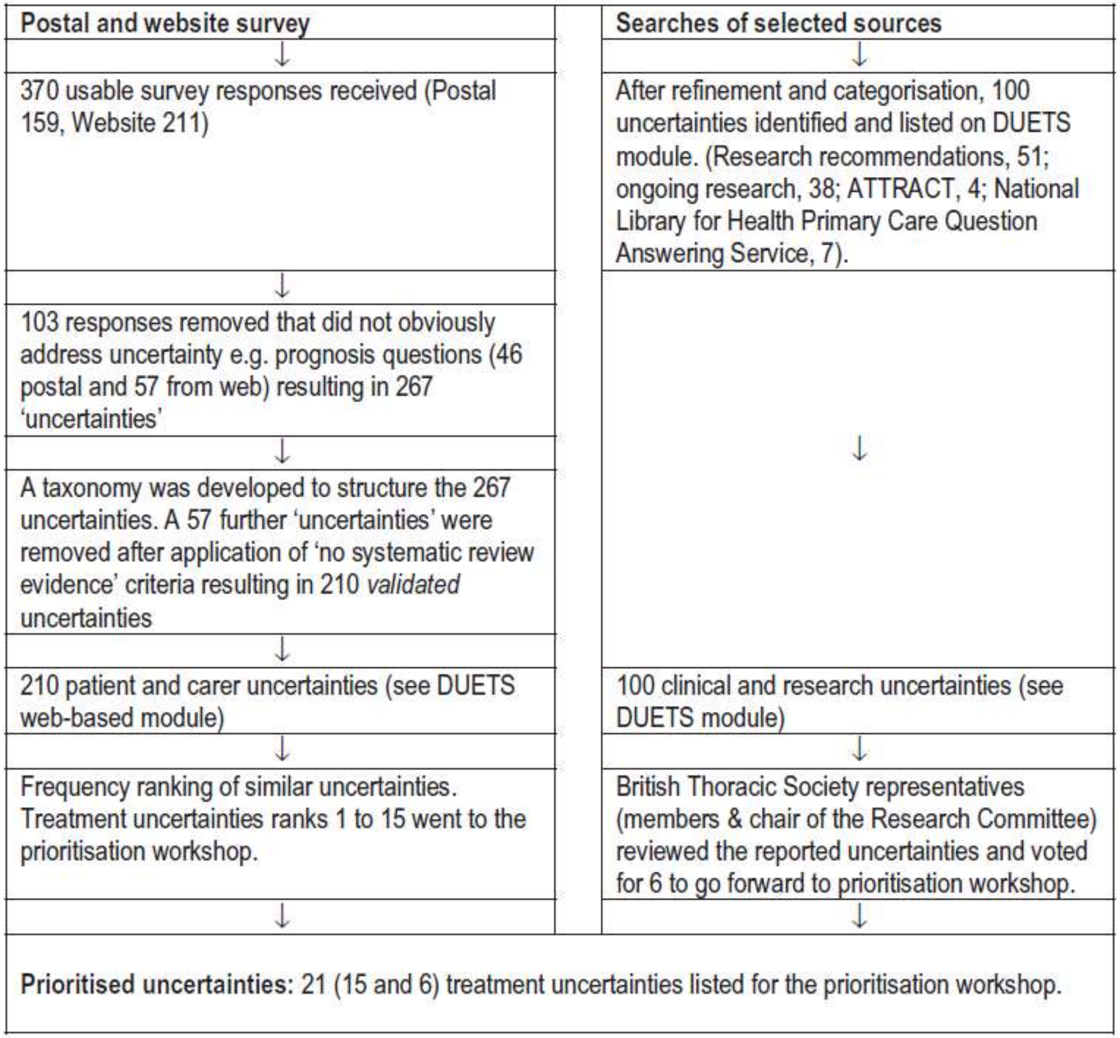
- Challenging task
- Strong, clear focus on eliciting and maintaining the patient's voice, incorporating health experience, addressing disparities
- The international perspective offers valuable lessons for this relatively new area in the US
- The US context is more complex, since there is no "unifying" perspective

  (i.e., no national health budget, health authority, or health care system)

Proposed PCORI process of public engagement in topic generation

- Proposed 6-phase process for lay members-clinicians to form priority setting partnerships (PSP) which generate & select research topics (Figure 2)
- Public = patients, families, carers, advocates, organizations
- The proposed PCORI-PSP is a hub for eliciting broader peer input, capturing health experience research, employing health disparities analyses, & considering systematic reviews to produce research themes & topics

Figure 1. The JLA Asthma WP process to identify treatment uncertainties. DUETs, Database of Uncertainties about the Effects of Treatments; JLA, James Lind Alliance; WP, Working Partnership.



Postal and website survey

↓

370 usable survey responses received (Postal 159, Website 211)

↓

103 responses removed that did not obviously address uncertainty e.g. prognosis questions (46 postal and 57 from web) resulting in 267 'uncertainties'

↓

A taxonomy was developed to structure the 267 uncertainties. A 57 further 'uncertainties' were removed after application of 'no systematic review evidence' criteria resulting in 210 *validated* uncertainties

↓

210 patient and carer uncertainties (see DUETS web-based module)

↓

Frequency ranking of similar uncertainties. Treatment uncertainties ranks 1 to 15 went to the prioritisation workshop.

↓

Searches of selected sources

↓

After refinement and categorisation, 100 uncertainties identified and listed on DUETS module. (Research recommendations, 51; ongoing research, 38; ATTRACT, 4; National Library for Health Primary Care Question Answering Service, 7).

↓

100 clinical and research uncertainties (see DUETS module)

↓

British Thoracic Society representatives (members & chair of the Research Committee) reviewed the reported uncertainties and voted for 6 to go forward to prioritisation workshop.

↓

Prioritised uncertainties: 21 (15 and 6) treatment uncertainties listed for the prioritisation workshop.

Elwyn G, Crowe S, Fenton M, Firkins L, Versnel J, Walker S, Cook I, Holgate S, Higgins B, Gelder C. Identifying and prioritizing uncertainties: patient and clinician engagement in the identification of research questions. J Eval Clin Pract. 2010 Jun;16(3):627-31.

1. How might this PCORI-PSP approach work in the US?

- Partnerships in the UK are built on professional societies and advocacy groups (e.g., Asthma UK and British Thoracic Society) http://www.lindalliance.org/
- Similar partnerships could be encouraged or supported in the US
- Ideally, such a process could also enhance clinical practice guideline development adhering to recent standards—including use of high-quality systematic reviews[1]

1. Clinical Practice Guidelines We can Trust 2011. The National Academy Press, 2011

Proposed PCORI-PSPs—considerations

- Expectations should be realistic
  - (JLA has 16 condition-specific partnerships since 2004; 8 have published research priorities)
- Use of existing systematic reviews is key—
  - as may be involvement of systematic review groups
- Experience suggests predictable challenges
  - engaging clinicians, moving beyond advocates, & need for structured support
- Providing complementary activities may be part of success
  - multiple mechanisms of patient engagement in research & evidence-based health inquiry exists in the UK (e.g., DUETS)

2. What about engaging other constituencies?

- PCORI specified including others in lay & clinical communities, researchers
- Multiple opportunities and avenues for access could increase engagement of the “public” and these others
    - e.g., web nominations, exploration forums, networking
- Other perspectives (e.g., health systems or purchasers/payers) further complement condition-focused PSP
    - e.g., IOM top priorities for health care quality transformation (2003):
      12 disease/health conditions, 4 population-specific, 2 intervention-specific,
      2 cross-cutting

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How James Lind Alliance (JLA) priorities for asthma compare with other solicitations:

- 10 JLA PSP Priorities for Asthma
  - 226 asthma-specific questions
  - Patient advocates-clinicians

- AHRQ’s Effective Health Care Program
  - 451 nominations: 5 asthma or cross-cutting health conditions
  - Public (website, topic exploration forums)

- Integrated health care delivery system
  - 310 nominations: 8 asthma or chronic disease management
  - Clinician/health system leaders (web-based survey)

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Patient-clinician priorities
Public nominations
Clinical/health system leaders topics

- Adverse **effects of medications** in adults ✓✓✓ *
- Adverse **effects of medications** in children ✓
- **Comorbidity** and asthma management ✓✓
- Self-management
- **Education to manage** adverse effects of medications ✓ ✓ *
- Managing allergy triggers
- Role of **complementary therapies** ✓
- **Breathing exercises** ✓
- **Education for asthma control** ✓✓
- Asthma **care management** approaches ✓✓ ✓✓✓✓ *
- Psychological interventions for adults

* top 50

Considerations in comparing approaches

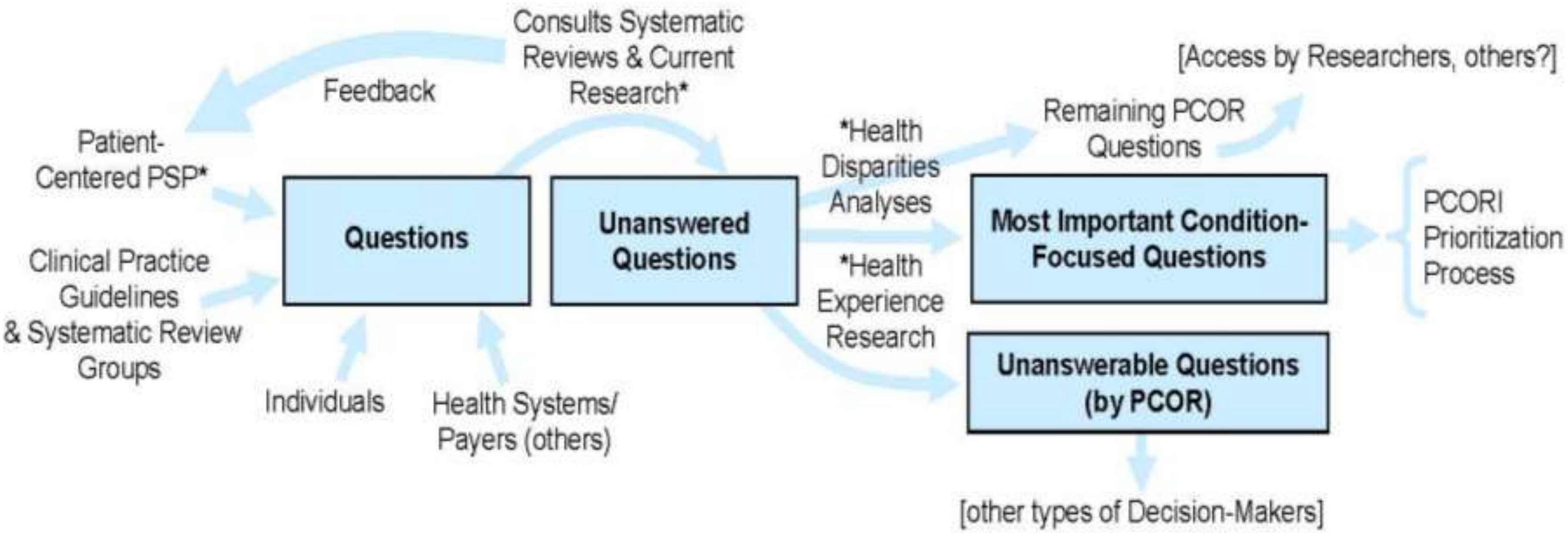
- Some unique, many complementary topics- reflecting dissemination and new research

- Responses are framed by the questions, as well as the nominator's perspective:
  - "Uncertain treatment effects" (JLA)
  - "Future research on tests or treatments"
  - "Important comparative effectiveness/safety research"
  - "Studies to make your life better"

- Will PCORI want "information needs" or ?

Building other constituencies into a PCORI-PSP* type approach

Expanding Condition-Specific PCOR Topic Generation



Consults Systematic Reviews & Current Research*

Feedback

Patient-Centered PSP*

Clinical Practice Guidelines & Systematic Review Groups

Questions

Unanswered Questions

Individuals

Health Systems/ Payers (others)

*Health Disparities Analyses

*Health Experience Research

[Access by Researchers, others?]

Remaining PCOR Questions

Most Important Condition-Focused Questions

PCORI Prioritization Process

Unanswerable Questions (by PCOR)

[other types of Decision-Makers]

*Proposed PCORI-Priority Setting Partnerships (PSP)

Recommendations for engaging other constituencies and perspectives

- Construct a system for patient-engagement that interrelates patient needs with those of other constituencies
  - one that connects more broadly to health research and health care improvement

- Connect with existing types of initiatives (beyond professional societies and advocacy groups)
  - query/inquiry systems (e.g., Hayes, ECRI)
  - quality Improvement initiatives (e.g., VA QUERI, primary care network)
  - learning networks (e.g., Medicaid Medical Directors)
  - researcher-community partnerships (e.g., Community-based participatory research initiatives)
  - community initiatives around health disparities

- Open up opportunities for “just in time” participation
  - (i.e., make it easy to do the right thing at the right time)

Who does similar or related work in the US?

Effective Health Care Program

Helping You Make Better Treatment Choices

Participate in activities featuring EHC Program research and publications

New! Patient Decision Aids

Explore Your Treatment Options

Share Your Success Stories!

See What's New in CME/CE

Measuring Your Blood Pressure at Home

Now Open for Comment

Draft Documents and White Papers

Feb. 7–Mar. 6, 2012
Future Research Needs for Diagnosis of Obstructive Sleep Apnea

Feb. 7–Mar. 6, 2012
Closing the Quality Gap Series: Prevention of Health Care-Associated Infections

Feb. 9–Mar. 8, 2012
Future Research Needs for

News and Announcements

Product Announcements

Mar. 2, 2012
Interventions to Modify Healthcare Provider Adherence to Asthma Guidelines -- Research Protocol

Feb. 29, 2012
Fecal DNA Testing in Screening for Colorectal Cancer in Average Risk Adults -- Final Research Review

Feb. 29, 2012
New! Spanish Consumer Summary on Comparative Effectiveness of

Most Popular

Researcher Tools

Overview
Methods Guide for Effectiveness and Comparative Effectiveness Reviews

Research Report - Final
Registries for Evaluating Patient Outcomes: A User's Guide: 2nd Edition

Most Viewed This Week

Consumer Summary

3. Who else is doing similar or related work to the JLA in the US?

- Engaging patients or consumer groups: Consumers United for Evidence; NCI/other NIH institutes; CTSA Community Engagement components; CBPR initiatives individual advocacy groups
- Providing access to evidence-based information: NLM; Consumers Union
- Conducting systematic reviews: EPC program; Cochrane review groups
- Undoubtedly large number of others not listed here

4. How generalizable & applicable is the proposed PCORI-PSP approach to the US?

- The recommended approach is condition-focused (e.g., asthma) while the PCORI draft research priorities are not
- The recommended approach may not produce rapid results or be broad-based enough
- Without infrastructure development and an overall, comprehensive system, the recommended approach may not produce similar results
- Important to ensure that the recommended partnership structure is that most valued by US patients

What else might be needed to apply the proposed PCORI-PSP approach to the US?

- The paper lays out principles, processes, methods for patient engagement
- Considering the motivation for participation in the US
  - meeting people where they are
  - maximizing the impact of their expertise
  - meeting their immediate needs
  - respecting their time and investment

Conclusions

- The Nass, Levine, Yancy paper has identified a promising best practice (JLA PSP) & lays out important considerations, methods, and processes for engaging patients in generating PCOR topics
- The prototype may need more modification to apply to the US than suggested in the paper
- Complementing this approach and integrating it into a broader system could be important

Comments & Questions

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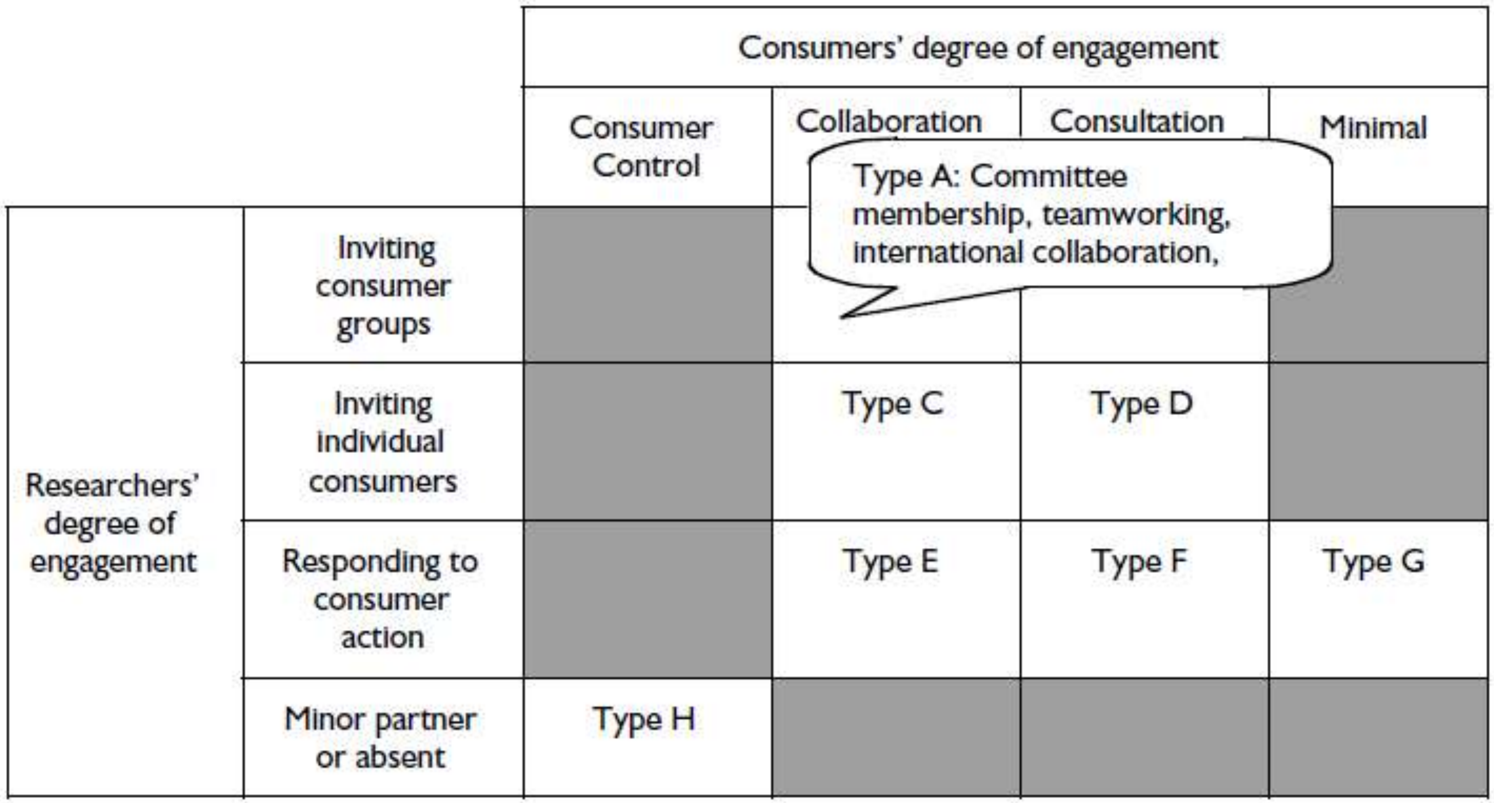
Figure 3. Framework for describing Type A consumer involvement in research agenda setting: inviting consumer group involvement through collaboration



		Consumers' degree of engagement			
		Consumer Control	Collaboration	Consultation	Minimal
Researchers' degree of engagement	Inviting consumer groups	▨	Type A: Committee membership, teamworking, international collaboration,		▨
	Inviting individual consumers	▨	Type C	Type D	▨
	Responding to consumer action	▨	Type E	Type F	Type G
	Minor partner or absent	Type H	▨	▨	▨

Patient-clinician priorities
Public nominations
Clinical/health system leaders topics

- Adverse **effects of medications** in adults **(CE of diff treatments-3 topics)* top 50**
- Adverse **effects of medications** in children **(CE of diff treatments)**
- **Comorbidity** and asthma management (comorbid asthma-2 topics)
- Self-management
- **Education to manage** adverse effects of medications (literacy impacts) **(pt-centered approaches to medication adherence)* top 50**
- Managing allergy triggers
- Role of **complementary therapies** (Buteyko breathing)
- **Breathing exercises** (Buteyko breathing)
- **Education for asthma control** (literacy; interventions to modify adherence)
- Asthma **care management** approaches (literacy; adherence interventions) **(CE of asthma care management/health system alternatives-3 topics) (pt-centered med adherence) * top 50**
- Psychological interventions for adults

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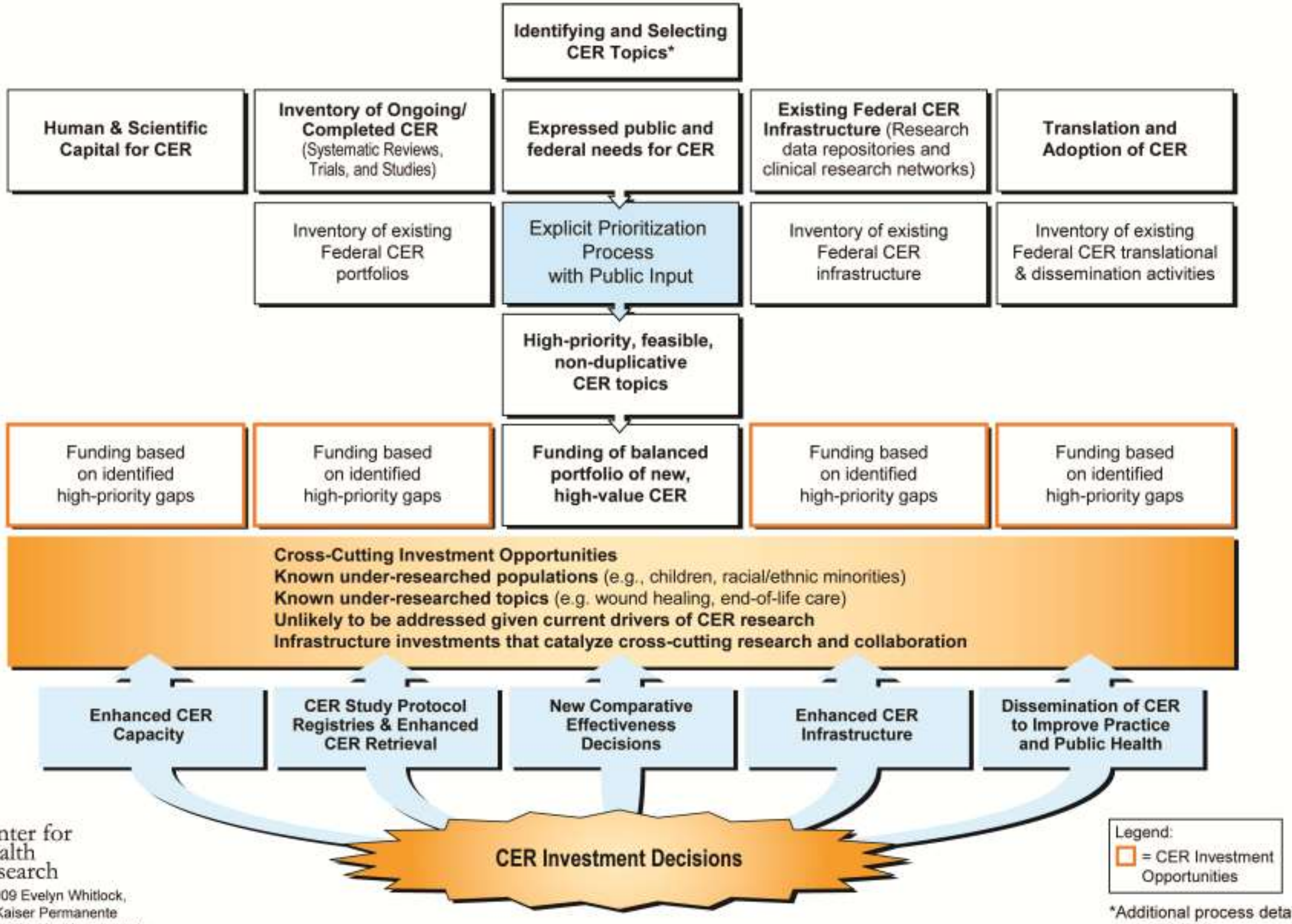
Overview of Framework for Coordinated, Comprehensive Federal Investments in CER



Identifying and Selecting CER Topics*

Human & Scientific Capital for CER

Inventory of Ongoing/ Completed CER (Systematic Reviews, Trials, and Studies)

Expressed public and federal needs for CER

Existing Federal CER Infrastructure (Research data repositories and clinical research networks)

Translation and Adoption of CER

Inventory of existing Federal CER portfolios

Explicit Prioritization Process with Public Input

Inventory of existing Federal CER infrastructure

Inventory of existing Federal CER translational & dissemination activities

High-priority, feasible, non-duplicative CER topics

Funding based on identified high-priority gaps

Funding based on identified high-priority gaps

Funding of balanced portfolio of new, high-value CER

Funding based on identified high-priority gaps

Funding based on identified high-priority gaps

Cross-Cutting Investment Opportunities
Known under-researched populations (e.g., children, racial/ethnic minorities)
Known under-researched topics (e.g. wound healing, end-of-life care)
Unlikely to be addressed given current drivers of CER research
Infrastructure investments that catalyze cross-cutting research and collaboration

Enhanced CER Capacity

CER Study Protocol Registries & Enhanced CER Retrieval

New Comparative Effectiveness Decisions

Enhanced CER Infrastructure

Dissemination of CER to Improve Practice and Public Health

CER Investment Decisions

Legend:
☐ = CER Investment Opportunities

*Additional process details supplied in Figure 1

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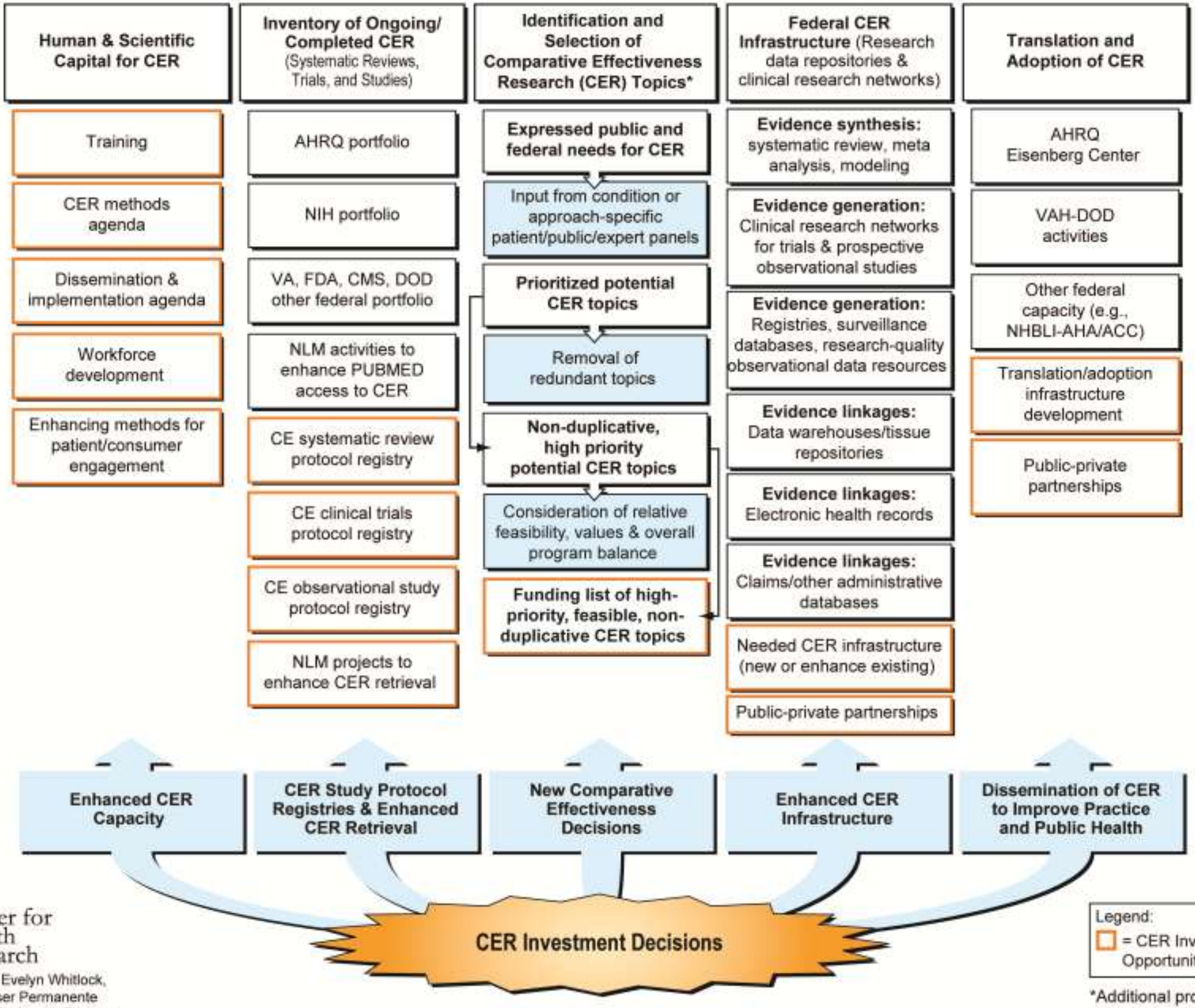
Framework for Coordinated, Comprehensive Federal Investments in CER



Human & Scientific Capital for CER

- Training
- CER methods agenda
- Dissemination & implementation agenda
- Workforce development
- Enhancing methods for patient/consumer engagement

Inventory of Ongoing/ Completed CER (Systematic Reviews, Trials, and Studies)

- AHRQ portfolio
- NIH portfolio
- VA, FDA, CMS, DOD other federal portfolio
- NLM activities to enhance PUBMED access to CER
- CE systematic review protocol registry
- CE clinical trials protocol registry
- CE observational study protocol registry
- NLM projects to enhance CER retrieval

Identification and Selection of Comparative Effectiveness Research (CER) Topics*

- **Expressed public and federal needs for CER**
- Input from condition or approach-specific patient/public/expert panels
- **Prioritized potential CER topics**
- Removal of redundant topics
- **Non-duplicative, high priority potential CER topics**
- Consideration of relative feasibility, values & overall program balance
- **Funding list of high-priority, feasible, non-duplicative CER topics**

Federal CER Infrastructure (Research data repositories & clinical research networks)

- **Evidence synthesis:** systematic review, meta analysis, modeling
- **Evidence generation:** Clinical research networks for trials & prospective observational studies
- **Evidence generation:** Registries, surveillance databases, research-quality observational data resources
- **Evidence linkages:** Data warehouses/tissue repositories
- **Evidence linkages:** Electronic health records
- **Evidence linkages:** Claims/other administrative databases
- Needed CER infrastructure (new or enhance existing)
- Public-private partnerships

Translation and Adoption of CER

- AHRQ Eisenberg Center
- VAH-DOD activities
- Other federal capacity (e.g., NHBLI-AHA/ACC)
- Translation/adoption infrastructure development
- Public-private partnerships

Enhanced CER Capacity

CER Study Protocol Registries & Enhanced CER Retrieval

New Comparative Effectiveness Decisions

Enhanced CER Infrastructure

Dissemination of CER to Improve Practice and Public Health

CER Investment Decisions

Legend:
🟧 = CER Investment Opportunities

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Primary Priority Conditions

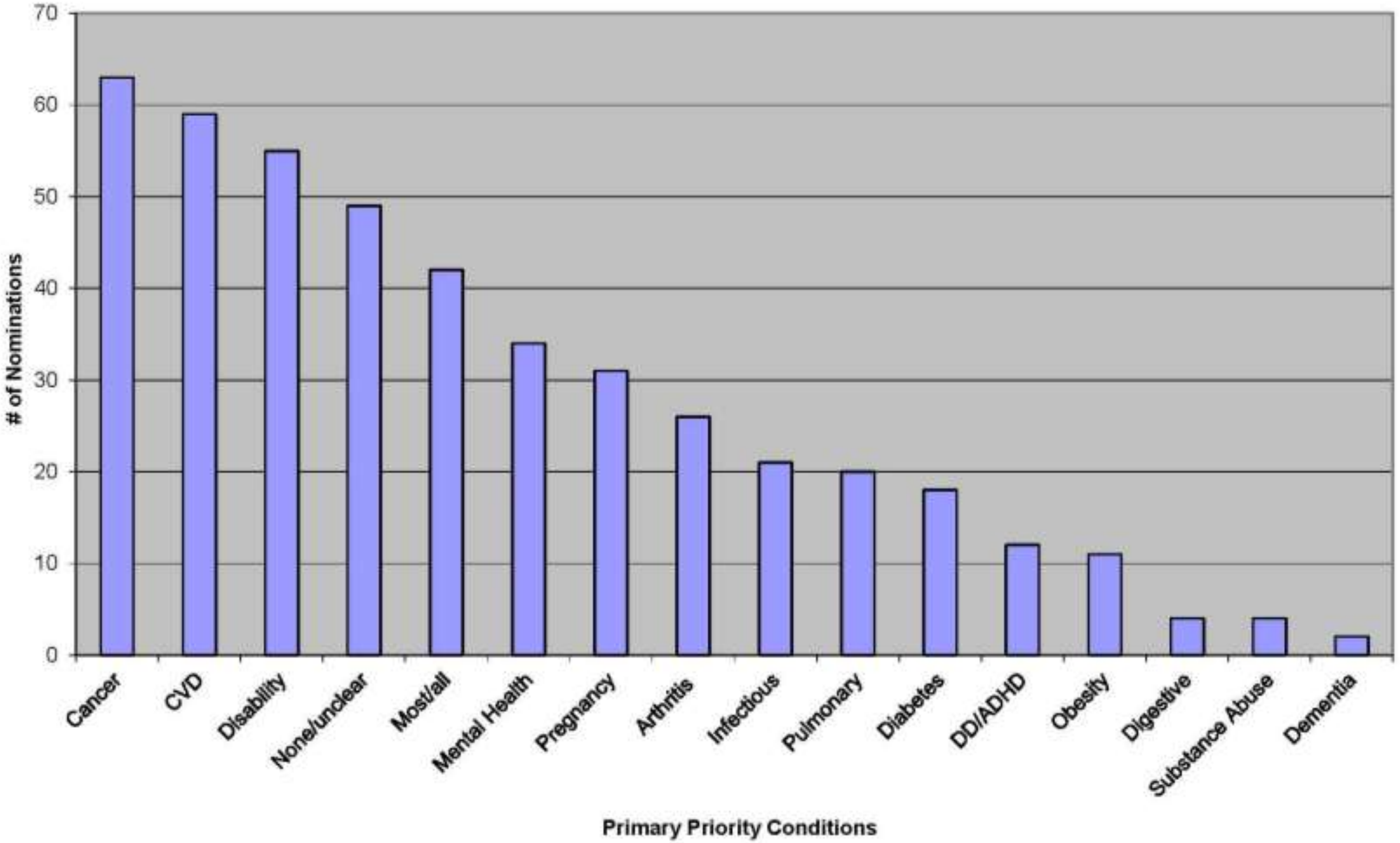

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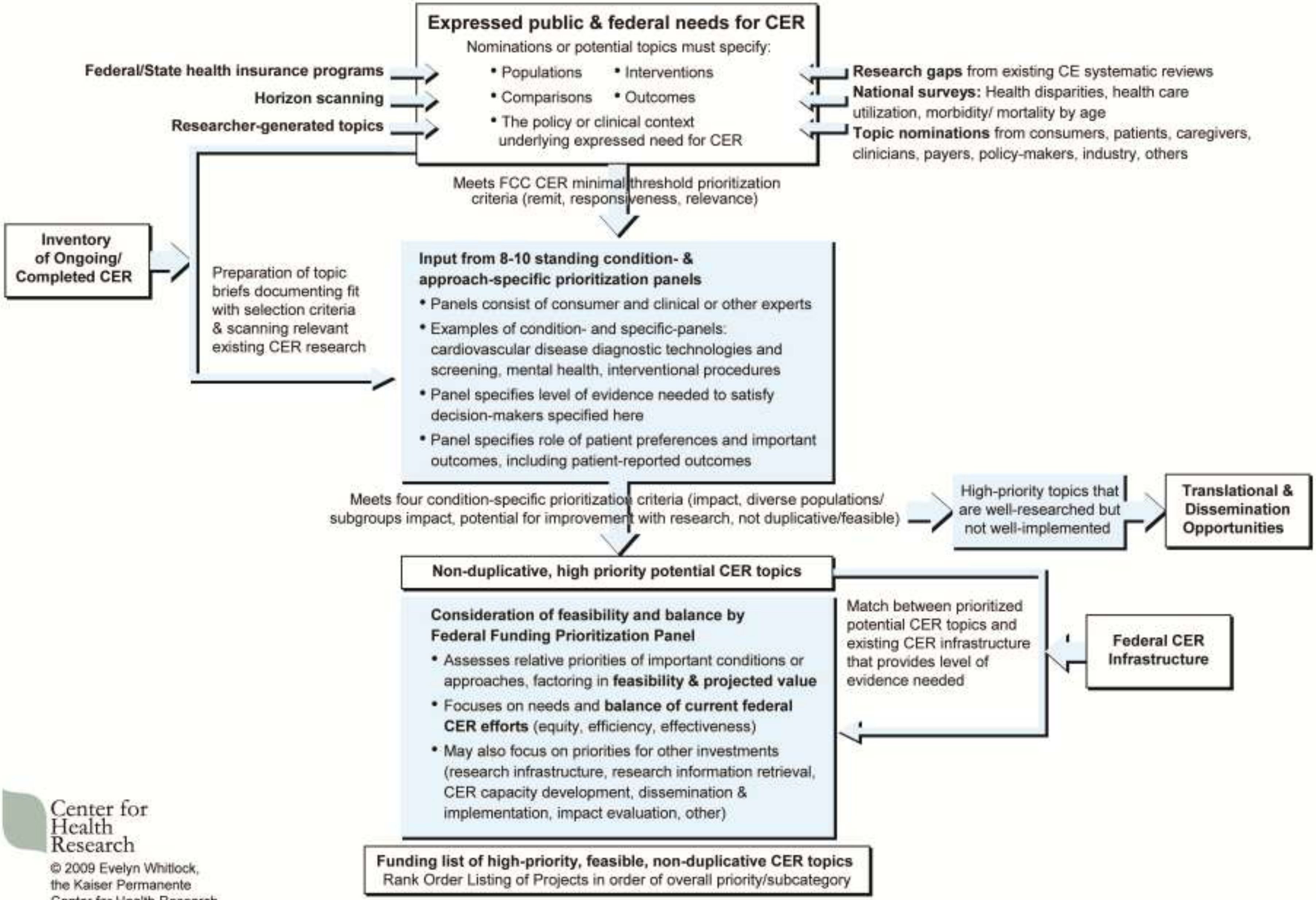
Figure 1. Identification & Selection (Prioritization Process) of Comparative Effectiveness Research (CER)



Federal/State health insurance programs

Horizon scanning

Researcher-generated topics

Expressed public & federal needs for CER

Nominations or potential topics must specify:

- Populations
- Interventions
- Comparisons
- Outcomes
- The policy or clinical context underlying expressed need for CER

Research gaps from existing CE systematic reviews

National surveys: Health disparities, health care utilization, morbidity/ mortality by age

Topic nominations from consumers, patients, caregivers, clinicians, payers, policy-makers, industry, others

Meets FCC CER minimal threshold prioritization criteria (remit, responsiveness, relevance)

Inventory of Ongoing/ Completed CER

Preparation of topic briefs documenting fit with selection criteria & scanning relevant existing CER research

Input from 8-10 standing condition- & approach-specific prioritization panels

- Panels consist of consumer and clinical or other experts
- Examples of condition- and specific-panels: cardiovascular disease diagnostic technologies and screening, mental health, interventional procedures
- Panel specifies level of evidence needed to satisfy decision-makers specified here
- Panel specifies role of patient preferences and important outcomes, including patient-reported outcomes

Meets four condition-specific prioritization criteria (impact, diverse populations/ subgroups impact, potential for improvement with research, not duplicative/feasible)

High-priority topics that are well-researched but not well-implemented

Translational & Dissemination Opportunities

Non-duplicative, high priority potential CER topics

Consideration of feasibility and balance by Federal Funding Prioritization Panel

- Assesses relative priorities of important conditions or approaches, factoring in **feasibility & projected value**
- Focuses on needs and **balance of current federal CER efforts** (equity, efficiency, effectiveness)
- May also focus on priorities for other investments (research infrastructure, research information retrieval, CER capacity development, dissemination & implementation, impact evaluation, other)

Match between prioritized potential CER topics and existing CER infrastructure that provides level of evidence needed

Federal CER Infrastructure

Funding list of high-priority, feasible, non-duplicative CER topics

Rank Order Listing of Projects in order of overall priority/subcategory

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PCORI research priorities

1. **Assessment of Prevention, Diagnosis and Treatment Options** - Comparing the:
    - effectiveness and safety of alternative prevention, diagnosis, and treatment options to
    - see which ones work best for different people with **a particular health problem.**
2. **Improving Healthcare Systems** - Comparing health system-level approaches to improving access, supporting patient self-care, innovative use of health information technology, coordinating care for complex conditions, and deploying workforce effectively. Health systems (fed and nonfed)
3. **Communication and Dissemination Research** - Comparing approaches to providing comparative effectiveness research information and supporting shared decision-making between patients and their providers.
4. **Addressing Disparities** - Identifying potential differences in prevention, diagnosis or treatment effectiveness, or preferred clinical outcomes across patient populations and the healthcare required to achieve best outcomes in each population.
5. **Accelerating Patient-Centered Outcomes Research and Methodological Research** - Improving the nation’s capacity to conduct patient-centered outcomes research, by building data infrastructure, improving analytic methods, and training

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Other resources in the UK

- The **James Lind Alliance Guidebook**: an evidence-based guide to working with patients, carers and clinicians to set priorities for health research. Contains a range of good practice examples, tools, templates and other resources. www.JLAguidebook.org.
- The **British Medical Association's Patient Liaison Group Glossary** produced to help patients and carers understand the roles of healthcare professionals – who work in the NHS. www.bma.org.uk/patients_public/whos_who_healthcare/index.jsp
- **Current Controlled Trials** allows users to search, register and share information about randomised controlled trials. www.controlled-trials.com
- **DUETs (Database of Uncertainties about the Effects of Treatments)** has been established to identify and publish patients' and clincians' questions about the effects of treatments which cannot be answered by referring to up-to-date systematic reviews of existing research evidence. www.library.nhs.uk/DUETs
- **Healthtalkonline - Clinical Trials** is the gateway to many video and audio interviews with patients about their experience of clinical trials. www.healthtalkonline.org/medical_research/clinical_trials
- **Healthtalkonline –** provides videos and audios about patients' health experiences
- **INVOLVE** promotes and supports active public involvement in NHS, public health and social care research.www.invo.org.uk
- The **James Lind Library** has been created to help people understand fair tests of treatments in health care by illustrating how fair tests have developed over the centuries. www.jameslindlibrary.org
- The **NHS Evidence** service provides easy access to a comprehensive evidence base for everyone in health and social care who takes decisions about treatments or the use of resources.www.evidence.nhs.uk
- The **National Institute for Health Research (NIHR)** commissions and funds NHS, social care and public health research that is essential for delivering its responsibilities in public, health and personal social services. Its role is to develop the research evidence to support decision making by professionals, policy makers and patients, make this evidence available, and encourage its uptake and use. www.nihr.ac.uk
- **NIHR Evaluation, Trials and Studies Coordinating Centre (NETSCC)** is home to a growing number of research programmes and is part of the NIHR. It manages the following research programmes: Efficacy and Mechanism Evaluation; Health Services and Delivery Research; Health Technology Assessment; and Public Health Research. www.netscc.ac.uk
- **People in Research** aims to help members of the public make contact with organisations that want to actively involve people in clinical research. It has been developed by the UK Clinical Research Collaboration, INVOLVE and others.www.peopleinresearch.org
- The **Royal Society of Medicine** (RSM) aims to provide a broad range of educational activities and opportunities for doctors, dentists, and veterinary surgeons, including students of these disciplines; and allied health-care professionals. www.rsm.ac.uk