Stakeholder and Community Engagement in Patient-Centered Comparative Effectiveness Research

Thomas Concannon, PhD

September 28, 2016
Clinical and Translational Science Awards (CTSA) Program

- National Institutes of Health (NIH) program
- Launched in 2006
- A national consortium of 64 institutions
- **Mission:** to develop innovative solutions that will improve the efficiency, quality and impact of the process for turning observation in the laboratory, clinic and community into interventions that improve the health of individuals and the public
Spectrum of Clinical and Translational Research

Bench to Bedside…

...to Widespread Clinical Practice…

...to Public Health…

...to Health Policy

Translation (T1)  Translation (T2)  Translation (T3)  Translation (T4)
Tufts CTSI’s Mission & Purpose

Established in 2008 to translate research into better health

- Stimulate and expedite innovative clinical and translational research, with the goal of improving the public’s health
- *Entire spectrum* of clinical and translational research is critical to meeting the promise and the public’s needs of biomedical science
39 Tufts CTSI Partners

13 Tufts Schools & Centers
Cummings School of Veterinary Medicine
Fletcher School of Law & Diplomacy
Friedman School of Nutrition Science & Policy
Graduate School of Arts & Sciences
Institute for Clinical Research & Health Policy Studies at Tufts Medical Center
Jean Mayer USDA Human Nutrition Research Center on Aging
Sackler School of Graduate Biomedical Sciences
School of Dental Medicine
School of Engineering
School of Medicine
Tisch College of Citizenship & Public Service
Tufts Center for the Study of Drug Development
Tufts Innovation Institute

7 Tufts-Affiliated Hospitals
Baystate Medical Center
Lahey Clinic
Maine Medical Center
New England Baptist Hospital
Newton-Wellesley Hospital
St. Elizabeth’s Medical Center
Tufts Medical Center

10 Community-Based Partners
Action for Boston Community Development (ABCD)
Asian Community Development Corporation
Asian Task Force Against Domestic Violence
Asian Women for Health
Boston Chinatown Neighborhood Center
Center for Information and Study on Clinical Research Participation
Greater Boston Chinese Golden Age Center
Health Resources in Action
Museum of Science, Boston
New England Quality Care Alliance

6 Industry/Non-Profit Partners
Blue Cross Blue Shield of Massachusetts
Eli Lilly and Company
Institute for Systems Biology and P4 Medicine Institute
Minuteman Health Network
Pfizer, Inc.
Tufts Health Plan

3 Academic Partners
Brandeis University
Northeastern University
RAND Corporation
How Can CTSI Help?

• Connections with other researchers, industry, the community, and policy-makers across the Tufts CTSI network and national CTSA consortium via our Navigators & Research Collaboration team.

• Consultations on comparative effectiveness, one health, research process improvement and stakeholder and community engagement projects and grants, as well as regulatory issues and other areas of translation.

• Study design and data analysis (pre- and post-award) through the Biostatistics, Epidemiology, and Research Design (BERD) Center, including drop-in sessions.
How Can CTSI Help?

- 24/7 clinical trial support through our Clinical and Translational Research Center (CTRC).
- Informatics tools for electronic data capture (REDCap), resource sharing, and collaboration.
- Training & professional development including MS and PhD degrees, certificate programs, seminars & workshops, and paid career development awards and fellowships.
- Funding through one-year interdisciplinary pilot studies grants that support the initial stages of research.
How to Request Tufts CTSI Services

• Visit www.tuftsctsi.org and submit a request
Live seminars are recorded for our I LEARN site. Seminar videos can be viewed at any time, and are free!

Welcome to I LEARN, the Tufts CTSI interactive education website. Tufts CTSI I LEARN is a new resource that offers a comprehensive library of educational courses in clinical and translational research for both professional development and CME credit. Building on our unique curriculum, we are offering some of our courses and workshops in an online learning format, combining professionally videotaped recordings of live lectures with other learning materials to transcend the traditional in-classroom experience.
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- Issued every Monday at 8AM
- Sign up on our website or at http://eepurl.com/C4d9X
For more information: www.tuftsctsi.org
Questions?
Stakeholder and Community Engagement in Patient-Centered Comparative Effectiveness Research

Thomas Concannon, PhD
September 28, 2016
Learning Objectives

At the end of this talk, you will be able to describe

• a rationale for engaging stakeholders
• successful frameworks for identifying and recruiting stakeholders, and
• basic principles for planning engagement activities
• how well-intentioned but poorly planned engagements can go wrong
Motivation (a Success Story)

‘This is only Round One. Lower the price or we’ll escalate.’

“After [the demonstration], they buckled and lowered the price by 20%. From then on, the industry said it’s probably smarter to try to talk to [activists] and placate them as much as we can.”

Six months later, the FDA reduces the standard dose by half.

-Peter Staley on ACT-UP demonstrations in response to the $10,000/year price of AZT.

Peter Staley, 1989
ACT UP emerged in 1987

In the first decade of the pandemic, people living with HIV faced

• Devastating illness
• High mortality rates: 31 in 1981 and 18,447 in 1990
• Inaction of most policy makers
• Grindingly slow pace of research

ACT UP was founded to take “direct action to end the AIDS crisis.”
Its members went everywhere...

No corner of health care was off limits
Members engaged with health care decision makers in:

- Government
- Industry
- Insurance
- Employment

And they became self-taught experts in:

- Drug development and markets
- Virology, immunology, biostatistics
- Regulatory affairs
...and achieved dramatic change

- Drug marketing and pricing (Burroughs Wellcome)
- Accelerated drug development (NIH & FDA)
- Alternatives to strict placebo control (NIH & FDA)
- Community research initiatives (NIH & AHCPR-now AHRQ)
- Health care delivery (HHS—Ryan White Care Act)
- Updated definition of AIDS (CDC, NIH)
HIV treatment is a home-run for drug therapy

ACT UP became part of the success story that culminated in discovery, development, and rapid uptake of effective treatment for millions of people in the US and across the globe.
What’s the upshot?

ACT UP became *part of the success story* that culminated in discovery, development, and rapid uptake of effective treatment for millions of people in the US and across the globe.
Twenty years later: stakeholder engagement reaches academic and industry research

- New funding for CER (ARRA and ACA 2008-present)
- Requirements for patient and other stakeholder engagement
- PCORI has developed detailed guidance on engagement
- PCORI guidance has influenced funding from AHRQ, NIH and other HHS agencies
“This is hard and I don’t know if it’s worth it.”
If researchers engage patients and other stakeholders, will there be more success stories?
Roadmap

1. Motivation (ACT UP)
2. A framework for engagement
3. How well are researchers doing?
4. Motivation (2009 remix)
5. What have we learned?
Roadmap

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A New Taxonomy for Stakeholder Engagement in Patient-Centered Outcomes Research

Thomas Concannon, Paul Meissner, Jo Anne Grunbaum, Newell McElwee, Jeanne-Marie Guise, John Santa, Patrick Conway, Denise Daudelin, Elaine Morrato, Laurel Leslie
Guiding Questions

1. Who is a CER stakeholder?

2. What kind of research qualifies as CER?

3. How can researchers start engaging stakeholders?
Definitions

**Stakeholder** – An individual or group who is responsible for or affected by health- and healthcare-related decisions that can be informed by research evidence.

**Stakeholder Engagement** – A bi-directional and sustained relationship between stakeholder and researcher that results in informed decision-making about the selection, conduct and use of research.
Definitions

**Stakeholder** – An individual or group who is responsible for or affected by health- and healthcare-related decisions that can be informed by research evidence.

**Stakeholder Engagement** – A bi-directional and sustained relationship between stakeholder and researcher that results in informed decision-making about the selection, conduct and use of research.
Introduces Three Items

1. 7Ps Framework – types of stakeholders
2. 6 Stage Model of CER – stages of research
3. A Plan-Do-Study-Act approach to stakeholder and community engagement
<table>
<thead>
<tr>
<th>Category</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients and Public</td>
<td>Current and potential consumers of patient-centered health care and population focused public health, their caregivers, families and patient and consumer advocacy organizations.</td>
</tr>
<tr>
<td>Providers</td>
<td>Individuals (e.g. nurses, physicians, mental health counselors, pharmacists, and other providers of care and support services) and organizations (e.g. hospitals, clinics, community health centers, community based organizations, pharmacies, EMS agencies, skilled nursing facilities, schools) that provide care to patients and populations.</td>
</tr>
<tr>
<td>Purchasers</td>
<td>Employers, the self-insured, government and other entities responsible for underwriting the costs of health care.</td>
</tr>
<tr>
<td>Payers</td>
<td>Insurers, Medicare and Medicaid, state insurance exchanges, individuals with deductibles, and others responsible for reimbursement for interventions and episodes of care.</td>
</tr>
<tr>
<td>Policy Makers</td>
<td>The White House, Department of Health and Human Services, Congress, states, professional associations, intermediaries, and other policy-making entities.</td>
</tr>
<tr>
<td>Product Makers</td>
<td>Drug and device manufacturers</td>
</tr>
<tr>
<td>Principal Investigators</td>
<td>Other researchers and their funders</td>
</tr>
</tbody>
</table>
The Six Stage Model of CER

Translational Spectrum of Comparative Effectiveness Research at Tufts CTSI

Evidence Prioritization
(Qualitative / Quantitative Identification)

Evidence Generation
(Randomized Control Trials, Registries, Observations, Non-Trials)

Evidence Synthesis
(Systematic Reviews, Meta Analysis)

Evidence Interpretation and Integration
(Cost-Effectiveness Analysis, Decision Analysis)

Dissemination and Application
(Guidelines, Policy, Social Sciences, Implementation Science)

Feedback and Assessment
(Qualitative Evaluation, Data Monitoring, Quality Monitoring, Reassessment)
Roadmap

1. Motivation (ACT UP)
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3. How well are researchers doing?
4. Motivation (2009 remix)
5. What have we learned?
Research Question

What are researchers *reporting about engagement*?
A systematic review of what we (researchers) have been publishing

Four steps:

1. Describe what we want to know
2. Search and screen literature
3. Extract data
4. Evaluate what we find
## Stakeholders

<table>
<thead>
<tr>
<th>Category Subcategory</th>
<th>Name</th>
<th>Organization</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients and the public</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Consumer</td>
<td>Grant P. Thompson</td>
<td>Consumer Reports Panel</td>
</tr>
<tr>
<td>Consumer</td>
<td>Gerald Rasmussen</td>
<td>Consumer Reports Panel</td>
</tr>
<tr>
<td>Providers</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Provider group</td>
<td>Judy Bradford</td>
<td>Fenway Institute/Fenway Health</td>
</tr>
<tr>
<td>Payers and Purchasers</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Self-insured employer</td>
<td>Lawrence Becker</td>
<td>Xerox</td>
</tr>
<tr>
<td>Policy Makers</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Federal research</td>
<td>JoAnne Grunbaum</td>
<td>Centers for Disease Control &amp; Prevention</td>
</tr>
<tr>
<td>Product Makers</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Pharmaceutical</td>
<td>Eleanor Perfetto</td>
<td>Pfizer</td>
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<tr>
<td>Principal investigators</td>
<td></td>
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</tr>
<tr>
<td>Health services</td>
<td>Julie Lynch</td>
<td>University of Massachusetts Boston</td>
</tr>
<tr>
<td>Clinical</td>
<td>Radley (Chris) Sheldrick</td>
<td>Tufts Medical Center</td>
</tr>
</tbody>
</table>
What do we want to know?

1. Types of stakeholders?
2. Stages of CER?
3. Types of engagement?
4. Special provisions for patients?
We searched published literature with key words

1. Stakeholders – patients through principal investigators
2. Stages of research – prioritization through feedback and assessment
3. PCOR and CER

76 terms
A summary of what we found

Search Strategy
2,062 Abstracts

Abstract Screen: 1,787 rejected
- Stakeholders not engaged / not CER or PCOR

Full Text Retrieval
275 Articles

Full Text Screen: 180 Rejected
- Stakeholders not engaged / not CER or PCOR (118)
- Non-US Settings (62)

Full Text Review
95 Articles

Original Research
36 Articles

Research Programs
34 Articles

Research Syntheses
25 Articles
Stakeholder Type

- Patients and the Public
- Providers
- Payers
- Policy Makers
- Product Makers
- Principal Investigators
- Purchasers
- Other

Articles (n)
Seven-Item Reporting Questionnaire

1. What types of stakeholders?
2. A priori target number(s) used and met?
3. How was balance considered and achieved?
4. Methods to identify, recruit and enroll stakeholders?
5. Engagement before, during and after research?
6. Methods and modes of engagement?
Seven-Item Reporting Questionnaire

7. Impact of engagement on:

• the **relevance** of research questions;
• the **transparency** of the research process;
• the **adoption** of evidence into practice settings?
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Motivation (a Spectacular Failure)

”[F]eedback about the recommendations …makes it clear that we need to have better messages.”

Vice Chair of USPSTF on the widespread opposition to the Mammography Screening Guideline.

Diana Petitti, 2009
USPSTF includes some experts and engages with some stakeholders...

Has 16 volunteer members who are experts in prevention, evidence-based medicine, and primary care

Engages partner organizations such as medical societies, insurers and consumer organizations

- *Before* guideline development: topic identification
- *After* guidelines are completed: dissemination
...but does not engage with all stakeholders

Some stakeholders are excluded *during* guideline development

- If they do not have methodological expertise (patients)
- If they have a perceived conflict of interest (industry, payers, employers, subspecialties)
Breast cancer screening review and guidelines were re-issued in 2009...

Review
Mammography screening reduces breast cancer mortality on average by
- 15% in women ages 39-49
- 14% in women ages 50-59
- 32% in women ages 60-69

Younger women are more likely to have false positive diagnoses from mammography screening
Breast cancer screening review and guidelines were re-issued in 2009...

Recommendations
Routine, biennial mammography for women ages 50-74
Mammography for women younger than 50 only after considering individual factors and patient preferences
  • Should be available but not routine
Women of all ages should talk with their doctors about their risks for breast cancer and their preferences for screening
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  • Should be available but NOT ROUTINE
Women of all ages should talk with their doctors about their risks for breast cancer and their preferences for screening
The release unleashed vehement opposition from radiologists, oncologists, patients advocacy groups
The release unleashed vehement opposition from *groups that were excluded from guideline development*

- New guidelines would “turn back the clock in the war on breast cancer.”
- Bi-partisan legislation guaranteed coverage of annual screening
- Many physicians and institutions resisted the guidelines
- Guidelines did not substantially alter screening practices
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Lessons

1. Engage with all stakeholders
   • Exclusions can backfire
   • Use a structured process to identify and recruit stakeholders
   • All stakeholders are experts on their own views, including patients
   • Conflict of interest can be managed and is not an excuse to exclude industry and subspecialists
Lessons

2. How you engage may be as important as that you engage
   • Prepare everyone
   • Same place, same time
   • Sustained relationships
   • Expert-led
   • Choose modes and methods carefully
   • Get advice
Lessons

3. Engagement is like any other activity in research
   • Doing it right is no guarantee of success
   • It may require tradeoffs with other goals of research
Thank you