Identifying Effective Health Care Services for Adults with Disabilities

Why Study Designs and Outcome Measures Matter

July 7, 2011

Presentation at the Center on Health Care Effectiveness (CHCE) Issue Forum
About CHCE

- A resource for policymakers, the public, and other stakeholders
- Offers broad-based expertise and rigorous methods related to:
  - Comparative effectiveness research (CER)
  - Policy analyses and evidence syntheses relevant to developing and using CER
  - Delivery-system research on implementing CER in clinical practice
  - Technical assistance for policymakers and others on using evidence to promote effective care
More than 150 researchers at Mathematica and the Center for Studying Health System Change

Purpose
- To provide objective evidence and policy analysis informing difficult decisions about “what works best for whom” in health care

Recent activities
- Created an online list of recent and ongoing CER-relevant projects at Mathematica to connect researchers and policymakers who have similar interests
About CHCE (cont’d.)

- **Upcoming forums**
  - Policy Levers to Promote Shared Decision Making

- **Recent publications**
  - Comparative Effectiveness of Care Coordination for Adults with Disabilities: A Conceptual Typology and Systematic Evidence Review
  - The Implications of Comparative Effectiveness Research for Academic Medicine

- **Learn more**
  - Visit our website: http://www.mathematica-mpr.com/chce/
Today’s Agenda

- **Presentation**
  - Project background and methodology
    - Matthew Kehn, M.P.P., research analyst
  - Proposed solutions: how we match study designs to research questions
    - Jeff Ballou, Ph.D., senior researcher
  - Proposed criteria for assessing the relevance of outcomes
    - Debra Lipson, M.H.S.A., senior researcher

- Discussants’ remarks

- Q&A
Discussants

- Lisa I. Iezzoni, M.D., M.Sc.
  - Professor of Medicine, Harvard Medical School
  - Director, Mongan Institute for Health Policy, Massachusetts General Hospital

- Shoshanna Sofaer, Dr.P.H.
  - Robert P. Luciano Professor of Health Care Policy, School of Public Affairs, Baruch College

- Sean Tunis, M.D., M.Sc.
  - Founder and Director, Center for Medical Technology Policy

- Judy Zerzan, M.D., M.P.H.
  - Chief Medical Officer/Deputy Medicaid Director, Colorado Department of Health Care Policy and Financing
Project Background and Methodology

Matthew Kehn
Funding and Project Origins

- American Recovery and Re-Investment Act (ARRA): a major national investment in CER
  - $1.1 billion for CER
  - HHS secretary received $400 million to “accelerate the development and dissemination of CER”
  - Work presented today is part of this investment

- FCC-CER report emphasizes the importance for CER related to people with disabilities

- HHS contract with MPR to conduct CER on interventions to improve health outcomes and quality of life for people with disabilities
CER is “…the conduct and synthesis of systematic research comparing different interventions and strategies to prevent, diagnose, treat, and monitor health conditions”

Purpose is “to inform patients, providers, and decision makers, responding to their expressed needs, about which interventions are most effective for which patients under specific circumstances”

Source: http://www.hhs.gov/recovery/programs/cer/draftdefinition.html

FCC-CER = Federal Coordinating Council for Comparative Effectiveness Research
FCC-CER’s Definition Guides ARRA Investment (cont’d.)

- **Interventions may include:**
  - Medications and procedures
  - Medical and assistive devices and technologies
  - Behavioral change strategies
  - Delivery-system interventions

- **CER “must assess a comprehensive array of health-related outcomes for diverse patient populations”**

Source: http://www.hhs.gov/recovery/programs/cer/draftdefinition.html
Patient-Centered Outcomes Research Institute (PCORI):
- Informs health care decisions
- Provides evidence on the effectiveness, benefits, and harms of various treatment options for different patients
- Compares drugs, medical devices, tests, surgeries, and strategies for delivering health care

PCORI recognizes that the patient’s voice should be heard in the care decision-making process

Research will be responsive to:
- The preferences, values, and experiences of patients in making health care decisions
- The impact that diseases have on daily life

http://www.pcori.org/aboutus.html
CER Is Important to the Disability Community

- 33 million American adults with disabilities (Erickson et al. 2010)

- Disability-related health care expenditures estimated at $400 billion in 2006 (Anderson et al. 2011)

- CER can help people with disabilities make informed decisions about health care, but:
  - Study designs must account for the heterogeneity of this population and the wide range of services needed
  - The outcomes and measures reported must be meaningful to the decision makers in the disability community
Scope of Presented Work

- To develop criteria for determining the appropriate study design for answering disability-related CER questions
  - Research brief: *Expanding the Possibilities for Timely and Useful Evidence: Matching Study Designs to Research Questions in Disability-Related Comparative Effectiveness Research*

- To develop criteria for selecting the most useful and appropriate outcomes measures when conducting disability-related CER
  - Issue brief: *Choosing Wisely: Selecting Outcomes for Comparative Effectiveness Research on Services for Adults with Disabilities*
Study design criteria

- Review and synthesis of existing standards
  - Producers of systematic reviews (e.g., Cochrane, AHRQ, Campbell Collaboration)
  - Clinical guideline developers (e.g., USPSTF)
  - Developers of evidence-assessment instruments (e.g., GRADE Working Group, CONSORT)
  - Policymakers (e.g., NICE)
- Technical expert panels
Methodology (cont’d.)

- Outcomes measures criteria
  - Review and synthesis of existing health and disability outcomes in recent frameworks
    - Health framework (e.g., Institute of Medicine [IOM], National Institutes of Health [NIH])
    - Disability frameworks (e.g., Sofaer et al., Patrick)
  - Technical expert panels
Matching Study Designs to Research Questions

Jeff Ballou
Internal validity
- The intervention caused the observed outcomes

External validity
- Findings are applicable beyond the study’s sample

*Good* randomized controlled trials (RCTs) have high internal validity

Many observational studies have high external validity

Evidence hierarchies focus primarily on internal validity
Despite their high internal validity, even strong RCTs:
- Often lack external validity
- Can be time consuming and expensive
- Might be unethical or even infeasible

Observational study designs are subject to confounding and “rank lower”

Consequently, decision makers often lack the evidence they need
- Either unavailable or available too late
- Results in greater reliance on expert opinion
Disability-Related CER Is Even More Difficult

- Broad heterogeneity among people with disabilities
  - Nature and mix of limitations
  - Severity of disability
  - Presence of comorbidities

- Range of interventions for people with disabilities
  - Medical or surgical
  - Complex services involving multiple providers
  - Environmental modification

- Will this work in typical practices or in residential settings?
Not all research questions require RCTs
- Traditional evidence hierarchies should be applied with caution

In some cases, strong observational studies can provide informative and timely evidence

But when should a given design be used?
- Greater judgment required of decision makers
- Guidance on selecting designs can aid both decision makers and researchers

This issue extends beyond disability research
- PCORI Methodology Committee’s translation table
Using Evidence for Decision Making

1. What is the question?
   - What evidence is required?
   - What is the decision maker’s timeframe?
   - What is the decision maker’s population?

2. What study designs will be informative?

3. What study designs are available?
   - Some compromise will generally be required

4. How should decision makers evaluate the body of evidence?
   - Use existing standards but context-specific hierarchies
   - Pay special attention to disability-relevant criteria
     - For example, inclusion criteria and replications
# What Evidence Is Required?

<table>
<thead>
<tr>
<th>Higher Internal Validity</th>
<th>Higher External Validity</th>
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<tbody>
<tr>
<td>Costly to implement</td>
<td>Diverse populations</td>
</tr>
<tr>
<td>OR</td>
<td>OR</td>
</tr>
<tr>
<td>Might cause significant harm</td>
<td>Difficult to replicate</td>
</tr>
<tr>
<td></td>
<td>OR</td>
</tr>
<tr>
<td></td>
<td>Tailored to specific settings</td>
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<table>
<thead>
<tr>
<th>Lower Internal Validity</th>
<th>Lower External Validity</th>
</tr>
</thead>
<tbody>
<tr>
<td>Criteria for highest internal validity are not met</td>
<td>Can be replicated in different settings</td>
</tr>
<tr>
<td>AND/OR</td>
<td>AND</td>
</tr>
<tr>
<td>Imminent risk of harm without intervention</td>
<td>No coordination required</td>
</tr>
<tr>
<td></td>
<td>AND</td>
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<td>Easy to participate</td>
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Pragmatic clinical trials (PCTs)
- When highest internal and external validity are needed

A good PCT (or two) might solve your problem

But trials are often unavailable
- Issues of feasibility
- Question requires a timely answer
  - “Coverage with evidence development” is a possibility

Might need to weigh the relative importance of highest internal and external validity
Examples

1. What is the comparative effectiveness of a new medication to ameliorate the effects of Alzheimer’s disease versus the standard of care?
   - Concern that potential harms could outweigh benefits
     • Requires high internal validity (RCT)
   - Is appropriate use uncomplicated for providers and caregivers?
     • Highest external validity might not be required
2. What is the effectiveness of power versus manual wheelchairs for improving the independence of working-age adults with physical disabilities and no other impairments?

- Less concern about potential for harm
- Proper use of wheelchair is relatively uncomplicated
- Designs with modest internal and external validity are acceptable
3. Are people with disabilities who participate in a peer-mentoring program less likely to develop secondary conditions than people who do not participate?

- Risk and costs are relatively low
- Intervention could be complicated to implement
  - Diverse pairings of adults with disabilities and peer mentors
- Designs with modest internal validity but high external validity are appropriate
Broader Ongoing Initiatives

- Encouraging and strengthening CER methods
  - PCORI Methodology Committee
  - AHRQ Methods Symposia

- Developing standards for observational studies
  - Standards for reporting
  - Standards for evaluating research quality

- Increasing the effectiveness of RCTs
  - Bayesian/adaptive designs

- Ultimate goal: more high-quality, timely, relevant evidence
Criteria for Selecting Relevant Outcomes

Debra Lipson
For CER to be practical and useful, outcomes must be meaningful to decision makers
- People with disabilities, caregivers
- Clinicians, provider organizations
- Purchasers, policymakers

How do we choose the right outcomes, given:
- The large set of potentially relevant outcomes of services for people with disabilities?
- The diversity of the audience?
### So Many Choices . . .

<table>
<thead>
<tr>
<th>Health Outcomes</th>
<th>Disability Outcomes</th>
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</thead>
<tbody>
<tr>
<td>Safe</td>
<td>Patient experience</td>
</tr>
<tr>
<td>Physical health (symptoms, function)</td>
<td>Disabling processes (disease, impairment, function)</td>
</tr>
<tr>
<td>Effective</td>
<td>Family experience</td>
</tr>
<tr>
<td>Mental health (affect, behavior, cognition)</td>
<td>Independence and community integration</td>
</tr>
<tr>
<td>Patient centered</td>
<td>Family caregiving burden</td>
</tr>
<tr>
<td>Social health (relationships, function)</td>
<td>Service receipt, social support, and physical environment</td>
</tr>
<tr>
<td>Timely</td>
<td>Provider experience</td>
</tr>
<tr>
<td>Efficient</td>
<td>Functional status, independence, community participation</td>
</tr>
<tr>
<td>Equitable</td>
<td>Health status</td>
</tr>
<tr>
<td></td>
<td>Prevention of secondary conditions</td>
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</tbody>
</table>
Sources for Selection Criteria

- Guidelines for judging the relevance of outcomes in traditional clinical research
- Cochrane principles for choosing relevant outcomes in systematic reviews of evidence
- FCC-CER’s recommendations on services for people with disabilities
- Consultation with the technical expert panel
  - Disability researchers, consumer advocates, CER methodologists, and “real-world” decision makers
1. When in Doubt, Ask the Audience

- Start with core measure sets and measures recommended by major stakeholders

- But such measures may not be the best choice
  - Often products of consensus
  - Stakeholder groups may not be representative
  - Tend to rely on readily available data
  - Intended for quality monitoring, public reporting, or provider payment purposes, not for CER

- So, ask representatives of intended CER users
  - Makes best use of limited research funds
  - Can help set priorities
  - May refine outcome measures
Basic logic models can clarify relationship between intervention and plausible outcomes

Complex interventions require greater specification
- Of relationships between activities and how they interact to produce expected outcomes
- Of the outcomes produced by actors operating at different levels of health and social support systems
- Environmental influences, especially financing

Select outcomes over which actors have more control
In each care-delivery model, the services over which the care coordinator has influence differ, and so outcomes will also differ.
3. Choose Outcomes to Suit Each Subgroup

- Include subsets of outcomes for each group
  - Employment outcomes for working-age adults
  - End-of-life care preferences for older, frail adults

- Need for and use of services varies by:
  - Age, living arrangement, informal support
  - Type and number of disabilities: sensory, cognitive, physical/ambulatory, intellectual, mental illness
  - Severity of disability; need for help with ADLs / IADLs
  - Coexisting chronic medical conditions: number, type, severity, stage of illness

- When measuring outcomes at the system level, control for these differences
4. Present Results and Compare Tradeoffs

- People with disabilities hold different values
  - Longer life vs. shorter life expectancy but higher quality of life
  - Short-term vs. long-term effects

- Present results in a way that shows tradeoffs
  - Summary tables with:
    - Magnitude of effects for all outcomes
    - Relative risks for all outcomes in control versus treatment groups
Discussants

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  - Professor of Medicine, Harvard Medical School
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  - Robert P. Luciano Professor of Health Care Policy, School of Public Affairs, Baruch College
Q&A

- Live and web audience

- **Moderator**
  - Eugene Rich, M.D., Director, CHCE, Mathematica

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  - Jeff Ballou, Mathematica
  - Lisa I. Iezzoni, Harvard Medical School, MA General Hospital
  - Matthew Kehn, Mathematica
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For More Information

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