# 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**

MATHEMATICA
Policy Research

# 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**

# About CHCE (cont'd.)

- 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**

# FCC-CER's Definition Guides ARRA Investment

- **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

# ACA and New Support for CER

- **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***

# Methodology

- **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

# Background and Context

- **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**

# Conducting High-Impact CER Is Difficult

- **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?**

# Matching Designs and Questions

- **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?

| Higher Internal Validity | Higher External Validity |
|---|---|
| Costly to implement<br>OR<br>Might cause significant harm | Diverse populations<br>OR<br>Difficult to replicate<br>OR<br>Tailored to specific settings |
| **Lower Internal Validity** | **Lower External Validity** |
| Criteria for highest internal validity are not met<br>AND/OR<br>Imminent risk of harm without intervention | Can be replicated in different settings<br>AND<br>No coordination required<br>AND<br>Easy to participate |

# Which Study Designs Are Informative and Available?

- **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

# Examples (cont'd.)

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**

# Examples (cont'd.)

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

# Challenge to CER Researchers

- **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 . . .

| Health Outcomes | | Disability Outcomes | |
|---|---|---|---|
| Crossing the Quality Chasm—IOM (2001) | Patient-Reported Outcomes Measurement Information System—NIH (2007) | Model for Promoting Health for People with Disabilities—Patrick (1997) | Measures of Care Coordination for Persons with Disabilities in Medicaid Managed Care—Sofaer et al. (2000) |
| Safe | Physical health (symptoms, function) | Disabling processes (disease, impairment, function) | Patient experience |
| Effective | Mental health (affect, behavior, cognition) | Independence and community integration | Family experience |
| Patient centered | Social health (relationships, function) | Service receipt, social support, and physical environment | Family caregiving burden |
| Timely | | Quality of life | Provider experience |
| Efficient | | | Functional status, independence, community participation |
| Equitable | | | Health status<br>Prevention of secondary conditions |

# Sources for Selection Criteria

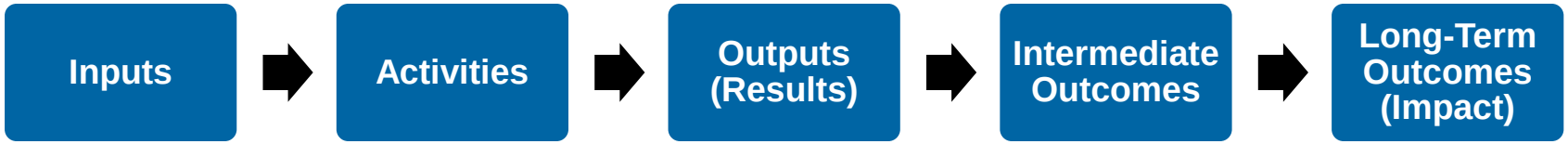
- Guidelines for judging the relevance of outcomes in traditional clinical research
- Cochrane principles for choosing relevant outcomes in systematic reviews of evidence
- FCC-CER's recommendations on services for people with disabilities
- Consultation with the technical expert panel
  - Disability 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**

# 2. Select Outcomes Linked to Goals

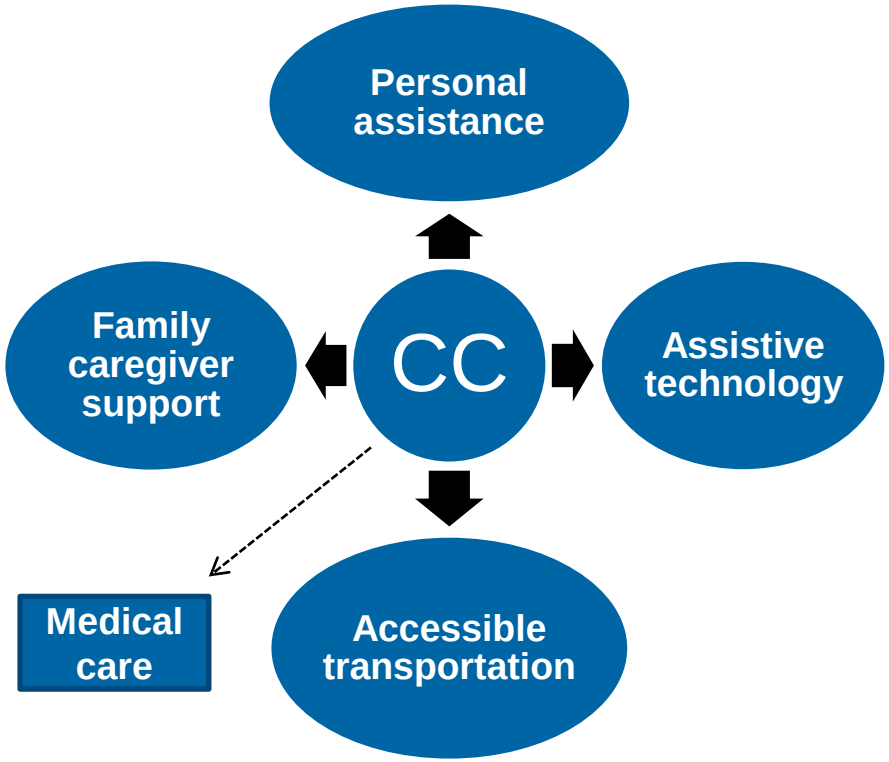
- **Basic logic models can clarify relationship between intervention and plausible outcomes**

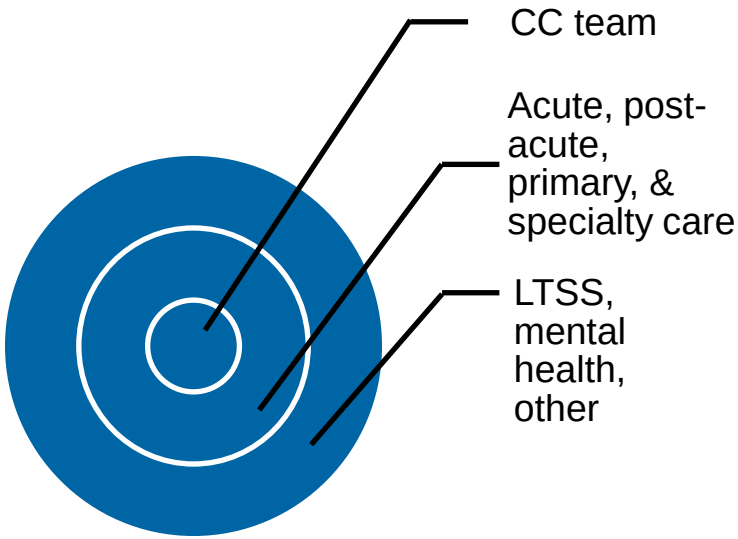



- **Complex interventions require greater specification**
  - **Of relationships between activities and how they interact to produce expected outcomes**
  - **Of the outcomes produced by actors operating at different levels of health and social support systems**
  - **Environmental influences, especially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

# Example: Outcomes of Care Coordination

**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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  - **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**
- **Discussants**
  - **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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