

JULY 2011

Choosing Wisely: Selecting Outcomes for Comparative Effectiveness Research on Services for Adults with Disabilities

by Debra J. Lipson, Matthew Kehn, and Eugene Rich

In 2008, 33 million U.S. adults lived with a disabling condition, a number that is expected to grow in the coming years as the population ages. The costs for their health care are significant, with \$400 billion, or almost 27 percent of the nation's total health care spending in 2006, going toward disability-related care. Yet many individuals lack the information they need to choose wisely among available treatments, and clinicians do not always have adequate medical evidence to inform their decisions. Furthermore, policymakers do not have the data to know which programs should continue or be revamped. The federal government began to address these gaps by allocating \$1 billion through the American Recovery and Reinvestment Act of 2009 for comparative effectiveness research (CER). The Patient-Centered Outcomes Research Institute (PCORI), funded by the Affordable Care Act, is taking this investment one step further to build a research base for CER. This brief lays out four criteria to help researchers select and report on the outcomes that matter most to adults with disabilities and to the providers and others responsible for their care.

Building a Research Base

Approximately 33 million American adults, or 14 percent of the adult population, lived with disabling conditions in 2008 (Erickson et al. 2010). Disability-related health care expenditures were nearly \$400 billion in 2006, almost 27 percent of the nation's total spending on health care (Anderson et al. 2011). On a personal level, living independently is a challenge for people with disabilities, as is securing a job for adults of working age. From access to health care to participating in the community, a disability can influence nearly every aspect of an individual's life.

In terms of health care decision making, individuals with disabilities lack the information they need to choose the services that are best for them. Clinicians

do not have adequate medical evidence to select the best treatments; and policymakers need more evidence on program effectiveness. PCORI will build on the comparative effectiveness research base established under ARRA, advancing the quality and relevance of evidence to help patients and their providers make more informed decisions. This commitment from the federal government affirms the notion that CER can play an important role in helping individuals with disabilities and their providers choose a personalized mix of services (Federal Coordinating Council on CER 2009).

Relevant Outcomes: Too Much of a Good Thing?

The complete set of outcomes relevant to the disability community—which

includes people with disabilities, clinicians, caregivers, provider organizations, purchasers, and policymakers—is too large for any single study. The set includes outcomes typically examined in CER studies of health services and medical technologies as well as a broader set of outcomes typically examined in disability research (Table 1). Two examples in the first category stand out. In its landmark report, *Crossing the Quality Chasm*, the Institute of Medicine (2001) determined that a high-quality health care system must be safe, effective, patient-centered, timely, efficient, and equitable. The National Institutes of Health developed the Patient Reported Outcomes Measurement Information System, or PROMIS (Cella et al. 2007). This set of highly reliable instruments and patient-reported outcomes for studies

of clinical treatment effectiveness is based on the World Health Organization’s definition of health, which covers physical health, mental health, and social well-being.

Two studies illustrate the broader set of outcomes that are typically examined in disability research. They include not only some outcomes examined in CER studies of health services and clinical effectiveness, but also the quality of life in general, and independence and community integration in particular. For instance, in the Model of Health Promotion for People with Disabilities, Patrick (1997) divided outcomes into four domains: (1) disabling processes, (2) independence and community integration, (3) receipt of health services, social supports, and environmental modifications, and (4) quality of life. Similarly, Sofaer and colleagues (2000) organized the outcome measures of coordinated care delivered to people with disabilities in Medicaid managed care plans into two categories that embrace both traditional health outcomes and disability outcomes: (1) long-term outcomes such as the patient’s and the family’s experience of care, the family caregiving burden, patient health and functional status, and independence

and community participation, and (2) intermediate outcomes that are measures of care coordination processes, such as less delay in the receipt of services, fewer conflicting prescriptions, and a reduction in preventable emergency room visits, hospitalizations, and nursing home placements (not shown in Table 1).

Criteria for Selecting Relevant Outcomes

In many respects, the wide array of outcomes available to researchers who design CER studies of disability services and models of care is an advantage. But the question is, how do researchers determine which outcomes are relevant to a given study and thus enhance the value of their CER? The four selection criteria described below can help them decide. The criteria were drawn from the following sources:

- Guidelines developed by Guyatt and Rennie (2002) for judging the relevance of outcomes in more traditional clinical research
- Criteria in Higgins and Green (2008) for choosing relevant outcomes when conducting systematic reviews of evidence according to Cochrane principles

- Recommendations from the Federal Coordinating Council on CER (2009) for conducting CER on services and care coordination for people with disabilities
- Consultation with members of a technical expert panel consisting of disability researchers, consumer advocates, CER methodologists, and “real world” decision makers such as state Medicaid directors

1. When in doubt, ask the audience

Researchers can improve the value of their CER on disability services by identifying the audience(s) for the study and selecting the outcomes that these individuals care about most. For example, there are many possible outcomes of care coordination, but people with disabilities and their caregivers may be interested in the most patient- or family-centered services, while purchasers may be interested in services that add up to greater cost savings.

Recent efforts to identify core sets of outcome measures can provide a useful starting point for researchers. For instance, Galantowicz (2010) developed quality outcomes for Medicaid home and community-based waiver services, and McDonald and colleagues (2010) developed outcomes for care coordination. But limited budgets may prevent researchers from exploring all outcomes in a set, in which case they should not assume which measures are the most relevant. Instead, they may need to ask representative decision makers directly about what they want to know and which comparisons are most appropriate. This approach might be especially useful when decision makers are interested in several outcomes or when an outcome is more nuanced than it appears. For example, purchasers are interested in more than cost-savings. As one expert panel member explained, “If there’s a better way of doing something, a Medicaid official would look at whether the innovation produces a better outcome, such as keeping people at home rather than an institution, for the same price.”

Table 1.
OUTCOMES RELEVANT TO CER ON SERVICES FOR ADULTS WITH DISABILITIES

Health Outcomes		Disability Outcomes	
Crossing the Quality Chasm (IOM 2001)	PROMIS (NIH 2007)	Model for Promoting Health for People with Disabilities, Patrick (1997)	Framework for 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 & 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
			Prevention of secondary conditions

2. Select outcomes that are closely linked to intervention goals

Researchers should choose outcomes that can be directly influenced by the intervention. For instance, improved health status and/or reduced therapeutic complications are appropriate outcomes for a study designed to measure the effects of alternative health services. Likewise, in an evaluation of the comparative effectiveness of a medical home model for adults with disabilities, it makes sense to measure changes in the use of health services because medical homes are intended to coordinate the full range of acute, primary, specialty, preventive and long-term care services. But it would be inappropriate to measure changes in health services utilization in an evaluation of the comparative effectiveness of a care coordination program if the care coordinators have control over nonmedical support services (such as personal care and home-delivered meals) but not over primary or specialty medical care.

In many cases, the outcomes that an intervention can influence are not long-term outcomes, but short-term results or outputs that are proximate to the intervention itself (process-of-care measures, for example). This point is especially relevant to care coordination; as Sofaer and colleagues cautioned, “If other quality dimensions [of services delivered] are poor, even the best efforts of those coordinating care may fail to have the desired [health] outcomes.”

3. Choose outcomes that matter to people with different types of disabilities

The same services and models of care are often delivered to people with various types of disabilities. While some measures should be standardized to compare outcomes for everyone receiving services, the heterogeneity of the adult population with disabilities means that outcomes relevant to each person are likely to differ. For example, what is meaningful to young adults with intellectual disabilities is not likely to be meaningful to frail older adults. Subsets

of outcomes should reflect this diversity. Outcomes for working-age adults with disabilities could include employment and participation in community life. Outcomes for the frail elderly could include whether care plans are consistent with individual preferences for end-of-life care. If there are several instruments for measuring outcomes, researchers should choose the ones that have been shown to be reliable for each subgroup of people with disabilities. In CER studies that measure outcomes at the level of the provider, program, or delivery system, researchers should use risk-adjusted measures that control for severity of condition or degree of disability in each study population.

4. Present results in a way that helps decision makers weigh potential tradeoffs

Because decision makers value some outcomes more than others, CER studies should present results on different outcomes in a manner that helps decision makers assess the possible tradeoffs associated with certain services or models of care versus others. For instance, in making a decision about where to live, some patients may be willing to accept an environment that is less safe or that presents a greater risk of not getting all services in a timely fashion in exchange for more physical activity, autonomy, or control that comes with living and receiving services in a home or in a community-based setting than in an institution. Individuals with a terminal illness often face a trade-off between a longer life and the quality of that life; even if a given treatment has a good chance of extending life, it may reduce mobility or increase pain, diminishing the quality of life. In some cases, decision makers want to know about the effects of an intervention over time, since they may differ from short-term effects.

In addition, the results of CER studies should be presented in ways that help decision makers judge for themselves the costs and benefits of a given treatment or model of care delivery. Some

principles have already been established to help researchers do this effectively. For example, according to the Cochrane guidelines on how to present evidence from systematic reviews, summary tables should show critical information on (1) the amount of evidence in each review for all important outcomes, regardless of whether the results are positive or negative, (2) the quality of the evidence, which may vary by outcome, (3) the risk or prevalence of the outcome in the comparison group versus the treatment group; and (4) the absolute or relative magnitude of the effects (Higgins and Green 2008).

Enhancing the Value of CER Studies

A broad range of outcomes can be used to conduct CER on alternative services and care models for adults with disabilities. For an individual CER study, the appropriate outcomes to measure and report will vary with the specifics of the intervention, the types of disabilities in the study populations, and nature of the decisions to be informed by the study. By applying the criteria described in this brief, researchers can enhance the value of CER studies and give decision makers better information to judge the value of alternative services for people living with disabilities.

For more information, contact Debra Lipson, senior researcher, dlipson@mathematica-mpr.com.

Mathematica® is a registered trademark of Mathematica Policy Research, Inc.

This issue brief is based on a project funded by the U.S. Department of Health and Human Services.

References

- Anderson, W.L., J.M. Wiener, E.A. Finkelstein, and B.S. Armour. "Estimates of National Health Care Expenditures Associated with Disability." *Journal of Disability Policy Studies*, vol. 21, no. 4, 2011, pp. 230-240.
- Cella, D., S. Yount, N. Rothrock et al. on behalf of the PROMIS cooperative group. "The Patient Reported Outcomes Measurement Information System (PROMIS): Progress of an NIH Roadmap Cooperative Group During Its First Two Years. *Medical Care*, vol. 45, no. 5, 2007, pp. S3-S11.
- Erickson, W., C. Lee, and S. Von Schrader. "2008 Disability Status Report: The United States." Ithaca, NY: Cornell University Rehabilitation Research and Training Center on Disability Demographics and Statistics, 2010. Available at <http://www.ilr.cornell.edu/edi/disabilitystatistics/>. Accessed July 30, 2010.
- Federal Coordinating Council for Comparative Effectiveness Research. "Report to the President and Congress." Washington, DC: U.S. Department of Health and Human Services, June 2009. Available at www.hhs.gov/recovery/programs/cer/cerannualrpt.pdf. Accessed on August 9, 2010.
- Galantowicz, S. "Environmental Scan of Measures for Medicaid Title XIX Home and Community-Based Services." AHRQ Publication No. 10-0042-EF. Rockville, MD: Agency for Healthcare Research and Quality, June 2010.
- Guyatt, G., and D. Rennie, eds. "Users Guides to the Medical Literature: Essentials of Evidence-Based Clinical Practice." Chicago: American Medical Association, 2002.
- Higgins, J., and S. Green, eds. *Cochrane Handbook for Systematic Reviews of Interventions*. Hoboken, NJ: John Wiley and Sons, 2008.
- Institute of Medicine. *Crossing the Quality Chasm: A New Health System for the 21st Century*. Washington, DC: National Academy Press, 2001.
- McDonald K.M., E. Schultz, L. Albin et al. "Care Coordination Measures Atlas, Version 3." AHRQ Publication No. 11-0023-EF. Rockville, MD: Agency for Healthcare Research and Quality, November 2010.
- Patrick, D.L. "Rethinking Prevention for People with Disabilities. Part I: A Conceptual Model for Promoting Health." *American Journal of Health Promotion*, vol.11, pp. 257-260.
- Sofaer, S., B. Kreling, and M. Carmel. "Coordination of Care for Persons with Disabilities Enrolled in Medicaid Managed Care: A Conceptual Framework to Guide the Development of Measures." Washington, DC: Department of Health and Human Services, Office of the Assistant Secretary for Planning and Evaluation, December 2000. Available at <http://aspe.hhs.gov/daltcp/reports/carecoor.pdf>.