Listening Session of the Federal Coordinating Council for Comparative Effectiveness Research

University of Illinois at Chicago Forum
Chicago, IL
May 13, 2009

Introduction

The purpose of this meeting was to continue to gather public input from a broad range of diverse stakeholders on priorities, concerns, and ideas about how comparative effectiveness research (CER) can empower patients and providers and improve care for all Americans.

The 2009 American Recovery and Reinvestment Act authorized $1.1 billion for comparative effectiveness research, including $300 million for the Agency for Healthcare Research and Quality, $400 million for the National Institutes of Health, and $400 million for the Secretary of Health and Human Services to support CER. The same law also created a 15-member Federal Coordinating Council for Comparative Effectiveness Research, which will assist federal agencies in coordinating comparative effectiveness and related health services research. The Council will submit a report to Congress on these priorities and recommendations by June 30, 2009.

The Council held its first listening session in Washington, D.C., on April 14. The third and final listening session will be held in Washington, D.C., on June 10. The Council will also be taking online written comments until the June 10th, 2009.

Recommendations

The Council obtained public comment from a wide range of speakers. A summary of key comments and recommendations follows.

Why CER Matters

- CER funding is crucial to reforming the practice of medicine to increase the quality, safety, cost benefits, and effectiveness of what providers bring to patients on a daily basis.

Knowledge Transfer

- The Council must pay attention to the transfer of knowledge from research findings to the clinical arena.
- CER should focus on what approaches and incentives to dissemination and adoption are most effective, and under what circumstances.

- CER should address the question of when it is most effective to target change at the organizational level, at the community level, or at the individual level.
- CER should examine which interventions can improve clinician and patient adoption and use of evidence-based care.
- Dissemination and implementation of what is known is crucial (i.e., culture of medicine care delivery needs to be data-driven).

Develop Infrastructure

- The success of community-engaged CER with the reach and sophistication to address racial and ethnic health disparities will ultimately depend upon community organizations possessing the infrastructure and capacity to support translational sciences and expanded programs of research and discovery in the service of improved community health.
- There is a need for a national, interoperable data exchange platform into which physicians, practices, and so forth, can both deposit information and access information for benchmarking purposes and to improve quality and efficiency.
- The Federal CER enterprise must build infrastructure and train future researchers (particularly in the areas of mental health and substance abuse prevention and treatment).
- Clinical technology organizations can serve to pool data from disparate sources into a unified dataset for both reporting and analysis.
- There is a need to build the infrastructure to conduct research that will address disparities as it relates to people of color. (*from* Open Comment Period)

Prevention

- CER should be focused on research for prevention, and the proposal review process should include experts on prevention.
- It is important to include CER studies not only on clinical prevention services but also on community prevention services.

Transparency

- Transparency is critical to reducing bias and rebuilding trust.
- Detailed, timely, and clearly written disclosures of the methods used will enable the users of CER findings to trust but verify.
- CER researchers should pledge to reduce bias.
- Researchers should show results prior to adjustments, as well as adjusted results.
- Research papers should disclose in detail the methods and metrics used, analytics behind statistical tests, the extent to which exclusions are met or not met, and the comparability of populations compared.
- There should be an open peer-review process, in a timely manner, at the beginning, middle, and end of a study.

- Consider compensating official, conflict-free peer reviewers at a rate that is competitive with rates paid to those conducting research.
- A structured approach to method disclosures should be considered.

Consider Real-World Settings

- The reach of CER includes both diagnostic and therapeutic procedures and systems.
- Sometimes comparative effectiveness can be determined by assessing technology and using quantitative metrics rather than via an expensive and sometimes lengthy clinical trial involving a substantial number of patients to achieve statistical certainty.

Consider Behavioral Incentives

- Research is needed to identify appropriate ways to incentivize patients for healthier behaviors.
- The Council should think broadly about not only medications, but also behavioral treatments and system-level interventions.
    - It can be more cost effective to pay an incentive if people quit the behavior (i.e., smoking) than using medicines on a lot of people on whom they are not successful.
    - When doing a comparative effectiveness study on how to improve outcomes among diabetic patients, it makes sense to think about testing behavioral approaches to weight loss reduction as well as medication-based approaches to improving outcomes among diabetics.

Consider Registries

- Comprehensive health registries should be included in any comparative effectiveness program.

Cost

- CER should not be focused on looking for cheaper treatments, and it should not be the basis for coverage decisions.
- The Council should consider funding projects that look at the cost of different services.
- The Council should consider costs. There is no question that if we don't consider costs, we will have to give up something. And that something we give up may be something we'd value very much in terms of health benefits.
- Understanding of the cost implications of therapies should be a central element of comparative effectiveness research. (*from* Open Comment Period)
    - Incorporation of costs is an integral part of informed decision-making.
    - Information about costs enables understanding about the direct differences in terms of clinical benefit as well as the value of interventions and whether or not they represent efficient use of resources.

Research Methodology

- Funded CER projects should be practical, include both primary research and secondary analysis, encourage people to focus on how different interventions are actually provided in the real world, and encourage people to present results that facilitate decision making.
- The Federal CER enterprise must be designed broadly to span treatment, prevention, promotion, and health-determinant interventions designed for both people and populations. It should also consider joint effects and span traditional research boundaries (i.e., targeted depression and diabetes treatments).
- The Council should consider explicitly using value of information principles and tools to prioritize comparative effectiveness research.
    - These approaches begin with ideas such as burden of illness and then turn to quantitative assessments of the likelihood that practice will change as a result of the research that is done.
- The most effective comparison efforts use improvement and health outcomes as the measure of effectiveness. They also compare comprehensive care protocols and teams outcomes, not individual medications or isolated procedures.
- The Council should fund studies where there is a community-friendly methodology, such as community participatory research. (*from* Open Comment Period)
- CER should use a variety of study designs to generate evidence about comparative effectiveness, comparative safety, and cost effectiveness. Observational studies, pragmatic clinical trials, and other study designs offer complementary information with varying research requirements. (*from* Open Comment Period)

Stakeholder Involvement

- The Federal CER enterprise must be guided by consumer and family input.

Take Advantage of Technology

- Patient compliance is a seldom-accounted-for variable in CER. CER studies should consider incorporating compliance-verification technology (low cost, miniature, unobtrusive, compliance verification devices) into orthotics, medication dispensers, and therapies.

Prioritize Around Understudied Areas

- Federal spending on CER should focus on priorities and areas in which the private sector is not active.
- This is incremental spending; thus, funding that is already going through NIH and other institutes should not be crowded out by what is done here.
- Comparative effectiveness research that is localized in a single disease may be less of a priority than questions that cross over diseases.

Focus on Populations

- At least one quarter of CER funds should be devoted to population interventions.

Focus on Care Delivery

- CER should study the best models for organizing and delivering care to patients with or at risk of chronic conditions (particularly diabetes).
- CER should include studies to compare current, more traditional models of chronic care delivery with genuinely team-based, patient-centered models of care that include patient education and self-care as an integral component.
- The Council should consider three recommendations related to health care delivery systems:
    - optimal practice models for delivering patient care
    - strategies for using IT (clinical decision support) to implement CER
    - best practices for disseminating and implementing CER

Address Sub-Populations

- The Council should ensure appropriate representation via oversampling of African Americans and other sub-populations in clinical trials.
- It is important to be aware that the use of retrospective comparative effectiveness data is flawed vis-à-vis sub-populations.
- There must be community-based research and patient-centered effectiveness.

Other Specific Research Priorities

- CER should be focused on producing research on clinical outcomes, taking into account ethnic, racial and gender differences in response to available treatments.
- CER should look at the impact of non-medical services (such as providing housing for the homeless) on cost-effective and clinical outcomes for chronically medically ill populations.
- CER should gather, evaluate, and compare the benefits of adding heparin to burn treatment prior to surgery.
- CER is needed on the clinical effectiveness, identification, and appropriateness of care for individuals with chronic conditions, including Alzheimer's disease:
    - A systematic review of the research on the effectiveness of physical, occupational and speech therapy for individuals with chronic conditions to identify therapy approaches that seem to be most effective for these individuals or particular subgroups thereof. Use the results of this review to conduct randomized clinical trials.
    - Compare medical and care coordination approaches for older people with serious medical conditions and dementia.
    - Compare approaches to improve difficult transitions for individuals with Alzheimer's disease, particularly from home or nursing home to the hospital and back.

    - Compare the different approaches to increased identification and recognition of dementia in older hospital patients in order to reduce complications, rapid declines in function, delirium, falls and lengths of stay for patients with Alzheimer's and other dementias during hospitalization.
- Research is needed that compares optimal medical management, percutaneous coronary interventions, and coronary artery bypass grafting in sub-populations.
- Research should be considered on the treatment of localized prostate cancer in African Americans and other sub-populations.
- CER is needed to test the total effects of medication on the frail elderly.
- CER is needed on the use of pain medication with and without an antidepressant in reducing frequency and intensity of pain in nursing home patients.
- CER should identify the most effective ways to encourage the adoption and active use of the diabetes education model, particularly in federal and state health programs, employer-sponsored plans, and health systems in underserved areas. In addition, CER should assess the expansion of the diabetes education model to other high priority chronic conditions.
- Comparative analysis across data sets (Medicare, pharmacy, laboratory, etc.) is needed in order to inform prescribing physicians on a wide range of what works best, and what does not, for patients with various complex conditions.
- CER is needed around prevention and treatment of oral diseases, including dental caries.
- The Federal government should take the leading role in supporting the generation of new evidence that directly compares relevant treatment alternatives. (*from* Open Comment Period)
    - Research is needed to evaluate the comparative effectiveness and cost effectiveness of medications and other medical technologies. Patients and providers need this information to make informed treatment decisions when considering treatment alternatives.
- CER is needed into a specific medical diet for children with epilepsy. If CER were done, it would allow the medical diet to be offered as an option, along with standard drug therapy. (*from* Open Comment Period)

Council's Questions to Panelists

Ms. Tanden asked Ms. Andriukaitis to clarify whether she meant that cost should never be a consideration or just not the primary motivation for CER. Ms. Andriukaitis replied that cost should not be the primary motivation, as too often people put cost into a separate silo and make decisions without regard to efficacy.

Dr. Clancy asked Dr. Lubin-Johnson to elaborate on the statement that collective decisions be made by all stakeholders. Dr. Lubin-Johnson replied that research needs to incorporate input from all parties affected, including patients (who receive a treatment), physicians (who administer a treatment), and insurers and hospitals (who administer the dollars).

Mr. Pacheco asked Ms. Jenson to share models dealing with multicultural kinds of populations in terms of prevention strategies. He noted that very little research has been done on preventive strategies for minority populations, and asked how the National Commission on Prevention Priorities (NCPP) might address those types of populations given the lack of data. Ms. Jenson said that while the NCPP's early research focused on population-wide estimates, NCPP is now developing Markoff models that offer the opportunity to get different estimates, for different populations, of the effectiveness and the use of different services, to the extent that such data are available. She added that NCPP is constrained by the lack of good research (and data) about the effectiveness of different types of services for different populations.

Ms. Tandeen asked Ms. Jenson to elaborate on her view about the importance of cost-effectiveness. Ms. Jenson noted that in reaching out to state and local public health departments, policy makers, employers, and others who might use research on community preventive services, all of them said that they need cost information to make decisions.

Dr. Conway asked Dr. Giger what research might look at effectiveness in imaging services in order to make the connections to patient outcomes and to build the evidence base to inform clinicians and patients. Dr. Giger said that one approach would be to assess which imaging procedure leads to the best clinical outcomes; a second approach is to look at modalities and physical parameters (e.g., what is the right resolution? how good is the image from this particular imaging system?). Dr. Giger indicated that there are a number of factors that need to be looked at, including preclinical evaluation of both the imaging system and reader performance, the radiologist's performance, and whether the service is cost-effective in clinical practice.

Dr. Clancy asked Dr. Wilson to what extent he believed that journal editors are doing a good job right now in addressing bias and conflict of interest. Dr. Wilson replied that it was not so much an issue of what journal editors are doing. Rather, he said, paying peer reviewers would improve peer reviews. He added that reviewers should be assessed prior to being selected to ensure they have an understanding of the methodologies being used in the research.

Dr. Clancy told the panel that the Council is going to struggle with the question of how to think about infrastructure. She said that if the panel were to think broadly about buckets of research, what should they be? Ms. Andriukaitis said she would like to see research on acute and early interventions. Dr. Wilson said that he'd like to see research on the operational aspects of primary, secondary and tertiary prevention (e.g., What's the best method of delivery of the uncontroversial treatments and preventions that we do?). Mr. Bendixen discussed the need for data systems (e.g., homelessness and Medicaid data) to talk to each other. Dr. Lubin-Johnson suggested the need to look at how to deliver care in a way that helps patients both get that care and adhere to proposed treatment regimens, and prevents subsequent, untoward effects of chronic diseases. Dr. Giger suggested that it was important to expedite and pre-evaluate systems for screening, detection, and treatment, to expedite the transfer of those to public use.

Dr. Cary asked Dr. Mersch for clarification on whether patients are aware that compliance-verification technology is being used. Dr. Mersch replied that there are currently two trains of thought on the issue (to prompt the patient or to not to let him or her know).

Dr. Valuck asked Dr. Aronson how Blue Cross Blue Shield health plans use CER information. Dr. Aronson replied that it was used in a variety of ways, and that CER would support benefit-design decisions.

Mr. Pacheco asked Dr. Manderscheid whether he had any specific strategies for increasing the representation of minority researchers in comparative effectiveness? Dr. Manderscheid pointed to a small SAMHSA program to increase the number of minority clinicians working in the behavioral health field, which he said was a model he thought was replicable for CER. He offered to provide Mr. Pacheco with additional background information on the program.

Dr. Conway asked Dr. Hurwitz whether he thought that the specialties would provide open access to their data should the Federal Government decide to fund further development and analysis of specialty databases. He also asked whether the specialties might be a vehicle for disseminating results. Dr. Hurwitz said while he couldn't speak for all of the specialty organizations, he felt that the response on contributing data had to date been positive. Regarding dissemination, Dr. Hurwitz said that while his specialty could not use the database to show results, it could perhaps find a way to make results available for best practices.

Dr. Valuck continued the discussion about databases, asking whether discussions about creating a joint orthopedic registry (in tandem with CMS and other HHS agencies) could serve as a potential basis for CER among other things. Dr. Hurwitz said that, speaking for his own organization, he would be in favor of using the data to assess a wide variety of best practices.

Dr. Casale asked Mr. Cappleman what it would take to provide support to the safety net providers so that they are full participants in CER. Mr. Cappleman said that many smaller medical health care centers do not have the infrastructure and are hence dependent on collaborations and linking up with other organizations.

Dr. Clancy continued the discussion, asking Mr. Cappleman if he might be able to provide any written follow-up on lessons learned by his organization. Mr. Cappleman noted that one project his organization is currently undertaking is the development of toolkits for health care clinics to help them put in place best practices and standardized approaches to lab testing (from the time the physician orders the test through follow-up). Dr. Schafer added that the current EHR cost infrastructure is killing safety net clinics. She suggested that the Council consider funding a few experiments about how to at least start using the diagnostic information from practice management systems to jumpstart the safety net clinics.

Ms Tanden asked Dr. Meltzer to elaborate on his remarks that CER criteria should focus on the likelihood that something will be applied (i.e., in practice). Dr. Meltzer said that there are times when there is a study that, while it may be a great idea, concerns something that no one will do—or where the barriers to implementation are too high or the infrastructure is prohibitively expensive. He said that the Council should try to take these factors into account in deciding what it will fund. How likely is it that we would get new information? And, then, how likely is it that that information, in turn, would change practices? he asked. After Ms. Tanden further suggested that he was recommending that the Council consider whether research will be high impact, Dr. Meltzer said that high impact has two components: the probability of an impact, and the magnitude of that impact.

Dr. Clancy followed up with Dr. Meltzer, asking whether the models he had discussed had been verified. Dr. Meltzer said that, in one sense, you can only validate at an expected value (and thus not really validate). He added, however, that the models had been used to describe the likely outcomes of the study.

Dr. Hudson asked Drs. Meltzer, Webster, and Marucha what steps their institutions were taking to train the next generation of clinicians, nurses, dentists, and family practitioners to carry out CER. He also asked whether the Council should consider training a priority. All three panelists said that they felt that training people to do CER was incredibly important. Dr. Marucha added that it was important to build a culture in both the research community and in clinical training programs to make research and evidence-based practice the norm rather than the exception.

Panelists

Access Community Health Network
James Cappleman, Research Coordinator,

AIDS Foundation of Chicago
Arturo Bendixen, Vice President for Programs and Partnerships

American Association of Diabetes Educators
Lana Vukovljak, CEO

American Association of Physicists in Medicine
Maryellen Giger, President (and Professor of Radiology)

American Board of Orthopaedic Surgery
Shepard Hurwitz, Executive Director,

American Society of Health-System Pharmacists
Cynthia Reilly, (telephonically), Director

AnalytiCare, LLC
Brett Kilpatrick, Manager Partner

Blue Cross Blue Shield Association
Naomi Aronson, Executive Director, TEC

Health Metric Systems, Inc.
Elsa Schafer, Chief Executive Officer,

Feinberg School of Medicine of Northwestern University
James Webster, Professor of Medicine, and Immediate Past President, Chicago Board of Health

M2S, Inc.
Meredith Mitchell (Telephonically), Product Manager

Medicare Advocacy Project, Alzheimer's Association
Leslie Fried (telephonically), Director

NAMI (National Alliance on Mental Illness) of Greater Chicago
Suzanne Andriukaitis, Executive Director

National Medical Association
Niva Lubin-Johnson, Chair, Board of Trustees

Partnership for Prevention
Jennifer Jenson, Managing Senior Fellow

Point Source, Inc.
Steven Mersch, President

Population Health Impact Institute
Thomas Wilson (telephonically), Epidemiologist

Saliba Burns, Wounds, Problems Institute
Michael Saliba, Chairman

SRA International
Ron Manderscheid (Telephonically), Director, Mental Health and Substance Use Programs

The University of Chicago
David Meltzer, Associate Professor and Director

University of Illinois at Chicago
Phillip Marucha, Associate Dean for Research

University of Pennsylvania School of Medicine and Wharton School of Business
Kevin Volpp

University of Virginia
Scott Wallace, Batten Fellow, Darden School

Open Public Comment Speakers

Center for Pharmacoeconomic Research, University of Illinois at Chicago
Todd Lee, Associate Professor

Charlie Foundation
Beth Zupec-Kania

Health Care Consortium of Illinois
Margaret Davis, Advocacy Director

Council Members

Agency for Healthcare Research & Quality
Carolyn Clancy

Centers for Medicare and Medicaid Services
Tom Valuck

Department of Defense
Michael Kilpatrick

Food and Drug Administration
Elizabeth Handley

Health Resources & Services Administration
Deborah Parham Hopson

National Institutes of Health
Lynn Hudson

Office of Disability
Michael Marge

Office of Minority Health, HHS
Guadalupe Pacheco (for Garth Brown)

Office of the Secretary, HHS
Neera Tanden

Substance Abuse and Mental Health Services Administration
Pete Delany

Veterans Health Administration
Margaret Cary

Council Staff
Patrick Conway, Executive Director
Cecilia Rivera Casale, Deputy Director