



Improving Evidence for Health Care Decision Making Conference Report April 1, 2005

On April 1, 2005, the Health Industry Forum convened a meeting to discuss strategies for improving the availability of medical evidence to support health care decision-making. The impetus for this meeting arose out of current discussions among the Centers for Medicare and Medicaid Services (CMS), the Agency for Healthcare Research and Quality (AHRQ), the Institute of Medicine (IOM) and other stakeholders about new collaborative initiatives to establish registries and other coordinated data-gathering activities that would result in practical actionable medical evidence for physicians and patients. These new initiatives could build upon current CMS initiatives – such as the recent decision to require that data be submitted to registries as a condition of coverage for implantable cardioverter defibrillators (ICDs). Such registries could provide important information about the safety of drugs, devices, and medical treatments that would not be available otherwise. They also could shed light on the efficacy and comparative effectiveness of treatments as they are used in the “real world.” But registries and other new data collection efforts are still controversial. Many critical issues -- such as governance, funding, and implementation -- remain unresolved.

Speakers included Carolyn Clancy, M.D., Director, AHRQ, Michael McGinnis M.D., Senior Scholar, Institute of Medicine, Helen Darling, President, National Business Group on Health, Sean Tunis, M.D., Chief Medical Officer, CMS, Jack Rowe M.D., Chairman and CEO, Aetna Inc., and David Brennan, President and CEO, AstraZeneca Pharmaceuticals.

While there were areas of controversy, speakers and other participants generally agreed that:

- There is a pressing need for actionable evidence to support patients and providers in medical decisions;
- Obtaining this evidence will require new initiatives such as registries, observational studies and coverage under protocol; and
- Public/private partnerships are the most desirable and feasible method of undertaking these efforts.

A number of challenging policy issues remain to be resolved, however, including:

- How these efforts will be organized, located and funded;
- How priorities will be established;
- How responsibility for analyzing and interpreting these new data will be assigned;
- How information will be disseminated to patients and physicians;
- How public trust in the data will be established; and
- How future incentives for innovation will be protected as coverage becomes more closely linked to evidence-based medicine.

Panel I: Improving the Availability of Practical Clinical Research

Carolyn Clancy, Director of AHQR, began the meeting by presenting the following conclusions about evidence-based decision making:

- Clinicians and patients make decisions every day, whether they have information or not;
- There is an important opportunity to provide the best available information to patients and physicians – even if that information is not definitive –in way that can be understood;
- Evidence is dynamic and needs to be systematically revisited over time;
- Transparency is critical;
- Enhanced evaluation should not stifle innovation; and
- Continued collaboration between the public and private sectors is essential.

Dr. Clancy first discussed the challenges of creating actionable knowledge for decision making. Effective diffusion of new knowledge faces formidable obstacles. Infrastructure to disseminate evidence-based practices is under-developed and the proliferation of new diagnostic and treatment options can overwhelm providers. A multitude of new studies are released daily, often with results that are not easily interpreted for use in daily practice.

Dr. Clancy then focused on the role of AHQR. Congress has recently directed AHQR to conduct and support research with a focus on outcomes. This initiative will require AHQR to create a “three-legged” process that incorporates: 1) knowledge synthesis; 2) knowledge generation; and 3) knowledge dissemination. To fulfill this mission, an inter-agency Steering Committee has worked to develop prioritization criteria for the new AHQR activities. The Committee considered such issues as a condition’s prevalence, health burden, cost, and representation in the Medicare population. The committee also considered the potential for improving clinical and financial outcomes through effective application of evidence-based treatment. Using these criteria, a priority list of conditions was developed that includes, but is not limited to, ischemic heart disease; cancer, COPD/Asthma, stroke, and diabetes.

Moving forward, the Agency’s analysis will move from measuring outcomes broadly within conditions to answering questions about the effectiveness of specific interventions, for specific patients, in specific treatment settings. Furthermore, effective dissemination will require new forms of communication tailored to different patient and provider audiences incorporating a variety of different media.

Ideally, these activities could support three distinct types of decisions. First, policymakers could use the information to set policies (do we fund heart transplants?). Second, the health care system, defined broadly, could use this information to address important issues about system design (where do we locate heart transplant services?). Finally, clinicians will be able to use the information from these studies to guide clinical policy and decision making (who should receive heart transplants?). Building new systems to effectively develop and disseminate new information will require careful planning and the involvement of a multitude of stakeholders. It may be essential to develop a common information

infrastructure. An important element of this effort may include observational studies and registries.

Michael McGinnis of the Institute of Medicine laid out a case for greater investment in evidence-based research. He noted that the variation in spending on health care by geographic region has persisted for many years, even as clinical guidelines have been developed. Dr. McGinnis argued that:

- Only a small amount of federal and private research funds are devoted to studying outcomes of existing medical treatments;
- Randomized controlled studies are too expensive and impractical to generate needed evidence;
- Better information is needed on drugs, devices and treatments;
- Evidence is needed more quickly;
- Innovation must be safeguarded in this process; and
- Public trust in this process is essential.

Dr. McGinnis also stated his belief that this initiative can be most successfully structured as a public/private cooperative effort. The IOM offers a respected and independent venue to help coordinate new initiatives and is working with a variety of stakeholders on a process to expand these activities. Dr. McGinnis announced that the IOM will be convening a forum on evidence-based medicine to bring stakeholders together and distributed a statement of purpose for the new initiative (attached).

Helen Darling from the National Business Group on Health (NBGH) began her remarks by noting her organization's strong support for this effort. Health care spending is growing at an unsustainable rate. The nation faces a tremendous unfunded liability from its promise to fund health care to seniors through Medicare. At the same time, health care spending is crowding out other important public policy priorities such as education. Employer spending on health care is far outpacing wage growth. As these trends continue, more people will get "skinny" benefit packages of limited value. One alternative future direction would be designing affordable benefits packages that incorporate evidence-based benefit design. NBGH has established a national committee to examine this option. Any transition to evidence-based benefit packages would need to be accompanied by support to help individuals make more effective health care decisions.

Gail Wilensky of Project HOPE started the discussion by inquiring whether specific health benefits language based on evidence-based medicine would survive legal challenges, given that the courts tend to define benefits broadly. Ms. Darling thought that if the plans use very specific language, and are careful about disclosure, the courts would uphold such language. Dan McGowan of HIP Health Plan of New York expressed doubts, noting that external appeals processes now in place in many states have generally sided with individuals rather than plans on coverage decisions. Dr. Wilensky noted that the controversy will be particularly acute for situations where a particular treatment, drug or device may be useful for some patients but not for others.

Stuart Altman, the moderator of the meeting, pointed out that contract language where either all patients or no patients are covered for a particular treatment is probably more defensible in court than those where coverage is patient specific. Mark Pauly of the Wharton School noted that evidence-based medicine has limits, and studies will only provide information about the probability of a particular outcome, not certainty for any particular patient. The courts, he stated, may take a dim view of this uncertainty and could feel pressure to force payers to cover any patient who might potentially benefit, even if the odds of a successful outcome are small. Myrl Weinberg of the National Health Council responded that patients with chronic conditions must be educated about these efforts, and reinforced the earlier view that coverage decisions in this gray zone could increase health plan liability.

Kathy Buto of Johnson & Johnson expressed potential concerns with “all or nothing” approaches to covering new technologies. If payers refuse to cover new drugs, then developing sufficient evidence about these drugs will be difficult. A rational policy that allows for introduction of new drugs, devices, or treatments, as data is collected, must be developed. Dr. Altman suggested an approach where payers would provide coverage, but only if data is collected that would allow for credible evidence to be developed over time.

Karen Ignagni of America’s Health Insurance Plans (AHIP) raised several issues that stand in the way of expanded use of EBM: 1) inadequate public and private research investment; 2) the lack of an apparatus for setting research priorities; 3) limited mechanisms for effectively diffusing new medical knowledge to physicians; 4) the lack of a coordinated infrastructure for technology assessment; and 5) the lack of a system for post-marketing surveillance of new products. Building a critical path to address these problems will require a public-private partnership.

Marc Berger of Merck remarked that although there are a variety of efforts now underway in the federal government, different agencies seem to be separately addressing different aspects of the problem without much coordination. The government has underinvested in this type of research and needs to put more focus on addressing methodological challenges. However, perhaps more important is the need to develop a much more coordinated agenda so that federal agencies address these issues in a consistent and complementary way. Furthermore, there is need for a coordinated public message about these efforts.

Gail Shearer of Consumers Union discussed her organization’s efforts to disseminate evidence for consumers. Publications like Consumer Reports “Best Buy Drugs” provide comparative information in a readily understood format about comparative efficacy and cost of certain classes of pharmaceuticals.

Sean Tunis of CMS noted that much of the discussion had assumed a top-down decision-making process, but a bottom-up approach may be more likely, as patients are forced to spend more of their own funds directly and as physicians are increasingly paid for their performance. A key challenge is to channel useable evidence to rank and file patients and physicians.

Dr. McGinnis talked about a vision for a national trust for evidence-based medicine. The initial efforts to develop this initiative would encompass three activities: 1) creation of registries; 2) development of priorities; and 3) creation of a mechanism for stakeholders to

participate in this process. Dr. Clancy commented that this is something people care a lot about and to some extent it doesn't matter where the activities are housed as long as they move forward.

Alan Muney of Oxford Health Plans raised the question of support from the physician community, noting that the biggest elephant in the room is that organized medicine is not driving this agenda. Physicians have long resisted measurement, but their active involvement is needed for this initiative to have a real impact on the health care system. Carol McCall of Humana suggested that additional work is also needed to increase patient demand for practical clinical research.

Panel II: Developing an Infrastructure to Support Practical Clinical Research

Sean Tunis of CMS began by discussing the need for a new “evidence initiative.” Substantial variation in health care spending and utilization persists over time in part because of a lack of appropriate actionable evidence. The two main sources of clinical research in the U.S. today primarily focus on discovery (NIH funded studies) and regulatory approval (industry studies). A third pillar of research focused on decision support is essential. The existing clinical research enterprise is not designed to provide actionable evidence for patients and providers, therefore a new evidence initiative is essential. Dr. Tunis outlined CMS' evidence objectives, foremost of which is providing better information for patients and physicians in the context of expanded incentives for effective decision-making. Other objectives include:

- Supporting innovation while ensuring systematic learning about optimal use;
- Expanding the capacity of the clinical research enterprise to produce information oriented to decision makers; and
- Moving towards a system in which delivery and evaluation are a single process.

Dr. Tunis then discussed strategies for combining coverage with evidence development or “coverage under protocol.” Under this approach, coverage is linked to prospective data collection – retaining evidence-based medicine as the conceptual framework for coverage and payment decisions. Payers will increasingly need to move from passive users of information to active collectors. Coverage under protocol could be used to inform decisions about promising, potentially high value drugs, devices, and treatments under investigation.

Putting these systems in place requires setting clinical research priorities. Dr. Tunis outlined some of the key issues in priority setting, including safety and side effects; off label uses and new combinations of approved uses; potential risks and benefits for population sub-groups; improved understanding of patients and provider types excluded from clinical trials; comparative effectiveness; additional outcomes not measured in trials; and non-regulated or lightly-regulated new techniques. Dr. Tunis briefly discussed the effort already underway at CMS, including the lung-volume reduction surgery registry; PET scans for patients with suspected Alzheimer's disease; the implantable defibrillator; and off-label uses of approved cancer drugs. Dr. Tunis concluded by discussing conceptual and implementation issues. Many issues such as priority setting, funding, governance, methodology, and infrastructure require substantial development.

Jack Rowe of Aetna echoed the need for expanded investment in developing evidence spanning from registries to clinical trials to large observational databases. While CMS may set the initial parameters, it is crucial for industry to work closely with CMS. Health plans are willing to work cooperatively with federal agencies and many plans would be willing to help fund registries for post-market surveillance as long as there is an equitable financing mechanism. Since this effort requires a public/private collaboration, Dr. Rowe argued that IOM would be a perfect institution to house this effort. He concluded by reiterating the need to set clear priorities and perhaps establish a “registry of registries” to support coordination across myriad efforts presently underway.

David Brennan of AstraZeneca expressed his support for the centrality of the physician-patient relationship in these discussions and felt that this was “good news” for the pharmaceutical industry. There is substantial room for expanded post-marketing surveillance; however, Mr. Brennan argued that improving patient care should be the primary goal rather than cost. While much discussion around cost containment has revolved around prescription drug spending, pharmaceuticals have remained at approximately 10 percent of health spending for years. Making a real impact on spending growth will require a much broader look at practices across the health care system. Mr. Brennan also expressed support for a transparent process to ensure that the concepts of evidence-based medicine are used appropriately.

Barbara McNeil of Harvard Medical School noted that the kind of observational data that registries produce may be problematic. Provider and patient characteristics can be important confounders for this type of data. Using observational data correctly will mean identifying elements that can be measured before registries are created. Joseph Jackson from Pfizer reinforced Dr. McNeil’s comments, stating that care is required in interpreting observational data.

Dr. Tunis commented on the importance of conducting “value of information” analyses before creating registries. A committee of stakeholders will need to establish priorities and to consider how many registries should be created, for what conditions, and when. Dr. Rowe stipulated that registries should be used where the long term utility and safety of a drug, device or treatment are unclear. Registries are generally better for evaluating safety than efficacy. Currently, health plans perform individual technology assessments, as new drugs, devices and treatments are introduced. Since these efforts are highly duplicative, a coordinated industry-wide technology assessment process may be preferable.

Joseph Jackson of Pfizer cautioned the group to proceed carefully when going from randomized clinical trials to observational studies, since what happens in the real world may differ substantially – for example, patients don’t stay on their medications. Dr. Berger commented that registries are good for studying safety and rare events, but that for other types of analyses, new methodologies are necessary “to separate the wheat from the chaff” in observational data. Dr. Tunis noted that observational studies have many limitations, but they provide a good start given the need for expanded practical data describing “real world outcomes.” As more costs are shifted to patients, demand for this kind of information, in a usable format, will grow.

Christobel Selecky of LifeMasters commented that the discussion seems to have separated the question of clinical effectiveness from cost effectiveness. She suggested that policy makers not just push the cost issue off on the health plans, but rather acknowledge that the cost problem is a societal issue.

Ms. Ignagni asked how these initiatives would be structured, noting that it was still unclear who would be responsible for thinking about the broad strategic questions, with NIH, AHRQ, IOM, FDA, and private groups all playing an important role. Dr. Altman noted that the private sector has much greater flexibility than the government and that this is another important reason why a public/partnership makes sense.

Ms. Darling commented that it is important to create a structure for this initiative that is viewed as trustworthy by public and not tied too closely to any particular interest group. Ms. Weinberg noted that her organization is conducting surveys help understand what information consumers think that they need. John Rother of AARP concurred, stating that this is “urgent work.”

Dr. Pauly commented that registry data sounded like a “public good” and asked whether researchers would have broad access to the information. Dr. Rowe responded that thoughtful data standardization is important, with collection, methods of analysis and availability of data important factors to be resolved. Karen Williams of the National Pharmaceutical Council commented that greater consensus is needed on how data will be interpreted. Stakeholders need to be a part of the coverage decisions arising from these efforts, given that patients will still want access to drugs, devices, and treatments that evidence-based medicine suggests will have only limited benefits for them.