Statement

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Considerations in Setting National Priorities for Comparative Effectiveness Research to be conducted under the American Recovery and Reinvestment Act of 2009

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The Association of American Medical Colleges (AAMC) welcomes the opportunity to submit this statement for the record in junction with the March 20, 2009 public forum convened by the Institute of Medicine Committee on Comparative Effectiveness Research Priorities. The AAMC applauds the Committee for holding this public forum, underscoring the importance of input from a broad range of stakeholders relevant to setting priorities for research on comparative effectiveness of health care services.

The AAMC is a not-for-profit association representing all 130 accredited U.S. and 17 accredited Canadian medical schools; nearly 400 major teaching hospitals and health systems, including 68 Department of Veterans Affairs medical centers; and 94 academic and scientific societies. Through these institutions and organizations, the AAMC represents 109,000 faculty members, 67,000 medical students, and 104,000 resident physicians.

The U.S. health care system faces a crisis of access, cost, and quality that must be addressed now. The Association of American Medical Colleges (AAMC) and its members believe that ensuring access to safe, high-quality, appropriate, and affordable patient-centered health care is, and should continue to be, the focal point of all health care reform discussions. Broadly defined, access is the timely, efficient, and effective provision of the most appropriate treatment for all in the most appropriate setting. Clearly research is essential to achieving this goal. Additional research is required to determine the most effective treatments to address the health care needs and concerns of specific patients. We understand this is the type of knowledge provided through comparative effectiveness research. Just as important is research that will clarify the best approaches to achieving timely, efficient, and effective provision of the services required by specific patient conditions—particularly those that rely upon the unique resources of teaching hospitals and physicians.

The U.S. health care system is recognized for discovering and providing life-saving treatments for many of the most difficult diseases and conditions and for educating a highly skilled workforce of clinicians and scientists. Yet, at the same time, many believe that in its current form, it is on an unsustainable course. The current system is costlier than other nations' health care delivery systems, does not provide insurance coverage for all, does not adequately emphasize preventive and primary care services, and is characterized by wide variation in utilization and the quality of care delivered.

In the last few decades, landmark developments in genetics, bioengineering, neuroscience, and molecular and structural biology have vastly increased our understanding of the causes of disease and raised new possibilities for treatment and prevention. However, there is a gap between the pace of scientific and technological advancements and the successful translation of this science into effective medical and health practices at the bedside, in the clinic, and in the community.

Clinical effectiveness research, and related research on knowledge translation, patient engagement, and health system transformation are key to converting biomedical discoveries into effective new approaches to the diagnosis, treatment, and prevention of human illnesses. The AAMC believes that medical schools and major teaching hospitals are uniquely situated to provide both the institutional support and the rigorous training necessary to conduct high quality, comparative effectiveness research and to nurture physician-scientists equipped to exploit scientific opportunities.

I. The AAMC's Health Care Reform Principles

The AAMC and its members are committed to the following principles and believe that academic medicine must play a pivotal role in improving health and health care and in achieving positive changes in the health care system. We believe that, with a concerted national effort from both the private and public sectors, the goal of affordable, quality health care for all is achievable and sustainable within the next decade. The AAMC's reform principles are available at: http://www.aamc.org/newsroom/pressrel/2008/081028.htm. In summary they state that:

- Health care coverage that is affordable, transportable, and continuous, and that combines the best of public and private systems, should be available to all.
- The health care delivery system must be restructured to facilitate health promotion and disease prevention while providing high-quality, cost-effective diagnosis and treatment of illness as well as palliative care.
- Health care financing mechanisms should be sustainable, equitable, explicit, accountable, and promote efficiency and quality.
- Existing programs that serve defined populations should be maintained until superior alternatives can fully replace them.
- The supply of health care practitioners must be adequate and reflect the population and its health care needs.
- Any reconfiguration of the health care system should recognize and provide stable support for the costs inherent in health research, technology development, and the provision of necessary specialized services to the broader society.

II. Background on Priorities for Comparative Effectiveness

In the US, policy makers, politicians, health professionals, and voters agree on few things. But they now agree that the US health care system must be improved. Millions of Americans do not receive much needed, highly effective health care while at the same time clinical services of little or unknown benefit are widely provided. There are various causes cited for these failings, yet there is a growing consensus that the consequences are grave. Rising health care costs are unsustainable. Health services researchers note poorly understood but dramatic regional and local variations in care and patients often do not receive the benefit of well proven, highly effective treatments. Scholars and policy experts assert that if medical practice were more evidence-based, substantial savings could be achieved without loss of access or quality of care. Because much of this evidence base is now lacking, clinicians are often faced to make decisions based upon limited data. Accordingly, better information on clinical effectiveness is seen to be a critical element of the solution to the national health care costs and quality deficits.

Many policymakers note the current production of comparative effectiveness research, "evaluating the impact of the different options that are available for treating a given medical condition for a particular set of patients" has been inadequate. Therefore a broad range of stake holders have agreed on the need for public investments to provide more and better information

on comparative clinical effectiveness, and Congress has concurred, providing $1.1 Billion through the ARRA to "jumpstart" this work.

AAMC and its members recognize that one important aspect of the IOM Committee's work is to identify specific clinical questions that are highest priority to be answered through new investments in CER. This is an important undertaking and through our nearly 400 major teaching hospitals and health systems, our 94 academic and scientific societies and our many thousands of clinical faculty, our constituents have deep insights relevant to these questions. Doubtless many will be contributing to your process through their specific affiliations. Therefore, AAMC will focus its comments on the question of the" highest priorities for developing new or enhanced systems, alliances, or capacities to sustain a national comparative effectiveness research enterprise."

III. Summary of Recommendations

1. **The AAMC strongly supports investments to further develop research methods to support the national comparative effectiveness research enterprise**
2. **The AAMC identifies as another high priority robust, sustained investment in research training in the disciplines relevant to comparative effectiveness research to enhance the skill, supply and diversity of the research workforce**
3. **The AAMC strongly supports investments to develop and sustain the national research infrastructure for CER as among the highest priorities for developing the national comparative effectiveness research enterprise.**
4. **The AAMC strongly supports further research to inform clinical care and the development of delivery system reforms.**

IV. Recommendations

1. **The AAMC strongly supports investments to further develop research methods to support the national comparative effectiveness research enterprise.**

Perhaps the least visible, but important, CER work is the development of robust research methods. A host of different techniques and approaches have developed in the past 50 years after the emergence of the randomized controlled clinical trial as a standard of evidence. Of course, RCTs can not be the only form of evidence used to determine relative clinical effectiveness, given their many limitations, including cost, timeliness, and the challenge of applying evidence to clinically relevant subpopulations.. New sources of data offer important opportunities; for example, use of electronic health records is expanding, especially among teaching hospitals and faculty group practices. For many health plans, claims data are being linked to clinical information as part of Pay for Performance (P4P) programs, creating richer administrative data sets for research. Clinical registries are expanding for a variety of procedural interventions, devices and therapeutics. All of these trends lead to increased options for secondary data analysis relevant to CER. With these and other expanded data sources, increased numbers of observations, and greater richness of clinical detail, new analytic methods aiding valid inference

from observational research should be a high priority. Moreover, every effort must be made to utilize these new sources of data while protecting the privacy of patient information.

2. **The AAMC identifies as another high priority robust, sustained investment in research training in the disciplines relevant to comparative effectiveness research to enhance the skill, supply and diversity of the research workforce**

Systematic development of the CER workforce will also be important. Comparative effectiveness research represents the fullest expression of the community application side of "translational research." Thus, there are many features of the discipline of CER that are relevant to the "discipline of clinical and translational science" fostered by the NIH and the "homes for clinical and translational research training" being developed in leading academic medical centers. Within the broad area of clinical and translation research, the skilled scholar of CER must have expertise not only in traditional clinical trial design, but also pragmatic/practical clinical trials and Bayesian modeling/ adaptive trial design, quasi-experimental/observational studies of clinical effectiveness, meta-analysis, clinical outcomes measurement and utility assessment..

3. **The AAMC strongly supports investments to develop and sustain the national research infrastructure for CER as among the highest priorities for developing the national comparative effectiveness research enterprise.**

In addition to the further development of the intellectual discipline and human resources for CER, investments will be required to develop and sustain key infrastructure relevant to efficient conduct of comparative effectiveness research. One fundamental need is for expanded networks for evidence review, the fundamental building block for comparative effectiveness research. The most substantive federal effort to date is AHRQ's Evidence Based Practice Centers, but such a network will likely need substantial expansion. With the rapid evolution of health information technology, and clinical data, there will be opportunities to augment existing networks of clinical data and to establish and sustain new clinical research databases that could encourage the timely conduct of CER projects. Many CER questions will need to address questions of effectiveness in patients with specific, multiple conditions, cared for in a variety of settings. Therefore CER studies will be facilitated by clinical research networks that are multidisciplinary, and can address multiple conditions, clinical circumstances, and practice settings. These must be able to quickly undertake large scale clinical trials, preferably using community-based clinical resources.

With the publication of the 2006 Report of its Task Force II on Clinical Research, AAMC has endorsed major investments by academic medicine in these areas. Among the prominent recommendations of this report are that "academic medical institutions should establish collaborations with community healthcare providers and practice-based research networks to broaden the diversity and size of the population base for translational and clinical research and to increase opportunities for health services, epidemiological, and outcomes research.:" and that they "should explicitly recognize and vigorously promote translational and clinical research as a core mission." Accordingly, many leading academic medical centers have already made substantial initial investments in the kinds of interdisciplinary research teams and academic – community partnerships needed for efficient conduct of successful, clinically relevant CER.

4. **The AAMC strongly supports further research to inform clinical care and the development of delivery system reforms.**

Clinicians' decisions are informed not only by their training and existing knowledge, but also by the timely, relevant availability of new knowledge. Physicians and others must treat patients on a daily basis for whom no relevant clinical trials exist that fully capture the conditions and preferences of single individuals. As a result, many are forced to make decisions which have little or no data to inform them. Further investment in clinical information useful to the practicing clinician must be developed alongside health services research which informs the systems and processes which facilitate this care delivery.

V. Conclusion

The problems of cost and quality that beset our health care delivery system do not lend themselves to easy solution. As indicated by our reform principles, the AAMC and its members believe that new investments in biomedical, clinical effectiveness, and health services research are key to discovering the answers to the nations' health care crisis. Medical schools and major teaching hospitals are uniquely situated to provide both the institutional support and the rigorous training necessary to conduct high quality, comparative effectiveness research and to nurture physician-scientists equipped to exploit scientific opportunities.

The AAMC's members provide the only environment where clinical care, research, and the training of the next generation of health care providers and researchers occur side by side. Any reconfiguration of the health care system must also recognize and provide stable support for the costs inherent in innovative clinical research, training, and technology development. Such research, when integrated with the development of medical and health systems knowledge, is the keystone to a vibrant "learning" health care system. This work will become increasingly important as we strive to enhance the evidence base for clinical care.