

Clinical Trials Have a Role in Comparative Effectiveness Research, Expert Contends

As the United States invests in comparative effectiveness research (CER) and examines the use of a multitude of research methods, at least one researcher cautions “not leaving randomized clinical trials by the side of the road because they are not going to become obsolete any time soon.”

Sean Tunis, the founder of the Center for Medical Technology Policy, added “we are not going to be able to abandon any time in the near future, experimental methods including traditional randomized clinical trials to answer key questions in comparative effectiveness” at a June 9 forum hosted by the Brookings Engelberg Center for Health Care Reform and The Hamilton Project that examined the key questions surrounding CER, which received a major boost from the American Recovery and Reinvestment Act of 2009 with a \$1.1 billion allocation for federal CER initiatives.

Tunis was tasked with developing a position paper for the forum on strategies to improve comparative effectiveness research methods and data infrastructure.

Although traditional clinical trials will still be important, Tunis noted that “it will be necessary to go beyond the current approaches to conducting clinical and health services research.” These include: systematic reviews of existing research, including meta-analysis; decision modeling, with or without cost information;

retrospective analysis of existing clinical or administrative data, including natural experiments; prospective non-experimental studies, including registries, which observe patterns of care and outcomes, but do not assign patients to specific study groups; and experimental studies, including randomized clinical trials.

Experimental Studies Continue To Be Crucial

“Experimental studies will continue to be a crucial source of CER information, and for those questions that are best addressed with these methods, it is critically important to develop study designs and infrastructure that will generate credible and relevant information, as quickly and as inexpensively as possible,” the position paper said.

“Many CER questions will not be adequately addressed by analyzing routinely collected data from large administrative databases or electronic medical records, given the widely recognized challenges of making valid comparisons between alternative strategies for managing health conditions, without being able to confidently account for unmeasured differences between comparison groups,” the paper said. “Some clinical and policy decisions will require experimental evidence because of the potential to do harm by widely applying potentially incorrect findings from non-experimental studies,” the paper said.

“Studies that are faster, less costly, and more generalizable will often be acceptable as long as there is room for error in the decision-making process,” the paper said. “Where there is less tolerance for error, experimental research will still be necessary, and new design techniques and implementation strategies will be required to make this research less resource-intensive and more broadly applicable.”

Tunis said that all of the “methods have a role. The key is to make sure that you are using right methodology for the right question. Experimental studies will have a crucial role in comparative effectiveness research and the key thing is using them when they are necessary and being able to design them as simply as possible, so that they can be done as cheaply, as quickly, and as efficiently as possible.”

He noted that critics contend “randomized controlled trials are not generalizable because they exclude all kinds of patients. That is not a fixed characteristic of randomized trials. Randomized trials can be designed

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