Making Comparative Effectiveness Research Work: Looking Back and Looking Ahead

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Now that health care reform has finally become law and the health policy community has had a chance to contemplate the nearly 3000 pages of relevant legislation, I will resume my occasional series for CHPE by reflecting on key elements of this wide-sweeping legislation. I will start with a long-standing interest of mine – comparative effectiveness research. As I've previously recounted in these columns, by 2007 a broad range of stakeholders were calling for expanded public investment in comparative effectiveness research (CER), but disagreements had emerged related to four fundamental issues: definition, funding, direction, and uses of CER. The recently passed Patient Protection and Affordable Care Act (PPACA) sets in law a general approach to resolving these controversies, but key differences were reflected in the House of Representatives proposal. Indeed, negotiations remained quite active behind the scenes in an effort to compromise on these key issues, until it became clear that the "reconciliation" approach to passing health care reform would not allow further resolution. Not surprisingly, these controversies may continue to influence the further development and implementation of publically funded CER.

Interestingly, on the Hill, consensus emerged on the usually thorny issue of "who should pay for CER?" Both the House and Senate proposals established a trust fund from an assessment on public and private health insurance. Private insurers had endorsed this approach in 2007, and many policy makers favored it as well, fearing the annual appropriations process allows for too many opportunities for political interference with the CE research agenda. Intense opposition also developed in 2007 however, when legislation adopting this approach was decried as the "mystery midnight tax." While the prospect of universal health insurance may reduce some large employer objections to a CER tax, there is plenty of opportunity for resistance to this public support for CER to re-emerge before the levy goes into effect in 2013. And there is also the need to renew the funding of CER in 2019 since it sunsets in the PPACA.

Not surprisingly, the issue of "How can CER be used?" has remained a topic of intense advocacy. The PPACA settled a Recovery Act debate, inserting the term "clinical" into comparative effectiveness research. This seems to have been done in part to preclude research focused on cost comparisons, like "cost-effectiveness analysis." In the same vein, there are explicit prohibitions on the use of CER funds to calculate quality adjusted life years. There are also various legislative declarations that findings from CER cannot determine CMS coverage policy. In the face of relentless growth in health care costs and the hopes that more and better CER can improve the value of this spending, it seems inevitable, however, that controversy will continue on how CER can be used to "bend the curve" of health care spending.

Similarly intense controversy emerged during the Recovery Act debate over "who should direct CER", when the proposed Federal Coordinating Council for Comparative Effectiveness Research was denounced as the "Federal Euthanasia Board." Sadly, but not surprisingly, this useful tool for good government, designed to coordinate CER efforts across relevant agencies, got caught up in anti-government rhetoric and was repealed by the PPACA. The new Patient Centered Outcomes Research Institute is established as an expressly "outside government" entity. With its multi-stakeholder governance of high stakes health care research, its control of a large tax-supported trust fund, and its complex and potentially overlapping relationship to existing federal research agencies (especially AHRQ and NIH), the implementation of this new Institute presents unique policy challenges. For example, how shall this private entity's Board interpret the congressionally mandated "preference" to use AHRQ or NIH to conduct research within their authority?

Perhaps the biggest question left to this multi-stakeholder Board to decide is "what is CER?" When initially proposed by policy makers, CER was to solve the problem that "little rigorous evidence is available about which treatments work best for which patients." (Orszag, NEJM, 2007). The CER proposal passed by the House (but not the Senate) in 2007 expressly focused on informing decisions "at the point of care." Subsequent advocacy from a wide array of stakeholders has so broadened this interpretation that in the PPACA, CER can include "…delivery…integrative health practices, and any other strategies…used in the …management…of illness or injury in individuals." Of course each trust fund dollar spent to answer a health services research question (especially one that could have been addressed through current NIH or AHRQ appropriations) is one less dollar to resolve a pressing clinical controversy. This can delay needed answers at the bedside and possibly facilitate continued use of a profitable but valueless product or service. Such delays certainly might be in some stakeholder's interest, but not the taxpayers'!

The PPACA builds on the important public investment in CER made by the Recovery Act. As with other elements of health reform, however, several controversial political and policy difficulties in implementing CER have been deferred for debate at a later time and place. Stay tuned! The resolution of these issues may prove key to determining the ultimate success of CER as a strategy for improving US health care.