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Prospects For Comparative Effectiveness Research Under Federal Health Reform

*Eleanor D. Kinney, J.D., M.P.H.**

I. INTRODUCTION

Comparative Effectiveness Research is a type of health services research that could possibly transform the health care sector of the United States. At least that is what many federal policy makers and third party payers are hoping as health care costs continue to rise at alarming rates. In 2009, national health expenditures grew four percent to \$2.5 trillion, or \$8,086 per person, and accounted for 17.6 percent of the Gross Domestic Product (“GDP”).¹ This increase in health care costs threatens the success of newly enacted health reform as well as existing public and private health insurance programs and plans.

This paper describes federally sponsored comparative effectiveness research and policy. In addition to laying out aspirations and apprehensions about the use of comparative effectiveness research, the paper addresses the prospects for comparative effectiveness research as a successful strategy for bending the proverbial cost curve in health care expenditures.

II. FEDERAL EFFORTS TO PROMOTE COMPARATIVE EFFECTIVENESS RESEARCH

The 2009 economic stimulus legislation, the American Recovery and Reinvestment Act of 2009 (“ARRA”),² authorized \$1.1 billion to the National Institutes of Health, the Agency for Healthcare Research and Quality (“AHRQ”) and the Office of the Secretary of the Department of Health and Human Services (“DHHS”) to fund comparative effectiveness

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1. Ctrs. for Medicare & Medicaid Servs., NHE Fact Sheet National Health Expenditure Data (June 14, 2011), https://www.cms.gov/NationalHealthExpendData/25_NHE_Fact_Sheet.asp#TopOfPage.

2. American Recovery and Reinvestment Act of 2009, Pub. L. No. 111-5 § 804, 123 Stat. 115 (codified at 42 U.S.C. § 299b-8) [hereinafter ARRA].

research. The ARRA established a Federal Coordinating Council for Comparative Effectiveness Research, which became the basis of Sarah Palin's allegation that the health reform would establish "death panels" to ration care,³ but whose actual task is to coordinate comparative effectiveness research across the federal government.⁴

A. *Work of the Institute of Medicine*

ARRA also directed and funded the Institute of Medicine ("IoM") to develop a definition of comparative effectiveness research as well as research priorities.⁵ Congress delegated the task of priority setting for comparative effectiveness to the IoM to facilitate consultation with interested stakeholders and to mitigate concerns that the federal government was setting priorities directly.⁶

Immediately upon enactment in February 2009, the IoM appointed the Committee on Comparative Effectiveness Research Prioritization. The Committee issued its report, *Initial National Priorities for Comparative Effectiveness Research*, in June 2009.⁷ The Committee's report defined comparative effectiveness research as follows:

CER is the generation and synthesis of evidence that compares the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care. The purpose of CER is to assist consumers, clinicians, purchasers, and policy makers to make informed decisions that will improve health care at both the individual and population levels.⁸

Two key elements that are embedded in this definition: (1) direct comparison of effective interventions, and (2) studies involving patients who are typical of day-to-day clinical care.⁹ The IoM Committee then

3. Eleanor D. Kinney, *The Real Truth about Death Panels: Comparative Effectiveness Research and the Health Reform Legislation*, OKLA. CITY U. L. REV. (forthcoming 2011).

4. ARRA § 804(a) (codified at 42 U.S.C. § 299b-8(a)). See U.S. DEP'T HEALTH & HUMAN SERVS., FED. COORDINATING COUNCIL FOR COMPARATIVE EFFECTIVENESS RESEARCH, REPORT TO THE PRESIDENT AND CONGRESS (June 30, 2009), <http://www.hhs.gov/recovery/programs/cer/cerannualrpt.pdf>.

5. ARRA § 804 (codified at 42 U.S.C. § 299b-8). See John K. Iglehart, *Prioritizing Comparative-Effectiveness Research - IoM Recommendations*, 361 NEW ENG. J. MED. 325 (2009); Harold C. Sox & Sheldon Greenfield, *Comparative Effectiveness Research: A Report From the Institute of Medicine*, 153 ANNALS INTERNAL MED. 203 (2009).

6. Sox & Greenfield, *supra* note 5, at 203.

7. Inst. of Med., *Comparative Effectiveness Research Prioritization* (Sept. 14, 2010), <http://iom.edu/Activities/Research/CERPriorities.aspx>.

8. INST. OF MED., COMM. ON COMPARATIVE EFFECTIVENESS RESEARCH PRIORITIZATION, INITIAL NATIONAL PRIORITIES FOR COMPARATIVE EFFECTIVENESS RESEARCH 13 (Nat'l Acad. Press 2009).

9. Sox & Greenfield, *supra* note 5.

selected one hundred topics for comparative effectiveness research after obtaining extensive input from professional organizations and the public.¹⁰

B. The Patient Protection and Accountable Care Act and Comparative Effectiveness Research

In March 2010, Congress enacted and President Obama signed into law the Patient Protection and Affordable Care Act (“PPACA”),¹¹ as amended by the Health Care and Education Reconciliation Act of 2010.¹² The joined legislation is called the Affordable Care Act (“ACA”). This legislation initiated comprehensive health reform for the health care sector of the United States, including increasing access to health care coverage through expansion of public programs and reform of the private health insurance market. Of several initiatives to improve the quality and control the cost of healthcare services in the ACA, the most important is support for comparative effectiveness research through the establishment of the Patient-Centered Outcomes Research Institute (“PCORI”).

The ACA defines “comparative clinical effectiveness research” and “research” follows:

The terms ‘comparative clinical effectiveness research’ and ‘research’ mean research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of 2 or more medical treatments, services, and items described in subparagraph (B).¹³

Subparagraph (B) describes the medical products, procedures and services subject to comparative effectiveness research under the act as follows:

The medical treatments, services, and items described in this subparagraph are health care interventions, protocols for treatment, care management, and delivery, procedures, medical devices, diagnostic tools, pharmaceuticals (including drugs and biologicals), integrative health practices, and any other strategies or items being used in the treatment, management, and diagnosis of, or prevention of illness or injury in,

10. Inst. of Med., 100 Initial Priority Topics for Comparative Effectiveness Research, <http://iom.edu/~media/Files/Report%20Files/2009/ComparativeEffectivenessResearchPriorities/Stand%20Alone%20List%20of%20100%20CER%20Priorities%20-%20for%20web.ashx>.

11. Patient Protection and Affordable Care Act, Pub. L. No. 111-148, 124 Stat. 119 (2010) [hereinafter ACA]. Upon enactment of the ACA, the Federal Coordinating Council created in the ARRA terminated. *Id.* § 6303.

12. Health Care and Education Reconciliation Act of 2010, Pub. L. No. 111-152, 124 Stat. 1029 (2010).

13. ACA § 6301(a).

individuals.¹⁴

The ACA establishes a new organization, PCORI, to conduct and supervise federally-funded comparative effectiveness research. The ACA defines the purpose of the PCORI as follows:

The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis that considers variations in patient subpopulations, and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services, and items described in subsection (a)(2)(B).¹⁵

The PCORI has a unique structure.¹⁶ It is a private, nonprofit entity governed by a public-private sector board of directors appointed by the Comptroller General and organized under the District of Columbia Nonprofit Corporation Act.¹⁷ It is independently funded through a federal trust fund and contributions from the Medicare program trust funds and from private health plans and insurers.¹⁸

The specific duties of the PCORI are straightforward and described in the statute in great detail.¹⁹ The duties all concern developing and executing a research project agenda. Several “duties” pertain to establishing processes to ensure the quality of the research, the proper dissemination of research results, and the transparency and integrity of the research process. The statute is unusually detailed in the degree to which it specifies processes for developing methodologies for comparative effectiveness research and other aspects of PCORI’s supervision of research.

The ACA imposed several important limits on the use of PCORI comparative effectiveness research.²⁰ Specifically, the statute provides: “Nothing in this section shall be construed . . . to permit the Institute to mandate coverage, reimbursement, or other policies for any public or

14. *Id.*

15. *Id.*

16. This section describing the structure of the PCORI draws on Eleanor D. Kinney, *Comparative Effectiveness Research under the Patient Protection and Affordable Care Act: Can New Bottles Accommodate Old Wine?* AM. J. L. & MED. (forthcoming 2011).

17. ACA § 6303(b)(1)-(2).

18. ACA § 6301(d)-(e).

19. ACA § 6301(c).

20. *Id.*

private payer. . . .”²¹ Nor can the PCORI develop or employ a “dollars per-quality adjusted life year” or similar measures that discount the value of a life because of disability as a “threshold” to establish what type of health care is cost effective or recommended.²² Further, the ACA prohibits the Centers for Medicare & Medicaid Services (“CMS”), except with complete transparency and with extensive procedural safeguards, from using such measures as a threshold to determine Medicare coverage or reimbursement or in other incentive programs.²³ As will be discussed below, these limits were imposed to address concerns among patients, consumers and providers as well as more conservative politicians that the federal government would use the results of comparative effectiveness research to ration health care based on such bloodless criteria.

III. THE ASPIRATIONS FOR COMPARATIVE EFFECTIVENESS RESEARCH

The aspirations for comparative effectiveness research are huge. One indication of their enormity is the amount of money that Congress and the Obama administration have allocated for comparative effectiveness research. As indicated above, ARRA authorized \$1.1 billion for comparative effectiveness research.²⁴ This is an astounding sum of money for health services research. The allocation to the AHRQ alone was more than its total budget of \$370 million for fiscal year 2009.²⁵ The entire budget of the National Institutes of Health including funds for all medical research in fiscal year 2011 is \$31.2 billion.²⁶

In a 2009 interview in the *New York Times Magazine*, President Obama talked about his expectations for comparative effectiveness research. In a famous statement, he said:

So when Peter Orszag²⁷ and I talk about the importance of using comparative-effectiveness studies as a way of reining in costs, that’s not an attempt to micromanage the doctor-patient relationship. It is an attempt to say to patients, you know what, we’ve looked at some objective studies out here, people who know about this stuff, concluding that the blue pill, which costs half as much as the red pill, is just as effective, and you might want to go ahead and get the blue one. And if a

21. ACA § 6301.

22. ACA § 6301(c).

23. *Id.*

24. *See* ARRA, *supra* note 2.

25. Agency for Healthcare Research & Quality, Fiscal Year 2009 Budget at a Glance (May 2009), <http://www.ahrq.gov/about/fy09glance.htm>.

26. National Inst. of Health, NIH Budget (May 2011), <http://www.nih.gov/about/budget.htm>.

27. The former Director of the Office of Management and Budget in the Obama Administration and a great supporter of comparative effectiveness research.

provider is pushing the red one on you, then you should at least ask some important questions.²⁸

The Obama administration sees comparative effectiveness research as the silver crowbar to bend the healthcare cost curve without compromising quality. The work of Dr. John Wennberg and colleagues, which has demonstrated that variation in the practice of medicine is great with unexplained variation in services among different geographic areas for the same conditions,²⁹ greatly influenced the Obama administration and its embrace of comparative effectiveness research. Dr. Wennberg and colleagues have established the Dartmouth Atlas Project, which uses Medicare data to analyze utilization of health care services in different geographic markets and by individual hospitals and affiliated physicians.³⁰ This body of research was dramatized in a widely read and very influential article in the *New Yorker* magazine.³¹ The *New Yorker* article describes the variations between two Texas cities in a most illustrative manner:

Between 2001 and 2005, critically ill Medicare patients received almost fifty per cent more specialist visits in McAllen than in El Paso, and were two-thirds more likely to see ten or more specialists in a six-month period. In 2005 and 2006, patients in McAllen received twenty per cent more abdominal ultrasounds, thirty per cent more bone-density studies, sixty per cent more stress tests with echocardiography, two hundred per cent more nerve-conduction studies to diagnose carpal-tunnel syndrome, and five hundred and fifty per cent more urine-flow studies to diagnose prostate troubles. They received one-fifth to two-thirds more gallbladder operations, knee replacements, breast biopsies, and bladder. They also received two to three times as many pacemakers, implantable defibrillators, cardiac-bypass operations, carotid endarterectomies, and coronary-artery stents. And Medicare paid for five times as many home-nurse visits. The primary cause of McAllen's extreme costs was, very simply, the across-the-board overuse of medicine.³²

The basic aspiration is that results of comparative effectiveness research

28. David Leonhardt, *After the Great Recession*, N.Y. TIMES MAG. (May 3, 2009), available at <http://query.nytimes.com/gst/fullpage.html?res=980CE7D8153BF930A35756C0A96F9C8B63&pagewanted=all>.

29. See John E. Wennberg et al., *Professional Uncertainty and the Problem of Supplier-Induced Demand*, 16 SOC. SCI. & MED. 811 (1982); John E. Wennberg & John Gittelsohn, *Small Area Variation in Health Care Delivery* 182 SCI. 1102 (1973); John E. Wennberg & John Gittelsohn, *Variations in Medical Care Among Small Areas*, 246 SCI. AM. 120 (1982).

30. The Dartmouth Atlas of Health Care, <http://www.dartmouthatlas.org/>

31. Atul Gawande, *The Cost Conundrum: What a Texas Town can Teach us about Health Care*, NEW YORKER (June 1, 2009), http://www.newyorker.com/reporting/2009/06/01/090601fa_fact_gawande?currentPage=all.

32. *Id.*

will permeate the practice of medicine over time and greatly reduce inexplicable and often costly variations in healthcare services. Ideally payers should use the results in determining the content of health plans. Patients, physicians, and other providers would have no difficulty with these plan changes because these changes would be based on sound and persuasive evidence developed in realistic clinical settings.

IV. APPREHENSIONS ABOUT COMPARATIVE EFFECTIVENESS RESEARCH

There is much concern within the healthcare sector about comparative effectiveness research. Patients, consumers, and their physicians are concerned that comparative effectiveness research results will be used to ration care.³³ This position has been voiced very effectively by conservative politicians.³⁴ Physicians and other providers have mixed views on comparative effectiveness research. Physicians are concerned about the ability to tailor clinical care to individual patients who might need different therapies and approaches than conventional practice would dictate.³⁵

Pharmaceutical and medical device manufacturers are very concerned about comparative effectiveness research as well. These industries have made it known that they do now want either the Food and Drug Administration or CMS, which makes Medicare coverage policy for new medical treatments, to have authority to mandate comparative effectiveness research, especially involving clinical trials. Such mandates would greatly complicate the research and development process, which is already cumbersome, timely and expensive.³⁶ There is a real concern that comparative effectiveness research could show that profitable products may not be more advantageous than less costly therapies.

A most insightful article in the *Bloomberg Businessweek* in 2010 aptly described the apprehensions of the pharmaceutical and medical device industries in reporting on remarks of John L. Sullivan, Director of Research at Leerink Swann and its Healthcare Investment Strategist:³⁷

Still, “the savings can be substantial if you’re drawing a clinical study

33. See Kinney, *supra* note 3.

34. See Jonathan Chait, *Why Do Conservatives Oppose Comparative Effectiveness Research?*, NEW REPUBLIC BLOG (March 11, 2011, 3:31 PM), <http://www.tnr.com/blog/jonathan-chait/85120/why-do-conservatives-oppose-comparative-effectiveness-research>.

35. Alan M. Garber & Sean R. Tunis, *Does Comparative-Effectiveness Research Threaten Personalized Medicine?* 360 NEW ENG. J. MED. 1925, 1925 (2009).

36. Scott Gottlieb, *The FDA Should not Mandate Comparative-Effectiveness Trials*, AM. ENTERPRISE INST. FOR PUB. POL’Y RES. (June 2011), <http://www.aei.org/docLib/HPO-2011-05-g.pdf>.

37. Alex Nussbaum et al., *Health Care Reform’s Cost-Cutting Scalpel*, BLOOMBERG BUSINESSWEEK (Mar. 26, 2010), available at http://www.msnbc.msn.com/id/36043001/ns/business-bloomberg_businessweek/.

conclusion that a generic drug works as well as a branded drug,” says Leerink Swann’s Sullivan, who suggests that therapies used by large numbers of people are likely to be researchers’ first targets. That means that Pfizer’s (PFE) cholesterol blockbuster, Lipitor, could be in the crosshairs, Sullivan says, along with anti-inflammatory drugs such as Johnson & Johnson’s (JNJ) Remicade, and heart stents made by Medtronic (MDT) and Boston Scientific (BSX).³⁸

Of interest, much thought has been given to the methodology of comparative effectiveness research.³⁹ For a major concern of policy-makers, health services researchers and practicing physicians is that the research reflects the conditions of conventional medical practice with a variety of patients. Many medical professionals maintain that findings from current clinical trials used to test the safety and efficacy of pharmaceutical products and medical devices do not reflect the conditions of medical practice and thus their findings are less relevant to medical practitioners in conventional clinical settings.

V. CONCLUSION

In sum, there are a host of questions about comparative effectiveness research that will dog comparative effectiveness research and the use of its results. Such questions include:

- Will comparative effectiveness research provide the basis for government rationing?
- Will public and private payers use comparative effectiveness research results to change coverage policy?
- Will comparative effectiveness research threaten the clinical autonomy of physicians?
- Will comparative effectiveness research thwart personalized medicine?
- Will comparative effectiveness research impose undue burdens on pharmaceutical and medical device manufacturers in bringing new products to market?
- Will comparative effectiveness research stifle innovation in pharmaceuticals and medical devices?

Comparative effectiveness research will certainly provide empirical information about options for medical care. It will also give public and

38. *Id.*

39. See e.g., Susan D. Horn & Julie Gassaway, *Practice-Based Evidence Study Design for Comparative Effectiveness Research*, 45 MED. CARE S50 (2007); Brian R. Luce et al., *Rethinking Randomized Clinical Trials for Comparative Effectiveness Research: The Need for Transformational Change*, 151 ANNALS INTERNAL MED. 206 (2009); Gail R. Wilensky, *Developing a Center for Comparative Effectiveness Information*, 7 HEALTH AFF. w572-85 (2006).

private payers the information needed to make cost cutting decisions. Great care must attend comparative effectiveness research and medical products to ensure that new products come to market and innovation continues.

However, the real success of comparative effectiveness research is up to patients, consumers and physicians. Drs. Sox and Greenfield have aptly described the interest in comparative effectiveness research and the conditions needed for its success in the changing world of medical practice:

In the end, whether CER [“comparative effectiveness research”] will realize its potential will depend on other features of health care reform legislation, the profession’s ability to mobilize the best in its members, and the ability of individual patients to engage their physicians in a dialogue about their own care.⁴⁰

40. Sox & Greenfield, *supra* note 5, at 5.