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CONGRESS APPROVES US$1.1 BILLION FOR COMPARATIVE EFFECTIVENESS RESEARCH

I. INTRODUCTION

As part of the US$787 billion economic stimulus bill President Obama signed into law on February 17, 2009, Congress appropriated US$1.1 billion to various federal agencies to conduct and fund comparative effectiveness research (CER) studies. Such studies are intended to identify which treatments and products are most effective through systematic retrospective reviews of completed studies or by new prospective clinical trials specifically designed to compare the effectiveness of various treatments.

This landmark appropriation clearly indicates that the Obama Administration, Congress, and a broad group of stakeholders in the health policy arena have embraced the concept of CER. However, numerous unresolved and potentially contentious issues remain. In particular, some industry and patient groups are concerned that a focus on the comparative cost of medical treatments could drive payment and coverage decisions in a way that limits patient access to certain therapies or constrains physicians' choices. In addition, incorporating the results of CER studies into medical practice presents significant practical challenges, including whether the results of comparative studies will help physicians to achieve any better clinical results or cost savings.

The stimulus law, the American Recovery and Reinvestment Act of 2009[1], leaves the US Department of Health and Human Services (HHS) broad discretion in designing and conducting its CER program, so these concerns remain important for stakeholders. In addition, the law likely is only a down payment on government funding for CER studies, and this debate may resurface if Congress later considers broader healthcare reform legislation.

II. ECONOMIC STIMULUS LAW

A. Summary

The stimulus law appropriates US$1.1 billion to the Agency for Healthcare Research and Quality (AHRQ), the National Institutes of Health (NIH), and HHS to conduct or support CER studies. In particular, the law appropriates US$400 million to HHS to:

> accelerate the development and dissemination of research assessing the comparative effectiveness of health care treatments and strategies, through efforts that: (1) conduct, support, or synthesize research that compares the clinical outcomes, effectiveness, and appropriateness of items, services, and procedures that are used to prevent, diagnose, or treat diseases, disorders, and other health

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> conditions; and (2) encourage the development and use of clinical registries, clinical data networks, and other forms of electronic health data that can be used to generate or obtain outcomes data.

An additional US$300 million is appropriated to increase funding for CER performed by AHRQ, which involves systematic retrospective studies to synthesize findings of completed clinical trials,[2] and US$400 million to the NIH (which may be distributed to its institutes or the Common Fund established under § 402A(c)(1) of the Public Health Service Act) to conduct or support CER.

The stimulus law creates a Federal Coordinating Council for Comparative Effectiveness Research (the Council) to "foster optimum coordination" between various CER-related activities conducted or supported by the federal government. The President, acting through the HHS Secretary, will appoint a 15-member, government-only panel of healthcare experts—half of whom must be physicians or others with clinical expertise—to coordinate federal CER efforts and advise the president and Congress on CER concerns, such as infrastructure needs, organizational expenditures, and opportunities for optimum coordination of CER efforts. The Council must include one senior officer or employee from AHRQ, the Centers for Medicare & Medicaid Services (CMS), NIH, the Office of the National Coordinator for Health Information Technology, the Food and Drug Administration (FDA), the Department of Veterans Affairs (VA), and the office within the Department of Defense responsible for the Military Health Care System. Members of the Council must be appointed within 30 days of enactment.

Priority setting under the new law will begin quickly. The Council must produce an "initial report" by June 30, 2009, which will describe current federal CER activities and make recommendations for research activities to be supported under the new funding. The law also calls for the Institute of Medicine (IOM) to publish a report by June 30, 2009, that "includes recommendations on the national priorities for comparative effectiveness research to be conducted or supported with the funds provided in this paragraph [the US$400 million appropriated to HHS] and that considers input from stakeholders." Before using any of the appropriated funds, the HHS Secretary must submit to Congress an "operating plan…[that] detail[s] the type of research being conducted or supported, including the priority conditions addressed." The operating plan for FY 2009 is due by July 30, 2009.

B. Key Provisions

The CER provision ultimately enacted includes several changes from earlier versions, which sparked concern in the healthcare industry. Most of the debate centered around whether research funded under the bill would focus on comparative cost or comparative clinical effectiveness. In January 2009, the House passed a version of the CER provision that did not distinguish between clinical effectiveness and cost-effectiveness research. The report accompanying the House-passed bill included the following statement:

> By knowing what works best and presenting this information more broadly to patients and healthcare professionals, those items, procedures, and interventions that are most effective to prevent, control, and treat health conditions will be utilized, while those that are found to be less effective and in some cases, more expensive, will no longer be prescribed.

The idea that more expensive or less effective treatments "will no longer be prescribed" suggested that the CER provision could ultimately be used to inform coverage decisions that would limit beneficiary access to treatments. This also raised concerns that the program could lead to government rationing of healthcare.

Less than two weeks later, the Senate passed its version of the CER provision, which omitted the controversial report language and focused on "comparative *clinical* effectiveness," rather than merely "comparative effectiveness." This revision was intended to allay concerns that the government would sponsor comparative cost-effectiveness studies and then use them to restrict patient access to certain medical technologies.

However, the final conference report version—like the House-passed version—omits the word "clinical" from its descriptions

2 Section 1013 of the 2003 Medicare Modernization Act (MMA) gave AHRQ the authority to evaluate and synthesize the results of clinical studies.

of "comparative effectiveness." The joint explanatory statement accompanying the conference report notes that the final bill "deletes without prejudice the term 'clinical.'"

The final stimulus law also lacks associated report language similar to the problematic statements in the House report. In fact, the joint explanatory statement emphasizes that the House-Senate conferees do not intend government-funded CER research "to be used to mandate coverage, reimbursement, or other policies for any public or private payer." In addition, the report notes that "a 'one-size-fits-all' approach to patient treatment is not the most medically appropriate solution to treating various conditions," and the legislation "ensure[s] that subpopulations are considered" when conducting government-funded CER.

The law also includes the following "rules of construction" barring the newly created CER Council from *mandating* any coverage or payment policies based on the results of CER studies:

> Nothing in this section shall be construed to permit the Council to mandate coverage, reimbursement, or other policies for any public or private payer… None of the reports submitted under this section or recommendations made by the Council shall be construed as mandates or clinical guidelines for payment, coverage, or treatment.

III. FUTURE LEGISLATION?

The law's new framework for government-funded CER could be fleshed out or revised in future healthcare legislation. In recent years, various groups have supported the development of better and more reliable comparative information about health products and services and weighed in on the features a longer-term CER structure should have.

The Medicare Payment Advisory Commission (MedPAC), an independent governmental body that advises the Medicare program on policy and administration, has encouraged Congress to create a comparative effectiveness program. MedPAC has argued that CER has "the potential to promote care of higher value and quality in the public and private sectors."[3]

In 2007, MedPAC recommended that Congress create an independent, public-private entity to produce and disseminate reliable CER information for healthcare services.[4] More recently, MedPAC expanded on that recommendation and offered detailed options for Congress on how to structure the entity's governance and funding[5] but did not make specific structural recommendations.

The nonpartisan Congressional Budget Office (CBO) also has expressed support for the concept of CER. At a 2007 Congressional hearing, Peter Orszag, then-CBO director, testified that expanded CER, "if linked to changes in incentives for providers and patients, offers a promising mechanism for reducing healthcare costs to a significant degree over the long term while maintaining or improving the health of Americans."[6] Dr. Orszag is now Director of the Office of Management and Budget (OMB), which oversees federal spending.

MedPAC and the CBO have also suggested that comparative *cost* effectiveness research can be a useful tool. In its recent reports, MedPAC did not rule out the possibility that cost effectiveness analysis could play some role, though it maintained that comparative clinical effectiveness should be the "primary mission" of a CER entity.[7] The CBO has noted that assessing comparative cost effectiveness of treatments would result in "a somewhat larger effect on healthcare spending" than studying comparative clinical effectiveness alone "because it would help highlight cases in which the additional benefits of a more costly treatment are relatively small."[8]

Other groups have been less restrained about advocating cost effectiveness analyses. For example, in December 2008, an IOM panel released a report calling for comparative effectiveness and cost effectiveness research as part of a modernized HHS.[9] The IOM report presented state-level Medicare data that failed to show any relationship

3 MedPAC, "Report to the Congress: Promoting Greater Efficiency in Medicare" 30 (June 2007).

4 *Id.* at 29-54.

5 MedPAC, "Report to the Congress: Reforming the Delivery System" 107-137 (June 2008).

6 Testimony of Peter R. Orszag, Director of the Congressional Budget Office, before the House Committee on Way and Means Subcommittee on Health (June 12, 2007).

7 MedPAC, "Report to the Congress: Reforming the Delivery System" 108 (June 2008).

8 Congressional Budget Office, "Key Issues in Analyzing Major Health Insurance Proposals" 146 (Dec. 2008).

9 Institute of Medicine, "HHS in the 21th Century: Charting a Course for a Healthier America" 85 (Dec. 12, 2008) (prepublication copy).

between healthcare spending and healthcare quality; instead, "the state with the *highest*-quality care, is at the low-cost end…whereas the two states where care is most expensive…have among the lowest quality ratings."[10] The panel recommended that HHS "establish a capability for *assessing the comparative value—including clinical and cost effectiveness*—of medical interventions and procedures, preventive and treatment technologies, and methods of organizing and delivering care."[11] But the three industry representatives on the 15-member panel dissented from this recommendation, primarily expressing concern with its focus on cost effectiveness.[12]

The most speculative aspect of this issue is whether, when, and to what extent, the practical application of CER findings could actually reduce costs for healthcare payors. The CBO has consistently found that although CER may yield eventual cost savings, any budgetary recoupment of federal outlays will be incremental, likely will occur far in the future, and will depend heavily on what incentives exist for providers and payors to change behavior.

In a 2007 score of CER legislation introduced by Representative Pete Stark (D-CA), the CBO projected that it would be "a decade or more" before any new comparative effectiveness research would yield significant savings in healthcare spending.[13] Nevertheless, the score noted that such research is important because of the rate of growth of healthcare spending and substantial geographic disparities in healthcare spending that appear unrelated to differences in healthcare quality or outcomes. The CBO also has noted that little evidence exists about "whether the added benefits of more effective but more expensive services are sufficient to warrant their added costs."[14]

In a December 2008 report, the CBO again expressed skepticism about CER's near-term benefits, calculating that a federal program to fund CER would increase the federal deficit by US$860 million over 10 years.[15] Toward the end of that period, however, "the annual reduction in healthcare spending is estimated to be slightly larger than the increased spending on research," suggesting savings beyond the 10-year window.[16] The CBO warned that predicting such savings is difficult because it is unclear what results the new CER studies would produce.

These CBO scores could create a difficult situation for Congress if future efforts to overhaul the healthcare system stay within the "pay-go" rules that Congressional Democrats put into effect at the beginning of the last Congress.[17] Even if a comparative effectiveness program is expected to yield long-term savings, any savings that accrue outside CBO's 10-year budget window are not reflected in its score (assuming that existing pay-go rules apply). Ultimately, broader national spending priorities—rather than the particular details of any future CER proposal—may determine whether policymakers will be willing to make another expensive short-term investment that would yield savings, if any, only in the long term.

Moreover, CBO and other groups have warned that CER data alone is not enough to achieve significant cost savings: to realize savings in healthcare spending, any new focus on comparative effectiveness must also include incentives for patients, physicians, and payors to change behavior based on that information. That issue raises the specter, once again, that future initiatives could authorize Medicare to use CER results to shape coverage or payment policies or to influence prescribing patterns.

Senate Finance Committee Chairman Max Baucus (D-MT) was supportive of the "short-term funds" for comparative effectiveness research included in the stimulus law, but also recently stated in a colloquy with other Senators[18] that he plans to reintroduce a broader comparative effectiveness

10 *Id.* at 88.

11 *Id.* at 85.

12 *Id.* at 173-88.

13 Letter from Peter R. Orszag, Director of the Congressional Budget Office, to Rep. Pete. Stark (Sept. 5, 2007) (scoring section 904 of H.R. 3162 (110th Congress), the Children's Health and Medicare Protection Act of 2007).

14 Peter R. Orszag & Philip Ellis, "The Challenge of Rising Health Care Costs—A View from the Congressional Budget Office," 357 *New England J. Med.* 1793, 1795 (Nov. 1, 2007).

15 Congressional Budget Office, "Budget Options, Volume 1: Healthcare" (Dec. 2008).

16 *Id.* at 86.

17 The stimulus bill was exempt from the pay-go rules.

18 Colloquy of Chairman Baucus, Senators Hatch, Conrad, Enzi, Menendez, and Carper of the Committee on Finance, Regarding Comparative Effectiveness Research (Jan. 27, 2009).

bill to provide a "long-term framework" for CER.[19] Key Finance Committee Republicans expressed guarded support for CER legislation. Senator Enzi (Ranking Member of the Senate Committee on Health, Education, Labor, and Pensions (HELP)) stressed that he supports research on clinical effectiveness, not cost effectiveness, and that CER should "consider differences in how people respond to treatments." Senator Hatch, Ranking Member of the Finance Committee, similarly commented that he supports CER that is focused on clinical effectiveness, not cost. He also noted that CER should not be a "one size fits all" solution: "[W]e all know that what works best for one, does not always work the same for the other." How the new stimulus law's CER provision is implemented will be important for many reasons, but (among other things) it could affect the shape of any future CER legislation and will certainly frame the debate over any future legislation.

IV. CONCLUSION

Although much of the policy debate on this issue focuses on potential impacts of CER—such as cost savings or limited access to therapies—questions remain about how CER studies can actually be performed, and whether meaningful data can be produced. In particular, it is difficult for long-term clinical studies to take account of new therapies that come to market mid-study, or the variability of real world patient populations. Thus, this appropriation of funds is merely the start of what will be a long process to set research priorities and design studies to produce meaningful results. Nonetheless, the newly enacted CER provision in the economic stimulus law should increase the body of knowledge comparing the effectiveness of various therapies and treatments. The law provides broad discretion to HHS to design its CER activities, and it will be important for industry to remain involved and encourage the agency to implement this CER program in a transparent and rigorous manner that preserves choice for physicians and patients.

19 In July 2008, Senator Baucus introduced a major bill (S. 3408) that has been seen as one of the leading models for broader CER legislation. S. 3408 would have created a quasi-governmental, nonprofit research institute to fund comparative effectiveness studies. The institute would contract with government agencies (such as AHRQ) and private entities to conduct both systematic reviews of existing studies as well as new clinical trials comparing the clinical effectiveness of various treatment options. The institute also would control priority-setting for CER research. Unlike the stimulus bill, S. 3408 envisioned a priority-setting body with broad stakeholder representation, including patient and healthcare consumers; practicing physicians; public payors (Medicare, other federal health programs, and state health programs); private payors; pharmaceutical, device, and technology manufacturers or developers; quality measurement or decision support organizations; and independent health services researchers. Also unlike the stimulus bill, S. 3408 would have relied on public and private financing for CER studies. Private health insurance plans would pay annual per-covered-life fees. The Medicare trust fund would also would pay a per-beneficiary fee.

We hope that you have found this client advisory useful. If you have additional questions, please contact your Arnold & Porter attorney or:

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