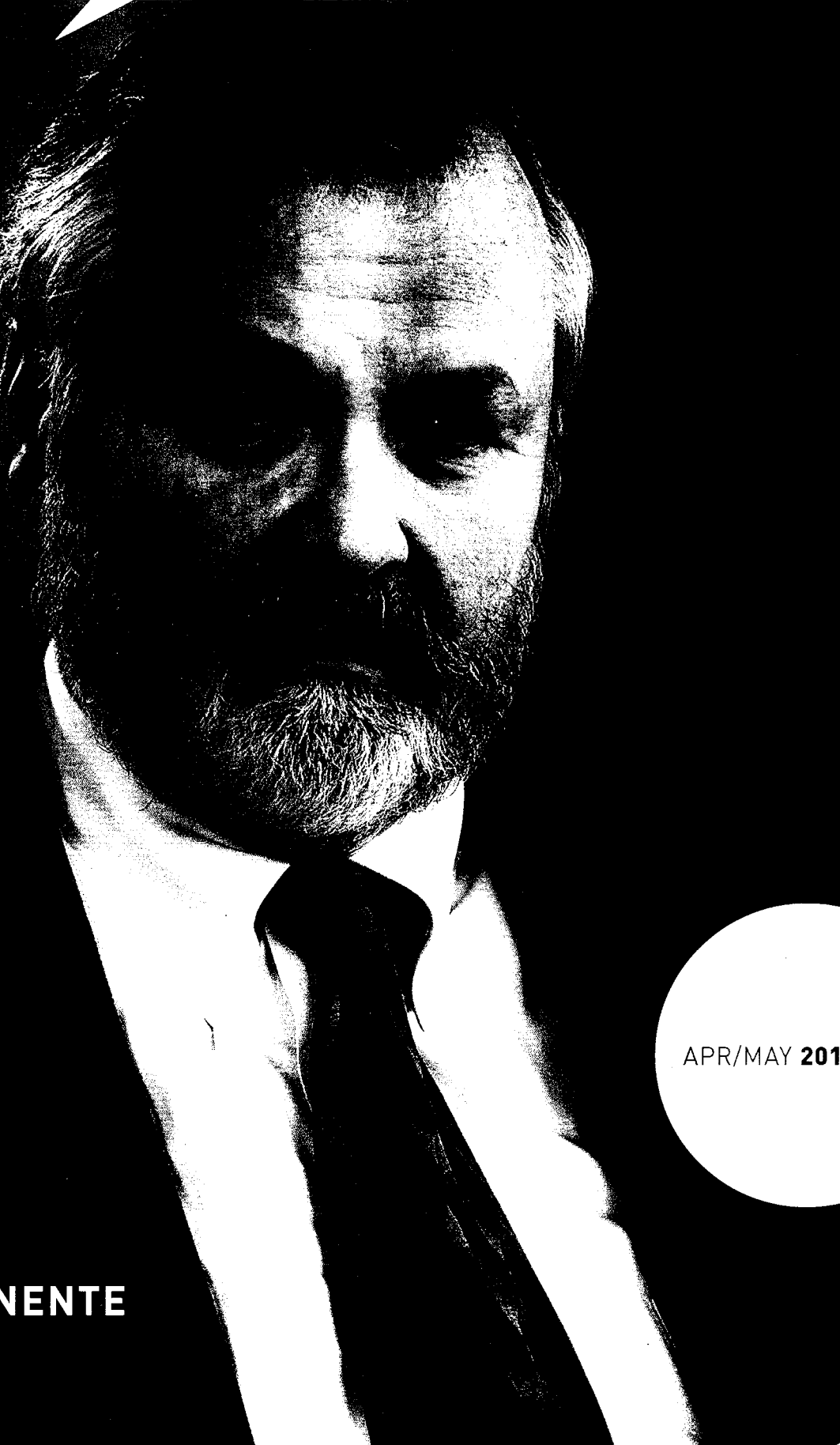


HCE

EXCHANGE

Key Issues: Real Solutions



APR/MAY 2010

KAISER PERMANENTE
Key to the future

HEALTH CARE REFORM: COMPARATIVE EFFECTIVENESS RESEARCH

STAKEHOLDERS SHOULD UNDERSTAND AND MONITOR DEVELOPMENTS IN COMPARATIVE RESEARCH PRIORITIES, METHODOLOGIES AND COMMUNICATION OF RESEARCH FINDINGS.

BY SHEILA D. WALCOFF AND
PAUL W. RADENSKY M.D.,
MCDERMOTT WILL & EMERY, LLP

The recently enacted Patient Protection and Affordable Care Act (PPACA) built on federal efforts to support and direct research comparing patient treatments. Drug manufacturers, diagnostics companies, medical device manufacturers and health services providers should carefully monitor and selectively engage in the formal and informal processes that will shape the development of the Patient Centered Outcomes Research Institute, conduct of research and communication of research findings.

Background

Section 6301 of the PPACA amends Title XI of the Social Security Act [42 USC 1301 et seq.] to add a new Part D on comparative clinical effectiveness research. Under the PPACA, comparative clinical effectiveness research means research that evaluates and compares the patient health outcomes and benefits of two or more medical treatments or services. Such treatment and services are defined broadly to include protocols for treatment, care management and delivery; procedures; diagnostic tools; medical devices; therapeutics;

and any other strategies used to treat, diagnose or prevent illness or injury. (See 42 U.S.C. 1181(a)(2)(A)-(B)).

The PPACA establishes a new Patient Centered Outcomes Research Institute (PCORI) responsible for the identification, prioritization and execution of such comparative effectiveness research. The PCORI will be structured as a tax-exempt independent government corporation overseen by a board of governors that includes the directors of the Agency for Health Care Research and Quality (AHRQ) and the National Institutes of Health (NIH), plus 17 members appointed by the Comptroller General (U.S. Government Accountability Office). The PPACA requires three industry representatives on the board.

The PCORI is responsible for setting national clinical comparative effectiveness research priorities and is directed to enter into contracts to manage the funding and conduct of research, with preference given to AHRQ and NIH. The PCORI will be responsible for establishing a standing research methodologies committee (comprising up to 15 members, each of whom is appointed by the Comptroller General) to develop standards for clinical comparative effectiveness, but the PCORI will conduct no research itself. Nevertheless, the PCORI is required to appoint advisory panels that are expert in carrying out randomized clinical trials under the PCORI's research project agenda and to appoint an expert advisory panel for purposes of assisting in the design of research studies and in determining the value and feasibility of conducting research studies for rare diseases.

ABOUT THE AUTHOR

Paul W. Radensky, M.D. and **Sheila D. Walcoff** are partners in the law firm of McDermott Will & Emery LLP. Dr. Radensky is a recognized authority on the full range of legal, regulatory and reimbursement issues pertaining to pharmaceutical, biotechnology, medical device, and clinical laboratory development and marketing. Ms. Walcoff focuses her practice on personalized medicine and other federal regulatory and science policy matters, counseling a broad range of clients on policy, government affairs advocacy, business strategy and communications. They can be reached at pradensky@mwe.com or swalcoff@mwe.com.

Research will be funded by the newly established Patient Centered Outcomes Research Trust Fund (PCORTF), which shall receive appropriations from private insurance based taxes. However, this funding source is time limited, and any amounts remaining in the PCORTF after September 30, 2019, will no longer be available for research and must be transferred back to the general treasury.

For purposes of developing the research agenda and supporting such research, the PCORI will have access to Medicare and Medicaid data. Within 90 days of receipt of findings or the completion of the research, the PCORI must publish research findings and any limitations, as well as what further research may be needed, in a manner useful to clinicians, patients and the general public in making health care decisions. Such findings may not include practice guidelines, coverage recommendations, or payment or policy recommendations. Importantly, there is no requirement that the findings communicated to the public be consistent with U.S. Food and Drug Administration approved labeling of regulated products. Federal payors are not prohibited from using research findings to inform payment, coverage and treatment decisions. However, comparative research findings alone may not be used to deny coverage.

Congress already added significant funding and direction to federally sponsored comparative effectiveness research in 2009 when it appropriated \$1.1 billion for comparative effectiveness research through AHRQ; NIH; and the U.S. Department of Health and Human Services, Office of the Secretary, under the American Recovery and Reinvestment Act (the Stimulus Act). The Stimulus Act created a Federal Coordinating Council for Comparative Clinical Effectiveness Research charged with comparative effectiveness research priority setting and development of strategies to support translation and dissemination of comparative effectiveness research findings. This Council was terminated upon enactment of the PPACA provisions establishing the PCORI.

Implications for Personalized Medicine

The PPACA specifies that research design should account for individual differences and sub-populations, but the ability to use and misuse data will remain ever present, since research findings will be greatly influenced by the methodological standards that will be developed under the auspices

of the PCORI's Methodology Committee. Measures will be developed to assess quality through comparative effectiveness, but can also assess value, which will likely include an evaluation of comparative cost and patient compliance factors, such as treatment regimen and unpleasant or harmful side effects. It is important to assess how widely a study reporting results across a population/sub-population applies to individuals or smaller sub-populations with the larger studied population.

Bending the Cost Curve

Comparative effectiveness research findings have the potential to identify savings in the health system and improve patient outcomes, but the most effective treatments are not necessarily the least costly treatments. The development of a balanced agenda with appropriate methodological standards, as well as identifying the appropriate context to communicate effectively such findings to all stakeholders, including clinicians, patients and payors, will be essential to those overarching objectives. In addition, it is important for those reviewing or using comparative effectiveness data to understand what the clinical findings show separate from economic outcomes. These endpoints taken together help assess value, but economic endpoints should not blur or confuse the presentation of the comparative clinical outcomes.

Implications for Stakeholders

Given the broad implications of the establishment of the new PCORI and its role across the research enterprise, medical products industry and health delivery system, stakeholders should understand and monitor developments in comparative research priorities, methodologies and communication of research findings. Product manufacturers and service providers, particularly in areas where regulated products or services are targeted at specific subpopulations, should consider engaging proactively in the formal and informal processes that will shape the development of the PCORI and ongoing research.