Preface

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The Medicare Modernization Act of 2003 (MMA) launched a new era in national health policy in the United States. In addition to increasing access to outpatient prescription medications through a new federal program, this landmark legislation creates new opportunities and a renewed national interest in improving the quality, safety, and effectiveness of health care across all settings. The Part D drug benefit is in the forefront of this interest because the appropriate use of medications holds the potential to improve the health of many Americans as well as the effectiveness of nearly all other health care services in which pharmaceuticals are an integral component. Nevertheless, expanded access and use of drug therapies also holds the potential for poor outcomes resulting from the adverse drug effects of medications, suboptimal treatment response, inappropriate choice of therapy, and gaps in knowledge on the true effects of a therapy across all patient populations and conditions for which it is used. These results are especially true as treatments, over time, are used in a broader spectrum of patients, clinical settings, and conditions than studied in premarketing clinical trials. Furthermore, premarketing studies are generally not designed to evaluate multiple endpoints, detect rare adverse effects, evaluate health outcomes over long periods, or compare all treatment options available to patients and their health care providers. As the federal government becomes a major payer of prescription drugs while also providing insurance coverage for hospital and medical care through Medicare, a new national focus has emerged on understanding the outcomes, effectiveness, and comparative effectiveness of treatments in clinical practice. This includes understanding the balance of risks and benefits for all forms of therapies as they are used beyond clinical research settings in routine clinical care.

The need of patients, health care providers, and policy makers for objective information about the outcomes and effectiveness of therapeutic interventions was specifically addressed in Section 1013 of the MMA. Section 1013 authorized the Agency for Healthcare Research and Quality (AHRQ) to conduct systematic reviews and research on topics of highest priority to Medicare, Medicaid, and the State Children’s Health Insurance Program (SCHIP). To implement Section 1013, AHRQ established the Effective Health Care program with a goal to increase knowledge about the balance of risks and benefits of therapies so that health care decisions are improved. The program is comprised of 3 components that work collaboratively to evaluate, synthesize, develop, and disseminate scientific evidence about the effectiveness of various treatment options. The first component of the program is the Evidence-based Practice Centers (EPCs), which conducts systematic reviews of available scientific evidence about the effectiveness of different treatments and identifies gaps in the existing knowledge base. To help fill these gaps, the program supports new research and the development of new methods through the Developing Evidence to Inform Decisions about Effectiveness (DEcIDE) research network and the Centers for Education and Research on Therapeutics (CERTs) program. Most authors of articles in this supplement are also investigators affiliated with the DEcIDE and CERTs centers. The third component of the program is the John M. Eisenberg Clinical Decisions and Communications Science Center, which translates the
program’s scientific findings into nontechnical language and specific formats to better communicate to different audiences.

To develop valid scientific evidence that can be used to inform decisions about the effectiveness of treatments, advances are needed in research methods, analytic tools, measurement, and study designs. This is particularly important to do using approaches that can be applied in actual clinical care settings and taking advantage of existing data that are collected as part of health care delivery in a growing number of information systems. These advances will need to overcome the current limitations of observational and quasi-experimental methods created by issues such as unmeasured confounding, selection bias and other biases, and poor measurement instruments. Furthermore, they will need to be rigorously applied and appropriately interpreted in light of any limitations. The value of new methods will partially depend on the level of evidence they provide to generate new hypotheses, support existing hypotheses, or provide conclusive information about the relationship between a therapy and its outcomes.

As the lead Federal agency for improving the quality, safety, and effectiveness of health care, AHRQ’s broad mission includes supporting science that can be applied to solving problems facing the nation’s health care systems. In light of this mission, the new Part D benefit, and the goals of the Effective Health Care program, AHRQ sponsored a 2-day symposium of researchers who presented new approaches to designing studies, identifying sources of data for research, and developing methodologies for studying health outcomes, safety, effectiveness, and comparative effectiveness. The proceedings of the symposium have been independently peer-reviewed and compiled to form this Medical Care supplement. This supplement is meant to serve as a resource to scientists investigating the safety and effectiveness of treatments, particularly the effects of therapies whose efficacy has been previously established through randomized controlled trials. This supplement is not intended to endorse particular research methods or address all the challenges of conducting observational and quasi-experimental research. Instead, it brings together the current thinking of experts in the field to publicly begin a national discussion about ways science can be appropriately used to provide valid and useful information for improving health outcomes and take advantage of the growing sources of electronic health information, including Part D data, for improving public health.

I extend my sincere thanks to the many people who contributed to the production of this journal supplement. I am particularly grateful to Dr. Kathleen Lohr and her colleagues at the RTI DEcIDE center who led this project and helped shape its direction. Without question, the symposium and this supplement would not have been completed without Kathy’s leadership, experience, and dedication. I also acknowledge and thank Dr. Brian Strom of the University of Pennsylvania DEcIDE and CERT, who cochaired the symposium. His advice and intellectual guidance were invaluable to the success of the symposium and supplement. The project is also indebted to several staff at AHRQ, particularly Jean Slutsky who directs the Center for Outcomes and Evidence and is the leader of the Effective Health Care program. The project benefited significantly from her support and assistance. I am also immensely thankful to each of the authors for their contributions, particularly in meeting a compressed publication timeline.

As the pace of discovery and development rapidly brings new therapies to clinical care, there is an expanding need for research on the safety and effectiveness of these therapies. The methods and approaches described in this supplement form a foundation for developing robust scientific evidence that can be used to assist patients, providers, and policy makers in making informed decisions about treatments. As the nation embarks upon a new era in health policy with the Part D benefit, these methods and those that will be developed in the future hold great potential for advancing the field of epidemiology, science of observational research, and ultimately improving the outcomes of health care.