This product is part of the Pardee RAND Graduate School (PRGS) dissertation series. PRGS dissertations are produced by graduate fellows of the Pardee RAND Graduate School, the world's leading producer of Ph.D.'s in policy analysis. The dissertation has been supervised, reviewed, and approved by the graduate fellow's faculty committee.

DISSERTATION

Effects of Trial Design on Participation and Costs in Clinical Trials, with an Examination of Cost Analysis Methods and Data Sources

MEREDITH L. KILGORE

This document was submitted as a dissertation in March 2004 in partial fulfillment of the requirements of the doctoral degree in public policy analysis at the Pardee RAND Graduate School. The faculty committee that supervised and approved the dissertation consisted of Jacob Klerman (Chair), Dana Goldman, and Emmett Keeler.

PARDEE RAND GRADUATE SCHOOL

The Pardee RAND Graduate School dissertation series reproduces dissertations that have been approved by the student's dissertation committee.

Published 2004 by the RAND Corporation
1700 Main Street, P.O. Box 2138, Santa Monica, CA 90407-2138
1200 South Hayes Street, Arlington, VA 22202-5050
201 North Craig Street, Suite 202, Pittsburgh, PA 15213-1516
RAND URL: http://www.rand.org/
To order RAND documents or to obtain additional information, contact
Distribution Services: Telephone: (310) 451-7002;
Fax: (310) 451-6915; Email: order@rand.org

ABSTRACT

This dissertation comprises a series of studies conducted as part of the Cost of Cancer Treatment Study (CCTS). The specific aims include exploring theoretical issues concerning the problem of representativeness in trial design with an explicit investigation of the causes of the under-representation of older adults in clinical cancer trials; comparing sources of data and modeling approaches for estimating treatment costs in health services research; and estimating the impact of clinical trial participation on prescription drug costs.

An exploration of the sample size requirements for power and significance levels in clinical trials suggests that proportional representation of subpopulations in trials will often not allow valid inferences to be drawn about differential treatment effects. Where differential treatment effects in subpopulations are suspected, targeted trials should be undertaken. Under-representation of older cancer could be accounted for by exclusion criteria based on comorbid conditions that disproportionately afflict the elderly.

Data from patient interviews, medical records abstraction, provider billing records, and Medicare claims were compared as data sources for estimating health care utilization rates and costs; the data were compared in terms of completeness and accessibility. Medicare claims contain data on all covered services, including charges, and reimbursements. The costs of Medicare data compare favorably with other sources of comparable quality, but claims data are missing for individuals in managed care and do not include information on prescription drugs. Provider billing records, however, constituted a poor data source, primarily because providers were unwilling or unable to provide these records. Medical records provide accessible, detailed data on service utilization, but not costs. Self-reported health services utilization generally agreed with other sources on inpatient care but not with respect to outpatient services. Cost estimates for utilization measures were derived from administrative data using hedonic regression models.

Prescription drug costs and out-of-pocket drug expenditures were compared for patients enrolled in cancer trials and for similar cancer patients with who did not participate in trials. Trial participation was associated with higher prescription drug costs, but that did not result in any significant difference in out-of-pocket expenditures for participants. These results were robust to a variety of modeling approaches.