IDENTIFYING THE IMPACT OF A TREATMENT ON OUTCOMES AND COSTS USING OBSERVATIONAL DATA: OVERCOMING SELECTION BIAS

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OBJECTIVES: Selection bias in observational studies is described. Instrumental variable estimation is presented in detail as a method of identification of treatment effects. Practical issues related to the proper implementation of this technique are stressed. Propensity score methods are also discussed.

PARTICIPANTS WHO WOULD BENEFIT: Those who develop and use decision/cost-effectiveness models.

In an ideal world all clinical decisions would be based on evidence from large comprehensive scientific studies. In the complex world of actual practice—the world of pharmacoeconomics—such evidence is often impractical or impossible to obtain. Models are used to help decision-makers make plausible and useful inferences in the absence of perfect information. Despite the broad application of models in this context, the process and conclusions of modeling exercises are frequently suspect. Concerns include: (1) failure to use the best available data; (2) inaccessibility of the model code; (3) lack of validation; and (4) difficulty interpreting the results of technical analyses. Despite recommendations from the US Preventive Services Task Force intended to address these concerns, models are still held in low regard. As part of the Stroke PORT project, a simulation model of stroke development and outcomes was developed. This model has since been expanded to accommodate a variety of potential interventions and data sources and is currently designated the Duke Stroke Policy Model (DSPM). One of the major objectives of the DSPM has been to address the above concerns. Our approach has been to (1) use the best data; (2) vigorously promote an open-code public-use application, available on the Internet; (3) perform rigorous validation; and (4) develop user-friendly applications based on the validated core code, tailored to different user groups. Researchers, teachers and public health professionals are encouraged to use the DSPM for academic and other non-commercial applications, and to engage with the developers to promote the continuing improvement of the model. In this workshop, we will (1) lay out the set of conditions for assessing the quality of models, (2) provide examples from the DSPM of how these conditions may be met, and (3) engage participants in a discussion of cases in other clinical areas.

PARTICIPANTS WHO WOULD BENEFIT: Researchers who use, evaluate, or conduct observational studies.

While estimates of treatment outcomes and costs from randomized clinical trials are still considered the gold standard, the use of observational data for these estimates is common. Observational data are less costly to obtain, readily available, and represent actual practice. However, there is always a danger of selection bias when these data are used to identify the costs or outcomes of one particular treatment versus another. This is true even when quality risk adjustment methods and propensity score methods are employed. Instrumental variable estimation and propensity score methods have both been proposed to overcome the selection bias problem in observational studies. We describe selection bias and how instrumental variables analysis and propensity score methods overcome the problem. We then explain how one would apply these techniques correctly to common research problems emphasizing the importance of choosing when propensity score methods are sufficient or when instrumental variables analysis should be used.

PATIENT REGISTRIES: THEIR VALUE IN OUTCOMES RESEARCH

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OBJECTIVES: To provide a detailed overview of the registry concept and implementation, emphasizing its research value as well as its potential role in support of new product development and marketing and in the development and testing of disease management programs.

PARTICIPANTS WHO WOULD BENEFIT: Persons who have responsibility for planning or conducting outcomes research or disease management programs in the pharmaceutical industry, hospitals, or managed care.

There is growing enthusiasm for the use of health-care claims data bases representing thousands or even millions of covered lives to assess the potential value of medical interventions, including pharmaceuticals and disease management programs. However, evaluations of the effectiveness and safety of such interventions conducted in this manner may be confounded by clinical or patient differences between treatment groups that are not captured in readily-available databases. Patient registries designed to meet specific research objectives can provide a more robust source of data for outcomes research and disease management by linking administrative data bases, medical records, patient questionnaires, death certificates, etc. The result is comprehensive longitudinal information on diagnosis, treatment, course of illness, outcomes, quality of life, and costs of the diseases of interest. By seeking the informed consent of participants the registry can also obviate growing public concerns about the research use of confidential health-care data. Workshop participants will...