Right Tool, Right Time, Right Reason

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Previously in these pages we have discussed the potential of pharmacoeconomic research to manage the delicate balance between cost of care and quality of care. Now more than ever, our nation's healthcare statistics in economic and demographic terms point to the necessity of this valuable tool. Healthcare costs total more than 13% of our gross domestic product each year. And we are living longer than ever before. The National Center for Health Statistics reports that since 1990 life expectancy in the United States has dramatically increased. At the turn of the last century, less than half of all Americans lived past age 65, but today more than 80% of Americans can expect to live beyond age 65. When we add in the potential of genomics and other rapidly emerging health technologies to the convergence of rising costs and a large aging population, the imperative of pharmacoeconomics becomes clear. But to utilize this valuable tool, it is important to understand how it works alongside a clinical trial.

A pharmacoeconomic evaluation differs in focus from the traditional clinical trial in two ways. First, a trial with economic evaluation is more concerned with extrapolating to what happens in "real life" rather than under controlled conditions. The economic study is more interested in answering questions relative to effectiveness as opposed to efficacy. Second, the economic component of the trial is concerned with additional and different outcomes. Where a traditional clinical trial primarily focuses on medical indicators (eg, blood pressure, blood sugar, temperature, etc.), a clinical study with economic evaluation also determines the effects on resource consumption, productivity, and/or quality of life.

A common question arises as to the ideal time for conducting pharmacoeconomic studies. While these studies may take place at any of the clinical development stages, it is becoming increasingly important to have these data as soon as possible after approval by the Food and Drug Administration. To accomplish this, discussion and planning for pharmacoeconomic evaluation should begin in the early stages of drug development. Studies designed to evaluate the cost of disease and costs associated with current treatments can begin early in the development phases. Quality-of-life instrument development and validation also can begin in these stages. Cost-effectiveness and costs associated with toxicities and treatment failures can initially begin in Phase III. However, because Phase III trials are rigidly controlled, much of the pharmacoeconomic data profile of a drug will be generated after the drug is marketed. Once a drug reaches market, either retrospective or prospective pharmacoeconomic studies may be designed using pharmacoepidemiological and pharmacoeconomic methodologies. Pharmacoepidemiology studies are frequently employed to further study the efficacy and safety of drugs after they reach market. Epidemiological data with regard to the disease and treatment under investigation can yield highly important data in the economic evaluation of specific drug therapy.

In cancer research, pharmacoeconomics is increasingly playing a key role in the economic evaluation of new therapies. In general terms, economic endpoints are just starting to be measured during the clinical trial in addition to clinical endpoints. The possible relationships between pharmacoeconomic evaluations and clinical trials are threefold: (1) the economic evaluation may be a secondary objective of a trial designed primarily for safety and efficacy; (2) the economic evaluation may be the principal purpose of a clinical trial; or (3) the economic evaluation may be done retrospectively on clinical data obtained in previous trials.

Beyond the merits of a well designed clinical trial, the additional element in a pharmacoeconomic evaluation is a system to monitor or estimate resource consumption (direct costs and benefits), lost productivity through morbidity and/or premature death (indirect costs and benefits), and the impact of disease on quality of life. Quality of life is of course a paramount concern among cancer patients and oncologists. Currently there are more than 300 cancer medicines in development in the United States. The annual costs associated with diagnosing and treating cancer already top $107 billion. With such dramatic spending to fight cancer and many more treatments in the pipeline, the ability to ascertain some measure of quality of life in return is critical. Cost pressures will only increase alongside the price tag of these new treatments. Pharmacoeconomic assessment, particularly in terms of quality-of-life outcomes, will play an increasingly important role in documenting the outcomes of oncology treatment and in justifying these new therapies.

Pharmacoeconomic research adds a valuable tool for healthcare professionals at all levels. As clinical researchers and practitioners, we understand much about the natural progression of disease. With pharmacoeconomics, we also have the ability to estimate several variables that may impact cost-of-illness and quality-of-life issues. Pharmacoeconomics helps us better predict a wide range of outcomes related to our research endeavors. In an era of intense cost considerations and remarkable new medications, pharmacoeconomics is the right tool at the right time, for the right reason. OS

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