Concluding Discussion

Pharmacoeconomic Factors Related to the Treatment of Schizophrenia

Dr. H. Meltzer: Readers of this Supplement should find answers to questions such as: How should various stakeholders evaluate cost effectiveness? Is cost-utility analysis an accepted form of research? Why should managed care companies or government agencies be interested in pharmacoeconomic analysis? Why is the pharmaceutical industry concerned about pharmacoeconomic data? Why is it important to the pharmaceutical industry that the audience evaluating these data is knowledgeable?

Readers should also have discovered that current opinion about decision analysis seems to be unfavorable, and more information about using databases to collect pharmacoeconomic data is needed.

We seem to agree that the incremental advances in outcome possible with the newer psychotropic drugs are worth the cost of these agents from a societal perspective, although there is a crying need for pharmacoeconomic studies that prove this point. Clearly, we have not yet produced data that will make payers stand up and take notice.

Dr. Awad: My research fantasy is to simultaneously conduct 2-year prospective studies, one that uses modeling and cost-utility analysis and also uses more traditional approaches. Politicians listen politely when I discuss cost-utility and quality-adjusted years of life, but they are moved to action by issues related to quality of life.

Dr. H. Meltzer: The National Institutes of Mental Health (NIMH) in both Canada and the United States are interested in multicenter trials comparing various services. It is time for a group to think ambitiously about designing an ideal study with an adequate sample size, but will the methodology be rigorous enough to pass peer review?

Dr. D. Meltzer: Cost-utility analysis may not be perfect, but it is as useful as any other pharmacoeconomic method available today. Funding agencies either must accept cost-utility analysis or completely refuse to fund pharmacoeconomic studies.

Dr. Manning: I sit on the NIMH Health Services Study Section. Researchers commonly promise to include elements of cost-effectiveness analysis in a clinical study. Reviewers would be receptive to a large study that addressed some of the methodological issues that we have discussed today.

Dr. D. Meltzer: Most costs of treating patients with serious mental illness are borne by the states. Perhaps a coalition of state agencies and academic centers could be created for such a study.

Dr. Shon: It is naive to think that state mental health agencies will immediately agree to participate in the kind of study that is normally conducted in a university setting. The dynamics are absolutely different. Some of the issues that are germane to research methodology are absolutely unimportant to politicians and administrators.

Dr. Manning: The sample size must be large enough to provide useful information at the endpoint.

Dr. Diamond: Some public policy issues are of general concern. For example, if we designed a system that effectively tracked people with mental illness who are jailed, public policy makers would be interested in the data, particularly if we created a standardized method of inexpensively collecting information and provided financial and technical support for implementing the data collection. There may be other overlapping agendas that would provide us with the opportunity to conduct a naturalistic observation that would lead to useful cost-effectiveness data.

If states and managed-care organizations learned how to improve data collection, it would be relatively easy and inexpensive to collect specific data on a large number of subjects. This information would complement results from more rigorous controlled studies. In the Wisconsin Medicaid Managed Care Project, we're trying to build quality-of-life outcome indicators into the pilot project, even though we lack a substantial research budget. If states had standardized data collection systems, it would be easy to mine the data at a later date when research funds are available.

Dr. D. Meltzer: The most useful information is likely to come from states with an existing data infrastructure.

Dr. H. Meltzer: Data collectors must be well trained.

Dr. Diamond: If agencies within a state can agree that a specific set of outcome measures will be used statewide, the data are likely to be useful even if the people filling out the forms lack training and the information is not completely standardized. These data would complement, not replace, controlled trials. Different kinds of information gathered from multiple points of view will augment our knowledge.

Dr. Mahmoud: Perhaps it would be premature to launch immediately into a large prospective randomized trial without seriously considering the possibility of establishing an observational cohort—such as the Framingham study—or even conducting a large trial that would help us establish answerable questions.

Dr. D. Meltzer: Haven't we always been skeptical about the quality of such data?

Dr. Mahmoud: We need to discover how precise the point estimates have to be when the study sample is extremely large.

Dr. D. Meltzer: We have to be as concerned about potential bias as we are about precision.

Dr. Mahmoud: I agree. Furthermore, I would add that sample size and generalizability are problems. So far, no one to my knowledge has been able to show a statistical difference in total costs between 2 schizophrenia drug treatments because of issues relating to sample size and extreme variability in service use. In addition, prospective studies require informed consent. It is well understood that, in schizophrenia, any study that requires informed consent will have a nonrepresentative population because 30% to 40% of patients refuse to consent to a randomized trial. To learn about long-term outcome for a representative sample of patients, we need a study that does not require informed consent.

Dr. D. Meltzer: It is possible to randomize by regions.

Mr. Weisburd: I think it's incumbent upon you who do these studies to recognize that pharmacoeconomic issues are important in a bigger arena, and your randomized studies will be like lead balloons to legislators who fund treatment. The sample population in randomized studies doesn't smell like the schizophrenic population that I know. The way individuals with schizophrenia are treated in this country has ramifications for law enforcement and public safety.

Many public decision makers who appropriate funds for treatment are angry at the ineffectual nature of the mental health systems. I felt that anger when I testified before a local board of supervisors, many of whom were my friends. They see little progress and so requests for more funds fall on deaf ears.

Now that improved pharmaceutical agents are available and we have a clearer grasp of the nature of the illness, researchers need to recognize some of the social forces on schizophrenia when they design studies. They need to be aware of the political arena where the battle for funds is fought. If several politicians had sat through the discussion today, we would have a hard time convincing them to pay for schizophrenia treatment.

Dr. H. Meltzer: I think we all agree that we still lack convincing data. Research on outcomes and cost-effectiveness must also be presented in a form that will make public policy makers and managed care decision makers pay attention because only those who pay for health insurance have the resources to require managed care organizations to provide needed mental health services.