Abstract

Outcomes research on treatments for schizophrenia has identified a number of efficacious interventions. The degree to which such scientific knowledge influences the care delivered in everyday practice depends on a large number of patient, practitioner, service system, and other social factors. The current atmosphere for change in the health care delivery system poses both risks and opportunities to improve care for persons with this disorder. Scientific knowledge about treatment outcomes must inform this rapid evolution of practice, policy, and research to ensure that effective treatments are preserved and available for all who need them and that new treatments continue to be developed, evaluated, and disseminated.


The past 40 years have brought major advances in treatments for persons with schizophrenia. The efficacy of many of these treatment advances has been rigorously assessed through the funding of research. Still, it must be asked, to what degree is the treatment of persons with schizophrenia in the late 20th century based on scientific evidence that the treatments work? It is difficult to know the degree to which scientifically based information on treatment outcomes influences the delivery of services and the formulation of mental health policy. Surely, efforts by society to help persons with schizophrenia must proceed even in a knowledge vacuum, and it is unrealistic, perhaps unwise, to expect that perfect scientific knowledge alone can form the basis for most treatment and policy decisions. Nonetheless, we correctly expect that scientific knowledge on cost-effectiveness will inform and enhance the art of practice and policy development. Furthermore, at this time of high sensitivity to cost-effectiveness, practices lacking demonstrated effectiveness may perish. It is useful then to consider the implications for practice, policy, and future research of our current knowledge base about the outcomes of treatments for schizophrenia.

What Do We Know?

Table 1 summarizes in broad strokes conclusions from the intervention studies reviewed in the articles elsewhere in this issue. Brief inspection of this table reveals that most of what we know relates to the alleviation of symptoms and that available treatments are effective primarily at reducing psychotic symptoms and preventing relapse. Effective treatment for deficit symptoms is not yet demonstrated. Furthermore, we lack a knowledge base about the impacts of most interventions on domains of outcome other than symptoms and rehospitalization. To some extent this is because studies have shown inconsistent or negative results for these other outcomes in controlled studies, but primarily it is because studies have focused on symptomatic outcomes.

By using empirical data from controlled studies, we can speak...
Table 1. Summary of known intervention outcomes

<table>
<thead>
<tr>
<th>Interventions</th>
<th>Psychotic symptoms and thought disorder</th>
<th>Secondary negative symptoms</th>
<th>Primary negative symptoms</th>
<th>Cognitive task performance</th>
<th>Ancillary symptom complexes</th>
<th>Other outcomes</th>
</tr>
</thead>
<tbody>
<tr>
<td>Conventional antipsychotics</td>
<td>+ Remission and relapse</td>
<td>+</td>
<td>?/−</td>
<td>ND</td>
<td>−</td>
<td>ND</td>
</tr>
<tr>
<td>Clozapine</td>
<td>+ Remission and relapse</td>
<td>+</td>
<td>?/−</td>
<td>ND</td>
<td>+ Depression</td>
<td>ND</td>
</tr>
<tr>
<td>Risperidone</td>
<td>+ Remission and relapse</td>
<td>+</td>
<td>ND</td>
<td>ND</td>
<td>+ Hostility</td>
<td>ND</td>
</tr>
<tr>
<td>Adjunctive pharmacologic agents</td>
<td>ND (Antipsychotic augmentation for lithium)</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>+ Depression</td>
<td>ND</td>
</tr>
<tr>
<td>Electroconvulsive therapy</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>+ Depression</td>
<td>ND</td>
</tr>
<tr>
<td>Psychological therapies</td>
<td>−</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>+ Life skills with skills training</td>
</tr>
<tr>
<td>Family interventions</td>
<td>+ Relapse</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
</tr>
<tr>
<td>Vocational rehabilitation</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>+ Work activities</td>
<td>ND</td>
</tr>
<tr>
<td>CM and ACT</td>
<td>+ Relapse (ACT)</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>+ FS for some rehabilitation CM models</td>
<td></td>
</tr>
<tr>
<td></td>
<td>ND (CM)</td>
<td>ND</td>
<td>ND</td>
<td>ND</td>
<td>+ RO for CM and ACT</td>
<td>ND</td>
</tr>
</tbody>
</table>

Note.—ACT = assertive community treatment; CM = case management; FS = functional status; FWB = family well-being; QOL = quality of life; RO = resources and opportunities; SAT = satisfaction with life.

1A "+" indicates that there is substantial evidence from controlled trials that the treatment has a positive impact on the outcome. A "−" indicates that there is substantial evidence from controlled trials that the treatment is not effective for the outcome. A "?/+" indicates some suggestive, but not definitive, evidence of a positive impact, and "?/−" indicates suggestive, but not definitive, evidence of lack of a positive impact. "ND" means "not determined," that is, there is insufficient evidence to make any statement about treatment effects.

confidently about the value for most patients of antipsychotic medications, both the conventional agents (Dixon et al. 1995, this issue) and the newer ones (Buchanan 1995, this issue; Umbrecht and Kane 1995, this issue), and of family interventions (Dixon and Lehman 1995, this issue) for
reducing the risk of relapse. Furthermore, adjunctive pharmacotherapies and electroconvulsive therapy are valuable options for treating patients who do not achieve adequate responses to antipsychotics or who experience persistent ancillary symptoms of depression, anxiety, or aggression (Johns and Thompson 1995, this issue). Assertive community treatment and certain forms of case management offer clear advantages for selected patients at high risk for noncompliance, relapse, or other sources of instability (Scott and Dixon 1995a, this issue).

Beyond this, several commonly used forms of treatment lack an adequate empirical base of demonstrated efficacy. These include various psychological interventions (Scott and Dixon 1995b, this issue) and vocational rehabilitation (Lehman 1995, this issue). Notably, however, there is a growing body of evidence that skills training and at least one form of vocational rehabilitation (i.e., supported employment) enhance functional outcomes. For a few interventions, such as insight-oriented psychotherapy and sheltered employment programs, the consistently negative data raise serious questions about their value.

What does this state of knowledge mean for practice, policy, and research?

Implications for Practice
The obvious implication of these findings is that practitioners must offer treatments with demonstrated efficacy and refrain from providing treatments that have been shown to offer no benefits. Unfortunately, this is not a simple task.

There are compelling reasons why the outcomes data presented should inform but not dictate practice. Most of the research reviewed has been efficacy research, that is, studies of outcomes when treatments are administered under relatively ideal conditions to carefully screened groups of patients. Effectiveness research under usual practice conditions is generally lacking. Therefore, it is difficult to know how treatments with established efficacy may need to be modified to achieve maximum effectiveness in various practice settings for various subgroups of patients. For example, pharmacotherapies that are highly efficacious may need to be modified for patients with complicating medical or substance use disorders. Family interventions may not be helpful to patients who have no contact with their families. The clinician must evaluate a far wider range of issues (psychopathology, personal attributes, psychosocial environment, stress sensitivity, treatment compliance, and pattern of illness over time) than is taken into account in research study designs. Hence, the data should not dictate, but rather guide, individualized care planning.

This caveat aside, there is reason to believe that current practice can be substantially improved in the light of the outcome research available. Practitioners must ask themselves whether they are trained to offer the interventions that have demonstrated efficacy, including the various pharmacotherapies, family interventions, and assertive community treatment, and those for which efficacy data are growing, including skills training and supportive employment.

Many currently practicing physicians received their psychopharmacology training before most of the research reported here. Current practice may not adequately reflect efficacy data on the use of more moderate doses of antipsychotic agents and the addition of adjunctive agents. It was not that long ago that "mega-dose" antipsychotic and "rapid neuroleptization" therapies were viewed as state of the art and that "polypharmacy," the use of adjunctive benzodiazepines and other agents, was viewed as poor practice. It is not known how many physicians still hold these views. Furthermore, the advent of new antipsychotic agents and the promise of several more over the next few years pose a challenge for all prescribing practitioners to ensure that their psychopharmacology skills remain current.

Most practitioners are not trained in certain techniques with documented efficacy: assertive community treatment and the newer family interventions, which emphasize psychoeducation, problem solving, communication skills, and support. Practitioners trained in the older forms of family therapy prominent in the 1960s and 1970s may have little knowledge about the types of family interventions reviewed herein. Furthermore, continued application of dynamic therapy oriented around family psychogenesis is to be rejected as divisive between families and clinicians and as based on therapeutic hypotheses devoid of scientific validation. Assertive community treatment may be even less well understood by practitioners. Recent studies of programs that allege to offer assertive community treatment suggest that much is lost in the implementation of this concept in most locales.
emphasize the necessity of interdisciplinary training. Clinic structures must continue education and in-service practitioners opportunities for communication. Agencies should offer pharmacy formularies need to reflect efficacious interventions, it is incumbent upon serv- tions, technical assistance in identifying barriers to implementation within the agency, and practical solutions to deal with these barriers.

There are many reasons why the care that a person receives may not be consistent with current scientific information. These may include failure to respond to indicated therapies, the need to tailor treatments to individual patient circumstances, lack of insurance coverage to pay for indicated care, lack of available providers with treatment expertise, lack of up-to-date knowledge among providers, failure of patients to return for follow-up care or referrals, lack of coordination among multiple providers caring for the same patients, and others. Some of these sources of variability in treatment are recognized as consistent with best clinical practice, while others represent failures in the health care system and the ability of providers to meet the needs of their patients. Those that represent failures in providing comprehensive, coordinated, and effective care should be targets for improvements.

Implications for Policy

The Nation is in the throes of major health care system change despite the recent failure of the Clinton health security initiative. It is clear that within the next few years, most persons, including those under Federal health programs such as Medicaid and Medicare, will receive their health care through some form of managed care. Given the prime motivation of cost-containment, the most pressing policy questions relate to what services are effective while offering good value for their cost.

The outcome data summarized in this issue convey at least three messages to any health care reform effort. First, there are efficacious treatments for schizophrenia that must be supported (Keith and Matthews 1993; Hall and Mark, in press). These proven benefits are at least as great as mainline medical and surgical therapeutics (Davis and Wang 1994; Keith and Matthews 1993). The existing data base offers a strong argument against those who would diminish the value of interventions for schizophrenia and who advocate for only pharmacological management.

Second, the data argue for the value of specific treatments, including the range of pharmacotherapies, family interventions, assertive community treatment, skills training, and supported employment. Health care policies need to reinforce the value of these interventions to payers and to insist that these efficacious services are covered by insurance plans. One needs only to look at the recent struggles over payment for the new antipsychotic agents to appreciate the basic problems that can and will arise. Many insurance plans do not reimburse for family interventions, and assertive community treatment can be more easily operated under capitation payment, which still does not exist in most locales. It is not clear who
will pay for supported employment or other rehabilitation services.

Third, this array of interventions can best be offered under comprehensive insurance packages, and these need to be promoted by insurance reform, particularly under managed care. An ongoing problem of the fee-for-service system has been the fragmentation of services for persons with schizophrenia and other chronic disorders. Although it carries many perils for persons with long-term disabilities, current health care reform can provide the impetus for comprehensive, "capitated" care plans that offer a full range of services consistent with both effectiveness and efficiency. Carving up this package to accommodate the limitations of individual providers must be avoided; instead, policy decisions should promote comprehensive service packages offered by providers with the capacity to furnish the full range of interventions with proven efficacy.

Capitation creates financial incentives for providers to offer cost-effective services, but it may be difficult to distinguish efficiency from undertreatment. Data on performance and outcomes are needed to guide clinical decision-making under capitated conditions. If providers are not held accountable, capitation may only create an incentive to provide less treatment rather than cost-effective treatment.

Implications for Research

This review carries reasons for both hope and humility. It is encouraging that a sufficient scientific data base exists to assert that certain reforms are needed in current practice and policies. Still, it is striking how much more we need to know. A full agenda for schizophrenia research cannot be laid out here, but fortunately, two major reports by experts brought together under the auspices of the National Institute of Mental Health have described this agenda in detail (Keith and Matthews 1989; National Institute of Mental Health 1991). We will highlight some priorities.

Available treatments focus primarily on the psychotic symptoms of schizophrenia and are reasonably effective at controlling these in the majority of patients. Most patients experience symptom reduction with pharmacotherapy, and relapse rates are reduced by more than half with maintenance treatment. Certain family intervention approaches further reduce relapse rates, and selected psychosocial treatments appear beneficial in occupational and social functioning. Despite effective treatment, most patients remain symptomatic and/or vulnerable to relapse. Persevering impairments are common, and long-term outcome, although heterogeneous, still represents significant morbidity for most patients. Outcomes in other domains, especially the deficit symptoms and impaired functional status, remain quite limited. This points to the need for basic research that provides better understanding of the etiologies and mechanisms of these impairments and the need to develop treatment technologies derived from new knowledge. Major breakthroughs in prevention or treatment depend on advances in basic knowledge. However, even modest advances in therapeutics can produce substantial human and economic benefit, and the recent increase in efforts to design and test new pharmacologic and psychosocial treatments is most promising. Hopefully, improvements in treatments for these areas of impairment will also contribute to improved patient satisfaction with treatments and quality of life.

Clinical research is needed to examine the relative efficacy of available treatments, especially options that represent substantial cost differentials. Many treatment research areas require further data. In light of the cost-cutting focus in health care today, efforts are needed to evaluate some commonly used interventions for which adequate outcome assessments are lacking. These include the various forms of psychological interventions (e.g., supportive psychotherapies) and psychosocial rehabilitation programs. The widespread use of these interventions suggests that they are viewed as valuable and may be critical to adequate community-based care for persons with schizophrenia. Yet the lack of an empirical data base supporting their efficacy may threaten their future. It is imperative that careful clinical research be done to assess their impacts.

Research should continue on psychosocial interventions that show promise when combined with the use of antipsychotic medications. These include family interventions, disease-specific forms of psychological treatments, skills training, and supported employment.

Clinical research should also attend to a fuller range of outcomes (noted in table 1). These outcomes represent the range of priorities of various "stakeholders": researchers, practitioners, patients, families, and payers. A more comprehensive approach to outcomes assessment
will ensure that studies are viewed as relevant and informative to these various stakeholders, who function as effective advocates at various levels. Furthermore, we must ask for whom outcomes are improved. Clinical research on treatment-relevant subgroups will aid in better treatment matching and more judicious allocation of resources. In short, we need to know which treatments enhance which outcomes for which patients.

Health services research has a responsibility on at least two levels. Clinical services research needs to address the translation of efficacious interventions into practice. To what degree are efficacious treatments used in practice? Who receives them and what are the patient and provider determinants of these practice patterns? What is the cost-effectiveness in practice of interventions with known efficacy? What are the barriers to the adoption of efficacious treatments in practice and how can these be overcome? Service systems research must tackle these questions at the system level. What organization and financing strategies promote services that incorporate the most effective treatments?

Conclusions

The outcomes research on the treatment of schizophrenia reviewed in this issue constitutes a substantial basis for action in the arenas of practice, policy, and research. The current changes in our health care systems and efforts to reduce government spending pose major risks and provide opportunities to use innovative approaches for improving the care of persons with this disorder. Although science alone cannot dictate the directions that treatment and services take, our current and ongoing research base must be promoted as a guide to decisions at the clinical, agency, State, and national levels to ensure that persons with schizophrenia have access to the most effective interventions available.

References


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Through a 5-year grant from the National Institute of Mental Health, the American Psychiatric Association (APA) Program for Minority Research Training in Psychiatry (PMRTP) is seeking to increase the number of minority psychiatrists entering the field of psychiatric research.

The program provides medical students and psychiatric residents with funding for stipends, travel expenses, and tuition for an elective or summer experience in a research environment, with special attention paid to the career development in research of trainees. In addition, stipends are available for a limited number of 1- or 2-year postresidency fellowships for minority psychiatrists. Training takes place at research-oriented departments of psychiatry in major U.S. medical schools and other appropriate sites throughout the country. An individual at the site (the research "mentor") is responsible for overseeing the research training experience.

The program, administered by the APA’s Office of Research, includes outreach efforts to identify minority medical students and residents who are potential researchers and to put them in touch with advisors who counsel them about careers in psychiatric research. Additional activities assist fellows and alumni in their research career development.

The director of the PMRTP is Harold Alan Pincus, M.D.; the project manager is Ernesto Guerra. An advisory committee of senior researchers and minority psychiatrists developed guidelines for applicants and criteria for selection. The members of this committee evaluate and select trainees, oversee the research training experiences, and play a role in evaluating the effectiveness of the program.

The deadline is December 1, 1995, for applications for residents seeking a year or more of training as postresidency fellows. For medical students and other residents, applications are due 3 months before training is to begin.

For more information about the PMRTP, please call the toll-free number for the PMRTP, 1-800-852-2390 or 202-682-6225, or write to Dr. Pincus at the American Psychiatric Association, 1400 K Street, NW, Washington, DC 20005.