

Issues in Studying the Effectiveness of Health Services for Children

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Objectives. To discuss issues in studying the effectiveness of health services for children, suggest areas in which more research is needed, and recommend strategies for future research.

Principal Findings. Issues that should be considered include the choice of perspective, which will help determine the interventions studied and the measures of effectiveness and cost-effectiveness chosen. Unique challenges in this area include the fact that serious measurable morbidity is relatively uncommon in children, that causal relationships between services and outcomes may be difficult to establish, and that standard measures of cost-effectiveness may fail to accurately measure important benefits, such as reduced parental anxiety. More research is needed on high-risk and health-promoting behaviors, on critical parent behaviors, on classifying children by vulnerability status, on modes of delivery of preventive care, and on violence prevention.

Recommendations. Group-randomized designs and observational research designs that take advantage of natural variations in practice may be increasingly useful in effectiveness studies. Parent- and patient-reported measures of health status and quality of life should be made briefer and more practical for routine use, and better measures of cost-effectiveness are needed. Future research efforts can best be supported by the concerted efforts of various constituencies, including health plans, providers, patients, researchers, and the government.

Key Words. Effectiveness, health services, research, children

Health policymakers, clinicians, and other advocates need high-quality evidence about effectiveness in order to recommend appropriate services for children. Ongoing changes in healthcare delivery, including the trend toward managed care among children with private insurance and with Medicaid, have increased both the opportunity and the need for research on the effectiveness of children's health services. In this article, we (1) define effectiveness and cost-effectiveness; (2) comment on challenges in studying the effectiveness of health services, particularly for children; (3) describe topics for which

more effectiveness research is needed, and (4) discuss strategies for future research.

THE HEALTHCARE SYSTEM AND CHILDREN'S HEALTH SERVICES

Until the late 1970s, fee-for-service was the dominant mode of healthcare financing in the United States. New health interventions were sometimes adopted based on evidence about their effectiveness, but were sometimes adopted based only on anecdotal experience. Cost-effectiveness was often a secondary consideration, if it was considered at all; there was little incentive to limit resource use.

In recent years, healthcare in the United States has been paid for increasingly under capitation and other managed care financing arrangements, which puts the provider at risk if resources are overspent. Compared with practices under fee-for-service reimbursement, new interventions tend to be subjected to closer scrutiny of their effectiveness and cost-effectiveness before they are adopted. It is reasonable to be concerned about whether current financial arrangements have driven the pendulum too far in the direction of possible underutilization of appropriate care. For example, managed care plans may not always include pediatric subspecialists on their lists of preferred providers. However, the current interest in assessing the effectiveness and cost-effectiveness of health services may provide an opportunity to enhance appropriate services while limiting ineffective ones.

We define health services for children as any organized effort whose primary purpose is to improve the health of children and their families. Health services include traditional medical services such as care delivered in hospitals, outpatient clinics, and community health centers. They also include other types of service such as school-based programs to reduce violent behavior or promote healthy nutritional choices (Grossman et al. 1997; Whitaker et al. 1994).

Health services are only one example of socially supported efforts that affect the health of children. Other such efforts include food and housing

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subsidies, educational programs, social services, and judicial and legislative interventions. Health services may play a smaller role in determining health than other factors such as socioeconomic status (Adler et al. 1993; Bodenheimer and Grumbach 1995). Studies of health services often will need to take other services and other factors into account when trying to determine the specific effects of health services on health outcomes.

DEFINING EFFECTIVENESS AND COST-EFFECTIVENESS

Effectiveness refers to the success of an intervention in actual practice, while efficacy refers to its success under ideal circumstances. For example, interventions with high biological efficacy, such as measles vaccination, may have diminished effectiveness in actual populations if programs to implement them are imperfect (The National Vaccine Advisory Committee 1991). The various possible reasons for gaps between healthcare effectiveness and its efficacy include financial barriers and non-financial barriers to its delivery. (Halfon, Inkelas, and Wood 1995) For example, the measles epidemic of the early 1990s resulted in part from diminished vaccine coverage. A lack of insurance coverage; limited clinic hours; missed opportunities to immunize children at preventive and non-preventive clinic visits; competing family priorities; and parents' concern about the risks of immunizations are among the possible obstacles to vaccine delivery (Cutts, Orenstein, and Bernier 1992; Szilagyi et al. 1993; D. Wood et al. 1995).

Cost-effectiveness refers to the relative balance (which is often formally expressed as a ratio) between the costs and the benefits of a service. It is an increasingly important consideration in health policy decisions. It is important to note that "cost-effective" does not necessarily mean "cost-saving" (Doubilet, Weinstein, and McNeil 1986). Many health interventions, even preventive ones, do not save money (Tengs et al. 1995). Rather, a service should be called cost-effective if its benefits are judged worth the costs.

Recently, a consensus panel supported by the National Institutes of Health published recommendations that define standards for conducting cost-effectiveness analysis (Gold et al. 1996). Cost-effectiveness analysis is only one of several methods that can be used for the economic evaluation of health services (Drummond, Stoddart, and Torrance 1987). Although these methods are useful, an intervention cannot be cost-effective without being effective. In analyses of health services for children, evidence about effectiveness is

sometimes limited (U.S. Congress, Office of Technology Assessment [OTA] 1988).

DEFINING PERSPECTIVES, INTERVENTIONS, AND OUTCOMES

In effectiveness research, it is important to define the perspective from which the question is being asked and the target audience for the answer. Possible individual perspectives include those of patients, parents, clinicians, clinical policymakers, administrators, and purchasers, usually employers. The societal perspective is defined as including the effects on and costs to all of these entities.

The choice of perspective will define both the type of intervention to be studied and the outcome measures to be used. For example, there are various possible interventions that involve spacer devices for delivery of inhaled asthma medications. For patients, parents, and pediatricians, it would be useful to know not only the efficacy of spacer devices for inhaled asthma medications, but also the efficacy of education to help patients use them properly. For a clinical policymaker, it would be useful to know the actual effectiveness of such education when it is being delivered by diverse physicians in busy clinic settings, compared with the effectiveness of alternative or similar educational efforts, such as counseling by pharmacists or group classes led by health educators. For an administrator, it would be helpful to know whether providing insurance coverage for spacer devices or asthma education programs would be cost-effective because those interventions improve health outcomes and/or reduce future asthma-related morbidity. Other perspectives include those of government agencies, professional organizations, and advocacy groups.

The perspective adopted will also determine the types of outcomes that will be used as measures of effectiveness. Types of outcomes include mortality; morbidity; health-related quality of life (which encompasses physical, mental, and social health status); patient/parent satisfaction; utilization; and cost (Spilker 1996). Frequently, research studies address one type of outcome, most typically morbidity, but do not include other important types. This may be appropriate in early trials of the efficacy of an intervention, for instance, varicella vaccination. However, in today's healthcare environment, decisions are driven not only by clinical effectiveness but also by patient satisfaction and cost. Research studies that address all types of outcomes will be increasingly needed to guide policy decisions.

Effectiveness may also be evaluated using intermediate outcomes, also termed process measures. For example, an immunization program may be judged either by the vaccination coverage rate (the process measure) or by the reduction in actual disease (the “hard” outcome). The process measure is appropriate when the outcome measure is either insensitive or difficult to obtain and has already been strongly linked with the process measure. The choice among process measures and other outcome measures will depend on the target audience and on the decision that the effectiveness information is supposed to help inform. For example, in comparing mailed versus telephone reminders for childhood immunizations, the incremental cost per additional child appropriately immunized, compared with the next most effective strategy or with no intervention, is an appropriate outcome measure (Lieu et al. In press).

SPECIAL CHALLENGES IN STUDYING THE EFFECTIVENESS OF CHILDREN’S HEALTH SERVICES

There is an acknowledged paucity of solid information about the effectiveness of even traditional medical services for children (OTA 1988). Data on the effectiveness of services provided outside of medical settings, and data on cost-effectiveness, are even more scant.

The challenges of doing outcomes research in children are deep-seated. Among the most important are these: (1) many interventions aim for improved health versus reduced morbidity, yet measures of parent- and patient-reported health status are not routinely collected; (2) interventions may have effects in the distant future or in sectors other than healthcare; and (3) cost-effectiveness measures are difficult to translate into measures comparable to those used for adult interventions. We now describe these and other challenges in detail together with their possible solutions.

Serious measurable morbidity is relatively uncommon. Hospitalization rates have been used as a convenient measure of outcomes for children’s health services (The National Committee for Quality Assurance 1993). However, only 2–10 percent of children with asthma, the most common cause of hospitalization among U.S. children, will be hospitalized in a given year (Lieu et al. 1997; Weitzman et al. 1992). Likewise, among newborns who experience short hospital stays (48 hours or less), only 1–6 percent will be rehospitalized during the next two weeks, and serious long-term sequelae from infection or

hyperbilirubinemia are even more uncommon (Catz et al. 1995; Newman and Maisels 1992). If controlled trials attempted to assess the effectiveness of alternative interventions to reduce these outcomes, huge sample sizes (e.g., 25,000 or more in the case of newborn rehospitalization) would be needed.

Measuring hospitalization rates will not accurately reflect the effectiveness of most children's health services, which aim to promote healthy physical and psychological development as well as to prevent morbidity. Thus, outcomes for effectiveness research ideally should include non-hospital morbidity, such as acute outpatient visits, days of activity limitation, school-loss, parent work-loss, and disease-related decreased quality of life.

Patient- and parent-reported measures of health status are not brief enough to be routinely collected. Several carefully developed child health status measures, both general and disease-specific, already exist, and more are under development (Juniper et al. 1996a,b; Landgraf, Abetz, and Ware 1996; Starfield et al. 1995; Stein 1990). At least one instrument is available in a computer-based, interactive form. Unfortunately, existing child health status measures are not necessarily practical for routine use in monitoring outcomes. None are as brief as the SF-12, a 12-item instrument for adults; the Child Health Questionnaire contains 28 items (Landgraf, Abetz, and Ware 1996). Although some of these instruments are self-administered and require only 15 minutes of respondent time, it is not realistic to expect staff in busy clinical settings to routinely collect them from parents. Thus, health status measurement currently requires dedicated research staff and is limited to prospectively designed studies.

The appropriate choice of outcome may differ depending on which perspective is chosen. Perceptions of health status may differ when measured from the child's versus the parent's perspective (Guyatt et al. 1997; P. R. Wood et al. 1994). Little evidence is available about whether a study of the effectiveness of a given health service has different results depending on whether the child or the parent served as the respondent in health status measures.

Beyond this, clinicians tend to view biological and psychological morbidity as important outcomes, while parents tend to identify other outcomes as also important. These include communication, coordination of care, ease of access to care, respect, self-efficacy, and shared decision-making. In addition, parents from varied cultural backgrounds may have varied perspectives about which health outcomes are most important. More studies of the effects of healthcare system changes on these important patient-centered outcomes are warranted.

Causal relationships between services and outcomes may be difficult to establish for several reasons. For example, recommended anticipatory guidance at visits for one-year-olds includes a discussion of nutrition and strategies to promote social competence and constructive family relationships (Green 1994). However, anticipatory guidance in the medical setting is conducted intermittently and very briefly. Thus, its effects are likely to be less, and more difficult to study, than those of more ambitious interventions, such as focused educational programs for violence prevention or smoking cessation. As health outcomes, good nutritional and social behaviors are challenging to study, partly because they may develop over several years. In addition, these behaviors are affected by many other family, social, and environmental influences aside from the healthcare system.

Studies of vulnerable populations are logistically difficult to undertake. Children at high risk for medical and social problems are a logical focus for effectiveness research since the potential gain from effective services is high; however, there are many logistical challenges to conducting research in such populations (Epstein 1997). These include language barriers, limited literacy, limited telephone accessibility of the respondents, lack of computerized outcome data in many settings, and higher rates of nonresponse and loss to follow-up (Epstein 1997; Sisk et al. 1996). The increase in the numbers of children in managed Medicaid arrangements and the ability of managed care organizations to collect data on these patients may provide new opportunities for studies of vulnerable populations.

Children's interventions may lead to cost savings in non-medical settings. Many cost-effectiveness analyses limit their perspective to financial effects from the perspective of a health plan or a healthcare payer. In contrast, interventions such as home visiting for high-risk infants or varicella vaccination may lead to significant cost savings outside of the medical system, such as reduced social service costs or reduced parent work-loss costs (Lieu et al. 1994; Olds et al. 1997). The societal perspective includes work-loss costs and other non-medical costs, as well as direct medical costs. An analysis conducted from the societal perspective may lead to results different from the results of one conducted from the health plan perspective, which typically includes only direct medical costs (Lieu et al. 1994; Taplin, Thompson, and Conrad 1988). The societal perspective should be used in the base case (primary) analysis whenever possible because it is the most inclusive approach (Gold et al. 1996).

Standard measures of cost-effectiveness may fail to capture important benefits of health services for children. Many adult health services, including mammograms, Pap smears, and colorectal cancer screening, have been

assessed using a common outcome measure: dollars per life-year saved. Many health services for children do not prevent mortality, so it is often difficult to compare their cost-effectiveness to that of adult health services. One possible way of addressing this problem would be to use another standard measure of benefit: quality-adjusted life-years (LaPuma and Lawlor 1990). This measure gives credit for morbidity prevented; for example, to prevent a year of severe disability might be worth some portion of a quality-adjusted life year (Drummond, Stoddart, and Torrance 1987).

Unfortunately, the methods established to derive quality-adjusted life-years are best suited to evaluating chronic disease states. In contrast, many health services for children reduce morbidity in acute conditions (e.g., treatment of otitis media); reduce pain (e.g., combination vaccines for infants); enhance desirable behaviors (e.g., healthy nutritional choices); or provide psychological benefits such as reassurance for parents. A decision analysis that addressed the testing and treatment of fever in young children was one of the few pediatric studies that used quality-adjusted life-years (Downs, McNutt, and Margolis 1991). Other methods, such as cost-benefit analysis and valuation of benefits using willingness-to-pay questions, might be better for evaluating selected health services for children.

Limited research has been conducted outside of university-based populations. Although this issue is not unique to children, existing knowledge about effectiveness is sometimes limited by the fact that studies tend to be conducted in university-based populations, which may differ from populations in other practice settings. It is important that effectiveness research also be conducted in office-based populations and in managed care systems. The Pediatric Research in Office Settings network and the HMO Research Group are consortia that support research in these settings (Wasserman 1997).

Financial constraints limit support for research on children's health services. On average, children consume less healthcare than adults. There has not been as broad a constituency, nor as much funding, to support effectiveness research. Many adult interventions center on medications, tests, or technology, for which pharmaceutical or laboratory companies need to provide clinical effectiveness data and are willing to fund controlled trials. There is much less such activity in pediatrics. Other potential funders include managed care organizations that may need research support for disease management programs. However, because the health problems of adults account for more total costs than those of children, managed care plans might give priority to studies of adult health services. There has also been

less support for children's health research than for adult research from federal agencies, but this may be due in part to the limited number of researchers in children's health services (Forrest, Simpson, and Clancy 1997).

TOPICS ON WHICH MORE EFFECTIVENESS RESEARCH IS NEEDED

Rationale for Choosing Topics

Given the very broad range of health services for children, how should we choose which areas deserve more effectiveness research in the next five to ten years? Those services that have the greatest potential to improve health or reduce morbidity among the entire population of U.S. children seem like logical priorities for effectiveness research. One such type of service would be that which affects the health of very broad populations, even if the effect of the service is relatively small. For example, outpatient follow-up services after newborn hospital discharge might have a small effect on any single patient, but a large effect on population health, because the service would affect the majority of all newborns from uncomplicated deliveries. A second type of service for which effectiveness data would be important would be a service that has a potential large effect on a narrower population. For example, services to prevent teen pregnancy may possibly have large effects even though they would be aimed at a subset of adolescents.

In addition, we believe that setting priorities among topics for research should use more than a purely utilitarian calculus, that is, "the greatest good for the population equals the number of patients affected times the effect of the intervention." Some research priority should be reserved for groups of children with increased vulnerability to adverse outcomes, for example, those with special health needs, in foster care, or without homes or medical insurance. More research is also needed on the effectiveness of mental health services for children.

Priorities for effectiveness research will also be driven, at least in part, by the financial and logistical costs of various services. New, high-cost, or high-risk interventions, such as extracorporeal membrane oxygenation or intravenous respiratory syncytial virus immunoglobulin for premature infants, will appropriately be subjected to more rigorous effectiveness and cost-effectiveness research than existing or low-cost interventions. The effects of reductions in existing services, particularly when they may affect vulnerable populations, need to be carefully studied.

PRIORITY RESEARCH QUESTIONS

One of the work groups convened during the meeting, "Improving Quality of Health Care for Children," was asked to reach consensus on six questions for which more effectiveness research was needed. After a modified Delphi process, in which each panel member suggested questions and commented on other questions that had been suggested, a vote was taken. The priority questions chosen are listed in Table 1.

In addition to those questions chosen by the work group, we suggest the following selected priority topics for health services research that looks at the effects of various organization and delivery methods on health outcomes:

Health Insurance Expansions. Currently, the federal and state governments are supporting expanded health insurance coverage for children, either by raising the income limits for Medicaid or by starting new programs to subsidize care for the near-poor. As more families are offered coverage under these programs, several issues that need to be addressed: What, for instance, are the most effective mechanisms of outreach to get the target populations enrolled? Will offering government-subsidized insurance have a "crowding-out" effect in which employers stop offering health insurance for employees' children or employed parents decline employment-based insurance for children? Will formerly uninsured children cost more than insured children, or will they have special problems gaining access to care in the private sector?

Managed Care Medicaid. The numbers of children insured by Medicaid who are served in managed care systems have increased over the past decade (Felt-Lisk and Yang 1997; Health Care Financing Administration 1997). The diversity of these systems provides a natural experiment in which to study the associations between various features of managed care organizations (e.g., payment mechanisms, provider feedback, disease management programs) and health outcomes (Epstein 1997). Little is known about the effectiveness of expanded "customer services" to help low-income populations access and use care appropriately in managed care systems. More information is needed on defining the strategies that could help ease these families' transitions into managed care settings and optimize the appropriateness of their preventive as well as acute care utilization.

Specialty Care. The health effects of specific practices in managed care, such as gatekeeper approaches that may limit access to specialty care (Cartland and Yudkowsky 1992), should be scrutinized. For any given condition, it may be less productive to ask whether specialists or generalists deliver better care than to ask whether it is more effective or cost-effective to increase

Table 1: Priority Questions for Research on the Effectiveness of Health Services for Children

Questions and Subquestions

1. How do we effectively change high-risk behaviors and promote healthy behaviors in children and adolescents, e.g., smoking, alcohol, drugs, exercise, nutrition, sexual behavior?
 - What is the role of acute healthcare in supporting these interventions?
 - What is the “natural history” of the behaviors requiring change?
 - Which persist; which are extinguished over time?
 - If we change high-risk behaviors, do these changes persist?
2. How can we identify and study the influence of critical parent behaviors which affect children’s health?
 - How do we categorize and identify critical behaviors?
 - How do we effectively intervene to change these behaviors?
 - What is the relative importance of different parental behaviors in determining health outcomes?
3. What is the impact on future functioning of early identification and effective treatment of mental health problems in children and adolescents?
 - What are key mental health problems for identification and intervention?
 - How do we increase identification of these problems in different settings?
 - How do alternative forms of financing affect outcomes of mental health disorders?
 - What are the efficacious treatments for children and adolescents?
 - How do we identify and account for co-morbidities?
 - What is the natural history of mental health problems in children and adolescents?
4. Does classification of children by vulnerability status and intensive intervention on identified high-risk groups affect health outcomes?
 - How do we identify and measure the features of vulnerability?
 - Vulnerability for what? e.g., poor health outcomes; failure to complete school, maintain employment, form intimate relationships, etc.
 - What are effective interventions?
 - What is an “intensive” intervention?
5. What is the effect of different periodicity schedules, alternative modes of delivery, different sites of delivery, and different practitioner types on health maintenance outcomes?
 - What is the effect of alternative modes of delivery? e.g., group health maintenance visits?
 - What is the effect of different provider types, such as PNPs, FPs, or pediatricians?
 - What aspects of health maintenance are most effective?
 - What is the effect of panel size and productivity demands on the delivery of health maintenance care?
 - Is it more cost-effective to adapt health maintenance care to the needs of different children, e.g., minimal care for healthy children from stable families and more intensive interventions for vulnerable children?
 - What standards of evidence are necessary in order to make health maintenance recommendations?
6. What are the most effective means of preventing violence and violent injury in children?
 - How do we intervene at different levels (e.g., child, family, community)?
 - What is the effect of violent behavior on healthcare outcomes for children and adolescents?
 - Can violence and violent injury be reduced through educational interventions with parents or schools?

continued

Table 1: (Continued)

Questions and Subquestions

- Do post-violent event counseling and intervention change the risk of subsequent violent behavior?
 - What is the role of the healthcare system in changing violent behavior?
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Note: Work group participants were David Bergman (chair), Robert Brook, Katherine Kauffer Christoffel, Sarah Horwitz, George Isham, Mary Kennedy, Tracy Lieu, Harold Perl, James Perrin, John Santelli, Mark Shuster, and David Stevens.

specialty care versus providing advanced training to generalists for chronic illnesses such as asthma or diabetes. A second important question is this: For what conditions do pediatric subspecialists achieve outcomes superior to those of adult subspecialists, and at what cost?

Center-Based Care and Allied Health Services. For certain diseases, such as cystic fibrosis and some types of childhood cancers, center-based care (in which all patients in a given region are served by a few centers devoted to their condition) is believed to be associated with better outcomes. Less consensus exists about the effectiveness of center-based care for other conditions. In addition, health policymakers need more information about the effectiveness of speech, occupational, and physical therapy for children.

GENERAL RECOMMENDATIONS

Research Designs. For new interventions, such as the case management of pediatric asthma or the introduction of new childhood vaccines, randomized trials of efficacy will often be a key step preceding effectiveness studies. One common problem in office or managed care settings is that a planned intervention, such as physician education to reduce unnecessary antibiotic utilization, often cannot be applied randomly to individual patients. Randomization by larger units, such as clinics, hospitals, or counties, has been used in these situations; appropriate statistical methods, such as generalized estimating equations or mixed-effects linear models, should be selected (Donner, Birkett, and Buck 1981; Liang and Zeger 1986; McCullagh and Nelder 1983).

Only a small fraction of children's health services can be studied by randomized trials, which are costly, time-consuming, and sometimes not feasible in real-life settings. Randomized trials usually require long time-lines for organization and recruitment; results from a trial initiated today may not be generalizable in the future if the healthcare system changes dramatically.

In this rapidly changing healthcare environment, patient populations in either randomized or non-randomized studies may experience changes in care which may confound the evaluation of selected interventions.

In some situations, such as chronic conditions like asthma (which have natural regression to the mean after exacerbations), pre- and post-intervention designs do not provide useful information, and external controls should be used if randomization is not possible. Carefully designed observational designs, such as cohort, case-control, and cross-sectional studies, may still provide acceptable evidence of causation, but they need to be interpreted thoughtfully (LeBaron et al. 1997; Newman, Browner, and Hulley 1988; Thompson 1997).

Further Development of Parent- and Patient-Reported Outcome Measures. The paucity of brief, validated survey instruments that measure health outcomes from the perspectives of parents and patients is a key barrier to effectiveness research. Much better research would be possible if health outcome surveys were brief enough to be integrated into the quality monitoring efforts of providers of care to large populations, including Medicaid and managed care plans. However, the collection of survey data is costly and is likely to expand only if the marketplace demands it.

Monitoring of outcomes from the parents' and patients' perspectives seems a likely future trend due to the increasing sophistication of healthcare purchasers and consumers. For example, the Foundation for Accountability (FACCT) recommends measures for asthma and other diseases that take patient-centered outcomes (functional status, work-loss, and satisfaction, for instance) into account. The FACCT measures may be adopted for monitoring by more health plans in the near future. If such outcome measures were routinely available in computerized data, longitudinal studies would be feasible, and studies of the effects of practice variations on outcomes would be much less costly.

It is important to remember that much routine quality monitoring will continue to rely on practical process measures, such as immunization coverage rates, that careful research has related to appropriate outcomes. However, health services researchers should seek to increase the use of parent- and patient-reported outcome measures.

Costs and Cost-Effectiveness. It is necessary to address the gaps in information on the cost-effectiveness of most children's health services. Studies of the effectiveness of services should routinely collect data on the costs of services and outcomes, because this information is useful to policymakers in their decisions about adopting a service and putting it in place. Collecting

cost and cost-effectiveness data should become increasingly feasible given the integration of cost data into the monitoring systems of many health plans. Efforts are needed to develop measures of cost-effectiveness for children's interventions that both capture important benefits and show comparisons with cost-effectiveness measures for adult interventions. Comparability is important because when resources are limited, services for children may need to compete for priority with services for adults.

Decision Analysis and Meta Analysis. Resources to support longitudinal, empirical trials are limited; yet projections of longitudinal outcomes are often important in the evaluation of possible policy options for children. For this reason, more research training should be made available in quantitative modeling methods such as decision analysis. These modeling methods can be used to integrate existing data and expert opinion to project future health outcomes and costs of alternative policies.

An explicit assessment of the strength of evidence is important for developing guidelines and deciding on coverage. Review articles should begin to grade the strength of evidence on effectiveness, adopting systems for grading like that of the U.S. Preventive Services Task Force (U.S. Preventive Services Task Force 1996). Meta analysis is a formal quantitative approach to synthesizing evidence from multiple trials, and it should be used when enough similar trials are available (Pettiti 1994).

The Need to Organize Constituencies. In children's health services, no one constituency by itself has the resources to promote better effectiveness research. Health plans and payers have access to populations of children and determine the data that are routinely collected on those populations, while researchers with the methodological skills reside mostly at universities. Government agencies play a role both as providers and as monitors of care. Increased collaboration among these entities, and improved coordination of research funding across government, foundation, and private-sector agencies, will be needed (Forrest, Simpson, and Clancy 1997).

CONCLUSIONS AND RECOMMENDATIONS

National trends in healthcare systems, including the increasing managed care enrollment and the regionalization of specialty services, are changing the organization and nature of many health services for children. Special issues in studying these services include the difficulty of establishing causal relationships between certain services and their outcomes; the need for briefer survey

instruments to measure parent- and patient-centered outcomes; and the fact that standard measures of cost-effectiveness may fail to measure accurately the important benefits of health services for children. More research is needed on high-risk and health-promoting behaviors, on critical parent behaviors, on classifying children by vulnerability status, on modes of delivery of preventive care, and on violence prevention. Future research can be supported most effectively by the concerted efforts of various constituencies, including health plans, providers, patients, researchers, and the government.

REFERENCES

- Adler, N. E., W. T. Boyce, M. A. Chesney, S. Folkman, and S. L. Syme. 1993. "Socioeconomic Inequalities in Health." *Journal of the American Medical Association* 269 (24): 3140-45.
- Bodenheimer, T. S., and K. Grumbach. 1995. *Understanding Health Policy*, 1st ed. Stamford, CT: Appleton & Lange.
- Cartland, J. D., and B. K. Yudkowsky. 1992. "Barriers to Pediatric Referral in Managed Care Systems." *Pediatrics* 89 (2): 183-92.
- Catz, C., J. W. Hanson, L. Simpson, and S. J. Yaffe. 1995. "Summary of Workshop: Early Discharge and Neonatal Hyperbilirubinemia." *Pediatrics* 96 (4, Part 1): 743-45.
- Cutts, F. T., W. A. Orenstein, and R. H. Bernier. 1992. "Causes of Low Preschool Immunization Coverage in the United States." *Annual Review of Public Health* 13: 385-98.
- Donner, A., N. Birkett, and C. Buck. 1981. "Randomization by Cluster: Sample Size Requirements and Analysis." *American Journal of Epidemiology* 114 (6): 906-14.
- Doubilet, P., M. C. Weinstein, and B. J. McNeil. 1986. "Use and Misuse of the Term "Cost Effective" in Medicine." *The New England Journal of Medicine* 314 (4): 253-55.
- Downs, S. M., R. A. McNutt, and P. A. Margolis. 1991. "Management of Infants at Risk for Occult Bacteremia: A Decision Analysis." *Journal of Pediatrics* 118 (1): 11-20.
- Drummond, M. F., G. L. Stoddart, and G. W. Torrance. 1987. *Methods for the Economic Evaluation of Health Care Programmes*. New York: Oxford University Press.
- Epstein, A. M. 1997. "Medicaid Managed Care and High Quality: Can We Have Both?" *Journal of the American Medical Association* 278 (19): 1617-21.
- Felt-Lisk, S., and S. Yang. 1997. "Changes in Health Plans Serving Medicaid, 1993-1996." *Health Affairs* 16 (5): 125-33.
- Forrest, C. B., L. Simpson, and C. Clancy. 1997. "Child Health Services Research: Challenges and Opportunities." *Journal of the American Medical Association* 277 (22): 1787-94.
- Gold, M. R., J. E. Siegel, L. B. Russell, and M. C. Weinstein, ed. 1996. *Cost-Effectiveness in Health and Medicine*. New York: Oxford University Press.

- Green, M., ed. 1994. *Bright Futures: Guidelines for Health Supervision of Infants, Children, and Adolescents*. Arlington, VA: National Center for Education in Maternal and Child Health.
- Grossman, D. C., H. J. Neckerman, T. D. Koepsell, P. Y. Liu, K. N. Asher, K. Beland, et al. 1997. "Effectiveness of a Violence Prevention Curriculum Among Children in Elementary School: A Randomized Controlled Trial." *Journal of the American Medical Association* 277 (20): 1605-11.
- Guyatt, G. H., E. F. Juniper, D. H. Feeny, and L. E. Griffith. 1997. "Children and Adult Perceptions of Childhood Asthma." *Pediatrics* 99 (2): 165-68.
- Halfon, N., M. Inkelas, and D. Wood. 1995. "Nonfinancial Barriers to Care for Children and Youth." *Annual Review of Public Health* 16: 447-72.
- Health Care Financing Administration. 1997. Medicaid Managed Care State Enrollment. URL: <http://www.hcfa.gov/medicaid/pntrtn3.htm>.
- Juniper E. F., G. H. Guyatt, D. H. Feeny, P. J. Ferrie, L. E. Griffith, and M. Townsend. 1996a. "Measuring Quality of Life in Children with Asthma." *Quality of Life Research* 5 (1): 35-46.
- Juniper, E. F., G. H. Guyatt, D. H. Feeny, P. J. Ferrie, L. E. Griffith, and M. Townsend. 1996b. "Measuring Quality of Life in Parents of Children with Asthma." *Quality of Life Research* 5 (1): 27-34.
- Landgraf, J. M., L. Abetz, and J. E. Ware. 1996. *The Child Health Questionnaire: A User's Manual*. Boston, MA: The Health Institute, New England Medical Center.
- LaPuma, J., and E. F. Lawlor. 1990. "Quality-Adjusted Life-Years." *Journal of the American Medical Association* 263 (21): 2917-21.
- LeBaron, C. W., M. Chaney, A. L. Baughman, E. F. Dini, E. Maes, V. Dietz, and R. Bernier. 1997. "Impact of Measurement and Feedback on Vaccination Coverage in Public Clinics, 1988-1994." *Journal of the American Medical Association* 277 (8): 631-35.
- Liang, K-Y., and S. Zeger. 1986. "Longitudinal Data Analysis Using Generalized Linear Models." *Biometrika* 73 (1): 13-22.
- Lieu, T. A., S. L. Cochi, S. B. Black, M. E. Halloran, H. R. Shinefield, S. J. Holmes, et al. 1994. "Cost Effectiveness of a Routine Varicella Vaccination Program for U.S. Children." *Journal of the American Medical Association* 271 (5): 375-81.
- Lieu, T. A., C. P. Quesenberry, A. M. Capra, M. E. Sorel, K. E. Martin, and G. R. Mendoza. 1997. "Outpatient Management Practices Associated with Reduced Risk of Pediatric Asthma Hospitalization and Emergency Department Visits." *Pediatrics* 100 (3, Part I): 334-41.
- Lieu, T. A., A. M. Capra, J. Makol, S. B. Black, and H. R. Shinefield, for the Immunization Message Study Group. 1998. "Effectiveness and Cost-Effectiveness of Letters, Automated Telephone Messages, or Both for Underimmunized Children in a Health Maintenance Organization." *Pediatrics* 101 (4)/URL:<http://www.pediatrics.org/cgi/content/full/101/4/e3>.
- McCullagh, P., and J. A. Nelder. 1983. *Generalized Linear Models*. New York: Chapman and Hall.
- The National Committee for Quality Assurance. 1993. *Health Plan Employer Data*

- and Information Set and Users' Manual, Version 2.0. Washington, DC: National Committee for Quality Assurance.
- The National Vaccine Advisory Committee. 1991. "The Measles Epidemic: The Problems, Barriers, and Recommendations." *Journal of the American Medical Association* 266 (11): 1547-52.
- Newman, T. B., W. S. Browner, and S. B. Hulley. 1988. "Enhancing Causal Inference in Observational Studies." In *Designing Clinical Research*, edited by S. B. Hulley and S. R. Cummings, p. 98. Baltimore, MD: Williams & Wilkins.
- Newman, T. B., and M. J. Maisels. 1992. "Evaluation and Treatment of Jaundice in the Term Newborn: A Kinder, Gentler Approach." *Pediatrics* 89 (5, Part 1): 809-18.
- Olds, D. L., J. Eckenrode, C. R. Henderson, H. Kitzman, J. Powers, R. Cole, et al. 1997. "Long-Term Effects of Home Visitation on Maternal Life Course and Child Abuse and Neglect." *Journal of the American Medical Association* 278 (8): 637-43.
- Pettiti, D. 1994. *Meta-Analysis, Decision Analysis, and Cost-Effectiveness Analysis*. New York: Oxford University Press.
- Sisk, J. E., S. A. Gorman, A. L. Reisinger, S. A. Glied, W. H. DuMouchel, and M. M. Hynes. 1996. "Evaluation of Medicaid Managed Care." *Journal of the American Medical Association* 276 (1): 50-55.
- Spilker, B., ed. 1996. *Quality of Life and Pharmacoeconomics in Clinical Trials*. Philadelphia: Lippincott-Raven.
- Starfield, B., A. W. Riley, B. F. Green, M. E. Ensminger, S. A. Ryan, K. Kelleher, et al. 1995. "The Adolescent Child Health and Illness Profile: A Population-Based Measure of Health." *Medical Care* 33 (5): 553-66.
- Stein, R. E. 1990. "Functional Status II(R): A Measure of Child Health Status." *Medical Care* 28 (11): 1041-55.
- Szilagyi, P. G., L. E. Rodewald, S. G. Humiston, R. F. Raubertas, L. A. Cove, C. B. Doane, et al. 1993. "Missed Opportunities for Childhood Vaccinations in Office Practices and the Effect on Vaccination Status." *Pediatrics* 91 (1): 1-7.
- Taplin, S. H., R. S. Thompson, and D. A. Conrad. 1988. "Cost-Justification Analysis of Prenatal Maternal Serum *alpha-feto* Protein Screening." *Medical Care* 26 (12): 1185-202.
- Tengs, T. O., M. E. Adams, J. S. Pliskin, D. G. Safran, J. E. Siegel, M. C. Weinstein, and J. D. Graham. 1995. "Five Hundred Life-Saving Interventions and Their Cost-Effectiveness." *Risk Analysis* 15 (3): 369-90.
- Thompson, R. S. 1997. "Systems Approaches and the Delivery of Health Services." *Journal of the American Medical Association* 277 (8): 670-71.
- U.S. Congress, Office of Technology Assessment. 1988. *Healthy Children: Investing in the Future*. Report OTA-H-345. Washington, DC: Government Printing Office.
- U.S. Preventive Services Task Force. 1996. *Guide to Clinical Preventive Services*, 2nd ed. Baltimore, MD: Williams & Wilkins.
- Wasserman, R. C. 1997. "Research in Private Pediatric Practice and the Challenge of Network Research." *Current Opinion in Pediatrics* 9 (5): 483-86.
- Weitzman, M., S. L. Gortmaker, A. M. Sobol, and J. M. Perrin. 1992. "Recent Trends

- in the Prevalence and Severity of Childhood Asthma." *Journal of the American Medical Association* 268 (19): 2673-77.
- Whitaker, R. C., J. A. Wright, T. D. Koepsell, A. J. Finch, and B. M. Psaty. 1994. "Randomized Intervention to Increase Children's Selection of Low-Fat Foods in School Lunches." *Journal of Pediatrics* 125 (4): 535-40.
- Wood D., C. Donald-Sherbourne, N. Halfon, M. B. Tucker, V. Ortiz, J. S. Hamlin, et al. 1995. "Factors Related to Immunization Status Among Inner-City Latino and African-American Preschoolers." *Pediatrics* 96 (2): 295-301.
- Wood, P. R., H. A. Hidalgo, T. J. Prihoda, and M. E. Kromer. 1994. "Comparison of Hispanic Children's and Parents' Responses to Questions About the Child's Asthma." *Archives of Pediatric and Adolescent Medicine* 148: P43.