Insurance Coverage, Physician Recommendations, and Access to Emerging Treatments

Growth Hormone Therapy for Childhood Short Stature

Beth S. Finkelstein, PhD; J. B. Silvers, PhD; Ursula Marrero, MSSA; Duncan Neuhauser, PhD; Leona Cuttler, MD

Context.—There is concern in both the medical community and the general public about mechanisms of medical decision making and the interplay of physician and insurer decisions in determining access to care.

Objective.—To examine the medical process influencing access to growth hormone (GH) therapy for childhood short stature by comparing coverage policies of US insurers with the treatment recommendations of US physicians.

Design and Participants.—Independent national representative surveys were mailed to insurers (private, Blue Cross/Blue Shield, health maintenance organizations, programs for Children with Special Health Care Needs, and Medicaid programs, n=113), primary care physicians (n=1504), and pediatric endocrinologists (n=534) with response rates of 75%, 60%, and 81%, respectively. Each survey included identical case scenarios. Primary care physicians were asked decisions about referrals to pediatric endocrinologists. Endocrinologists were asked GH treatment recommendations. Insurers were asked coverage decisions for GH therapy.

Main Outcome Measures.—Insurer coverage decisions for GH in specific case scenarios were compared with the recommendations of primary care physicians and pediatric endocrinologists.

Results.—Physician recommendations and insurance coverage decisions differed strikingly. For example, while 96% of pediatric endocrinologists recommended GH therapy for children with Turner syndrome, insurer policies covered GH therapy for only 52% of these children. Overall, referral and treatment decisions by physicians resulted in recommendations for GH therapy in 78% of children with GH deficiency, Turner syndrome, or renal failure; of those recommended for treatment, 28% were denied coverage by insurers. Similarly, GH therapy would be recommended by physicians for only 9% of children with idiopathic short stature, but insurers would not cover GH for the vast majority of these children. Furthermore, the data indicated considerable variation among insurers regarding coverage policies for GH (P<.01).

Conclusions.—Access to GH therapy differs depending on the type of insurance coverage. The deep discord between physician recommendations and insurance coverage decisions, exemplified by these findings, represents a major challenge to mechanisms of health care decision making, access, and costs.

THE INFLUENCE of insurance on access to health care, especially access to expensive specialist care and treatment, is a major concern.1,4 However, little is known about the spectrum of coverage across insurers or about the agreement between physician recommendations and insurer policies for specific therapies. These concerns are particularly important for emerging and semielective treatments related to quality of life (such as treatments for short stature, infertility, obesity, and aging5), in which consensus about optimal utilization may be lacking.

For editorial comment see p 703.

In this article, we focus on insurer and physician decisions regarding growth hormone (GH) therapy for childhood short stature for several reasons. First, GH therapy is representative of many treatments whose use depends on a process involving primary care physicians, specialists, and insurers. Pediatric endocrinologists are the specialist group almost exclusively responsible for prescribing GHs for short children and are considered experts in the area.5 However, the overall impact of their decisions on GH use is influenced by referrals from primary care physicians and coverage decisions of insurers. Second, GH therapy is at times semielective, is rarely needed for life-threatening situations, and is very costly (approximately $14 000 per year for a child weighing 20 kg).8 Finally, optimal GH use has been the subject of debate.5,10 Traditionally, GH therapy has been used for children who have classical GH deficiency (GHD)11,12 due to a lack of natural GHs. However, the medical literature and available guidelines suggest...
diverse criteria for defining GHD and identifying appropriate candidates for treatment.7,23,24 Physicians may and do prescribe GHs for conditions other than GHD.5,12,14-16 The literature indicates that GHs may benefit children with short stature due to conditions such as Turner syndrome and chronic renal insufficiency (CRI). Moreover, GH use has been suggested for certain short children who do not have a defined medical disorder (ie, familial, constitutional, or idiopathic short stature).5,11,12,16 These children constitute the largest number of candidates for GH therapy and represent the first major threshold in nontraditional GH use that may be followed by other applications, including GHs and derivatives for aging, the acquired immunodeficiency syndrome, and obesity.4

In this study, we asked 3 major questions: (1) What are insurer policies for the coverage of GH therapy in the treatment of childhood short stature? (2) Are insurer policies comparable to the recommendations of expert physicians? (3) How do insurer policies for GH therapy interact with physician decisions in influencing GH utilization and costs?

METHODS

Insurer Survey and Sample

A written survey instrument was developed by a team of experts in endocrinology, survey methodology, and health care financing. The preliminary survey was pretested with administrative and medical personnel employed by insurers. The final questionnaire used an experimental design based on survey methodology in which each insurer was asked to report the following: (1) general policies for GH therapy; (2) whether their organization would cover all or part of the costs of GH therapy for childhood short stature due to specific medical conditions such as GHD, CRI, or Turner syndrome. These conditions were selected to include disorders for which GH therapy was approved (GHD, CRI) or not approved (Turner syndrome) by the FDA. The survey was mailed to the medical director of each organization in late 1994 and early 1995, with follow-up telephone calls. A second mailing was sent to nonresponders. Several measures were taken to ensure accuracy of the data. We asked each respondent to indicate how well the survey responses captured their organization’s policies (choices included fully, partially, and not at all). We also asked each respondent to indicate how much involvement he or she personally has in decisions made regarding coverage of GH therapy (choices included never involved, involved in an advisory capacity, direct involvement with setting policies, oversee implementation and compliance with policies, and approve all cases seeking coverage for GH therapy). In addition, after receipt of the completed survey, each insurer was sent a personalized summary of the information for verification.

Surveys of Primary Care Physicians and Endocrinologists

We conducted separate national surveys of primary care physicians and pe-
diagnostic endocrinologists to address referral practices and treatment decisions, respectively, for childhood short stature.

The primary care physician survey was mailed to 1504 practitioners (equal numbers of family practitioners and general pediatricians), selected at random by the Division of Survey and Data Resources of the American Medical Association from the same 16 states described under the insurer survey.21 The survey included the same case descriptions as the insurer questionnaire. Physicians were asked whether they would refer each case to a pediatric endocrinologist for further evaluation, using a 5-point scale (categories included definitely would not, not likely to, not sure, likely to, definitely would refer) with the last 2 categories taken as decisions to refer.

A separate survey was sent to all members of the Lawson Wilkins Pediatric Endocrine Society, the largest professional group of pediatric endocrinologists in the United States and the only one devoted exclusively to endocrine disorders in children (n=534, excluding physicians involved in survey development and/or employed by the government or industry). Data from that survey were recently reported5 and are presented here for comparison with results from insurers and primary care physicians. The endocrinologists were asked to indicate whether they would recommend GH therapy for children with CRI or Turner syndrome, and for cases of idiopathic short stature matching those presented to primary care physicians and insurers, with additional medical information provided on the bone age x-ray film.

Determining the Potential Cohort and Costs for GH Therapy

Table 1.—Characteristics of Insurer Groups Regarding General and Specific Coverage Policies for GH Therapy

<table>
<thead>
<tr>
<th>Medical Conditions</th>
<th>GH Deficiency</th>
<th>Turner Syndrome</th>
<th>Chronic Renal Insufficiency</th>
</tr>
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<tbody>
<tr>
<td>Private %</td>
<td>100</td>
<td>50</td>
<td>79</td>
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<tr>
<td>BC/BS %</td>
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<tr>
<td>P (x2)</td>
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<td>Weighted average, %</td>
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*GH indicates growth hormone; BC/BS, Blue Cross/Blue Shield; HMO, health maintenance organization; CSHCN, Children With Special Health Care Needs; and PE, pediatric endocrinologist.
†Insurers differed, x2, P < .01.
‡Includes 1 or more of the following: PEs, adult endocrinologists (internal medicine), pediatricians, primary care physicians, nephrologists, and/or “any” physician.

RESULTS

Insurer Policies

The response rate for insurers was 75% (private insurers 80%, BC/BS 50%, HMOs 64%, CSHCN programs 82%, and Medicaid programs 84%). Over 80% of respondents indicated that they had direct involvement with setting policies for GH coverage, oversaw implementation and compliance with policies, or approved all cases seeking GH therapy within their organization. Faxed verification of responses indicated that the data were accurate.

Table 2.—Percentage of Insurers Covering GH Therapy for 3 Medical Conditions

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together with insurer responses about policies for GHs, was therefore used to construct weighted average approval rates for GH coverage. The weighted average indicates that for each of the 4 cases with idiopathic short stature, 10% to 13% of children would have insurance that covers GH treatment. For children with defined medical conditions, 94% of children with classical GHD and slightly more than half of children with Turner syndrome (52%) or CRI (58%) are likely to have insurance coverage for GH therapy.

Comparison of Physician Management with Insurer Coverage

We compared the proportion of primary care physicians who would refer short children to endocrinologists, the proportion of endocrinologists who would recommend GH therapy, and the weighted mean insurance coverage of GH treatment for several causes of short stature. There was considerable discord between physician recommendations and insurance policies for all conditions other than GHD. For example, whereas 96% and 68% of pediatric endocrinologists recommended GH therapy for Turner syndrome and CRI, respectively, 91% of the cohort of children with idiopathic short stature were not recommended for GH treatment by physicians. Thus, 91% of the cohort are not recommended for GH treatment by physicians, leaving only 9% to be considered by insurer groups. Yet, insurer groups, on average, do not cover GH treatment for 89% of these remaining children. Therefore, only 1% of the initial cohort of children with idiopathic short stature would ultimately access GH treatment.

For the 3 medical causes of short stature (GHD, Turner syndrome, and CRI) (Figure 3, right), 22% of children are excluded by the 2 physician decisions, leaving 78% for insurer considerations. Insurer groups, on average, do not cover the costs of therapy for 26% of these children, leaving 56% of the initial cohort of children with both physician recommendations and treatment coverage.

Modeling Overall Numbers and Costs

Using the results illustrated in Figure 3 together with estimated costs of therapy,6 we estimated the potential cost of GH treatment for US children. At the high end of potential costs, if all short, slow-growing US children ages 4 to 15 years (approximately 1 million) obtained GH treatment, potential annual costs could reach over $18 billion. Physician recommendations and insurer coverage policies, ascertained from the current data, would reduce this amount to approximately $357 million. If only children with 1 of the 3 medical conditions (GHD, Turner syndrome, and CRI), a physician recommendation for GH, and insurance coverage for GH were treated, there would be approximately 13,400 candidates, at an annual cost of $196 million. Thus, the potential costs to the United States for GH treatment of children could range from a low of $196 million to a high of $18 billion.

COMMENT

The overall importance of insurance coverage is clear. Patients without either public or private insurance often have reduced access to medical care and poor medical outcomes, compared with insured patients.13 However, the influence of type of insurance on access to and use of specific treatments—particularly in the context of physician recommendations—is not well understood. The current analyses provide insight into the role of insurance in treatment utilization and processes for specialized medical therapies.

The data indicate significant variation among US insurers regarding coverage of GHs, with insurers covering 94% of children with classical GHD and slightly more than half of children with Turner syndrome (52%) or CRI (58%) are likely to have insurance coverage for GH therapy. The percentage of primary care physicians who would refer each of the cases, the percentage of endocrinologists who would recommend growth hormone (GH) therapy, and the proportion of children for whom GH therapy would be approved by insurers is shown.
policies for GH therapy, supporting the concept that there are major discrepancies in access to treatment as a function of third-party payers. These findings are consistent with the few earlier retrospective assessments for other conditions, undertaken in selected locations and populations 31-35 and extend them by conducting analyses across insurance groups and geographic sites.

The contrast between physician recommendations for GH therapy and the coverage decisions of insurers is striking. Primary care physicians appear to use discretion in referring short children to specialists who, in turn, are fairly selective in their treatment recommendations (ie, the overwhelming majority would not recommend GH treatment for a moderately short child such as case A). Despite the sequential medical process that favors relatively limited GH use, insurance adds a further significant level of restriction. For Turner syndrome, almost half of the children recommended for GH treatment by endocrinologists would not, on average, have insurance that covered treatment, and 42% of children with CRI who are recommended for treatment would not have coverage. For idiopathic short stature, pediatric endocrinologists were selective in their recommendations based on physiological patient characteristics, whereas insurer policies were less responsive to these characteristics. Thus, for 3 of 4 cases presented, far fewer children would have insurance coverage for GH therapy than would be recommended for treatment by physician experts. The insurer therefore plays a critical role in influencing access to this treatment and, in conjunction with other determinants, is instrumental in limiting GH therapy to 1% of the initial cohort of children with idiopathic short stature and 56% of children with 1 of 3 defined medical conditions.

While these findings cannot establish whether current practices are right or wrong, questions remain as to the appropriate role of the insurer and about how final decisions should be made regarding access to treatments.2,3,34 The discord between physicians and insurers may result, in part, from current debates about what constitutes deficiency and disease, and what testing criteria ought to be used in the determination of a GH disorder.4,7,10,11 Nevertheless, judging from the data, many payers do not agree with the expert opinion of pediatric endocrinologists. The apparent disconnecting by insurers of physician recommendations may not be limited to GHs. Reports within the lay press35,36 suggest similar conflicts in other settings, such as bone marrow transplants in cancer patients. Federal agencies have begun to consider such issues.37 The current data, to our knowledge, are the first to systematically analyze and quantify the implications of disagreements between physicians and insurers. Although disagreements may be difficult to resolve, our findings underscore their importance in determining differential access to treatment and, as such, indicate the necessity of serious efforts toward resolution.

In interpreting the findings, several limitations are noted. It is possible that coverage policies reported from an insurer’s national headquarters (eg, private insurers) may not reflect local variation or “tailor-made” individual policies, although respondents to our survey did indicate that policies are consistent across product lines. Categorical policy decisions may also be mutable by pressures such as lawsuits32 and individual lobbying. The absence of global claims data precludes comparison with these survey results, although the figures derived from our survey data are consistent with those reported elsewhere. In addition, although our response rates were generally high, plans not responding might have different policies from those of the responders. The increasing trend toward state Medicaid managed care plans may impose different prescribing and approval patterns within a more restrictive environment. It is also recognized that some children and their families may be self-referred to endocrinologists, while some children with distinct medical disorders may be missed or misdiagnosed at an early point in the sequential decision process. Furthermore, we assumed independent decision making when modeling exclusion processes. It is possible that physician decisions and insurer decisions are influenced by each other, although we controlled for insurance in the survey to prescribing physicians by indicating the out-of-pocket costs of GH treatment (after insurance coverage) for the cases presented. Physician and insurer decisions may also be altered by future changes in treatment costs, although the price of GH has remained
relatively stable despite new manufacturers and the loss of Orphan Drug status in 1995.

In summary, this study provides insight into the sequential process of medical decision making found in most nonemergency or subspecialty care. It illustrates how medical referral and expert opinion serve as powerful forces in limiting treatment utilization to a subset of potential patients even in cases as ambiguous as short stature of unknown cause. The fact that insurance coverage further limits access may simply reflect a reasonable cost-benefit calculus at work. Alternatively, it may represent inappropriate denial of care. Nonetheless, the discrepancy between physician treatment recommendations and insurance coverage, exemplified by the current findings, constitutes a critical challenge to health care delivery with serious ramifications for access, costs, and outcomes.

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References


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