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JAMA. 1998;279(9):663-668 (doi:10.1001/jama.279.9.663)

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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.

JAMA. 1998;279:663-668

THE INFLUENCE of insurance on access to health care, especially access to expensive specialist care and treatment, is a major concern.¹⁻³ 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 semiselective treatments related to quality of life (such as treatments for short stature, infertility, obesity, and aging⁴), 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 semiselective, is rarely needed for life-threatening situations, and is very costly (approximately \$14 000 per year for a child weighing 20 kg).⁶ Finally, optimal GH use has been the subject of debate.⁷⁻¹² Traditionally, GH therapy has been used for children who have classical GH deficiency (GHD)^{11,13} due to a lack of natural GHs. However, the medical literature and available guidelines suggest

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Presented in part at the 14th annual meeting of the Association for Health Services Research, Chicago, Ill, June 17, 1997.

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diverse criteria for defining GHD and identifying appropriate candidates for treatment.⁷⁻¹³ Physicians may and do prescribe GHs for conditions other than GHD.^{5,12,14-15} 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).^{4,9,12,15} 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 Food and Drug Administration (FDA) and for which physician consensus to recommend or use GHs ranges from moderate (CRI) to very high (GHD, Turner syndrome)⁶; and (3) whether their organization would cover GH therapy for case scenarios of children who are short but do not have classical GHD or other defined medical disorders (ie, idiopathic short stature). (Note: GH therapy was approved by the FDA for Turner syndrome on December 31, 1996, after this survey took place.)

The format for the case presentations of children with idiopathic short stature involved systematic variation of experi-

mental variables to create a controlled environment for the study of GH coverage decisions. Insurers were presented with 4 case scenarios (cases A-D; Figure 1), differing only in the physiological growth variables concerning degree of short stature (height, 2 or 3 SDs below the mean for the child's age) and the rate of growth (the 3rd to 10th percentile or below the third percentile for age, designated as 4.5 and 3.2 cm per year [1.8 and 1.3 in per year],¹⁶ respectively). The case descriptions were designed to be representative of the population of relatively short, slow-growing children (height, >1.5 SDs below the mean, growth rate below the 10th percentile for age¹⁶) with the common and often difficult-to-differentiate conditions of idiopathic short stature, familial short stature, and constitutional delay in growth and development.^{4,13,17} The case descriptions were also designed to match the cases presented in separate surveys (described below) to primary care physicians for referral decisions, and to pediatric endocrinologists for treatment recommendations. Case descriptions included information typically used by each group (eg, insurer surveys indicated that, for each case scenario, GH therapy had been "prescribed by an authorized physician"). To control for other variables, the clinical context of the scenarios was described in detail. The patient presented was a 10-year-old boy or girl (the average age of children presenting to endocrinologists for short stature and the approximate midrange of potential candidates¹⁸) with no other abnormalities on physical examination, normal peak GH levels (15 µg/L) in response to a stimulation test,^{5,7,13} and all other laboratory test results normal (including free thyroxine, thyrotropin, complete blood cell count, erythrocyte sedimentation rate, urinalysis, chemistry profile, and, in females, karyotype), excluding the diagnosis of classical GHD or other medical causes of short stature.

All state Medicaid agencies were surveyed since preliminary investigation indicated likely state-to-state variation.¹⁹ Because some state public programs for Children with Special Health Care Needs (CSHCN)²⁰ act as the payer of last resort for the underinsured and uninsured, we surveyed these programs from 16 states (Alabama, Arizona, California, Florida, Georgia, Indiana, Maine, Maryland, Michigan, Minnesota, Nebraska, New Hampshire, New Mexico, North Carolina, Ohio, and South Carolina) chosen by stratified sampling according to variation in physician-to-population ratio and geographic diversity,²¹ and to match the sampling frame used for the survey of primary care phy-

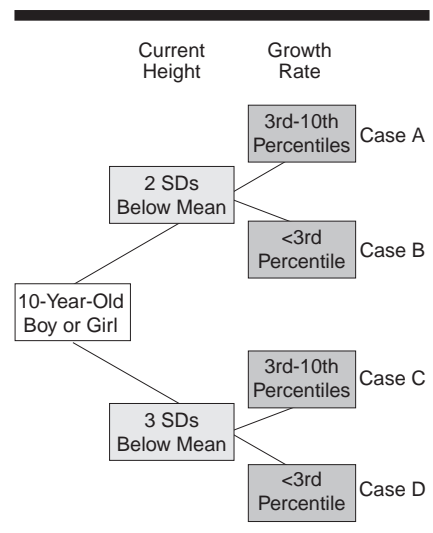


Figure 1.—Cases of idiopathic short stature presented (cases A-D) to insurers. The 4 cases are described in detail in the "Methods" section. Case A was moderately short and slow growing (height, 2 SD below the mean; growth rate, 4.5 cm per year). Successive children (cases B-D) presented progressively more severe short stature and/or slower growth rates.

sicians as described below. The distribution of health insurer coverage in the 16 states is representative of the US population. To capture private insurer decisions, Blue Cross and Blue Shield (BC/BS) agencies from these states were surveyed. In addition, we targeted the largest 5 private US indemnity insurers²² (60% of all lives covered by such plans), and the largest 25 individual health maintenance organizations (HMOs) in the United States in 1993 (covering 35% of all individuals enrolled in HMOs).²³

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-

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

	Private, %	BC/BS, %	HMO, %	CSHCN, %	Medicaid, %
Do you cover all or part of the costs of GH therapy, if prescribed by an authorized physician?†					
Always cover GH therapy	0	0	0	0	28
Sometimes cover GH therapy	100	100	100	78	63
Never cover GH therapy	0	0	0	22	9
Which physician groups are authorized to prescribe GH therapy?†					
PE only	25	13	63	60	18
PE or other physicians‡	75	87	37	40	82

*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.

†Insurer groups differed, χ^2 , $P < .01$.

‡Includes 1 or more of the following: PEs, adult endocrinologists (internal medicine), pediatricians, primary care physicians, nephrologists, and/or "any" physician.

diatric 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

Census data on the US population of children,²⁴ together with assessments of disease prevalence from the medical literature,^{14,25,26} were used to determine the potential number of US children eligible

for GH treatment because of the 3 medical disorders (GHD, Turner syndrome, and CRI). In evaluating the number of children represented by the 4 cases of idiopathic short stature, we used census data and the conditional presence of low or very low growth rates within designated height categories, estimated using the Delphi method²⁷ with a panel of 8 pediatric endocrinologists. Since the primary care physician may not diagnose the cause of short stature in children with medical conditions such as Turner syndrome, his or her decision to refer would typically be based on stature and growth patterns alone. Therefore, referral patterns for the 4 cases of idiopathic short stature, based on height and growth rate, were used to estimate referrals by primary care physicians for children with medical causes of short stature. The cost of GH therapy, ascertained from the literature⁶ and corroborated by pharmacists and GH manufacturers, was combined with standard dosages⁶ to yield an annual cost of \$700 per kilogram of body weight (applied to each case presented based on average weights).¹⁶

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.

All insurers reported having a policy for the coverage of GH therapy, with the exception of 9 state Medicaid programs. As shown in Table 1 (top), 28% of Medicaid agencies reported that they *always*

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

Insurer Groups	Medical Conditions		
	GH Deficiency	Turner Syndrome	Chronic Renal Insufficiency
Private	100	50	75
BC/BS	100	25	37
HMO	100	62	56
CSHCN	71	50	36
Medicaid	91	81	79
P (χ^2)	.06 (8.0)	<.01 (12.65)	<.02 (11.87)
Weighted average, %	94	52	58

*GH indicates growth hormone; BC/BS, Blue Cross/Blue Shield; HMO, health maintenance organization; and CSHCN, Children With Special Health Care Needs.

cover GH therapy if prescribed by an authorized physician and almost one quarter (22%) of CSHCN programs reported that they *never* cover any part of GH costs. Other payers fell between these 2 extremes, covering GH therapy only under certain circumstances.

Heterogeneity also existed among third-party payers as to the type of physician authorized to prescribe GHs. As shown in Table 1 (bottom), CSHCN programs and HMOs tend to restrict GH coverage to prescriptions from pediatric endocrinologists, whereas most Medicaid agencies and private insurers allow other physician groups (including pediatric endocrinologists, adult endocrinologists [internal medicine], pediatricians, primary care physicians, nephrologists, and/or "any physician") to prescribe GHs.

Insurer policies for GH treatment varied strikingly for conditions other than classical GHD. For example, coverage policies for GH treatment of CRI varied significantly among insurer groups, with approvals ranging from 36% of CSHCN programs to 79% of Medicaid programs ($P < .02$, Table 2). For Turner syndrome, GH coverage ranged from 25% of BC/BS insurers to 81% of Medicaid programs ($P < .02$, Table 2). For each of the 4 children with idiopathic short stature, variation in coverage was marked ($P < .001$). None of the private and BC/BS insurers approved GHs for any of the cases presented, whereas approximately half (48%-50%) of Medicaid agencies approved GHs for each case. Eight percent of the CSHCN programs approved GHs for cases C and D, and 6% of HMOs approved coverage for case D.

Based on the literature,²⁸⁻³⁰ the distribution of insurance coverage for US children was assessed as follows: private insurance (24%), BC/BS (25%), Medicaid (21%), HMO (17%), and CSHCN/uninsured (13%). There are no data indicating different patterns of insurance for short stature children; this distribution,

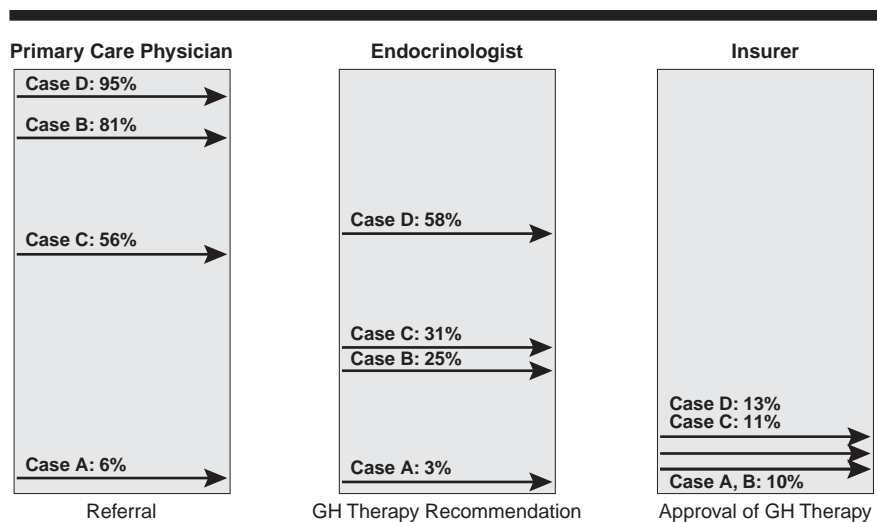


Figure 2. —Comparison of physician referrals, treatment recommendations, and insurer decisions for 4 case scenarios (A-D) of children with idiopathic short stature. The percentage of primary care physicians who would refer each of the cases, the percentage of endocrinologists who would recommend growth hormone (GH) therapy, and the proportion of children for whom GH therapy would be approved by insurers is shown.

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, insurers would cover GH therapy for only 52% and 58% of these children, respectively.

For the 4 cases of idiopathic short stature, the differences between physician recommendations and insurance coverage were particularly marked, as illustrated in Figure 2. Among primary care physicians, decisions to refer children to specialists ranged from 6% (for a moderately short and slow-growing child, case A) to 95% (for a very short and very

slow-growing child, case D). While less than 5% of endocrinologists recommended GHs for the first child (case A), recommendations rose progressively depending on the height and growth rate of the child, with 58% of endocrinologists recommending GHs for the most severely affected child (case D). By contrast, insurer decisions were more categorical, with approvals for coverage of 10% to 13% of children in each of the 4 cases. Thus, there was a 2- to 4-fold mismatch between the recommendations of endocrinologists and the coverage policies of insurers, with at least 1 reversal (ie, proportion of insurers approving GH was greater than physician recommendations).

Understanding the Sequential Decision Process for GH Treatment

Combining the initial cohort of children with GHD, Turner syndrome, CRI, and idiopathic short stature with the survey results for (1) primary care physicians' referral decisions, (2) endocrinologists' GH treatment recommendations, and (3) insurer approvals of GH coverage allows assessment of the proportion of children excluded at each step of the decision process (Figure 3). While we recognize that families play a key role in GH use by the emphasis placed on stature, physician-seeking behaviors, and willingness to undertake treatment,¹⁸ data are lacking on the factors that determine parental decisions and therefore the models assume that all potential candidates would present to primary care physicians to initiate the process. Approximately 66% of children with idiopathic short stature are likely not to be

referred to an endocrinologist by a primary care physician (Figure 3, left); of those referred, 74% are likely not to be recommended for GH treatment by endocrinologists. 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 28% 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.^{1,3} 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 processes for specialized medical therapies.

The data indicate significant variation among US insurers regarding coverage

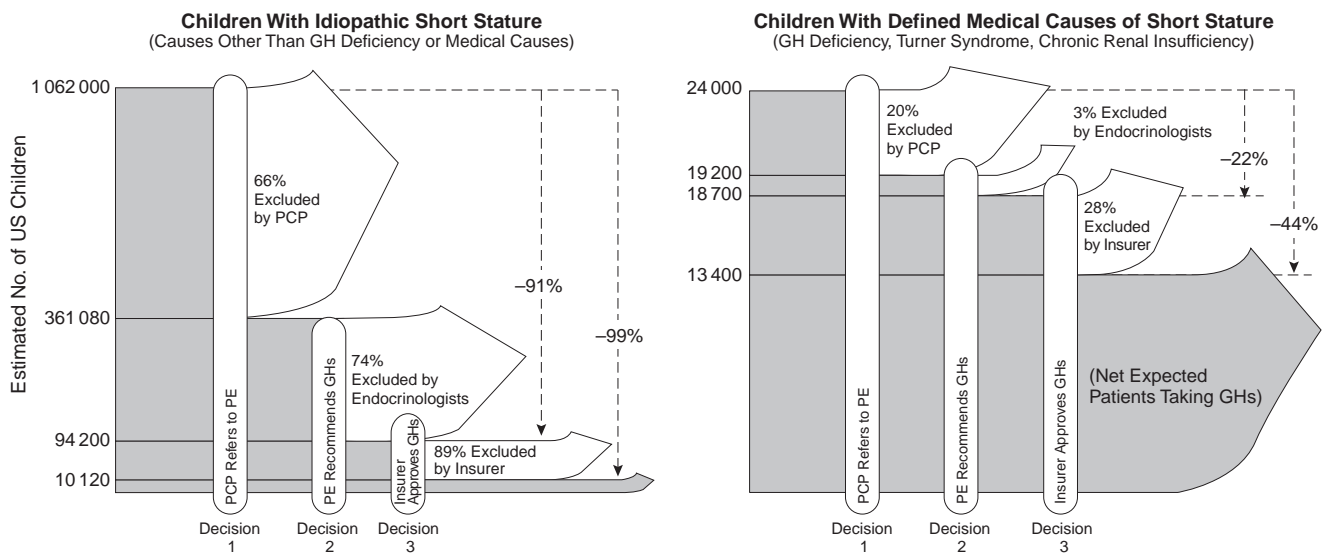


Figure 3.—Sequential decision process for growth hormone (GH) therapy in children with idiopathic short stature (left) and 3 defined medical causes of short stature (GH deficiency, Turner syndrome, and chronic renal insufficiency) (right). The proportion of US children (age range, 4-15 years) excluded at each step of the process is shown. PCP indicates primary care physician; PE, pediatric endocrinologist.

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,³¹⁻³² 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.^{2,33,34} 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 discounting by insurers of physician recommendations may not be limited to GHs. Reports within the lay press^{35,36} 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 non-emergency 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 cur-

rent findings, constitutes a critical challenge to health care delivery with serious ramifications for access, costs, and outcomes.

This work was supported by a grant from the National Institutes of Health. Ms Finkelstein's work was supported in part by a National Research Service Award (T32 HS00059-03) from the Agency for Health Care Policy and Research.

We thank all the insurers, primary care physicians, and endocrinologists who participated in this study.

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