

Workshop Findings: Recommendations for Improving Clinician and Payer Communication About Growth Hormone Use

After contributing to the Consultants' Conference's presentations and discussion sessions, participants were divided into 3 workshops to examine the issues relating to growth hormone use and reimbursement, to identify areas that need resolution, to make recommendations, and to arrive at a consensus on a plan for future action. Each of the workshops included clinician and payer participants and chairs to maximize interaction and communication. Findings from each of the workshops are presented below.

■ Workshop #1: Pediatric Patients

Cochairs: Ora Pescovitz, MD, and Harvey P. Katz, MD

Findings presented by Dr. Pescovitz

The agenda for the participants in this workshop was divided into 3 parts: 1) identification of areas where there were no controversies; 2) identification of areas that remain controversial; and 3) recommendations for a process by which the defined controversies could be resolved.

At present, there is no disagreement regarding the use of growth hormone in children with classic growth hormone deficiency, Turner syndrome, and chronic renal insufficiency before organ transplantation.

There is, however, considerable controversy regarding the use of growth hormone in children with idiopathic short stature, partial or transient growth hormone deficiency, constitutional delay of growth, poor growth after renal transplantation, intrauterine growth retardation, and steroid-dependent growth failure.

The plan of action regarding non-controversial areas is for clinicians

and payers to continue to cooperate. For the controversial areas, however, the workshop participants agreed that a series of "next steps" were necessary.

The participants offered a number of suggestions for consideration and discussion, including a recommendation that a committee appointed by the board of directors of the Lawson Wilkins Pediatric Endocrine Society (LWPES) issue a policy recommendation to clarify the use of growth hormone in children with "controversial" indications. Those issuing the policy recommendation would include clinicians who are members of the society, academicians and research investigators, pharmaceutical companies, payers, and representatives of regulatory agencies.

The participants recommended that Dr. Ron Rosenfeld, one of the chairs of the Consultants' Conference, write a letter to the LWPES board of directors to inform it of the progress that took place at the conference and to communicate the recommendation of an LWPES-appointed committee.

The participants also suggested a timetable to be used for follow up of its plan of action. The first phase

would consist of monitoring the progress made by LWPES in appointing members to a policy recommendation group. The second phase would involve reconvening the Consultants' Conference some time between September 2000 and March 2001 to:

- Address recommendations made by the policy group and learn more about its recommendations.
- Discuss plans to implement these recommendations.
- Develop a plan of action to deal with new issues.

The workshop participants agreed that it was important for the consultants to reconvene to evaluate the policy recommendations made by the LWPES group. Such a measure would encourage extensive evaluations of the recommended policies by pediatricians and pediatric endocrinologists in cooperation with colleagues in adult medicine and adult endocrinology, as well as with representatives of the payer community.

A final area of consensus arising from this workshop was the issue of the high cost of growth hormone as it applies to all growth hormone use in pediatric patients. During the course of the conference, it was mentioned many times that if growth hormone therapy were not so costly there would be little need for the conference. Workshop participants also raised several ancillary issues pertaining to cost, such as who is going to pay for developing policy recommendations, who should reconvene the Consultants' Conference, and who should pay for it? Answers to these questions were not arrived at during the workshop.

■ Workshop #2: Adult Patients

Cochairs: Vivien Bonert, MD, and Pete Fullerton, PhD

Findings presented by Dr. Bonert

Areas of consensus outweighed areas

of dissent in this workshop during which several key points were raised. The major point was that adult growth hormone deficiency is a clearly defined syndrome that is caused by some organic or pituitary-hypothalamic disease and that it should be recognized as such.

Clinical features that contribute to morbidity and mortality in those with the syndrome include decreased bone mineral density and increased risk of fracture, a 3-fold greater incidence of cardiovascular mortality in these patients compared with normal controls, a 3-fold increase in cerebrovascular mortality compared with controls, and unfavorable changes in body composition (ie, a decrease in lean body mass and an increase in body fat). The long-term morbidity and mortality associated with cardiovascular disease and the adverse changes in bone mineral density were of particular concern to workshop participants representing the payer community because of their enormous impact on healthcare costs.

The payers raised several concerns regarding growth hormone abuse among adults and explained why such abuse has led to some resistance to reimbursement for growth hormone therapy in the past. Stories in the lay press about normal adults who "feel great" because they took growth hormone and with documented instances of adults who abused the healthcare system to garner reimbursement for nonapproved indications have contributed to the reasons why payers are wary of reimbursing growth hormone use in adults.

However, the clinicians participating in this workshop noted that growth hormone has extremely beneficial short-term and long-term effects in adults with growth hormone deficiency and that payers should not deny reimbursement for growth hormone treatment in such patients as they attempt to screen out those who would abuse it.

The workshop participants recommended that growth hormone, at an appropriate dose, be provided to adults with organic growth hormone deficiency stemming from pituitary or hypothalamic disorders or trauma. They also agreed that insulin-like growth factor-1 (IGF-1) was a suitable marker with which to monitor both the safety and efficacy of growth hormone therapy in these patients.

Regarding the steps to be taken next and a plan of action, the workshop participants agreed that the Pituitary Society should generate a policy statement that clearly specifies approved and nonapproved indications for growth hormone use in adults as well as appropriate dosing (including the maximum dose) and monitoring. Because the workshop participants felt the statement should be one that could be used by clinicians and payers alike, 2 of the participants who are leaders of the Pituitary Society offered to draft a preliminary statement that would reflect input from both communities. The resultant policy statement from the Pituitary Society will then be sent to medical directors of insurance companies.

Although the Growth Hormone Research Society has issued a statement regarding growth hormone use in adults, the workshop participants, particularly those representing payers, felt it was more focused on diagnosis than on treatment and more on academic research findings than on clinical practice. Thus, the Pituitary Society was selected to draft the policy statement.

**■ Workshop #3:
Transitioning Patients**

Cochairs: Stephen LaFranchi, MD, Baha Arafah, MD, and John Bloom, MD

Findings presented by Dr. Bloom

Consultants participating in this workshop explored areas of agreement and disagreement and arrived at

a set of recommendations that covered both. They agreed that transitioning patients with structural lesions (eg, status post radiation, multiple pituitary hormone deficiencies, and isolated growth hormone deficiency) be evaluated before continuing, discontinuing, or restarting growth hormone therapy. They also agreed to wait for a policy statement from the Growth Hormone Research Society before deciding whether 1 test or no test was necessary to proceed.

For adolescents with isolated idiopathic growth hormone deficiency, the participants agreed that the following baseline tests were needed before deciding to continue or discontinue growth hormone: IGF-1 level, lipoprotein profile, dual-energy X-ray absorptiometry (DEXA) of both bone density and body composition, and a reliable and validated quality-of-life assessment. They also agreed that the baseline tests should be done after the patient has been off growth hormone for at least 72 hours and that a growth hormone stimulation test should be done with 2 pharmacologic agents. Values below 5 ng/mL indicate ongoing growth hormone deficiency requiring continuation of treatment, whereas values between 5 ng/mL and 10 ng/mL indicate uncertainty thus warranting vigorous monitoring, and values above 10 ng/mL are considered normal.

For follow-up monitoring, the workshop participants recommended physical examination, measurements of IGF-1 levels and lipoprotein levels, and DEXA scans. Normal DEXA scans for 2 years suggest that growth hormone therapy could be discontinued.

Dosage should be based on the 50th percentile IGF-1 level for the patient's age, starting with 2 to 3 mg/kg/day and increased to tolerance.

The next step should be convening a transitioning consensus conference by a national organization similar to the LWPES to draft a policy statement.