FDA advisory panel okays Genentech's Nutropin

Genentech's new hGH should be approved for treating growth failure in children with chronic renal insufficiency.

WASHINGTON, D.C.—Genentech's (S. San Francisco, CA) Nutropin—its newer recombinant version of human growth hormone (hGH)—should be approved for treating growth failure in children with chronic renal insufficiency (CRI) until these children receive kidney transplants. That's what the Food and Drug Administration's (FDA, Bethesda, MD) Endocrinologic and Metabolic Drugs Advisory Committee unanimously recommended recently.

Genentech's Protropin hGH is currently marketed for treating children with hGH inadequacies, which leads to reduced growth. Protropin differs from Nutropin in containing methionine as its N-terminal amino acid, giving it one extra amino acid, and by subtle ways in which it is folded during production. Nutropin, in fact, is identical to Eli Lilly's (Indianapolis, IN) hGH product, which will lose its orphan-drug status in March. At that point, Genentech plans to replace Protropin with Nutropin to treat hGH inadequacy, pending FDA approval. Genentech has conducted clinical trials showing that Nutropin is as effective as Protropin in treating children with hGH inadequacy and has filed for FDA approval to market Nutropin for this indication. Genentech believes that, since Nutropin is a more faithful replica of genuine hGH, it is a better product.

The FDA advisory committee reviewed data from clinical trials involving nearly 200 young individuals with CRI who were treated with Nutropin. CRI, a condition in which there is severe damage to the kidneys, leads to a variety of health problems, including severe and ordinarily irreversible growth retardation. Though children with CRI produce hGH, the hormone is inhibited from working. Therapeutic doses of hGH override this inhibition, however, allowing hGH to induce growth.

Indeed, Genentech's clinical-trial data show that height in the normal range was achieved by 65 percent of CRI patients treated for two years with Nutropin and that normal-range height was achieved by 91 percent of CRI patients treated for at least three years with Nutropin. Because CRI affects only about 3,000 children in the U.S., FDA has granted Nutropin orphan-drug status for this disease.

Kidney-transplant procedures cannot be done on very young CRI patients and, without pretransplant hGH treatments, such patients will never "catch up" in stature to their healthy peers. CRI patients who receive hGH and then receive kidney transplants will discontinue hGH treatment, because normal growth should resume.

In terms of safety, Nutropin does not appear to cause any major side effects in CRI patients. Although in other clinical studies hGH treatment seems to have been associated with the development of leukemia in a small fraction of patients, the evidence for this association is "in dispute," according to FDA officials. The CRI clinical studies showed no such association, but they were on too small a scale to put much confidence in that finding. Hence, this and other safety-related issues should continue to be monitored in phase-IV, postmarketing surveillance studies, according to the advisory panel.

—Jeffrey L. Fox

Biotech executives loathe price controls

Price controls will cause 77 percent of biotech executives to curtail expansion plans.

NEW YORK—Fully 68 percent of U.S. biotech executives believe that U.S. health-care reform will result in price controls on new drugs, with such controls carrying dire consequences. Indeed, 77 percent of these same executives say that price controls will cause them to curtail expansion plans, 74 percent say that such controls will result in the industry losing its leading edge, and 67 percent say that price controls will force them to cut back on new-product financing.

So reports KPMG Peat Marwick (Princeton, NJ) in a recent survey entitled "The Biotechnology Industry and Health-Care Reform: The Views of Senior Executives." The survey is based on interviews with 100 chief executive officers and senior-level executives of U.S. biotech firms, with 59 of these firms privately held and 41 publicly held.

Currently, the Clinton administration's health-care-reform package includes indirect price controls. An Advisory Council on Breakthrough Drugs would assess the "reasonableness" of new drug prices by examining company information on the cost of a drug and by considering company pricing of a drug in other countries. Also, Medicare would receive a 17 percent rebate on the average nationwide price of each drug it reimburses, with that rebate higher if the drug price has increased faster than inflation or, in the case of a new drug, if the price is deemed "unreasonable."

Among other survey highlights:

- Fully 59 percent of executives believe that the only way even the strongest firms will survive health-care reform is through mergers and acquisitions. So most executives believe that over the next five years the number of public firms will remain constant, at between 200 and 250, with mergers and acquisitions offsetting initial public offerings.
- Over the next 18 months of health-care-reform debate, 77 percent of executives see joint ventures or corporate alliances as a "likely" funding approach. Sixty percent see contract research as a likely approach, while 53 percent see the public markets as a likely approach.
- Eighty-one percent of executives believe that health-care reform will force them to add staff to judge the clinical utility and cost effectiveness of their products before they're approved. And 58 percent believe that the biggest impact of health-care reform will be greater product cost effectiveness.
- Though 95 percent of executives believe that health-care reform will occur in the foreseeable future, only 23 percent believe that it will take effect in 1994. Forty-three percent see it happening in 1995, 17 percent in 1996, and 12 percent after 1996.

—B.J. Spalding