Human growth hormone shows promise in treating cystic fibrosis symptoms

October 4 2010, By Colin Poitras

(PhysOrg.com) -- A study finds that while the hormone can treat the symptoms, its impact on the disease is unclear.

Human growth hormone can be used successfully to treat some symptoms of cystic fibrosis and prevent hospitalizations, but its impact on the disease itself remains unknown, according to a new report produced by the University of Connecticut/Hartford Hospital Evidence-based Practice Center.

Cystic fibrosis is an inherited, chronic, multi-organ disease caused by a defective gene, in which the body produces thick mucus that clogs the lungs and leads to life-threatening lung infections. The disease also makes it difficult for the pancreas to work, hampering the ability to absorb food. Common signs are salty tasting skin, difficulty breathing, chronic lung infections, poor weight gain, and shorter height. Typically detected in childhood, the disease stunts the patient's growth and usually leads to early death.

The report finds that the use of human growth hormone increases height and weight, may improve lung functioning, and may strengthen the bones of patients with cystic fibrosis. Researchers found evidence suggesting that human growth hormone therapy reduces the need for hospitalizations, but could find no evidence that the therapy prolongs life or improves health-related quality of life. They also found that human growth hormone raises blood sugar modestly, which may over time lead to the development of diabetes in some patients.

“Patients with cystic fibrosis and their families have long looked for ways to manage this disease,” says Dr. Carolyn Clancy, director of the federal Agency for Healthcare Research and Quality (AHRQ), which funded the study. AHRQ is the health services research arm of the U.S. Department of Health and Human Services. “This report gives patients and their families excellent information that they can use – in consultation with their doctors – to make decisions about care.”

The UConn report – Effectiveness of Recombinant Human Growth Hormone (rhGH) in the Treatment of Patients with Cystic Fibrosis – was published online today in the journal *Pediatrics*.

Cystic fibrosis afflicts about 30,000 children and adults in the United States. In the 1950’s, few children diagnosed with cystic fibrosis lived to attend elementary school, but advances in antibiotic therapy over the past 25 years have raised the median age of survival to 37 years old.

Recombinant human growth hormone (rhGH) is an anabolic agent approved by the U.S. Food and Drug Administration for a variety of uses, including the treatment of growth hormone deficiency, chronic kidney failure, and small size in children. Human growth hormone therapy requires that patients inject the medication every day for several months.

“Unfortunately, individuals with cystic fibrosis need frequent hospitalizations and are more likely to develop osteoporosis or be small for their age,” says C. Michael White, director of the UConn/Hartford Hospital Evidence-Based Practice Center and the study’s lead author. “Human growth hormone therapy has been shown to increase height by 1¼ inches and weight by 3 pounds, prevent between one and two hospitalizations a year, increase the strength of the bones, and improve some measures of lung health in poorly growing children. It is thought that increases in height and weight may create increased lung capacity,

creating some reserve lung function when infections set in and thus preventing hospitalizations."

In their meta-analysis, the researchers reviewed 56 studies starting from 1950. After screening the studies for various terms of eligibility (weeding out duplicates and those not involving human subjects, for example), the researchers finished with a net group of 10 studies. The researchers focused on seven key questions examining the benefits and harms associated with using rhGH in patients with cystic fibrosis.

While the findings are encouraging, White cautioned that individuals should consult with their physicians in determining the best course of treatment.

"While the therapy was well tolerated in trials, children or their caregivers would need to get a shot of this medication every day for six to 12 months, which can be tough for children who are already taking other medicines every day," says White, a professor in the UConn School of Pharmacy. "It costs $300 to $600 per week, but since the medication can reduce costly hospitalizations, health plans may be willing to cover the cost of therapy."

The University of Connecticut/Hartford Hospital Evidence-based Practice Center, located on the campus of Hartford Hospital, is one of 14 evidence-based practice centers certified by AHRQ in North America. The UConn/Hartford Hospital center was selected by AHRQ in July 2007.

Evidence-based practice centers conduct systematic reviews and meta-analyses in order to expand the health care knowledge base so that both private and public health care organizations can make more informed health care decisions, formulate national guidelines, and devise standards of practice. Research developed by evidence-based practice centers is

used by federal agencies for Medicare and Medicaid, the FDA, the National Institutes of Health, and the Centers for Disease Control and Prevention, among others. The information is also used by managed care organizations, national medical societies, and nonprofit healthcare organizations, as well as clinicians and patients.

Provided by University of Connecticut