UC San Diego Health

UCSD Medical Center and Children's Hospital To Participate in Clinical Study for Short Stature

September 19, 2005 |

UCSD Medical Center and Children's Hospital and Health Center have launched a clinical trial to evaluate the potential benefit of a therapy to treat children with growth failure caused by growth hormone abnormalities. The study is currently underway and will enroll 160 patients at 40 medical centers across the United States.

"This is the first new prospective treatment in thirty years to provide an alternative therapy for short stature," said Michael Gottschalk, M.D., UCSD Professor of Pediatrics and principal investigator of the trial. Until now the only option has been to administer growth hormone replacement.

Gottschalk explains that in healthy individuals, growth hormone is secreted into the bloodstream where it binds with cell receptors and stimulates the production of an insulin-like growth factor into the bloodstream. This growth factor is the principal hormone necessary for children's bones and organs to grow properly. Endocrinologists have long recognized that children with short stature are growth hormone-deficient and have low Insulin-like Growth Factor-1 (IGF-1) levels.

"Primary deficiency of IGF-1 production afflicts an estimated 30,000 children evaluated for short stature in the United States," Gottschalk said. "Traditionally, physicians have used growth hormone replacement to raise the levels and improve growth rates. However, there are many short stature children who are not deficient ingrowth hormone, but there is no known cause for their disease."

Gottschalk said new insights into the growth process have revealed that a significant portion of these children most likely have short stature caused by a lack of IGF-1 production. Because these children are less sensitive to growth hormone than other children, traditional growth hormone therapy is not an optimal treatment. This new understanding of IGF-1 as a cause of short stature, has led to the clinical development of recombinant human insulin-like growth factor, or rhIGF-1, as a potential replacement therapy for children with this disease. Children in the study will receive rhIGF-1, structurally and functionally identical to IGF-1, which is naturally produced by the human body. The biopharmaceutical firm, Tercica, which manufactures rhIGF-1, is the study sponsor.

According to Gottschalk, a child with short stature is medically defined as being shorter than the 3rd percentile on the growth chart of all children the same age and gender. Current estimates suggest that Primary IGF-1 Deficiency (IGFD) causes short stature 1.5 times more often than growth hormone deficiency. IGFD is diagnosed in children who have normal or elevated secretion of endogenous growth hormone and whose height and serum IGF-1 levels are below normal.

Primary IGFD can be caused by abnormalities of the growth hormone receptor or the growth hormone signaling pathway. If untreated, Primary IGFD may lead, in children and adults, to a range of other metabolic disorders, including lipid irregularities, decreased bone density, obesity, and insulin resistance.

The study follows a previous clinical trial of rhIGF-1 in children with Severe Primary IGFD. In the previous phase researchers demonstrated that compared to pre-treatment growth patterns, on average the children gained an additional inch per year for each year of therapy over the course of eight years.

UCSD is conducting the study at Children's Hospital and Health Center. The study seeks children with Primary IGFD who have not yet entered puberty, are below a certain height for their age and sex, have an IGF-1 level below the normal range, are found *not* to be growth hormone-deficient, and have not been treated with recombinant human growth hormone, (rhIGF-1) or other growth disorder medications, or medications for hyperactivity and/or attention deficit disorders (ADHD/ADD). Children will be enrolled until researchers fill all study slots nationwide.

Parents who think their children might qualify for this study can call **858-966-8130** or email cmlee@chsd.org to receive information. Children who qualify will receive study medication, medical evaluations, and all other study-related procedures at no cost.

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