Insulin-Like Growth Factor Treatment of Growth Disorders

Ron Rosenfeld

Oregon Health and Sciences University President, STAT5, LLC

Patients with growth hormone (GH) insensitivity, typically resulting from mutations affecting the GH receptor (GHR), GHR signaling cascade and insulin-like growth factor-1 (*IGF-1*) gene, are, generally, unresponsive to GH therapy. Beginning in the 1990's, clinical trials of IGF-1 administration in such patients demonstrated both short- and long-term efficacy, although not to the degree observed with GH treatment of naïve GH deficient patients. Adverse effects, including hypoglycemia, lymphoid overgrowth, benign intracranial pressure and coarsening of facial features, have been observed, but, in general, have proven to be transient. As demand for IGF-1 treatment for children currently labeled as idiopathic short stature increases, it will be important to have controlled clinical trials of GH, vs. IGF-1 vs. combination GH + IGF-1.

Table 1. Long-term results of insulin-like growth factor-1 (IGF-1) treatment

	Ranke et al (11)		Chernausek et al (13)	
Years on IGF-1	**n**	**cm/yr**	**n**	**cm/yr**
1	15	8.8	59	8
2	13	7	54	5.9
3	12	6.8	48	5.5
4	15	5	39	4.8
5		5.2	21	4.9
6	6	6.3	20	5
7			16	4.7
8			14	4.6

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