

Treatment of Duchenne's Muscular Dystrophy With Growth Hormone Inhibitors

Although major advances are being made in isolating the gene for Duchenne's muscular dystrophy, the basic mechanism of this disorder is still unknown. It may be several years before the function of the gene is understood. In the meantime, the disease continues its relentless deterioration process in affected males.

An interesting clinical observation reported by Zatz et al five years ago has led to some very important therapeutic implications. The report was about a boy with Duchenne's muscular dystrophy who also had growth hormone deficiency (GHD) and a relatively benign course. When compared with other affected individuals in his family, he was very much less severely affected. For this reason, Zatz et al undertook to utilize growth hormone (GH) antagonists in the treatment of Duchenne's muscular dystrophy. Specifically, she treated one of two identical

twins with the disease in a double-blind controlled study. The treatment involved the use of the GH antagonist mazindol. After one year of the therapeutic trial, the code was broken and the identical twin boys were compared. The twin being treated by GH antagonist was significantly less severely affected after a year of therapy than his brother, who had typical progression of his disease.

In the same issue of the *American Journal of Medical Genetics*, Zatz et al report the follow-up on the patient observed five years ago. The boy is still alive and functional at 18 years of age, while the other affected members of his family had already died or been non-ambulatory by the same age.

Zatz M, Betti RTB, Frota-Pessoa O. *Am J Med Gen* 1986;24:549-566, 567-572.

Editor's comment—*Duchenne's muscular dystrophy is one of the most common and distressing of single gene disorders. The basic mechanism of the disease has eluded definition, and until this report of Zatz et al, there has been no*

real hope for interrupting the progressive deterioration of affected boys. Advances in genetics are frequently made by experiments of nature and observation; the insight to appreciate that the boy who was affected with GHD was actually doing "well" with regard to his Duchenne's muscular dystrophy was extremely important. The observation that the identical twin who was treated with GH antagonist is significantly better than his non-treated twin raises the possibility that cell growth may have some role in Duchenne's muscular dystrophy. It may well be that smaller cells somehow do better and survive longer in the presence of the Duchenne's muscular dystrophy gene. It may be that boys with Duchenne's muscular dystrophy begin to do poorly with the increased turnover of cells and that GHs stimulate this turnover. Whatever the case, this important clinical observation may well lead to immediate treatment for Duchenne's muscular dystrophy. In addition, it is interesting to speculate whether GH antagonist may offer potential therapy in other degenerative diseases.