RESEARCH REPORTED IN NEW ENGLAND JOURNAL OF MEDICINE INDICATES GAUCHER DISEASE CAN BE TREATED MORE EFFECTIVELY, LESS EXPENSIVELY

LA JOLLA, CALIFORNIA Dec. 2, 1992 -- Investigators at The Scripps Research Institute (TSRI) in La Jolla, California have reported in this week's issue of the New England Journal of Medicine that Gaucher disease patients can be treated more effectively and less expensively than previously thought.

Alglucerase (Ceredase) is a form of treatment for Gaucher disease, a hereditary disorder that causes enlargement of the liver and spleen, anemia, bleeding and bone pain. This drug, which is manufactured by Genzyme Corporation of Boston, Massachusetts, has been designated "the world's most expensive medicine" by both the Wall Street Journal and the New York Times. Treatment for a 155 pound patient for one year at the dose recommended by Genzyme costs $380,000.

Now, a group of investigators headed by Ernest Beutler, M.D., TSRI's director of the Department of Molecular and Experimental Medicine, provides data supporting a low-dose, less expensive treatment schedule. "In an era when we need to make more judicious use of our health care resources, this is good news for patients and insurance companies," Beutler said.
Although Genzyme recommends that patients be treated with 60 units per kilogram once every two weeks, Beutler noted that "the medication disappears from the blood and tissues within a day. That's why we believed that more frequent administration of very small doses would be more effective. Now, there is proof that this strategy works."

Eleven patients were treated with the new low-dose, high-frequency schedule of Ceredase in the General Clinical Research Center of Scripps Clinic and Research Foundation. Their response to treatment, as reported in the New England Journal, was indistinguishable from that obtained when high doses were given every two weeks.

"And, the treatment costs less than a quarter as much," Beutler said.

A member of the National Academy of Sciences, Beutler is an internationally-known physician/scientist who was recently awarded the "Annual Award from the National Gaucher Disease Foundation" for his studies of low-dose Ceredase and of the molecular biology of Gaucher disease.

He stated that "both the team headed by Dr. Roscoe Brady at the National Institutes of Health and Genzyme Corporation deserve a great deal of credit for making this very effective new treatment available to patients with Gaucher disease. However, its availability to sufferers from this disease both here and abroad has been seriously compromised by its high cost."
"What is at issue here is the most effective way to administer the treatment, and it is unlikely that the administration of a drug which is gone from the body within 24 hours will be optimally effective if given only every two weeks," Beutler said.

"The results of the studies we have performed are good news for patients suffering from the disease who were denied access to the treatment because of its high cost," he added. "It is good news, too, for the insurance companies and for the health care system as a whole. We all recognize how inadequate the health care dollars are to meet the needs of the afflicted. The dosage schedule that we recommend will save hundreds of millions of dollars of health care resources."

"The only bad news is for Genzyme Corporation whose representatives have continued to promote the high dose/low frequency schedule to patients with Gaucher disease," Beutler said. "Their sales, which according to Newsweek magazine were $100 million in the first full year, will probably diminish as a result of this more rational use of Ceredase."

Beutler added that Genzyme currently suggests that the very high dosage they recommend can be halved every six months. "Even if this strategy is effective (and there are no published data that indicate that it is) the same scaling down of the high-frequency, low-dose program could be achieved. Thus, the cost of treatment will always be much higher than for the program that we have shown to be fully effective."
Although the more frequent administration of the drug advocated by Beutler is less convenient than Genzyme’s once every-two-weeks treatment, "the drug can be given safely at home by a nurse, family member or the patient, and this is how most of our patients receive the drug," he said.

Other members of the Beutler team included Michael Figueroa, M.D., now practicing in Redding, California; Andrea Kay, M.D., now assistant director, Medical Oncology/Immunology with Knoll Pharmaceutical Corporation; James Koziol, Ph.D., head of the Division of Biostatistics at TSRI; Terri Gelbart, who assisted Beutler with data analysis; Paul Garver, M.D. and Dennis Thurston, M.D., who performed the radiologic measurements at Scripps Clinic to document the findings; and Barry Rosenbloom, M.D., of Los Angeles.

Beutler noted that "the very costly studies that were performed were made possible by the resources of the NIH-sponsored General Clinical Research Center and the Sam and Rose Stein Foundation."

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