ENZYMEnE REPIJACEI,ENT TIIERAPY FOR GAUCHER DISEASE TO BE DISCUSSED AT MAJOR MEETING OF CLINICAL RESEARCHERS

LA JOLLA, CALIF. May 2, 1991 -- Recent research indicating the medical benefits of a more cost-effective therapy for Gaucher disease will be discussed by Andrea C. Kay, M.D. of Scripps Clinic and Research Foundation at the May 4 Clinical Meetings in Seattle, Washington.

The Clinical Meetings are an annual event attended by clinical researchers who belong to the Association of American Physicians, the American Society for Clinical Investigations, and the American Federation for Clinical Research.

Kay's presentation, titled "Enzyme Replacement Therapy in Type I Gaucher Disease," reports on successful clinical trials with six Scripps Clinic patients who have severe Gaucher disease. Kay was part of a team led by Ernest Beutler, M.D., head of Scripps Clinic's Department of Molecular and Experimental Medicine.

A lipid storage disease characterized by the deficiency of an enzyme required to degrade complex molecules in cells, Gaucher disease is particularly common among the Jewish population, but found in others, as well. It varies from a rapidly fatal disorder, with death occurring in the first year of life, to a disease so benign that it is diagnosed quite by accident. Treatment has been

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unsatisfactory, with the only cure achieved by bone marrow transplantation.

In the 1970s, American researchers sought to treat the disease with enzyme replacement therapy, but were unsuccessful largely due to the shortage of a commercially-available enzyme. Within recent years, an enzyme product was manufactured and found to be effective in treating Gaucher disease. However, the cost to administer the enzyme to a 154-pound patient would be about $350,000 a year.

The Scripps Clinic study investigated the efficacy of administering smaller doses of the enzyme on a more frequent basis. Beutler and Kay note that the patients responded well to the smaller doses, and the cost for treatment was decreased by more than 75 percent.

The Scripps Clinic study began in January 1990 with patients given one-fourth of the enzyme dose traditionally used. The enzyme was administered on alternate days or three times a week. Results included a decrease in the size of enlarged livers and spleens, as well as gradually improved liver function tests, peripheral blood counts, and enzyme levels.

In addition to Beutler and Kay, researchers included A. Saven, P. Garver, D. Thurston, A. Dawson and B. Rosenbloom. Studies were carried out in Scripps Clinic's General Clinical Research Center, which is funded by the National Institutes of Health. Additional funding was received from Sam and Rose Stein.

In her prepared remarks for the May 4 Clinical Meetings, Kay will estimate that at least 5,000 patients with Gaucher disease in the U.S. could benefit from treatment. "Even if many of these are children or small adults, the cost of enzyme alone for these patients would approach $1 billion per year..." at the current
therapeutic doses recommended by the enzyme manufacturer.

She will also note that the availability of enzyme therapy "provides Gaucher patients with a safe and effective therapy for the first time. It is incumbent upon us to determine treatment strategies that will be economically feasible as well as effective."

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