Medical Xpress

Enzyme therapy slows kidney function decline

April 8 2009

For men with Fabry disease, enzyme replacement therapy (ERT) with agalsidase alfa slows deterioration of kidney function, reports a study in the online edition of the *Journal of the American Society of Nephrology* (*JASN*). "The results provide further evidence that ERT with agalsidase alfa may slow the progression of kidney disease, provided that ERT is initiated early in the disease process," comments Michael L. West, MD (Dalhousie University, Canada).

The researchers pooled the results of three previous clinical trials of ERT with agalsidase alfa in 108 men with Fabry disease—a rare genetic disorder. Without treatment, Fabry disease causes progressive loss of kidney function, eventually leading to end-stage renal disease.

During treatment with an inactive placebo, kidney function declined rapidly. By comparison, during treatment with agalsidase alfa (1 to 4.5 years), the rate of decline slowed considerably. The response to treatment was not as good for patients with lower initial kidney function. "This underlines the importance of prompt diagnosis and intervention in patients with Fabry disease," adds Dr. West.

The study was one of the largest ever of men with Fabry disease and included the most accurate techniques of measuring kidney function. The study also had important limitations: it used data from different studies performed at different times; it represented a relatively narrow range of patient characteristics, specifically excluding children and women; and it lacked adequate data for full statistical analysis.

While not approved for use in the US, Agalsidase alfa is approved for use in over 40 countries including Canada, European countries, Argentina, Australia, and Japan. A related drug called agalsidase beta is approved in the US.

Dr. West has received research funding and other fees from Shire Human Genetic Therapies Inc. and other drug companies.

More information: The study entitled, "Agalsidase Alfa and Kidney Dysfunction in Fabry Disease," will appear online at jasn.asnjournals.org/ on April 8, 2009, doi 10.1681/ASN.2008080870.

Source: American Society of Nephrology (news : web)