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HORMONE MIMIC REDUCES LIVER DAMAGE CAUSED BY COMMON GENETIC KIDNEY DISEASE

Octreotide Provides Benefits for Both the Liver and Kidney in Patients with ADPKD

Washington, DC (April 26, 2010) — A hormone mimic called Octreotide may be effective for treating polycystic liver disease (PLD) caused by ADPKD, according to a study appearing in an upcoming issue of the *Journal of the American Society Nephrology* (JASN). The study is the first clinical trial performed in the United States to test the effects of this agent in PLD.

In addition to causing kidney failure, ADPKD also often leads to PLD, a condition characterized by multiple variably-sized cysts in the liver. Octreotide mimics the somatostatin hormone that regulates the secretion of several other hormones in the body. Somatostatin exerts its effects by blocking both the formation of the chemical cyclic AMP and the secretion of fluids by cells, two factors thought to play a role in the development of kidney and liver cystic diseases.

Marie Hogan, MD, PhD (Mayo Clinic College of Medicine) and her colleagues designed a clinical trial to examine whether Octreotide could shrink the cyst-filled livers of patients with PLD. The randomized, double-blind, placebo-controlled trial enrolled 42 patients with severe PLD caused by ADPKD (34 patients) or autosomal dominant PLD (8 patients). (Autosomal dominant PLD is a genetic form of PLD not caused by ADPKD.) Patients received Octreotide or placebo, and treatments were administered as monthly injections.

After one year, liver volume decreased by an average of approximately 5% in patients taking Octreotide but slightly increased (by approximately 1%) in patients taking placebo. Octreotide also had an effect on the diseased kidneys of patients with ADPKD. Among these patients, total kidney volume remained practically unchanged in the Octreotide group but increased by more than 8% on average in the placebo group. Kidney function was similar in both groups of patients. Octreotide was well tolerated, and treated individuals reported an improved perception of bodily pain and physical activity.

“In summary, Octreotide slowed the progressive increase in liver volume and total kidney volume, improved health perception among patients with PLD, and had an acceptable side effect profile,” said Dr. Hogan.

Study co-authors include Tetyana Masyuk, PhD, Linda Page, Vickie Kubly, Eric Bergstralh, Xujian Li, Bohyun Kim, MD, Bernard King, MD, James Glockner, MD, PhD, David Holmes III, PhD, Sandro Rossetti, MD, PhD, Peter Harris, PhD, Nicholas LaRusso, MD, and Vicente Torres, MD, PhD (Mayo Clinic College of Medicine).

In reviewing the results of Dr. Hogan's study in an accompanying editorial, Robert Schrier, MD (University of Colorado Denver) noted that they support the findings of earlier animal and human studies and give hope that increases in polycystic liver and kidney volume can be attenuated in patients with ADPKD. He added that additional, larger studies are needed to verify the results and to demonstrate whether Octreotide can provide meaningful health benefits to patients.

Disclosures: Funding support for the study was provided by Mayo Foundation for Medical Education and Research and Novartis USA. Dr. Hogan received partial funding support for this study from Novartis USA. Dr. LaRusso and Dr. Masyuk are named inventors on pending patent applications filed by Mayo Clinic claiming methods for using somatostatin analogs to treat polycystic liver disease. Dr. Schrier, the author of the editorial, is a consultant for Otsuka Pharmaceuticals.

The article, entitled "Randomized Clinical Trial of Long-Acting Somatostatin for Autosomal Dominant Polycystic Kidney and Liver Disease," (doi 10.1681/ASN.2009121291) and accompanying editorial, "Randomized Intervention Studies in Human Polycystic Kidney and Liver Disease," (doi 10.1681/ASN.2010030262) will appear online at <http://jasn.asnjournals.org/> on April 29, 2010.

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