



## **Interim Results From Independent Study of H.P. Acthar® Gel in Patients With Advanced Diabetic Nephropathy Presented at American Society of Nephrology 43rd Annual Meeting**

**--New data suggest Acthar may represent a novel pathway for the treatment of proteinuria due to diabetic nephropathy--**

**--Diabetic nephropathy represents the most common cause of end-stage renal disease in the U.S.--**

DENVER, Nov. 22, 2010 /PRNewswire/ -- On November 20 at the American Society of Nephrology (ASN) 43rd Annual Meeting, interim results presented from an ongoing, randomized prospective study conducted independently by James A. Tumlin, MD, at the University of Tennessee College of Medicine Chattanooga indicated that 9 of 15 patients (60%) with advanced diabetic nephropathy who are currently enrolled in the study and have completed or are still undergoing a six month course of treatment with H.P. Acthar® Gel (repository corticotropin injection) have exhibited a clinically significant decrease in proteinuria and achieved stabilized renal function. Excessive proteinuria, or urinary protein loss, is a known high risk factor for end-stage renal disease (ESRD).

Acthar is not currently indicated for the treatment of proteinuria associated with diabetic nephropathy.

"Our interim analysis suggests that Acthar may reduce proteinuria and stabilize renal function in patients with advanced diabetic nephropathy," stated Dr. Tumlin. "The use of Acthar could represent a novel pathway for the management of proteinuria associated with diabetic nephropathy. Given the very limited number of treatments available for these patients, further clinical evaluation of Acthar in patients with diabetic nephropathy is needed."

This is an exploratory, investigator-initiated study -- conducted independently by Dr. Tumlin but sponsored through a research grant from Questcor. The study currently has 15 patients enrolled. These patients have either completed or are still undergoing a six month course of Acthar therapy, and so far 9 have exhibited either a complete or partial response to therapy. In this study, a complete response was defined as a reduction of proteinuria to less than 300 mg per 24 hours. A partial response was defined as a greater than 50% reduction in proteinuria per 24 hours, with proteinuria remaining above 300 mg per 24 hours. All patients enrolled had either confirmed type I or II diabetes and >3000 mg proteinuria per 24 hours on angiotensin-converting enzyme inhibitors (ACE) alone or >2000 mg per 24 hours on combination ACE/angiotensin receptor blockers (ARB) or other protein lowering agent. Patients enrolled in the study receive daily Acthar injections for a six month treatment period.

Analysis of interim study results showed that in patients who responded to Acthar therapy proteinuria was reduced from an average of 7515 +/- 681 mg per 24 hours to an average of 2500 +/- 397 mg per 24 hours, an average decrease of 67%. Despite the study patients suffering from advanced diabetic nephropathy (mean glomerular filtration rate of 42.0 ml/min), serum creatinine, a key measure of kidney function, remained stable over the six month period (2.50 +/- 0.3 to 2.7 +/- 0.3 mg/dl). For patients responding to Acthar treatment, the reductions in proteinuria observed during the treatment period have typically persisted following the completion of the course of treatment, with follow-up periods extending up to six months post-treatment to date.

Despite having advanced diabetic nephropathy, none of the 15 enrolled study patients have progressed to ESRD during the course of the study or on follow-up. In contrast, over the same time period 10 of 25 (40%) case cohort control patients with advanced diabetic nephropathy followed by Dr. Tumlin have progressed to ESRD and have been initiated on hemodialysis.

"Diabetic nephropathy is the most common cause of end-stage renal disease and represents an enormous burden for the US healthcare system. Unfortunately, there are currently few treatment options available for these patients" said Don M. Bailey, President and CEO of Questcor Pharmaceuticals. "Interim results from this small, independent investigator-initiated study are encouraging and suggest that further exploration of Acthar in patients suffering from diabetic nephropathy is indeed warranted."

Diabetic nephropathy is typically defined by macroalbuminuria—that is, a urinary albumin excretion of more than 300 mg in a 24-hour collection—or macroalbuminuria and abnormal renal function as represented by an abnormality in serum creatinine, calculated creatinine clearance, or glomerular filtration rate (GFR). Clinically, diabetic nephropathy is characterized by a progressive increase in proteinuria and decline in GFR, hypertension, and a high risk of cardiovascular morbidity and mortality.

According to ASN estimates, more than 500,000 patients in the US suffer from ESRD and this figure is expected to grow by 50% over the next 20 years. While there are a number of different disorders of the kidney that can result in ESRD, 44% of the more than 100,000 new ESRD cases annually in the US are caused by diabetic nephropathy. Dialysis and kidney transplant

are the only treatment alternatives available for patients who progress to ESRD.

A brief abstract of Dr. Tumlin's study was submitted for consideration to ASN in June 2010. This abstract, available at [www.asn-online.org](http://www.asn-online.org), provides a portion of the data available for analysis at that point in time. Dr. Tumlin's poster presentation on November 20 provided an expanded set of data which became available as the study further progressed.

### **About H.P. Acthar® Gel**

H.P. Acthar® Gel is a natural adrenocorticotrophic hormone (ACTH) designed to provide a prolonged release after intramuscular or subcutaneous injection. Acthar is indicated for the treatment of acute exacerbations of multiple sclerosis in adults, and as monotherapy for the treatment of IS in infants and children under 2 years of age. It is also indicated to induce a diuresis or a remission of proteinuria in the nephrotic syndrome without uremia of the idiopathic type or that due to lupus erythematosus, and for the treatment of several other diseases and disorders. For more information, please visit [www.acthar.com](http://www.acthar.com).

### **About Questcor**

Questcor Pharmaceuticals, Inc. is a biopharmaceutical company whose products help patients with serious, difficult-to-treat medical conditions. Questcor markets H.P. Acthar® Gel (repository corticotropin injection), which is indicated for the treatment of acute exacerbations of multiple sclerosis in adults, and as monotherapy for the treatment of IS in infants and children under 2 years of age. It is also indicated to induce a diuresis or a remission of proteinuria in the nephrotic syndrome without uremia of the idiopathic type or that due to lupus erythematosus, and for the treatment of several other diseases and disorders.

Questcor also markets Doral® (quazepam), which is indicated for the treatment of insomnia characterized by difficulty in falling asleep, frequent nocturnal awakenings, and/or early morning awakenings. For more information, please visit [www.questcor.com](http://www.questcor.com).

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