



## **New Data on H.P. Acthar® Gel in the Treatment of Nephrotic Syndrome Presented at the American Society of Nephrology 43rd Annual Meeting**

**--Analysis included patients with advanced kidney disease who had previously failed multiple therapies--**  
**--Results in idiopathic membranous nephropathy, one of the more common forms of primary nephrotic syndrome, appear promising--**

DENVER, Nov. 22, 2010 /PRNewswire/ -- On November 20 at the American Society of Nephrology 43rd Annual Meeting, Columbia University Assistant Professor of Clinical Medicine Andrew Bomback, MD, presented results from a patient case series assessing the potential therapeutic value of H.P. Acthar® Gel (repository corticotropin injection) as a treatment for patients with nephrotic syndrome, a kidney disorder characterized by excessive loss of urinary protein. Initial data presented at the meeting revealed that 9 of 11 patients (82%) with nephrotic syndrome due to idiopathic membranous nephropathy (iMN) who were treated with Acthar achieved complete or partial remission of proteinuria. Nephrotic syndrome is a known risk factor for progression to end-stage renal disease (ESRD).

Overall, this initial assessment of Acthar in kidney disease included 24 patients with nephrotic syndrome of various etiologies, including the 11 patients with iMN. Over an average total follow-up period of 8 months, which included a typical Acthar treatment period of 6 months, 11 patients (46%) achieved a complete or partial remission, with four patients (17%) achieving complete remission. Among the 11 patients with iMN, three achieved complete remission (27%) and six achieved partial remission (55%), despite having previously failed an average of 2.4 other therapies. The analysis defined complete remission as stable or improved renal function with final proteinuria falling to <500 milligrams per day, and partial remission as stable or improved renal function with greater than or equal to 50% reduction in proteinuria and final proteinuria of 500-3500 milligrams per day.

"This represents the first modern clinical evaluation involving the use of Acthar for the treatment of nephrotic syndrome," said Dr. Bomback. "These early data suggest that Acthar may be a viable treatment option for resistant nephrotic syndrome due to idiopathic membranous nephropathy. We look forward to further study of this promising treatment option."

Acthar is 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. Acthar is also indicated for the treatment of exacerbations associated with multiple sclerosis in adults, was recently approved by the FDA as monotherapy for the treatment of infantile spasms in infants and children under 2 years of age, and is indicated for the treatment of several other diseases and disorders.

"This new data is encouraging, and demonstrates that Acthar is a potentially important treatment option for patients with nephrotic syndrome due to idiopathic membranous nephropathy. It is particularly noteworthy that many of these patients had previously failed to respond adequately to current treatment options, but they did respond to Acthar," said Don M. Bailey, President and CEO of Questcor Pharmaceuticals. "This initial clinical evaluation has reinforced our strong interest in fully understanding and maximizing the clinical benefits of Acthar in nephrotic syndrome. We are awaiting the availability of further clinical data from trials already in progress."

Nephrotic syndrome results from damage to the kidney glomeruli, tiny blood vessels that filter wastes and excess water from the blood and send them to the bladder as urine. It is characterized by excessive loss of protein in the urine, a condition known as proteinuria. This can be caused by a number of underlying diseases and disorders, including iMN, primary focal segmental glomerular sclerosis (FSGS) and other conditions. Based on epidemiology data and extensive market research with nephrologists, Questcor estimates that there are approximately 8,000 patients in the US with nephrotic syndrome due to iMN, and a similar number due to primary FSGS. There are also a number of other less common causes as well. Patients suffering from these types of idiopathic nephrotic syndrome often progress to ESRD if their kidney disease is not appropriately treated or if they do not respond adequately to treatment. 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. There are a number of disorders of the kidney that can result in ESRD and more than 100,000 new ESRD cases are diagnosed annually in the US. Dialysis and kidney transplant are the only treatment alternatives for patients with ESRD.

A brief abstract of Dr. Bomback's case series analysis 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. Bomback's free communication 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. The Company 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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