Clinical Trial Results Indicate Acthar May Significantly Reduce Proteinuria in Patients with Nephrotic Syndrome Due to Idiopathic Membranous Nephropathy

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- Clinical results appear to correlate with reduction in anti-PLA2R antibodies

ANAHEIM, Calif., April 21, 2014 /PRNewswire/ -- Questcor Pharmaceuticals, Inc. (Nasdaq: QCOR) today announced that results from an investigator-initiated clinical study involving two major academic research centers examining the dosing and effectiveness of H.P. Acthar® Gel (repository corticotropin) in 20 patients with nephrotic syndrome due to idiopathic membranous nephropathy (iMN) have been published in the journal *Nephrology Dialysis Transplantation.* The paper appears in the current online edition of the journal.

The full article can be accessed on the *Nephrology Dialysis Transplantation* website: http://ndt.oxfordjournals.org.

The results demonstrate that Acthar can be a potentially useful therapy for inducing remission of proteinuria in patients suffering from nephrotic syndrome secondary to iMN. The study also found that clearance of anti-PLA2R antibodies, which are an immunological marker of iMN disease activity, typically preceded or paralleled improvements in proteinuria as a result of Acthar treatment in patients with detectable antibody levels. Acthar was generally well tolerated by patients during the course of the study, with the most common side effects being mood changes, weight gain and transient insomnia. The study was conducted at the Mayo Clinic and the University of Toronto, with funding provided through a research grant from Questcor.

"This important independent academic study adds significantly to the growing body of clinical evidence for Acthar in the treatment of patients suffering from idiopathic types of nephrotic syndrome," said Steve Cartt, Chief Operating Officer of Questcor. "As a company, we continue to actively support both company-sponsored research and independent academic research evaluating Acthar in on-label indications such as the one studied in this trial, as well as in other autoimmune and inflammatory disorders having high unmet medical need."

About the Study Design
The study involved patients with biopsy proven iMN who were randomized 1:1 to receive Acthar at a dose of either 40 or 80 units administered twice weekly following an initial induction period. Changes in proteinuria, albumin, cholesterol profile, estimated glomerular filtration rate and serum anti-PLA2R antibodies were assessed at baseline and in response to treatment along with tolerance and safety.

Highlights of the Study Results
Baseline characteristics included mean proteinuria (9.1±3.4 g/day), albumin (2.7±0.8 g/dL), estimated glomerular filtration rate (77±30 ml/min) along with elevated total and low-density lipoprotein (LDL) cholesterol. By 12 months of follow-up, there was a significant improvement in proteinuria in the entire cohort, decreasing to 3.87±4.24 g/day ($p < 0.001$) with significant improvements in serum albumin ($p=0.001$), total cholesterol ($p < 0.001$) and LDL ($p=0.001$). A > 50% decrease in proteinuria was noted in 65% (13/20) of the patients with a trend towards better outcomes among patients who received greater cumulative doses.

About Nephrotic Syndrome
Nephrotic syndrome is a nonspecific kidney disorder characterized by a number of signs of disease including, but not limited to, proteinuria, hypoalbuminemia and edema. It is characterized by an increase in permeability of the capillary walls of the glomerulus leading to the presence of high levels of protein passing from the blood into the urine (proteinuria at least 3.5 grams per day per 1.73m^2 body surface area); low levels of protein in the blood (hypoproteinemia or hypoalbuminemia), ascites and in some cases, edema; high cholesterol (hyperlipidemia or hyperlipemia) and a predisposition for coagulation. If inadequately treated, nephrotic syndrome can lead to end stage renal disease and may require long-term dialysis or renal transplant in some patients.

About Questcor
Questcor Pharmaceuticals, Inc. is a biopharmaceutical company focused on the treatment of patients with serious, difficult-to-treat autoimmune and inflammatory disorders. Questcor also provides specialty contract manufacturing services to the global pharmaceutical industry through its wholly-owned subsidiary BioVectra Inc. For more information about Questcor, please visit www.questcor.com.

About H.P. Acthar® Gel
Questcor's H.P. Acthar Gel (repository corticotropin injection), is an injectable drug approved by the FDA for the treatment of 19 indications. Of these 19 indications, Questcor currently generates substantially all of its net sales from the following on-label indications: the treatment of proteinuria in the nephrotic syndrome of the idiopathic type, or NS, the treatment of acute exacerbations of multiple sclerosis, or MS, in adults, the treatment of infantile spasms, or IS, in infants and children under two years of age, and the treatment of certain rheumatology related conditions, including the treatment of the rare and closely related neuromuscular disorders dermatomyositis and polymyositis. With respect to nephrotic syndrome, the FDA has approved Acthar 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." Questcor is also exploring the possibility of developing other on-label indications and the possibility of pursuing FDA approval of additional indications not currently on the Acthar label where there is high unmet medical need. For more information about Questcor, please visit www.acthar.com.

Cautionary Statements Related to Forward-Looking Statements
Note: Except for the historical information contained herein, this press release contains forward-looking statements that have been made pursuant to the Private Securities Litigation Reform Act of 1995. These statements relate to future events or our future financial performance. In some cases, you can identify forward-looking statements by terminology such as "believes," "continue," "could," "ensuring," "estimates," "expects," "growth," "may," "momentum," "plans," "potential," "remain," "should," "start," "substantial," "sustainable" or "will" or the negative of such terms and other comparable terminology. These statements are only predictions. Actual events or results may differ materially. Factors that could cause or contribute to such differences include, but are not limited to, the following:

- Our reliance on Acthar for substantially all of our net sales and profits;
- The lack of patent protection for Acthar; and the possible FDA approval and market introduction of additional competitive products;
- Our ability to continue to generate revenue from sales of Acthar to treat on-label indications associated with NS, rheumatology-related conditions, MS, or IS, and our ability to develop other therapeutic uses for Acthar;
- Research and development risks, including risks associated with Questcor's work in the area of NS, and the ability of research and development to generate successful results; and
- Other risks discussed in Questcor's annual report on Form 10-K for the year ended December 31, 2013 as filed with the Securities and Exchange Commission, or SEC, on February 26, 2014, and other documents filed with the SEC.

The risk factors and other information contained in these documents should be considered in evaluating Questcor's prospects and future financial performance.

Questcor undertakes no obligation to publicly release the result of any revisions to these forward-looking statements, which may be made to reflect events or circumstances after the date of this release.

For more information, please visit www.questcor.com or www.acthar.com.

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