



Questcor to Commence Phase 2 Study of Acthar for ALS

July 24, 2013

ANAHEIM, Calif., July 24, 2013 /PRNewswire/ -- Questcor Pharmaceuticals, Inc. (NASDAQ: QCOR) announced today that patient screening will commence in connection with Questcor's Phase 2 Study to explore the safety and tolerability of H.P. Acthar® Gel (repository corticotropin injection) in patients with Amyotrophic Lateral Sclerosis (ALS). ALS -- often referred to as Lou Gehrig's disease -- is a progressive, degenerative disease affecting motor neurons. ALS is a serious debilitating disease that significantly alters one's quality of life. According to the ALS Association, the average life expectancy from the time of diagnosis for someone affected by this disorder is about two to five years.

The study will seek to enroll up to 40 patients in an 8-week randomized, open-label trial designed to explore the safety and tolerability of four dosing regimens of Acthar. Patients who successfully conclude the initial 8-week trial will then have the option to participate in a 28-week open-label extension with a 3-week taper and one week follow-up period. The study will also examine whether Acthar provides any functional improvement to ALS patients.

"Questcor has focused its expanding R&D efforts on devastating medical conditions for which patients are in need of new treatment options," said Dr. David Young, Questcor's Chief Scientific Officer. "We are continually understanding more about the mechanism of action of Acthar and how it might provide benefit to patients in need. Initial non-clinical research efforts provided us with data suggesting that Acthar may potentially provide therapeutic benefit to patients with ALS, leading us to conduct this important phase 2 study to further explore this possibility."

"In addition to this ALS trial, Questcor has on-going trials in idiopathic membranous nephropathy, systemic lupus erythematosus, and diabetic nephropathy," continued Dr. Young. "These trials, combined with many mechanism of action studies and numerous Questcor-sponsored investigator initiated studies, are aimed at building the body of evidence surrounding the safety and efficacy of Acthar in both on-label and potential new indications."

For more information on studies related to Acthar, investors are encouraged to visit <http://www.clinicaltrials.gov>.

About ALS

Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disease that affects nerve cells in the brain and the spinal cord. Motor neurons reach from the brain to the spinal cord and from the spinal cord to the muscles throughout the body. The progressive degeneration of the motor neurons in ALS eventually leads to their death. When the motor neurons die, the ability of the brain to initiate and control muscle movement is lost. With voluntary muscle action progressively affected, patients in the later stages of the disease may become totally paralyzed. The effect of this disease is not only a significant decrease in one's quality of life but also a decrease in life expectancy to an average of two to five years. At this time there is an unmet medical need for ALS treatments that significantly improve quality of life and/or extend survival.

ALS was first described in 1869 by French neurologist Jean-Martin Charcot, but it wasn't until 1939 that Lou Gehrig brought national and international attention to the disease when he abruptly retired from baseball after being diagnosed with ALS. Most commonly, the disease strikes people between the ages of 40 and 70, and as many as 30,000 Americans have the disease at any given time.

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. Questcor's primary product is H.P. Acthar® Gel (repository corticotropin injection), an injectable drug that is approved by the FDA for the treatment of 19 indications. Of its 19 FDA-approved 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 markets for 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 such as ALS. Questcor also has agreed to acquire certain international rights for Synacthen® (tetracosactide) and Synacthen Depot®, and has licensed the right to develop and seek FDA approval for these products in the United States. For more information about Questcor, please visit www.questcor.com. For more information about Acthar, including important safety information, please visit www.acthar.com.

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," "estimates," "expects," "growth," "intent," "may," "plans," "potential," "should," "substantial," "will," or "would" 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:

- Research and development risks, including risks associated with Questcor's work in the area of ALS;
- 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 competitive products;
- The results of any pending or future litigation, investigations or claims, including with respect to the investigation by the United States Attorney's Office for the Eastern District of Pennsylvania regarding the Company's promotional practices and litigation brought by certain shareholders arising from the federal securities laws, currently pending in the United States District Court for the Central District of California;
- Our ability to effectively manage our growth, including the expansion of our sales forces, and our reliance on key personnel; and
- Other risks discussed in Questcor's annual report on Form 10-K for the year ended December 31, 2012 as filed with the Securities and Exchange Commission, or SEC, on February 27, 2013, 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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