March 15, 2017

Catalyst Pharmaceuticals Announces Positive Data from Investigator-Sponsored Trial of Firdapse® in treating MuSK Antibody Positive Myasthenia Gravis

- *Catalyst intends to proceed to U.S. multi-center pivotal trial*
- *Expects additional data to be presented at upcoming medical congresses in 2017*

CORAL GABLES, Fla., March 15, 2017 (GLOBE NEWSWIRE) -- Catalyst Pharmaceuticals, Inc. (Catalyst) (Nasdaq:CPRX), a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare neuromuscular and neurological diseases, today announced positive top-line results from the investigator-sponsored trial evaluating Firdapse® (Amifampridine Phosphate) as a treatment for myasthenia gravis patients with anti-MuSK antibodies (MuSK-MG). MuSK-MG, is an ultra-rare sub-population of myasthenia Gravis (MG) patients which is a debilitating neuromuscular disease, and there are currently no FDA approved therapies for this specific form of MG. Both of the co-primary efficacy endpoints of change from baseline (CFB) in total Quantitative Myasthenia Gravis (QMG) score (p=0.0003) and CFB in total Myasthenia Gravis Activities of Daily Living (MG-ADL) score (p=0.0006) were statistically and clinically significant in this seven patient trial. Several secondary efficacy measures also achieved statistical significance. Amifampridine phosphate was well tolerated in this population of patients.

The study was conducted by a team of researchers led by Renato Mantegazza, MD, Director, Department of Neuroimmunology and Neuromuscular Diseases, Fondazione Istituto Neurologico Carlo Besta in Milan, Italy, a major referral center for MuSK-MG patients. The study was designed as a randomized (1:1), double-blind, placebo-controlled, crossover, outpatient study to evaluate the safety, tolerability and potential efficacy of amifampridine phosphate in patients diagnosed with MuSK-MG. Catalyst provided funding, study drug, and placebo for this trial.

Dr. Mantegazza, the principal investigator of this trial, stated, "Our prospective study evaluating amifampridine phosphate for the symptomatic relief of antibody positive MuSK-MG was statistically significant in demonstrating that it can be an important treatment option. Not only are the results statistically significant, but more importantly, there was a large clinical benefit to the patients. Current treatments for MuSK-MG patients are often inadequate and these patients often face a lifetime of severe complications, including difficulty walking, talking, swallowing, and breathing normally, and in some cases their disease may be life-threatening and require hospitalization and intensive care. Amifampridine phosphate may offer us an effective treatment option. I look forward to the day when I can use this drug in routine clinical practice of treating MuSK-MG patients."

Dr. Silvia Bonanno, one of the investigators from the Istituto Neurologico Carlo Besta, is planning to present these results at the 13th International Conference on Myasthenia Gravis and Related Disorders in May, 2017 in New York City, provided her abstract is accepted as a Hot Topic Short Talk. This conference is organized by the Myasthenia Gravis Foundation of America and the New York Academy of Sciences.

"These data announced today should allow us to accelerate our MuSK-MG program over the coming months, as we expect to consult with our external experts and regulatory agencies on a pivotal clinical development plan," said Patrick J. McEnany, Catalyst's Chief Executive Officer. "I would like to thank Dr. Mantegazza, his associates at Carlo Besta Neurological Institute, and the patients that participated in this important clinical trial."

"While several effective treatment options exist for the anti-acetylcholine receptor form of myasthenia gravis (AcHR-MG), MuSK-MG has been particularly refractory to current MG treatment options and represents an unmet medical need in the MG community of patients," stated Gary Ingenito MD, Ph.D., Catalyst's Chief Medical Officer. Dr. Ingenito continued: "If the significant clinical effect observed in this trial is reproduced in a multicenter trial, amifampridine phosphate would, upon approval, likely become the first line standard of care for MuSK-MG. Based on these results we intend to discuss with FDA conducting a registration trial in the United States evaluating amifampridine phosphate for the symptomatic treatment of patients with MuSK-MG."

About the Clinical Trial

The MuSK-MG "proof-of-concept" trial was a randomized, double blind, placebo-controlled, single site, outpatient, investigator-sponsored, clinical trial to evaluate the safety and efficacy of amifampridine phosphate in myasthenia gravis patients with a positive serological test for the anti-MuSK antibodies (MuSK-MG). Catalyst provided the investigational drug, placebo, and funding for this clinical trial.

Patients were enrolled into the trial and were titrated to an effective dose of amifampridine phosphate for a period of at least 4 weeks in a "run-in" phase of the trial. Following achievement of a dosage that effectively managed the patient's symptoms, the patients were randomized 1:1 into one of two crossover treatment groups. There were three treatment periods in this crossover design, referred to as a "switch-back" crossover design, that enables both the determination of the effect of the treatment as well as the correction of any "subjective carryover" from earlier treatment periods into later treatment periods. Carryover in crossover designs is a common concern of regulatory agencies, and this design effectively corrects for and eliminates the effects of carryover from the efficacy assessments. Each treatment period lasted 1 week for a total of treatment duration of 3 weeks alternating with either amifampridine phosphate or placebo. The co-primary efficacy endpoints of change from base (CFB) in total Quantitative Myasthenia Gravis (QMG) score and CFB in total Myasthenia Gravis Activities of Daily Living (MG-ADL) score at the 7th day of each period were assessed using a mixed carryover effects statistical model of Kunert and Stufken. Secondary endpoints consisted of, in order, CFB in Myasthenia Gravis Composite (MGC) Score, CFB in Neurological Institute Carlo Besta-Myasthenia Gravis (NICB-MG) score, proportion of patients with a 2, or larger, point reduction in MG-ADL, proportion of patients with a 3, or larger, reduction in MGC, CFB in Myasthenia Gravis Quality of Life, 15 domain, (MG-QoL 15) score, and CFB in Fatigue Severity scale (FSS). Catalyst's funding of this study included the use of a CRO, and this study was run as a well-controlled trial suitable for regulatory submission.

About MuSK Myasthenia Gravis

About 15% of MG patients test negative for the acetylcholine receptor antibody. These patients have seronegative (SN) MG. Approximately 40-50% of these patients with SNMG (equating to an estimate of approximately 4,500 patients in the United States) test positive for the anti-MuSK antibody. MuSK is a protein that is required for the maintenance of the neuromuscular junction and patients with the anti-MuSK antibody are identified as having MuSK-MG. MuSK-MG is a clinically distinguishable, more severe form of MG. The disease is characterized by a predominance in females, a prevalent involvement of cranial and bulbar muscles, high incidence of respiratory crises and a resistance to treatment. Although many patients with MuSK MG are presently treated with anticholinesterase inhibitors or immunosuppressants, such patients do not generally respond adequately to these treatments.

About Catalyst Pharmaceuticals

Catalyst Pharmaceuticals is a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases, including Lambert-Eaton myasthenic syndrome (LEMS), congenital myasthenic syndromes (CMS), MuSK myasthenia gravis and infantile spasms. Firdapse® has received Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) for the treatment of LEMS and Orphan Drug Designation for LEMS, CMS and myasthenia gravis. Firdapse is the first and only approved drug in Europe for symptomatic treatment in adults with LEMS.

Catalyst is also developing CPP-115 to treat refractory infantile spasms and possibly refractory Tourette's Disorder. CPP-115 has been granted U.S. Orphan Drug Designation for the treatment of infantile spasms by the FDA and has been granted E.U. Orphan Medicinal Product Designation for the treatment of West syndrome by the European Commission. In addition, Catalyst is developing a generic version of Sabril® (vigabatrin).

Forward-Looking Statements

This press release contains forward-looking statements. Forward-looking statements involve known and unknown risks and uncertainties, which may cause Catalyst's actual results in future periods to differ materially from forecasted results. A number of factors, including whether the receipt of breakthrough therapy designation for Firdapse will expedite the development and review of Firdapse by the FDA or the likelihood that the product will be found to be safe and effective, the timing of Catalyst's second trial evaluating Firdapse for the treatment of LEMS and whether the trial will be successful, whether Catalyst's assumptions in its updated business plan will be accurate and the impact of unanticipated events or delays in projected activities on Catalyst's cash requirements and on Catalyst's ability to get to an accepted NDA submission for Firdapse without the need for additional funding, what clinical trials and studies will be required before Catalyst can resubmit an NDA for Firdapse for the treatment of CMS and whether any such required clinical trials and studies will be successful, whether any NDA for Firdapse resubmitted to the FDA will ever be accepted for filing, the timing of any such NDA filing or acceptance, whether, if an NDA for Firdapse is accepted for filing, such NDA will be given a priority review by the FDA, whether any future trial evaluating Firdapse for the treatment of MuSK-MG will be successful and whether Catalyst can obtain the funding required to conduct such trial, whether Firdapse will ever be approved for commercialization, whether Catalyst will be the first company to receive approval for amifampridine (3,4-DAP), giving it 5-year marketing exclusivity for its product, whether CPP-115 will be determined to be safe for humans, what additional testing will be required before CPP-115 is "Phase 2 ready", whether CPP-115 will be determined to be effective for the treatment of refractory infantile spasms or possibly Tourette's Disorder, or for any other indications, whether Catalyst can successfully design and complete a bioequivalence study of its version of vigabatrin compared to Sabril that is acceptable to the FDA, whether any such bioequivalence study the design of which is acceptable to the FDA will be successful, whether any ANDA that Catalyst

submits for a generic version of Sabril will be accepted for filing, whether any ANDA for Sabril accepted for filing by the FDA will be approved (and the timing of any such approval), whether any of Catalyst's product candidates will ever be approved for commercialization or successfully commercialized, and those other factors described in Catalyst's Annual Report on Form 10-K for the fiscal year 2015 and its other filings with the U.S. Securities and Exchange Commission (SEC), could adversely affect Catalyst. Copies of Catalyst's filings with the SEC are available from the SEC, may be found on Catalyst's website, or may be obtained upon request from Catalyst. Catalyst does not undertake any obligation to update the information contained herein, which speaks only as of this date.

Investor Contact

Brian Korb

The Trout Group LLC

(646) 378-2923

bkorb@troutgroup.com

Media Contacts

David Schull

Russo Partners

(212) 845-4271

david.schull@russopartnersllc.com

Company Contact

Patrick J. McEnany

Catalyst Pharmaceuticals

Chief Executive Officer

(305) 529-2522

pmcenany@catalystpharma.com

Primary Logo

Source: Catalyst Pharmaceuticals, Inc.

News Provided by Acquire Media