December 8, 2016

Catalyst Pharmaceuticals Provides Update on its Clinical Trial for Patients with Congenital Myasthenic Syndromes

- *Allowing adult CMS patients to participate*
- *Number of patients increased to approximately 20*
- *Additional site added*

CORAL GABLES, Fla., Dec. 08, 2016 (GLOBE NEWSWIRE) -- Catalyst Pharmaceuticals, Inc. (Catalyst) (Nasdaq:CPRX), a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases, today provided an update on its clinical trial with amifampridine phosphate in patients with congenital myasthenic syndromes (CMS). After discussions with the U.S. Food and Drug Administration (FDA) the study has been expanded beyond pediatric patients to include adult CMS patients and the enrollment size has been increased to approximately 20 patients. Further, there are now a total of five sites participating in the study:

- Children's Healthcare of Atlanta
- Johns Hopkins Pediatric Neurology
- Boston Children's Hospital
- Nationwide Children's Hospital
- University of California, Los Angeles, Department of Neurology

Amifampridine phosphate, Firdapse®, has received Breakthrough Therapy Designation from the FDA for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), as well as orphan drug designations for LEMS, CMS, and Myasthenia Gravis.

Patrick J. McEnany, Catalyst's Chief Executive Officer said, "We are pleased to enhance and expand our clinical study to include adults with CMS, as we evaluate the efficacy and safety of amifampridine phosphate in patients of all ages diagnosed with certain genetic mutations of CMS. Assuming the data from our study is positive, we will work towards including data and information on the benefits of amifampridine phosphate for CMS in our new drug application that we plan to submit for Firdapse. Additionally, we also expect to include in a new submission the positive results seen to date in children with CMS who are currently being treated with amifampridine phosphate under an investigator treatment IND. We continue to believe that we will report top-line results from this study in the second half of 2017."

Dr. Gary Ingenito, Ph.D., Catalyst's Chief Medical Officer said, "Though CMS is primarily diagnosed in infancy and childhood, adult patients who have been diagnosed with other neuromuscular diseases have been found to have CMS; thus, we are pleased to add this population to our clinical study. Genetically-confirmed CMS patients older than two years of age diagnosed with acetylcholine receptor defect, Rapsyn deficiency, MuSK deficiency, Dok-7 deficiency, SYT2 mutations, SNAP25B deficiency, and fast channel syndrome may be eligible for this study. Genetic testing will be provided to potential participants whose CMS has not been genetically-confirmed within an eligible diagnosis."

Additional information about this trial (NCT02562066) can be found on www.clinicaltrials.gov.

About Congenital Myasthenic Syndromes

Congenital myasthenic syndromes, or CMS, are rare neuromuscular disorders comprising a spectrum of genetic defects and is characterized by fatigable weakness of skeletal muscles with usual onset at or shortly after birth or early childhood; in rare cases symptoms may not manifest themselves until later in childhood or adulthood. The severity and course of the disease are variable, ranging from minor symptoms to progressive disabling weakness; symptoms may be mild, but sudden severe exacerbations of weakness or even sudden episodes of respiratory insufficiency also occur.

Congenital myasthenic syndromes are rare, estimated at one-tenth that of myasthenia gravis, which in itself is rare. Based on currently available information, Catalyst estimates that there are between 1,000 and 1,500 CMS patients in the United States.

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), infantile spasms, and Tourette's Disorder. Firdapse for the treatment of LEMS has received Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) 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 infantile spasms, epilepsy and other neurological conditions associated with reduced GABAergic signaling, like post-traumatic stress disorder and 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 clinical 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 the investigator-sponsored study evaluating Firdapse for the treatment of MuSK-MG 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 Firdapse will ever be approved for commercialization, whether Catalyst will be the first company to receive approval for amifampridine (3,4-DAP), giving it 7-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 infantile spasms, post-traumatic stress disorder, Tourette's Disorder or 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.

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