October 31, 2017

Catalyst Pharmaceuticals Completes Enrollment in Second Phase 3 Trial of Firdapse® in Patients with Lambert-Eaton Myasthenic Syndrome

-- ***Top-Line Results Expected in Early December*** --

-- ***Required Abuse Liability Studies Completed*** --

CORAL GABLES, Fla., Oct. 31, 2017 (GLOBE NEWSWIRE) -- Catalyst Pharmaceuticals, Inc. (Nasdaq:CPRX), a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating, chronic neuromuscular and neurological diseases, today announced the completion of enrollment in the LMS-003 Phase 3 trial evaluating Firdapse® (amifampridine phosphate) in patients with Lambert-Eaton Myasthenic Syndrome (LEMS).

"Completion of enrollment in LMS-003 marks a significant milestone for Catalyst as well as the LEMS community as we take another step toward advancing treatment for LEMS patients," commented Patrick J. McEnany, Chief Executive Officer of Catalyst. "We would like to express our thanks to the dedicated investigators and clinical site coordinators who are conducting the trial and also patients with LEMS and their caregivers who are participating. We look forward to reporting top-line results in early December."

As previously reported, Catalyst is also conducting preclinical abuse liability studies required by the FDA's guidance for "Assessment of Abuse Potential of Drugs" that was finalized in January 2017. Catalyst has completed all three studies for Self-Administration, Physical Dependence, and Drug Discrimination. Top-line results indicate that amifampridine phosphate does not exhibit abuse potential in these assessment models. Catalyst remains confident that no further preclinical or clinical studies for abuse liability will be required.

Catalyst's second Phase 3 trial, LMS-003, is a double-blind, placebo controlled withdrawal trial, which enrolled a total of 26 subjects. The co-primary endpoints remain the same as Catalyst's first Phase 3 trial evaluating Firdapse for the treatment of LEMS, including change from Baseline Quantitative Myasthenia Gravis (QMG) score, and change in Subject Global Impression (SGI) score.

Catalyst is also developing Firdapse for the treatment of MuSK antibody positive myasthenia gravis (MuSK-MG) and congenital myasthenic syndromes (CMS).

About Catalyst Pharmaceuticals

Catalyst Pharmaceuticals is a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating, chronic neuromuscular and neurological diseases, including Lambert-Eaton myasthenic syndrome (LEMS), congenital myasthenic syndromes (CMS), MuSK antibody positive 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 any additional abuse liability studies of Firdapse will be required by the FDA before Catalyst can resubmit an NDA for Firdapse, whether Catalyst's assumptions as to the availability of funding to meet its anticipated working capital

requirements in future periods will be accurate and the impact of unanticipated events or delays in projected activities on Catalyst's cash requirements, 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 Catalyst can successfully complete a registration trial evaluating Firdapse for the treatment of MuSK-MG that is acceptable to the FDA, whether any such future trial evaluating Firdapse for the treatment of MuSK-MG will be successful, whether Catalyst can obtain the funding required to conduct such a 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 2016 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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