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Catalyst Pharmaceuticals Announces FDA Orphan Drug Designation of Firdapse for the Treatment of Myasthenia Gravis

CORAL GABLES, Fla., Sept. 02, 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 announced that the U.S. Food and Drug Administration (FDA) has granted the company orphan drug designation for Firdapse® (amifampridine phosphate) for the treatment of myasthenia gravis.

Myasthenia Gravis caused by antibodies to the muscle-specific kinase (MuSK-MG) is a rare disease that is estimated to inflict 5-8% of all myasthenia gravis patients (equating to an estimate of approximately 4,500 patients in the United States). MuSK antibodies identify a clinically distinguishable, more severe form of MG. The disease is characterized by a predominance in females, prominent bulbar involvement, more severe clinical condition and 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.

"We are pleased that the FDA has granted Orphan Drug designation to Firdapse for myasthenia gravis, as it provides Catalyst with a number of benefits through development and commercialization," stated Patrick J. McEnany, Chief Executive Officer of Catalyst. He continued, "We are currently supporting an investigator-sponsored, randomized, double-blind, placebo controlled study evaluating Firdapse for the treatment of patients with MuSK-MG, and we anticipate the investigator reporting top-line results from this study in early 2017. If this trial is successful, and subject to the availability of funding, we hope to initiate a registration quality trial in the U.S. evaluating Firdapse for the treatment of patients with MuSK-MG."

About Orphan Drug Designation

Orphan Drug designation is granted by the FDA's Office of Orphan Products Development for drugs that are expected to provide significant therapeutic advantage over existing treatments and that target conditions affecting 200,000 or fewer U.S. patients annually. Orphan Drug designation qualifies a company for several benefits under the Orphan Drug Act of 1983. The benefits apply across all stages of drug development and include an accelerated approval process; seven years of market exclusivity following marketing approval; tax credits on U.S. clinical trials; eligibility for Orphan Drug grants; and waiver of Prescription Drug User Fee Act (PDUFA) and certain other administrative fees.

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, what study design for a second trial evaluation of Firdapse for the treatment of LEMS will be acceptable to the FDA, the timing of such trial, and whether it 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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