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Catalyst Pharmaceuticals Receives Special Protocol Assessment (SPA) from the FDA for Second Phase 3 Clinical Trial Evaluating Firdapse for the Treatment of LEMS

CORAL GABLES, Fla., Oct. 31, 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, announced today that it has reached an agreement with the U.S. Food and Drug Administration (FDA) under a Special Protocol Assessment (SPA) for the protocol design, clinical endpoints, and statistical analysis approach to be taken in Catalyst's upcoming Phase 3 study evaluating Firdapse® (amifampridine phosphate) for the symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS).

A SPA is a process by which sponsors ask the FDA to evaluate the protocol of a proposed clinical trial to determine whether it adequately addresses scientific and regulatory requirements for the purpose identified by the sponsor. A SPA agreement indicates concurrence with the adequacy and acceptability of specific critical elements of protocol design, endpoints and analysis. Additionally, it provides a binding agreement with FDA's review division that a pivotal trial design, conduct, and planned analysis adequately addresses the scientific and regulatory objectives in support of a regulatory submission for drug approval. However, final marketing approval depends upon the results of efficacy, the safety profile, and an evaluation of the risk/benefit of treatment demonstrated in the Phase 3 clinical trial, among other requirements. For further information regarding the SPA process, please visit the FDA website at www.fda.gov.

"Receipt of this SPA agreement is a major milestone which provides us with a clearly defined development and regulatory pathway for Firdapse in the treatment of LEMS," said Patrick J. McEnany, Catalyst's Chief Executive Officer. "We would like to thank the FDA for its engagement and guidance in this process."

Catalyst intends to conduct its second Phase 3 trial (designated as LMS-003) at two clinical trial sites, one on the east coast of the United States and one on the west coast of the United States. This double-blind, placebo controlled withdrawal trial will include approximately 28 subjects, so that the trial is adequately powered, and will have the same co-primary endpoints as Catalyst's first Phase 3 trial evaluating Firdapse for the treatment of LEMS. Further, the FDA has agreed to allow Catalyst to enroll patients from its expanded access program as study subjects in this second trial. Finally, after further discussion with the FDA in connection with the SPA request, this second trial will be a parallel design and not a cross-over design.

Final details of the Phase 3 clinical trial will be available at the launch of the study on www.clinicaltrials.gov. As previously reported, Catalyst expects to initiate this trial before the end of this year.

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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