December 16, 2015

Catalyst Pharmaceuticals Announces Top-Line Results in Phase 1(b) Trial of CPP-115

CORAL GABLES, Fla., Dec. 16, 2015 (GLOBE NEWSWIRE) -- **Catalyst Pharmaceuticals, Inc.** (Nasdaq:CPRX), a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases, today announced top line results from its Phase 1(b) double-blind, placebo controlled safety and tolerance study of CPP-115 in normal healthy volunteers.Â The results showed significant increases in brain levels of the surrogate marker for potential efficacy, gamma-aminobutyric acid (GABA).Â The main adverse effect of prolonged elevated brain GABA, somnolence, was also observed.

While the primary objective of this study was to obtain safety and tolerance data for CPP-115 administered over 14 days, brain GABA levels were measured as a surrogate marker of potential efficacy, since CPP-115 is a second generation GABA aminotransferase inhibitor.Â Specifically, this study examined GABA levels in both the POC (Parietal-Occipital Cortex) a grey matter rich region thought to be associated with epilepsy, and which was previously studied for vigabatrin, and the SMA (Supplementary Motor Area), which is thought to be associated with Tourette's Disorder.Â The maximum brain GABA increases, in both brain regions, ranged from about 150% to over 200% of baseline levels, as measured by magnetic resonance spectroscopy (MRS).

Patrick J. McEnany, Catalyst's Chief Executive Officer stated, "These data are promising, because they provide evidence that CPP-115 significantly raises brain GABA, a mechanism known to effectively treat epilepsy, infantile spams, and potentially Tourette's disorder.Â CPP-115 was also found to be even more potent than anticipated, which may indicate that a lower dose of CPP-115 has the potential to effectively treat these diseases. Â A low drug dose may reduce or eliminate the risk of visual field defects, a side effect associated with vigabatrin."

Steven Miller, Ph.D., Catalyst's Chief Operating Officer and Chief Scientific Officer stated, "We expect to receive the full results of this study early next year, including additional data from laboratory safety tests, pharmacokinetic modeling, and brain levels of CPP-115 as determined from MRS data of the patients in this study. Once we obtain the full results of this study, we intend to determine the steps required to make CPP-115 "Phase 2 ready," including a Phase 1 dose ranging study evaluating CPP-115 at lower doses."

AboutÂ Catalyst Pharmaceuticals

Catalyst PharmaceuticalsÂ 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. Catalyst's lead candidate, Firdapse for the treatment of LEMS, recently completed testing in a global, multi-center, double-blinded randomized pivotal Phase 3 trial resulting in positive top-line data on both co-primary endpoints. Firdapse for the treatment of LEMS has received Breakthrough Therapy Designation from theÂ U.S. Food and Drug AdministrationÂ (FDA) and Orphan Drug designations for LEMS and CMS. Firdapse is the first and only European approved drug 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 theÂ FDAÂ and has been granted E.U. orphan medicinal product designation for the treatment of West Syndrome by theÂ European Commission. In addition, Catalyst is developing a generic version of Sabril® (vigabatrin).***Â***

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 breakthroughÂ therapy designation forÂ FirdapseÂ will expedite the development and review ofÂ FirdapseÂ by theÂ FDAÂ or the likelihood that the product will be found to be safe and effective,Â what clinical trials and studies will be required before Catalyst can submit an NDA for Firdapse for the treatment of CMS and whether any such required clinical trials and studies will be successful,Â whether an NDA forÂ FirdapseÂ will ever be accepted for filing by theÂ FDA, 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 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 spasm, 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 files 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 2014 and its other filings with theÂ U.S. Securities and Exchange CommissionÂ (SEC), could adversely affect Catalyst. Copies of Catalyst's filings with the SECÂ are available from theÂ 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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