August 30, 2017

Catalyst Pharmaceuticals Provides Update on the Status of its Firdapse Development Activities

-- FDA grants Special Protocol Assessment agreement for Phase 3 MuSK-MG trial --

-- Enrollment for second Phase 3 trial for LEMS nearing completion --

-- Expected NDA submission for Firdapse moves from end of 2017 to first quarter 2018 --

CORAL GABLES, Fla., Aug. 30, 2017 (GLOBE NEWSWIRE) -- Catalyst Pharmaceuticals, Inc. (Catalyst) (Nasdaq:CPRX), a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating neuromuscular and neurological diseases, today provided an update on the status of its Firdapse® (amifampridine phosphate) development activities.

Patrick J. McEnany, Chief Executive Officer of Catalyst, stated: "We are pleased to report that we have reached an agreement with the FDA under a Special Protocol Assessment (SPA) for the trial design, clinical endpoints and statistical analysis approach for our Phase 3 clinical trial evaluating Firdapse for the treatment of patients with MuSK antibody positive Myasthenia Gravis (MuSK-MG). We appreciate the FDA's engagement and guidance during the SPA process, and we look forward to dosing the first patients in this trial in early 2018."

Mr. McEnany continued, "Over the last few weeks, our management team has carefully examined the timelines for our development plans for Firdapse, including all necessary steps that are required for us to submit a complete new drug application (NDA) for Firdapse for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS). Those efforts have led us to slightly refine our timeline for the planned submission to the FDA of our NDA, which we now expect will be submitted in the first quarter of 2018. Previously we had reported that we expected to submit our NDA by the end of this year."

Dr. Steven Miller, Catalyst's Chief Operating Officer and Chief Scientific Officer added, "The additional short term preclinical studies that the FDA required us to complete before we could submit another NDA for amifampridine phosphate are abuse liability studies that the FDA and Controlled Substance Staff require for the evaluation of the potential effect of any new chemical entities on behaviors relating to abuse liability and dependence/withdrawal effects. Our discussions with the FDA regarding the protocols for these studies took longer than initially expected and, as a result, we now expect to have the final report in December."

Second Phase 3 clinical trial (LMS-003) evaluating Firdapse for the treatment of LEMS

Catalyst expects to complete enrollment shortly and to announce top-line results from its confirmatory Phase 3 study (LMS-003) in the fourth quarter of this year.

Preclinical Abuse Liability Studies

Catalyst is conducting three 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 the studies for Self-Administration and Physical Dependence, and has obtained top-line results indicating that amifampridine phosphate does not exhibit abuse liability in these two assessment models. The third study for Drug Discrimination is currently ongoing and is expected to be completed in the fourth quarter of this year. Catalyst remains confident that all three studies will indicate that amifampridine phosphate does not have any abuse liability and that no further preclinical or clinical studies for abuse liability will be required, although there can be no assurance.

Submission of NDA for Firdapse for LEMS

Catalyst intends to request a confirmatory pre-NDA meeting with the FDA to discuss the proposed NDA filing package. Since the briefing package to the FDA, to be submitted prior to this meeting, needs to include results from the LMS-003 trial and findings from the three abuse liability studies, Catalyst believes that any pre-NDA meeting that may be granted will not take place until December 2017 or January 2018. As a result, Catalyst has concluded that the NDA submission for Firdapse for LEMS is likely to be submitted (assuming the results of its studies are successful) in the first quarter of 2018 rather than before the end of 2017 as previously reported.

Registration trial evaluating Firdapse for the treatment of MuSK-MG

Catalyst has just recently received agreement from the FDA on a special protocol assessment (SPA) for a study evaluating the safety and efficacy of amifampridine phosphate treatment in patients with MuSK antibody positive myasthenia gravis (MuSK-MG). This is a particularly severe form of myasthenia gravis that affects about 3,000 to 4,800 patients in the US, for which there are no approved effective therapies and is therefore an unmet medical need. The protocol that the FDA has reviewed is for a multi-site, international (US and Italy), double-blind, placebo-controlled, clinical trial that is targeted to enroll about 60 subjects diagnosed with MuSK-MG. The trial will employ a primary endpoint of Myasthenia Gravis Activities of Daily Living (MG-ADL) and a secondary endpoint of Quantitative Myasthenia Gravis Score (QMG). At the FDA's request, the trial will also enroll up to 10 generalized myasthenia gravis patients who will be assessed with the same clinical endpoints, but achieving statistical significance in this subgroup of patients is not required and only summary statistics will be provided. Catalyst anticipates that enrollment in this trial will commence in the first quarter of 2018, and that it will take about 12 months to complete the enrollment for the trial.

Phase 3 clinical trial (CMS-001) evaluating Firdapse for the treatment of Congenital Myasthenic Syndromes (CMS)

Catalyst recently reported that it expects to report top-line data from its ongoing clinical trial evaluating Firdapse for the treatment of CMS in the first half of 2018, and Catalyst continues to believe that this timeline remains accurate. Catalyst also hopes to include in any NDA that it submits for Firdapse for LEMS those limited types of CMS that are generally considered mechanistically similar to LEMS. Catalyst intends to confirm with the FDA at any pre-NDA meeting that it may be granted, that any such inclusion will not slow down the FDA's review of a submitted NDA for Firdapse for LEMS.

Development of commercial plan for Firdapse

As previously reported, Catalyst intends to recommence its efforts to refine and begin implementation of its commercialization plan for Firdapse before the end of this year.

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Detailed information about each of Catalyst's ongoing studies and trials can be found in the Company's filings with the Securities and Exchange Commission, including its Annual Report on Form 10-K for 2016 and its Quarterly Report on Form 10-Q for the period ended June 30, 2017.

About Special Protocol Assessment

A Special Protocol Assessment (SPA) is a process that allows the sponsor of a clinical trial to submit their proposed trial design (clinical trial protocol and also the statistical analysis plan, if desired) to the FDA for review and concurrence prior to initiating the clinical trial. The FDA responds to these submissions within 45 days either with agreement, or with disagreement, and may provide written comments in either case. Should the FDA disagree, a new submission with revised documents would be necessary in order to reach agreement, and meetings with the FDA prior to any SPA submission may be necessary in some cases. As stated in the FDA's "Guidance for Industry: Special Protocol Assessment, ...a special protocol assessment documents our agreement that the design and planned analysis of a study can adequately address objectives in support of a regulatory submission. However, final determinations for marketing application approval are made after a complete review of a marketing application and are based on the entire data in the application." Therefore, the possibility exists that the Agency may determine that a clinical trial is inadequate at a later date in spite of having agreed to it in the past.

About Catalyst Pharmaceuticals

Catalyst Pharmaceuticals is a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating 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 that trial will be successful, 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, 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 has sufficient funding to conduct such a trial, whether Firdapse will ever be approved for commercialization and successfully commercialized, whether Catalyst will be the first company to receive approval for amifampridine (3,4-DAP), giving it 5-year marketing exclusivity for its product, 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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