November 27, 2017

Catalyst Pharmaceuticals Announces Positive Top-Line Results from Second Phase 3 Clinical Trial of Firdapse® in Patients with Lambert-Eaton Myasthenic Syndrome

-- Treatment with Firdapse Achieved Statistical Significance for Co-Primary Endpoints and Secondary Endpoint --

-- Positive Results to Support Planned NDA Submission in the First Quarter of 2018 --

-- Conference Call Today At 8:30 am ET --

CORAL GABLES, Fla., Nov. 27, 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 positive top-line results from a second Phase 3 clinical trial (LMS-003) of Firdapse® (amifampridine phosphate tablets equivalent to 10 mg amifampridine) for the symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS). Firdapse received Breakthrough Therapy designation from the FDA in August 2013 and this clinical trial was conducted using a protocol agreed to by the FDA through the Special Protocol Assessment (SPA) process.

This trial had two prospectively defined co-primary endpoints. The first of these, quantitative myasthenia gravis score (QMG), achieved a statistically significant p-value of 0.0004, and the second, subject global impression (SGI), achieved a statistically significant p-value of 0.0003. More importantly, a clinically significant difference of 6.4 points was observed between the Firdapse and placebo groups for the QMG endpoint. Firdapse was well tolerated and showed a similar safety profile to that seen in earlier studies. All p-values reported are based on the entire intent to treat (ITT) population of patients that enrolled in this trial.

The prospectively defined secondary endpoint for the physician's clinical global impression of improvement (CGI-I) achieved statistical significance (p-value 0.0020). Further, the exploratory endpoints had the following results: (i) the triple timed up and go (3TUG) endpoint achieved statistical significance (p-value 0.0112), (ii) the evaluation of the QMG-Limb domains endpoint achieved statistical significance (p-value 0.0010), and (iii) the most bothersome symptom (MBS) endpoint was not statistically significant, but showed a positive trend (p-value 0.0572).

"Data from the LMS-003 trial continue to demonstrate that amifampridine phosphate has a significant magnitude of effect in treating the symptoms of LEMS patients," said Perry Shieh, MD, PhD, Associate Professor of Neurology at the David Geffen School of Medicine at UCLA, and principal investigator of this clinical trial. "The findings are especially meaningful given the need for FDA-approved therapies which may transform the lives of patients suffering from LEMS."

"We are extremely pleased with the top-line efficacy and safety results from this second Phase 3 trial, which reinforces the potential of Firdapse to be an important treatment for patients suffering from LEMS. We look forward to presenting further data in future publications and at medical conferences. We remain on track to submit our NDA to the FDA in the first quarter of 2018," stated Patrick J. McEnany, President and CEO of Catalyst Pharmaceuticals. "Catalyst remains committed to the development of therapies that will improve the function and lives of people with rare neurodegenerative diseases."

"Catalyst has once again been able to demonstrate that Firdapse is able to have a positive, clinically significant effect in the symptomatic treatment of LEMS," stated Gary Ingenito, MD, Ph.D., Chief Medical Officer of Catalyst Pharmaceuticals. "We thank all those individuals who participated in the LMS-003 trial, in order to have the possibility of an FDA approved medication accessible to the LEMS population."

About the Clinical Trial (LMS-003)

This clinical trial was designed as a double blind, randomized, "withdrawal trial" in which LEMS patients who were currently receiving Firdapse in Catalyst's Expanded Access Program (EAP) were invited to participate in LMS-003, and upon completion of screening were treated with either Firdapse or placebo (randomly assigned, 1:1) during a 4-day randomization period. Prior to initiation of this trial, the FDA agreed to the design through the Special Protocol Assessment (SPA) process. A total of 26 patients completed the randomized treatment. In a trial of this design, the clinically significant findings, when present, are worsening of symptoms in the placebo group.

All endpoints were evaluated as a change in the assessment from day 0 to day 4 in the randomization phase of the trial (a

"change from baseline", or CFB). Although the protocol allowed for subjects to be "rescued" if the treatment during the randomization phase resulted in an intolerable level of symptoms or effect on ambulation, no subjects required rescue and all 26 subjects completed the randomization phase to provide pre- and post-treatment assessment data. All patients who participated in the trial are continuing to participate in Catalyst's EAP following completion of the trial.

About the Clinical Trial Endpoints

The protocol for this trial specified 2 co-primary endpoints, QMG score and SGI. The QMG score is a physician-rated semi-quantitative evaluation consisting of 13 assessments (each rated 0 to 3), which are totaled to obtain a QMG score, and includes tests for arm strength, leg strength, face and neck muscle performance, swallowing, speech, grip strength, forced respiration, and gaze impairment. The second co-primary endpoint, the SGI score, is a subjective scale on which the patients rate their satisfaction with the effects of Firdapse or placebo on their LEMS symptoms with scores from 1 ("Terrible") to 7 ("Delighted").

The CGI-I score was a secondary endpoint for the clinical trial and captures the investigator's overall impression of improvement or worsening of the patient's symptoms over the course of treatment with Firdapse. This 7-point scale is subjectively scored at the conclusion of treatment by the investigator based on changes in symptoms, behavior, and functional abilities, with scores ranging from 1="Very much improved" to 7="Very much worse".

Triple timed up and go (3TUG) was an exploratory endpoint and is a functional test that assesses a patient's ability to rise from a chair, walk 10 feet, turn around, walk back, and sit down. This is repeated three consecutive times, without rest, and the total time needed to complete the assessment is recorded. The proportion of subjects that demonstrated a change of 20%, or more (worsening in the case of a discontinuation design like this trial), for each treatment group was statistically evaluated. Researchers have established that a change of 20% or more, is considered a clinically significant change, and this type of statistical evaluation is referred to as a responder analysis.

The patient's most bothersome symptom assessment (MBS) was an exploratory endpoint and is an evaluation of how much a patient's most bothersome disease symptom, identified prior to treatment, bothers them after treatment. At randomization, the patient identifies their most bothersome symptom and rates how much, on a scale of 0 to 3, the symptom bothered them prior to ever being treated with Firdapse. After the end of the treatment phase, the patient is again asked to rate how much the previously identified symptom bothered them during the last day of the treatment period, on a scale of 0 to 3, and the change in the patient's assessment of their most bothersome symptom before and after treatment is analyzed as the MBS endpoint.

The evaluation of the QMG-Limb domain scores was an exploratory endpoint and is an assessment of the patient's arm and leg function using the four QMG domains that are related to a patient's ability to move their arms and legs. This subset of scores (each rated 0 to 3) were totaled and statistically evaluated as the QMG-Limb domain score.

About the Safety and Tolerability of Amifampridine

Firdapse was safe and well tolerated by the patients in the trial. The majority of adverse events (AEs) in each category occurred in the group assigned to placebo for this withdrawal design, as they experienced a return of their LEMS symptoms. There were no serious adverse events during the trial.

About Lambert-Eaton Myasthenic Syndrome (LEMS)

Lambert-Eaton Myasthenic Syndrome, or LEMS, is a rare autoimmune disorder, most often characterized by muscle weakness of the limbs. The disease is caused by an autoimmune reaction where antibodies are formed against voltage gated potassium channels in the connection between nerves and the muscles they communicate with. In approximately 50% of cases, LEMS is associated with an underlying malignancy, most commonly small-cell lung cancer, and in some individuals, LEMS is the first symptom of such malignancy. LEMS generally affects the extremities, especially the legs. As the disease most affects the parts of limbs closest to the trunk, difficulties with climbing stairs or rising from a sitting position are commonly noted. Physical exercise and high temperatures tend to worsen the symptoms. Other symptoms occasionally seen include weakness of the muscles of the mouth, throat, and eyes. Individuals affected with LEMS also may have a disruption of the autonomic nervous system, including dry mouth, constipation, blurred vision, impaired sweating, and/or hypotension.

Conference Call

Catalyst management will host a conference call and webcast today at 8:30 am ET to discuss these positive top-line results from the Firdapse LMS-003 clinical trial. Interested participants and investors may access the conference call by dialing (877) 407-8912 for domestic and Canadian callers or (201) 689-8059 for international callers. Those interested in listening to the conference call live via the internet may do so by visiting the Investors page of the Company's website

at www.catalystpharma.com and clicking on the webcast link on the Investors home page. A webcast replay will be available on the Catalyst website for 30 days following the call by visiting the Investor page of the Company's website at www.catalystpharma.com.

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, spinal muscular atrophy (SMA), 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 (i) whether the results of the LMS-003 trial, combined with the results of the Company's previous Phase 3 trial, will be acceptable to the FDA as support for an approval of Firdapse for the treatment of LEMS, (ii) whether any NDA submitted for Firdapse will be accepted by the FDA, and the timing of any such submission and acceptance, (iii) whether any additional abuse liability studies of Firdapse will be required by the FDA before Catalyst can resubmit an NDA for Firdapse, (iv) 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, (v) whether, if an NDA for Firdapse is accepted for filing, such NDA will be given a priority review by the FDA, (vi) whether Firdapse will ever be approved for commercialization, (vii) whether Catalyst will be the first company to receive an approval for amifampridine (3,4-DAP), giving it 5-year marketing exclusivity for its product, and (viii) 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.

Investor Contact

Brian Korb

The Trout Group LLC

(646) 378-2923

bkorb@troutgroup.com

Company Contact

Patrick J. McEnany

Catalyst Pharmaceuticals

Chief Executive Officer

(305) 420-3200

pmcenany@catalystpharma.com

Media Contact

David Schull

Russo Partners

(212) 845-4271

david.schull@russopartnersllc.com

Primary Logo

Source: Catalyst Pharmaceuticals, Inc.

News Provided by Acquire Media