May 18, 2016

Catalyst Pharmaceutical's Firdapse Phase 3 Study Published in Muscle & Nerve

CORAL GABLES, Fla., May 18, 2016 (GLOBE NEWSWIRE) -- Catalyst Pharmaceuticals, Inc. (Nasdaq:CPRX), a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases, today reported on the publication of detailed results from the LMS-002 Phase 3 study of amifampridine phosphate (Firdapse®) in patients with Lambert-Eaton myasthenic syndrome (LEMS). The study results were published in the May 2016 issue of *Muscle & Nerve* (volume 53, number 5), authored by Dr. Shin Oh, et al.

As previously reported by Catalyst, its LMS-002 Phase 3 study was a multi-center, randomized, double blind, placebo-controlled "withdrawal trial" in which all patients were initially treated with Firdapse followed by treatment with either Firdapse or placebo during a 2-week randomization period. The study assessed the safety and efficacy of amifampridine phosphate in patients with LEMS, an ultra-rare autoimmune disease resulting in debilitating muscle weakness and other, possibly severe, symptoms. A total of 38 patients completed the trial. In this trial design, the clinically significant findings, when present, are worsening of symptoms in the placebo group.

Summary of Clinical Trial Results for Firdapse

- Co-primary endpoints
  - The primary endpoint of change in quantitative myasthenia gravis score, or QMG, at day 14 reached statistical significance (p=0.0452).
  - The primary endpoint of change in subject global impression, or SGI, at day 14 was also statistically significant (p=0.0028).
- Secondary endpoints
  - The secondary endpoint for the physician's clinical global impression of improvement, or CGI-I, reached statistical significance (p=0.0267).
  - The secondary endpoint of change in walking speed at day 14 showed a worsening of 9.67 ft/min in the placebo group. As expected, this was a quantitative worsening in walking speed in the placebo group, but the magnitude of the change relative to the variance inherent in this test prevented reaching statistical significance for this endpoint with this small sample size.

"Publication in *Muscle & Nerve*, a leading medical publication devoted to neuromuscular disorders and treatments, further validates the strength of the Phase 3 data that showed a significant benefit for patients taking Firdapse," stated Gary Ingenito, M.D., Ph.D., Chief Medical Officer of Catalyst. "I would also like to reiterate our appreciation to all the patients and physicians who participated in LMS-002 as we continue to work towards gaining FDA approval of Firdapse."

Shin J. Oh, M.D., Distinguished Professor Emeritus, University of Alabama at Birmingham, noted, "These findings demonstrate that amifampridine phosphate is safe and effective in the symptomatic treatment of LEMS. This is an important milestone in the development of Firdapse towards an FDA approval and access for all patients with LEMS who may benefit."

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. Firdpase for the treatment of LEMS has received Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) and Orphan Drug Designations for LEMS and CMS. Firdapse is the first and only drug approved 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 evaluating Firdapse for the treatment of LEMS will be acceptable to the FDA, the timing of such trial, and whether such trial will be successful, 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 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 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 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 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.

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 Contacts

David Schull

Matt Middleman, M.D.

Russo Partners

(212) 845-4271

(212) 845-4272

david.schull@russopartnersllc.com

matt.middleman@russopartnersllc.com

Primary Logo

Source: Catalyst Pharmaceuticals, Inc.

News Provided by Acquire Media