April 23, 2014

Catalyst Announces Initiation of an Expanded Access Program to Provide Firdapse at No Cost to U.S. Patients with Lambert-Eaton Myasthenic Syndrome

CORAL GABLES, Fla.--(BUSINESS WIRE)-- Catalyst Pharmaceutical Partners, Inc. announced today it has begun the process required to establish an expanded access program (EAP) to make available in the United States its investigational potassium channel inhibitor, Firdapse™ (amifampridine phosphate or 3,4-DAP phosphate), to patients diagnosed with Lambert-Eaton Myasthenic Syndrome (LEMS) through their neuromuscular disease specialists. The product, currently in Phase 3 development, will be provided at no cost until sometime after approval. Program enrollment will begin in the next few months, pending FDA review and approvals.

"As part of our ongoing commitment to the LEMS community, we are making Firdapse available to those who would like access to this investigational drug, but are unable to participate in clinical studies or travel to sites where it is available," said Patrick J. McEnany, CEO of Catalyst Pharmaceutical Partners, Inc.

Patients diagnosed with LEMS or physicians treating such patients can call toll free 1-844-FIRDAPSE (1-844-347-3277) to inquire about this program.

"In previously published clinical trials, amifampridine has been shown to reduce neuromuscular weakness and symptoms of autonomic dysfunction in LEMS patients while being well tolerated," said Shin J. Oh, MD, Distinguished Professor Emeritus of Neurology, University of Alabama at Birmingham School of Medicine. "I am pleased that the company will be able to make its drug available to patients, who need it, while diligently pursuing FDA approval."

Expanded access is a mechanism supported by regulatory agencies for getting investigational treatment to patients who have a life threatening or severely debilitating disease and who cannot be satisfactorily treated with an alternative therapy approved by the FDA. Expanded access programs are not required by regulatory agencies. These programs are initiated and supported by drug manufacturers in recognition of the promise an unapproved drug may hold for patients facing a life-threatening or severely debilitating disease.

About Lambert-Eaton Myasthenic Syndrome (LEMS)

LEMS is an autoimmune neuromuscular disease in which the release of acetylcholine is decreased at the neuromuscular junction, resulting in muscle weakness. Patients with LEMS typically present with fatigue, muscle pain and stiffness that is generally more marked in the proximal muscles, most often muscles of the legs and trunk. Other symptoms may include reduced reflexes, drooping eyelids, facial weakness and swallowing problems. Patients often report a dry mouth, impotence, constipation and light-headedness on standing. On occasion, these problems can be life threatening when the weakness involves respiratory muscles. Approximately 50 percent of patients diagnosed with LEMS have an underlying malignancy, most commonly small cell lung cancer. LEMS is therefore regarded as a paraneoplastic syndrome (a condition that arises as a result of cancer elsewhere in the body).

About Firdapse

Firdapse, amifampridine phosphate or 3,4-diaminopyridine phosphate (3,4-DAP), is a potassium channel inhibitor. By blocking this ion channel, Firdapse increases the nerve repolarization time, which causes an increase in the influx of calcium, thereby causing more acetylcholine to be released, restoring muscle fiber contraction thus relieving muscle weakness caused by LEMS. In addition to LEMS, other potential orphan neuromuscular indications for Firdapse include certain types of Myasthenia Gravis and Congenital Myasthenic Syndrome, among others.

Firdapse has been granted orphan drug and breakthrough therapy designations by the U.S. FDA, and orphan medicinal product designation in the European Union for the treatment of LEMS. It is approved and commercialized in the E.U. by BioMarin Pharmaceutical. Catalyst recently announced it has reached its patient enrollment target for its Phase 3, double-blind, placebo-controlled, clinical trial evaluating the safety and efficacy of Firdapse for the treatment of LEMS. Upon completion of the Phase 3 trial, assuming it is successful, Catalyst expects to begin submitting a rolling new drug application with the FDA in 2015.

About Catalyst's Commitment to LEMS Patients

Catalyst is committed to bringing all patients diagnosed with LEMS a quality, safe and effective, FDA approved product, regardless of financial circumstances. The Company is already working to establish a patient assistance program that will be designed to help patients without insurance, those in Medicare Part D, or Medicaid, and those facing financial challenges. Catalyst will work hard to understand the needs of patients and is committed to helping them afford their medication.

About Catalyst Pharmaceutical Partners

Catalyst Pharmaceutical Partners, Inc. is a specialty pharmaceutical company focused on the development and commercialization of prescription drugs targeting rare (orphan) neuromuscular and neurological diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS), infantile spasms, and Tourette Syndrome. In 2012, Catalyst licensed Firdapse from BioMarin, and Catalyst assumed management of the Phase 3 pivotal trial, which was initiated by BioMarin. Firdapse is the first and only European approved drug for symptomatic treatment in adults with LEMS.

Catalyst is also developing a potentially safer and more potent vigabatrin analog (designated CPP-115) to treat infantile spasms, and epilepsy, as well as other neurological conditions associated with reduced GABAergic signaling, like post-traumatic stress disorder and Tourette syndrome. 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. For more information, please visit www.catalystpharma.com.

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 the anticipated timing of the receipt of top-line results from the double-blind, placebo-controlled portion of the Phase 3 trial of Firdapse, whether historic metrics of patients enrolled in the trial who complete the run-in phase of the trial and are randomized into the double-blind, placebo-controlled portion of the trial will continue to apply, such that at least 36 patients will be randomized into the double-blind, placebo-controlled portion of the trial from the patients already enrolled in the trial, whether the Phase 3 trial will be successful, 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, whether an NDA for Firdapse will ever be accepted for filing by the FDA, the timing of any such NDA filing or acceptance, whether Catalyst will be the first company to receive an approval for 3,4-DAP, giving it 7-year marketing exclusivity for its product, 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 2013 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.

Photos/Multimedia Gallery Available: http://www.businesswire.com/multimedia/home/20140423005359/en/

Media/Investor Contacts
LaVoie Health Science
David Connolly or , 617-374-8800
dconnolly@lavoiehealthscience.com
or
Aurora Krause, 617-374-8800
akrause@lavoiehealthscience.com
or
Company Contact
Catalyst Pharmaceutical Partners, Inc.
Patrick J. McEnany, 305-529-2522
Chief Executive Officer
pmcenany@catalystpharma.com

Source: Catalyst Pharmaceutical Partners, Inc.