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Catalyst Pharmaceuticals Announces Phase 2 Study of Firdapse® in Ambulatory Patients with Spinal Muscular Atrophy (SMA)

CORAL GABLES, Fla., Nov. 21, 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 the initiation of a company-sponsored, adequate and well-controlled, proof-of-concept clinical trial evaluating safety, tolerability and efficacy of Firdapse® (amifampridine phosphate) as a symptomatic treatment for patients with Spinal Muscular Atrophy (SMA) Type 3.

The study will be conducted by a team of researchers led by Lorenzo Maggi, MD, and Giovanni Baranello, MD, of the Fondazione Istituto Neurologico Carlo Besta in Milan, Italy, a major referral center for SMA patients. The study is designed as a randomized (1:1), double-blind, 2-period, 2-treatment, crossover, outpatient proof-of-concept study to evaluate the safety, tolerability and potential efficacy of amifampridine in ambulatory patients diagnosed with SMA Type 3. The study is planned to include approximately 12 patients, and Catalyst anticipates reporting top-line results from the study in the first half of 2019.

Patrick J. McEnany, Chief Executive Officer of Catalyst, said, "We are very pleased that Drs. Maggi and Baranello and their team at the Fondazione Istituto Neurologico Carlo Besta have agreed to conduct this study for Catalyst of Firdapse in patients diagnosed with Spinal Muscular Atrophy Type 3. If the results from this trial support the safety and efficacy of Firdapse as a treatment for ambulatory patients with SMA Type 3, we intend to submit an application for orphan drug designation and to pursue a clinical program required to obtain approval of Firdapse for this indication."

Studies in SMA animal models and SMA patients have shown that the neuromuscular junction, or NMJ, displays significant structural and functional defects that precede overt disease symptoms, suggesting that impaired NMJ function may contribute to SMA pathogenesis and symptoms. Defects in the NMJ appear to precede degeneration of motor neurons suggesting that abnormal formation and/or lack of maintenance of this structure may be a key contributor to the disease pathogenesis. These defects have been observed at both the pre- and postsynaptic components of the NMJ, which likely contribute to the failure to maintain the NMJ and muscle innervation in mouse models of SMA. Finally, NMJ dysfunction has been observed in SMA patients, mainly in the Type 3 subgroup, which contributes to the symptoms associated with the disease, and also is the primary reason for Catalyst's choice to limit this new trial to SMA Type 3 patients.

Amifampridine enhances neuromuscular junction transmission and therefore may help to preserve the NMJ and motor neurons in these patients. Catalyst hopes that Amifampridine treatment will provide symptomatic relief to SMA Type 3 patients, and the assessment of this symptomatic relief, should it occur, is the primary outcome variable of this new trial. In addition, as amifampridine enhances NMJ transmission, it is also hoped that the enhanced NMJ function will slow the progression of the nerve degeneration pathology of SMA. However, due to the duration of the treatment periods in this new trial, it will not be possible in this trial to assess the long-term progression of SMA in these patients.

About Spinal Muscular Atrophy (SMA) Type 3

Spinal Muscular Atrophy (SMA) is a spectrum of genetic disorders of the Survival Motor Neuron (SMN) protein that affects the function of the neuromuscular junction. The pathogenesis may, in part, progress due to the lack of retrograde signaling from dysfunctional neuromuscular junctions leading to nerve damage and ultimately nerve cell death. As a spectrum of genetic disorders of the SMN protein, the condition varies in severity and the disease has been classified into Types (SMA Types 1 through 4) based primarily on clinical symptoms of the disease. The overall incidence of SMA is believed to be 1 in 6,000 to 10,000 live births, with over half of the cases diagnosed as SMA Type 1. Due to the poor prognosis of SMA Type 1 patients, the actual prevalence is lower, since well over half of the SMA patients are Type 1 and have a very short life span. Due to the heterogeneity of the disease and the variations in life expectancy, prevalence is difficult to determine and not well defined for the different types of SMA. Current estimates place the prevalence of SMA Types 2 and 3 at about 1.5 per 100,000 people, with the majority of these being SMA Type 3 due to the longer life span of SMA Type 3 patients. Based on currently available data, Catalyst estimates the prevalence of SMA Type 3 in the United States to be about 2,500 to 3,500 patients.

SMA Type 3 (sometimes called Kugelberg-Welander disease) includes clinically heterogeneous patients. They typically reach all major motor milestones in childhood and independent walking by adulthood. However, during infancy they typically have proximal muscular weakness. Some might need wheelchair assistance in childhood, whereas others might continue to walk and live productive adult lives with minor muscular weakness. Patients who lose ambulation often develop scoliosis

and other medical problems related to poor mobility and muscle tone, such as obesity and osteoporosis. Two subgroups of severity have been suggested based on the probability of being able to walk by age 10 and on the subsequent probability of losing the ability to walk by age 40. Significant differences in losing the ability to walk have been observed in relation to those with an onset of weakness before (SMA 3a) and after (SMA 3b) age 3.

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, 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 Firdapse will provide symptomatic relief to SMA Type 3 patients or slow the progression of the nerve degeneration pathology of SMA, (ii) whether Catalyst's estimate of patients in the U.S. with SMA Type 3 will be determined to be accurate, (iii) whether Catalyst can obtain orphan drug designation for this indication, (iv) whether this proof-of-concept study evaluating Firdapse for the treatment of SMA Type 3 will be successful, (v) even if this trial is successful, what other future trials and studies will be required for Catalyst to seek approval to commercialize Firdapse for the treatment of SMA Type 3, (vi) whether Firdapse will ever be approved for commercialization for the treatment of any disease, and (vii) 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

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