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Catalyst Pharmaceuticals Observes Rare Disease Day 2016

CORAL GABLES, Fla., Feb. 29, 2016 (GLOBE NEWSWIRE) -- **Catalyst Pharmaceuticals, Inc.** (Nasdaq:CPRX), 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, today announced its support for Rare Disease Day® (www.rarediseaseday.org), always held today, the last day of February and dedicated to providing education and awareness about rare diseases. This year's theme is "Patient Voice-*Join us in making the voice of rare diseases heard*." Rare Disease Day is observed around the globe and is led in the U.S. by the National Organization for Rare Disorders (NORD) (http://rarediseases.org), a patient advocacy organization committed to the identification, treatment and cure of rare disorders through programs of education, advocacy and research. Catalyst collaborates with NORD as a corporate member. Global Genes (https://globalgenes.org/), one of the leading rare disease patient advocacy organizations in the world that promotes the needs of the rare disease community and of which Catalyst is also a corporate member, is another organization that supports patient activities to raise rare disease awareness on this special day.

"Providing education and awareness about rare diseases is a major focus for Catalyst," said Patrick J. McEnany, chairman and CEO of Catalyst. "We are pleased to support the efforts of the rare disease community to find ways to broaden the awareness of rare diseases and improve access to treatments."

In support of rare disease awareness, Catalyst has added a patient engagement section to its website (www.catalystpharma.com) that provides stories from patients with Lambert-Eaton myasthenic syndrome (LEMS) and their struggles with diagnosis and living with the disease.

According to the U.S. National Institutes of Health, a disease is rare if it affects fewer than 200,000 people. Nearly 1 in 10 Americans live with a rare disease—affecting 30 million people—and two-thirds of these patients are children. There are nearly 7,000 identified rare diseases, and with only approximately 450 U.S. Food and Drug Administration medical treatments approved for all of these diseases, many of these patients may receive no treatment for their disorders.

About Lambert-Eaton Myasthenic Syndrome (LEMS)

LEMS is a severely debilitating, rare autoimmune disease that affects approximately 1 in 100,000 people. The most common clinical presentations are proximal muscle weakness, easy fatigability that may lead to difficulty walking and climbing stairs, and cholinergic dysautonomia.

About Congenital Myasthenic Syndromes (CMS)

CMS is a rare autoimmune inherited neuromuscular disorder that is characterized by fatigable weakness of the skeletal muscles with onset at or shortly after birth or early childhood. In some cases, CMS symptoms may include sudden severe exacerbations of weakness or sudden episodes of respiratory insufficiency. Prevalence is estimated at 1-2 in 500,000 people.

About Tourette's Disorder

Tourette's Disorder is a hereditary neurological movement disorder that is characterized by repetitive motor and vocal tics. Symptoms may include involuntary movements of the extremities, shoulders, and face accompanied by uncontrollable sounds and, in some cases, inappropriate words.

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. Catalyst's lead candidate, Firdapse for the treatment of LEMS, has completed testing in a global, multi-center, double-blinded randomized pivotal Phase 3 trial resulting in positive top-line data. Firdapse 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 European approved drug 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 what additional supporting information will be required before the FDA will accept an NDA filing for Firdapse, whether any additional clinical studies or trials will be required before the FDA will accept an NDA filing for Firdapse for LEMS, 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 clinical trials and studies will be required before Catalyst can file an NDA for Firdapse for the treatment of CMS and whether any such required clinical trials and studies will be successful, the timing of any future NDA acceptance, whether, if an NDA for Firdapse is accepted for filing, such NDA will be given a priority review by the FDA, 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 the investigator-sponsored study evaluating Firdapse for the treatment of MuSK-MG will be successful, whether CPP-115 will be determined to be safe for humans, 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 2014 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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