February 27, 2017

Catalyst Pharmaceuticals Supports Rare Disease Day® and Joins Global Movement to Raise Important Awareness for Rare Diseases

CORAL GABLES, Fla., Feb. 27, 2017 (GLOBE NEWSWIRE) -- Catalyst Pharmaceuticals, Inc. (Nasdaq:CPRX), (Catalyst) a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases, today announced that it has joined forces with 30 million Americans and health care advocates around the world for Rare Disease Day® on February 28. Rare Disease Day is an annual awareness day dedicated to elevating public understanding of rare diseases and calling attention to the special challenges they present to patients and families.

Catalyst continues to support patient organizations that provide disease awareness for Lambert-Eaton Myasthenic Syndrome (LEMS) and Congenital Myasthenic Syndromes (CMS). These are rare diseases in which patients are often misdiagnosed with other diseases prior to getting a definitive diagnosis, thus educating the patient and physicians at medical congresses is an important outreach to the rare disease community to facilitate proper and prompt diagnoses.

"Completing one phase 3 clinical study and enrolling a second phase 3 clinical study for LEMS indicates our commitment to the LEMS community for patients to have access to an FDA approved treatment" says Patrick J. McEnany, Chairman and CEO of Catalyst. "In addition, we have expanded our CMS phase 3 clinical study to include adults in recognition of the need to provide access to a potential treatment across the entire CMS population. We are pleased to continue to support the efforts of the rare disease community to find ways to broaden the awareness of rare diseases and improve access to treatments."

According to the National Institutes of Health (NIH), 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 nearly half of these patients are children. There are more than 7,000 rare diseases and only approximately 450 FDA-approved medical treatments.

Rare Disease Day takes place every year on the last day of February (February 28 or February 29 in a leap year)—the rarest date on the calendar—to underscore the nature of rare diseases and what patients face. It was established in Europe in 2008 by EURORDIS, the organization representing rare disease patients in Europe, and is now observed in more than 80 nations. Rare Disease Day is sponsored in the U.S. by the National Organization for Rare Disorders (NORD)®, the largest and leading independent, nonprofit organization committed to the identification, treatment, and cure of rare diseases and one which Catalyst actively supports. Additionally, Catalyst supports Global Genes in their efforts to continue to get patients with rare diseases to take an active role in raising awareness about their disease.

For more information about Rare Disease Day in the U.S., go to http://www.rarediseaseday.us. For information about global activities, go to www.rarediseaseday.org. To search for information about rare diseases, visit NORD's website www.rarediseases.org and Global Gene's website https://globalgenes.org/world-rare-disease-day.

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. 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 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, the timing on Catalyst's second trial evaluating Firdapse for the treatment of LEMS and whether the trial will be successful, whether Catalyst's assumptions in its updated business plan will be accurate and the impact of unanticipated events or delays in projected activities on Catalyst's cash requirements and on Catalyst's ability to get to an accepted NDA submission for Firdapse without the need for additional funding, 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 the investigator-sponsored study evaluating Firdapse for the treatment of MuSK-MG 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 ever 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 spasms, 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 submits 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