February 12, 2018

Catalyst Pharmaceuticals Announces Plans to Resubmit New Drug Application for Firdapse®

- Positive meeting with the FDA about the resubmission of an NDA for Firdapse
- Resubmission of NDA for Firdapse on schedule for end of the first quarter

CORAL GABLES, Fla., Feb. 12, 2018 (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 results of its recent Type C meeting with the U.S. Food and Drug Administration (FDA). Prior to the meeting, Catalyst had provided the FDA with its preliminary data package for the proposed NDA resubmission, including clinical, non-clinical, regulatory and abuse liability elements. The preliminary data package included the recently reported positive top-line results from a required second, confirmatory Phase 3 clinical trial (LMS-003) of Firdapse® for the symptomatic treatment of LEMS, as well as the recently completed FDA-required abuse liability studies demonstrating that Firdapse does not have abuse potential. The minutes of the meeting received from the FDA reflect the FDA's advice to Catalyst that its proposed filing package will be sufficient for resubmission of an NDA for Firdapse, and Catalyst currently anticipates resubmitting its NDA for Firdapse® for LEMS to the FDA by the end of the first quarter of 2018.

As previously reported, Catalyst is presently conducting a Phase 3 double-blind placebo-controlled clinical trial evaluating Firdapse® for the treatment of congenital myasthenic syndromes (CMS), and Catalyst expects to report top line results from the trial in the second half of this year. Catalyst is currently evaluating its options for the most appropriate and efficient path forward to hopefully include CMS in any approved labeling for Firdapse.

"We remain on track to resubmit our NDA for Firdapse in the first quarter of this year," stated Patrick J. McEnany, President and CEO of Catalyst Pharmaceuticals. "Based on our discussions with the FDA, we believe that we have a clear regulatory pathway forward, and we will continue to work collaboratively with the FDA as we seek to bring FDA approved therapies forward for the treatment of LEMS and CMS."

About Lambert-Eaton Myasthenic Syndrome (LEMS)

Lambert-Eaton Myasthenic Syndrome, or LEMS, is a rare autoimmune disorder, most often characterized by muscle weakness of the limbs. The disease is caused by an autoimmune reaction where antibodies are formed against voltage gated potassium channels in the connection between nerves and the muscles they communicate with. In approximately 50% of cases, LEMS is associated with an underlying malignancy, most commonly small-cell lung cancer, and in some individuals, LEMS is the first symptom of such malignancy. LEMS generally affects the extremities, especially the legs. As the disease most affects the parts of limbs closest to the trunk, difficulties with climbing stairs or rising from a sitting position are commonly noted. Physical exercise and high temperatures tend to worsen the symptoms. Other symptoms occasionally seen include weakness of the muscles of the mouth, throat, and eyes. Individuals affected with LEMS also may have a disruption of the autonomic nervous system, including dry mouth, constipation, blurred vision, impaired sweating, and/or hypotension.

About Congenital Myasthenic Syndromes (CMS)

Congenital myasthenic syndromes, or CMS, are rare neuromuscular disorders comprising a spectrum of genetic defects and is characterized by fatigable weakness of skeletal muscles with usual onset at or shortly after birth or early childhood; in rare cases symptoms may not manifest themselves until later in childhood or adulthood. The severity and course of the disease are variable, ranging from minor symptoms to progressive disabling weakness; symptoms may be mild, but sudden severe exacerbations of weakness or even sudden episodes of respiratory insufficiency also occur. Congenital myasthenic syndromes are rare, estimated at one-tenth that of myasthenia gravis, which in itself is rare. Based on currently available information, Catalyst estimates that there are between 1,000 and 1,500 CMS patients in the United States.

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. 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 the results of the LMS-003 trial, combined with the results of the Company's previous Phase 3 trial, will be acceptable to the FDA as support for an approval of Firdapse for the treatment of LEMS, (ii) whether the results of the abuse liability studies undertaken by Catalyst will be acceptable to the FDA as support for an approval of Firdapse, (iii) whether any NDA submitted for Firdapse will be accepted by the FDA, and the timing of any such submission and acceptance, (iv) 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, (v) whether, if an NDA for Firdapse is accepted for filing, such NDA will be given a priority review by the FDA, (vi) whether Firdapse will ever be approved for commercialization, (vii) whether Catalyst will be the first company to receive an approval for amifampridine (3,4-DAP), giving it 5-year marketing exclusivity for its product, and (viii) 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.

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