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## **Catalyst Pharmaceuticals Announces Publication of CPP-115 Clinical Efficacy Data for Infantile Spasms in Epilepsy & Behavior Case Reports**

CORAL GABLES, Fla., Sept. 19, 2016 (GLOBE NEWSWIRE) -- Catalyst Pharmaceuticals, Inc. (Nasdaq:CPRX), a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases, today announced that the journal of *Epilepsy & Behavior Case Reports* has accepted for publication a case report on the efficacy of CPP-115 in a child with refractory infantile spasms.

The case report presents a child treated with CPP-115 through an investigational new drug protocol who experienced a significant reduction of seizures with no evidence of retinal dysfunction. The research paper made the following conclusions:

- | The case study suggests sustained efficacy and tolerability of CPP-115 in treating epileptic spasms
- | It reported reduction in seizure frequency and documented improvements in the EEG interictal and ictal record temporally associated with CPP-115 initiation for this patient
- | It reported that in the context of refractory infantile spasms and associated morbidity, mortality, and poor neurodevelopmental outcomes, CPP-115 is potentially a promising alternative to vigabatrin therapy

Prior to treatment with CPP-115, the patient had failed ten drugs and the ketogenic diet, and had approximately 100 seizures per day. One year after starting CPP-115 and coming off of clobazam and vigabatrin, the patient's reported seizures have seen a marked reduction in frequency and his cognition and behavior have improved.

"There is a significant unmet medical need in the area of refractory infantile spasms, as parents of children who have infantile spasms have a very difficult choice when it comes to treatment options, weighing both drug-related risks and adequate treatment," said Patrick J. McEnany, Chairman and CEO of Catalyst. "Our pediatric epilepsy experts advise us that approximately half of the children diagnosed with infantile spasms are refractory to the medications approved for the treatment of infantile spasms."

### **About the Published Paper**

The research paper was authored by Kyra Doumlele, Erin Conway, Julie Hedlund, Patricia Tolete, Dr. Orrin Devinsky, New York University School of Medicine, Comprehensive Epilepsy Center, New York, New York. The manuscript can be accessed at <http://www.sciencedirect.com/science/article/pii/S2213323216300391>.

### **About West Syndrome / Infantile Spasms**

An infantile spasm is a type of seizure seen in an epilepsy syndrome of infancy and childhood known as West Syndrome. The onset of infantile spasms is usually in the first year of life, typically between 4-8 months. Spasms often occur in clusters of up to 100 at a time, and infants may have dozens of clusters and several hundred spasms per day. Infantile spasms usually stop by age five, but may be replaced by other seizure types. Many underlying disorders, such as birth injury, metabolic disorders and genetic disorders can give rise to spasms, making it important to identify them (symptomatic IS). In some children, no cause can be found (cryptogenic IS). Mental retardation occurs in 70-90% of persons with infantile spasms, usually involving severe to profound retardation. Early control of seizures is critical for reducing developmental delays and levels of mental retardation, but ~5% of infants with this condition eventually die from complications caused by the seizures.

### **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® for the treatment of LEMS has received Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) 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, what study design for a second trial evaluation of Firdapse for the treatment of LEMS will be acceptable to the FDA, the timing of such trial, and whether it 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.*

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