

# Catalyst Pharmaceutical Partners and BioMarin Pharmaceutical Enter Into Strategic Collaboration for Firdapse(TM) in North America

# BioMarin to Make \$5 Million Strategic Equity Investment in Catalyst

#### Catalyst Licenses North American Rights to Firdapse,™ Phase III Orphan Drug for the Treatment of Lambert-Eaton Myasthenic Syndrome (LEMS)

# Catalyst Expects Top-Line CPP-109 Phase II(b) Data During First Half of November

CORAL GABLES, Fla., Oct. 31, 2012 (GLOBE NEWSWIRE) -- Catalyst Pharmaceutical Partners, Inc. (Nasdaq:CPRX) and BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that they have entered into a strategic collaboration for the rights to Firdapse<sup>™</sup> iNorth America. Firdapse<sup>™</sup> is an orphan product, which has been approved in the uropean Union (EU) and is undergoing a Phase III clinical trial in the United States, for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS), a rare, debilitating and sometimes fatal autoimmune disease with the primary symptoms of muscle weakness. The key components of the collaboration include Catalyst licensing the exclusive North American rights to Firdapse<sup>™</sup> and and making a \$5 million investment in Catalyst to rapidly advance the Firdapse<sup>™</sup> program inthe United States.

Patrick J. McEnany, Catalyst's Chief Executive Officer, stated: "As part of this arrangement, we are gaining access to a latestage U.S. orphan drug targeting LEMS, a disease of the central nervous system for which there is not currently an effective treatment approved in the United States. Our existing product candidates are focused on addiction and central nervous system orphan indications like Infantile Spasms/West Syndrome and Tourette's Disorder, and adding Firdapse<sup>™</sup> is consistent with our product development strategy. The relationship with BioMarin is exciting and strategically important, as it provides Catalyst with another orphan drug candidate and near-term funding towards the completion of the currently underway Phase III trial for Firdapse<sup>™</sup>."

Under the terms of the collaboration, Catalyst and BioMarin have entered into a:

- Convertible Promissory Note and Note Purchase Agreement under which BioMarin has invested \$5 million in Catalyst, which will convert on a mandatory basis into Catalyst common stock at a future date. The conversion price will be based on the dollar weighted average of Catalyst's common stock during the 15 business day period prior to the conversion date. Catalyst has covenanted to BioMarin that the \$5 million investment will be used solely for the purpose of developing Firdapse™ ithe United States.
- License Agreement in which Catalyst receives the exclusive rights to Firdapse<sup>™</sup> for all indications North America. Catalyst will be responsible for all future costs of developing and commercializing Firdapse<sup>™</sup> North America, and will share equally the cost of various post-marketing studies in the EU, the data from which is also anticipated to be included in the Firdapse<sup>™</sup> registration package ime United States. Subject to certain criteria, Catalyst will also owe royalty payments to BioMarin, and milestone and royalty payments to the former shareholders of Huxley Pharmaceuticals and to a third-party licensor of the rights being sublicensed to Catalyst.

Mr. McEnany continued: "Additionally, as previously announced, we are continuing to communicate closely with the Department of Veterans Affairs Cooperative Studies Program (VACSP), the collaborator responsible for the management, verification and statistical analyses of the data being collected in our Phase II(b) trial for the treatment of cocaine addiction. Based on information received to date, we continue to expect that we will receive and be in a position to report the top-line results from our trial during the first half of November 2012."

# About Firdapse™

Firdapse<sup>™</sup>also known as amifampridine phosphate, 3,4-diaminopyridine or 3,4-DAP, is a potassium channel blocker. It delays repolarization of the pre-synaptic neuron, causing voltage gated Ca2+ channels to remain open longer. The increased Ca2+ influx causes more acetylcholine to be released, making it more likely that a muscle action potential will be initiated, thereby reducing muscle weakness. BioMarin acquired the rights to Firdapse<sup>™</sup> as part of its acquisition **d**fuxley Pharmaceuticals in 2009. Since then, BioMarin has commercialized Firdapse<sup>™</sup> in the EU for LEMS, where it has orphan medicinal product designation.

In the United States, where it also has orphan drug designation, Firdapse<sup>™</sup> is in a Phase III, multicenter, doublehind, placebocontrolled, randomized discontinuation study followed by an open-label extension period to evaluate the efficacy and safety of Firdapse<sup>™</sup> in patients with LEMS. Upon completion of this transaction, Catalyst will be responsible for the overall management and continuing this already initiated study. The estimated enrollment for the U.S. Phase III study is 30 LEMS patients. In addition to LEMS, other potential orphan central nervous system indications for Firdapse<sup>™</sup> include Myasthenia Gravis and Congenital Myasthenic Syndrome, among others.

# About United States Orphan Drug Designation

Orphan drug designation is granted by the U.S. Food & Drug Administration (FDA) Office of Orphan Drug Products to promote the development of drugs and biologics for the treatment of rare diseases and disorders that affect fewer than 200,000 persons in the United States. The key benefit includes a 7-year period of market exclusivity if Firdapse<sup>™</sup> is the first of its type approvec for the specified indication or if it demonstrates superior safety, efficacy or a major contribution to patient care versus another drug of its type previously granted the designation for the same indication. Other potential benefits include tax credits for certain clinical research costs, annual grant funding, clinical trial design assistance and waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

# About LEMS

Lambert-Eaton Myasthenic Syndrome is a rare autoimmune disease with the primary symptoms of muscle weakness. The muscle weakness in LEMS is caused by autoantibodies to voltage gated calcium channels leading to a reduction in the amount of acetylcholine released from nerve terminals. The prevalence of LEMS is estimated at approximately 3,000 patients in the United States and Canada. Approximately 50 percent of LEMS patients diagnosed have small cell lung cancer. Patients with LEMS typically present with fatigue, muscle pain and stiffness. The weakness is generally more marked in the proximal muscles, particularly of the legs and trunk. Other problems include reduced reflexes, drooping of the eyelids, facial weakness and problems with swallowing. Patients often report dry mouth, impotence, constipation and feelings of light headedness on standing. These problems can be life threatening when the weakness involves respiratory muscles. A diagnosis of LEMS is generally made on the basis of clinical symptoms, electromyographic testing and the presence of autoantibodies against voltage gated calcium channels.

# **About Catalyst Pharmaceutical Partners**

Catalyst Pharmaceutical Partners, Inc. is a development-stage specialty pharmaceutical company focused on the development and commercialization of prescription drugs targeting diseases and disorders of the central nervous system. Catalyst has two products in development and is currently evaluating its lead product candidate, CPP-109 (vigabatrin, a GABA aminotransferase inhibitor), for the treatment of cocaine addiction and Tourette's Disorder. CPP-109 has been granted "Fast Track" status by the FDA for the treatment of cocaine addiction. Catalyst also expects to evaluate CPP-109 for the treatment of other addictions. Catalyst is also developing CPP-115, another GABA aminotransferase inhibitor that is more potent than vigabatrin and has reduced side effects (e.g., visual field defects, or VFDs) from those associated with vigabatrin. Catalyst is planning to develop CPP-115 for several indications, including drug addiction, epilepsy and for use in other selected central nervous system indications. CPP-115 has been granted orphan drug designation for the treatment of infantile spasms by the FDA and orphan medicinal product designation by the European Commission. Catalyst believes that it controls all current intellectual property for drugs that have a mechanism of action related to the inhibition of GABA aminotransferase. For additional information, please visit <u>www.catalystpharma.com</u>.

# **About BioMarin Pharmaceutical**

BioMarin Pharmaceutical Inc. develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. Its product portfolio comprises four approved products and multiple clinical and pre-clinical product candidates. Approved products include Naglazyme® (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; Kuvan® (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; and Firdapse<sup>™</sup> (amifampridine), which has been approved by theuropean Commission for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS). Product candidates include GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase III clinical development for the treatment of MPS IVA, amifampridine phosphate (3,4-diaminopyridine phosphate), which is currently in Phase III clinical development for the treatment of LEMS in the U.S., PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU, BMN-701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase I/II clinical development for the treatment of Pompe disease, BMN-673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase I/II clinical development for the treatment of genetically-defined cancers, and BMN-111, a modified C-nutriuretic peptide, which is currently in Phase I clinical development for the treatment of achondroplasia. For additional information, please visit www.bmrn.com.

#### Forward-Looking Statements

This press release contains forward-looking statements. Forward-looking statements involve known and unknown risks and uncertainties which may cause actual results in future periods to differ materially from the statements made herein. A number of factors, including whether Firdapse<sup>TM</sup> will be approved for commercialization in the U.S., whether Catalyst will have sufficient resources to complete the development of Firdapse<sup>TM</sup> in the U.S., whether Catalyst's current Phase II(b) trial evaluating its product candidate CPP-109 for the treatment of cocaine addiction will be successful, whether Catalyst's current product candidates, CPP-109 and CPP-115, will ever be approved for commercialization in the U.S., and those other factors described in Catalyst's and BioMarin's filings with the U.S. Securities and Exchange Commission (SEC), could adversely affect the forward-looking statements made in this release. Copies of Catalyst's and BioMarin's filings with the SEC, may be found on the respective company's website or may be obtained upon request from the respective company. Neither Catalyst nor BioMarin undertake any obligation to update the information contained herein, which speaks only as of this date.

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Source: Catalyst Pharmaceutical Partners, Inc.

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