

## BioMarin Acquires Huxley Pharmaceuticals, Inc.

**Sole Product of Huxley is a Proprietary Form of 3,4-Diaminopyridine, Amifampridine Phosphate Positive Opinion for Approval of Amifampridine Phosphate for the Treatment of Lambert Eaton Myasthenic Syndrome (LEMS) Issued by European Regulatory Authorities on October 23, 2009 Official Approval and Product Launch Expected in Q1 2010 Conference Call and Webcast to Be Held Today at 5:30 p.m. ET**

PRNewswire-FirstCall  
NOVATO, Calif.

BioMarin Pharmaceutical Inc. announced today that it has acquired Huxley Pharmaceuticals, Inc. (Huxley), which has rights to a proprietary form of 3,4-diaminopyridine (3,4-DAP), amifampridine phosphate, for the rare autoimmune disease Lambert Eaton Myasthenic Syndrome (LEMS). Last week, the Committee for Medicinal Products for Human Use of the European Medicines Evaluations Agency adopted a positive opinion recommending approval of amifampridine phosphate for LEMS. If approved by the European Commission, amifampridine phosphate will be the first approved treatment for LEMS, thereby conferring orphan drug protection and providing ten years of market exclusivity in Europe. Huxley licensed the rights to 3,4-DAP from EUSA Pharma, which was developing the product after acquiring the rights from the original developer, Assistance Publique Hopitaux de Paris (AP-HP).

"This acquisition represents a natural extension of BioMarin's core business operations and strategy. LEMS is a rare, serious and debilitating autoimmune disease treated by neuromuscular specialists," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "This deal leverages our existing European infrastructure and commercial capabilities and provides the opportunity for near-term revenue growth and operating income growth. We expect to launch the product in Europe in the first quarter of 2010, and are evaluating the best development strategy for amifampridine phosphate in LEMS in the U.S. and in other indications in the U.S. and Europe. We will also evaluate development of amifampridine phosphate in other indications including multiple sclerosis. We expect this deal to be dilutive in 2010 and accretive in 2011 and beyond."

Stephen Aselage, Senior Vice President and Chief Business Officer of BioMarin added, "3,4-DAP is currently the treatment of choice for LEMS. Although its use has been limited as an unapproved product due to regulatory restrictions and limited availability of drug product, 3,4-DAP has been studied in six randomized controlled trials and has been shown to improve muscle strength in LEMS patients as measured by a variety of means. 3,4-DAP has been widely recommended for use in LEMS, and the introduction of amifampridine phosphate will enable regular access to a high quality, stable proprietary product."

Bryan Morton, President and Chief Executive Officer of EUSA Pharma said, "We are pleased now to be working with BioMarin to develop and commercialize 3,4-DAP. BioMarin is well placed to launch this important treatment for LEMS, and to continue its future broader development."

Under the terms of the agreement, BioMarin paid Huxley stockholders \$15.0 million upfront and will pay an additional \$7.5 million upon final European Commission approval of amifampridine in LEMS, which is expected in late 2009 or early 2010. Additionally, Huxley stockholders are eligible to receive up to approximately \$36.0 million in milestone payments if certain annual, cumulative sales and U.S. development milestones are met. In addition, successful development of multiple sclerosis will result in milestone payments to EUSA.

### Conference Call Details

BioMarin will host a conference call and webcast to discuss the acquisition of Huxley Pharmaceuticals today, Monday, October 26, at 5:30 p.m. ET. This event can be accessed on the investor section of the BioMarin website at [www.BMRN.com](http://www.BMRN.com).

Date: October 26, 2009  
Time: 5:30 p.m. ET  
U.S. / Canada Dial-in Number: 866.788.0544  
International Dial-in Number: 857.350.1682  
Participant Code: 53851486  
Replay Dial-in Number: 888.286.8010  
Replay International Dial-in Number: 617.801.6888  
Replay Code: 48079886

About LEMS

Lambert Eaton Myasthenic Syndrome (LEMS) is a rare autoimmune disease with the primary symptoms of muscle weakness. 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 four to ten per million, or approximately 2,000 to 5,000 patients in the EU and 1,200 to 3,100 patients in the U.S. 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 a dry mouth, impotence, constipation and feelings of light headedness on standing. On occasion 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.

Current treatment of LEMS can consist of strategies directed at the underlying malignancy if one is present. Unfortunately, therapy of small cell lung cancer is limited and outcomes are generally poor. Immunosuppressive agents have been tried but success is limited by toxicity, and difficulty administering the regimens. A mainstay of therapy has been 3,4-DAP but its use in practice has been limited by the drug's availability. This problem will be addressed by the introduction of BioMarin's product.

#### About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises three 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; and Kuvan® (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany. Other product candidates include PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU and GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase I/II clinical development for the treatment of MPS IVA. For additional information, please visit [www.BMRN.com](http://www.BMRN.com). Information on BioMarin's website is not incorporated by reference into this press release.

#### About Huxley Pharmaceuticals

Huxley Pharmaceuticals, Inc. is private life sciences company founded and managed by Aceras BioMedical, LLC. Aceras is dedicated to funding and developing novel innovations that address unmet medical needs.

#### About EUSA Pharma

EUSA Pharma is a transatlantic specialty pharmaceutical company focused on in-licensing, developing and marketing late-stage oncology, pain control and critical care products. The company currently has eight marketed products, including Caphosol® for the treatment of oral mucositis, a common and debilitating side-effect of radiation therapy and high dose chemotherapy, Erwinase® and Kidrolase® for the treatment of acute lymphoblastic leukemia, Collatamp® G, a surgical implant impregnated with the antibiotic gentamicin, ProstaScint® for imaging the extent and spread of prostate cancer and Quadramet® for the treatment of pain in patients whose cancer has spread to the bones. EUSA also has several products in development.

#### About AP-HP

Assistance Publique - Hopitaux de Paris (AP-HP) is a public health federation that gathers together 37 hospitals for the Paris metropolitan area. Its activities are medical care (750 medical units / 52 medical and biological specialties), education (37 formation centers), and research performed either independently (AP HP labs and pharmaceutical departments), in partnership with hosted research units (112 INSERM and 30 CNRS units), or in partnership with external entities (public and private).

#### Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the expectations of the development and potential approval of Huxley's 3,4-Diaminopyridine product for the treatment of LEMS. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities, particularly the pending decision by the European Commission on the Marketing Authorization Application for

such product, our success in the commercialization of such product, if approved; results and timing of current and planned preclinical studies and clinical trials related to such product; our ability to successfully manufacture the product ; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's 2008 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

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