

BioMarin Announces Agreement With Repligen for Pre-clinical Compounds

Includes Compounds for Potential Treatment of Friedreich's Ataxia

SAN RAFAEL, Calif., Jan. 21, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has entered into an agreement to purchase Repligen Corporation's histone deacetylase inhibitor (HDACi) library and related intellectual property. Potential applications of the HDACi portfolio include Friedreich's ataxia and other neurological disorders. Under the terms of the agreement, BioMarin will provide Repligen with an upfront payment of \$2 million and potential future milestone and royalty payments for the development, regulatory approval and commercial sale of compounds included in this agreement.

"This agreement represents a natural extension of BioMarin's business strategy of acquiring promising pre-clinical programs that we can successfully develop and commercialize for people with serious, unmet medical needs," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "Friedreich's ataxia is a neurological disorder that affects tens of thousands of young people in the developed world and we look forward to the opportunity to move a compound into the clinic in this indication."

About Friedreich's ataxia

Friedreich's ataxia (FA) is a progressive, neurological disorder that affects approximately 20,000 people in the United States and Europe, typically resulting in wheelchair dependence in young adulthood and early death due to cardiac failure. It is caused by mutations in the FXN gene, and is inherited in an autosomal recessive manner. FXN mutations result in reduced expression of frataxin protein, manifesting in progressive neurological and cardiac damage. Major neurological symptoms include muscle weakness and ataxia, a loss of balance and coordination. These symptoms typically appear between 10 and 15 years of age, but FA has been diagnosed in people from ages 2 to 50 with earlier onset associated with a more severe course.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises four approved products and multiple clinical and pre-clinical product candidates. Approved products include Naglazyme® (galsulfase) for MPS VI, a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for 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® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include VIMIZIM™ (N-acetylgalactosamine 6-sulfatase), formally referred to as GALNS, which successfully completed Phase 3 clinical development for the treatment of MPS IVA, PEG PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 3 clinical development for the treatment of PKU, BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer, BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 1/2 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

Vimizim™ is our trademark, and BioMarin®, Naglazyme®, Kuvan®, Firdapse® are registered trademarks of BioMarin Pharmaceutical Inc.

Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC.

CONTACT: Investors:

Traci McCarty

BioMarin Pharmaceutical Inc.

(415) 455-7558

Media:

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451

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