BioMarin Acquires Zacharon Pharmaceuticals

Lead Program Focused on the Oral Treatment of MPS III
Acquisition Augments Existing Analytical Capabilities and Expertise in Glycobiology

SAN RAFAEL, Calif., Jan. 7, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has acquired Zacharon Pharmaceuticals, a private biotechnology company based in San Diego focused on developing small molecules targeting pathways of glycan and glycolipid metabolism.

Zacharon drug discovery projects include two ongoing lead optimization programs, inhibition of heparan sulfate synthesis for MPS III and other MPS disorders, and inhibition of ganglioside synthesis for diseases such as Tay Sachs and Sandhoff. Zacharon's proprietary SensiPro® platform is a powerful technology for analysis of specific carbohydrate structures and therefore the identification of candidate drugs to treat those conditions.

Hank Fuchs, M.D., Executive Vice President and Chief Medical Officer of BioMarin said, "Zacharon's lead program, focused on reducing the accumulation of heparan sulfate, offers the exciting prospect of treating both the CNS and peripheral manifestations of MPS III, and potentially other MPS disorders, with an orally bioavailable small molecule. In general, reducing the synthesis of the target substrate alleviates the burden on the compromised lysosomal system, and this therapeutic approach has been clinically validated with other enzyme inhibitors. Zacharon's deep expertise in glycobiology has generated additional programs for treating lysosomal storage disorders that we expect to progress, and we will leverage that expertise to continue to build BioMarin's existing research and development pipeline into a sustainably leading pipeline."

"The acquisition of Zacharon will further expand our glycobiology expertise and will support our lysosomal storage disease drug development efforts," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We are committed to investing in our advancing pipeline, which has evolved through a combination of internal development and targeted acquisitions, such as this."

Under the terms of the stock purchase agreement, BioMarin paid $10 million upfront for 100 percent of Zacharon's share capital and may make potential additional payments for clinical, regulatory and commercial milestones. The costs to be incurred in 2013 of acquiring Zacharon and absorbing its operations is covered by the previously provided 2013 R&D expense guidance.

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 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™ (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include BMN-110 (N-acetylgalactosamine 6-sulfatase), formally referred to as GALNS, which successfully completed Phase III clinical development for the treatment of MPS IVA, 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-natriuretic peptide, which is currently in Phase I clinical development for the treatment of achondroplasia. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

The BioMarin Pharmaceutical Inc. logo is available at http://www.globenewswire.com/newsroom/prs/?pkgid=11419

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: expectations related to the Zacharon development programs, and the development of BioMarin's other programs, including GALNS; and actions by regulatory authorities. 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: results and timing of current and planned preclinical studies and clinical trials; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning each of the described products and product candidates; 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 2011 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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