

## Press Release

### BioMarin Acquires ZyStor Therapeutics, Inc.

- **High Affinity ZC-701 Fusion Enzyme Replacement Therapy Demonstrates Superior Potency and Efficacy to Myozyme in Nonclinical Studies**
- **Phase I/II Clinical Study In Late Onset Pompe Expected to Start in Q1 2011**
- **Conference Call and Webcast to Be Held Today at 5:00 p.m. ET**

NOVATO, Calif., Aug 17, 2010 /PRNewswire via COMTEX/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) announced today that it has acquired ZyStor Therapeutics, Inc. (ZyStor), a privately-held biotechnology company developing enzyme replacement therapies (ERT) for the treatment of lysosomal storage disorders. ZyStor's lead product candidate is ZC-701, a novel fusion of insulin-like growth factor 2 and alpha glucosidase (IGF2-GAA) in development for Pompe disease.

Under the terms of the agreement, BioMarin acquired ZyStor for \$22 million upfront and up to an additional \$93 million if certain development, regulatory and commercial milestones are achieved. There are no royalties owed. The FDA has accepted an investigational new drug (IND) application for ZC-701, investigational product has been manufactured and a clinical study is expected to start in Q1 2011.

In vitro studies demonstrate that ZC-701 has more than ten times higher affinity for the mannose-6-phosphate receptor compared to Myozyme, which enables delivery of higher levels of enzyme to the lysosomes of muscle cells of Pompe patients. Studies in the Pompe mouse model indicate that ZC-701 clears glycogen to lower levels in skeletal, heart and diaphragm muscle compared to Myozyme and at similar levels compared to second generation compounds that have been tested. Many experts believe that an enzyme with more efficient uptake into muscle cells would lead to more effective treatment of the disease. Over the next several months BioMarin plans to recruit clinical research hospitals that can conduct clinical studies and finalize the clinical protocol and expects the first patient dosed in the first quarter of 2011.

"The acquisition of ZyStor gives us the opportunity to introduce a superior product to fulfill an unmet medical need and is a perfect fit in our core business. It not only provides us with a promising product candidate for Pompe disease but also an exciting new platform technology," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "ZC-701 has been shown to be more effective in the Pompe mouse model than commercially available replacement enzymes for Pompe disease. In addition, ZyStor's proprietary Glycosylation Independent Lysosomal Targeting (GILT) technology is applicable to other ERTs and has the potential to deliver more enzyme to lysosomes compared to traditional mannose-6-phosphate targeted approaches. Also, relative to our internal candidate for Pompe, BMN-103, ZC-701 has a faster clinical development timeline, lower development costs, significantly lower cost of goods and lower capital investment."

Mr. Bienaime continued, "As for the potential market opportunity, the incidence of Pompe is 1 in 40,000 births. The total market for Pompe is estimated at more than \$1.0 billion, assuming 3,000 to 6,000 patients have access to high value therapeutics and an average cost of therapy that is comparable to other enzyme replacement therapies. We look forward to keeping you updated on this and other programs in our pipeline."

Loren Peterson, President and Chief Executive Officer of ZyStor said, "We are very pleased to conclude this transaction with BioMarin for the development of ZC-701. BioMarin has a proven track record of successfully and expeditiously developing value-added therapies for orphan diseases, with particular strength in the field of enzyme replacement therapies for lysosomal storage disorders. Using our proprietary GILT technology, we believe that ZC-701 has the potential to be a more potent therapy for the treatment of Pompe disease."

### Conference Call Details

BioMarin will host a conference call and webcast today, Tuesday, August 17, at 5:00 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: August 17, 2010  
Time: 5:00 p.m. ET  
U.S. / Canada Dial-in Number: 866.783.2143  
International Dial-in Number: 857.350.1602  
Participant Code: 81596241  
Replay Dial-in Number: 888.286.8010  
Replay International Dial-in Number: 617.801.6888  
Replay Code: 32632590

### About Pompe Disease

Pompe disease, a lysosomal storage disorder, is a progressive degenerative disease of the heart muscle, diaphragm and skeletal muscle. It is caused by a deficiency in the lysosomal enzyme acid alpha glucosidase which leads to the accumulation of glycogen in myocyte lysosomes and results in cell death. The incidence is one in 40,000 births. There are two main forms of Pompe disease: adult onset with an incidence of one in 57,000 births and infantile onset with an incidence of one in 138,000 births. The current

standard of care is Genzyme's Myozyme and Lumizyme. Prognosis with standard of care is stabilization of the disease or minor improvements for the majority of adult onset patients.

### **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(R) (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme(R) (Iaronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; Kuvan(R) (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; and Firdapse(TM) (amifampridine phosphate), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Other product candidates include GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in clinical development for the treatment of MPS IVA and PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU. 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 ZyStor Therapeutics, Inc.**

ZyStor Therapeutics, Inc. is a privately held biotechnology company based in Milwaukee, Wisconsin developing a novel class of targeted protein therapeutics incorporating the company's proprietary Glycosylation Independent Lysosomal Targeting (GILT) technology. Instead of using mannose-6-phosphate (M6P) containing oligosaccharides to target the mannose-6-phosphate receptor and deliver proteins to lysosomes, this proprietary technology uses a peptide tag to target the M6P receptor with high affinity. ZyStor's lead product, ZC-701, has a much higher affinity for the M6P receptor compared to commercially available enzymes for Pompe disease and has been shown to be substantially more effective in clearing glycogen storage in the Pompe mouse model. In addition, preliminary studies indicate that GILT-tagged proteins actually induce more M6P receptor to appear on the cell surface compared to conventional replacement lysosomal enzymes, which may be particularly important for tissues that do not normally express high levels of M6P receptor, like skeletal muscle. GILT also offers substantial manufacturing advantages over making highly mannose-6-phosphorylated proteins. ZC-701 is a new molecular entity with unique composition of matter and claims to at least 2025, as well as 12 years of data exclusivity under the new healthcare reform. BMO Capital Markets acted as financial advisor to ZyStor in this transaction.

### **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 efficacy of ZC-701, an enzyme replacement therapy for the treatment of Pompe disease. 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, 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 2009 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.

BioMarin(R), Naglazyme(R) and Kuvan(R) are registered trademarks of BioMarin Pharmaceutical Inc.

Firdapse(TM) is a trademark of BioMarin Huxley Ltd.

Aldurazyme(R) is a registered trademark of BioMarin/Genzyme LLC.

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