BioMarin InvestorRoom

BioMarin Reports Encouraging Preliminary Data on BMN 110 for MPS IVA

Phase III Trial Expected to Start by Q4 2010 or Q1 2011 Conference Call and Webcast to Be Held Today at 5:00 p.m. ET

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NOVATO, Calif.

BioMarin Pharmaceutical Inc. today announced an update on the Phase I/II trial for BMN 110 or N-acetylgalactosamine 6-sulfatase (GALNS), intended for the treatment of the lysosomal storage disorder Mucopolysaccharidosis Type IVA (MPS IVA), or Morquio A Syndrome. Preliminary clinical data from the first 24 weeks of the study (12 weeks at 0.1mg/kg and 12 weeks at 1.0 mg/kg) have been evaluated, and BioMarin plans to announce top-line results for the full 36-week study after completion of dosing at 2.0 mg/kg in the second quarter of 2010.

Key Observations:

- -- Keratan sulfate (KS) levels fall within a few weeks after the start of therapy.
- -- Improvements in 6-minute walk distance and 3-minute stair climb at 24 weeks are consistent with those observed with clinical studies for MPS I, MPS II, and MPS VI.
- -- The frequency and severity of infusion reactions appear comparable to those observed with Naglazyme and Aldurazyme.

"Although still early, we are encouraged by these initial signals of efficacy of GALNS enzyme replacement therapy for Morquio disease. Additional results will become available following the 2.0 mg/kg dose phase, but compared to other studies we have conducted in MPS diseases, we feel encouraged by the reduction in KS and improvements in walk distance and stair climb. Based on these results, we feel more confident about endurance as a primary endpoint for a Phase III trial and that a Phase III trial can be conducted as expeditiously as previous trials of enzyme replacement therapy," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We plan to work closely with the FDA and other health authorities to finalize a Phase III protocol after the completion of the current study and have increased confidence that we will initiate a Phase III registration-enabling program by the fourth quarter of 2010 or the first quarter of 2011."

Jean-Jacques Bienaime, Chief Executive Officer of BioMarin added, "Based on these data, we are more optimistic about the GALNS program as we continue to move closer to providing a treatment option for Morquio patients. The number of Morquio patients identified already exceeds the number of MPS VI patients on Naglazyme, and an ERT for Morquio will fit perfectly into our global commercial infrastructure without the need for significant additional commercialization costs."

Mr. Bienaime continued, "2010 is shaping up to be an eventful year for BioMarin. In addition to results from the Morquio Phase I/II trial expected in the second quarter, we expect to report Phase II PEG-PAL results in mid-2010 and Phase I BMN 195 results in the third quarter of 2010. We look forward to keeping you updated on the progress of our clinical programs."

GALNS Phase I/II Clinical Trial Design

The Phase I/II study is designed as an open-label, within-patient dose escalation trial in approximately 20 patients followed by a treatment continuation phase. During the dose escalation phase of the study, subjects receive weekly intravenous infusions of GALNS in three consecutive 12-week dosing intervals: 0.1 mg/kg for twelve weeks, 1.0 mg/kg for twelve weeks and 2.0 mg/kg for twelve weeks. The objectives of the Phase I/II study are to evaluate safety, pharmacokinetics, pharmacodynamics, clinical response to therapy and to identify the optimal dose of GALNS for future studies.

The company has successfully developed and manufactures two FDA-approved enzyme replacement therapies for the treatment of MPS I and MPS VI. Naglazyme® (galsulfase) for MPS VI is wholly developed and commercialized by BioMarin. Aldurazyme® (laronidase) for MPS I is manufactured by BioMarin and marketed by Genzyme Corporation.

Conference Call Details

BioMarin will host a conference call and webcast to discuss the preliminary results of the Phase I/II trial of GALNS for MPS IVA today, Thursday, February 4, at 5:00 p.m. ET. This event can be accessed on the investor section of the BioMarin website at www.BMRN.com.

Date: February 4, 2010
Time: 5:00 p.m. ET
U.S. / Canada Dial-in Number: 800.299.9630
International Dial-in Number: 617.786.2904
Participant Code: 29296665
Replay Dial-in Number: 888.286.8010
Replay International Dial-in Number: 617.801.6888
Replay Code: 25068117

About MPS IV

Mucopolysaccharidosis IVA (MPS IVA, also known as Morquio A Syndrome) is a disease characterized by deficient activity of N-acetylgalactosamine-6-sulfatase (GALNS) causing excessive lysosomal storage of keratan sulfate (KS). This excessive storage causes a systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of the thorax impairs respiratory function, and odontoid hypoplasia and ligamentous laxity cause cervical spinal instability and potentially cord compression. Other symptoms may include hearing loss, corneal clouding, and heart valvular disease. Initial symptoms often become evident in the first five years of life. Depending on severity of the disease, age of diagnosis will vary.

The rate of incidence of MPS IVA is as yet unconfirmed and varies among different populations but estimates vary between 1 in 200,000 live births and 1 in 250,000 live births. There are several studies that have documented the incidence as high as 1 in 76,000 live births in Northern Ireland. The estimated prevalence is between 1,000 and 1,500 patients in the U.S., EU and Japan and between 1,500 to 2,000 patients in the rest of the world for a total of 2,500 to 3,000 patients. Approximately 400 patients worldwide are currently registered in The International Morquio Organization (IMO) survey and over 100 patients are already registered in the BioMarin MorCAP registry program.

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(TM) (amifampridine phosphate), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Other product candidates include PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU; GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase I/II clinical development for the treatment of MPS IVA and BMN 195, which is currently in Phase I clinical development for the treatment of Duchenne Muscular Dystrophy. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the development of its program for MPS IVA, and particularly the timing and conduct of clinical trials related thereto, expectations regarding other clinical and preclinical programs, and the potential market for GALNS, if approved. 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 results of current and planned pre-clinical trials related to BioMarin's development programs and particularly the enzyme replacement therapy for MPS IVA; the content and timing of decisions by the U.S. Food and Drug Administration, EMEA and other regulatory agencies, particularly with respect to the enzyme replacement therapy for MPS IVA, the actual number of MPS IVA patients in the developed world; 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. 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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Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC.

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