BioMarin Reports Positive Results for Phase I/II Trial for BMN 110 for MPS IVA

GALNS Demonstrates Benefits in Endurance and Pulmonary Function Endpoints Phase III Trial Expected to Start by Q4 2010 Conference Call and Webcast to Be Held Today at 5:00 p.m. ET

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BioMarin Pharmaceutical Inc. today announced positive results for 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. The company expects to initiate a pivotal Phase III trial in the fourth quarter of 2010.

Highlights from the Phase I/II study:

-- Endurance improvements with GALNS were consistent with, and in some cases, better than those observed in pivotal studies of approved enzyme replacement therapies.
-- Clinically meaningful improvements in two measures of endurance (6-minute walk distance and 3-minute stair climb) were achieved at both 24 weeks and 36 weeks as compared to baseline. (See Table 1 below).
-- Clinically meaningful improvements in two measures of pulmonary function (forced vital capacity and maximum voluntary ventilation) were achieved at 36 weeks as compared to baseline. (See Table 1 below).
-- Keratan sulfate levels decreased shortly after the initiation of treatment and fell further as the study progressed. (See Table 1 below).
-- The frequency and severity of infusion reactions were comparable to those observed with Naglazyme and Aldurazyme.

"We are encouraged that clinically significant improvements in endurance and pulmonary function can be detected with GALNS treatment even in a relatively short study of a heterogeneous population. This gives us confidence that we can design a robust 24 to 36 week Phase III clinical study with a measure of endurance as the primary endpoint and several additional supportive endpoints. We will be working to reach an agreement with regulatory authorities on a powerful and large Phase III trial protocol that minimizes regulatory risk and captures the maximum amount of clinical benefit expeditiously," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We are on track to initiate a Phase III registration-enabling program in the fourth quarter of 2010."

Dr. Chris Hendriksz, Clinical Lead for Inherited Metabolic Disorders and Director for Lysosomal Storage Disorders at Birmingham Children's Hospital in Birmingham, UK added, "The data from the Phase I/II trial are extremely encouraging as they clearly demonstrate that GALNS has an important, positive effect. The data supports my long-held observation that this disorder is much more than only a bone disease. The greatest misconception about Morquio is that it is a skeletal dysplasia only disease and that there is very little to gain from therapy. Patients with Morquio look forward to breathing easier, playing with their friends outside and living a more normal life. Patients experienced benefits that were quite remarkable and evidently rather quickly. This is an exciting milestone for the MPS IV community as BioMarin's program moves one step closer to providing the first much-needed and possibly life-changing treatment option for Morquio patients."

Jean-Jacques Bienaime, Chief Executive Officer of BioMarin added, "Assuming the GALNS clinical program is successful, we are very optimistic about the commercial potential of the product. There are over 600 MPS IV patients identified to date which is more than the number of patients on commercial Naglazyme therapy. Since there is no neurocognitive component to the disease and no competing treatment, we expect that all patients may be candidates for therapy. The large identified patient base also provides an accessible pool from which to draw patients for the Phase III trial. GALNS is not only our most advanced clinical program, it also has the potential to be our largest and most profitable product because of the manufacturing and commercial infrastructure that is already in place."

Table 1. Overall Summary of Efficacy Data

	Statistic	Week 24	Week 36
Endurance	N	15	15
Change in 3 Minute Stair Climb from Baseline (stairs per minute)	Mean (p-value)	6.9 (p=0.01)	8.9 (p=0.03)
	Median (p-value)	10.3	7.3 (p=0.01) (p=0.06)
Change in 6 Minute Walk Test from Baseline (meters)	N	16	16
	Mean	17 (p=0.36)	15 (p=0.38)
	Median	38 (p=0.09)	19 (p=0.34)
Change in 6 Minute Walk Test from Baseline in Patients with Baseline Walk Distance </= 325 meters(1) (meters)	N	12	12
	Mean	38 (p=0.01)	28 (p=0.14)
	Median	42 (p=0.02)	24 (p=0.18)
Biomarker	N	18	18
Percent Change in Urine KS(2) (% decrease from Baseline)	Mean	28% (p<0.01)	41% (p<0.01)
	Median	31% (p<0.01)	47% (p<0.01)
Pulmonary function	N	16	16
Percent Increase in FVC(3)	Mean	<1% (p=0.98)	11% (p=0.06)
	Median	<1% (p=0.85)	11% (p=0.01)
Percent Increase in MVV(4)	N	13	14
	Mean	11% (p=0.09)	11% (p=0.04)
	Median	6.4% (p=0.15)	10% (p=0.06)

(1) The Phase I/II trial for GALNS did not select for baseline walk distance and consequently, a relatively healthier patient population was enrolled as compared to the Naglazyme pivotal Phase III trial. The Naglazyme pivotal Phase III trial had an inclusion criterion of baseline 6-minute walk distance no greater than 270 meters. As a basis for comparison, for 6-minute walk distance at 24 weeks, the Naglazyme mean change from baseline was 48 meters, and the median change from baseline was 19 meters.

(2) keratan sulfate; (3) forced vital capacity; (4) maximum voluntary ventilation

T-test for mean comparison; Wilcoxon signed-rank test for median comparison

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 results of the Phase I/II trial of GALNS for MPS IVA today, Thursday, April 29, at 5:00 p.m. ET. Dr. Chris Hendriksz, Clinical Lead for Inherited Metabolic Disorders and Director for Lysosomal Storage Disorders at Birmingham Children's Hospital will participate in the call. This event can be accessed on the investor section of the BioMarin website at www.BMRN.com.

Date: April 29, 2010
Time: 5:00 p.m. ET
U.S. / Canada Dial-in Number: 800.804.6921
International Dial-in Number: 857.350.1667
Participant Code: 38613991
Replay Dial-in Number: 888.286.8010
Replay International Dial-in Number: 617.801.6888
Replay Code: 77798822

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. Over 400 patients worldwide are currently registered in The International Morquio Organization (IMO) survey and nearly 200 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 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 2009 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.

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

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