BioMarin Phase 3 Study of GALNS for the Treatment of MPS IVA Meets Primary Endpoint

Company Plans to Submit Marketing Applications Starting in 1Q 2013
Conference Call and Webcast to be Held Today at 8:30 a.m. ET

SAN RAFAEL, Calif., Nov. 5, 2012 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the pivotal Phase 3 study of GALNS met the primary endpoint of change in six-minute walk distance compared with placebo at 24 weeks in subjects receiving weekly infusions of GALNS at the dose of 2 mg/kg (p=0.0174). MOR-004 was a randomized, double-blind, placebo-controlled study evaluating two doses of GALNS (BMN-110, N- acetylgalactosamine-6-sulfatase) for the treatment of patients with the rare lysosomal storage disorder Mucopolysaccharidosis Type IVA (MPS IVA), also called Morquio A Syndrome. Patients dosed with GALNS at 2 mg/kg every other week did not show a meaningful or statistically significant change from baseline compared to placebo. The company also announced preliminary data from the MOR-005 extension study which suggests that clinical benefits continue to improve with further dosing with GALNS. Only a limited number of patients have reached the 36 or 48 week points of total time on treatment in the extension study, and the results will be updated when the study is completed. The company confirmed that based on the results of MOR-004, and following planned discussions with regulatory authorities, it expects to submit marketing applications starting in the first quarter of 2013.

Treatment with GALNS Significantly Improves Primary Endpoint

The primary endpoint of the study, change in six-minute walk distance at 24 weeks, was statistically significant in patients dosed with GALNS at 2 mg/kg every week with a mean increase of 22.5 meters (p=0.0174) over placebo. In MOR-004, patients dosed at 2 mg/kg every week showed an improvement in six-minute walk distance at week 12 compared to baseline and showed continued improvement at week 24. Preliminary analysis of a subset of the patients in the MOR-005 extension study who have reached the 36 week and 48 week timepoints in the study also showed further improvement at weeks 36 and 48.

Treatment with GALNS Improves Both Secondary Endpoints

On the secondary endpoint of three-minute stair climb, patients dosed with GALNS at 2 mg/kg every week showed a trend toward improvement at 24 weeks of 1.1 additional stairs per minute over placebo. In MOR-004, patients dosed with GALNS at 2 mg/kg every week showed an improvement in three-minute stair climb performance at week 12 compared to baseline and showed continued improvement at week 24. Preliminary analysis of a subset of patients in the extension study (MOR-005) who have reached the 36 week and 48 week timepoints in the study also showed further improvement in three-minute stair climb performance.

In the other secondary endpoint, urinary keratan sulfate (KS) levels, patients dosed with GALNS at 2 mg/kg every week showed consistent and robust reduction in urinary KS with a mean difference from baseline as compared to placebo of 40.7 percent (p less than 0.0001). Preliminary analysis of a subset of patients in the extension study (MOR-005) who have reached the 36 week and 48 week timepoints in the study showed this level of reduction was maintained.

Treatment with GALNS Improves Pulmonary Function

Pulmonary function, as defined by maximum voluntary ventilation (MVV) was measured at 24 weeks. In MOR-004, patients dosed with GALNS at 2 mg/kg every week showed a trend toward improvement from baseline of 10.3 percent over placebo. Preliminary analysis of the subset of patients who reached the 48 week timepoint showed a reduction in the improvement, though an increase over baseline was maintained.

Pulmonary function, as defined by forced vital capacity (FVC) was measured at 24 weeks. In MOR-004, patients dosed with GALNS at 2 mg/kg every week showed a trend toward improvement from baseline of 3.3 percent over placebo. Preliminary analysis of the subset of patients who reached the 48 week timepoint showed continued improvement.

Safety Summary

In MOR-004, GALNS was generally well-tolerated and adverse events were similar to those seen in clinical trials of other enzyme replacement therapies. The most common adverse events occurring in more than 25 percent of treated patients included vomiting, pyrexia, headache, nausea and cough. Serious adverse events that were thought to be related to study drug occurred in 3.4 percent of the weekly group, 1.7 percent of the every other week group and 0 percent in the placebo group. There were no deaths and no patients withdrew from the study

due to an adverse event.

Infusion-associated reactions were generally mild to moderate and manageable with symptomatic treatment and/or infusion rate modification. Of the 1,345 total number of infusions in the weekly dose group, 17 infusions (1.3 percent) were interrupted or discontinued due to an adverse event. All patients subsequently resumed dosing.

"The positive results from this pivotal study will help support GALNS as the first therapy available to help the approximate 3,000 people worldwide suffering from MPS IVA -- a rare, degenerative, life-threatening genetic condition with no available therapy," said Hank Fuchs, M.D., Chief Medical Officer at BioMarin. "We are very pleased with the clarity that the MOR-004 study has provided us with respect to the appropriate dosing of GALNS. The weekly 2 mg/kg dose provided a statistically significant and clinically meaningful improvement in the study's primary endpoint, and positive trends toward improvement in other clinically meaningful endpoints, including three-minute stair climb and pulmonary function tests. By contrast, the 2 mg/kg every other week dose was shown to be similar to placebo on the primary and clinical secondary and tertiary endpoints. We look forward to reviewing the results of this study with regulatory authorities, and applying for marketing authorizations starting in the first quarter of 2013."

"The GALNS clinical program is currently the highest development priority at BioMarin, and this positive Phase 3 study serves as a potentially transformative milestone for the company," said Jean-Jacques Bienaimé, CEO of BioMarin. "We are applying our track record of success in developing novel treatments for orphan diseases and our existing commercial infrastructure for Naglazyme to bring GALNS to patients as rapidly as we can."

Top-line Results: Primary, and Secondary and Tertiary Endpoints

6 Minute Walk Distance

	Change from Baseline in Meters (Observed data)			**Change vs Placebo** (Modeled treatment effect)
	Placebo	**2.0 mg/kg/every other week**	**2.0 mg/kg/ weekly**	**2.0 mg/kg/weekly**
MOR-004				
Week 12				
Mean	12.7	13.5	23.7	
n	59	59	58	
Week 24				
Mean	13.5	14.9	36.5	22.5
n	59	58	57	p=0.0174*
MOR-005 (Interim data)				
Week 36				
Mean	N/A	17.0	46.6	
n	N/A	27	27	
Week 48				
Mean	N/A	3.8	45.4	
n	N/A	18	13	

3 Minute Stair Climb

	Change from Baseline in Stairs per

	Minute (Observed data)			Change vs Placebo (Modeled treatment effect)
	Placebo	**2.0 mg/kg/every other week**	**2.0 mg/kg/ weekly**	**2.0 mg/kg/weekly**
MOR-004				
Week 12				
Mean	2.9	3.6	3.6	
n	59	59	58	
Week 24				
Mean	3.6	3.4	4.8	1.1
n	59	58	57	p=0.4935*
MOR-005 (Interim data)				
Week 36				
Mean	N/A	5.3	6.5	
n	N/A	27	27	
Week 48				
Mean	N/A	5.8	7.1	
n	N/A	18	16	

Urinary Keratan Sulfate				
	Percentage Change from Baseline (ug/mg) (Observed data)			**% Change vs Placebo** (Modeled treatment effect)
	Placebo	**2.0 mg/kg/every other week**	**2.0 mg/kg/ weekly**	**2.0 mg/kg/ weekly**
MOR-004				
Week 12				
Mean	(1.6)	(20.9)	(41.8)	
n	56	58	55	
Week 24				
Mean	(4.4)	(35.2)	(45.1)	(40.7)
n	55	57	54	p less than 0.0001*
MOR-005 (Interim data)				
Week 36				
Mean	N/A	(30.2)	(45.8)	

n	N/A	25	19	
Week 48				
Mean	N/A	(31.7)	(45.9)	
n	N/A	9	10	

Pulmonary Function

	Percentage Change from Baseline (Observed data)			**% Change vs Placebo** (Modeled treatment effect)
	Placebo	**2.0 mg/kg/every other week**	**2.0 mg/kg/ weekly**	**2.0 mg/kg/ weekly**
Maximum Voluntary Ventilation (MVV)				
MOR-004				
Week 24				
Mean	2.4	6.1	10.8	10.3
n	50	52	49	p=0.0943*
MOR-005 (Interim data)				
Week 48				
Mean	N/A	15.5	1.1	
n	N/A	16	9	
Forced Vital Capacity (FVC)				
MOR-004				
Week 24				
Mean	1.5	4.1	4.9	3.3
n	53	55	55	p=0.3041*
MOR-005 (Interim data)				
Week 48				
Mean	N/A	6.3	6.1	
n	N/A	16	12	

* P-values are based on the pre-specified primary statistical analysis model. Mean changes vs placebo are the treatment effects based on this model and may differ slightly from the means of the observed data.

Study Design

The MOR-004 Phase 3 study was a randomized, double-blind placebo-controlled clinical study to evaluate the efficacy and safety of 2.0 mg/kg/week and 2.0 mg/kg/every other week of GALNS in patients with MPS IVA. The primary endpoint was the change in six-minute walk distance from baseline to week 24 compared to placebo, and the secondary endpoints were the changes in three-minute stair climb test and urine KS levels compared to placebo.

MOR-005 is the extension study for the pivotal Phase 3 study for GALNS (MOR-004). Patients from the two treatment arms of 2 mg/kg/week and 2 mg/kg/every other week remained on the same dose, and the placebo patients were randomized into one of the treatment groups. Preliminary data presented in this press release includes only patients from the two active treatment arms of MOR-004 who have reached 36 and 48 weeks of total treatment time as of September 14, 2012.

To be eligible for the study, subjects had to be at least five years of age, have a documented clinical diagnosis of MPS IVA and have an average screening six-minute walk test distance ≥30 meters and ≤325 meters.

This is the largest Phase 3 enzyme replacement therapy (ERT) study to date with 176 patients treated at 31 sites in 17 countries.

The baseline measurements, including age, six-minute walk distance, three-minute stair climb and urinary KS were well-balanced across the three arms of the study.

Results presented are preliminary and subject to final analysis. Complete results will be presented at the WORLD Symposium in mid-February 2013.

Conference Call Details

BioMarin will host a conference call and webcast to discuss results for the GALNS Phase 3 trial today, Monday, November 5, at 8:30 a.m. ET. This event can be accessed on the investor section of the BioMarin website at www.BMRN.com.

Date: November 5, 2012
Time: 8:30 a.m. ET
U.S. / Canada Dial-in Number: 877.303.6313
International Dial-in Number: 631.813.4734
Conference ID: 64771689

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 64771689

About MPS IVA

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. 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.

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 GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase III clinical development for the treatment of MPS IVA, amifampridine phosphate (3,4-diaminopyridine phosphate), which is currently in Phase III clinical development for the treatment of LEMS in the U.S., 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-nutriuretic 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: the expectations related to the continued clinical development of its product candidate GALNS; expectations regarding the final analysis of the GALNS Phase 3 clinical trial data; expectations for the results of the extension trial of 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 of GALNS; the final analysis of the results of the Phase 3 trial of GALNS; our ability to successfully manufacture our products and product candidates; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning GALNS 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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