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Press Release

BioMarin Provides Update on GALNS Phase 1/2 Extension Study (MOR-100)

Ongoing study suggests GALNS sustains improvements in endurance and respiratory function for at least 2 years

NOVATO, Calif., July 28, 2011 /PRNewswire via COMTEX/ --

BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) today announced an update on the GALNS Phase 1/2 extension study (MOR-100) in which patients have continued treatment on an ongoing basis.

Patients originally enrolled in the initial Phase 1/2 study of GALNS (MOR-002) were continued on therapy in a new extension study (MOR-100). In the MOR-100 study, patients were treated at a 2.0 mg/kg/week dose and have been followed for an additional 24 weeks. Previously, these patients were dosed at 0.1 mg/kg/week, 1.0 mg/kg/week and 2.0 mg/kg/week for 12 weeks each in the Phase 1/2 study (MOR-002). Following the dose escalation phase, patients were then dosed at 1.0 mg/kg/week for an additional 36 to 48 weeks as part of the MOR-002 extension, before continuing on to MOR-100. Taken together, these patients have received GALNS for approximately two years.

"We now have data from MPS IVA patients that have been on GALNS for approximately 2 years and the data continues to look promising," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "Looking at the Phase 1/2 data in totality, we now see that the endurance and pulmonary function improvements are durable and clinically meaningful compared to baseline, and similar in magnitude to what we have observed during the development of Naglazyme and Aldurazyme. These data are encouraging in regard to the ongoing Phase 3 study in that the benefits of GALNS appear consistently sustained, and no new risks have emerged. Enrollment in the Phase 3 study is accelerating and its execution is BioMarin's highest product development priority."

Preliminary highlights, including information on patients treated for 24 additional weeks at 2.0 mg/kg/week are provided below. Data from the extension study will be submitted for presentation at the World Lysosomal Storage Disease conference in February 2012.

Highlights of MOR-100 Extension Study:

- No new or consistent patterns of clinically meaningful or treatment limiting adverse events were reported.
- Measures of 6-minute walk distance, 3-minute stair climb and pulmonary function all generally improved and were sustained for at least 2 years.
- For the group of patients with baseline 6-minute walk distance ≤325 meters, mean walk distance remains consistently improved for the entire period of observation. Specifically, 24 weeks after resuming 2.0 mg/kg/week dosing in MOR-100, the mean 6-minute walk distance was 38 meters. This is roughly equivalent to that observed after the first 24 weeks of the original Phase 1/2 trial (MOR-002). The ongoing Phase 3 study is using mean improvement in 6-minute walk distance in patients with baseline walk distance ≤325 meters as the primary endpoint.
- 6-minute walk distance improvements were greater in the subgroup of patients with baseline walk distance ≤325 meters. However, variability of this test increases with longer-term follow-up, likely as a result of issues related to the progressive nature of Morquio syndrome. Several measures to reduce variability have already been incorporated in the ongoing Phase 3 trial: large sample size, selection of patients not expected to require surgical correction of skeletal deformity in the subsequent 24 weeks, duplicate measures of 6-minute walk test, conclusion of the trial at 24 weeks, extensive oversight of the performance of the walk test according to ATS criteria and inclusion of placebo for comparative purposes.
- 3-minute stair climb and pulmonary function have consistently improved over the entire duration of the study. 24 weeks after returning to 2.0 mg/kg, patients can now climb a mean of 13.6 stairs per minute and FVC has improved a mean of 15.9%, compared to 6.9 stairs per minute and 1.5% reported at the end of the first 24 weeks of the Phase 1/2 trial. Stair climb and pulmonary function are secondary and tertiary endpoints, respectively, in the ongoing prospective Phase 3 trial.
- The data from the extension study to date support 6-minute walk distance as the primary endpoint and patients with baseline walk distance ≤325 meters as an entry criteria in the ongoing GALNS Phase 3 trial, given the large and durable effect of the drug on these measures in this population.

Simon Jones, Consultant in Paediatric Metabolic Medicine, St. Mary's Hospital, Manchester UK commented, "I'm pleased with the outcome of the GALNS program to date. The longer-term data look good with sustained improvements in endurance and pulmonary function. Patients feel stronger and function better than I expected. All in all, we look forward to the results of the Phase 3 study as it is well designed and will, if successful, support introduction of an important product to patients in need."

Hank Fuchs, M.D., Chief Medical Officer of BioMarin added, "It's remarkable that improvements in endurance are sustained in spite of the severe and progressive nature of Morquio syndrome. Patients with Morquio syndrome can miss therapy, for example, due to the need for corrective surgery. In spite of that, resumption of therapy returns patients to their original state of health or better."

Table 1 - Summary of Efficacy Results Through Week 24 of MOR-100 Extension Study

	Statistic	MOR-002 Week 24	MOR-002 Week 36	MOR-100 Week 0	MOR-100 Week 12	MOR-100 Week 24
	Dose	1.0 mg/kg	2.0 mg/kg	1.0 mg/kg for previous 36-48 weeks, Begin 2.0 mg/kg	2.0 mg/kg	2.0 mg/kg
ENDURANCE						
6 Minute Walk Test - change from Baseline (meters)	N Mean (Std. Dev.) Median	16 17.3 (74.0) 37.6	16 14.6 (65.2) 18.9	15 13.9 (98.6) 14.0	15 21.8 (85.4) 15.7	14 29.1 (79.1) 6.8
6 Minute Walk Test - change from Baseline (meters) (for patients with baseline walk distance ≤ 325m)	N Mean (Std. Dev.) Median	12 38.0 (45.2) 42.4	12 28.2 (61.0) 24.4	11 37.3 (79.8) 14.0	11 40.0 (77.0) 15.7	11 38.3 (81.8) 2.6
3 Minute Stair Climb Test - change from Baseline (steps/minute)	N Mean (Std. Dev.) Median	15 6.9 (8.9) 7.3	15 8.9 (14.3) 10.2	14 14.5 (14.3) 13.1	14 13.9 (15.2) 14.6	12 13.6 (19.6) 10.4
PULMONARY FUNCTION						
FVC - Percent Change from Baseline	N Mean (Std. Dev.) Median	15 1.5 (16.2) 0.0	15 7.2 (16.0) 10.0	14 11.5 (15.5) 6.9	N/A	13 15.9 (16.8) 12.0
MVV - Percent Change from Baseline	N Mean (Std. Dev.) Median	13 11.0 (21.5) 6.4	14 10.5 (17.4) 10.0	13 11.1 (16.4) 10.6	N/A	12 10.3 (23.2) 0.8

***** After Week 36 in MOR-002, patients were dosed at 1.0 mg/kg/week for 36 to 48 weeks. At Week 0 of MOR-100, patients were returned to 2.0 mg/kg/week dosing.
** Results were impacted by several patients with extenuating circumstances (knee procedures, car accident, unplanned extended drug holiday) after week 24 of MOR-002.

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), 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, and 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. 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, including, without limitation, statements about: the expectations related to the continued clinical development of its product candidate 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; 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 2010 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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