BioMarin Provides Preliminary Data From Ongoing Phase 1/2 Pivotal Study of BMN 190 for Treatment of CLN2 Disorder, a Form of Batten Disease

Preliminary Results Show Evidence of Disease Stabilization

SAN RAFAEL, Calif., Jan. 12, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced interim results from its Phase 1/2 pivotal study for BMN 190 or cerliponase alfa, a recombinant human tripeptidyl peptidase 1 (rhTPP1), to treat of patients with late infantile CLN2 disease, a form of Batten disease. Interim data indicates that in all nine of the BMN 190 patients who have been followed for at least six months and up to 15 months, the treatment appears to show stabilization of the disease compared to the natural history based on a standardized measure of motor and language function.

The primary end point of the study is a standardized mobility and language score using a CLN2-specific rating scale. The scale separately measures performance of mobility and language with normal function in each being a score of three and no function being a score of zero. The highest score possible is six.

According to data from a natural history study of the disease, patients generally lose one point every six months and generally lose most language and mobility functioning over a two to four year period in this rapidly progressive disease. In the nine BMN 190 patients treated for more than six months and up to 15 months, six patients showed no net change in their CLN2 rating scale score, while the other three showed a decline of one point.

In addition, seven of the nine patients in the BMN 190 study for more than six months were matched to between one and 12 individuals from the natural history data set according to baseline age and disease severity. (For two of the BMN 190 patients, there is no matched patient in the natural history database based on age and disease severity.) All seven BMN 190 patients with six months of treatment and at least one control match had better walk/talk scores as of their last evaluation than their natural history counterparts.

Additional detailed information on the interim preliminary results from the nine patients who have been on BMN 190 for more than six months and their matched natural history counterparts can be found at http://www.bmrn.com/pdf/JPMPresentation011215.pdf.

"This trial represents the essence of BioMarin's commitment to patients with fatal rare diseases and no treatment options. This initial look at the data is encouraging, and this therapy may make a meaningful difference for children with this form of Batten disease. We look forward to working with the regulatory authorities to determine if this single study will support regulatory approval as quickly as possible," said Jean-Jacques Bienaimé, Chief Executive Officer.

There is no approved treatment that can prevent, stop, or reverse CLN2 disorder. Palliative care—to reduce seizures—and physical rehabilitative care—to help children retain muscle function for as long as possible—are currently the available treatment options for patients with this rare disease.

"This interim data represents an important step on a journey to develop a treatment for CLN2 disorder that may be able to slow the course of this fatal disease," said Angela Schulz, M.D. Ph.D., Department of Paediatrics, University Medical Center Hamburg-Eppendorf. "We appreciate the commitment of the children and their families who are participating in this study."

"We welcome BioMarin's update on nine of the 24 patients participating in this clinical trial and appreciate the efforts they are making in developing a treatment for this deadly ultra-rare disease," said Andrea West, Chief Executive, Batten Disease Family Association.

"We are pleased about the progress that BioMarin is making in developing a treatment for CLN2 disorder," said Margie Frazier, Ph.D., Executive Director, Batten Disease Support and Research Association. "This is a notable moment, which holds promise for the children and families who are affected by this disease."

The Phase 1/2 pivotal study is an open-label, dose-escalation study in patients with late infantile CLN2 disease, a form of Batten disease. The primary objectives are to evaluate the safety and tolerability of BMN 190 or cerliponase alfa and to evaluate effectiveness using a CLN2 disorder-specific rating scale score in comparison with natural history data after 48 weeks of treatment. Secondary objectives are to evaluate the impact of treatment on brain atrophy in comparison with CLN2 natural history after 48 weeks of treatment and to characterize pharmacokinetics and immunogenicity. The study enrolled 24 subjects at five clinical sites for a

planned treatment duration of 48 weeks. Complete results are expected in Q4 2015.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five approved products and multiple clinical and pre-clinical product candidates. Approved products include VIMIZIM® (elosulfase alfa) for MPS IVA, a product wholly developed and commercialized by BioMarin; Naglazyme® (galsulfase) for MPS VI, a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for MPS I, a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; KUVAN® (sapropterin dihydrochloride) Powder for Oral Solution and 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 pegvaliase (PEGylated recombinant phenylalanine ammonia lyase, formerly referred to as BMN 165 or PEG PAL), which is currently in Phase 3 clinical development for the treatment of PKU, talazoparib (formerly referred to as BMN 673), a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer, reveglucosidase alfa (formerly referred to as BMN 701), a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase 3 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia, cerliponase alfa (formerly referred to as BMN 190), a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of CLN2 disorder, a form of Batten disease, which is currently in Phase 1, BMN 270, an AAV-factor VIII vector, for the treatment of hemophilia A and BMN 250, a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of MPS IIIB.

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: BioMarin's development programs for BMN 190 or cerliponase alfa, and generally the timing and results of the Phase 1/2 pivotal trial of BMN 190 or cerliponase alfa. 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 clinical trials of BMN 190 or cerliponase alfa; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; 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 2013 Annual Report on Form 10-K, as amended, and the factors contained in BioMarin's reports on Form 8-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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