BioMarin Launches kNOWyourDuchenne, Comprehensive Program for Patients With Duchenne Muscular Dystrophy to Understand Their Genetic Mutation

Adds Novel Program to Established Offering of Personalized Patient Support Services

SAN RAFAEL, Calif., Sept. 1, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced the introduction of kNOWyourDuchenne, a program to help families and physicians obtain and interpret genetic testing for patients with Duchenne muscular dystrophy. This novel program will identify specific genetic mutations leading to Duchenne, which may help guide clinicians and caregivers to potential treatments in development. Interpretation of the test will be performed by an independent lab director. Currently, genetic test results may not fully inform families or physicians of what potential treatments a child might be eligible for on the basis of that child's mutation. Duchenne muscular dystrophy is the most common fatal genetic disorder diagnosed in childhood, affecting approximately 1 in every 3,500 live male births.

"Creating the kNOWyourDuchenne program fits with BioMarin's mission to provide highly personalized support and services to patients," said Chuck Bucklar, Vice President Commercial Operations. "Every family with Duchenne has specific needs unique to them, and BioMarin has a decade of experience in tailoring support services based on individual need. The first step to provide support to patients with Duchenne is to make sure that each person with Duchenne is tested and has access to an independent genetic counselor to provide an accurate interpretation of the test results."

BioMarin will provide genetic interpretation and testing services through

kNOWyourDuchenne to patients and families at no cost throughout the duration of the program. kNOWyourDuchenne is accessible to eligible Duchenne patients.

"It is important for children and their families with Duchenne muscular dystrophy to know their mutation in order to understand if they qualify for a clinical trial, or to know if they might be candidates for future treatments. Having access to an expert to interpret the genetic test results is critical to determine the most appropriate care," said Patrick Pallansch, Vice President, Medical Affairs.

Today, there are no treatments available in the US to target the genetic basis of Duchenne muscular dystrophy. Potential new therapies that target the cause of the disease are being studied. Patients, families, and physicians need to know the specific genetic mutation and understand what potential targeted therapy might address the underlying genetic cause of the disease. BioMarin is developing new treatments to target the underlying genetic cause of Duchenne, which would require a genetic test to determine if a patient might benefit from a treatment.

BioMarin has an established track record of providing support services to patients, including diagnostics for the diseases where it is developing treatments or is providing approved treatments for rare genetic diseases. Since 2005, BioMarin's case management system has been assisting patients and physicians to access therapy, to navigate insurance reimbursement processes and to provide diagnostic services to confirm disease diagnosis. The kNOWyourDuchenne program builds on BioMarin's expertise in personalized patient support services.

To access information about kNOWyourDuchenne and request access to genetic interpretation and testing services, go

to www.kNOWyourDuchenne.com or call 1-844-DUCHENNE (382-4366).

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a severely debilitating childhood neuromuscular disease caused by mutations in the dystrophin gene. This results in the absence or defect of the dystrophin protein, which is important in connecting the cytoskeleton of muscle fibers to the extracellular matrix. As a result, patients suffer from progressive loss of muscle strength, often rendering them wheelchair-bound before the age of 12 years. Respiratory and cardiac muscle can also be affected by the disease and most patients die in early adulthood due to respiratory and cardiac failure. Because the Duchenne gene is found on the X-chromosome, it primarily affects boys.

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 drisapersen, an exon skipping oligonucleotide, for which a marketing application has been

submitted to FDA and EMA for the treatment of patients with Duchenne muscular dystrophy (DMD) with mutations in the dystrophin gene that are amenable to treatment with exon 51 skipping, 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, BMN 044, BMN 045 and BMN 053, exon skipping oligonucleotides, which are currently in Phase 2 clinical development for the treatment of Duchenne muscular dystrophy (exons 44, 45 and 53), 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: kNOWyourDuchenne, a program to provide genetic testing and interpretation of test results and expectations about new treatments for

Duchenne muscular dystrophy. 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: our ability to enroll sufficient numbers of test centers, obtaining appropriate institutional and governmental approvals, the number of patients and physicians who use the kNOWyourDuchenne program; 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 2014 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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