BioMarin Announces EMA Grants Accelerated Assessment for Cerliponase Alfa, Experimental Treatment for a Form of Batten Disease

EU and U.S. Marketing Application Submissions Planned for Mid-Year 2016
Company Seeking Priority Review in the U.S.

SAN RAFAEL, Calif., May 03, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the European Medicines Agency (EMA) has granted BioMarin's request for accelerated assessment for the planned cerliponase alfa Marketing Authorization Application (MAA). Accelerated assessments are granted on the grounds that a product may satisfy an unmet medical need and is of major interest from the point of view of therapeutic innovation and public health. Accelerated assessment has the potential to shorten EMA's review procedure. However, at any time during the MAA assessment, the EMA may decide to continue the assessment under standard assessment timelines, and most applications that initially qualify for accelerated assessment are ultimately reviewed on a standard timeline.

Cerliponase alfa is a recombinant human tripeptidyl peptidase 1 (rhTPP1) intended for the treatment of children with CLN2 disease, a form of Batten disease. CLN2 disease is a rapidly progressing, fatal neurodegenerative disease with no approved treatments.

The company expects to submit the cerliponase alfa MAA to the EMA and the Biologics License Application to the U.S. Food and Drug Administration (FDA) by mid-year. If the cerliponase alfa MAA is accepted by the EMA, then an opinion from the Committee for Medicinal Products for Human Use (CHMP) is anticipated in the first quarter of 2017, and, if positive, a decision from the European Commission could be received in the first half of 2017.

"We are pleased that the EMA has recognized the need to bring a therapy to children with this particular form of Batten disease as quickly as possible. This could be the first therapeutic option available for these children," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin. "We look forward to entering the regulatory review phase for cerliponase alfa on a global basis. CLN2 disease represents a significant unmet medical need for those affected, and our hope is to offer a life-altering treatment option to patients worldwide."

In the U.S., BioMarin is seeking to shorten the regulatory review time by requesting Priority Review. Priority Review status is designated by the FDA to drugs that offer major advances in treatment or provide a treatment where no adequate therapy exists. The FDA will evaluate this request following submission of the planned Biologics License Application (BLA). Cerliponase alfa has been granted Orphan Drug Designation by the FDA and EMA and Breakthrough Therapy designation by the FDA.

Early Access Program

BioMarin is planning to implement an early access program to provide limited access to treatment for additional CLN2 patients prior to obtaining marketing approval. An early access program will be limited in scope and number of participants, and will be conducted under a protocol. We expect that the program will be conducted at centers that have participated in the cerliponase alfa study. Those sites have experience administering this drug directly to the brain and would ensure continued patient monitoring. The program is expected to begin in at least one site by the end of Q3 2016, and the timing of additional sites will vary by country and individual site. The overall scope, eligibility criteria and details of this program are still being determined. BioMarin must adhere to specific legal procedures for each country and has begun these preparations at risk with the goal of being ready to dose patients by the end of Q3 2016. BioMarin will provide additional details about the scope and timing of this program as they become available.

About Cerliponase Alfa

Cerliponase alfa is a recombinant form of human TPP1, the enzyme deficient in patients with CLN2 disease. It is an enzyme replacement therapy designed to restore TPP1 enzyme activity and break down the storage materials that cause CLN2 disease. In order to reach the cells of the brain and central nervous system, the treatment is delivered directly to the fluid surrounding the brain (cerebrospinal fluid) using BioMarin's patented technology.

For additional information regarding the investigational product cerliponase alfa, please contact BioMarin Medical Information at medinfo@bmrn.com.

About CLN2 Disease

The neuronal ceroid lipofuscinoses (NCLs) are a heterogeneous group of lysosomal storage disorders that includes the autosomal recessive neurodegenerative disorder CLN2 disease. CLN2 disease is caused by mutations in the TPP1/CLN2 gene resulting in deficient activity of the enzyme tripeptidyl peptidase 1 (TPP1). In the absence of TPP1, lysosomal storage materials normally metabolized by this enzyme accumulate in many organs, particularly in the brain and retina.

Buildup of these storage materials in the cells of the nervous system contribute to the progressive and relentless neurodegeneration which manifests as loss of cognitive, motor, and visual functions. Disease progression is rapid. The onset of symptoms is typically between ages two and four. Patients typically present initially with language delay and seizures, followed by movement disorders, motor deterioration, dementia, blindness and early death. During the later stages of the disease, feeding and tending to everyday needs become very difficult and death typically occurs between ten and 16 years of age.

There is no approved treatment that can prevent, stop, or reverse CLN2 disease. Symptomatic care to treat the symptoms of the disease, prevent and treat complications, and attempt to preserve quality of life is the only available treatment options for patients with this rare disease.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. The company's portfolio consists of five commercialized products and multiple clinical and pre-clinical product candidates.

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 cerliponase alfa generally, the expectations regarding the impact of EMA Accelerated Assessment program, timing of regulatory filings with the FDA and EMA and its global regulatory process in general. 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 of current and planned clinical trials of cerliponase alfa; the content and timing of decisions by the FDA and EMA and other regulatory authorities; our ability to manufacture sufficient quantities of cerliponase alfa for clinical trials, commercial launch and other preapproval requirements; 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 2015 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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