BioMarin Announces EMA Validation of Brineura™ (Cerliponase Alfa) Marketing Authorization Application for Treatment of CLN2 Disease, a Form of Batten Disease

CHMP Opinion and EU Decision Expected Q3 2017

SAN RAFAEL, Calif., Sept. 15, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the European Medicines Agency (EMA) validated the Marketing Authorization Application (MAA) for Brineura™ (cerliponase alfa) to treat children with CLN2 disease, a form of Batten disease. Validation of the MAA confirms that the submission is accepted and starts the formal review process by the EMA's Committee for Human Medicinal Products (CHMP).

Earlier this year, the CHMP accepted BioMarin's request for accelerated assessment of the MAA on the grounds that Brineura is of major public health interest with the potential to have a major impact on medical practice for CLN2 patients in Europe. Accelerated assessment has the potential to shorten the EMA's review procedure. However, applications which are initially granted accelerated assessment frequently revert to standard assessment timelines. Assuming a positive opinion from the CHMP and standard assessment timing, a decision from the European Commission is anticipated by the third quarter of 2017. The EMA previously granted Brineura Orphan Drug Designation.

"CLN2 disease is a rapidly progressing, fatal neurodegenerative disease with no approved treatments. Based on the positive results of the pivotal study, we are working to bring this meaningful therapeutic option to patients and families in Europe as soon as possible," said Hank Fuchs, MD, Chief Medical Officer of BioMarin. "We greatly appreciate the CLN2 community's ongoing support and look forward to continuing to work with regulatory authorities in the coming months."

Children with CLN2 disease typically begin to present symptoms between the ages of two and four, with the majority of affected children losing their ability to walk and talk by approximately six years of age. Initial symptoms can include language delay and seizures, followed by movement disorders, motor deterioration, dementia and blindness. During the later stages of the disease, feeding and tending to everyday needs become very difficult, and death often occurs between eight and 12 years of age.

Marketing Applications

On July 27, 2016, the U.S. Food and Drug Administration (FDA) accepted for review the submission of a Biologics License Application (BLA) for cerliponase alfa. The Prescription Drug User Fee Act (PDUFA) goal date for a decision is April 27, 2017. The FDA granted cerliponase alfa Priority Review status, which is designated to drugs that offer major advances in treatment or provide a treatment where no adequate therapy exists. Cerliponase alfa was previously granted Orphan Drug Designation and Breakthrough Therapy Designation by the FDA. The Agency has advised that they plan to hold an advisory committee meeting at a date to be confirmed per their usual practice of notification in the Federal Register.

Early Access Program

BioMarin has implemented an early access (compassionate use) program as planned to provide experimental drug for additional CLN2 patients prior to obtaining marketing approval. The program is limited in scope and number of participants, and is being conducted under a protocol. The program initially is being conducted at centers that have participated in the cerliponase alfa study. The program began in August 2016 in Hamburg, Germany and Columbus, OH, U.S.A. We continue to work on opening the other sites, while adhering to specific legal and regulatory procedures for each country. In order to assure fairness in inclusion, enrollment decisions will be made independent of BioMarin. In addition, the identities of participants are confidential to protect the privacy of the patients and families.

About Cerliponase Alfa

Brineura is a recombinant form of human tripeptidyl peptidase 1 (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 Brineura, please contact BioMarin Medical Information at medinfo@bmrn.com.

About CLN2 Disease

CLN2 disease is caused by mutations in the TPP1/CLN2 gene, resulting in deficient activity of the enzyme 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 progressive and relentless neurodegeneration, which manifests as loss of cognitive, motor and visual functions.

There is no approved treatment that can prevent, stop or reverse CLN2 disease. Symptomatic care to treat disease symptoms, prevent and treat complications, and attempt to preserve quality of life is the only currently available option 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, and specifically about regulatory filings for commercial approval of the product candidate, the results of the Phase 1/2 pivotal trial and an ongoing extension study of 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 of current and planned clinical trials of cerliponase alfa; the content and timing of decisions by the U.S. Food and Drug Administration, the European Medicines Agency 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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