FDA Accepts BLA for BioMarin's Cerliponase Alfa for CLN2 Disease, Form of Batten Disease

Potential First Treatment for Fatal, Rare, Brain Disease in Children

FDA Grants Priority Review Status and Breakthrough Therapy Designation

PDUFA Action Date is January 27, 2017

MAA Submitted to European Regulatory Authorities

SAN RAFAEL, Calif., July 27, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the U.S. Food and Drug Administration (FDA) accepted for review the submission of a Biologics License Application (BLA) for cerliponase alfa, an investigational therapy to treat children with CLN2 disease, a form of Batten disease. The Prescription Drug User Fee Act (PDUFA) goal date for a decision is January 27, 2017. BioMarin also has submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for cerliponase alfa, and it is undergoing validation at the Agency.

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 company also announced that the preliminary approved brand name for cerliponase alfa is Brineura™.

"CLN2 disease is a rapidly progressing, fatal neurodegenerative disease with

no approved treatments. The FDA recognized the potential of cerliponase alfa to help address this devastating condition, and we look forward to working closely with the Agency over the coming months," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We thank the community for its continued support, as well as the patients and families who dedicated their time to the clinical development of cerliponase alfa."

"This is an historic milestone for the Batten disease community. For the first time since it was originally described more than a century ago, there is a potential treatment for our children with CLN2 disease," said Margie Frazier, PhD, Executive Director of the Batten Disease Support and Research Association. "We appreciate BioMarin's continued commitment to pursue a therapy for this devastating disease and to advancing it quickly through the development process."

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 8 and 12 years of age.

Early Access Program

BioMarin is planning to implement an early access program to provide access to treatment for additional CLN2 patients prior to obtaining marketing approval. The program will be limited in scope and number of participants and will be conducted under a protocol. BioMarin expects that the program will be conducted initially 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 timing of the start of the program is on track, and the initial site is expected to be open during Q3 2016. The exact timing will vary by country of the sites participating. The overall scope and details of this program are still being determined. BioMarin must adhere to specific legal procedures for each country and has begun these preparations with the goal of being ready to dose patients in Q3 2016.

About Cerliponase Alfa

Cerliponase alfa 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) by intracerebroventricular (ICV) infusion, an established technique for delivering drugs to the brain.

For additional information regarding the investigational product cerliponase alfa, 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. 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.

Contacts:
Investors
Traci McCarty
BioMarin Pharmaceutical Inc.
(415) 455-7558

Media
Debra Charlesworth
BioMarin Pharmaceutical Inc.
(415) 455-7451

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