WORLDSymposium Recognizes BioMarin's Brineura® (cerliponase alfa) with 2018 New Treatment Award

SAN RAFAEL, Calif., Feb. 6, 2018 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that The WORLD *Symposium* 2018 awarded Brineura® (cerliponase alfa) the New Treatment Award, which recognizes important achievements in therapeutic advancements for lysosomal diseases. The award was presented on Monday, February 5.

"We are honored that Brineura has been recognized by the WORLD *Symposium* as the first approved therapy to treat a form of Batten disease," said Hank Fuchs, M.D., President of Worldwide Research and Development at BioMarin. "The approval of Brineura was a direct reflection of an unwavering commitment and partnership between families, advocates and physicians. We share this award with those communities and look forward to continuing to provide our support to advance the care of children with CLN2 disease."

Brineura is the only therapy approved by the U.S. Food and Drug Administration and European Union to treat children with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency. In the U.S., Brineura is approved to slow the loss of ambulation in children over 3 with late infantile CLN2. CLN2 disease is an ultra-rare, rapidly progressive fatal brain condition, which affects an estimated 1,200 to 1,600 children worldwide. Affected children first show symptoms around the age of 3 and completely lose the ability to walk and talk around 6 years of age. During the later stages of the disease, feeding and tending to everyday needs become very difficult with death usually occurring between 8 and 12 years of age.

Brineura was also awarded the 2017 *Popular Science* "Best of What's New" award in the health category. Each year, *Popular Science* reviews thousands of new products and innovations across 11 categories for its annual "Best of What's New" issue, selecting those that represent a significant step forward in their category.

This is also the second time BioMarin has received the WORLD *Symposium* New Treatment Award. BioMarin received the award in 2015 for the first and only therapy approved to treat mucopolysaccharidosis type IVA, also known as Morquio A syndrome.

About CLN2 Disease

Children with CLN2 disease typically begin experiencing seizures between the ages of 2 and 4 years old, preceded in the majority of cases by language development delay. The disease progresses rapidly with most affected children losing the ability to walk and talk by approximately 6 years of age. Initial symptoms are followed by movement disorders, motor deterioration, dementia, blindness, and death usually occurring between the ages of 8 and 12 years of age. During the later stages of the disease, feeding and tending to everyday needs become very difficult. BioMarin estimates the incidence of CLN2 disease is approximately one in 200,000 with up to 1,200 to 1,600 children in the regions of the world where BioMarin operates, many of whom are undiagnosed.

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 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.

About Brineura®

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 into the fluid surrounding the brain (cerebrospinal fluid) using BioMarin's patented technology.

For additional information regarding this product, please contact BioMarin Medical Information at medinfo@bmrn.com.

Indication

Brineura® (cerliponase alfa) is a prescription medication used to slow loss of ability to walk or crawl (ambulation) in symptomatic pediatric patients 3 years of age and older with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency.

Important Safety Information

Brineura is a prescription medicine. Before treatment with Brineura, it is important to discuss your child's medical history with their doctor. Tell the doctor if they are sick or taking any medication and if they are allergic to any

medicines. Your child's doctor will decide if Brineura is right for them. If you have questions or would like more information about Brineura, contact your child's doctor.

Brineura is only given by infusion into the fluid of the brain (known as an intraventricular injection) and using sterile technique to reduce the risk of infection. An intraventricular access device or port must be in place at least 5 to 7 days prior to the first infusion. Intraventricular access device-related infections were observed with Brineura treatment. If any signs of infection occur, contact your child's doctor immediately. Your child's intraventricular access device may need to be replaced over time.

Brineura should not be used in patients with active intraventricular access device-related complications (e.g., leakage, device failure, or device-related infection) and with shunts used to drain extra fluid around the brain.

Low blood pressure and/or slow heart rate may occur during and following the Brineura infusion. Contact your child's doctor immediately if these reactions occur.

Undesirable or hypersensitivity reactions related to Brineura treatment, including fever, vomiting, and irritability, may occur during treatment and as late as 24 hours after infusion. Your child may receive medication such as antihistamines before Brineura infusions to reduce the risk of reactions. Serious and severe allergic reactions (anaphylaxis) may occur. If a reaction occurs, the infusion will be stopped and your child may be given additional medication. If a severe reaction occurs, the infusion will be stopped and your child will receive appropriate medical treatment. If any signs of anaphylaxis occur, immediately seek medical care.

Safety and effectiveness in pediatric patients below 3 years of age have not been established.

The most common side effects reported during Brineura infusions included fever, problems with the electrical activity of the heart, decreased or increased protein in the fluid of the brain, vomiting, seizures, hypersensitivity, collection of blood outside of blood vessels (hematoma), headache, irritability, and increased white blood cell count in the fluid of the brain, device-related infection, slow heart rate, feeling jittery, and low blood pressure. Intraventricular device-related side effects included infection, delivery system-related complications, and increased white blood cell count in fluid of the brain.

These are not all of the possible side effects with Brineura. Talk to your child's doctor if they have any symptoms that bother them or that do not go away.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Please see accompanying full Prescribing Information, or visit www.Brineura.com.

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 six commercialized products and multiple clinical and pre-clinical product candidates.

For additional information, please visit www.BMRN.com.

Contact:

Investors:	*Media:*
Traci McCarty	*Debra Charlesworth*
BioMarin Pharmaceutical Inc.	*BioMarin Pharmaceutical Inc.*
(415) 455-7558	(*415) 455-7451*

SOURCE BioMarin Pharmaceutical Inc.
