Popular Science Names BioMarin's Brineura® (cerliponase alfa) One of the Top Health Innovations of 2017 with "Best of What's New" Award

SAN RAFAEL, Calif., Oct. 18, 2017 /PRNewswire/ -- BioMarin announced today that Brineura® (cerliponase alfa) has been 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.

Brineura is the first therapy approved by the U.S. Food and Drug Administration to treat children with a form of Batten disease, CLN2 disease. CLN2 disease is an ultra-rare and fatal pediatric brain disorder that affects less than one in a million U.S. residents. Brineura 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. It was approved in the U.S. in April 2017 and the European Union in June 2017.

"We are honored that Brineura has been recognized as one of the top scientific accomplishments in 2017 by *Popular Science*," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin. "We share this recognition with the Batten disease patient and medical research communities who played an integral role in making Brineura available to children affected by CLN2 disease. As the first enzyme replacement therapy to be directly administered to the brain, our hope is that this scientific breakthrough blazes a trail for more therapies to treat other neurodegenerative diseases in a meaningful way."

"Until this year, there was no approved treatment for children with any form of Batten disease. Our desire is that this therapy for the CLN2 form of the disease and this recognition of scientific achievement will encourage research into other forms of this devastating disease," said Margie Frazier, PhD, LISW-S, Executive Director of Batten Disease Support and Research Association. "Brineura represents a collaborative scientific effort among patients, industry and academia to bring forth a treatment that seemed impossible just five years ago."

Every year approximately 20 children are born in the U.S. with CLN2 disease. These affected children suffer from drug resistant seizures, movement disorder and completely lose the ability to walk and talk by the time they reach six years of age. During the later stages of the disease, feeding and tending to everyday needs become very difficult with death often occurring between eight and 12 years of age.

Brineura is the first enzyme replacement therapy to be directly administered to the brain, treating the underlying cause of the condition by helping to replace the deficient TPP1 enzyme. Using an established technique most often used in oncology – intraventricular administration – the therapy is delivered directly into fluid surrounding the brain, known as the cerebrospinal fluid.

"The 'Best of What's New' awards honor the innovations that shape the future," said Joe Brown, Editor-in-Chief, *Popular Science*. "From life-saving technology to incredible space engineering to gadgets that are just breathtakingly cool, this is the best of what's new."

Brineura was approved in less than four years from the start of the first clinical trial to approval, a significant achievement for a condition that progresses so rapidly.

About Best of What's New

Each year, the editors of *Popular Science* review thousands of products in search of the top 100 tech innovations of the year—breakthrough products and technologies that represent a significant leap in their categories. The winners, the Best of What's New, are awarded inclusion in the much-anticipated December issue of *Popular Science*, the most widely read issue of the year since the debut of Best of What's New in 1988. Best of What's New awards are presented to 100 new products and technologies in 11 categories: Automotive, Aerospace, Engineering, Entertainment, Gadgets, General Innovation, Security, Software, Home, Health and Recreation.

About Popular Science

Founded in 1872, *Popular Science* is the world's largest science and technology magazine with a circulation of 1.3 million and 6.8 million monthly readers. Each month, *Popular Science* reports on the intersection of science and everyday life, with an eye toward what's new and why it matters. *Popular Science* is published by Bonnier Active Media, a subsidiary of Bonnier Corporation.

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. Information on BioMarin's website is not incorporated by reference into this press release.

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Contact:

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

SOURCE BioMarin Pharmaceutical Inc.
