FDA Extends Market Exclusivity Six Months for BioMarin's Rare Disease Therapy KUVAN(R) (sapropterin dihydrochloride) Powder for Oral Solution and Tablets

New Convenient Form of KUVAN Now Available for the Treatment of Phenylketonuria (PKU)

SAN RAFAEL, Calif., April 14, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN), a global leader in the development and commercialization of therapies for rare genetic diseases, today announced that the Food and Drug Administration (FDA) granted KUVAN® (sapropterin dihydrochloride) Powder for Oral Solution and Tablets a six-month pediatric exclusivity extension. The FDA action extends KUVAN's market exclusivity to June 2015 and is based on studies submitted in response to a written request by the FDA to investigate the use of KUVAN in pediatric patients from birth to age 6. KUVAN formulated as Tablets and Powder for Oral Solution, is the first and only FDA-approved medication for PKU to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive phenylketonuria (PKU). KUVAN is to be used in conjunction with a Phe-restricted diet.

PKU or PAH Deficiency is a rare genetic condition in which the body cannot process Phe, an amino acid found in many foods. This can lead to too much Phe in the blood. Left untreated, high blood Phe levels can affect the brain, impair thinking, and cause behavioral problems.

The FDA has approved a new and convenient form of KUVAN, a powder for oral solution packaged in individual packets of 100 mg. KUVAN powder is to be dissolved in water, apple juice or a small amount of soft foods. This new formulation is a convenient alternative to tablets and provides an additional option for infants and small children. KUVAN powder dissolves rapidly and completely, and offers reduced acidity.

"Seven years after the approval of KUVAN Tablets, BioMarin remains committed to the PKU or PAH deficient community. The new powder form of KUVAN provides an additional option for parents with small children taking KUVAN, or for anyone who has trouble swallowing tablets," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We continue to invest in the PKU community with the introduction of this new solution and to advance another experimental PKU treatment with PEG PAL, a therapy in Phase 3 clinical development."

In January 2014, the American College of Medical Genetics and Genomics (ACMG) issued new practice guidelines, which support the need for lifelong management of PHE levels in patients with phenylketonuria or PKU. The new diagnosis and management guidelines provide the first update to recommendations for therapy of PKU since the 2001 National Institutes of Health Consensus statement.[1]

The new guidelines state that treatment of PKU should be initiated as early as possible and must be continued throughout adulthood and for life. Patients treated from the early weeks of life with initial good metabolic control, but who lose that control in later childhood or adult life, may experience both reversible and irreversible neuropsychiatric consequences. The guidelines specifically note that for appropriate patients use of KUVAN should be considered to help lower Phe.

About KUVAN®

First approved in December 2007, KUVAN is the first and only prescription medicine of its kind. When used in conjunction with diet, KUVAN can help lower blood Phe levels more than the use of diet alone. The newly revised American College of Medical Genetics and Genomics (ACMG) PAH-deficiency management guidelines (2013) specifically recommend trial therapy with KUVAN to assess patient responsiveness.

KUVAN (sapropterin dihydrochloride), formulated as Tablets and Powder for Oral Solution, is the first and only FDA-approved medication for PKU to reduce blood Phe levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin (BH4- responsive PKU). KUVAN is a pharmaceutical formulation of BH4, the natural cofactor for the PAH enzyme, which stimulates activity of the residual PAH enzyme to metabolize Phe into tyrosine. KUVAN is to be used in conjunction with a Phe-restricted diet.

KUVAN Powder for Oral Solution is available immediately. For more information about KUVAN, please visit www.KUVAN.com.

Important Safety Information

KUVAN® (sapropterin dihydrochloride) Tablets and KUVAN (sapropterin dihydrochloride) Powder for Oral Solution are approved to reduce blood Phe levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive Phenylketonuria (PKU). KUVAN is to be used with a Phe-restricted diet.

High blood Phe levels are toxic to the brain and can lead to lower intelligence and decrease in the ability to focus, remember and organize information. Any change you make to your diet may impact your blood Phe level. Follow your doctor's instructions carefully. Your doctor and dietitian will continue to monitor your diet and blood Phe levels throughout your treatment with KUVAN.

If you have a fever, or if you are sick, your Phe level may go up. Tell your doctor and dietitian as soon as possible so they can see if they have to adjust your treatment to help keep your blood Phe levels in the desired range.

KUVAN is a prescription medicine and should not be taken by people who are allergic to any of its ingredients. Tell your doctor if you have ever had liver or kidney problems, are nursing or pregnant or may become pregnant, have poor nutrition or are anorexic. Your doctor will decide if KUVAN is right for you. Tell your doctor about all the medicines you take.

The most common side effects reported when using KUVAN are headache, diarrhea, abdominal pain, upper respiratory tract infection (like a cold), throat pain, vomiting, and nausea.

To report SUSPECTED ADVERSE REACTIONS, contact BioMarin Pharmaceutical Inc. at 1-866-906-6100, or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

KUVAN is available by prescription only. To learn more, please visit www.kuvan.com for full prescribing information. If you have any questions about this information, please talk with your doctor.

About PKU or PAH Deficiency

Phenylketonuria (PKU) or phenylalanine hydroxylase (PAH) deficiency is a genetic disorder affecting approximately 50,000 diagnosed patients in the developed world and is caused by a deficiency of the enzyme phenylalanine hydroxylase (PAH), this enzyme is required for the metabolism of phenylalanine (PHE), an essential amino acid found in most protein-containing foods. If the active enzyme is not present in sufficient quantities, PHE accumulates to abnormally high levels in the blood and becomes toxic to the brain, resulting in a variety of complications including severe intellectual disability, seizures, tremors, behavioral problems and psychiatric symptoms. As a result of newborn screening efforts implemented in the 1960s and early 1970s, virtually all individuals with PKU or PAH deficiency under the age of 40 in developed countries are diagnosed at birth and treatment is implemented soon after. PAH deficiency can be managed with a PHE-restricted diet, which is supplemented by low-protein modified foods and PHE-free medical foods; however, the strict diet is difficult for most patients to adhere to the extent needed for achieving adequate control of blood PHE levels. KUVAN, the first and only prescription medicine of its kind, may help individuals with PAH deficiency lower blood PHE levels when used in conjunction with a PHE-restricted diet, more than the use of diet alone. To learn more about PAH deficiency, please visit www.PKU.com. Information on this website is not incorporated by reference into this press release. Some of the signs and symptoms of high blood PHE include:

- For infants and children: severe intellectual disability and developmental delay, skin rash (eczema), light-colored skin, eyes and hair (hypopigmentation)
- For teens and adults: lower intelligence, psychological and psychiatric symptoms like anxiety, depression and phobias, problems with memory and performing tasks (executive function), poor concentration and irritable mood among other things.
- For pregnant women: increased risk for the baby's growing brain, including risk of intellectual disability, increased risk for a small head (microcephaly) and other problems such as a heart malformation (congenital heart defect) and poor overall growth (intrauterine growth retardation). This teratogenic effect of PHE on the developing fetus is called Maternal PKU syndrome.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five approved products and multiple clinical and pre-clinical product candidates. Approved products include VIMIZIM™ (elosulfase alfa) for MPS IVA; Naglazyme® (galsulfase) for MPS VI; Aldurazyme® (laronidase) for MPS I, a product which BioMarin developed through a 50/50 joint venture with Genzyme, a Sanofi Company; KUVAN® (sapropterin dihydrochloride) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany and Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include PEG

PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 3 clinical development for the treatment of PKU, BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer, BMN 701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase 1/2 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 1 clinical development for the treatment of achondroplasia, BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease, which is currently in Phase 1, BMN 270, an AAV-factor VIII vector, for the treatment of hemophilia A and BMN 250, a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of MPS IIIB.

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

[1]https://www.acmg.net/docs/Phenylalanine_Hydrosylase_Deficiency_Practice_Guideline_AOP_Jan_2013.pdf

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