

# BioMarin Receives 2009 Corporate Award for the Development of Kuvan

## National Organization for Rare Disorders (NORD) Recognizes BioMarin's Efforts to Expand Treatment Options for Patients with PKU

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BioMarin Pharmaceutical Inc. announced today that it has received the 2009 Corporate Award from the National Organization for Rare Disorders (NORD), an organization dedicated to improving the lives of people with rare diseases. The award was presented on May 14 at NORD's annual "Partners in Progress" Gala in Washington, D.C. It recognizes BioMarin's significant achievements toward improving the lives of people living with phenylketonuria (PKU).

NORD honored BioMarin for the development of Kuvan(R) (sapropterin dihydrochloride) Tablets, the first and only prescription medication for PKU. Kuvan is indicated to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive phenylketonuria (PKU). PKU is a rare genetic disorder that can cause brain damage and various neurological complications, including mental retardation, mental illness, seizures, tremors and cognitive problems. Designated as an orphan disease, PKU occurs in about one out of every 12,000 to 15,000 live births in the United States.

"It is a great honor to receive this award from NORD," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "We are very pleased to be able to provide this important treatment option to PKU patients and we will continue to push our scientific research and product development efforts forward to meet the medical needs of PKU patients -- and those with other rare diseases."

Each year NORD selects organizations that have made a positive contribution to further the needs of the patient community and have inspired the public to do so as well. Since 2004, this is the third time BioMarin has been bestowed with this honor.

"Patients and families who live with PKU face a constant struggle to maintain their health balance, and BioMarin has been instrumental in giving PKU patients an opportunity to lower their Phe levels," said NORD president Peter Saltonstall. "We applaud BioMarin's efforts and look to more companies to make similar strides and provide treatment options to patients in rare disease communities."

In addition to attending the NORD Gala mid-month, BioMarin will participate in several PKU community events in support of PKU Awareness Month this May. Participation includes full sponsorship of a disease awareness campaign and a support program for patients who may require assistance with disease management. Locally, BioMarin employees will attend and sponsor community walks around the country and assist in fundraising efforts. For more information on PKU Awareness Month, visit [www.rarediseases.org](http://www.rarediseases.org).

### About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises three approved products and multiple clinical and pre-clinical product candidates. Approved products include Naglazyme(C) (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme(R) (Iaronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; and Kuvan(C) (sapropterin dihydrochloride) Tablets, a product for the treatment of phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany. Other product candidates include PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase I clinical development for the treatment of PKU and GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase I/II clinical development for the treatment of MPS IVA. For additional information, please visit [www.BMRN.com](http://www.BMRN.com). Information on BioMarin's website is not incorporated by reference into this press release

### About NORD

NORD, celebrating its 25th anniversary this year, is a federation of individuals' and patients' organizations. The organization advocates on behalf of the rare disease community and sponsors research grants and fellowships, educational services for patients and medical professionals and other patient assistance programs. Established by leaders of rare disease patient organizations who helped get the Orphan Drug Act of 1983 passed, NORD

represents the nearly 30 million Americans who have rare diseases with programs of advocacy, education, research, and patient services. For additional information, please visit [www.rarediseases.org](http://www.rarediseases.org).

#### About PKU

PKU, a genetic disorder affecting approximately 50,000 diagnosed patients in the developed world, is caused by a deficiency of the enzyme phenylalanine hydroxylase. PAH is required for the metabolism of phenylalanine, 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 mental retardation and brain damage, mental illness, seizures, tremors and limited cognitive ability. As a result of newborn screening efforts implemented in the 1960s and early 1970s, virtually all PKU patients under the age of 40 in developed countries have been diagnosed at birth. For more information about PKU, visit [www.pku.com](http://www.pku.com).

#### About Kuvan

Kuvan(R) (sapropterin dihydrochloride) Tablets is the first and only FDA-approved prescription drug for the treatment of phenylketonuria, or PKU, a lifelong genetic metabolic disorder that, when left untreated, can result in mental retardation and other neurological problems. Individuals with PKU cannot metabolize an essential amino called phenylalanine, or Phe, which is found in most foods; this results in a dangerous accumulation of Phe in the blood, which can be toxic to the brain. Kuvan is indicated to reduce blood 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. For full prescribing information, please visit <http://www.kuvan.com/TopNavPages/PatientInfo.aspx>.

BioMarin(R), Naglazyme(R) and Kuvan(R) are registered trademarks of BioMarin Pharmaceutical Inc.

Aldurazyme(R) is a registered trademark of BioMarin/Genzyme LLC.

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Web Site: <http://www.bmrn.com/>  
<http://www.kuvan.com/TopNavPages/PatientInfo.aspx>  
<http://www.rarediseases.org/>  
<http://www.pku.com/>

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