

# Kuvan Receives Priority Review Status From Health Canada

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BioMarin Pharmaceutical Inc. announced today that it has received notice of priority review status for Kuvan(R) (sapropterin dihydrochloride) as an oral treatment for patients with phenylketonuria (PKU) from Health Canada. Priority review provides for a shortened submission review of 180 days versus the standard twelve months. BioMarin plans to file a marketing application for Kuvan in Canada in the third quarter of 2009, and with priority review status, a decision for marketing approval is expected in the first half of 2010.

"Priority review status from Health Canada marks another milestone in our efforts to commercialize Kuvan worldwide as it is granted for serious, life-threatening or severely debilitating diseases for which no approved medical therapy exists in Canada," said Steve Aselage, Senior Vice President and Chief Business Officer of BioMarin. "Along with our partners Merck Serono and Asubio, we remain dedicated to serving the PKU community and providing a therapeutic option in the management of this disease."

## About Kuvan

Kuvan(R) (sapropterin dihydrochloride) Tablets are indicated in the United States 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.

The active ingredient in Kuvan, sapropterin dihydrochloride, is the synthetic form of 6R-BH4 (tetrahydrobiopterin), a naturally occurring enzyme cofactor that works in conjunction with phenylalanine hydroxylase (PAH) to metabolize Phe.

Kuvan has received orphan drug designation from both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Kuvan has received seven years of orphan exclusivity in the United States and ten years of market exclusivity in the E.U.

## 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. To learn more about PKU, please visit [www.PKU.com](http://www.PKU.com). Information on this website is not incorporated by reference into this press release.

## 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(R) (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme(R) (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; and Kuvan(R) (sapropterin dihydrochloride) Tablets, for 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 in clinical development for the treatment of PKU and GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase 1/2 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.

## Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the continued development and commercialization of Kuvan and BioMarin's other products and product candidates and actions by regulatory

authorities. 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: our success in the continued commercialization of Kuvan; the content and timing of decisions by the U.S. Food and Drug Administration, Health Canada and other regulatory authorities concerning each of the described products and product candidates; 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 2008 Annual Report on Form 10-K, as amended, and the factors contained in BioMarin's reports on Form 10-Q and 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.

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

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

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