

# United States Patent Office Issues Patent Covering the Approved Administration of Kuvan With Food

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BioMarin Pharmaceutical Inc. announced today that the United States Patent and Trademark Office has issued a patent covering the approved administration of Kuvan® (sapropterin dihydrochloride) with food for the treatment of phenylketonuria (PKU). The patent expires in 2024.

"We believe that the food effect patent, based on the discovery that food increases the bioavailability of Kuvan, along with our patents for once daily dosing and stable tablet formulation, will create significant barriers to therapeutically equivalent generic competition from entering the market for approximately ten years beyond orphan drug exclusivity," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "We remain committed to serving the PKU community with initiatives such as implementation of an outcomes study in the second quarter of next year and the ongoing development of a blood Phe monitor. We also look forward to results from various investigator-sponsored trials that, if positive, will strengthen the value proposition for the use of Kuvan."

## About Kuvan

Kuvan® (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® (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; and Kuvan® (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 currently in Phase II 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.

## Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: expectations related to patents associated with Kuvan. 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:

possible actions by others to challenge, invalidate or design around the patents; 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®, Naglazyme® and Kuvan® are registered trademarks of BioMarin Pharmaceutical Inc.

Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC.

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