

# Data to Be Presented at 58th Annual Meeting of The American Society of Human Genetics

PRNewswire-FirstCall  
NOVATO, Calif.

BioMarin Pharmaceutical Inc. announced today that data from clinical studies of Kuvan(R) (sapropterin dihydrochloride), Naglazyme(R) (galsulfase) and enzyme replacement therapies for MPS IVA (Morquio Syndrome) and Pompe disease will be presented at the 58th Annual Meeting of the American Society of Human Genetics (ASHG) in Philadelphia, Pennsylvania, November 11-15, 2008.

"We look forward to a number of poster presentations that will address a variety of investigator-sponsored trials now in progress. Results from these studies will add to our growing data set for Kuvan and Naglazyme and increase our understanding about the long term safety and efficacy of the drugs across different patient populations," said Chief Medical Officer Emil Kakkis, M.D., Ph.D.

The following posters will be featured:

- 590/W - "Implications of absence of clinical phenotype on Morquio A mice: Why rodents do not require skeleton keratan sulfate?"
- 752/W - "Intrathecal (IT) enzyme replacement therapy (ERT) for symptomatic spinal cord compression (SCC) in a MPS VI child: safety, efficacy, and pitfalls"
- 763/W - "Therapeutic response after two years of Galsulfase ERT in five adult patients with MPS VI"
- 2353/W - "MPS Brazil-Network: 4 years improving diagnosis and management of mucopolysaccharidoses in Brazil"
- 772/T - "PKU treatment with tetrahydrobiopterin (sapropterin) during pregnancy"
- 773/T - "A new strategy using sapropterin and placebo to determine the predictive value of mutation analysis towards identifying BH4 responsive hyperphenylalanemia"
- 774/T - "Interim results of a Phase II, multicenter, open-label study of sapropterin dihydrochloride in subjects with hyperphenylalaninemia related to primary BH4 deficiency"
- 767/T - "Differential response to Galsulfase therapy in brothers with mucopolysaccharidosis VI (Maroteaux-Lamy)"
- 778/T - "Sapropterin (Kuvan) is safe and effective in patients under 4 years of age with PKU"
- 677/F - "Lysosomal Disease Network, and WORLD Symposium 2009"
- 787/F - "A follow-up of ERT in two MPS VI patients with poorly engrafted bone marrow transplantation"
- 805/F - "An improved alpha-glucosidase enzyme for Pompe disease"

## About Kuvan

Kuvan (sapropterin dihydrochloride) Tablets is indicated 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. BioMarin and Merck Serono estimate that Kuvan could be a potential treatment option for approximately 30 percent to 50 percent of the estimated 50,000 identified PKU patients in the developed world.

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 market exclusivity in the United States. In November 2007, Merck Serono submitted a Marketing Authorization Application (MAA) to the EMA for sapropterin dihydrochloride as an oral treatment for patients suffering from HPA due to PKU or BH4 deficiency. If approved in the EU, it will receive 10 years of market exclusivity for this indication.

## About Naglazyme

Naglazyme (galsulfase) is the first drug approved to treat MPS VI in the United States and Europe. It is an enzyme replacement therapy, using a recombinant version of arylsulfatase B to replace or supplement low to non-existent levels of the natural enzyme in the body. The product received FDA approval in May 2005 and EMEA approval in January 2006.

## 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, 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 6R-BH4 for cardiovascular indications, which is currently in Phase 2 clinical development for the treatment of peripheral arterial disease and sickle cell disease, and PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 1 clinical development for the treatment of PKU. For additional information, please visit <http://www.bmrn.com/>. Information on BioMarin's website is not incorporated by reference into this press release.

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

Contact:

|                              |                              |
|------------------------------|------------------------------|
| Investors                    | Media                        |
| Eugenia Shen                 | Susan Berg                   |
| BioMarin Pharmaceutical Inc. | BioMarin Pharmaceutical Inc. |
| (415) 506-6570               | (415) 506-6594               |

First Call Analyst:

FCMN Contact: [eshen@bmrn.com](mailto:eshen@bmrn.com)

SOURCE: BioMarin Pharmaceutical Inc.

CONTACT: Investors, Eugenia Shen, +1-415-506-6570, or Media, Susan Berg, +1-415-506-6594, both of BioMarin Pharmaceutical Inc.

Web site: <http://www.bmrn.com/>

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