BioMarin Enrolls First Patient into Phase 2 Clinical Trial of Phenoptin for PKU

PRNewswire-FirstCall
NOVATO, Calif.

BioMarin Pharmaceutical Inc. (Nasdaq and SWX: BMRN) today announced that it has initiated its Phase 2 clinical trial of Phenoptin(TM) (sapropterin hydrochloride), an investigational oral, small molecule therapeutic for the treatment of the genetic disease phenylketonuria (PKU). Patients who respond to Phenoptin in the Phase 2 clinical trial, as measured by a greater than 30 percent reduction in blood phenylalanine (Phe) levels following eight days of treatment, will be eligible to enroll into the Phase 3 trial expected to begin in the first quarter of 2005.

Margretta Seashore, M.D., Director of Genetic Consultation Service in the Department of Genetics at Yale University School of Medicine, commented, "PKU patients in my clinic are excited about the potential of an oral therapeutic that could help them control blood Phe levels and ease the burden imposed by the highly restrictive diet required to manage the disease." Dr. Seashore continued, "For many medical geneticists that treat PKU patients, Phenoptin could become an important tool to improve the life-long management of PKU and to help patients avoid the neurological symptoms associated with PKU."

The Phase 2 trial will screen up to 400 PKU patients for a response to Phenoptin as defined by 30 percent or greater reduction in blood Phe levels. PKU patients over the age of 8 will receive 10mg/kg of Phenoptin daily for eight days. Responsive patients will be eligible to enroll in the Phase 3 clinical trial expected to begin in the first quarter of 2005.

About Phenoptin

Phenoptin(TM) (sapropterin hydrochloride, also known as 6R-BH4) is an investigational oral small molecule therapeutic for the treatment of primarily the moderate to mild forms of PKU. BioMarin received orphan drug designation for Phenoptin to treat PKU from both the U.S. Food and Drug Administration and European Medicines Evaluation Agency. BioMarin would receive seven years of market exclusivity in United States and 10 years in the European Union if it were first to receive approval for 6R-BH4 to treat PKU.

About PKU

PKU, a genetic disorder affecting at least 50,000 diagnosed patients under the age of 40 in the developed world, an estimated half of whom have the moderate to mild form of the disease, is caused by a deficiency of the enzyme, phenylalanine hydroxylase (PAH). PAH is required for the metabolism of 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 brain resulting in a variety of complications, including severe mental retardation and brain damage, mental illness, seizures and tremors, and cognitive problems. As a result of global newborn screening efforts implemented in the 1960s and early 1970s, virtually all PKU patients in developed countries have been diagnosed at birth. The only treatment currently available for PKU patients is a highly restrictive and expensive medical food diet that most patients find difficult to maintain.

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio is comprised of two approved products, Orapred(R) (prednisolone sodium phosphate oral solution) for severe asthma and Aldurazyme(R) (laronidase) for mucopolysaccharidosis I (MPS I), and multiple investigational product

candidates including rhASB (galsulfase), a Phase 3 product candidate for the treatment of mucopolysaccharidosis VI (MPS VI), and Phenoptin(TM) (sapropterin hydrochloride), a Phase 2 product candidate for the treatment of phenylketonuria (PKU). For additional information, please visit www.BMRN.com.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the development of its product candidate Phenoptin; expectations related to future clinical trials of Phenoptin; the efficacy Phenoptin; and the commercialization plans for Phenoptin. 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: slow patient enrollment rates for the Phenoptin trials; results and timing of clinical trials of Phenoptin; the content and timing of decisions by the FDA, the EMEA and other regulatory authorities concerning Phenoptin; the possible need to conduct additional clinical trials of Phenoptin; risks associated with the commercial scale-up of the manufacturing process of Phenoptin, including cost and viability risks; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Factors That May Affect Future Results" in BioMarin's 2003 Annual Report on Form 10-K and the factors contained in BioMarin's reports on Forms 10-Q and 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.

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

Orapred(R) is a registered trademark of Medicis Pediatrics, Inc. and is used under license.

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