BioMarin Announces Positive Results From Phase 3 Diet Study of Phenoptin for PKU

Phenoptin Significantly Increases Phenylalanine Tolerance in BH4-Responsive Patients; All Pre-Specified Endpoints Met

Conference Call to be Held Today at 12:00 p.m. EST

PRNewswire-FirstCall
NOVATO, Calif.

BioMarin Pharmaceutical Inc. (Nasdaq and SWX: BMRN) today announced positive results from the Phase 3 diet study of Phenoptin(TM) (sapropterin dihydrochloride), in combination with diet, in 4-12 year old patients. Phenoptin is an investigational oral small molecule, being developed in partnership with Merck Serono, for the treatment of patients with phenylketonuria (PKU), who have elevated phenylalanine (Phe) levels. The results show that all pre-specified efficacy and safety endpoints of the double-blind, placebo-controlled study were met. Phenoptin treatment caused a significant increase in phenylalanine tolerance as well as a reduction in blood phenylalanine levels. In addition, the data showed that Phenoptin was well tolerated in younger PKU patients who were under dietary control.

Dr. Harvey Levy, Professor of Pediatrics at Harvard Medical School and Senior Associate in Medicine and Genetics at Children's Hospital Boston, stated, "This is the first time a controlled study has demonstrated the potential of tetrahydrobiopterin, or 6R-BH4, to liberalize diet in PKU patients. If approved, Phenoptin offers patients who respond to BH4 a real opportunity to relax their diets and the possibility to perhaps reduce or even eliminate the need for nutritional supplementation from medical food."

Key findings from the study:

*In the primary endpoint, Phenoptin enabled a mean increase of 20.9 mg/kg/day in Phe supplementation for those patients on Phenoptin ($p<0.001$). Patients treated with Phenoptin were able to, on average, double their baseline intake. At week 10, they were able to take a mean total Phe intake of approximately 43.8 mg/kg/day, while maintaining controlled blood Phe levels. The mean Phe tolerance represents approximately half the amount of Phe in a normal diet.

*The two secondary endpoints were also met:

-- Phenoptin provided a mean reduction of 148.5 micromolars per liter (from a starting mean of 275.7 micromolars per liter) in blood Phe level from baseline to week 3 (prior to Phe supplementation) ($p<0.001$).

-- At the end of the study, patients on Phenoptin were able to increase their mean daily Phe supplement by a mean of 20.9 mg/kg versus a mean of 2.9 mg/kg for placebo patients ($p<0.001$).

*The incidence and types of adverse events were similar in both the placebo and Phenoptin groups and all reported events were mild or moderate in severity. There were two serious adverse events (one in the Phenoptin group and one in the placebo group), neither of which were considered drug-related. The most frequently reported adverse events were headache, abdominal pain, fatigue, and diarrhea.

The 11-week multi-center double-blind, placebo controlled Phase 3 study enrolled 90 patients between the ages of 4 and 12 years, with blood Phe levels below 480 micromolars per liter. Patients were screened for responsiveness to Phenoptin with an open-label one-week treatment at a dose of 20 mg/kg/day (Part 1). Of the 89 patients who completed Part 1, 50 subjects demonstrated a blood Phe reduction of at least 30%, and 45 were randomized to Phenoptin (20 mg/kg/day) or placebo in a 3:1 ratio, and enrolled in the 10-week double-blind, placebo-controlled portion of the study (Part 2). For the first three weeks, patients maintained their pre-existing restricted diet with no supplementation of phenylalanine. Thereafter, every other week, specific amounts of phenylalanine were added (or removed) to the restricted diet of each patient according to pre-defined blood phenylalanine levels. The maximum amount of Phe that could be added to a patient diet during the study was 50 mg/kg/day.

BioMarin and Merck Serono remain on track to file the NDA and MAA in the second and third quarters of 2007, respectively.

BioMarin will hold a conference call today, January 16, 2007, at 12:00 p.m. EST to discuss the Phase 3 diet study results. Dr. Harvey Levy, Professor of Pediatrics at Harvard Medical School and Senior Associate in Medicine and Genetics at Children's Hospital Boston, will participate on the call. This event can be accessed on the investor section of the BioMarin website at www.BMRN.com .

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Date:  January 16, 2007
Time:  12:00 p.m. EST
U.S. and Canada Toll-Free Dial in #:  800.659.1966
International Dial in #:  617.614.2711
Participant Code:  31390367
Replay Toll-Free Dial in #:  888.286.8010
Replay International Dial in #:  617.801.6888
Replay Code:  21090857

About Phenoptin
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Phenoptin is an investigational oral small molecule therapeutic for the treatment of PKU. The active ingredient in Phenoptin, 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. Preliminary clinical data have suggested that Phenoptin has a potential to produce significant reductions in blood Phe levels in the subset of patients who are BH4-responsive. BioMarin and Merck Serono estimate that Phenoptin could be a potential treatment option for approximately 30 percent to 50 percent of the estimated 50,000 individuals in the developed world who have been diagnosed with PKU.

Phenoptin received orphan drug designation to treat PKU from both the U.S. Food and Drug Administration (FDA) and European Medicines Agency

(EMEA). If Phenoptin becomes the first drug therapy approved for the treatment of PKU, Phenoptin would receive seven years of market exclusivity in the United States and 10 years in the European Union for this indication. Additionally, the FDA has granted Phenoptin Fast Track designation, which is designed to facilitate the development and expedite the review of new drugs that are intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs.

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). PAH is required for the metabolism of phenylalanine (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 fail to adhere to the extent needed for achieving adequate control of blood Phe levels. To learn more about PKU, please visit www.PKU.com . Information on this website is not incorporated by reference into this press release.

Positive Results from Phase 3 Clinical Study of Phenoptin for PKU

Positive results of a Phase 3, double-blind, placebo-controlled clinical study of Phenoptin (sapropterin dihydrochloride), an investigational oral small molecule for the treatment of phenylketonuria (PKU) were reported on March 15, 2006.

Results confirmed that all pre-specified primary and secondary endpoints were met and data demonstrated a statistically significant reduction at six weeks in blood phenylalanine (Phe) levels ($p<0.0001$) in patients receiving Phenoptin, compared with those receiving placebo.

About BioMarin

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 regarding filings with regulatory agencies; and the development of 6R-BH4 for other indications. 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: the results of ongoing clinical trials related to Phenoptin; results and timing of current and planned clinical trials of Phenoptin for the treatment of PKU; the content and timing of decisions by the U.S. Food and Drug Administration, the European Medicines Agency and other regulatory authorities concerning Phenoptin; results and timing of current and planned clinical trials of 6R-BH4 for other indications; 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 2005 Annual Report on Form 10-K, as amended, and the factors contained in BioMarin's reports on 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.

Forward-Looking Statements

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Contacts:
For BioMarin:
Investors and Media:
Eugenia Shen
BioMarin Pharmaceutical Inc.
415-506-6570
http://www.bmrn.com/

FCMN Contact: eshen@bmrn.com

SOURCE: BioMarin Phamaceutical Inc.

CONTACT: investors and media, Eugenia Shen of BioMarin Pharmaceutical Inc., +1-415-506-6570

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