

# BioMarin Named by CenterWatch as One of the Fastest Developers of Medicines

## Analysis Shows That BioMarin is a Leader in Speed and Efficiency of Bringing New Treatments to Patients

SAN RAFAEL, Calif., Sept. 15, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that CenterWatch, a leading source for global clinical trial information, has named it one of the fastest drug developers in the industry. CenterWatch reviewed 307 therapies approved between 2000 and 2013 and concluded that BioMarin ranked within the top five in all categories. The three assessed categories were clinical duration, NDA approval duration and total duration of approval. BioMarin was also among the fastest companies named in drug development for endocrine disorders.

Analysis showed that BioMarin took a median 61 months to bring a treatment through the entire regulatory process, from IND filing to approval. With regard to clinical duration, the time to take a therapy from IND filing through to NDA submission, the company was able to complete the process in a median 55 months, while needing just six months to complete the NDA approval process. BioMarin was also among the top five fastest companies in endocrine development with a median development time of 2.6 years. This compares to a median clinical phase of 5.3 years for endocrine development for all companies analyzed.

"The analysis by CenterWatch showed that BioMarin develops treatments 12 months faster than the industry median. For the patients we seek to treat, those with ultra-rare conditions, one year can make a lifetime of a difference," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We thank CenterWatch for conducting this analysis, which truly underscores our continued commitment to developing therapies that address unmet medical needs as quickly and efficiently as possible."

The results of the analysis were published in the September issue of CenterWatch Monthly, a newsletter providing clinical trial information to patients and professionals. This is the second such analysis conducted by CenterWatch.

### About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five 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; KUVAN® (sapropterin dihydrochloride) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS); and VIMIZIM® (elosulfase alfa) for the treatment of Morquio A (MPS IVA). Product candidates include: BMN 165 (PEGylated recombinant phenylalanine ammonia lyase), also referred to as PEG PAL, which is currently in Phase 3 clinical development for the treatment of PKU; talazoparib (BMN 673), a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer; BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 3 clinical development for the treatment of Pompe disease; BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia; and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1), which is currently in Phase 1 for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease.

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.

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### Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, implied statements about the development timelines of

BioMarin's product candidates. The historical median numbers should not be an indication of the future approval timelines as the development of each product candidate is unique. Actual development timelines involve risks and uncertainties such that actual results may differ materially from our historical median numbers. These risks and uncertainties include, among others: the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities, results and timing of current and planned clinical and preclinical studies related to such product; our ability to successfully manufacture the product; 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 2013 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. 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.

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