

# BioMarin Supports Observance of Rare Disease Day February 28

## **International Coalition Aims to Increase Public Awareness, Supportive Legislation and Drug Development For Patients with Rare Diseases; California Assembly Endorses State Date of Observance**

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NOVATO, Calif.

BioMarin Pharmaceutical Inc. announced today that it has partnered with industry and patient advocacy organizations in an international effort to raise awareness of rare diseases which affect nearly 30 million Americans and millions of others around the world. As part of this program, the company hosted a special event today at its Novato headquarters to honor patients and reaffirm its commitment to developing much-needed therapeutic treatments for rare diseases. Guests included patients, physicians and representatives from the National Organization for Rare Diseases (NORD), along with local civic leaders and BioMarin employees. Rare Disease Day 2010 has also been endorsed by the California Assembly, via Resolution 117, as an official State Day of Observance.

Rare Disease Day activities in the U.S. will include a nationwide network of activities for patients, care givers and the general public. The day is observed by industry, medical, research and patient communities and government entities such as the National Institutes of Health (NIH) Office of Rare Diseases Research and the Food and Drug Administration's (FDA) Office of Orphan Products Development.

"Our goal in supporting this program is to help educate the community and reinforce the importance of making rare diseases a public health priority," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "We commend patients for their courage and determination in battling these debilitating illnesses and we applaud all of the efforts that our medical researchers and organizations such as NORD are making to help support and alleviate the suffering of this under-represented patient population."

NORD, a U.S. based patient advocacy organization, works on behalf of the rare disease community, sponsoring research grants and fellowships, patient assistance programs and a variety of supportive and educational initiatives. NORD founders are responsible for spearheading orphan drug legislation, specifically the Orphan Drug Act of 1983.

"People with rare diseases face unique challenges compared to those with more common diseases," said NORD President and Chief Executive Officer, Peter L. Saltonstall. "They often have great difficulty obtaining accurate diagnoses and finding medical experts who recognize and understand the complexities of their rare disorders. It is our ongoing mission to work with our partners in industry, science and government to mobilize interest and improve medical care for these patients."

February 28 will mark the third annual Rare Disease Day, a concept that was launched in Europe in 2008 by the European Rare Disease Organization (EURODIS), a sister organization to NORD. Rare Disease Day will be observed in the United States, Canada, Europe, Australia and other Pacific regions, and will continue in the hopes of establishing a formalized global Rare Disease Day on the last day of February each year.

A rare disease (also known as an "orphan" disease) is one that affects fewer than 200,000 Americans. According to the NIH there are nearly 7,000 such diseases affecting an estimated 30 million people in the United States.

In 1983, the Orphan Drug Act was passed by Congress to create financial incentives for companies to develop treatments for rare diseases. Since then, approximately 360 orphan drugs and biologics have been approved by the FDA. While estimates indicate that up to 13 million Americans now benefit from these products, more than 15 million patients still remain untreated and underserved.

For more information about Rare Disease Day 2010 activities in the U.S., visit [www.rarediseases.org](http://www.rarediseases.org). For information about activities worldwide, go to [www.rarediseaseday.us](http://www.rarediseaseday.us).

### About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises four 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) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; and Firdapse(TM) (amifampridine phosphate), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Other product candidates include PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU; GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase I/II clinical development for the treatment of MPS IVA and BMN 195, which is currently in Phase I clinical development for the treatment of Duchenne Muscular Dystrophy. 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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About NORD

The National Organization for Rare Disorders ([www.rarediseases.org](http://www.rarediseases.org)) is a federation of individuals and patient organizations that was established in 1983 by patient leaders who worked to get the Orphan Drug Act passed. NORD provides advocacy in Washington, DC., on behalf of the rare disease community and sponsors research grants and fellowships, educational services for patients and medical professionals and other patient assistance programs and public awareness initiatives.

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Web Site: <http://www.bmrn.com/>  
<http://www.rarediseases.org/>  
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