

## **BioMarin to Present Research Results on Duchenne Muscular Dystrophy and Pompe Disease Programs at the 20th International Congress of the World Muscle Society**

SAN RAFAEL, Calif., Sept. 30, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) announced today that the company will present research results at the 20th International Congress of the World Muscle Society, including one oral presentation in Pompe disease and two posters each in Duchenne muscular dystrophy and Pompe disease programs. The meeting will be held in Brighton, England from September 30 to October 4.

During an oral session, preliminary clinical efficacy and safety data from an existing extension study of reveglucosidase alfa (BMN 701) in patients with late onset Pompe disease will be presented. Pompe disease is an inherited condition caused by the deficiency in the enzyme acid alpha-glucosidase, which leads to glycogen accumulation and progressive muscle weakening, including muscles essential for breathing. Reveglucosidase alfa is a novel fusion protein of insulin-like growth factor 2 and acid alpha-glucosidase, designed to target delivery to cell structures called lysosomes where the enzyme is most needed.

Results will also be presented on Duchenne muscular dystrophy, including those from a prospective natural history study to measure progression of physical impairment, activity limitation and quality of life in the condition. Duchenne muscular dystrophy is a progressive muscle disorder caused by mutations in dystrophin, a protein which plays an important structural role in muscle cells. Boys living with Duchenne experience progressive muscle weakness, resulting in serious medical complications and death.

A complete listing of the company's data presentations are as follows:

## Oral Presentation

### Title

G.O. 20: Reveglucosidase alfa (BMN 701), a GILT-tagged recombinant human acid alpha glucosidase (rhGAA), evaluation in late onset Pompe disease: Preliminary clinical efficacy and safety results of an extension study (72-week results)

### Authors

Roberts M, Hiwot T, Barohn R, Bratkovic D, Byrne B, Desnuelle C, Hughes D, Laforet P, Mengel E, Yang K, Heusner C, Dummer W

**Presentation: Sun., Oct. 4, 2015 from 8:45 - 9:00**

## Poster Presentations

### Pompe disease

#### Title

G.P. 8: An international, phase 3, switchover study of reveglucosidase alfa (BMN 701) in subjects with late onset Pompe disease (INSPIRE study)

#### Authors

Schooser B, Byrne B, Eyskens F, Hiwot T, Hughes D, Kissel J, Mengel E, Mozaffar T, Pestronk A, Roberts M, Sivakumar K, Statland J, Young P, Heusner C, Dummer W

**Guided Poster Session: Thurs., Oct. 1 from 14:30 - 16:00**

G.P. 14: Lung function tests (MIP, MEP, VC, FVC) predict ventilation and wheelchair use in late-onset Pompe disease

Roberts M, Mozaffar T, Young P, Johnson EM, Quartel A, Berger KI.

**Guided Poster Session: Thurs., Oct. 1 from 14:30 - 16:00**

### Duchenne Muscular Dystrophy

#### Title

G.P. 351: Exploring the route from exon skipping antisense oligonucleotide administration to functional effects in muscle of the mdx mouse model of Duchenne muscular dystrophy

#### Authors

Datson N, Weij R, Bijl S, Janson A, Vermue R, Testerink J, van Deutekom J

**Guided Poster Session: Fri., Oct. 2 from 16:30 - 18:00**

G.P. 389: A prospective natural history study to measure progression of physical impairment, activity limitation and quality of life in Duchenne muscular dystrophy

Goemans N, Wong B, McDonald C, Mason C, Hall A, Champion G

**Guided Poster Session: Fri., Oct. 2 from 16:30 - 18:00**

## **About BioMarin**

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. The company's portfolio consists of five commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit [www.BMRN.com](http://www.BMRN.com).

CONTACT: Investors:

Traci McCarty

BioMarin Pharmaceutical Inc.

(415) 455-7558

Media:

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451

---

<https://investors.biomin.com/2015-09-30-BioMarin-to-Present-Research-Results-on-Duchenne-Muscular-Dystrophy-and-Pompe-Disease-Programs-at-the-20th-International-Congress-of-the-World-Muscle-Society>