

BioMarin to Highlight Breadth of Innovative Development Pipeline at R&D Day on November 7th in New York

Key Research & Development Pipeline Products and Data to be Presented, Including:

- Vosoritide for the Treatment of Achondroplasia 42-Month Data Update
- Gene Therapy Product for the Treatment of Phenylketonuria Data and Program update

SAN RAFAEL, Calif., Nov. 1, 2018 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) will host an R&D Day from 8:30am (ET) to 11:30am (ET) on Wednesday, November 7, 2018 in New York City. BioMarin management and external experts will provide an update to the investment community on the Company's development portfolio, which is focused on innovative therapies to treat rare and ultra-rare diseases.

BioMarin R&D Day 2018 Agenda



Welcome and Opening Remarks

- *Update on Programs not Covered in Core Sessions*, presented by Hank Fuchs, M.D., President, Worldwide Research & Development, BioMarin

Vosoritide for Achondroplasia

- *42-Month Vosoritide Data Update*, presented by Jonathan Day, M.B.B.S., Ph.D., Executive Medical Director, Clinical Science, BioMarin
- *Drug Development Considerations in Achondroplasia*, presented by Ron Rosenfeld, M.D., Professor and Chair (emeritus) of Pediatrics, Oregon Health & Science University and Former Senior Vice President and Medical Director, Lucile Packard Foundation for Children's Health

Valoctocogene Roxaparvovec Gene Therapy for Hemophilia A

- *Regulatory Path Considerations*, presented by Geoff Nichol, M.B., Ch.B., M.B.A., Senior Vice President, Chief Medical Officer and Head of Global Clinical Development, BioMarin
- *Considerations for Manufacture of AAV-Based Gene Therapy Products*, presented by Robert Baffi, Ph.D., M.B.A., Executive Vice President, Technical Operations, BioMarin

Regulatory Policy Advances at the Food and Drug Administration

- *The Evolving Regulatory Landscape and Significance of Patient Engagement*, presented by Adora Ndu, Pharm.D., J.D., Executive Director and Head of Global Regulatory Policy, Research, and Patient Engagement, BioMarin

PKU Gene Therapy Data and Program update; R&D Pipeline Deep Dive

- *Genetics, Genomics and the Future of Us*, presented by Lon Cardon, Ph.D., Senior Vice President and Chief Scientific Officer, BioMarin

Closing Statements

- *Key Milestones and Catalysts*, presented by Jean-Jacques Bienaimé, Chairman and Chief Executive Officer, BioMarin

Live Video Webcast

Interested parties may access a live video webcast that will include audio and slides of the presentations [here](#). A replay of the meeting will be archived on the site for at least one week.

For those who choose not to listen and view the event via webcast, dial-in information for the audio portion of the webcast can be accessed using:

USA: (913) 227-1201
USA /Canada (toll free): (866) 705-2554
Conference ID: 6342187

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. The company's portfolio consists of seven commercialized products and multiple clinical and pre-clinical product candidates.

For additional information, please visit www.biomin.com. Information on BioMarin's website is not incorporated by reference into this press release.

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