

## **BioMarin to Present Pipeline at Upcoming R&D Day on Tuesday, September 12th**

*BioMarin Leadership and External Medical Experts to Discuss 7 Product Candidates in Clinical Development as well as ROCTAVIAN and VOXZOGO New Indication Opportunities*

*R&D Day to be Webcast Beginning at 8:00 a.m. Eastern Time September 12<sup>th</sup>*

SAN RAFAEL, Calif., Sept. 6, 2023 [/PRNewswire/](#) -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN), a global biotechnology company dedicated to transforming lives through genetic discovery, will host an R&D Day at 8:00 a.m. ET on Tuesday, September 12, 2023. BioMarin management and external experts will provide an update to the investment community on the company's development portfolio.

### **Key topics to be discussed at BioMarin's R&D Day:**

#### **Pipeline Programs:**

- *BMN 255 for hyperoxaluria in nonalcoholic fatty liver disease*
- *BMN 349 for alpha-1 antitrypsin deficiency*
- *BMN 331 for hereditary angioedema*
- *BMN 351 for Duchenne muscular dystrophy*
- *BMN 293 for myosin binding protein C3 hypertrophic cardiomyopathy*
- *New Candidate BMN 365 for plakophilin-2 (PKP2) arrhythmogenic cardiomyopathy*
- *New Candidate BMN 355 for long-QT syndrome types 2 and 3*

### **ROCTAVIAN panel discussion on severe hemophilia A and new indications:**

- Professor Johnny Mahlangu, MBBCh, MMed, FCPATH – Director Haemophilia Comprehensive Care, Charlotte Maxeke Johannesburg Academic Hospital, Professor of Haematology, University of the Witwatersrand and National Health Laboratory Service, Johannesburg
- Professor Amit Nathwani, MBChB, FRCP, RCPATH, Ph.D.- Director of the Katharine Dormandy Hemophilia Centre, Royal Free Hospital, London

- Guy Young, M.D. – Director, Hemostasis and Thrombosis Center and Clinical Coagulation Laboratory, Children's Hospital; Professor of Pediatrics, University of Southern California's Keck School of Medicine, Los Angeles

### **VOXZOGO panel discussion on achondroplasia and new indications:**

- Andrew Dauber, M.D. - Chief of Endocrinology, Children's National Hospital, Washington, D.C.
- Melita Irving, M.B.B.S. - Consultant in Clinical Genetics, Guy's and St. Thomas' NHS Foundation Trust, Evelina Children's Hospital, London, UK
- Bradley Miller, M.D., Ph.D. - Director, Division of Endocrinology, M Health Fairview Masonic Children's Hospital; Professor, Department of Pediatrics, University of Minnesota Medical School, Minneapolis, Minnesota

### **Live Audio Webcast**

An audio webcast of the presentations will be available live. You can access the webcasts at: <https://investors.biomarin.com/>. An archived version of the remarks will also be available through the Company's website for a limited time following the event.

### **About BioMarin**

Founded in 1997, BioMarin is a global biotechnology company dedicated to transforming lives through genetic discovery. The company develops and commercializes targeted therapies that address the root cause of the genetic conditions. BioMarin's unparalleled research and development capabilities have resulted in eight transformational commercial therapies for patients with rare genetic disorders. The company's distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options. For additional information, please visit [www.biomarin.com](http://www.biomarin.com).

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