

BioMarin to Present Innovative Pipeline at R&D Day on September 12th in New York

Live Audio Webcast to Begin at 8:00 a.m. Eastern Time

SAN RAFAEL, Calif., Sept. 11, 2023 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN), a global biotechnology company dedicated to transforming lives through genetic discovery, will update the investor community on the company's research and development portfolio, including early-stage product candidates and new indication opportunities with VOXZOGO and ROCTAVIAN. In addition, external clinicians from leading global institutions will participate in panel discussions.

"We look forward to sharing progress across our earlier-stage, next generation product candidates from the most diverse and high-potential pipeline in our company's history," said Hank Fuchs, M.D., president of Worldwide Research and Development at BioMarin.

Pipeline programs to be presented:

Therapeutic Focus	Candidate	Technology	Condition
Musculoskeletal/ Metabolic	BMN 255	Small molecule	Hyperoxaluria in nonalcoholic fatty liver disease
	BMN 351	Exon 51 oligonucleotide	Duchenne muscular dystrophy
Cardiovascular	BMN 293	AAV gene therapy	Myosin binding protein C3 hypertrophic cardiomyopathy
	BMN 365	AAV gene therapy	Plakophilin-2 (PKP2) arrhythmogenic cardiomyopathy
	BMN 355	Monoclonal antibody	Long QT syndrome types 2 and 3
Other	BMN 349	Small molecule	Alpha-1 antitrypsin deficiency
	BMN 331	AAV gene therapy	Hereditary angioedema

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.

Contacts:

Investors

Media

Traci McCarty

Andrew Villani

BioMarin Pharmaceutical Inc. BioMarin Pharmaceutical Inc.

(415) 455-7558

(628) 269-7393

SOURCE BioMarin Pharmaceutical Inc.

<https://investors.biomarin.com/2023-09-11-BioMarin-to-Present-Innovative-Pipeline-at-R-D-Day-on-September-12th-in-New-York>