

A MESSAGE FROM THE CHAIRMAN AND CEO

Dear Stockholder of BioMarin:

In 2022, BioMarin achieved record revenue and pivoted to GAAP profitability, making it a truly transformational year for the company and laying the foundation for long-term growth. The global launch of VOXZOGO for children with achondroplasia helped drive that revenue growth. We believe conditional European approval of our gene therapy for the treatment of severe hemophilia A, ROCTAVIAN, has set the stage for further growth in 2023, when we also hope to gain approval in the United States. Our pipeline is the most diverse in BioMarin's history and we expect to advance a number of early-stage programs in 2023.

Commercial Strength

The strength of our core enzyme replacement products and the ongoing launch of VOXZOGO, which we expect to accelerate further in 2023, helped drive 2022 revenue growth. More than 1,200 children in 32 countries around the world have now received VOXZOGO, which addresses the root cause of achondroplasia, the most common form of disproportionate short stature. In Japan, the medicine is approved for children from birth, and we have also filed for regulatory approval in the United States and Europe with the goal of expanding use of the medicine to include children of all ages.

Following European conditional approval of ROCTAVIAN, we are making important progress as we work through the complex process of securing reimbursement through outcomes-based agreements in Germany and other markets, preparing us for commercial use of the gene therapy in 2023. In parallel, we have prepared for a potential U.S. Food and Drug Administration approval of ROCTAVIAN by identifying hemophilia treatment centers and actively meeting with U.S. payers to prepare for the anticipated launch.

Advancing Our Pipeline

Our scientific legacy in advancing therapeutics to treat rare conditions and our industry-leading expertise in understanding the genetic causes of disease position us for our next chapter. We have pivoted to focus on genetic conditions with larger patient populations, as well as more common diseases with an underlying genetic component. As genetic sequencing becomes more commonly utilized, we are understanding to a greater level of detail the contributions of genetics to disease expression. Additionally, innovative medicines are increasingly sophisticated in design, making it possible to transform lives in ways that were previously unimaginable. We are uniquely positioned to take sustainable advantage of this context, with approximately 30 clinical and preclinical candidates advancing in our earlier-stage pipeline.

We are actively pursuing ways to reach a greater number of people with both severe hemophilia A and genetic short stature. In addition to working to bring VOXZOGO to the youngest children, we are evaluating its potential to treat other genetic forms of short stature, including hypochondroplasia, NPR2 deficiency and Noonan syndrome. We are also exploring a number of groups of patients who might benefit from treatment with ROCTAVIAN, in addition to those we hope to be included in our initial indication.

Beyond these areas, we are pleased with the progress of our early-stage pipeline. We have generated some early evidence of the ability to improve metabolism and protect patients from the damaging impact of recurrent kidney stones. And we are generating data that could lead to transformation in the care of people with hereditary angioedema. Further, we anticipate bringing three product candidates into the clinic this year: BMN 351 for Duchenne muscular dystrophy, BMN 349 for alpha-1 antitrypsin deficiency and BMN 293, a gene therapy for MYBPC3 hypertrophic cardiomyopathy.

World-Class, End-to-End Manufacturing

With more than two decades of operational experience, BioMarin is a leader in biologic and gene therapy production from supporting R&D and interacting with regulatory authorities to supplying the demand for our clinical and commercial therapies worldwide. The foundation of this leadership position is our wholly owned manufacturing facilities, which allow us to maintain world-class quality standards



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end-to-end and demonstrate significant flexibility. Along with the efficient production and delivery capabilities, we have the capacity to scale our manufacturing output to meet clinical and commercial demands. We operate three cGMP (current Good Manufacturing Practices) manufacturing plants in Novato, California and Shanbally, Ireland, which include a 18,000 square-foot, state-of-the-art gene therapy manufacturing facility in Novato.

Anticipating Sustained Profitability

In 2022, we executed on our growth strategy developed over the past several years, reaching a milestone that is transformative in the trajectory of a biotechnology company: GAAP profitability, which we anticipate sustaining for the foreseeable future. We achieved double-digit revenue growth in 2022 and expect to do the same in 2023, driven by the strength of our durable – and growing – core enzyme business, as well as the introduction of two products, VOXZOGO and ROCTAVIAN, which represent the largest opportunities in BioMarin’s history. Our focus on controlling operating expenses while significantly growing revenue has enabled us to deliver on both the top-line and bottom-line.

The strength and diversity of our base business in more than 75 countries around the world, and the potential of VOXZOGO and ROCTAVIAN, lay the foundation for value creation for all of our stakeholders. We believe we have built an organization capable of supporting the expansion of our marketed products and the development of an innovative pipeline, while at the same time sustainably increasing our profitability over the coming months and years.

Employee Experience

We are well aware that our talented employees are key to our success. We are focused on creating a working environment that provides opportunities for employees to learn and grow as leaders and technical experts, and fosters a sense of belonging, community and engagement. We are committed to diversity, equity and inclusion (DEI) throughout the employee lifecycle, from ensuring a respectful and fair experience for those we are recruiting, to retaining and developing those who contribute to our success. We offer robust and equitable rewards packages, career development opportunities, and benefits and wellbeing programs that are designed to support employees throughout their time at BioMarin. We also are dedicated to having an employee base that represents the diversity of the communities in which we operate.

Responsible Citizenship

We are committed to ensuring that we are acting in a socially and environmentally responsible way in the communities where we do business. As a global corporate citizen, BioMarin operates in a manner that protects the environment and provides a safe and healthy workplace. Creating a cleaner and more sustainable world is consistent with our values. We have developed a roadmap to assess and provide more transparency into our greenhouse gas (GHG) emissions and mitigation efforts, ultimately leading to a multi-year data collection and to the development of strategies to minimize the company’s carbon footprint. We strive to reduce our environmental impact in numerous other ways, including lowering water consumption, using solar energy and purchasing renewable energy, and reducing waste with reusable bioprocessing materials.

In closing, I am proud of the progress we made in 2022, as we built on the success of our first 25 years with continued growth of our enzyme replacement products and the introduction of important new therapies that address the unmet needs of larger patient populations. I would like to thank our more than 3,000 employees around the world, without whom our success would not be possible. On behalf of the entire company and our Board of Directors, I would also like to thank you for your investment in BioMarin. We look forward to delivering on more opportunities in 2023, as we continue to transform lives through genetic discovery.

Sincerely,



Jean-Jacques Bienaimé

Chairman and Chief Executive Officer
BioMarin Pharmaceutical Inc.

Forward-Looking Statements

This letter contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin) including, without limitation, statements regarding BioMarin’s strategies, growth and profitability as well as the progress of its early-stage programs, continued clinical development of its clinical programs and commercialization of its commercial products and product candidates. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. Such risks and uncertainties include those factors detailed in BioMarin’s filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption “Risk Factors” in BioMarin’s Annual Report on Form 10-K for the year ended December 31, 2022, as such factors may be updated by any subsequent reports. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation, to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise. See our Proxy Statement for our 2023 Annual Meeting of Stockholders for further information regarding our use of forward-looking statements.