# A MESSAGE FROM THE CHAIRMAN AND CEO

BIOMARIN PHARMACEUTICAL INC.
WWW.BMRN.COM
415.506.6700

BioMarin managed through the challenges brought about by the COVID-19 global pandemic with persistence and agility. Demand for our medicines drove strong results in 2020. With our foundation of products that constitute a strong base business, we drove positive operating cash flows, enabling the advancement of our next two potential significant product opportunities: vosoritide for achondroplasia and valoctocogene roxaparvovec gene therapy for severe hemophilia A. If vosoritide is approved and launched commercially, we would expect significant revenue growth starting in 2022. We also look toward our earlier-stage pipeline, which is designed to ensure a steady flow of new product opportunities on the medium and longer-term horizons.

## ANTICIPATED MILESTONES

In 2021, we have several important regulatory events that we expect will drive substantial value over the coming quarters. For vosoritide for the treatment of children with achondroplasia, in Europe we expect a Committee for Medicinal Products for Human Use (CHMP) opinion this June, followed by the European Commission decision in late summer, potentially leading to a commercial launch this fall. In the US, our target Food and Drug Administration (FDA) Prescription Drug User Fee Act (PDUFA) action date is August 20, 2021. Our decision to supplement the review with the two-year Phase 3 data, however, could result in a major amendment pushing the PDUFA target action date out by three months to November 2021.

BioMarin recently withdrew its Marketing Authorization Application (MAA) for valoctocogene roxaparvovec for severe hemophilia A and plans to resubmit the MAA along with 52-week results from the full Phase 3 study to the European Medicines Agency (EMA) in the second quarter of 2021. Assuming we remain on anticipated timelines in Europe, this could potentially lead to a CHMP opinion for valoctocogene roxaparvovec in the first half of 2022, with potential European launch in the third quarter of 2022. We recently announced that the FDA granted us Regenerative Medicine Advanced Therapy or "RMAT" designation, which is complementary to the Breakthrough Therapy Designation we received in 2017. The RMAT designation will provide FDA more options to leverage data for post-approval requirements, which could be important for gene therapies such as valoctocogene roxaparvovec. We recognize FDA's initial request to see two years of data from the Phase 3 study to evaluate the safety and efficacy of this investigational treatment. In the U.S., the FDA recommended that the Company complete the Phase 3 study and submit two-year follow-up safety and efficacy data on all study participants.

## R&D ENGINE: PROMISING EARLY STAGE PIPELINE

In our earlier-stage pipeline, we have multiple programs moving forward, including BMN 307 gene therapy for phenylketonuria (PKU). Based on encouraging Phe lowering and safety observed in the BMN 307 Phase 1/2 study with the low dose, we will be moving imminently to the next dose of 6e13vg/kg. We will share an update on BMN 307 once we have selected the dose for registration-enabling studies. We are conducting this study with material manufactured with a commercial-ready process to de-risk the program and to facilitate rapid clinical development. We are excited about the prospect of BMN 307 as it represents a potential third PKU treatment option in our PKU franchise and our second gene therapy development program.

We doubled our early-stage pipeline in 2020 via internal growth and external partnerships, advancing several pre-clinical programs spanning multiple modalities. With gene therapy, beyond our valoctocogene roxaparvovec and PKU programs, we are conducting Investigational New Drug (IND) enabling studies with BMN 331 gene therapy for hereditary angioedema. In addition, our collaboration with DiNAQOR AG, a gene therapy platform company, on hypertrophic cardiomyopathies is progressing well and demonstrates our commitment to developing promising gene therapy candidates. Together, we plan to select our candidate vector in 2021 for this program and to commence non-clinical development studies to enable a subsequent IND submission.

Our pre-clinical candidate, BMN 351, is an oligonucleotide therapy for the treatment of Duchenne Muscular Dystrophy. BMN 351 has demonstrated a high level of protein expression in disease models possessing skippable dystrophic mutations and at doses that are promising in regard to safety. Finally, at the advanced pre-clinical stage, the FDA granted BMN 255, our small molecule for a chronic renal disease, IND status and is progressing well. We plan to share updates across our earlier-stage pipeline at R&D Day later in 2021.

## FOCUS ON DIVERSITY

We are proud to have earned the top score of 100 on the 2021 Human Rights Campaign Corporate Equality Index for lesbian, gay, bisexual, transgender and queer (LGBTQ) LGBTQ-inclusive workplace policies and practices. It is an honor to be named a "Best Place to Work for LGBTQ Equality. BioMarin is committed to cultivating a diverse, equitable and inclusive community for our employees. We are focused on our most important asset, our people, to ensure we are the premier place to work and that our people can bring their best selves to work. More diverse and inclusive workforces drive financial outcomes, employee investment in work, creativity and innovation, which for BioMarin translates into more breakthrough therapies more rapidly and to more people with rare diseases.

# STEWARDS OF THE ENVIRONMENT

It is up to all of us to protect our planet. As global citizens, we strive to operate in a way that protects the environment and provides a safe and healthful workplace for personnel and the communities that we serve. Our Ireland manufacturing site is certified to ISO 14001 Environmental Management Systems and ISO 45001 Occupational Health and Safety Management Systems standards. Our Northern California manufacturing site also conforms to management system standards for environmental and occupational health and safety. We recently updated our website to include our Roadmap to Enhanced Greenhouse Gas Disclosures.

# POTENTIAL COMMERCIAL EXPANSION ON THE HORIZON

In 2021, we will continue our launch readiness efforts for vosoritide, which if approved, we expect to be our largest brand to date based upon potential revenues. We are actively preparing for possible launches in our two largest regions, North America and Europe, Middle East and Africa (EMEA) in the second half of the year. With two applications moving forward in parallel, we have two complementary and attractive commercial opportunities ahead.

# VALUE PROPOSITION

Looking forward, with respect to potential approvals and launches, we believe that the commercial market opportunities for vosoritide and valoctocogene roxaparvovec together significantly exceed the aggregate addressable market sizes of our current products. Therefore, we believe the combination of the positive operating results expected from BioMarin's base business, plus the growth potential from the Company's large, late-stage opportunities creates a compelling value proposition that is further bolstered by our commitment to early-stage, innovative rare disease research and development.

We remain grateful to our employees, who make possible our important work of delivering scientific breakthroughs and transformative treatments to people with rare diseases. We are inspired by the patients and their families who we serve and work tirelessly on their behalf. Our success is inextricably linked to the physicians and nurses who treat our patients, academic researchers, regulatory authorities, government officials, payers and the local communities where we live and operate. Together, we strive to address the unmet medical needs of people with rare diseases by creating new treatment paradigms.

Sincerely,

Jean-Jacques Bienaimé
Chairman and Chief Executive Officer

## *Forward-Looking Statements*

*This letter contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about BioMarin's expected continued revenue growth and the expected drivers of such growth; BioMarin's ability to reach expected milestones for products and product candidates in its pipeline and the timing of such milestones; the continued clinical development and commercialization of BioMarin's commercial products and product candidates; the possible approval and commercialization of BioMarin's product candidates, including vosoritide and valoctocogene roxaparvovec, and the resulting impact on BioMarin's revenues and growth; the potential market opportunities for vosoritide and valoctocogene roxaparvovec, including the statement that the commercial market opportunities for vosoritide and valoctocogene roxaparvovec together significantly exceed the aggregate addressable market sizes of our current products; actions by regulatory authorities; and other clinical development, regulatory interactions, manufacturing and commercial operations in 2021. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: BioMarin's ability to successfully commercialize its current or future commercial products; the results and timing of current and planned preclinical studies and clinical trials; BioMarin's ability to successfully manufacture its commercial products and product candidates; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities concerning BioMarin's products and product candidates; the market for BioMarin's products and product candidates; actual sales of BioMarin's commercial products; the impact of the COVID-19 pandemic on BioMarin's business and the foregoing factors; and 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, 2020, 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.*