A Message from the Chairman and CEO

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As we enter the next decade, our pace of scientific innovation continues to increase. In 2020, we celebrate one of our earliest breakthroughs, the 15th anniversary of the approval of Naglazyme® (galsulfase), which laid the groundwork for our leadership in rare diseases. Our approved products, our R&D engine, our manufacturing expertise, and the employees supporting BioMarin create a solid base for growth that we believe will support us in continuing our success into the new decade.

As I write this letter, we face an unprecedented pandemic that is heart wrenching and will profoundly impact our society. During this time, we gain a greater appreciation of the value of the life sciences industry as a force for good. Never before has there been so much hope and need in our industry's ability to change the course of disease.

During this uncertain time, we believe BioMarin is well-positioned to limit disruptions to our operations due to the essential therapies we provide for people with rare diseases. And while economic uncertainty related to the pandemic will touch every company, including ours, we are monitoring the situation very closely, adjusting where necessary and remaining focused on the long-term as we manage through this health crisis. BioMarin is an "essential business" based on the importance of our medicines, and as such, we are making every effort to ensure continued access to our therapies for our patients no matter where they are located. As scientific pioneers, we are comfortable navigating uncharted territory, an ability that serves us in this health crisis, as well as in our business overall.

Operational Excellence

We delivered record results for the full-year 2019, demonstrating our persistent emphasis on operational excellence. In parallel, our productive R&D engine advanced the next wave of product candidates expected to contribute significantly to top-line growth.

We begin the new decade with an established base business that is foundational to our financial strength. Our revenue growth and improvement in profitability also increased our operating cash flows. We have two potential blockbusters on the horizon that are all expected to drive meaningful growth.

With valoctocogene roxaparvovec, in the last 12 months we met the criteria necessary to submit applications for expedited review in the United States and Europe, submitted and were granted acceptance of both applications, completed enrollment in our pivotal Phase 3 study and produced material from our own GMP facility to prepare for a potential commercial launch later this year. In the United Sates, the Food and Drug Administration (FDA) set an action date of August 21, 2020, and we also expect the European decision in the second half of this year. Moving this development program from the first human administration to marketing application review in four years is an achievement in and of itself. The potential for valoctocogene roxaparvovec to completely change the treatment paradigm for severe hemophilia A is an even greater feat.

We want to thank the hemophilia community for providing BioMarin the opportunity and support to develop valoctocogene roxaparvovec. A major part of this support comes from the dedication and commitment of key opinion leaders in the field.

Also in 2019, with vosoritide for achondroplasia, we were pleased to have shared the highly statistically significant one-year results from the Phase 3 study demonstrating a strong increase in growth velocity across the 121 children ages 5 through 14 who participated. Based on recent meetings with health authorities in the U.S. and Europe, we plan to submit marketing applications to the FDA and the European Medicines Agency in the third quarter of 2020. As with valoctocogene roxaparvovec, we anticipate the highly innovative attributes of vosoritide will drive uptake from patients and meaningful contributions to the business upon potential approval and launch. There is tremendous unmet medical need in this patient population, as demonstrated by the level of interest from families seeking treatment for their very young children.

We believe the next two years present an enormous opportunity for value creation based on the following: potential launches in valoctocogene roxaparvovec and vosoritide, if approved, continued expansion of our PKU franchise with global Palynziq® (pegvaliase-pqpz) penetration and the development of PKU gene therapy, initial clinical development of a gene therapy for Hereditary Angioedema (HAE), our abundant cash reserves, our wholly owned manufacturing facilities and our global footprint.

As we begin a new decade having already built a strong base business, transitioned our pipeline to address larger rare indications, and laid the foundation for significant profitability if valoctocogene roxaparvovec and vosoritide are approved, we believe we have diversified risk and positioned ourselves for substantial success in both the near-term and long-term.

Value Proposition of a One Time Treatment for Severe Hemophilia A

In contemplating the potential commercial value of valoctocogene roxaparvovec, it is necessary to understand and acknowledge the significant burden and cost of severe hemophilia A given the current standard of care, chronic prophylactic FVIII. Designed as a single infusion with ongoing effects, valoctocogene roxaparvovec has the potential to dramatically change the treatment paradigm and how

we think about managing severe hemophilia A. The growing body of data from our clinical trials suggests that the potential benefits of treating patients with valoctocogene roxaparvovec over chronic, prophylactic FVIII therapy could provide meaningful cost savings to the healthcare system.

A recent publication has estimated the average annual cost of prophylactic FVIII to be between $700-750K. Modeling these annual costs forward for an adult male, an 18-year-old with life expectancy to 71 years, could result in an aggregate cost of $38 million of factor replacement, the predominant treatment, at today's costs. In the context of these high costs, and at a presumed price in line with other recently approved gene therapy products, cost-effectiveness of valoctocogene roxaparvovec could be expected in about two and a half years.

Promising Early Stage Pipeline

This year, we expect to start enrolling patients in the PHEarless Phase 2 study for BMN 307, our investigational gene therapy for PKU. This study could potentially be registration enabling as we are conducting it with material manufactured with a commercial-ready process to de-risk the program and 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 a second gene therapy development program, leveraging our learnings and capabilities from valoctocogene roxaparvovec.

Finally, our earlier-stage pipeline includes our BMN 331 gene therapy product candidate for HAE, a rare and potentially life-threatening genetic disease that causes swelling throughout the body, which could include choking because of swelling of the airway. We expect to complete preclinical work with BMN 331 this year, in anticipation of a possible clinical trial in early 2021. We are also moving forward this year to test vosoritide in Dominantly Inherited Short Stature (DISS) and anticipate a possible investigator sponsored trail later this year as part of a research collaboration with Children's National Hospital.

Our early pipeline employs our development strategy to build on our technical expertise of gene therapy in PKU and HAE, to explore developing one treatment for multiple indications as we are doing by investigating vosoritide in DISS, and to develop multiple treatments for one disease area like PKU with a potential third product candidate in development.

Driving Growth, Entering the Next Decade

As described earlier, we believe we have developed a clear and achievable strategy that is designed to position BioMarin for long-term success.

In conclusion, we are very pleased with our achievements in 2019 and full of anticipation for what lies ahead in the next decade. We are grateful to our employees who work tirelessly to pioneer treatments that advance the standard of care for patients with rare genetic diseases, the patients and their families who inspire us to deliver scientific breakthroughs, the physicians and nurses who treat our patients, academic researchers, regulatory authorities, government officials, payers, and the local communities where we live and operate.

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 continued revenue growth, financial success and opportunities for value creation; BioMarin's ability to reach expected milestones for products and product candidates in its pipeline; the continued clinical development and commercialization of BioMarin's commercial products and product candidates and the timing of such development and commercialization; the possible approval and commercialization of BioMarin's product candidates, the expected contributions of these product candidates to top-line growth, if approved, and the potential for these product candidates to provide meaningful cost-savings to the healthcare system and change treatment paradigms, if approved; actions by regulatory authorities and the timing of such actions; BioMarin's ability to navigate through and operate during the COVID-19 pandemic, including it being well-positioned to limit disruptions to its operations; BioMarin's diversification of risk and position for substantial success in both the near-term and long-term; BioMarin's strategy for long-term success and the clarity and achievability of that strategy; and other clinical development, regulatory interactions, manufacturing and commercial operations in 2020 and beyond. 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, as well as the potential impact of the COVID-19 pandemic on BioMarin's ability to continue such preclinical studies and clinical trials and the timing of such 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, including the potential impact of the COVID-19 pandemic on the regulatory authorities' abilities to issue such decisions and the timing of such decisions; the market for BioMarin's products and product candidates; actual sales of BioMarin's commercial products; 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, 2019, 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.