UNITED STATES DISTRICT COURT
NORTHERN DISTRICT OF CALIFORNIA

_____, Individually and On Behalf of All Others Similarly Situated, Plaintiff, v. BIOMARIN PHARMACEUTICAL INC., JEAN-JACQUES BIENAIMÉ, BRIAN R. MUELLER, and HENRY J. FUCHS, Defendants.	Case No. CLASS ACTION COMPLAINT FOR VIOLATIONS OF THE FEDERAL SECURITIES LAWS DEMAND FOR JURY TRIAL

Plaintiff _____ ("Plaintiff"), individually and on behalf of all other persons similarly situated, by Plaintiff's undersigned attorneys, for Plaintiff's complaint against Defendants, alleges the following based upon personal knowledge as to Plaintiff and Plaintiff's own acts, and information and belief as to all other matters, based upon, *inter alia*, the investigation conducted by and through Plaintiff's attorneys, which included, among other things, a review of the Defendants' public documents, conference calls and announcements made by Defendants, United States ("U.S.") Securities and Exchange Commission ("SEC") filings, wire and press releases published by and regarding BioMarin Pharmaceutical Inc. ("BioMarin" or the "Company"), analysts' reports and advisories about the Company, and information readily obtainable on the Internet. Plaintiff believes that substantial additional evidentiary support will exist for the allegations set forth herein after a reasonable opportunity for discovery.

NATURE OF THE ACTION

1. This is a federal securities class action on behalf of a class consisting of all persons and entities other than Defendants that purchased or otherwise acquired BioMarin securities between February 28, 2020 and August 18, 2020, both dates inclusive (the "Class Period"), seeking to recover damages caused by Defendants' violations of the federal securities laws and to pursue remedies under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 (the "Exchange Act") and Rule 10b-5 promulgated thereunder, against the Company and certain of its top officials.

2. BioMarin was founded in 1996 and is headquartered in San Rafael, California. BioMarin is a biotechnology company that develops and commercializes therapies for people with serious and life-threatening rare diseases and medical conditions. The Company's product candidates include, among others, valoctocogene roxaparvovec, an investigational adeno-associated virus ("AAV") gene therapy, which is in Phase 3 clinical development for the treatment of patients with severe hemophilia A.

3. Based on BioMarin's Phase 1/2 study results, the investing and medical community viewed valoctocogene roxaparvovec as a likely candidate for becoming the first gene therapy approved by the U.S. Food and Drug Administration ("FDA") for hemophilia in the U.S.

4. In May 2019, BioMarin announced the interim results from its Phase 3 study of valoctocogene roxaparvovec for adults suffering from severe hemophilia A. The results, although showing the treatment's effectiveness, disappointed the market because they indicated a reduction in durability of effectiveness from the results shown in the Phase 1/2 results. That is, under the Phase 3 results, it was not clear whether valoctocogene roxaparvovec's effectiveness would last as long as indicated in the Phase 1/2 study, and thus, whether valoctocogene roxaparvovec would be a single treatment, or one requiring more than one treatment over the life of a patient. These concerns echoed those that first arose in 2018, after the Company reported a disappointing two-year update from the Phase 1/2 study, which showed factor VIII levels waning over time.

5. Apparently dismissing these concerns, in December 2019, BioMarin submitted a Biologics License Application ("BLA") to the FDA for valoctocogene roxaparvovec for adults with hemophilia A based on the interim analysis of the ongoing Phase 3 study of valoctocogene roxaparvovec, as well as three-year data from the Company's Phase 1/2 study of valoctocogene roxaparvovec. BioMarin also announced that the European Medicines Agency ("EMA") validated the Company's Marketing Authorization Application ("MAA") for valoctocogene roxaparvovec for adults with severe hemophilia A, with the MAA review to commence in January 2020 under accelerated assessment.

6. Throughout the Class Period, Defendants made materially false and misleading statements regarding the Company's business, operational and compliance policies. Specifically, Defendants made false and/or misleading statements and/or failed to disclose that: (i) differences between the Phase 1/2 and Phase 3 study of valoctocogene roxaparvovec limited the reliability of

the Phase 1/2 study to support valoctocogene roxaparvovec's durability of effect; (ii) as a result, it was foreseeable that the FDA would not approve the BLA for valoctocogene roxaparvovec without additional data; and (iii) as a result, the Company's public statements were materially false and misleading at all relevant times.

7. On August 19, 2020, BioMarin announced receipt of a Complete Response Letter ("CRL") from the FDA to the Company's BLA for valoctocogene roxaparvovec. BioMarin advised investors that in the CRL, "the FDA introduced a new recommendation for two years of data from the Company's ongoing 270-301 study (Phase 3) to provide substantial evidence of a durable effect using Annualized Bleeding Rate (ABR) as the primary endpoint" and "recommended that the Company complete the Phase 3 Study and submit two-year follow-up safety and efficacy data on all study participants." In explaining the new recommendation, the "FDA concluded that the differences between Study 270-201 (Phase 1/2) and the Phase 3 study limited its ability to rely on the Phase 1/2 study to support durability of effect."

8. On this news, BioMarin's stock price fell $41.82 per share, or 35.28%, to close at $76.72 per share on August 19, 2020.

9. As a result of Defendants' wrongful acts and omissions, and the precipitous decline in the market value of the Company's securities, Plaintiff and other Class members have suffered significant losses and damages.

JURISDICTION AND VENUE

10. The claims asserted herein arise under and pursuant to Sections 10(b) and 20(a) of the Exchange Act (15 U.S.C. §§ 78j(b) and 78t(a)) and Rule 10b-5 promulgated thereunder by the SEC (17 C.F.R. § 240.10b-5).

11. This Court has jurisdiction over the subject matter of this action pursuant to 28 U.S.C. § 1331 and Section 27 of the Exchange Act.

12. Venue is proper in this Judicial District pursuant to Section 27 of the Exchange Act (15 U.S.C. § 78aa) and 28 U.S.C. § 1391(b). BioMarin is headquartered in this Judicial District, Defendants conduct business in this Judicial District, and a significant portion of Defendants' activities took place within this Judicial District.

13. In connection with the acts alleged in this complaint, Defendants, directly or indirectly, used the means and instrumentalities of interstate commerce, including, but not limited to, the mails, interstate telephone communications, and the facilities of the national securities markets.

PARTIES

14. Plaintiff, as set forth in the attached Certification, acquired BioMarin securities at artificially inflated prices during the Class Period and was damaged upon the revelation of the alleged corrective disclosures.

15. Defendant BioMarin is a Delaware corporation with principal executive offices located at 770 Lindaro Street, San Rafael, California 94901. BioMarin's common stock trades in an efficient market on the Nasdaq Global Select Market ("NASDAQ") under the symbol "BMRN."

16. Defendant Jean-Jacques Bienaimé ("Bienaimé") has served as BioMarin's Chairman and Chief Executive Officer at all relevant times.

17. Defendant Brian R. Mueller ("Mueller") has served as BioMarin's Executive Vice President, Chief Financial Officer ("CFO") since June 2020. Before his promotion to Executive Vice President, CFO, Mueller had served as BioMarin's Senior Vice President, Acting CFO since February 2020.

18. Defendant Henry J. Fuchs, M.D. ("Fuchs") has served as BioMarin's President of Worldwide Research & Development at all relevant times.

19. Defendants Bienaimé, Mueller, and Fuchs are sometimes referred to herein as the "Individual Defendants."

20. The Individual Defendants possessed the power and authority to control the contents of BioMarin's SEC filings, press releases, and other market communications. The Individual Defendants were provided with copies of BioMarin's SEC filings and press releases alleged herein to be misleading prior to or shortly after their issuance and had the ability and opportunity to prevent their issuance or to cause them to be corrected. Because of their positions with BioMarin, and their access to material information available to them but not to the public, the Individual Defendants knew that the adverse facts specified herein had not been disclosed to and were being concealed from the public, and that the positive representations being made were then materially false and misleading. The Individual Defendants are liable for the false statements and omissions pleaded herein.

21. BioMarin and the Individual Defendants are sometimes collectively, in whole or in part, referred to herein as "Defendants."

SUBSTANTIVE ALLEGATIONS

Background

22. BioMarin was founded in 1996 and is headquartered in San Rafael, California. BioMarin is a biotechnology company that develops and commercializes therapies for people with serious and life-threatening rare diseases and medical conditions. The Company's product candidates include, among others, valoctocogene roxaparvovec, an investigational AAV gene therapy, which is in Phase 3 clinical development for the treatment of patients with severe hemophilia A.

23. BioMarin touted valoctocogene roxaparvovec as a potential one-time treatment gene therapy for patients suffering from severe hemophilia A, which is marked by a deficiency of

the FVIII protein to help with blood clotting and to prevent painful, potentially life-threatening bleeding from even modest injuries. Both the market and medical community hoped study results would show that after a single treatment of the valoctocogene roxaparvovec gene therapy, patients suffering from severe hemophilia A would no longer require a prophylactic regimen of Factor VIII infusions administered intravenously because the gene therapy promised to potentially rid patients of the FVIII protein deficiency for the life of the patient. As such, valoctocogene roxaparvovec was considered a potential "blockbuster" treatment with a projected price tag of $1-$3 million per treatment, representing potentially billions of dollars in sales.

24. Based on BioMarin's Phase 1/2 study results, the investing and medical community viewed valoctocogene roxaparvovec as a likely candidate for becoming the first gene therapy approved by the FDA for hemophilia in the U.S. However, expectations of the treatment were somewhat reduced in 2018, after the Company reported a disappointing two-year update from the Phase 1/2 study, which showed factor VIII levels waning over time.

25. On May 28, 2019, BioMarin announced interim results from the Phase 3 study of valoctocogene roxaparvovec for adults suffering from severe hemophilia A (the "May 2019 Press Release"). That press release reported, among other results, a 94% reduction in mean Factor VIII usage, whereas, in May 2018, the Company reported a 98% drop in average FVIII usage, implying a diminishing treatment effect, and causing investors to question whether the Company anticipated working on a retreatment paradigm if it turned out that valoctocogene roxaparvovec's treatment effect diminished over time.

26. Following release of the May 2019 Press Release, BioMarin's stock price fell $4.53 per share, or 5.09%, to close at $84.50 per share on May 28, 2019. Despite this decline in BioMarin's stock price, the Company's securities continued to trade at artificially inflated prices

because of Defendants' misstatements regarding valoctocogene roxaparvovec's market viability based on the Phase 1/2 study and interim results from the Phase 3 study.

27. For example, in the May 2019 Press Release, Defendants touted that BioMarin's "investigational gene therapy, valoctocogene roxaparvovec, for adults with severe hemophilia A achieved pre-specified clinical criteria for regulatory review in the U.S. and Europe"; that, "[a]s of May 28, 2019, eight patients in the 20-patient cohort of the Phase 3 GENEr8-1 study achieved Factor VIII levels of 40 international units per deciliter (IU/dL), or more, at 23 to 26 weeks, meeting the pre-specified criteria for Factor VIII activity levels"; and that "[t]he company will meet with the [FDA] and [EMA] to review the phase 3 data and the other elements of a submission and intends to announce the timing for its planned marketing applications in 3Q 2019."

28. The May 2019 Press Release also quoted Defendants Fuchs, who touted, in relevant part, that "[r]eaching this pre-specified clinical endpoint is an important milestone that brings [BioMarin] one step closer to a potential regulatory submission in both the U.S. and Europe for valoctocogene roxaparvovec to treat adults with severe hemophilia A," that Defendants' "discussions with the FDA and EMA underscore the high level of unmet need in the hemophilia community," and that Defendants "look forward to continuing [their] productive dialogue on the submissions."

29. On July 8, 2019, BioMarin issued a press release announcing that "based on recent meetings with health authorities in the U.S. and Europe, the [C]ompany plans to submit marketing applications to both the [FDA] and [EMA] in 4Q 2019 for its investigational gene therapy, valoctocogene roxaparvovec, for adults with severe hemophilia A" (the "July 2019 Press Release"). That press release represented, in relevant part, that "[t]hese submissions will be based on the updated three-year Phase 1/2 data and the recently completed Phase 3 interim analysis of patients treated with material from the to-be-commercialized process"; that "[b]oth submissions

are expected to represent the first time a gene therapy product for any type of hemophilia will be reviewed for marketing authorization by health authorities"; that "[b]oth the FDA and EMA continue to prioritize interactions related to the review of valoctocogene roxaparvovec through the Breakthrough Therapy Designation program in the U.S and the Priority Medicines (PRIME) regulatory initiative in Europe"; and that "[v]aloctocogene roxaparvovec is one of the first therapies to go through the new PRIME initiative."

30. The July 2019 Press Release also discussed an "ISTH [International Society on Thrombosis and Haemostasis] Late-Breaking Abstract," touting, in relevant part, that "[e]arlier today, Professor Pasi presented data in a late-breaking abstract session on the efficacy and safety of valoctocogene roxaparvovec in an ongoing Phase 1/2 study at the 27th [ISTH] 2019 Congress"; that "[t]he three-year update of the 6e13 vg/kg dose cohort in the Phase 1/2 study demonstrated that bleed rate control and reduction in Factor VIII usage was maintained for a third year following a single administration of valoctocogene roxaparvovec"; that, "[i]n the year prior to study entry, the mean Annualized Bleed Rate" ("ABR") "was 16.3 and the median was 16.5"; that, "[o]ver three years, the ABR was reduced to a mean of 0.6 and a median of zero"; that "[t]his represents a 96% reduction in participants' mean ABR, and there is 100% resolution of target joints"; that "[t]here was also a 96% reduction in participants' mean annualized Factor VIII usage rate over three years, and all participants remain off Factor VIII prophylaxis"; that "Factor VIII levels sustained over a three-year period were sufficient to achieve striking hemostatic efficacy"; and that "Factor VIII expression has entered a plateau phase where the rate of decline has substantially slowed, which could be indicative of durable, long-term expression."

31. Additionally, the July 2019 Press Release quoted Defendant Fuchs, who touted, in relevant part, that Defendants "applaud the FDA's efforts to incorporate the patient voice in the regulatory review process"; that "[p]owerful and moving testimonials from clinical study

participants have helped serve as a critical element in the FDA's considerations of potentially the first commercially available gene therapy for any type of hemophilia"; that, "[a]s important, [Defendants] commend the EMA PRIME initiative for enabling enhanced interactions and early dialogue that have optimized our development plans and have helped speed up evaluation of this novel investigational gene therapy"; that Defendants "have been moving efficiently through the development process, in no small part because of [Defendants'] ability to treat clinical trial participants with valoctocogene roxaparvovec produced using [their] commercial process"; and that "[u]tilization of to-be-commercialized material during Phase 3 studies significantly de-risks the program, as no production or facility changes need to be made to support commercial demand."

32. On December 23, 2019, BioMarin issued a press release announcing that it had submitted a BLA to the FDA for its investigational AAV gene therapy, valoctocogene roxaparvovec, for adults with hemophilia A (the "December 2019 Press Release"). That press release represented, in relevant part, that the BLA's "submission is based on a Phase 3 interim analysis of study participants treated with material from the to-be-commercialized process, and the three-year Phase 1/2 data"; that "[s]ubject to completion of the FDA's filing review, BioMarin anticipates the BLA review to commence in February 2020"; and that "BioMarin will provide an update in February 2020"; while touting that "[t]his submission marks the first marketing application submission for a gene therapy product for any type of hemophilia in the United States."

33. The December 2019 Press Release further touted that, also on December 23, 2019, "the EMA validated the Company's [MAA] to the EMA" for valoctocogene roxaparvovec, and that "BioMarin anticipates the start of the MAA review to commence in January 2020 under accelerated assessment," which further indicated to investors valoctocogene roxaparvovec's readiness for marketing approval.

34. On February 20, 2020, BioMarin issued a press release announcing that the FDA "has accepted for Priority Review the [BLA] to the FDA for its investigational AAV5 gene therapy, valoctocogene roxaparvovec, for adults with hemophilia A" (the "February 2020 Press Release"). That press release represented, in relevant part, that "[t]his acceptance by the FDA marks the first marketing application accepted for a gene therapy product for any type of hemophilia in the United States"; that "[t]he application is based on a Phase 3 interim analysis of study participants treated with investigational product manufactured by the to-be-commercialized process and three-year Phase 1/2 data"; and that "[t]he FDA has informed the company that they are not currently planning to hold an advisory committee meeting to discuss the application."

35. The February 2020 Press Release also quoted Defendant Fuchs, who touted, in relevant part, that "[v]aloctocogene roxaparvovec has the potential to be the first gene therapy approved in any type of hemophilia"; that "the acceptance of this application and its priority review status marks a significant milestone for gene therapies in general and for the hemophilia community specifically"; that, "[a]s pioneers in gene therapy, [Defendants] are proud of the medical and technological innovation represented in valoctocogene roxaparvovec, which is possible because of the scientists who did the early research, clinical investigators, the hemophilia community and the people who work" at BioMarin; and that Defendants "look forward to working with the FDA to bring this groundbreaking therapy to people with hemophilia A."

Materially False and Misleading Statements Issued During the Class Period

36. The Class Period begins on February 28, 2020. On February 27, 2020, during after-market hours, BioMarin filed an annual report on Form 10-K with the SEC, reporting the Company's financial and operating results for the quarter and year ended December 31, 2019 (the "2019 10-K"). The 2019 10-K touted regulatory review of valoctocogene roxaparvovec for the treatment of severe hemophilia A, stating, in relevant part, that "[o]n February 20, 2020,

[Defendants] announced that the [FDA] accepted for priority review [the BLA] for valoctocogene roxaparvovec for the treatment of adults with severe hemophilia A"; that "[t]he Prescription Drug User Fee Act (PDUFA) target action date for the BLA has been set for August 21, 2020"; that "[o]n December 23, 2019, [Defendants] announced that the [EMA] validated [the MAA] for valoctocogene roxaparvovec, which has been in review under accelerated assessment since January 2020"; that "[t]he submissions are based on [BioMarin's] Phase 3 interim analysis and the three-year Phase 1/2 data of patients treated with valoctocogene roxaparvovec"; and that "[b]oth submissions represent the first time a gene therapy product for any type of hemophilia indication is under review for marketing authorization by health authorities."

37. The 2019 10-K also reported that "[o]n May 28, 2019, [BioMarin] announced a three-year update to [its] previously reported results from the Phase 1/2 dose-escalation study for valoctocogene roxaparvovec in patients with severe hemophilia A"; that this update "demonstrated that bleed rate control with the 6e13 vg/kg dose was maintained for a third year with a median [ABR] of 0 and mean ABR of 0.7 in that year"; that "factor VIII levels in the 6e13 vg/kg dose appeared to be approaching a plateau in year three"; that "[f]actor VIII levels measured with the chromogenic substrate" ("CS") "assay at the end of year three were mean and median of 32.7 international units per deciliter (IU/dL) and 19.9 IU/dl, respectively, compared with mean and median of 36.4 IU/dL and 26.2 IU/dL, respectively, at the end of year two"; and that, "[i]n January 2020, the New England Journal of Medicine . . . published results from the three-year update from the Phase 1/2 study"; all of which indicated to investors the market viability of valoctocogene roxaparvovec, and an increased likelihood of the FDA's acceptance of the BLA for that drug, given the updated Phase 1/2 study results.

38. With respect to BioMarin's Phase 3 study program for valoctocogene roxaparvovec, the 2019 10-K represented, in relevant part:

> The global Phase 3 study program with valoctocogene roxaparvovec includes two studies, one with the 6e13 vg/kg dose (GENEr8-1) and one with the 4e13 vg/kg dose (GENEr8-2). In July 2019, we announced the discontinuation of the GENEr8-2 study given the overwhelming preference by patients to be treated with the 6e13 vg/kg dose. The GENEr8-1 study is an open-label single-arm study to evaluate the efficacy and safety of valoctocogene roxaparvovec as well as evaluate superiority of the product candidate compared to standard of care. The primary endpoint is based on the factor VIII activity level achieved following treatment with valoctocogene roxaparvovec, and the secondary endpoint measures annualized factor VIII replacement therapy use rate and ABR. We announced on May 28, 2019 that data from a 20-patient cohort of the Phase 3 GENEr8-1 study achieved pre-specified clinical criteria for regulatory review in the U.S. and Europe. As of May 28, 2019, eight patients in the cohort achieved factor VIII levels of 40 or more IU/dL using the CS assay at 23 to 26 weeks, meeting the pre-specified criteria for factor VIII activity levels. As of the April 30, 2019 data cutoff, between weeks 23 to 26, seven of 16 study participants in the cohort reached or exceeded the pre-specified factor VIII levels of 40 IU/dL using the CS assay. Subsequent to the April 30, 2019 cutoff, one additional participant met that criteria, bringing the total to eight participants. For the 16 patients who had reached week 26 since administration of valoctocogene roxaparvovec by the April 30, 2019 cutoff, the estimated median ABR was zero and the estimated mean ABR was 1.5, representing a reduction of 85% from baseline levels where all patients were on standard of care. In addition, there was a 98% reduction in median annualized factor VIII usage and a 94% reduction in mean factor VIII usage annualized between weeks 5 and 26. Between weeks 23 and 26, the mean factor VIII level using the CS assay was 36 IU/dL and the median was 33 IU/dl. We have dosed 134 study participants in the full GENEr8-1 study and the 52-week results are anticipated in the first quarter of 2021.

While these statements further supported the market viability of valoctocogene roxaparvovec, and an increased likelihood of the FDA's acceptance of the BLA for that drug, which was based in part on the Phase 3 study's data, they nonetheless failed to disclose what, if any, impact the differences between the Phase 3 study and Phase 1/2 study would have on the reliability of the Phase 1/2 study to support the BLA for valoctocogene roxaparvovec.

39. Additionally, the 2019 10-K contained generic, boilerplate representations concerning risks inherent in BioMarin's potential failure to successfully obtain regulatory approval for one or more of its product candidates. For example, with specific respect to valoctocogene roxaparvovec, the 2019 10-K advised, *inter alia*:

> With respect to valoctocogene roxaparvovec, we may experience challenges specific to gene therapy that cause significant delays or unanticipated costs, or that cannot be solved. Although numerous companies are currently advancing gene therapy product candidates through clinical trials, the FDA has only approved a very small number of vector-based gene therapy products thus far. Moreover, there are very few approved gene therapy products outside the U.S. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for valoctocogene roxaparvovec in any jurisdiction. Regulatory requirements governing gene and cell therapy products are still evolving and may continue to change in the future. Regulatory review agencies and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our treatment candidate or lead to significant post-approval studies, limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring valoctocogene roxaparvovec to market could have a negative effect on our business and financial condition. Even if we do obtain regulatory approval, ethical, social and legal concerns about gene therapy arising in the future could result in additional regulations restricting or prohibiting sale of our product.

Plainly, this risk warning was a generic, catch-all provision that was not tailored to BioMarin's actual known risks regarding the BLA application for valoctocogene roxaparvovec, much less what, if any, impact the differences between the Phase 3 study and Phase 1/2 study would have on the reliability of the Phase 1/2 study to support the BLA for valoctocogene roxaparvovec.

40. Appended as an exhibit to the 2019 10-K were signed certifications pursuant to the Sarbanes-Oxley Act of 2002, wherein Defendants Bienaimé and Mueller certified that "the [2019 10-K] fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934," and that "the information contained in the [2019 10-K] fairly presents, in all material respects, the financial condition and results of operations of the Company."

41. On May 31, 2020, BioMarin issued a press release announcing "an update to its previously reported results of an open-label Phase 1/2 study of valoctocogene roxaparvovec, an investigational gene therapy treatment for adults with severe hemophilia A," with "[t]he data hav[ing] been submitted as a late-breaking abstract to the World Federation of Hemophilia . . . Virtual Summit" (the "May 2020 Press Release"). That press release reported, in relevant part,

that "[t]he four-year update for the 6e13 vg/kg and three-year update for the 4e13 vg/kg cohorts demonstrated that all subjects in both cohorts remain off prophylactic Factor VIII treatment since receiving their single dose of valoctocogene roxaparvovec"; that "[c]umulative mean [ABR] remain less than one (1) in both cohorts and below pre-treatment baseline levels"; that "[t]he mean ABR in year four for the 6e13 vg/kg cohort was 1.3, and the mean ABR in year three for the 4e13 vg/kg cohort was 0.5"; that "[o]ver the past year, six of the seven participants in the 6e13 vg/kg cohort and five of the six participants in the 4e13 vg/kg cohort remain free of spontaneous bleeds"; and that "Factor VIII activity levels declined commensurate with the most recent years' observations and remain in a range to provide hemostatic efficacy"; all of which further indicated to investors the market viability of valoctocogene roxaparvovec, and an increased likelihood of the FDA's acceptance of the BLA for that drug, given the updated Phase 1/2 study results.

42. Additionally, the May 2020 Press Release quoted Defendant Fuchs, who touted, in relevant part, that "BioMarin is proud to have advanced the community's knowledge of the potential for gene therapy to transform lives"; that "[i]n just over four years since starting clinical trials in patients, [BioMarin has] submitted applications for marketing authorizations globally"; that BioMarin "continue[s] to contribute to the growing body of scientific data in gene therapy for hemophilia A with five studies underway"; and that Defendants "continue to move forward with health authorities to make this treatment available for people with severe hemophilia A."

43. On June 17, 2020, BioMarin issued a press release announcing "additional data from its previously reported four-year update of an open-label Phase 1/2 study of valoctocogene roxaparvovec, an investigational gene therapy treatment for severe hemophilia A," the results of which "were presented during a late-breaking oral presentation at the World Federation of Hemophilia . . . Virtual Summit" (the "June 2020 Press Release"). Specifically, that press release provided several slides that purportedly further supported valoctocogene roxaparvovec as a gene

therapy treatment for severe hemophilia A, as well as discussions of the beneficial data observed in the individual cohorts of the Phase 1/2 study.

44. With respect to ABR and Factor VIII use in the 6e13 vg/kg Cohort, the June 2020 Press Release stated, in relevant part, that "[i]n the six study participants who were previously on Factor VIII prophylaxis in the 6e13 vg/kg cohort, the data showed substantial and sustained reductions in bleeding that required Factor VIII infusions"; that "[i]n the year prior to treatment with valoctocogene roxaparvovec, the mean [ABR] was 16.3 and the median was 16.5"; that "[d]uring the four years following treatment with valoctocogene roxaparvovec, the cumulative mean ABR was 0.8, which represents a 95% reduction from baseline"; that, "[i]n the fourth year, the mean ABR was 1.3 and the median was zero"; that "[t]here was a 96% reduction in mean Factor VIII usage to 5.4 infusions per year cumulatively over four years from the baseline of 135.6 infusions per year"; that "[a]mong all seven study participants in the 6e13 vg/kg cohort, 86% or six out of seven were bleed-free in the fourth year"; and that "[a]ll participants remain off Factor VIII prophylaxis therapy."

45. With respect to ABR and Factor VIII use in the 4e13 vg/kg Cohort, the June 2020 Press Release stated, in relevant part, that "[s]imilarly, in the six study participants in the 4e13 vg/kg cohort, the data showed substantial and sustained reductions in bleeding requiring Factor VIII infusions following treatment with valoctocogene roxaparvovec"; that "[a]ll participants remain off Factor VIII prophylaxis therapy"; that "[i]n the year prior to treatment with valoctocogene roxaparvovec, the mean ABR was 12.2 and the median was 8.0"; that "[t]he cumulative mean ABR was reduced by 93% to 0.9 with continued absence of target joint bleeds in 5 of 6 subjects during the three years observed, which represents a 93% reduction from baseline"; that "[d]uring the third year of follow-up, the mean ABR was 0.5 and the median was zero (0), and 67% or four out of six study participants were bleed-free"; that "[f]ive out of six

participants had no spontaneous bleeds"; and that "[t]here was a 96% reduction in mean Factor VIII usage to 5.7 infusions per year cumulatively over three years from the baseline of 142.8 infusions per year."

46. With respect to Factor VIII activity levels for 6e13 vg/kg and 4e13 vg/kg cohorts, the June 2020 Press Release stated, in relevant part, that "[f]or the 6e13 vg/kg and 4e13 vg/kg cohorts, mean Factor VIII activity levels over four and three years, respectively, support the observed reductions in bleed rates and annualized Factor VIII usage"; that "[a]ll study participants had severe hemophilia A at baseline, defined as less than or equal to 1 IU/dL of Factor VIII activity"; that "[a]t the end of the fourth-year post-infusion with valoctocogene roxaparvovec, all patients continue to produce their own endogenous factor with the mean Factor VIII activity level of the 6e13 vg/kg cohort at 24.2 IU/dL as measured by the [CS] assay and at 35.4 IU/dL as measured by the One-Stage" ("OS") "assay"; that "[t]he median Factor VIII activity levels at the end of the fourth year was 16.4 IU/dL as measured by the CS assay and 23.4 IU/dL as measured by the OS assay"; that "[t]hese measurements are based on six of the seven participants, as an evaluable sample for the seventh study participant was not available"; that "[m]ean Factor VIII activity levels over three years similarly support the observed reductions in bleed rates and annualized Factor VIII usage for the 4e13 vg/kg cohort"; that "[a]t the end of the third year post-infusion with valoctocogene roxaparvovec, mean Factor VIII activity level of the 4e13 vg/kg cohort was 9.9 IU/dL as measured by the CS assay and 14.9 IU/dL as measured by the OS assay"; and that "[t]he median Factor VIII activity levels at the end of the third year was 7.9 IU/dL as measured by the CS assay and 12.3 IU/dL as measured by the OS assay."

47. Additionally, the June 2020 Press Release touted BioMarin's "[r]obust [c]linical [p]rogram" for valoctocogene roxaparvovec, touting, in relevant part, that "[t]he global Phase 3 study of valoctocogene roxaparvovec at the 6e13 vg/kg dose (GENEr8-1) evaluates superiority of

valoctocogene roxaparvovec to the current standard of care, FVIII prophylactic therapy"; that "[t]he sample size of the GENEr8-1 study is approximately 130 total participants"; that "BioMarin has five clinical studies underway in its comprehensive gene therapy program for the treatment of severe hemophilia A"; that, "[i]n addition to the global Phase 3 study GENEr8-1, the Company is running a Phase 1/2 Study with the 6E13kg/vg dose of valoctocogene roxaparvovec in approximately 10 participants with pre-existing AAV5 antibodies"; that "[t]he Company is also running two additional and separate studies, one to study AAV seroprevalence in people with severe hemophilia A and one non-interventional study to determine baseline characteristics in people with hemophilia A"; and that "[p]articipants in the Phase 1/2 dose escalation study will continue to be monitored as part of the global program underway." All of these statements, in addition to the other statements from the June 2020 Press Release referenced herein, further indicated to investors the market viability of valoctocogene roxaparvovec, and an increased likelihood of the FDA's acceptance of the BLA for that drug.

48. Indeed, the June 2020 Press Release quoted Professor John Pasi, M.B., Ch.B., Ph.D., from Barts and the London School of Medicine and Dentistry and Chief Investigator for the Phase 1/2 study, who touted, in relevant part, that "[w]ith four years of data, this study represents the longest duration of clinical experience for any gene therapy in hemophilia A"; that "[i]t is exciting to observe that all study participants remain off Factor VIII prophylaxis therapy, while also experiencing a greater than 90 percent reduction in bleeding episodes from a single administration of valoctocogene roxaparvovec"; that "[t]hese data demonstrate the very real potential of a paradigm shift in the treatment of hemophilia A"; and that "ongoing research into gene therapies could represent an entirely new way to approach meeting the high unmet need in patients with severe hemophilia A."

49. Additionally, the June 2020 Press Release quoted Defendant Fuchs, who touted, in relevant part, that "BioMarin is committed to the bleeding disorders community with the most robust and advanced clinical development program for a potential first gene therapy in severe hemophilia A"; and that "[d]emonstrating a 96% reduction in exogenous Factor VIII usage as patients are now producing their own endogenous factor VIII is a potential benefit that [Defendants] hope to be able to offer as [they] work closely with regulators to seek approval and work to reduce the burden of hemophilia."

50. The statements referenced in ¶¶ 36-49 were materially false and misleading because Defendants made false and/or misleading statements, as well as failed to disclose material adverse facts about the Company's business, operational and compliance policies. Specifically, Defendants made false and/or misleading statements and/or failed to disclose that: (i) differences between the Phase 1/2 and Phase 3 study of valoctocogene roxaparvovec limited the reliability of the Phase 1/2 study to support valoctocogene roxaparvovec's durability of effect; (ii) as a result, it was foreseeable that the FDA would not approve the BLA for valoctocogene roxaparvovec without additional data; and (iii) as a result, the Company's public statements were materially false and misleading at all relevant times.

The Truth Emerges

51. On August 19, 2020, during pre-market hours, BioMarin announced receipt of a CRL from the FDA to the Company's BLA for valoctocogene roxaparvovec (the "August 2020 Press Release"). Specifically, BioMarin advised, in relevant part, that "[h]aving previously agreed with the [FDA] on the extent of data necessary to support the BLA, the FDA introduced a new recommendation for two years of data from the Company's ongoing [Phase 3 study] to provide substantial evidence of a durable effect using [ABR] as the primary endpoint"; that the FDA "first informed the Company of this recommendation in the CRL having not raised this at any time

during development or review"; that 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"; that the "FDA concluded that the differences between [the Phase 1/2 study] and the Phase 3 study limited its ability to rely on the Phase 1/2 study to support durability of effect"; that "[t]he Phase 3 study was fully enrolled in November 2019, and the last patient will complete two years of follow up in November 2021"; that "[t]he Company plans to meet with the [FDA] in the coming weeks to align on the next steps to obtain approval"; and that "[t]he ongoing valoctocogene roxaparvovec clinical trials will continue while BioMarin is exploring next steps to obtain approval."

52. The August 2020 Press Release also quoted Defendant Bienaimé, who represented, in relevant part, that Defendants "are surprised and disappointed that the FDA introduced new expectations for the first time in the Complete Response Letter."

53. Following BioMarin's issuance of the August 2020 Press Release, the Company's stock price fell $41.82 per share, or 35.28%, to close at $76.72 per share on August 19, 2020.

54. Although Defendants largely shifted the blame for the valoctocogene roxaparvovec BLA's rejection onto the FDA for moving the goal post forward in terms of what was required for the BLA's acceptance, analysts quickly noted that Defendants' should have been aware of issues with the BLA ahead of time. For example, an article published by *Fierce Biotech* on August 19, 2020, observed that "[t]hree-year data on [BioMarin's] candidate were reported last May but sparked concerns about the durability of the therapy after factor VIII levels seemed to fall off after 12 to 18 months, raising the possibility that patients might need to be re-dosed to maintain protection against bleeds," and that "it is durability the FDA has concerns over, though BioMarin contends this is the first it's heard about it." Similarly, an article published by *Evaluate Vantage* on August 20, 2020, remarked that "[t]he problem of [valoctocogene roxaparvovec]'s waning efficacy has been rumbling for some time," and that, "[a]s such BioMarin's claim that the US FDA

had moved the goalposts in demanding long-term data before approving [valoctocogene roxaparvovec] . . . a central point of yesterday's complete response letter, rings hollow." Additionally, RBC Capital analyst Kennen Mackay downgraded BioMarin to sector perform from outperform, cutting his share-price target to $92 from $123, noting that the FDA's action is "thesis-changing" and reduced management credibility.

55. As a result of Defendants' wrongful acts and omissions, and the precipitous decline in the market value of the Company's securities, Plaintiff and other Class members have suffered significant losses and damages.

Post-Class Period Developments

56. On September 9, 2020, BioMarin filed a current report on Form 8-K with the SEC, disclosing that the EMA had delayed the marketing application for valoctocogene roxaparvovec. Specifically, the Form 8-K advised, in relevant part, that "the Company received the [EMA] Joint Assessment Report (the 'Report') related to the EMA's ongoing review of the Company's [MAA] for valoctocogene roxaparvovec for severe hemophilia A"; that "[t]he Report requests that the Company submit to the EMA the full 52-week results from the 134 patients in the ongoing Phase 3 study of valoctocogene roxaparvovec with the 6e13 vg/kg dose"; that "[t]he Company expects the last patient will reach 52 weeks of follow-up in November 2020, and BioMarin is working with the EMA to enable a potential submission of the requested data by the end of the first quarter of 2021"; that, "[a]s a result of the EMA's request, the review of the Company's MAA has reverted from an accelerated assessment to a standard review"; and that "BioMarin plans to provide additional information about its expectations regarding the timing of the MAA review after the Company has further interactions with the EMA."

PLAINTIFF'S CLASS ACTION ALLEGATIONS

57. Plaintiff brings this action as a class action pursuant to Federal Rule of Civil Procedure 23(a) and (b)(3) on behalf of a Class, consisting of all those who purchased or otherwise acquired BioMarin securities during the Class Period (the "Class"); and were damaged upon the revelation of the alleged corrective disclosures. Excluded from the Class are Defendants herein, the officers and directors of the Company, at all relevant times, members of their immediate families and their legal representatives, heirs, successors or assigns and any entity in which Defendants have or had a controlling interest.

58. The members of the Class are so numerous that joinder of all members is impracticable. Throughout the Class Period, BioMarin securities were actively traded on the NASDAQ. While the exact number of Class members is unknown to Plaintiff at this time and can be ascertained only through appropriate discovery, Plaintiff believes that there are hundreds or thousands of members in the proposed Class. Record owners and other members of the Class may be identified from records maintained by BioMarin or its transfer agent and may be notified of the pendency of this action by mail, using the form of notice similar to that customarily used in securities class actions.

59. Plaintiff's claims are typical of the claims of the members of the Class as all members of the Class are similarly affected by Defendants' wrongful conduct in violation of federal law that is complained of herein.

60. Plaintiff will fairly and adequately protect the interests of the members of the Class and has retained counsel competent and experienced in class and securities litigation. Plaintiff has no interests antagonistic to or in conflict with those of the Class.

61. Common questions of law and fact exist as to all members of the Class and predominate over any questions solely affecting individual members of the Class. Among the questions of law and fact common to the Class are:

- whether the federal securities laws were violated by Defendants' acts as alleged herein;
- whether statements made by Defendants to the investing public during the Class Period misrepresented material facts about the business, operations and management of BioMarin;
- whether the Individual Defendants caused BioMarin to issue false and misleading financial statements during the Class Period;
- whether Defendants acted knowingly or recklessly in issuing false and misleading financial statements;
- whether the prices of BioMarin securities during the Class Period were artificially inflated because of the Defendants' conduct complained of herein; and
- whether the members of the Class have sustained damages and, if so, what is the proper measure of damages.

62. A class action is superior to all other available methods for the fair and efficient adjudication of this controversy since joinder of all members is impracticable. Furthermore, as the damages suffered by individual Class members may be relatively small, the expense and burden of individual litigation make it impossible for members of the Class to individually redress the wrongs done to them. There will be no difficulty in the management of this action as a class action.

63. Plaintiff will rely, in part, upon the presumption of reliance established by the fraud-on-the-market doctrine in that:

- Defendants made public misrepresentations or failed to disclose material facts during the Class Period;
- the omissions and misrepresentations were material;
- BioMarin securities are traded in an efficient market;

- the Company's shares were liquid and traded with moderate to heavy volume during the Class Period;
- the Company traded on the NASDAQ and was covered by multiple analysts;
- the misrepresentations and omissions alleged would tend to induce a reasonable investor to misjudge the value of the Company's securities; and
- Plaintiff and members of the Class purchased, acquired and/or sold BioMarin securities between the time the Defendants failed to disclose or misrepresented material facts and the time the true facts were disclosed, without knowledge of the omitted or misrepresented facts.

64. Based upon the foregoing, Plaintiff and the members of the Class are entitled to a presumption of reliance upon the integrity of the market.

65. Alternatively, Plaintiff and the members of the Class are entitled to the presumption of reliance established by the Supreme Court in *Affiliated Ute Citizens of the State of Utah v. United States*, 406 U.S. 128, 92 S. Ct. 2430 (1972), as Defendants omitted material information in their Class Period statements in violation of a duty to disclose such information, as detailed above.

COUNT I

(Violations of Section 10(b) of the Exchange Act and Rule 10b-5 Promulgated Thereunder Against All Defendants)

66. Plaintiff repeats and re-alleges each and every allegation contained above as if fully set forth herein.

67. This Count is asserted against Defendants and is based upon Section 10(b) of the Exchange Act, 15 U.S.C. § 78j(b), and Rule 10b-5 promulgated thereunder by the SEC.

68. During the Class Period, Defendants engaged in a plan, scheme, conspiracy and course of conduct, pursuant to which they knowingly or recklessly engaged in acts, transactions, practices and courses of business which operated as a fraud and deceit upon Plaintiff and the other members of the Class; made various untrue statements of material facts and omitted to state material facts necessary in order to make the statements made, in light of the circumstances under

which they were made, not misleading; and employed devices, schemes and artifices to defraud in connection with the purchase and sale of securities. Such scheme was intended to, and, throughout the Class Period, did: (i) deceive the investing public, including Plaintiff and other Class members, as alleged herein; (ii) artificially inflate and maintain the market price of BioMarin securities; and (iii) cause Plaintiff and other members of the Class to purchase or otherwise acquire BioMarin securities and options at artificially inflated prices. In furtherance of this unlawful scheme, plan and course of conduct, Defendants, and each of them, took the actions set forth herein.

69. Pursuant to the above plan, scheme, conspiracy and course of conduct, each of the Defendants participated directly or indirectly in the preparation and/or issuance of the quarterly and annual reports, SEC filings, press releases and other statements and documents described above, including statements made to securities analysts and the media that were designed to influence the market for BioMarin securities. Such reports, filings, releases and statements were materially false and misleading in that they failed to disclose material adverse information and misrepresented the truth about BioMarin's finances and business prospects.

70. By virtue of their positions at BioMarin, Defendants had actual knowledge of the materially false and misleading statements and material omissions alleged herein and intended thereby to deceive Plaintiff and the other members of the Class, or, in the alternative, Defendants acted with reckless disregard for the truth in that they failed or refused to ascertain and disclose such facts as would reveal the materially false and misleading nature of the statements made, although such facts were readily available to Defendants. Said acts and omissions of Defendants were committed willfully or with reckless disregard for the truth. In addition, each Defendant knew or recklessly disregarded that material facts were being misrepresented or omitted as described above.

71. Information showing that Defendants acted knowingly or with reckless disregard for the truth is peculiarly within Defendants' knowledge and control. As the senior managers and/or directors of BioMarin, the Individual Defendants had knowledge of the details of BioMarin's internal affairs.

72. The Individual Defendants are liable both directly and indirectly for the wrongs complained of herein. Because of their positions of control and authority, the Individual Defendants were able to and did, directly or indirectly, control the content of the statements of BioMarin. As officers and/or directors of a publicly-held company, the Individual Defendants had a duty to disseminate timely, accurate, and truthful information with respect to BioMarin's businesses, operations, future financial condition and future prospects. As a result of the dissemination of the aforementioned false and misleading reports, releases and public statements, the market price of BioMarin securities was artificially inflated throughout the Class Period. In ignorance of the adverse facts concerning BioMarin's business and financial condition which were concealed by Defendants, Plaintiff and the other members of the Class purchased or otherwise acquired BioMarin securities at artificially inflated prices and relied upon the price of the securities, the integrity of the market for the securities and/or upon statements disseminated by Defendants, and were damaged thereby.

73. During the Class Period, BioMarin securities were traded on an active and efficient market. Plaintiff and the other members of the Class, relying on the materially false and misleading statements described herein, which the Defendants made, issued or caused to be disseminated, or relying upon the integrity of the market, purchased or otherwise acquired shares of BioMarin securities at prices artificially inflated by Defendants' wrongful conduct. Had Plaintiff and the other members of the Class known the truth, they would not have purchased or otherwise acquired said securities, or would not have purchased or otherwise acquired them at the inflated prices that

were paid. At the time of the purchases and/or acquisitions by Plaintiff and the Class, the true value of BioMarin securities was substantially lower than the prices paid by Plaintiff and the other members of the Class. The market price of BioMarin securities declined sharply upon public disclosure of the facts alleged herein to the injury of Plaintiff and Class members.

74. By reason of the conduct alleged herein, Defendants knowingly or recklessly, directly or indirectly, have violated Section 10(b) of the Exchange Act and Rule 10b-5 promulgated thereunder.

75. As a direct and proximate result of Defendants' wrongful conduct, Plaintiff and the other members of the Class suffered damages in connection with their respective purchases, acquisitions and sales of the Company's securities during the Class Period, upon the disclosure that the Company had been disseminating misrepresented financial statements to the investing public.

COUNT II

(Violations of Section 20(a of the Exchange Act Against the Individual Defendants

76. Plaintiff repeats and re-alleges each and every allegation contained in the foregoing paragraphs as if fully set forth herein.

77. During the Class Period, the Individual Defendants participated in the operation and management of BioMarin, and conducted and participated, directly and indirectly, in the conduct of BioMarin's business affairs. Because of their senior positions, they knew the adverse non-public information about BioMarin's misstatement of income and expenses and false financial statements.

78. As officers and/or directors of a publicly owned company, the Individual Defendants had a duty to disseminate accurate and truthful information with respect to BioMarin's

financial condition and results of operations, and to correct promptly any public statements issued by BioMarin which had become materially false or misleading.

79. Because of their positions of control and authority as senior officers, the Individual Defendants were able to, and did, control the contents of the various reports, press releases and public filings which BioMarin disseminated in the marketplace during the Class Period concerning BioMarin's results of operations. Throughout the Class Period, the Individual Defendants exercised their power and authority to cause BioMarin to engage in the wrongful acts complained of herein. The Individual Defendants therefore, were "controlling persons" of BioMarin within the meaning of Section 20(a) of the Exchange Act. In this capacity, they participated in the unlawful conduct alleged which artificially inflated the market price of BioMarin securities.

80. Each of the Individual Defendants, therefore, acted as a controlling person of BioMarin. By reason of their senior management positions and/or being directors of BioMarin, each of the Individual Defendants had the power to direct the actions of, and exercised the same to cause, BioMarin to engage in the unlawful acts and conduct complained of herein. Each of the Individual Defendants exercised control over the general operations of BioMarin and possessed the power to control the specific activities which comprise the primary violations about which Plaintiff and the other members of the Class complain.

81. By reason of the above conduct, the Individual Defendants are liable pursuant to Section 20(a) of the Exchange Act for the violations committed by BioMarin.

PRAYER FOR RELIEF

WHEREFORE, Plaintiff demands judgment against Defendants as follows:

A. Determining that the instant action may be maintained as a class action under Rule 23 of the Federal Rules of Civil Procedure, and certifying Plaintiff as the Class representative;

B. Requiring Defendants to pay damages sustained by Plaintiff and the Class by reason of the acts and transactions alleged herein;

C. Awarding Plaintiff and the other members of the Class prejudgment and post-judgment interest, as well as their reasonable attorneys' fees, expert fees and other costs; and

D. Awarding such other and further relief as this Court may deem just and proper.

DEMAND FOR TRIAL BY JURY

Plaintiff hereby demands a trial by jury.