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, DANIEL SPIEGELMAN, 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 others 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 January 13, 2020 and September 3, 2021, 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 develops and commercializes therapies for people with serious and life-threatening rare diseases and medical conditions. The Company is developing, among other product candidates, BMN 307, an AAV5 mediated gene therapy, which is in a phase 1/2 clinical trial to normalize blood phenylalanine ("Phe") concentration levels in patients with phenylketonuria ("PKU"). The Company's Phearless Phase 1/2 study is evaluating BMN 307 in adults with PKU.

3. On November 7, 2018, BioMarin shared pre-clinical data of BMN 307, which demonstrated lifetime Phe corrections in mouse models, and announced that the Company was planning to file an investigational new drug application ("IND") for BMN 307 with the United States Food and Drug Administration ("FDA") in the second half of 2019. On January 13, 2020, the Company announced that the FDA granted IND status for BMN 307 for the treatment of PKU. On September 24, 2020, the Company announced that it had dosed the first human participant in the global Phearless Phase 1/2 study of BMN 307.

4. Throughout the Class Period, Defendants made materially false and misleading statements regarding the Company's business, operations, and compliance policies. Specifically, Defendants made false and/or misleading statements and/or failed to disclose that: (i) BMN 307 was less safe than BioMarin had led investors to believe; (ii) BMN 307's safety profile made it likely that the FDA would place a clinical hold on the Phearless Phase 1/2 study; (iii) accordingly, the Company had overstated BMN 307's clinical and commercial prospects; and (iv) as a result, the Company's public statements were materially false and misleading at all relevant times.

5. On September 5, 2021, BioMarin issued a press release announcing "that the [FDA] placed a clinical hold on the BMN 307 Phearless Phase 1/2 study", which "is evaluating BMN 307, an investigational AAV5-phenylalanine hydroxylase (PAH) gene therapy, in adults with [PKU]." BioMarin advised investors that "[t]he FDA's clinical hold was based on interim safety findings from a pre-clinical, non-GLP pharmacology study."

6. On this news, BioMarin's stock price fell $7.14 per share, or 8.4%, to close at $77.81 per share on September 7, 2021, the next trading day.

7. 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

8. 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).

9. 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.

10. 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.

11. 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

12. 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.

13. 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."

14. Defendant Jean-Jacques Bienaimé ("Bienaimé") has served as BioMarin's Chief Executive Officer at all relevant times. Bienaimé is also the Company's Chairman.

15. Defendant Brian R. Mueller ("Mueller") has served as BioMarin's Executive Vice President ("EVP") and Chief Financial Officer ("CFO") since June 29, 2020, prior to which he served as the Company's acting CFO since February 3, 2020, and Senior Vice President, Finance and Chief Accounting Officer.

16. Defendant Daniel Spiegelman ("Spiegelman") served as BioMarin's EVP and CFO from before the start of the Class Period until February 3, 2020, and remained as an employee and senior advisor of the Company until September 1, 2020.

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

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

19. 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.

SUBSTANTIVE ALLEGATIONS

Background

20. BioMarin develops and commercializes therapies for people with serious and life-threatening rare diseases and medical conditions. The Company is developing, among other product candidates, BMN 307, an AAV5 mediated gene therapy, which is in a phase 1/2 clinical trial to normalize blood Phe concentration levels in patients with PKU. The Company's Phearless Phase 1/2 study is evaluating BMN 307 in adults with PKU.

21. On November 7, 2018, BioMarin shared pre-clinical data of BMN 307, which demonstrated lifetime Phe corrections in mouse models, and announced that the Company was planning to file an IND for BMN 307 with the FDA in the second half of 2019. On January 13, 2020, the Company announced that the FDA granted IND status for BMN 307 for the treatment of PKU. On September 24, 2020, the Company announced that it had dosed the first human participant in the global Phearless Phase 1/2 study of BMN 307.

Materially False and Misleading Statements Issued During the Class Period

22. The Class Period begins on January 13, 2020, when BioMarin issued a press release announcing it would begin the Phearless Phase 1/2 study. That press release touted BMN 307's clinical prospects, stating, in relevant part:

> [B]oth the [FDA] and the Medicines and Healthcare Products Regulatory Agency (MHRA) in the U.K. have granted the Company Investigational New Drug (IND) status and approved its Clinical Trial Application (CTA), respectively, for its investigational gene therapy candidate BMN 307. BMN 307 is an AAV5-phenylalanine hydroxylase (PAH) gene therapy designed to normalize blood [Phe] concentration levels in patients with PKU. BMN 307 will be evaluated to determine whether a single dose of treatment can restore natural Phe metabolism, normalize plasma Phe levels, and enable a normal diet in patients with PKU.
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> The Company expects to start dosing patients in PHEARLESS, a Phase 1/2 study, in the first quarter of 2020 with product made at commercial scale from its award-winning gene therapy manufacturing facility. The Company is actively preparing regulatory submissions to open additional clinical sites in other countries. BMN 307 represents a potential third PKU treatment option from BioMarin and its second gene therapy clinical program. Both the FDA and European Medicines Agency have granted BMN 307 Orphan Status.

23. On February 26, 2020, BioMarin issued a press release announcing the Company's Q4 and full year 2019 financial results. The press release stated, in relevant part:

> Commenting on 2019 results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "Our performance in 2019 reflects the clinical, regulatory and financial goals we set for ourselves a year ago.
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> Mr. Bienaimé continued, "In addition to these later-stage regulatory and clinical milestones in 2019, we made significant progress advancing our early-stage pipeline. Building on the success of our phenylketonuria (PKU) franchise with Palynziq and Kuvan, we announced in January that both the United States and the United Kingdom health authorities had given the go-ahead to start dosing patients with PKU with our BMN 307 gene therapy in a Phase 1/2 study. We plan to treat patients with BMN 307 in the first quarter using product made at commercial scale from our award-winning gene therapy manufacturing facility.

24. That same day, BioMarin hosted an earnings call with investors and analysts to discuss the Company's Q4 and full year 2019 results (the "Q4 2019 Earnings Call"). During the scripted portion of the Q4 2019 Earnings Call, Defendant Fuchs stated, in relevant part:

> Turning now to BMN 307, our investigational gene therapy for phenylketonuria. We expect to start enrolling patients in the peerless Phase II study, which is a dose-escalation, dose-selection study later this quarter, with an expansion arm expected in the second half of the year. This study could potentially be registration enabling past that expansion as we're conducting it with material manufactured using a commercial-ready process to de-risk this program and facilitate rapid clinical development and registration. We are excited about the prospect of BMN 307 as it represents a potential third treatment for phenylketonuria in our franchise; and a second gene therapy development program, leveraging our learnings and capabilities from valrox.

25. On February 27, 2020, 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 stated, in relevant part:

> BMN 307 is a gene therapy product candidate that is designed to normalize blood [Phe] concentration levels in patients with PKU. On January 13, 2020, we announced that both the FDA and Medicines and Healthcare Products Regulatory Agency (MHRA) in the United Kingdom (U.K.) granted Investigational New Drug (IND) status and approved our Clinical Trial Application (CTA), respectively, for BMN 307. We expect to start dosing patients in PHEARLESS, a Phase 1/2 study of BMN 307, in the first quarter of 2020 using product made at commercial scale from our gene therapy manufacturing facility.

26. Appended to the 2019 10-K as an exhibit was a signed certification pursuant to the Sarbanes-Oxley Act of 2002 ("SOX") by Defendants Bienaimé and Mueller, attesting 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."

27. On April 29, 2020, BioMarin issued a press release entitled, "BioMarin Announces First Quarter 2020 Total Revenue Growth of 25% to $502 million." The press release listed as one of the "Key Program Highlights":

> - **BMN 307 gene therapy product candidate for phenylketonuria (PKU)**: On January 13, 2020 the Company announced that both the FDA and the Medicines and Healthcare Products Regulatory Agency (MHRA) in the U.K. have granted the Company Investigational New Drug (IND) status and approved its Clinical Trial Application (CTA), respectively, for BMN 307.
>
> The impact of COVID-19 has created uncertainty about when it will be safe for patients to be dosed in PHEARLESS, our Phase 1/2 study of BMN 307. The Company currently estimates that dosing will begin in the second half of 2020. In the meantime, new sites are currently being prepared to open and enroll patients. All subjects participating in the PHEARLESS study will receive product made at commercial scale from BioMarin's award-winning gene therapy manufacturing facility. Both the FDA and EMA have granted BMN 307 Orphan Drug Status.
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> Preclinical data with BMN 307 demonstrated a lifetime Phe correction sustained at 80 weeks in mouse models. BMN 307 is an AAV vector containing the DNA sequence that codes for the phenylalanine hydroxylase enzyme that is deficient in people with PKU.

28. That same day, BioMarin hosted an earnings call with investors and analysts to discuss the Company's Q1 2020 results (the "Q1 2020 Earnings Call"). During the scripted portion of the Q1 2020 Earnings Call, Defendant Fuchs stated, in relevant part:

> Moving to BMN 307, our investigational gene therapy for phenylketonuria, we're continuing to prepare new sites to open in order to enroll patients when it is safe to do so, given the COVID-19 circumstances. We're excited about the prospect of BMN 307, as it represents a third treatment for phenylketonuria in our PKU franchise and the second gene therapy development program, leveraging our learnings and capabilities from ROCTAVIAN. Currently, we expect the study to start later – we expect to start the study later in 2020.

29. On August 4, 2020, BioMarin issued a press release entitled, "BioMarin Announces Second Quarter 2020 Total Revenue Growth of 11% to $430 million." The press release listed as one of the "Key Program Highlights":

> - **BMN 307 gene therapy product candidate for phenylketonuria (PKU)**: On January 13, 2020 the Company announced that both the FDA and the

> Medicines and Healthcare Products Regulatory Agency (MHRA) in the U.K. have granted the Company Investigational New Drug (IND) status and approved its Clinical Trial Application (CTA), respectively, for BMN 307.
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> > Depending on the ongoing impact of COVID-19, the Company currently believes that dosing in Phearless, the Phase 1/2 study of BMN 307, could begin later in the third quarter. In the meantime, sites are being prepared to open and enroll patients. All subjects participating in the Phearless study will receive product made at commercial scale from BioMarin's award-winning gene therapy manufacturing facility. Both the FDA and EMA have granted BMN 307 Orphan Drug Status.

30. That same day, BioMarin hosted an earnings call with investors and analysts to discuss the Company's Q2 2020 results (the "Q2 2020 Earnings Call"). During the scripted portion of the Q2 2020 Earnings Call, Defendant Fuchs stated, in relevant part:

> Moving on to BMN 307, our investigational gene therapy for PKU, we are continuing to prepare new sites and depending on the ongoing impact with COVID-19, we believe we could begin data from the Phase 1, 2 study nicknamed Phearless later in the third quarter. We're 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 ROCTAVIAN.

31. On September 24, 2020, BioMarin issued a press release entitled, "BioMarin, Pioneer in Phenylketonuria (PKU) and Gene Therapy, Doses First Participant in Global PHEARLESS Phase 1/2 Study of BMN 307 Gene Therapy." The press release stated, in relevant part:

> BioMarin [. . .] announced today that it has dosed the first participant in the global PHEARLESS Phase 1/2 study with BMN 307, an investigational gene therapy for the treatment of individuals with PKU. BMN 307 is an AAV5-phenylalanine hydroxylase (PAH) gene therapy designed to normalize blood phenylalanine (Phe) concentration levels in patients with PKU by inserting a correct copy of the PAH gene into liver cells. BMN 307 will be evaluated to determine safety and whether a single dose of treatment can restore natural Phe metabolism, normalize plasma Phe levels, and enable a normal diet in patients with PKU.
>
> BioMarin will conduct this study with material manufactured with a commercial-ready process to facilitate rapid clinical development and potentially support approval. BMN 307 represents a potential third PKU treatment option in BioMarin's PKU franchise and a second gene therapy development program.

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> "BioMarin has been committed to the PKU community for more than 15 years and remains dedicated to the research and development of innovative therapies to advance the standard of care for people with PKU," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "Building upon our experience of delivering two approved PKU therapies to the PKU community, BMN 307 gene therapy combines BioMarin's leadership in the development of PKU therapies with our expertise in gene therapy development and manufacturing."
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> "PKU is a serious condition and many individuals struggle to manage their disorder on a daily basis. BioMarin is a pioneer in PKU treatments delivering the first two drug therapies to individuals with PKU. We applaud their unwavering commitment to drive research to bring a third treatment to the PKU community and for their substantial contributions to the overall body of scientific knowledge in PKU that they continue to make," said Christine S. Brown, MS, Executive Director, National PKU Alliance. "We are encouraged by BioMarin's efforts to develop a gene therapy that brings together their experience in PKU drug development, gene therapy development and gene therapy manufacturing. "

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> Both the FDA and European Medicines Agency have granted BMN 307 Orphan Drug Designation. The Company is actively preparing regulatory submissions to open additional clinical sites in other countries.

32. On October 2, 2020, BioMarin issued a press release entitled, "BioMarin, Pioneer in Phenylketonuria (PKU) and Gene Therapy, Receives FDA Fast Track Designation for PKU Investigational Gene Therapy, BMN 307." The press release stated, in relevant part:

> BioMarin [. . .], a pioneer in developing treatments for phenylketonuria (PKU) and gene therapies, announced today that the [FDA] has granted Fast Track designation to BMN 307, an investigational gene therapy for the treatment of individuals with PKU.
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> Fast Track designation is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fulfill an unmet medical need, enabling drugs to reach patients earlier. Clinical programs with Fast Track designation may benefit from early and frequent communication with the FDA throughout the regulatory review process. These clinical programs may also be eligible to apply for Accelerated Approval and Priority Review if relevant criteria are met, as well as Rolling Review, which means that completed sections of the Biologic License Application can be submitted for review before the entire FDA

application is complete. Both the FDA and European Medicines Agency have granted BMN 307 Orphan Drug Designation.

"Fast Track designation combined with our ability to conduct our clinical studies incorporating material manufactured using a commercial-ready process will further facilitate rapid clinical development of BMN 307 gene therapy," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "We are looking forward to working closely with the FDA, as well as other health agencies, to evaluate the safety and efficacy of this promising investigational gene therapy as we continue our unwavering 15-year commitment to advance the standard of care for people with PKU."

33. On November 5, 2020, BioMarin issued a press release entitled, "BioMarin Announces Third Quarter 2020 Total Revenues of $477 Million." The press release listed as one of the Company's "Key Program Highlights":

- **BMN 307 gene therapy product candidate for phenylketonuria (PKU)**: On September 24, 2020, the Company announced that it began dosing participants in PHEARLESS, the Phase 1/2 study of BMN 307. Both the FDA and EMA granted BMN 307 Orphan Drug Status. Additionally, the FDA has granted Fast Track status to BMN 307. Product for use in the Phase 1/2 study was made at commercial scale from BioMarin's award-winning gene therapy manufacturing facility.

34. That same day, BioMarin hosted an earnings call with investors and analysts to discuss the Company's Q3 2020 results (the "Q3 2020 Earnings Call"). During the scripted portion of the Q3 2020 Earnings Call, Defendant Fuchs stated, in relevant part, "[b]riefly on BMN 307, our investigational gene therapy for phenylketonuria, we're pleased to announce that testing hasn't been begun in the third quarter, and we have now treated two adults having phenylketonuria in the study. With a 307 study now underway, we continue to advance the next products in our earlier stage pipeline."

35. On February 25, 2021, BioMarin issued a press release announcing the Company's Q4 and full year 2020 financial results and corporate updates. The press release stated listed as one of the Company's "Key Program Highlights":

- **BMN 307 gene therapy product candidate for PKU**: The Company announced that it plans to dose escalate in PHEarless, the Phase 1/2 study

> of BMN 307 based on encouraging Phe lowering and safety signals observed in study participants who were treated with the lowest dose. Both the FDA and EMA granted BMN 307 Orphan Drug Status. Additionally, the FDA has granted Fast Track status to BMN 307. Product for use in the Phase 1/2 study was made at commercial scale from BioMarin's award-winning gene therapy manufacturing facility.

36. That same day, BioMarin hosted an earnings call with investors and analysts to discuss the Company's Q4 and full year 2020 results (the "Q4 2020 Earnings Call"). During the scripted portion of the Q4 2020 Earnings Call, Defendant Bienaimé stated, in relevant part:

> Moving to our earlier stage pipeline. We have numerous programs advancing this year. Starting with BMN 307 gene therapy for PKU, we are pleased to share that we are moving to the next higher dose in our Phase I/II studies, advancing the third potential treatment modality for our PKU franchise. We are encouraged by the Phe lowering and safety results observed in the first 2e13 dose cohorts in our Phase I/II study, and we are now ready to move the next dose of 2e13, which is similar to the ROCTAVIAN dose. Based on this early data from the 2e13 cohort and our prior steep dose response experience with ROCTAVIAN, we are optimistic that the 6e13 dose will be our optimal dose.

Also during the scripted portion of the Q4 2020 Earnings Call, Defendant Fuchs stated, in relevant part:

> Turning to BMN 307, our investigational gene therapy for PKU, results from the starting dose in the PHEARLESS Phase II study demonstrating meaningful Phe lowering in the first two subjects. The study will progress with a higher dose cohort, a three-fold higher dose 6e13 vested genomes per kilo. We're hopeful this test will be registration-enabling based on these early data from the 2e13 cohort in our prior steep first response experience with ROCTAVIAN. We are conducting this study with material manufactured with a commercial-ready process to derisk the program and facilitate rapid clinical development.
>
> We are excited about the prospects of BMN 307 as it represents a potential third treatment option in our PKU franchise and our second gene therapy development program. We look forward to sharing results from the dose confirmation phase of the study when we've selected dose registration-enabling studies. We doubled our early-stage pipeline in 2020 by internal growth and external partnerships, advancing several preclinical programs spanning multiple modalities. With gene therapy beyond our ROCTAVIAN and PKU programs, we're conducting IND-enabling studies with BMN 331 gene therapy for hereditary angioedema.

37. On February 26, 2021, 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, 2020 (the "2020 10-K"). The 2020 10-K touted the purported safety of BMN 307 observed in the Phearless Phase 1/2 study, stating, in relevant part: "On February 25, 2021, we announced that we plan to dose escalate in the PHEarless Phase 1/2 study of BMN 307, a gene therapy for the treatment of PKU, based on encouraging Phe lowering and safety signals observed in study participants who were treated with the lowest dose."

38. Appended to the 2020 10-K as an exhibit was a signed certification pursuant to SOX by Defendants Bienaimé and Mueller, attesting that "the information contained in the [2020 10-K] fairly presents, in all material respects, the financial condition and results of operations of the Company."

39. On April 29, 2021, BioMarin issued a press release announcing the Company's Q1 2021 financial results and corporate updates. The press release listed as one of the Company's financial highlights:

> ***Earlier-stage Development Portfolio (BMN 307, BMN 255, BMN 331, DiNA-001, Allen Institute Collaboration)***
>
> - BMN 307: Dose escalation in PHEarless, the Phase 1/2 study of BMN 307 continues based on encouraging Phe lowering and safety profile observed in study participants who were treated with the lowest dose.

40. That same day, BioMarin hosted an earnings call with investors and analysts to discuss the Company's Q1 2021 results (the "Q1 2021 Earnings Call"). During the scripted portion of the Q1 2021 Earnings Call, Defendant Fuchs stated, in relevant part, "[b]riefly, on the earlier stage pipeline, dose escalation has commenced with BMN 307, our investigational gene therapy for phenylketonuria. And the program continues based on the encouraging Phe lowering observed with a lower dose that's been tested so far." Further, when asked a question regarding the Company's decision to move to a higher dose in the Phearless Phase 1/2 study, Defendant Fuchs responded, in relevant part, ". . . we're very excited. The PKU market is not -- in spite of just fantastic work in spite of how good Palynziq is, there's plenty of opportunity to expand the PKU

market and 255 fits into our model of genetically validated and potentially transformative interventions. So, we're very excited about the programs." Finally, when asked to identify the key differences between BMN 307 and its competition, Defendant Fuchs responded:

> Well, AAV5 is a little bit better known to us than any other AAV might be to almost any other company just because of having a large amount of both, clinical experience, but also manufacturing experience. We have an enormous amount of preclinical data on almost every single nucleotide in the cassette. I mean, every single thing has kind of been tested for specific reasons when it comes to matters like codon optimizations or spacers or tails or those sorts of things. So, we leveraged a lot of the knowledge that we've gained in the building of ROCTAVIAN to build an even more potent phenylalanine hydroxylase.
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> And I think if you don't have just as much experience as our group has in terms of designing vectors, testing them and mice testing them in nonhuman primates and then ultimately bringing them to humans and being able to iterate what we've learned. If you don't have all that experience, then you're just sort of flying blind. I think that they can be encouraged that they got some expression with phenylalanine hydroxylase, but they're pulling back from that dose rather than leaning into a dose of even more effective. Because of our confidence in the safety profile of ROCTAVIAN and what we've seen so far, we're very confident that the dose level that we're at is going to produce meaningful -- extremely meaningful fee reduction levels. So, I think at the end of the day, the real answer to your question is, our view of the competition is that we're going to end up being more effective by virtue of having a better vector design, the details of which are kind of inside the guts of the vector?

41. On July 28, 2021, BioMarin issued a press release announcing the Company's Q2 2021 financial results and corporate updates. The press release listed as one of the Company's financial highlights:

> ***Earlier-stage Development Portfolio (BMN 307, BMN 255, BMN 331, DiNA-001, Allen Institute Collaboration)***
>
> - BMN 307: Dose escalation in PHEarless, the Phase 1/2 study of BMN 307 continues based on encouraging Phe lowering and safety profile observed in study participants who were treated with the lowest dose.

42. That same day, BioMarin hosted an earnings call with investors and analysts to discuss the Company's Q2 2021 results (the "Q2 2021 Earnings Call"). During the scripted portion of the Q2 2021 Earnings Call, Defendant Fuchs stated, in relevant part:

> Turning now to our earlier-stage pipeline and beginning with 307 gene therapy for phenylketonuria. The dose escalation portion of the study continues with incoming subjects now receiving a 6e13 vector genome per kilogram dose, based on encouraging signals from the 2e13 vector genome per kilo dose, we look forward to gathering a meaningful amount of data with the 6e dose before determining next steps. To remind you, we are targeting normal fee and normal diet, and we look forward to the readouts from additional subjects given the interest in gene therapy solutions for phenylketonuria.

Further, when asked to provide an update on additional patients that had been dosed in the Phearless Phase 1/2 study, Defendant Fuchs responded, in relevant part:

> We are, in fact, at a second dose level. So, we've dosed patients at the first entry dose level, the study which was 2e13 vector genomes per kilo. We saw some signs of Phe-lowering efficacy. So, it says to us that we're probably on the dose response curve. On the basis of that, we elevated the dose in the next group of patients. We haven't communicated the data but what we -- from that next group of patients. But what we have communicated is that based on what we've seen in the 2e group and based on what we've seen with ROCTAVIAN, we're encouraged to think that it's possible that the 6e group would be the group that we choose to dose expand.

43. The statements referenced in ¶¶ 22-42 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, operations, and compliance policies. Specifically, Defendants made false and/or misleading statements and/or failed to disclose that: (i) BMN 307 was less safe than BioMarin had led investors to believe; (ii) BMN 307's safety profile made it likely that the FDA would place a clinical hold on the Phearless Phase 1/2 study; (iii) accordingly, the Company had overstated BMN 307's clinical and commercial prospects; and (iv) as a result, the Company's public statements were materially false and misleading at all relevant times.

The Truth Emerges

44. On September 5, 2021, BioMarin issued a press release entited, "U.S. FDA Placed a Clinical Hold on BMN 307 Phearless Phase 1/2 Gene Therapy Study in Adults with PKU Based on Interim Pre-clinical Study Findings." The press release stated:

> BioMarin [. . .] announced today that the [FDA] placed a clinical hold on the BMN 307 Phearless Phase 1/2 study. The Phearless study is evaluating BMN 307, an

> investigational AAV5-phenylalanine hydroxylase (PAH) gene therapy, in adults with phenylketonuria (PKU). The FDA's clinical hold was based on interim safety findings from a pre-clinical, non-GLP pharmacology study.
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> The Company carried out this pre-clinical study to understand the durability of BMN 307 activity in mice bearing two germline mutations, which may predispose the mice to the development of malignancy. One mutation eliminated the PAH gene that's missing in PKU and the second rendered the animals immunodeficient. Of 63 animals treated, six of seven animals administered BMN 307 at the highest dose group (2e14 Vg/kg) had tumors on liver necropsy 52 weeks after dosing with evidence for integration of portions of AAV vector into the genome. No lesions were observed in any mice at 24 weeks. Five of these animals had adenomas and one had a hepatocellular carcinoma (HCC). The translatability of these findings to humans is uncertain and under further investigation.
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> To date, the Company has only dosed humans in the Phearless Phase 1/2 clinical study with lower doses of either 2e13 vg/kg or 6e13 vg/kg. Due in part to the risk previously identified by historical rodent studies, the liver health of Phearless study participants is regularly monitored. The Company will work with the Data Review Board and Principal Investigators to further evaluate the study participants who have been dosed and will continue to monitor them over the long-term. The clinical significance of these pre-clinical rodent findings has not been established and cancers due to AAV integration have not been observed in larger animals or humans. BioMarin is pausing further enrollment into this global Phase 1/2 study until the investigation of these findings is completed. The company is working with the FDA and other health authorities and will communicate next steps for the program when available.
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> "More than 3,000 patients have been treated with gene therapy, and there are no reports of cancers emerging as a consequence. Acknowledging the complexity of the issue as highlighted in this week's FDA discussion, integrational mutagenesis and resultant cancer formation has been observed in mice using other AAV vectors," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "Therefore, we plan to investigate these findings. For patients who have already received lower doses of these vectors, we will continue to carefully evaluate and monitor their health. We are committed to understand and mitigate any risk of cancer causation."

45. On this news, BioMarin's stock price fell $7.14 per share, or 8.4%, to close at $77.81 per share on September 7, 2021, the next trading day.

46. 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.

PLAINTIFF'S CLASS ACTION ALLEGATIONS

47. 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.

48. 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.

49. 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.

50. 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.

51. 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.

52. 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.

53. 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.

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

55. 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)

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

57. 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.

58. 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.

59. 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.

60. 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.

61. 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.

62. 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.

63. 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.

64. 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.

65. 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)

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

67. 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.

68. 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.

69. 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.

70. 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.

71. 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.