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UNITED STATES DISTRICT COURT

NORTHERN DISTRICT OF CALIFORNIA

SAN FRANCISCO DIVISION

In re BioMarin Pharmaceutical Inc. Securities Litigation	Case No. 3:20-cv-06719-WHO CLASS ACTION **AMENDED CLASS ACTION COMPLAINT FOR VIOLATIONS OF THE FEDERAL SECURITIES LAWS** DEMAND FOR JURY TRIAL

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Lead Plaintiff, Arbejdsmarkedets Tillægspension ("ATP" or "Lead Plaintiff"), by and through its counsel ("Lead Counsel"), bring this action individually and on behalf of all persons and entities who purchased the publicly traded common stock of BioMarin Pharmaceutical Inc. ("BioMarin" or the "Company") between March 3, 2020 and August 18, 2020, inclusive (the "Class Period") and were damaged thereby, arising from false and misleading statements by Defendants BioMarin, its Chief Executive Officer ("CEO") Jean-Jacques Bienaimé ("Bienaimé"), and President of Worldwide Research & Development Henry J. Fuchs ("Fuchs") (Bienaimé and Fuchs are collectively referred to as the "Individual Defendants" in this Complaint, and with BioMarin, the "Defendants").

Lead Plaintiff alleges the following upon information and belief, except as to those allegations concerning Lead Plaintiff, which Lead Plaintiff alleges upon personal knowledge. Lead Plaintiff's information and belief are based upon Lead Counsel's investigation, which included review and analysis of, *inter alia*: (i) BioMarin's filings with the United States Securities and Exchange Commission ("SEC"); (ii) Defendants' additional public statements, including those made in press releases, at investor conferences, and on earnings calls; (iii) analyst reports concerning BioMarin; (iv) interviews with former employees of BioMarin; and (v) other publicly available information regarding the Company. Lead Counsel's investigation into the factual allegations contained in this Complaint is continuing, and many of the relevant facts are known only by Defendants or are exclusively within their custody or control. Lead Plaintiff believes that substantial additional evidentiary support will exist for the allegations set forth in this Complaint after a reasonable opportunity for further investigation or discovery.

I. INTRODUCTION

1. This case arises from Defendants' materially false and misleading statements concerning BioMarin's application to the FDA for approval of the Company's most important new drug, a gene therapy for hemophilia called valrox. Investors were deeply focused on the progress of the FDA's review, and, in particular, the successful completion of a key regulatory inspection of the new valrox manufacturing facility, which the agency had made clear was a condition of approval (the "Preapproval Inspection"). Throughout the Class Period, Defendants assured investors that BioMarin was "working very closely" and "quite collaborative[ly]" with the FDA in connection with the valrox

application, that the Company was meeting or ahead of approval milestones, and, critically, that the Preapproval Inspection was scheduled to occur in the second quarter of 2020 – "significantly before" the August 2020 deadline for FDA to approve the treatment.

2. None of this was true. At the very start of the Class Period, the FDA warned BioMarin that its critical Preapproval Inspection of the valrox manufacturing facility would likely be delayed beyond the second quarter of 2020, severely jeopardizing valrox's approval by the August deadline. Further, Defendants admitted that beginning in mid-April 2020 through the end of the Class Period, the Company had "no dialogue whatsoever" with the agency – totally contrary to Defendants' Class Period statements that BioMarin was "working very closely" and "quite collaborative[ly]" with the FDA. As a result of the agency's silence, BioMarin was unable to address critical approval issues with the agency. The only break in the FDA's silence came in June 2020, when the FDA ***directly warned*** BioMarin that approval of valrox by the August 2020 deadline was highly improbable. As Defendants have now admitted, at a private meeting in June 2020, the FDA flagged serious concerns about valrox's efficacy. During that meeting, Defendants frantically sought reassurances from the FDA that its concerns would not impact approval, but the agency refused to provide any. At the same time, the FDA also told BioMarin it was cancelling the Preapproval Inspection that was required for agency approval. Defendants later admitted that, behind the scenes, BioMarin was "going crazy" and "scrambl[ing]" internally as a result of this cancellation. Rather than admit to the public that valrox's approval by the August 2020 deadline was, at a minimum, in serious jeopardy, Defendants continued to assure investors that the key valrox review milestones were being met and that, given valrox's imminent approval, BioMarin remained well ahead of competitors in bringing the first hemophilia gene therapy to market. Shortly after BioMarin's highly negative June 2020 meeting with the FDA, and while BioMarin's stock price was soaring because of Defendants' false and misleading statements, Defendant Fuchs sold nearly twenty-million dollars in BioMarin stock. On August 19, 2020, BioMarin admitted that the FDA had ***rejected*** the valrox application for August 2020 approval, just as the agency's private warnings to BioMarin had clearly signaled for months, causing BioMarin's stock price to plummet by 35% and resulting in billions of dollars in losses to BioMarin's investors.

3. BioMarin is a specialty pharmaceutical company that developed valrox to treat hemophilia A, a genetic disorder in which patients are unable to produce a critical blood clotting protein called "Factor VIII." Valrox belongs to a novel class of treatments called gene therapies, which treat disorders caused by genetic mutations by introducing healthy genetic material into the patient's cells. BioMarin touted valrox as a one-shot treatment that could save patients years of ongoing treatments for severe hemophilia.

4. Valrox represented BioMarin's first foray into the emerging field of gene therapy. Gene therapies are difficult to manufacture and, as a BioMarin executive explained, the Company "started off with very little expertise and capabilities within the company from a [gene therapy] manufacturing perspective." In 2016, the Company first attempted to develop its own manufacturing process for valrox and build a facility – located in Novato, California – capable of producing it (the "Novato Facility").

5. As Defendants Fuchs stated, the product had the potential to be "transformative for BioMarin." Given the incredible $2 million price tag BioMarin announced it would charge for a single dose of valrox, once fully launched, valrox could ***double*** BioMarin's revenue. As a result, BioMarin's investors were singularly focused on valrox's approval throughout the Class Period.

6. Critically, while BioMarin faced stiff competition from larger pharmaceutical companies like Pfizer and Roche, which were developing their own gene therapies for hemophilia A, valrox was poised to become the first treatment to enter the marketplace and, thus, gain a significant first-mover advantage against these well-funded rivals. Accordingly, obtaining accelerated FDA approval of valrox was of great importance to BioMarin and its investors. When, in December 2019, BioMarin submitted its Biologics License Application, or "BLA," to the FDA seeking approval to market valrox, it appeared to investors that BioMarin remained well positioned to be first to market.

7. Pursuant to federal law, the FDA set a deadline of August 21, 2020 to complete its review of the valrox BLA (known as the "PDUFA Date"). One of the most important steps in the FDA's approval process for any new drug is the completion of a Preapproval Inspection of the facilities in which the new drug will be manufactured, including a detailed review of the processes and controls involved in producing the drug. The FDA relies heavily on such inspections to ensure that the drug

can be safely and reliably commercially manufactured. Even in the case of routine drug applications, the FDA's manufacturing review is highly detailed. As BioMarin explained to investors, it is not "unusual to get 400 questions during the review process both clinical, nonclinical and CMC [Chemistry Manufacturing Controls]."

8. In valrox's case, both Defendants and investors understood that the Preapproval Inspection of BioMarin's brand-new Novato Facility – which had never been inspected by the FDA – would be a focal point of the FDA's BLA review. As Defendant Fuchs admitted, the FDA had made clear to BioMarin that a successful Preapproval Inspection of the Novato Facility was required for approval of the valrox application.

9. Just prior to the start of the Class Period, BioMarin released data from ongoing clinical trials that raised questions about the durability of valrox's benefit and the adequacy of BioMarin's manufacturing processes. Among other things, those data indicated that patients taking valrox manufactured by BioMarin at the Novato Facility (using the same processes that would be used to make the commercially marketed version of valrox) produced just half the critical clotting protein, Factor VIII, produced by patients taking valrox that had been made exclusively for use in clinical trials by a third-party laboratory (using the third party's manufacturing processes and controls). The data also indicated that the benefit provided by valrox declined over time, making patients' initial levels of Factor VIII even more important in assessing how long valrox's benefits would last. While Defendants claimed that other differences between the two trials were responsible for the decline in Factor VIII, both Defendants and investors understood that the FDA would be particularly focused on inspecting the Novato Facility to ensure that the Company's commercial manufacturing processes did not negatively affect valrox's efficacy. Indeed, shortly after BioMarin released these data, Dr. Peter Marks, the head of the FDA division responsible for reviewing the valrox BLA, publicly emphasized the significant "challenges in manufacturing gene therapy products," and, in particular, the difficulties in scaling production up from clinical formulation to commercial formulations—just as BioMarin proposed to do with valrox.

10. Given the importance of the valrox BLA to BioMarin, investors repeatedly sought, and Defendants provided, numerous reassurances throughout the Class Period that the FDA's review, and the critical Preapproval Inspection in particular, was on track or ahead of schedule.

11. Critically, Defendants repeatedly reassured investors that BioMarin had been in close dialogue with the FDA throughout the BLA approval process. For instance, at an investor conference on June 4, 2020, Defendant Fuchs responded to yet another analyst inquiry about the Preapproval Inspection, stating "Yes. We have a schedule [for the inspection]. And in fact, the [FDA] has been quite collaborative. . . . [T]he agency has ***signaled strongly*** that they intend to maintain their PDUFA action date."[1] Likewise, at an investor conference just a few days later, an analyst ***again*** asked Defendants for assurance regarding the critical Preapproval Inspection. Fuchs responded that "in regards to major metrics, the agency is actually ahead of their PDUFA mandated regulatory metrics in terms of time line" and "we're working very closely with them to keep things on track."

12. As a result of this purported close contact with the FDA, Defendants confidently assured investors that the review of the valrox BLA was proceeding successfully. For instance, on BioMarin's April 2020 earnings call, Bienaimé assured investors that BioMarin was "tracking to our milestones" in the BLA review, "and in some cases, [the FDA] is accelerating their work." Likewise, at a May 2020 investor conference, and in response to an analyst's direct question about the Preapproval Inspection, Bienaimé reiterated that the Preapproval Inspection would occur in the second quarter of 2020, "significantly before the PDUFA date. So we're on track in this respect."

13. These statements were false and misleading. In truth, as a former BioMarin executive explained, by the start of the Class Period, the FDA warned BioMarin that the Preapproval Inspection of the valrox facility would likely be delayed beyond the second quarter of 2020. FDA guidance provides that the agency conducts Preapproval Inspections at least two months prior to the PDUFA date in order to leave sufficient time to "address any deficiencies" prior to the deadline for agency action. Accordingly, Defendants knew by the start of the Class Period that approval of the valrox BLA by the August 21, 2020 PDUFA date was in serious jeopardy.

[1] Throughout this Complaint, all emphasis in quoted material is added unless otherwise noted.

14. Moreover, contrary to Defendants' statements that BioMarin was "working very closely with [FDA] to keep things on track," and that the agency had been "quite collaborative" and "strongly" signaled the Preapproval Inspection was on track, in reality BioMarin "had ***no dialogue whatsoever with the agency***" from mid-April 2020 until August 18, 2020, as Defendant Fuchs ultimately admitted. Thus, when Fuchs reassured investors in June 2020 that the FDA was "quite collaborative" and was "working very closely" with BioMarin through the review, BioMarin had actually "had no dialogue whatsoever" with the FDA for nearly two months.

15. Indeed, the only communications the FDA had with BioMarin after mid-April made clear that approval of the valrox BLA by the August 2020 PDUFA date was all but impossible. Defendants have now admitted that at BioMarin's "late-cycle meeting" with the FDA in mid-June 2020, and in a pre-meeting memorandum the agency sent to the Company a few days earlier, the FDA privately raised with BioMarin the very same concerns that formed the basis of the agency's decision denying the Company's BLA. Specifically, in that June 2020 meeting and memorandum, the FDA cited the reduced Factor VIII activity observed in patients taking the BioMarin-made formulation of valrox, and, as Fuchs admitted, questioned whether the "gene therapy is going to wear off" based on the trajectory of factor activity. The FDA also raised the possibility that manufacturing differences were driving the reduced efficacy. Significantly, though BioMarin sought reassurances from the FDA about the impact of those concerns on approval of the valrox BLA, the agency refused to provide any.

16. Defendants have further admitted that, at the same time as the disastrous June 2020 meeting, the FDA completely ***canceled*** the Preapproval Inspection on which valrox approval depended. Fuchs later acknowledged that, in the wake of that cancellation, BioMarin was "going crazy" and "scrambl[ing]" internally, and that, while BioMarin attempted to get the FDA to accept alternatives to a formal inspection, it "could never get any traction on that discussion."

17. Even after the FDA flagged serious concerns about valrox's efficacy and canceled the required Preapproval Inspection, Defendants continued to make concrete assertions of past and present facts falsely assuring investors that the valrox BLA review, including the Preapproval Inspection, was meeting, or ahead of, every milestone. For instance, at a June 24, 2020 investor conference, an analyst asked Defendants whether, in connection with the valrox BLA, they "still expect to have an inspection

in the first half of this year[.]" Despite the fact that the FDA had flagged concerns about valrox's efficacy and that BioMarin was internally "going crazy" and "scrambl[ing]" as a result of the cancellation of the Preapproval Inspection, Fuchs falsely assured investors that "we're on track for our PDUFA [date of] 8[/]21." Likewise, at an August 13, 2020 investor conference, Fuchs touted valrox's lead over competing therapies, claiming that BioMarin's competitors were "***so far behind***," while failing to disclose that, with the PDUFA date only a week away, the FDA had cancelled the Preapproval Inspection that was a condition of approval, making approval by the August 2020 date impossible under the FDA's own guidelines.

18. Notably, in July 2020, as BioMarin was internally "going crazy" about the FDA's cancellation of the Preapproval Inspection, but BioMarin's stock price was soaring as a result of Defendants' misleading statements, Defendant Fuchs sold nearly 60% of his BioMarin holdings, reaping nearly $20 million in proceeds. Fuchs' sales departed significantly from his prior trading patterns. Indeed, in the six months preceding the six-month Class Period (the "Control Period"), Fuchs did not sell a single share of BioMarin stock.

19. The truth was ultimately revealed on August 19, 2020, when BioMarin announced that the FDA would not approve valrox. Analysts were "shocked" and "surprised" by this news and immediately raised questions concerning management's credibility, noting the Company's recent "positive commentary on regulatory process[,]" management's "uber bullish" remarks "regarding [valrox]'s commercial prospects," and the fact that "[u]nder [breakthrough therapy designation], we would assume that [BioMarin] would have been aware of" the approval problems "well before the week of the [FDA's August 21, 2020 PDUFA action date]." On this news, BioMarin stock plummeted by $41.82 per share, more than 35%, wiping out billions in shareholder value.

II. JURISDICTION AND VENUE

20. The claims asserted herein arise under 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 (17 CF.R. § 240.10b-5).

21. This Court has jurisdiction over the subject matter of this action pursuant to 28 U.S.C. §§ 1331 and 1337 and Section 27 of the Exchange Act (15 U.S.C. § 78aa).

22. Venue is proper in this District pursuant to Section 27 of the Exchange Act (15 U.S.C. § 78aa) and 28 U.S.C. § 1391(b). BioMarin maintains its corporate headquarters in San Rafael, California, which is situated in this District, conducts substantial business in this District, and many of the acts and conduct that constitute the violations of law complained of herein, including dissemination to the public of materially false and misleading information, occurred in and/or were issued from this District.

23. 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.

III. PARTIES

24. Lead Plaintiff ATP is a Denmark-based pension fund established in 1964 that provides retirement allowances and other benefits to Danish citizens. At the end of 2020, ATP managed more than 959 billion DKK (approximately $156 billion USD) in net assets for the benefit of more than 5 million members. As reflected in ATP's certification on file with the Court (ECF No. 30-2), ATP purchased shares of BioMarin stock during the Class Period and suffered damages as a result of the violation of the federal securities laws alleged in this Complaint.

25. Defendant BioMarin is a Delaware corporation based in California and maintains its corporate headquarters at 770 Lindaro Street, San Rafael, CA 94901. The Company's common stock trades on the Nasdaq Stock Market ("NASDAQ") under the ticker symbol "BMRN." As of October 23, 2020, BioMarin had over 181 million shares of common stock outstanding.

26. Defendant Jean-Jacques Bienaimé ("Bienaimé") currently serves as BioMarin's Chairman and Chief Executive Officer ("CEO"). Defendant Bienaimé joined the Company as CEO and member of the Board of Directors in May 2005.

27. Defendant Henry J. Fuchs ("Fuchs") served as BioMarin's President of Worldwide Research & Development during the Class Period. Defendant Fuchs joined the Company in March 2009 as Senior Vice President and Chief Medical Officer.

IV. SUMMARY OF THE FRAUD

A. BioMarin Pharmaceutical Inc.

28. BioMarin is an American specialty biotechnology and pharmaceutical company founded in 1997 and headquartered in San Rafael, California. Historically, BioMarin has researched, developed, and commercialized pharmaceuticals to treat rare diseases and medical conditions. BioMarin describes itself as focused on "select[ing] product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products."

29. Specifically, BioMarin focuses on the development and commercialization of drugs with "orphan drug" status—drugs that treat medical conditions that are so rare they would not be profitable to produce without government assistance. In the United States, an orphan drug is one that treats a rare condition or disease that affects fewer than 200,000 people.

30. As described by BioMarin, the Company's focus on rare diseases and conditions requires the Company to achieve "significant market share and maintain high per-patient prices for [its] products to achieve profitability." To achieve these goals, BioMarin focuses on first-to-market orphan drugs, which can come with significant and highly lucrative exclusivity rights. Even more, orphan drugs are exempt from the Medicaid mandatory discount pricing established by the Affordable Care Act. The average annual cost of orphan drug treat exceeds "mainstream" drug treatment by nearly 1400%.

31. At the beginning of the Class Period, BioMarin's drug portfolio consisted of six FDA-approved orphan drugs. Just one of its drugs, Kuvan, accounted for nearly 30% of the Company's total net revenue in 2019, but the marketing of generic versions of Kuvan would begin in October 2020. BioMarin's patents for one of its other drugs, Aldurazyme, which accounted for approximately 6% of BioMarin's 2019 net revenue, would expire in 2021. Accordingly, by the beginning of the Class Period, BioMarin faced a threat to nearly one-third of its net revenue due to generic competition.

32. BioMarin was relying heavily on its pipeline of developmental drugs to defend against these threats to the Company's revenue. At the beginning of the Class Period, BioMarin's research and development pipeline consisted of only four products, including valrox. Valrox was BioMarin's

only new product with an expected release date in 2020. As discussed below, Defendants touted valrox as transformative for the Company, with potential to double its revenue. As of January 2020, Defendant Bienaimé announced that, with the expected valrox launch in 2020, "We anticipate for the first time in the history of the company, breakeven or slightly GAAP net income positive with valrox approval."

B. BioMarin's Development of Valrox Generates Significant Investor Enthusiasm

33. BioMarin developed valrox as a treatment for hemophilia A, an inherited bleeding disorder in which the blood does not clot properly. In hemophilia A patients, this condition is caused by the body's inability to correctly produce a critical clotting protein called "Factor VIII."

34. Valrox belongs to a relatively novel class of treatments known as "gene therapies." Gene therapies treat conditions caused by mutated or missing genes by introducing a normal copy of the affected genetic material into the body's cells. Genes inserted directly into the body do not usually function. Accordingly, gene therapies use a "vector," often a benign virus, to introduce the healthy genetic material into a patient's cells. *See* Figure 1, below. Valrox was designed to treat hemophilia A by using an adenovirus vector to deliver functional Factor VIII genes to patients who lacked them.

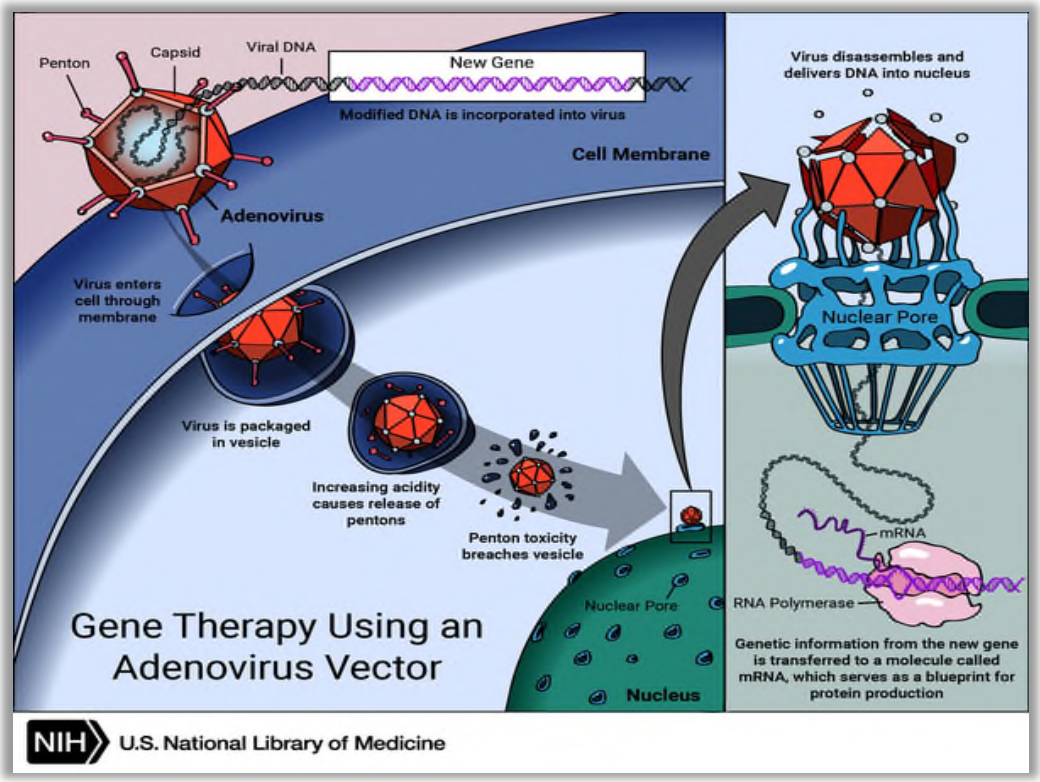

Figure 1. Gene therapy uses vectors, such as a benign adenovirus, to deliver healthy genetic material to cells and replace mutated or missing genes.

35. Given their novelty and complexity, gene therapies are costly and difficult to manufacture. As BioMarin's former President of Global Manufacturing and Technical Operations, Robert Baffi, explained at an October 2019 investor conference, "it costs a significant amount of money to build a facility [equipped to manufacture a gene therapy], but building the facility is only one part of it. It's not just the clean rooms and the HVAC systems and things of that nature. It's really development of the overall process and analytical controls along with the manufacturing and having well-trained personnel to run those processes, which are fairly complex."

36. BioMarin, however, was a newcomer to this developing field, as valrox was the first gene therapy BioMarin attempted to design and manufacture. Indeed, as Baffi explained at a November 2019 investor conference, BioMarin "started off with very little expertise and capabilities within the company from a [gene therapy] manufacturing perspective."

37. It was not until 2016, after BioMarin achieved initial favorable clinical trial results with a formulation of valrox manufactured by a third-party laboratory, that the Company first attempted to

develop its own manufacturing process for the drug and built a facility capable of producing it in Novato, California. BioMarin claimed to have successfully developed scalable manufacturing for valrox at its new Novato Facility in just a few short months, despite its inexperience with gene therapies.

38. Valrox generated enormous investor excitement because it was poised to be the first gene therapy approved for hemophilia A. Currently, hemophilia A is typically treated by intravenously administering a clotting factor concentrate, called "replacement therapy," to patients as many as two or three times a week. Replacement therapy is not only cumbersome for patients, it is expensive. Prophylactic replacement therapy can cost upwards of $400,000 annually.

39. Gene therapy, by contrast, consists of only a single, one-time infusion that, if effective, could greatly improve patients' quality of life. And, because currently available hemophilia A treatment is costly, insurers would be willing to pay handsomely for an effective one-time treatment. Indeed, BioMarin intended to charge as much as $2 million for a single infusion of valrox, making it the most expensive drug ever marketed.

40. Investors were particularly enthusiastic about valrox because it would also give BioMarin the "first-mover advantage" for gene therapy treatment for hemophilia, *i.e.*, a preexisting presence in the marketplace allowing the "first-mover" to preempt potential competition and gain significantly more market-share. While BioMarin faced stiff competition from larger pharmaceutical companies, including Pfizer and Roche, which were developing their own gene therapies for hemophilia A, Defendants repeatedly told investors that BioMarin had a meaningful head start against those rivals. As discussed below, BioMarin's first-mover advantage depended largely upon BioMarin employing every option available under the FDA's regulatory regime to accelerate review of valrox.

41. Given valrox's potential to transform the therapeutic landscape for hemophilia patients, analysts estimated that, once fully launched, the drug would ***double*** BioMarin's revenue, generating more than $1.5 billion in annual sales. In a May 2019 report, for instance, J.P. Morgan analysts explained, "given the high cost and inconvenience of therapy for hemophilia A, there is appetite for a one-time, potentially curative therapy." These analysts estimated that valrox alone would generate $1.6 billion in annual sales (as compared with BioMarin's total 2018 revenue of $1.5 billion) if

BioMarin captured one third of the severe hemophilia population. As Defendant Fuchs explained at a June 11, 2019 investor conference, "Now to put 1/3 in context, 117,000 patients in BioMarin territories . . . . even if you used a number like 10,000 and you said there's 3,000 patients who are going to be ready to take valrox in Year 1, even [if the treatment was priced at] $1 million a year, 3,000 patients is almost twice the revenue that we're currently experiencing, and so the demand size, the value that valrox represents is really substantial and ***transformative for BioMarin***."

42. In January 2020, BioMarin announced it was likely to target more than that $1 million price point. Speaking at the January 13, 2020 JPMorgan Healthcare Conference, Defendant Bienaimé disclosed that the Company "ha[s] done a lot of payer research in the U.S. and in Europe. . . . it looks like the U.S. payers are somewhat comfortable with a price around $2 to $3 million per therapy."

43. Following the Company's pricing announcement, analysts were even more emphatic that accelerated approval would be a transformative event for the Company. In a February 2020 report, for instance, Cowen analysts noted, "there is little question that Hem A is a large market and that, should it be successful, valrox would address a large opportunity." The Cowen analysts continued, "BioMarin estimates that overall there are 117K hemophilia A patients in its territories . . . with 18K in North America alone. Thus, ***even modest penetration with a price point around $2MM would represent significant revenue, as ~$1B in sales could be achieved for every 500 adult patients treated***."

44. Importantly, however, gene therapy patients cannot typically be re-dosed once treated, as, among other things, they develop antibodies to the vectors used to deliver the healthy genetic material. Accordingly, valrox would be medically effective and commercially successful if, and only if, it provided a durable and long-lasting reduction in bleeding events. If valrox's benefit was not long-lived, insurers would not view the treatment as cost-effective and health care providers would not prescribe it. As Evercore analysts observed in a February 2019 report, durability was the "key question" on which valrox's value proposition depended. These analysts explained, "The key question on valrox will be whether [patient factor levels] stabilize at 3 years or if they are continuing to decline at 3 years. If the effect isn't durable, the value proposition may be harder to communicate. As such, the error bars on hemophilia A peak sales potential remains extremely wide."

C. In May 2019, BioMarin Reports Clinical Trial Data That Heightens The Importance Of Regulatory Review of the Company's Manufacturing Processes and Controls for Valrox

45. Investors' focus on the durability of valrox's benefit was heightened when, on May 28, 2019, BioMarin released results for two important clinical trials of valrox.

46. By way of background, the FDA typically classifies clinical trials of new drugs into four phases: small phase I trials are conducted to evaluate the drug's safety in humans; larger phase II trials are conducted to evaluate efficacy; still larger phase III trials are conducted to compare the experimental drug to competing therapies in terms of both efficacy and safety; finally, large phase IV trials are conducted after the drug has been marketed to continue to evaluate safety and investigate efficacy for new indications.

47. BioMarin had initiated a Phase I/II trial of valrox in 2015 and a Phase III trial in 2017. Significantly, while patients in the Phase I/II trial received formulations of valrox manufactured in small batches by a third-party laboratory specifically for use in the Phase I/II trial (using the laboratory's own manufacturing processes and controls), patients in the Phase III trial received valrox infusions manufactured by BioMarin at its new Novato Facility using the same manufacturing processes and controls that BioMarin would use to mass produce the valrox sold to patients if the treatment was approved.

48. In the May 2019 announcement, BioMarin released results from both trials. First, BioMarin updated results from its ongoing combined Phase I/II trial of valrox, releasing a third year of efficacy data. The "primary endpoint"– the criteria defining success in the Phase I/II trial – was the level of Factor VIII activity achieved by patients. Initially, valrox had demonstrated encouraging efficacy. Normal Factor VIII activity in healthy humans ranges between 50 and 150 international units per deciliter ("IU/dL"). One year after infusion, Factor VIII activity levels for valrox patients taking the highest dose of therapy were within this zone of normal activity, with mean and median Factor VIII activity levels of 64.3 IU/dL and 60.3 IU/dL respectively. By the end of the second year after infusion, however, factor activity levels in those patients had declined significantly, with mean and median levels of 36.4 and 26.2. BioMarin had previously reported these data to the public in July 2017 and May 2018. In its May 2019 third-year update, BioMarin revealed that patient Factor VIII

levels had continued to decline, though the rate of decline was less severe, with mean and median Factor VIII activity levels of 32.7 IU/dL and 19.9 IU/dL respectively. *See* Figure 2.

FVIII activity level (IU/dL) time point	Mean (Chromogenic)	Median (Chromogenic)	Mean (One Stage)	Median (One Stage)
Weeks 23-26	68	57	127	100
Week 52	64	60	104	89
Week 104	36	26	59	46
Week 156	33	20	52	30

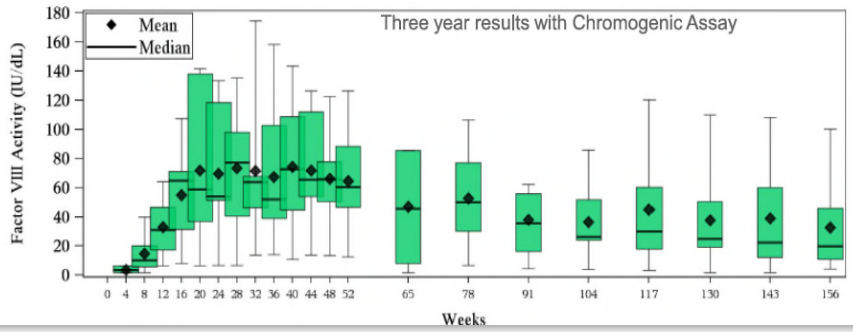

Figure 2. On May 28, 2019, BioMarin updated results for its Phase I/II study of valrox with a third year of efficacy data. These data showed continued decline in patients' Factor VIII activity levels.

49. Despite the continued decline in factor activity observed in the latest Phase I/II dataset, BioMarin management struck a confident tone, stating that "Rate of FVIII decline . . . slowed in year 3, and appears to be approaching a plateau."

50. Second, BioMarin released "interim results" from its ongoing Phase III trial of valrox (using the commercial formulation manufactured by BioMarin at the Novato Facility), providing investors their first glimpse of those data. BioMarin's Phase III results were more worrisome than the Phase I/II data. BioMarin's Phase III data showed that 26 weeks after infusion, Factor VIII activity for patients taking the highest dose of BioMarin-manufactured valrox was ***just half*** the Factor VIII levels expressed by Phase I/II patients (who received valrox produced by a third-party lab) after 26 weeks. In the 23 to 26-week timeframe, the mean and median Factor VIII activity in Phase III patients taking the highest dose of valrox was 36 IU/dL and 33 IU/dL compared with 68 IU/dL and 57 IU/dL for Phase I/II patients at the same timepoint. *See* Figure 3. Moreover, a higher-than-expected number of Phase III patients – 19% – did not respond to therapy, failing to generate factor activity above 5 IU/dL.

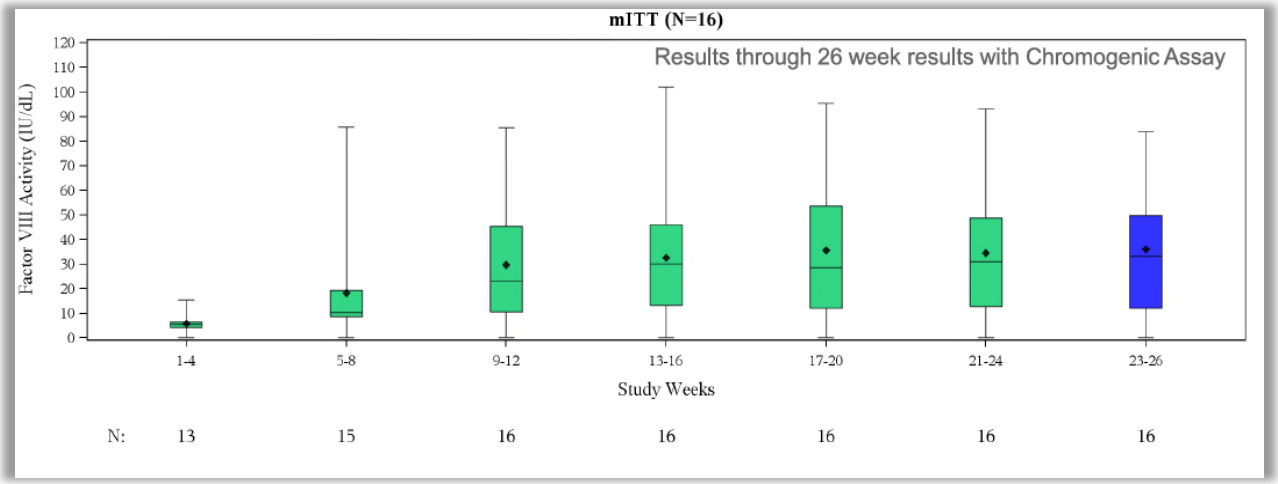

Figure 3. Also on May 28, 2019, BioMarin released the first interim results from its Phase III trial of valrox. These data showed that 26 weeks after infusion, patients taking the highest dose of valrox had only half the Factor VIII activity as Phase I/II patients at the same timepoint.

51. BioMarin acknowledged that Factor VIII expression observed in the Phase III trial was "a little bit lower than what we observed in the Phase I/II study" and that there was "some dispersion between the Phase II results and our Phase III results." Though BioMarin hypothesized that the observed decline in Factor VIII activity in Phase III patients was likely due to differences in steroid use amongst patient populations, the Company acknowledged that it was also possible the use of BioMarin-made product in the Phase III study was responsible for that trial's apparently inferior results.

52. For instance, on the Company's May 28, 2019 conference call, Fuchs stated that "it's possible that there are" some characteristics of the "product which have shifted the response a little bit to the left," *i.e.*, resulted in reduced factor activity. Bienaimé followed up on Fuchs' comments by alternatively hypothesizing that the decline in Factor VIII expression in Phase III patients "might be resulting from a change in the steroid protocol," *i.e.*, differences in steroid use between patients. Likewise, on a November 14, 2019 investor call, Baffi acknowledged that "one of the possibilities is that the variability [between results in the two trials] is due to the manufacturer of the product." And while Defendants continued to emphasize that they believed differences in steroid use between patients in the trials was the likeliest explanation for the decline in factor activity, they acknowledged that this was, as Fuchs stated on an October 23, 2019 investor call, a "hypothesis rather than necessarily a fact."

53. Analysts and investors were concerned about the apparent decline in Factor VIII expression amongst Phase III valrox patients and the potential causes of that decline. For instance, in a May 28, 2019 report, Evercore analysts stated, "The outstanding question now is what the implications are for the interim P3 results, and we wind up again having to wait to see durability of effect (and implications) in this new important cohort. BMRN is optimistic that the P2 portends a strong outlook for the P3 results, but since most of this field is in uncharted territory, uncertainty remains." The Evercore analysts continued, "Variability between P1/2 and P3 datasets adds risk that BMRN's extrapolated expression from P1/2 won't be accurate for P3. Difference in steroid protocol noted as a potential cause of the discrepancy, but this is only theoretical."

54. Certain analysts noted the potential impact of the switch to the Novato Facility on valrox's efficacy. For example, in a May 28, 2019 report, Canaccord analysts stated, "[Factor VIII] activity [in the Phase III data] squeaks by minimum regulatory threshold for accelerated approval at weeks 23-26, but . . . appears less robust than ph.1/2 . . . . Recall, changes made from ph.1/2 to ph.3 that may account for the drop in [Factor VIII] activity include prophylaxis corticosteroids to on demand, and ***new manufacturing process*** – currently unclear what is driving differences . . . ."

55. Nevertheless, given BioMarin's reassurances, investors remained confident that valrox would be timely approved. For instance, in the report discussed above, Canaccord analysts concluded, "we still see [valrox] as likely approvable given BioMarin's confidence in regulatory actions thus far . . . . Ph.1/2 data appear robust quelling durability questions in our view . . . ." Likewise, the Evercore analysts discussed above also concluded that "Overall we see valrox as a decent start for gene therapy . . . ***BMRN remains in the pole position for now***."

56. However, given the role BioMarin's manufacturing processes and controls might have played in valrox's apparently reduced efficacy in the Phase III trial, investors understood, and Defendants were well aware, that regulators would be particularly focused on thoroughly reviewing those manufacturing processes and controls. A key part of that in-depth review would be the agency's mandatory Preapproval Inspection of the Company's Novato Facility before it would approve the drug. Indeed, as Baffi had explained to investors at an October 2019 conference, even with respect to routine applications for approvals of biologics, the FDA "ask[s] a lot of questions" concerning the

manufacturing process. Baffi explained that it was not "unusual to get 400 questions during the review process both clinical, nonclinical and CMC [Chemistry Manufacturing Controls]."

57. The FDA was particularly focused on ensuring the adequacy, reliability, and reproducibility of manufacturing controls used in connection with gene therapies. In October 2019, shortly after BioMarin reported its anomalous Phase III valrox results, Dr. Peter Marks, the Director of FDA's Center for Biologics Evaluations and Research ("CBER") – the FDA division responsible for reviewing the valrox application – delivered an address at a clinical conference. In that address, Dr. Marks emphasized the significant manufacturing challenges gene therapies faced. Indeed, Dr. Marks stated that "[t]here are some challenges in [gene therapy] and ***many of them*** relate to manufacturing issues."

58. Almost as if speaking specifically about valrox, Dr. Marks further stated, "manufacturing turns out to be one of the most important aspects of developing [gene therapies], that's particularly true because oftentimes their effect in clinical development is very clear but making sure they're well-made and made consistently can be more challenging." As part of ensuring gene therapies are "well-made," slides presented by Dr. Marks at that conference again emphasized the importance of Preapproval Inspection. *See* Figure 4.

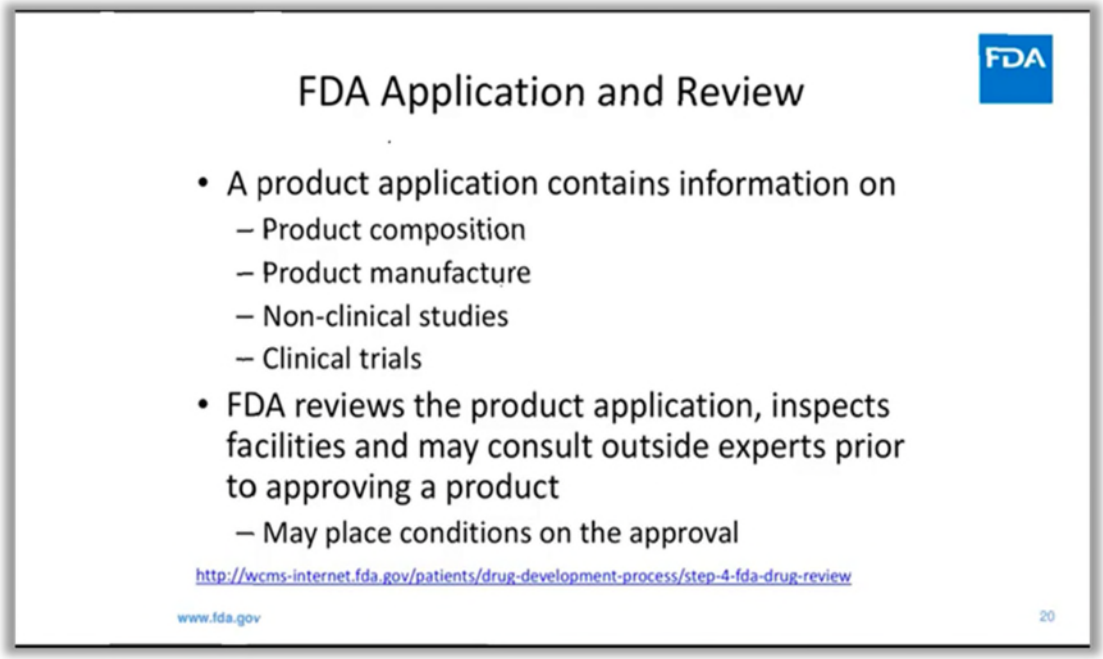

Figure 4. Slides presented by FDA's Director of CBER at an October 2019 clinical conference emphasized the importance of Preapproval Inspections of manufacturing facilities in evaluating applications for approval of novel gene therapies, like valrox.

59. At another conference that same month, Dr. Marks also raised concerns around transitioning from preclinical trials to commercial manufacturing for gene-therapy products. Dr. Marks emphasized that "challenges in manufacturing gene therapy products is one of the real areas where we still have a ways to go. We still have issues with process scale-up, which is not yet reproducible from clinical trial to commercial size lots."

60. Defendants were well aware of Dr. Marks' remarks. In fact, Baffi referenced them at a November 2019 investor conference, stating, "Dr. Marks highlighted his concerns about productivity, the scale, being able to produce enough product, the purification processes and the lack of international collaboration on requirements and expectations for these [gene therapy] programs." Defendants also understood that, as the FDA had never inspected BioMarin's Novato Facility, it would be particularly focused on the Preapproval Inspection performed in connection with the valrox application for FDA approval.

D. In December 2019, BioMarin Submits Its Application For Approval of Valrox to the FDA And the FDA Sets An August 21, 2020 Deadline To Act On the Application

61. On December 23, 2019, BioMarin submitted its Biologics License Application ("BLA") to the FDA seeking approval to market valrox. On February 20, 2020, the FDA accepted BioMarin's valrox BLA for review, beginning the agency's formal evaluation process of the application. Under the Prescription Drug User Fee Act ("PDUFA"), the FDA is required to complete its review of new drug applications within 10 months, or six months in the case of applications accepted for "accelerated" review. In an effort to maintain its lead over its larger, better financed, and more sophisticated competitors, BioMarin sought "accelerated" FDA approval of the valrox BLA. Accordingly, the FDA set a deadline to approve the valrox BLA – known in the industry as a "PDUFA date" – of August 21, 2020.

62. The PDUFA date for a new drug is a focus of intense interest for analysts, investors, and any company that makes a submission to the FDA. As described by a *Benzinga* analyst, "PDUFA dates are binary events that invariably serve as make-or-break catalysts for stocks" because they are the "deadlines by which the FDA reviews a new drug application before announcing its decision concerning the approvability/non-approvability of the drug." As noted by *The Motley Fool*, "[t]he FDA, as a matter of policy, does not publish an official list of PDUFA dates. However, many drug companies choose to release their PDUFA dates in the hopes that doing so will lead to an increase in their stock prices." Multiple investor sites and sources, including the investor tool BioPharmCatalyst, are devoted to calendaring and closely tracking PDUFA dates and company disclosures leading up to those dates.

63. BioMarin's BLA sought approval of valrox principally on the basis of the Phase I/II and interim Phase III Factor VIII data discussed above. Shortly after BioMarin submitted the valrox BLA, however, in January 2020, the FDA issued new guidance that was directly applicable to the valrox BLA titled "Human Gene Therapy for Hemophilia." As that guidance explains, Factor VIII activity is considered a "surrogate endpoint" – an indirect measure of efficacy – for hemophilia treatments, whereas reduction of a patient's "annualized bleeding rate," or ABR, is a direct measure of therapeutic benefit. That FDA guidance further explains that "[h]istorically, applicants of products

approved for treatment of hemophilia, (e.g., plasma-derived, recombinant replacement factors; bi-specific antibodies) have relied on annualized bleeding rate (ABR) to demonstrate clinical benefit," and despite its limitations continued to "recommend ABR as a primary endpoint to demonstrate clinical benefit." Nevertheless, that guidance further stated that "[f]actor activity may be considered as a surrogate endpoint for primary efficacy assessment under the accelerated approval pathway."

64. While both of BioMarin's trials collected ABR data, there was not yet enough data to demonstrate a statistically significant benefit associated with valrox on that direct endpoint. Accordingly, as discussed, BioMarin depended heavily on its factor activity data for approval of valrox. This put an even brighter spotlight on the observed decline in efficacy between the Phase I/II and III patients and the potential causes for that decline, including manufacturing-driven differences in the nature of the material used in the respective trials. Again, Defendants and investors understood that the FDA would be focused on thoroughly evaluating BioMarin's manufacturing processes and controls for valrox before it would approve the treatment.

65. Significantly, prior to submitting the valrox BLA, Defendants told investors that the Company had routine, detailed communications with the FDA about the application. In particular, valrox had received a Breakthrough Therapy designation from the FDA in October 2017. As the FDA explains, under federal law, sponsors of a Breakthrough Therapy-designated drug receive "[i]ntensive [agency] guidance on an efficient drug development program." Among other things, the FDA is required to "hold[] meetings with the sponsor and the review team throughout the development of the drug" and "provid[e] timely advice to, and interactive communication with, the sponsor regarding the development of the drug."

66. Defendants trumpeted the extensive guidance BioMarin had received from the agency about the valrox trials and anticipated application. For instance, at a June 2019 investor conference, Fuchs stated, "we've had quite a lot of encounters with both the Food and Drug Administration here in the United States and the European Medicines Agency in Europe because in the United States, we have Breakthrough Therapy designation . . . . And under those interactions, we've had fairly clear dialogue with them about requirements for registration."

E. Overview of the BLA Review Process

67. As discussed, PDUFA sets a timeline for the FDA to approve new drug and biologics applications: 10 months for standard review and 6 months for accelerated review. PDUFA, as well as agency regulation and policy, set up a number of milestones during the review process and a timeline for their completion. These milestones include, among other events, a "mid-cycle" communication between the FDA and company management to discuss the status of the pending application, critical communications concerning new drug labelling, a Preapproval Inspection of the manufacturing facilities, and a "late-cycle" meeting between the FDA and company management.

68. According to FDA guidance, the FDA will conduct a brief mid-cycle communication with the applicant to discuss, *inter alia*, the status of the review, any new information requests, and the projected date for the late-cycle meeting. Here, BioMarin later disclosed that the FDA's mid-cycle communication occurred in "mid-April."

69. Following the mid-cycle meeting, the FDA has numerous communications with the applicant. Among other things, the FDA holds multiple labeling reviews with the applicant, which agency guidance explains, "should be scheduled to [begin] approximately one to two weeks after the mid-cycle meeting," and, later, transmits draft labeling to the applicant. Appropriate labeling is, of course, a condition precedent to approval of any drug product. As CBER guidance explains, "Proper labeling of licensed and approved products is a requirement of the Food, Drug and Cosmetic Act (FD&C Act) and the Public Health Service Act (PHS Act)."

70. In addition, following the mid-cycle meeting, the FDA has multiple communications with the applicant concerning post-marketing commitments and requirements, such as additional studies, trials, or analyses the applicant may be required to perform following approval. Moreover, following the mid-cycle meeting, the FDA transmits discipline review letters, or comments on the application from members of the different review teams, to the applicant.

71. The FDA then conducts a "late-cycle" meeting with the applicant. According to FDA guidance, the "late-cycle meeting is intended to share information, identify deficiencies . . . and plan the rest of the review." Specifically, guidance provides that topics for discussion include "[m]ajor deficiencies identified to date" and "[i]nformation requests from the review team to the applicant,"

among others. That guidance further provides that individuals leading FDA review of the application attend this important meeting, including "the signatory authority for the application, review team members from appropriate disciplines, and appropriate team leaders and/or supervisors from disciplines for which substantive issues have been identified in the review to date." CBER guidance likewise explains that the late-cycle meeting is "held for applications subject to the PDUFA Program with the CBER Review Committee, CBER senior management, and the applicant to discuss the status of the review of the application late in the review cycle."

72. FDA guidance states that for applications, like the valrox BLA, that will not be discussed at a meeting of outside experts (called an Advisory Committee meeting), the late-cycle meeting "will generally occur not later than 3 months (standard review) or two months (priority review) prior to the PDUFA goal date," and that "[f]or expedited reviews that will not go to an [Advisory Committee]" – again, like the valrox BLA – "the meeting may be held earlier in the review cycle." In addition, in the case of an accelerated review, like valrox's, the FDA must deliver a "Late-Cycle Meeting Background Package" no later than two days before the meeting. The package includes a memorandum from the review team "outlining substantive application issues," including "deficiencies identified by primary and secondary reviews."

73. Thus, given the valrox BLA's August 21, 2020 PDUFA date, the FDA's late-cycle meeting with BioMarin would have occurred no later than June 21, 2020, and the Company would have received the Late-Cycle Meeting Background Package no later than June 19, 2020.

74. Finally, as noted above, one of the most critical steps in the approval process is the completion of the Preapproval Inspection of the facilities in which the new drug will be manufactured, including a review of all manufacturing controls and processes. Under the Food, Drug, and Cosmetic Act, the FDA may approve an application to market a new drug or therapy only if the methods used in, and the facilities and controls used for, the manufacture, processing, packing, and testing of the drug are found adequate, and ensure and preserve its identity, strength, quality, and purity. Likewise, FDA regulations state:

> A biologics license application shall be approved only upon examination of the product and upon a determination that the product complies with the standards established in the biologics license application and the requirements prescribed in the regulations in

> this chapter including but not limited to the good manufacturing practice requirements set forth in parts 210, 211, 600, 606, and 820 of this chapter.

75. As agency guidance explains, the FDA relies on Preapproval Inspections to assure itself that a manufacturing facility "named in a drug application is capable of manufacturing a drug, and that submitted data are accurate and complete," particularly, as in the case of the valrox BLA, if the manufacturing facility is new or the drug is a breakthrough therapy. As Fuchs explained to investors, a successful Preapproval Inspection of the valrox manufacturing facility was a "requirement" for approval of the Company's BLA. At a June 2019 investor conference, Fuchs stated,

> [W]e've had fairly clear dialogue with [the FDA] about requirements for registration. . . . . And then the last key criteria that they talked about was the CMC [Chemistry. Manufacturing, and Controls] package.[2] So for CMC considerations, we've completed our – what's called the PPQ [process performance qualification] campaign, which is we make material for the Phase III clinical trial, and then we make it again and resubmit that as part of the BLA.[3] And what they want to see, the agency, is that what you made for the clinical trials is represented in the PPQ material and then – and that you are ready for them to come to an inspection."

Again, as discussed above, questions concerning the role BioMarin's manufacturing processes and controls might have played in the disappointing interim Phase III valrox data made the Preapproval Inspection of BioMarin's Novato facility even more critical to approval.

76. FDA guidance provides that the agency will conduct Preapproval Inspections at least two months prior to the PDUFA date in order to leave sufficient time to "address any deficiencies" prior to the deadline for agency action. Indeed, the Preapproval Inspection is complex and thorough, and can take multiple weeks to complete. Moreover, once the Preapproval Inspection is complete, inspectors must compile a lengthy and detailed Establishment Inspection Report ("EIR"), which must be separately approved by the FDA Office of Compliance. And, as discussed above, any deficiencies identified in the EIR must be resolved before the agency can act on the BLA. Accordingly, the FDA's

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[2] The FDA requires applicants to submit a "Chemistry, Manufacturing, and Controls" package to demonstrate that a pharmaceutical or biologic has, among other things, consistent safety, quality, purity, and strength across product batches prior to the inspection.

[3] The PPQ requires the applicant to show objective measures that, in combination, the design of the manufacturing facility is appropriate, proper utility systems and equipment are being used and are operating within required specifications by properly trained personnel.

ability to approve a BLA by the PDUFA date deadline would be severely jeopardized were the Preapproval Inspection delayed beyond this timeframe.

77. Defendants repeatedly told investors throughout the Class Period that the FDA scheduled the Preapproval Inspection of BioMarin's Novato Facility to occur in the second quarter of 2020 and, as Defendants Bienaimé said on a May 14, 2020 investor call, "significantly before the PDUFA date."

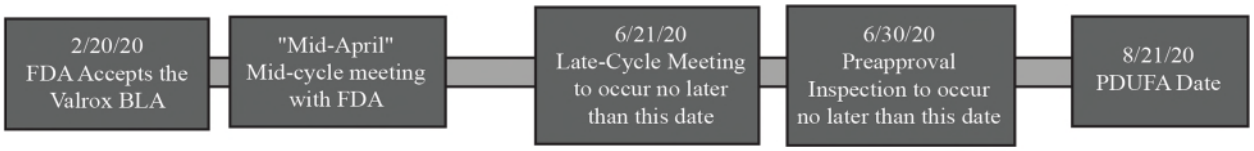

Figure 5. BioMarin's regulatory timeline for the valrox BLA.

F. The FDA Told Defendants Of Concerns About Valrox's Efficacy And Canceled The Valrox Preapproval Inspection

78. As discussed below and as Defendants ultimately ***admitted***, the FDA privately warned BioMarin that approval of the valrox BLA by the PDUFA date was highly unlikely. First, at the very start of the Class Period, the FDA warned BioMarin that the critical Preapproval Inspection would likely be delayed beyond the second quarter of 2020 raising serious concerns about the FDA's ability to approve the valrox BLA by the August deadline. Then, in June 2020 the FDA privately flagged to BioMarin the very same concerns about valrox's efficacy it later cited as the basis of its decision to deny the BLA and canceled the Preapproval Inspection the agency had told BioMarin was a condition of approval. Moreover, notwithstanding Defendants' statements that BioMarin was "working very closely" and "quite collaborative[ly]" with the FDA in connection with the valrox application, Defendants have now admitted the Company had had "no dialogue whatsoever" with the agency from mid-April 2020 to the end of the Class Period, apart from the deeply worrying June meeting. As a direct result of the FDA's silence, BioMarin was unable to address, or even discuss, key review milestones, such as labeling, with the FDA. Defendants' direct representations to the contrary, and their concrete assertions of fact about the progress of the valrox BLA review were materially false and misleading.

1. At the Start of the Class Period, The FDA Warned BioMarin That The Critical Valrox Preapproval Inspection Would Likely Be Delayed

79. According to a former BioMarin executive, the FDA warned BioMarin at the start of the Class Period that the Preapproval Inspection of the valrox facility would be delayed and that delay would likely extend beyond the second quarter of 2020. The FDA's warning and delay raised serious and significant questions about whether the FDA would be able to approve the valrox BLA by the August 21, 2020 PDUFA date.

80. The BioMarin Former Employee ("FE 1") was a senior business development executive from prior to the start of the Class Period to mid-2020. FE 1 stated that, in late February or early March of 2020, before COVID-19-related shutdowns became widespread, the FDA held a meeting with BioMarin personnel at which the agency provided "concrete feedback" concerning the Company's valrox BLA. Brinda Balakrishnan, Group Vice President of Corporate and Business Development and one of Bienaimé's direct reports, informed FE 1 that, during the pre-pandemic meeting, the FDA told BioMarin that the Preapproval Inspection of the Company's Novato facility would likely be delayed beyond the second quarter of 2020, seriously jeopardizing the agency's ability to approve valrox by the August 21, 2020 PDUFA date. FE 1 confirmed the Company's understanding that the inspection was a necessary condition for FDA approval of the valrox BLA.

81. FE 1 reported that as the PDUFA date drew closer, the FDA's failure to perform the Preapproval Inspection continued to generate anxiety inside BioMarin. FE 1's colleagues in both R&D and in manufacturing expressed concern, particularly after April 2020, that the FDA had failed to move the valrox CMC review forward. Indeed, FE 1 stated he was "very surprised" when Fuchs and Bienaimé told investors in August 2020 that the valrox BLA was on track when, in truth, the Preapproval Inspection had not occurred and the Company had received no subsequent communication from the FDA on this and other critical issues, including labeling.

82. Indeed, unbeknownst to investors, outside of the late-cycle meeting discussed below, BioMarin had no dialogue with the FDA from April 2020 until the agency issued its rejection of the valrox BLA in August 2020. As Defendant Fuchs later admitted at a September 16, 2020 investor conference, "So in April, we had the late – the mid-cycle meeting. It was our last meeting with anybody

and senior management at the FDA [present]. And from then until the receipt of the complete response letter, we had ***no dialogue whatsoever with the agency***," outside of the late-cycle meeting.

83. Defendants' own post-Class Period admissions – and analysts' stunned responses to those admissions – make clear that the FDA's silence following the mid-cycle meeting was highly unusual. For instance, at a September 10, 2020 investor conference Defendants admitted that until late August, BioMarin had only had a single meeting – the late-cycle meeting – with the FDA following the mid-cycle meeting. In response, a Citigroup analyst responded, "Wow. Got it. And you would assume that there would be more meetings with the Breakthrough Therapy designation typically, that's the case, right?" Fuchs admitted, "Yes. Yes." Indeed, as the Company explained, throughout valrox's development the Company was "meeting with the agency regularly" due to its "Breakthrough Therapy designation status" and that status "support[ed]" the Company's "frequent interactions with the[] health authorities," including the FDA.

84. Again, even in an ordinary review cycle, the FDA is supposed to have numerous critical reviews and communications with the applicant after the mid-cycle meeting, including multiple labeling reviews and transmission of draft labeling to the applicant; communications concerning post-marketing commitments and requirements; and transmission of discipline review letters. After the end of the Class Period, Defendants acknowledged that, because BioMarin had "no dialogue whatsoever" with the FDA during much of the review cycle, the Company was unable to address these key milestones and was only beginning to do so following the issuance of the CRL. For instance, at a September 16, 2020 investor conference, Fuchs stated, "So there has been no dialogue [with the FDA] . . . . And it's often in that dialogue that you can lay issues to rest and -- through [discussions about] labeling or post-approval studies, et cetera. So we're just going to be beginning that process, unfortunately, now after the CRL is received."

85. The FDA's sudden and highly unusual stonewalling of BioMarin was even more strange given valrox's Breakthrough Therapy designation, which, as discussed above, purportedly entailed "intensive" FDA communication with BioMarin during the BLA review process.

86. Accordingly, that BioMarin "had no dialogue whatsoever with the agency" following the mid-cycle meeting (outside of the highly negative communications discussed below), and that, as

a result, BioMarin was unable to address critical review milestones like labelling with the agency, thus raised the most glaring red flags that review of the valrox BLA was deeply troubled. Particularly given the agency's close dialogue with the Company prior to April of 2020, the FDA's sudden silence was highly unusual and the Company's inability to effectively communicate with FDA about the valrox BLA would have been deeply concerning to investors, had they been told the truth.

2. Despite The FDA's Warnings, Defendants Repeatedly Stated That The Valrox Preapproval Inspection Was Scheduled For the Second Quarter of 2020 and BioMarin was "Tracking To Our Milestones" To Meet The PDUFA Date

87. As discussed above, investors and analysts were deeply focused on whether the Preapproval Inspection would proceed in the second quarter of 2020 and sought confirmation that the valrox BLA was on track to meet the August 21, 2020 PDUFA date. The Company repeatedly assured investors of both.

88. On March 3, 2020, Defendant Bienaimé attended the Cowen HealthCare Conference and gave a presentation and speech to analysts and investors on the BLA, which Bienaimé announced had been accepted by the FDA two days earlier. Bienaimé assured analysts and investors that due to the strength of the Phase II data and the FDA's decision to grant accelerated review, the Company "anticipate[s] launching in the second half of the year." Bienaimé failed to disclose the FDA's warnings that the Preapproval Inspection would likely be delayed beyond the second quarter of 2020, thus substantially escalating the risk that FDA would not approve valrox by the PDUFA date.

89. Likewise, an analyst asked whether "there are any areas of major disagreement between BioMarin and the [FDA], whether it's in clinical data, clinical trial design, or manufacturing." Rather than disclose the FDA's decision to significantly delay the Preapproval Inspection, Bienaimé assured investors that the agency had raised no significant issues. Bienaimé stated that, "as of today, although [sic] we're having a substantial discussion going on. On the CMC front, there are some discussions, but we have been incorporating very closely with the FDA over the past 3 years. Actually, even when we design the manufacturing facility, we interacted with them. We've been interacting with them very closely on the development of in-process assay or release assay. So we believe we have a very good relationship with them." Defendants repeated these assurances during the Company's first quarter

2020 earnings call on April 29, 2020 with investors and analysts. Rather than disclose that valrox was facing a significant obstacle to approval because the FDA had significantly delayed the inspection it said was a precondition to thereto, Bienaimé stated that "[t]he confidence from the FDA is that we're tracking to our milestones and in some cases, they are accelerating their work. So having been through a bunch of these [approvals] recently, all the signs are pointing favorably."

90. Defendants Fuchs and Bienaimé continued to assure investors that the valrox PDUFA date was "holding firm" throughout May 2020. On May 14, 2020, Fuchs and Bienaimé attended the Bank of America Merrill Lynch Healthcare Conference and discussed valrox and the inspection. During the conference, an analyst from Bank of America Merrill Lynch asked, "from a manufacturing perspective, what gives you the confidence in the ultimate outcome here with respect to an inspection?" In response, Bienaimé reiterated that "the [Preapproval Inspection] by the FDA is scheduled significantly before the PDUFA date. So we're on track in this respect. I would say what makes us confident . . . . is based on all of our interactions we've had over the past 3 years or so, interacting with the FDA as we were building the plant, validating the plants, makes us already be confident that we're going to be in a good position with the FDA inspection." Defendant Fuchs also attended the May 19, 2020 RBC Global Healthcare Conference, where he emphasized that despite the ongoing COVID-19 pandemic, "[o]ur PDUFA action date of August 21 is holding firm. In spite of the pandemic, everything is going quite well, in review with the FDA."

91. The Company also touted the upcoming inspection in a May 31, 2020 press release stating "[t]he inspection of the facility by the FDA is expected to be complete during the second quarter, which would allow for potential licensure of the facility in the U.S. consistent with the August 21st PDUFA date."

92. In June 2020, Defendants assured investors that BioMarin had been in close contact with the FDA, and that the agency had reaffirmed that the BLA was on track for approval by the PDUFA date and, in particular that the second quarter 2020 inspection date held firm. During the June 4, 2020 Jefferies Healthcare Conference, attended by Defendants Fuchs and Bienaimé, an analyst noted that "[y]ou also mentioned that you guys are on track for FDA's inspection in second quarter, and we have less than a month left. So has the date been set up?" Fuchs responded "Yes. We have a

schedule. And in fact, ***the [FDA] has been quite collaborative***. . . .[T]he agency has ***signaled strongly that they intend to maintain their PDUFA action date***."

93. Likewise, at the June 9, 2020 Goldman Sachs Global Healthcare Conference, an analyst again asked Defendants to reaffirm that the all-important inspection was on schedule. Fuchs stated in response that the Company was in productive dialogue with the FDA, that the BLA was ahead of schedule, and that FDA had all the information it needed to approve the valrox application. Fuchs stated, "Well, we're in the middle of a mesh with the FDA. ***Any given day, it's like back and forth***. But what I would say is that in regards to major metrics, the agency is actually ahead of their PDUFA mandated regulatory metrics in terms of time line. So really, what remains are inspections and completion of reviews. They have all the information on file that they need to complete their reviews. And so ***we're working very closely with them to keep things on track***."

94. Remarkably, as discussed above and as Fuchs admitted after the Class Period, far from being "quite collaborative" and "strongly" signaling the Preapproval Inspection was on track, the FDA had "no dialogue whatsoever" with BioMarin since mid-April. Thus, at the time Defendants made these misleading assertions of past and present fact, BioMarin had encountered nearly two months of silence from the FDA at the very time that the Company should have been in active dialogue with the agency regarding issues essential to approval. As discussed, this silence was made all the more troubling by the fact that valrox's Breakthrough Therapy designation should have entailed exceptionally active communication with the FDA. Moreover, far from being ahead of schedule or having all the information needed to approve the BLA, the FDA had provided no assurances to BioMarin that it would forego the critical Preapproval Inspection that still had not taken place.

3. BioMarin Concealed the FDA's Warnings About The Risk to Valrox Approval While It Held A Critical Private Offering

95. At the same time that the Company concealed that FDA approval of the valrox application by the August 2020 PDUFA date was in serious jeopardy, the Company planned and completed a critical debt offering necessary to pay debts coming due imminently. On May 11, 2020, the Company announced a $550 million private debt offering at 1.25%, primarily to pay off the $375 million of BioMarin's 1.50% convertible notes that were due in full in October 2020. Defendants'

repeated assurances that valrox remained on schedule for approval were critical to BioMarin's ability to consummate the offering on favorable terms. BioMarin closed the private debt offering on May 19, 2020, with $585.8 million in net proceeds after initial purchasers exercised an option to purchase an additional $50 million of notes.

96. Both the May 2020 debt offering, and valrox's approval on the August 21, 2020 PDUFA date were of particular importance to BioMarin, given the projected negative impact of the COVID-19 pandemic on the Company's 2020 earnings, which created significant uncertainty for BioMarin's cashflow and ability to pay its October 2020 debt without significantly constraining its ability to finance development of the Company's all-important drug pipeline. As Defendant Bienaimé disclosed during the Company's April 29, 2020 first quarter 2020 earnings conference call, the Company expected "more meaningful business disruptions for the remainder of 2020 due to the pandemic."

4. In June 2020, The FDA Raised Concerns About Valrox's Efficacy In a Private Meeting With BioMarin and Canceled the Preapproval Inspection

97. In June 2020, the FDA provided additional warnings to BioMarin making clear that approval of the valrox BLA by the August 21, 2020 PDUFA date was highly improbable. As Defendants have now admitted, at BioMarin's late-cycle meeting with the FDA, and in the background package distributed to BioMarin prior to that meeting, the FDA privately raised with BioMarin the very same concerns that formed the basis of its decision denying the Company's BLA. Specifically, at that June 2020 meeting, the FDA flagged the disparity in efficacy between valrox's Phase I/II and Phase III trials, the possibility that the decline in factor activity in the Phase III study was driven by manufacturing differences, and questioned whether, based on the trajectory of Factor VIII activity observed in the Phase III study, the "gene therapy is going to wear off." Significantly, though, at the meeting, BioMarin sought reassurances from the FDA about the impact of its concerns on approval of the valrox BLA, the agency refused to provide any.

98. As Fuchs acknowledged at a September 9, 2020 investor conference, "During the late-cycle meeting, [FDA] observed this trajectory [in factor activity among valrox patients], the difference between the Phase II and the Phase III material, which like I said, that was – that meeting was in June . . . . And they first identified the issue in writing." Fuchs further admitted that, as discussed above,

despite BioMarin's urgent inquiries, the FDA never told BioMarin that the agency's concerns were resolved or would not impact the outcome of its review: "[W]e asked them, 'Where are you going with this?' And they said, 'Here's what we found.'"

99. Likewise, in response to analyst questions at a September 16, 2020 investor conference, Fuchs elaborated on the FDA's warnings at the June 2020 late-cycle meeting: "[I]n the middle of the process, [FDA] made note of the trajectory of the future of Factor VIII expression and they made note of thresholds. So for example, we said, they want to know, is there a – gene therapy is going to wear off. Is there a threshold level where a patient should revert to prophylactics therapy?" According to Fuchs, the FDA further stated at the late-cycle meeting, "given this trajectory, we are unable to establish that threshold" at which valrox will remain effective.

100. These warnings, including warnings that the FDA was unable "to establish a threshold" beyond which lowered factor activity (a surrogate endpoint) results in diminished clinical efficacy (such as bleeding), signaled serious agency concern with valrox's approvability. As discussed above, in January 2020, the FDA had issued guidance specifically concerning the approval of human gene therapies to treat hemophilia. The guidance states that "[a]pproval of GT products could be based on factor activity levels, if scientifically justified." The guidance advises "sponsors seeking accelerated approval based on factor activity levels," just like BioMarin, to "provide evidence, specific to their GT [gene therapy] product, that correlates the factor levels with relevant clinical outcomes." In other words, the guidance explains that in deciding whether to approve a drug based on the kind of data BioMarin submitted, the FDA will look to whether the sponsor has demonstrated a "threshold" above which increased factor activity translates into improved clinical outcomes. The fact that the FDA raised concerns about the absence of such data at, and prior to, the late-cycle meeting – and the fact that BioMarin sought reassurances from the FDA that these concerns did not threaten approval and the agency refused – was therefore highly significant.

101. Likewise, as discussed above, investors were focused on the possibility that BioMarin's manufacturing processes and controls contributed to the decreased factor activity observed in the Company's Phase III valrox data. Again, shortly after those results were released, the head of the FDA division responsible for reviewing the valrox BLA stated that the agency would carefully scrutinize

the adequacy of gene therapy manufacturing controls, specifically noting that manufactured material can behave differently than material prepared for clinical development. Accordingly, the fact that the FDA flagged "the difference between the Phase II and the Phase III material" at the late-cycle meeting – and, again, the fact that BioMarin sought reassurances from the FDA that these concerns did not threaten approval and the agency refused them – would also have been highly significant to investors.

102. As discussed above, BioMarin's late-cycle meeting would have occurred no later than June 21, 2020 – two months before the August 21, 2020 PDUFA date, under FDA guidelines. Indeed, "[f]or expedited reviews that will not go to an [Advisory Committee]," like the valrox review, "the meeting may be held earlier in the review cycle." In addition, the late-cycle meeting background package, in which Fuchs acknowledged the agency raised its concerns about valrox's efficacy, would have been provided to BioMarin no later than June 19, 2020, two days before the meeting.

103. After the end of the Class Period, Defendants further admitted that "at the same time" the FDA was raising concerns about valrox's efficacy with BioMarin – just prior to, and during, the June 2020 late cycle meeting – the agency told the Company it was no longer planning to conduct the Preapproval Inspection that was a condition of approval, and which Defendants had repeatedly assured investors would take place in the second quarter of 2020. While discussing BioMarin's late-cycle meeting with the FDA at the September 16, 2020 investor conference, Fuchs stated "at the same time, it's probably worth appreciating that they had communicated that they were going to inspect us, and then they changed their plans sensibly related to COVID . . . . [O]ur focus in this time [that the late-cycle meeting was occurring] was, well, they must have problems with inspection."[4] Defendants stated that BioMarin was "going crazy" and "scrambl[ing]" internally as a result of this cancellation. Again, as discussed above, Defendants understood that the valrox BLA could not be approved if the inspection did not occur. Fuchs further stated that while BioMarin attempted to get the FDA to accept

[4] While the reason the FDA canceled the Preapproval Inspection is irrelevant (since, whatever the reason, without the inspection, valrox could not be approved by the PDUFA date), the FDA did ***not*** tell BioMarin it had canceled the inspection because of COVID. As Defendants acknowledged after the Class Period, the FDA's CRL made clear that it had canceled the inspection because its concerns about valrox's efficacy precluded it from approving the drug, thus obviating the need for an inspection.

alternatives to a formal inspection, such as documentary submissions, it "could never get any traction on that discussion."

104. Despite the fact that the FDA had flagged concerns regarding valrox's efficacy, had refused to provide BioMarin with the reassurances it sought concerning the impact of those concerns on the outcome of the BLA, and that the Company was "going crazy" and "scrambl[ing]" internally as a result of the FDA's cancellation of the critical Preapproval Inspection, Defendants continued to assure investors that the valrox BLA was "on track." For instance, at a June 24, 2020 investor conference, an analyst asked Defendants whether, in connection with the valrox BLA, they "still expect to have an inspection in the first half of this year." In response, Fuchs stated, "Yes. That's going to – that's a good question to illuminate the thing that I said at the beginning, which is now that we're under 2 months away from PDUFA, I think our answer to these types of questions is going to be, ***we're on track for our PDUFA [date of] 8[/]21.***" Noting that approval of valrox by the PDUFA date has "got to be one of the most key questions that people have in their mind," Fuchs further assured investors that "we continue to anticipate approval of Roctavian [valrox] in the second half of the year based on the August 21 PDUFA action date." Fuchs' misleading factual assertions failed to disclose any of the issues discussed above, including that FDA had already canceled the critical Preapproval Inspection and that BioMarin could not "get any traction" on its efforts to push the agency to forego it.

105. Notably, at that same June 24 investor conference, Defendants suddenly told investors that they were "implement[ing] a lockdown on communication of our regulatory status to ensure that everyone has the same information at the same time, and I want to thank you for bearing with us." BioMarin's unexpected embargo on public disclosures concerning the valrox BLA – after months of positive commentary on the subject – was highly unusual and not mandated by any regulation.

106. Notwithstanding this "lockdown," at an August 13, 2020 investor conference, Fuchs touted valrox's lead over competing therapies developed by Roche/Spark and Pfizer/Sangamo. Fuchs stated, "I think [valrox] is generation one gene therapy. . . . Spark and Sangamo are generation 0.6 and 0.3, maybe even a higher degree of discount because ***they're so far behind***." Fuchs failed to disclose,

however, that, with the PDUFA date only a week away, the FDA had failed to perform the Preapproval Inspection, which, as Fuchs acknowledged, the FDA had told BioMarin was required for approval.

107. Analysts and market commentators trumpeted Fuchs' positive statements so close to the PDUFA date as highly encouraging. For instance, in an August 14, 2020 article, *Endpoints News* – a biotech financial media site – reported, "BioMarin CSO disses rivals for the hemophilia A gene therapy crown: Way behind." The reporters further noted, "[J]ust 7 days away from their PDUFA date, with high odds of success, the top execs clearly feel that they are way out front." These reporters were, of course, unaware of the truth: that the FDA had flagged serious concerns about valrox's efficacy months earlier, had canceled the critical Preapproval Inspection, and that the FDA had refused to provide any reassurances to BioMarin that neither its concerns about efficacy nor the absence of an inspection threatened approval.

108. In sum, while Defendants were repeatedly misleading investors that BioMarin was "working very closely" and "quite collaborative[ly]" with the FDA in connection with the valrox application, that the Company was meeting or ahead of approval milestones, and that the key manufacturing inspection would occur in the second quarter of 2020, the FDA had, in truth, had "***no dialogue whatsoever***" with BioMarin from mid-April on outside of the June late-cycle meeting, at which the agency flagged serious concerns about valrox's efficacy and cancelled the necessary Preapproval Inspection.

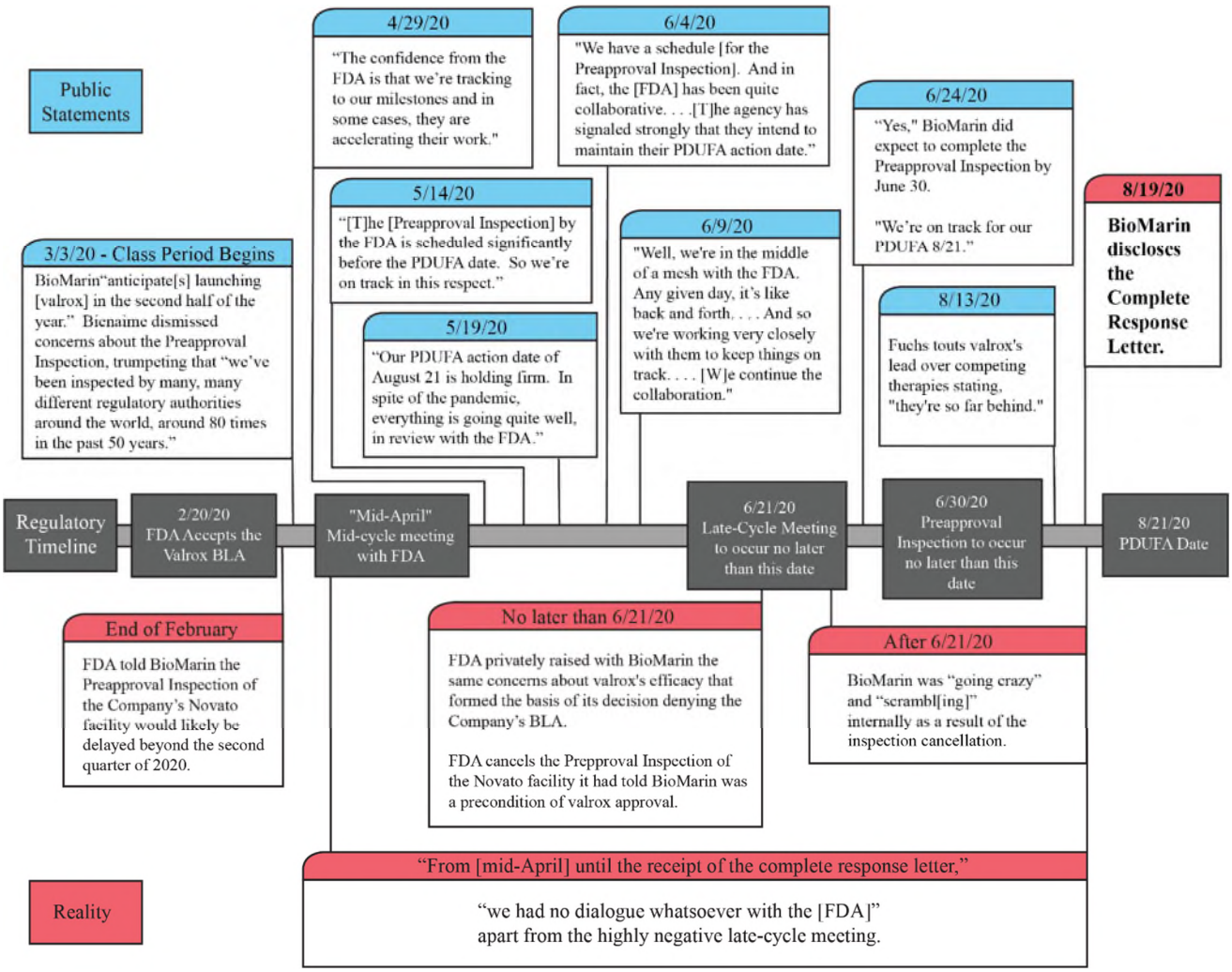

Figure 6. A selection of Defendants' false and misleading statements compared to the undisclosed reality within BioMarin.

G. Defendants Took Advantage of BioMarin's Inflated Share Price to Dump Millions of Dollars' Worth of Company Stock

109. At the same time that Defendants Bienaimé and Fuchs were issuing materially misleading statements to investors, they took advantage of BioMarin's inflated stock price to sell more than $30 million dollars' worth of their BioMarin holdings during the Class Period. By withholding news of the FDA's warnings, failure to engage with BioMarin, and cancellation of the Preapproval Inspection, Bienaimé and Fuchs avoided substantial losses on their insider sales.

110. Significantly, Defendant Fuchs sold ***$19.5 million*** worth of BioMarin stock – amounting to 59% of his holdings (and comprising 85% of all of his Class Period sales) – in just two transactions on July 13 (23,011 shares for $130) and July 20, 2020 (126,389 shares at $130), just weeks

after the FDA raised concerns about valrox's efficacy at the Company's late-cycle meeting and canceled the critical Preapproval Inspection. Delaying disclosure of this highly negative news until after his pre-planned July 2020 sales benefitted Fuchs enormously; had that negative information been disclosed just a few weeks earlier, Fuchs' proceeds would have been slashed significantly. Notably, Fuchs purportedly executed these trades pursuant to a 10b5-1 trading plan executed on March 11, 2020, ***after*** the FDA warned BioMarin that the Preapproval Inspection would likely be delayed beyond the second quarter of 2020, severely jeopardizing timely approval of the valrox BLA.

111. Defendants' insider trades were highly unusual and departed from their historical trading patterns. During the Class Period, Defendant Fuchs sold 64% of his BioMarin holdings for proceeds of nearly $23 million. During the six months that preceded the six-month Class Period ("Control Period"), Fuchs did not sell a single share of BioMarin stock. Additionally, Fuchs made no open market purchases of BioMarin stock during the Class Period. Likewise, Bienaimé sold more than 89,000 shares of BioMarin stock, or 23% of his holdings, for proceeds of more than $8.2 million, compared with Control Period-sales of 48,000 shares (13% of his holdings), for proceeds of less than $4 million.

	Shares Sold During Class Period	**Shares Sold During Control Period**	**Percentage of Holdings Sold During Class Period**	**Percentage of Shares Sold During Control Period**	**Class Period Sales Proceeds**	**Control Period Sales Proceeds**
Fuchs	186,613	None	64%	No sales	$22,888,787	$0
Bienaimé	89,000	48,000	23%	13%	$8,223,920	$3,945,500

Figure 7. Defendants' insider sales during the Class Period were significant and inconsistent with their pre-Class Period trading.

H. The Truth Is Finally Revealed

112. As the FDA's Friday, August 21, 2020 deadline to act on the valrox BLA under PDUFA approached, analysts eagerly awaited the news of the FDA's approval and were already incorporating the approval—which was expected, based on management's repeated assurances—into their valuation of the Company. For example, Credit Suisse wrote in an August 19, 2020 note (released before the

Company's statements later that day) that "BioMarin's [valrox] is poised to become the first approved hemophilia A gene therapy." Credit Suisse further wrote that "investors have a positive bias going into this event," and that "approval is widely expected," based on, among other things, "positive management commentary."

113. On August 19, 2020, before the market opened, BioMarin shocked the market by announcing receipt of a Complete Response Letter ("CRL") from the FDA to the Company's BLA. The FDA sends a CRL when it has completed its review of a new drug application, and it has decided it will not approve it for sale in its current form. A CRL will provide deficiencies with the drug application that the manufacturer must address before the FDA will review the application again.

114. In its press release disclosing the CRL, BioMarin stated that 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." 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.

115. Analysts were "shocked" and "surprised" by this news and immediately raised questions concerning management's credibility. For example, RBC Capital Markets wrote in an August 19, 2020 note that the CRL "comes as a massive surprise & changes our prior thesis." RBC Capital Markets further wrote that the massive stock price decline following the news left BioMarin shares "fairly valued," given, among other things, "increased mgmt credibility concerns." RBC analysts described the FDA's CRL as ***"a massive surprise," particularly in light of the Company's recent "positive commentary on regulatory process."*** The firm went on to write that it saw "limited upside [in a BioMarin investment] given that ***there could be mgmt credibility concerns***." Similarly, analysts at Piper Sandler characterized the FDA's decision to issue a CRL as a "major surprise" and were "shocked by this development as ***management, as recently as last week during our virtual West Coast bus tour, was uber bullish regarding [valrox]'s commercial prospects***, saying 2021 consensus was 'not heroic,'" *i.e.*, not ambitious enough. Moreover, a JPMorgan analyst questioned whether the Company had been fully transparent with investors regarding its discussions with the FDA. Specifically, the analyst noted that "[u]nder [breakthrough therapy designation], ***we would assume***

that [BioMarin] would have been aware of this data deficiency well before the week of the [FDA's August 21, 2020 action date]."

116. Notably, in investor conferences during the fall of 2020, securities analysts pressed Defendants to describe the rationale the FDA had articulated in the CRL driving its denial of the valrox BLA. At a September 16, 2020 investor conference, Defendant Fuchs responded to one such inquiry by explaining, "Well, what they told us was that the differences in the Factor VIII expression at weeks 23 to 26 between the Phase II and the Phase III study precluded them from projecting the durability – sorry, the trajectory of Factor VIII expression and precluded them from demonstrating a threshold level above which you need to be to achieve bleeding. So that's what they said was the underlying mechanism." Likewise, at a November 16 investor conference, Fuchs explained, "When we got the CRL, we conveyed what was the substantial element of the CRL, which was that the difference in the 2 trials, the Phase II and the Phase III trial resulted in differences in Factor VIII activity at an initial time point. And because of other differences in the trial, how they were conducted, the starting materials for the Phase I/II trial versus the Phase III trial and the use of corticosteroids[.]" As discussed above, Defendants' responses make clear that the FDA had already flagged those very concerns with BioMarin at the June late-cycle meeting, months earlier.

V. DEFENDANTS' MATERIALLY FALSE AND MISLEADING STATEMENTS DURING THE CLASS PERIOD

117. As discussed above, throughout the Class Period Defendants made numerous false and misleading statements and omissions about the progress of the FDA's review of the valrox BLA. Defendants' false and misleading statements and omissions are set forth below.

A. Cowen HealthCare Conference (March 3, 2020)

118. On March 3, 2020, Defendant Bienaimé attended the Cowen HealthCare Conference on behalf of BioMarin. During the conference, Bienaimé stated that the Company "anticipate[s] launching" valrox "in the second half of [the] year."

119. At that Cowen investor conference, an analyst asked Bienaimé whether "there are any areas of major disagreement between BioMarin and the agency, whether it's in clinical data, clinical trial design or manufacturing[?]" Bienaimé responded, "On the CMC front, there are some

discussions, but we have been incorporating very closely with the FDA over the past 3 years. Actually, even when we design the manufacturing facility, we interacted with them. We've been interacting with them very closely on the development of in-process assay or release assay. So we believe we have a very good relationship with them."

120. Defendant Bienaimé's statements were materially misleading when made and omitted material facts necessary to make the statements not misleading. It was misleading for Defendants to state, for example, that the Company "anticipate[s] launching" valrox "in the second half of [the] year" and assuring investors that the FDA had not raised any "major" issues as part of its BLA review while failing to disclose that the FDA had informed Defendants that it was delaying the Preapproval Inspection and that the delay likely would extend beyond the second quarter of 2020, seriously jeopardizing approval of the valrox BLA by the August 2020 PDUFA date.

121. Analysts credited Bienaimé's statements. In a March 3, 2020 report, Cowen and Company maintained its "Outperform" rating for BioMarin stock and specifically noted that "Management appears quite confident in the FDA approval of valrox on or around its 8/21 PDUFA date." The report also highlighted that "BMRN notes that a manufacturing inspection of the valrox plant has already been planned, and is confident that manufacturing will support approval. BioMarin has worked with the FDA on the design of valrox's facility and process controls, and in fact noted that it has designed 50 or 60 assays for process validation with input of the Agency."

B. First-Quarter 2020 Earnings Conference Call (April 29, 2020)

122. On April 29, 2020, the Company held its first quarter 2020 earnings conference call with analysts and investors. During the call, a SunTrust analyst asked Defendants why they were confident "that there won't be any more delays" on U.S. approval of valrox. Defendant Fuchs responded, "The confidence from the FDA is that we're tracking to our milestones and in some cases, they are accelerating their work. So having been through a bunch of these [approvals] recently, all the signs are pointing favorably."

123. Defendant Fuchs' statements were materially false and misleading when made and omitted material facts necessary to make the statements not misleading. It was misleading for Fuchs to state, for example, that BioMarin and FDA were either "tracking to our milestones" for the BLA

review or accelerating them, while failing to disclose that valrox was facing a significant obstacle to approval because the FDA had informed Defendants that it was delaying the Preapproval Inspection and that the delay likely would extend beyond the second quarter of 2020, seriously jeopardizing approval of the BLA by the PDUFA date.

124. Analysts credited Fuchs' statements. For example, in an April 29, 2020 report, Guggenheim Partners stated, "Our key takeaways from the company's 1Q call were 1) [valrox] is on track for its Aug PDUFA with the FDA[.]" In another report published that day, Cowen and Company maintained its "Outperform" rating on BioMarin stock and noted that "[valrox] is on track for an approval on its Aug. PDUFA and a pre-approval site inspection by the FDA is expected to be conducted during Q2." The report further noted that "[w]e also think it likely that [valrox] will outpace consensus estimates. . . . [T]here will be a proportion of Hem A patients who will 'cue up' as they think gene therapy is the 'holy grail.'" Credit Suisse maintained its "Outperform" rating, noting that "US regulators plan to inspect the manufacturing facility" for valrox "in Q2'20 and based on recent interactions with the FDA, US approval timelines appear on track." Piper Sandler also maintained its "Overweight" rating and noted "the all-important US valrox August approval remains on track (PDUFA 8/21/20)."

C. Bank of America Merrill Lynch Healthcare Conference (May 14, 2020)

125. On May 14, 2020, Defendants Bienaimé and Fuchs attended the Bank of America Merrill Lynch Healthcare Conference on behalf of BioMarin. At the conference, a Bank of America Merrill Lynch analyst asked Bienaimé and Fuchs "where [the Company is] with respect to CMC[,] [a]nd from a manufacturing perspective, what gives you confidence in the ultimate outcome here with respect to an inspection? Maybe just help us with the inspection and the manufacturing facility." Bienaimé responded that "***the inspection by the FDA is scheduled significantly before the PDUFA date. So we're on track in this respect***. I would say what makes us confident . . . . is based on all our interactions we've had over the past 3 years or so, interacting with the FDA as we were building the plant, validating the plants, makes us already confident that we're going to be in a good position with the FDA inspection."

126. Defendant Bienaimé's statements were materially false and misleading when made and omitted material facts necessary to make the statements not misleading. It was misleading for Bienaimé to state, for example, that "the inspection by the FDA is scheduled significantly before the PDUFA date. So we're on track in this respect," while failing to disclose that valrox was facing a significant obstacle to approval because the FDA had informed Defendants that it was delaying the Preapproval Inspection and that the delay likely would extend beyond the second quarter of 2020, seriously jeopardizing approval of the valrox BLA by the August 2020 PDUFA date. Likewise, it was misleading for Defendants to tout BioMarin's extensive interactions with the FDA in connection with the valrox BLA, while failing to disclose that, deeply troublingly, the Company had "had no dialogue whatsoever with the [FDA]" concerning the valrox BLA for weeks, and, as a result, BioMarin was unable to address critical issues essential to approval with the agency, including valrox's labelling.

D. RBC Global Healthcare Conference (May 19, 2020)

127. On May 19, 2020, Defendant Fuchs attended the RBC Global Healthcare Conference on BioMarin's behalf. Fuchs stated that "***Our PDUFA action date of August 21 is holding firm. In spite of the pandemic, everything is going quite well, in review with the FDA***."

128. Defendant Fuchs' statements were materially false and misleading when made and omitted material facts necessary to make the statements not misleading. It was misleading for Fuchs to state that "[o]ur PDUFA action date of August 21 is holding firm" and that "everything is going quite well, in review with the FDA," when, in truth, (1) the FDA had informed Defendants that it was delaying the Preapproval Inspection and that the delay likely would extend beyond the second quarter of 2020, seriously jeopardizing approval of the valrox BLA by the August 2020 PDUFA date; and (2) deeply troublingly, the Company had "had no dialogue whatsoever with the [FDA]" concerning the valrox BLA for weeks, and, as a result, BioMarin was unable to address critical issues essential to approval with the agency, including valrox's labelling.

129. Analysts credited the statements. In a May 19, 2020 report, RBC Capital Markets highlighted that "On [valrox], mgmt. reiterated the regulatory review of [valrox] for hemophilia A remains on track (August 21 PDUFA)."

E. BioMarin Press Release (May 31, 2020)

130. In a May 31, 2020, BioMarin press release, Defendants stated, "The inspection of the [Novato] [F]acility by FDA is expected to be completed during the second quarter, which would allow for potential licensure of the facility in the U.S. consistent with the August 21st PDUFA date."

131. Defendants' statements were materially false and misleading when made and omitted material facts necessary to make the statements not misleading. It was misleading for Defendants to state that "[t]he inspection of the facility by FDA is expected to be completed during the second quarter," when, in truth, (1) the FDA had informed Defendants that it was delaying the Preapproval Inspection and that the delay likely would extend beyond the second quarter of 2020, seriously jeopardizing approval of the valrox BLA by the August 2020 PDUFA date; and (2) deeply troublingly, the Company had "had no dialogue whatsoever with the [FDA]" concerning the valrox BLA for weeks, and, as a result, BioMarin was unable to address critical issues essential to approval with the agency, including valrox's labelling.

F. Jefferies Healthcare Conference (June 4, 2020)

132. On June 4, 2020, Defendants Fuchs and Bienaimé attended the Jefferies Healthcare Conference on behalf of BioMarin. A Jefferies analyst asked "[y]ou also mentioned that you guys are on track for FDA's inspection [for the valrox BLA] in second quarter, and we have less than a month left. So has the date been set up?" Fuchs stated "Yes. We have a schedule. And in fact, the agency has been quite collaborative . . . . [T]he agency has signaled strongly that they intend to maintain their PDUFA action date."

133. Defendant Fuchs' statements were materially false and misleading when made and omitted material facts necessary to make the statements not misleading. It was misleading for Defendants to state that the FDA had scheduled the valrox Preapproval Inspection for the second quarter of 2020 and had "signaled strongly that [it] intend[ed] to maintain" the PDUFA date, when, in truth, the FDA had informed Defendants that it was delaying the Preapproval Inspection and that the delay likely would extend beyond the second quarter of 2020, seriously jeopardizing approval of the valrox BLA by the August 2020 PDUFA date. It was additionally misleading for Defendants to reassure investors that the FDA had "been quite collaborative" and had "signaled strongly that [it]

intend[ed] to maintain [the] PDUFA" date for the valrox BLA, while failing to disclose that, deeply troublingly, the Company had "had no dialogue whatsoever with the [FDA]" concerning the valrox BLA for over a month, and, as a result, BioMarin was unable to address critical issues essential to approval with the agency, including valrox's labelling.

134. Analysts credited the false and misleading statements. In a June 4, 2020 report, Jefferies noted "FDA inspection for manufacturing facility has been scheduled; document review has begun; consistent with timely approval by PDUFA date (8/21/20)." Jefferies rated BioMarin stock a "Buy."

G. Goldman Sachs Global Healthcare Conference (June 9, 2020)

135. On June 9, 2020, Defendants Fuchs and Bienaimé attended the Goldman Sachs Global Healthcare Conference on BioMarin's behalf. At the conference, a Goldman Sachs analyst asked Defendants, "[A]s you near this August 21 PDUFA date [for the valrox BLA], could you just comment on what gating factors are – still are there ahead of the PDUFA and commercialization?" Defendant Fuchs responded, "***Well, we're in the middle of a mesh with the FDA. Any given day, it's like back and forth. But what I would say is that in regards to major metrics, the agency is actually ahead of their PDUFA mandated regulatory metrics in terms of time line***. So really, what remains are inspections and completion of reviews. They have all the information on file that they need to complete their reviews. ***And so we're working very closely with them to keep things on track***. . . . [W]e continue the collaboration."

136. Defendant Fuchs' statements were materially false and misleading when made and omitted material facts necessary to make the statements not misleading. It was misleading for Defendants to state that BioMarin was "in the middle of a mesh with the FDA," that "[a]ny given day, it's like back and forth," and that the Company was "working very closely with them to keep things on track," when, in truth, and deeply troublingly, the Company had "had no dialogue whatsoever with the [FDA]" concerning the valrox BLA for over a month and, as a result, BioMarin was unable to address critical issues essential to approval with the agency, including valrox's labelling. It was additionally misleading for Defendants to state that "the agency is actually ahead of their PDUFA mandated regulatory metrics in terms of time line," while failing to disclose that the FDA had informed

Defendants that it was delaying the Preapproval Inspection and that the delay likely would extend beyond the second quarter of 2020, seriously jeopardizing approval of the valrox BLA by the August 2020 PDUFA date.

H. Bank of America Merrill Lynch Napa Healthcare Conference (June 24, 2020)

137. On June 24, 2020, Defendant Fuchs attended the Bank of America Merrill Lynch Napa Healthcare Conference on behalf of BioMarin. At the conference, a Bank of America Merrill Lynch analyst asked Defendants, "can you comment at all if you still expect to have an inspection in the first half of this year?" Defendant Fuchs responded, "Yes. That's going to – that's a good question to illuminate the thing that I said at the beginning, which is now that we're under 2 months away from PDUFA, I think our answer to these types of questions is going to be, we're on track for our PDUFA [date of] 8[/]21."

138. Also at the June 24, 2020 Defendant Fuchs stated "We're so pleased with the clinical benefit demonstrated with [valrox] that it continues to be durable at the 4 years and counting . . . . So we continue to anticipate approval of [valrox] in the second half of this year based on the August 21 PDUFA action date."

139. Additionally, Defendant Fuchs stated, "And I think the hemostatic efficacy even at low factor level statement under – and some level doesn't do enough justice to the profundity of the clinical benefit that, that patient – those patients who are even in that [lower factor] range, still experiencing profound benefits many years later. And those are our lowest patients. And so I think that's a pretty comforting thing as to think about the fact that the expectations for a clinical benefit are really, can be fairly large."

140. Defendants Fuchs' statements were materially false and misleading when made and omitted material facts necessary to make the statements not misleading. It was misleading for Defendants to state that "[y]es," the Company still expected to have an inspection in the first half of the year, that "we're on track for our PDUFA 8[/]21," and that "we continue to anticipate approval of [valrox] in the second half of this year based on the August 21 PDUFA action date," when, in truth, BioMarin had already received additional warnings that approval of the valrox BLA by the August 21,

2020 PDUFA date was in serious jeopardy, including: (1) at the private late-cycle meeting between the FDA and BioMarin, and in the pre-meeting memorandum the agency provided to BioMarin, the FDA flagged concerns about valrox's efficacy, including the decline in Factor VIII activity among valrox patients observed in the Phase III data; (2) while BioMarin sought reassurances from the FDA that these concerns would not affect approval, the agency refused to provide them; (3) the FDA canceled the Preapproval Inspection that was a condition of approval; and (4) while BioMarin requested that the FDA accept alternatives to this critical inspection, Defendants acknowledged after the Class Period that BioMarin "could never get any traction on that discussion" and BioMarin was "going crazy" and "scrambl[ing]" internally as a result of this cancellation. It was additionally misleading for Defendants to state that BioMarin was on "on track for our PDUFA" date, while failing to disclose that, deeply troublingly, the Company had "had no dialogue whatsoever with the [FDA]" concerning the valrox BLA since the mid-cycle meeting – apart from the dismal late cycle meeting and cancellation of the Preapproval Inspection – and, as a result, BioMarin was unable to address critical issues essential to approval with the agency, including valrox's labelling.

141. It was also false and misleading for Defendant Fuchs to tout the durability of valrox's supposed benefit and to state that "the clinical benefit demonstrated with [valrox is] that it continues to be durable at the 4 years and counting" that the "patients who are even in that [lower factor] range, still experiencing profound benefits many years later," and that "the expectations for a clinical benefit are really, can be fairly large," while failing to disclose that, at the private late-cycle meeting between the FDA and BioMarin, and in the pre-meeting memorandum the agency provided to BioMarin, the FDA specifically flagged concerns about the durability of any benefit valrox might confer, including the decline in Factor VIII activity among valrox patients observed in the Phase III data, BioMarin's inability to establish a threshold level beyond which lowered factor activity results in diminished clinical efficacy, and questioned whether, based on the trajectory of Factor VIII activity observed in the Phase III study, the "gene therapy is going to wear off."

I. BioMarin Press Release (August 4, 2020)

142. On August 4, 2020, the Company issued a press release titled "BioMarin Announces Second Quarter 2020 Total Revenue Growth of 11% to $430 Million." In the August 4, 2020 press

release, Defendant Bienaimé is quoted as stating, "With the outcome of the Priority Review of our [valrox] BLA anticipated August 21, 2020, our commercial team is preparing to launch what we believe is the most innovative product yet for people with bleeding disorders."

143. Defendant Bienaimé's statements were materially false and misleading when made and omitted material information necessary to make them not misleading. It was misleading for Defendants to state that BioMarin anticipated a positive "outcome of the Priority Review of our [valrox] BLA" and that the Company was "preparing to launch" valrox while failing to disclose that BioMarin had already received additional warnings that approval of the valrox BLA by the August 21, 2020 PDUFA date was in serious jeopardy, including: (1) at the private late-cycle meeting between the FDA and BioMarin, and in the pre-meeting memorandum the agency provided to BioMarin, the FDA flagged concerns about valrox's efficacy, including the decline in Factor VIII activity among valrox patients observed in the Phase III data; (2) while BioMarin sought reassurances from the FDA that these concerns would not affect approval, the agency refused to provide them; (3) the FDA canceled the Preapproval Inspection the agency had told BioMarin was a condition of approval; and (4) while BioMarin requested that the FDA accept alternatives to this critical inspection, Defendants acknowledged after the Class Period that BioMarin "could never get any traction on that discussion" and BioMarin was "going crazy" and "scrambl[ing]" internally as a result of this cancellation; and (5) with the valrox PDUFA date just two weeks away, the FDA had still not performed the critical Preapproval Inspection and had refused to accept any substitutes for an in-person inspection, notwithstanding BioMarin's pleas for an alternatives. It was additionally misleading for Defendants to state that BioMarin was "preparing to launch" valrox, while failing to disclose that, deeply troublingly, the Company had "had no dialogue whatsoever with the [FDA]" concerning the valrox BLA since the mid-cycle meeting – apart from the dismal late cycle meeting and cancellation of the Preapproval Inspection – and, as a result, BioMarin was unable to address critical issues essential to approval with the agency, including valrox's labelling.

J. Canaccord Genuity Growth Conference (August 13, 2020)

144. On August 13, 2020, Defendant Fuchs attended the Canaccord Genuity Growth Conference on behalf of BioMarin. At that investor conference Fuchs touted valrox's lead over

competing therapies developed by Roche/Spark and Pfizer/Sangamo. Fuchs stated, "I think [valrox] is generation one gene therapy. . . . Spark and Sangamo are generation 0.6 and 0.3, maybe even a higher degree of discount because ***they're so far behind***."

145. Defendant Fuchs' statements were materially false and misleading when made and omitted material information necessary to make them not misleading. It was misleading for Defendant Fuchs to tout valrox's lead over competing therapies and state that those competing therapies are "so far behind" valrox, while failing to disclose that BioMarin had already received additional warnings that approval of the valrox BLA by the August 21, 2020 PDUFA date was in serious jeopardy, including: (1) at the private late-cycle meeting between the FDA and BioMarin, and in the pre-meeting memorandum the agency provided to BioMarin, the FDA flagged concerns about valrox's efficacy, including the decline in Factor VIII activity among valrox patients observed in the Phase III data; (2) while BioMarin sought reassurances from the FDA that these concerns would not affect approval, the agency refused to provide them; (3) the FDA canceled the Preapproval Inspection that was a condition of approval; (4) while BioMarin requested that the FDA accept alternatives to this critical inspection, Defendants acknowledged after the Class Period that BioMarin "could never get any traction on that discussion" and BioMarin was "going crazy" and "scrambl[ing]" internally as a result of this cancellation; and (5) with the valrox PDUFA date just one week away, the FDA had still not performed the critical Preapproval Inspection and had refused to accept any substitutes for an in-person inspection, notwithstanding BioMarin's pleas for an alternatives. It was additionally misleading for Defendant Fuchs to tout valrox's lead over competing therapies, while failing to disclose that, deeply troublingly, the Company had "had no dialogue whatsoever with the [FDA]" concerning the valrox BLA since the mid-cycle meeting – apart from the dismal late cycle meeting and cancellation of the Preapproval Inspection – and, as a result, BioMarin was unable to address critical issues essential to approval with the agency, including valrox's labelling.

VI. SUMMARY OF SCIENTER ALLEGATIONS

146. Numerous allegations set forth above and summarized below give rise to the strong inference that Defendants intentionally or recklessly misled investors about BioMarin's valrox BLA. These allegations include, but are not limited to, the following:

147. ***First***, Fuchs' and Bienaime's post-Class Period admission make clear they received warnings directly from the FDA indicating approval of the valrox BLA by the August 2021 PDUFA was in serious jeopardy. Defendants have admitted that at the FDA's late-cycle meeting with BioMarin (one of two major agency meetings held during the BLA review cycle), and in the pre-meeting memorandum delivered to the Company, the agency raised concerns about the valrox efficacy data, specifically flagging the significant decline in factor activity observed in BioMarin's Phase III data, the concern that the decline was driven by BioMarin's commercial manufacturing processes, and questioned whether, based on the trajectory of factor activity observed in the Phase III study, the "gene therapy is going to wear off." Defendant Fuchs stated that, at that meeting, he and his colleagues sought reassurances from FDA about the impact of these observations on valrox's BLA but the agency refused to provide any such reassurances. Moreover, Defendants have further admitted that, at the same time that the FDA raised these warnings, it also told BioMarin it was canceling the Preapproval Inspection it had told the Company was necessary to secure approval of the valrox BLA. Defendants' admissions thus establish that at the same time they were telling investors that, for instance, "we're on track for our PDUFA [date of] 8[/]21," they were aware of highly material facts making approval of the valrox BLA by the August 21, 2020 PDUFA date highly improbable. Likewise, the FDA told BioMarin in February or early March, that the valrox Preapproval Inspection would likely be delayed beyond the second quarter 2020 timeframe, severely jeopardizing approval of the Company's BLA by the August 2020 deadline. Accordingly, Defendants' admissions concerning both the FDA's affirmative warnings give rise to a strong inference of scienter.

148. ***Second***, Defendants have also admitted that from the time of the mid-April mid-cycle meeting until it received the CRL in late August, BioMarin "had no dialogue whatsoever with the agency," outside of the late-cycle meeting. Accordingly, Defendants' admissions establish that at the same time they were responding to ***direct analyst inquiries*** by reassuring investors that BioMarin was maintaining a close dialogue with the FDA – that the agency "has been quite collaborative," that BioMarin was "working very closely with them to keep things on track," and "was in the middle of a mesh with the FDA" – the Company had in truth had "no dialogue whatsoever with the agency" for nearly two months. Importantly, as a result, BioMarin was unable to address critical issues essential

to approval with the agency, including valrox's labelling, and missed significant approval milestones. Again, Defendants' admissions concerning the FDA's troubling silence give rise to a strong inference of scienter.

149. ***Third***, that Defendants' misstatements concerned the most significant events relating to one of BioMarin's most significant products supports a strong inference of scienter, particularly as BioMarin was a relatively small company with management focused on valrox's approval in 2020. As of the start of the Class Period, BioMarin's portfolio included only six FDA-approved drugs and four drugs in development. Vosoritide had completed clinical trials but was not yet submitted for FDA approval and likely will not be approved until 2021 or later, BMN 307 had just received approval to begin clinical trials, and BMN 331 was in and remains in a pre-clinical stage.

150. As alleged above, Defendants told investors prior to and during the Class Period that valrox was poised to be the first gene therapy marketed for treatment of severe hemophilia, and as a first mover in this relatively large market, "the value that valrox represents is really substantial and ***transformative for BioMarin***." Indeed, Defendants stated that even if valrox penetrated only a third of the hemophilia market, the drug would ***double*** BioMarin's ***Company-wide*** revenue. Valrox's enormous revenue potential was particularly significant for BioMarin, as its launch was slated to coincide with Kuvan's, which accounted for nearly 30% of the Company's revenue, loss of patent exclusivity in October 2020. Thus, BioMarin's Fall 2020 launch of valrox was particularly significant for the Company since it was expected to offset a meaningful hit to its revenue. And, of course, the FDA's review of the valrox BLA was critical to valrox's commercial success, and therefore, one of the most critical issues facing BioMarin during the Class Period.

151. Given valrox's importance to BioMarin, Defendants Bienamé and Fuchs discussed the drug at ***every*** investor conference and on ***every*** earnings call during, and for months before, the Class Period. Indeed, as alleged above, Defendants gave detailed, data-laden responses to analyst questions about the valrox BLA, the Company's interactions with the FDA, and valrox's clinical data. For instance, at a March 3, 2020 investor conference, Defendant Bienaimé provided a detailed account of BioMarin's discussions with the FDA on specific aspects of the valrox BLA dealing with manufacturing, such as "the development of in-process assay or release assay." Notably, following

BioMarin's disastrous late-cycle meeting with the FDA, Defendants suddenly refused to provide detailed responses to specific analyst questions concerning the valrox BLA, but nevertheless continued to assure investors that the application was "on track" and that BioMarin's competition was "far behind."

152. ***Fourth***, while Plaintiff need not allege any motive to plead scienter, that Defendants sold millions of dollars' worth of BioMarin stock following the FDA's warnings, including the Company's highly negative late-cycle meeting with the FDA and after the agency's cancellation of the critical Preapproval Inspection further support an inference of scienter. During the Class Period, as negative facts about the valrox BLA accumulated, Defendant Fuchs sold approximately 64% of his BioMarin holdings, reaping proceeds of nearly ***$22.9 million***. Significantly, Defendant Fuchs sold ***$19.5 million*** worth of BioMarin stock – amounting to 59% of his holdings and ***85%*** of his Class Period sales – in just two transactions on July 13 and July 20, 2020, just weeks after the FDA raised concerns about valrox's efficacy at the Company's late-cycle meeting and canceled the critical Preapproval Inspection. Delaying disclosure of this highly negative news until after his pre-planned July 2020 sales benefitted Fuchs enormously; had those sales been executed just a few weeks later – after the market learned that valrox would not be approved by the August 21 PDUFA date – Fuchs' proceeds would have been slashed by more than a third. Fuchs' trading during the Class Period was highly unusual, departing from his historical trading patterns: during the six months preceding the start of the six-month Class Period (the "Control Period"), Fuchs did not sell ***a single share*** of BioMarin stock. Additionally, Fuchs made no open market purchases of BioMarin stock during the Class Period.

153. Likewise, Defendant Bienaimé sold 89,000 shares of BioMarin stock, 23% of his BioMarin holdings during the Class Period, for proceeds of more than $8.2 million. Like Defendant Fuchs, Bienaimé's sales were inconsistent with his prior trading patterns. Bienaimé's sales during the Control Period were approximately half his Class Period sales: during the Control Period, he sold 48,000 shares of BioMarin stock, 13% of his holdings, for proceeds of under $4 million.

154. ***Fifth***, while Plaintiff need not allege any motive to plead scienter, that BioMarin was conducting a $600 million May 2020 private offering to raise capital needed to pay off $375 million in notes due in October 2020 provided a powerful incentive for Defendants to conceal the FDA's

warnings that approval of the valrox BLA was in jeopardy. Delaying disclosure of negative news about BioMarin's most important drug allowed Defendants to raise nearly $600 million through the May 2020 private offering on favorable terms. As alleged above, the negative impact of the COVID-19 pandemic on BioMarin's earnings significantly heightened the importance of the May 2020 offering, providing a powerful motive for Defendants to conceal the truth.

VII. LOSS CAUSATION

155. Lead Plaintiff incorporates by reference the allegations set forth above. During the Class Period, Defendants publicly disseminated materially false and misleading statements and omitted material facts concerning the Company's operations and its true financial condition.

156. The material misrepresentations and omissions included issues concerning (i) the FDA's Preapproval Inspection of the Company's Novato Facility necessary for the valrox BLA approval and (ii) the likelihood of the FDA's approval of the valrox BLA on the August 21, 2020 PDUFA date. The conduct alleged and the materially false and misleading statements and omissions made during the Class Period caused BioMarin's common stock to trade at artificially inflated prices, closing as high as $131.03 per share during the Class Period, operating as a fraud on investors in the Company's common stock.

157. When the truth was disclosed on August 19, 2020, BioMarin's stock price declined significantly as the artificial inflation as removed from the stock price.

158. On August 19, 2020, during pre-market hours, BioMarin issued a press release titled "BioMarin Receives Complete Response Letter (CRL) from FDA in Valoctocogene Roxaparvovec Gene Therapy for Severe Hemophilia A." The press release disclosed that the receipt of the CRL meant that the valrox BLA "application is complete and that the application is not ready for approval in its present form." In its press release disclosing the CRL, BioMarin stated that 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."

159. On this news, the price of Company's common stock fell $41.82 per share, or 35.28%, to close at $76.72 per share on August 19, 2020, on a high trading volume of 34 million shares.

160. On the same day, multiple analysts published reports explaining the significance of the disclosure. In an August 19, 2020 report, RBC Capital Markets wrote that "We see BMRN's [valrox] CRL as thesis-changing," reduced the stock's price target from $123 to $92 and downgrading it from "Outperform." RBC Capital Markets noted "[t]his complete response letter comes as a near-complete surprise to us only days ahead of the August 21 PDUFA and after positive commentary on regulatory progress at our Virtual Healthcare Conference, their recent [valrox]/HemeA KOL webinar & their recent Q2 financial results call. . . . [I]t dramatically changes our [valrox]-centric bull thesis." The report further noted that the CRL "increased mgmt credibility concerns."

161. It was entirely foreseeable that Defendants' materially false and misleading statements and omissions discussed herein would artificially inflate the price of BioMarin's stock. It also was foreseeable to Defendants that the revelation of the truth concerning the valrox BLA delay would cause the price of the Company's stock price to fall as the artificial inflation caused by Defendants' misstatements and omissions was removed. Thus, the stock price declines described above were directly and proximately caused by Defendants' materially false and misleading statements and omissions.

VIII. CLASS ACTION ALLEGATIONS

162. Lead Plaintiff brings this action as a class action pursuant to Rule 23 of the Federal Rules of Civil Procedure on behalf of all persons who purchased the publicly traded common stock of BioMarin during the Class Period (the "Class"). Excluded from the Class are Defendants and their families, directors, and officers of BioMarin and their families and affiliates.

163. The members of the Class are so numerous that joinder of all members is impracticable. The disposition of their claims in a class action will provide substantial benefits to the parties and the Court. As of October 23, 2020, BioMarin had over 181 million shares of common stock outstanding, owned by numerous investors.

164. There is a well-defined community of interest in the questions of law and fact involved in this case. Questions of law and fact are common to the members of the Class, which predominate over questions which may affect individual Class members, including:

(a) Whether Defendants violated the Exchange Act;

(b) Whether Defendants omitted and/or misrepresented material facts;

(c) Whether Defendants' statements omitted material facts necessary in order to make the statements made, in light of the circumstances under which they were made, not misleading;

(d) Whether the Individual Defendants are personally liable for the alleged misrepresentations and omissions described herein;

(e) Whether Defendants knew or recklessly disregarded that their statements and/or omissions were false and misleading;

(f) Whether Defendants' conduct impacted the price of BioMarin common stock;

(g) Whether Defendants' conduct caused the members of the Class to sustain damages; and

(h) The extent of damage sustained by Class members and the appropriate measure of damages.

165. Lead Plaintiff's claims are typical of those of the Class because Lead Plaintiff and the Class sustained damages from Defendants' wrongful conduct.

166. Lead Plaintiff will adequately protect the interests of the Class and has retained counsel experienced in class action securities litigation. Lead Plaintiff has no interests that conflict with those of the Class.

167. A class action is superior to other available methods for the fair and efficient adjudication of this controversy. Joinder of all Class members is impracticable.

IX. INAPPLICABILITY OF STATUTORY SAFE HARBOR

168. None of Defendants' alleged false and misleading Class Period statements were forward-looking. Instead, Defendants made concrete misstatements concerning past or present facts, including misstatement concerning the steps that the Company and the FDA were purportedly undertaking at the time those misstatements were made, which were necessary to receive a decision by the August 21, 2020 PDUFA date.

169. To the extent any of Defendants' alleged false and misleading Class Period statements are considered forward-looking (which they should not be), BioMarin's "Safe Harbor" warnings accompanying those statements were ineffective to shield those statements from liability.

170. Defendants are also liable for any false or misleading forward-looking statements pleaded herein because, at the time each such statement was made, the speaker knew the statement was false or misleading and the statement was authorized and/or approved by an executive officer of BioMarin who knew that the statement was false.

X. PRESUMPTION OF RELIANCE

171. At all relevant times, the market for BioMarin's common stock was an efficient market for the following reasons, among others:

(a) BioMarin common stock met the requirements for listing, and was listed and actively traded on the NASDAQ, a highly efficient and automated market;

(b) As a regulated issuer, BioMarin filed periodic public reports with the SEC and the NASDAQ;

(c) BioMarin regularly and publicly communicated with investors via established market communication mechanisms, including through regular disseminations of press releases on the national circuits of major newswire services and through other wide-ranging public disclosures, such as communications with the financial press and other similar reporting services; and

(d) BioMarin was followed by several securities analysts employed by major brokerage firm(s) who wrote reports which were distributed to the sales force and certain customers of their respective brokerage firm(s). Each of these reports was publicly available and entered the public marketplace.

172. As a result of the foregoing, the market for BioMarin common stock promptly digested current information regarding BioMarin from all publicly available sources and reflected such information in the price of BioMarin common stock. Under these circumstances, all purchasers of BioMarin common stock during the Class Period suffered similar injury through their purchase of BioMarin common stock at artificially inflated prices and the presumption of reliance applies.

173. A Class-wide presumption of reliance is also appropriate in this action under the Supreme Court's holding in *Affiliated Ute Citizens of Utah v. United States*, 406 U.S. 128 (1972), because the Class' claims are grounded on Defendants' material omissions. Because this action involves Defendants' failure to disclose material adverse information regarding BioMarin business and operations—information that Defendants were obligated to disclose—positive proof of reliance is not a prerequisite to recovery. All that is necessary is that the facts withheld be material in the sense that a reasonable investor might have considered them important in making investment decisions.

Given the importance of the FDA's denial of expedited approval for valrox and the impact that could have on the Company's future revenue growth, that requirement is satisfied here.

COUNT I

For Violations of Section 10(b) of the Exchange Act and Rule 10b-5 Against Defendants BioMarin, Bienaimé, and Fuchs

174. Lead Plaintiff repeats and realleges each and every allegation contained above as if fully set forth herein.

175. During the Class Period, Defendants carried out a plan, scheme, and course of conduct which was intended to and, throughout the Class Period, did: (i) deceive the investing public, including Lead Plaintiff and other Class members, as alleged herein; and (ii) cause Lead Plaintiff and other members of the Class to purchase BioMarin common stock at artificially inflated prices.

176. Defendants: (i) employed devices, schemes, and artifices to defraud; (ii) made untrue statements of material fact and/or omitted to state material facts necessary to make the statements not misleading; and (iii) engaged in acts, practices, and a course of business which operated as a fraud and deceit upon the purchasers of the Company's common stock in an effort to maintain artificially high market prices for BioMarin common stock in violation of Section 10(b) of the Exchange Act and Rule 10b-5, promulgated thereunder.

177. Defendants, individually and in concert, directly and indirectly, by the use, means or instrumentalities of interstate commerce and/or of the mails, engaged and participated in a continuous course of conduct to conceal adverse material information about the Company's financial well-being, operations, and prospects.

178. During the Class Period, Defendants made the false statements specified above, which they knew or recklessly disregarded to be false and misleading in that they contained misrepresentations and failed to disclose material facts necessary in order to make the statements made, in light of the circumstances under which they were made, not misleading.

179. Defendants had actual knowledge of the misrepresentations and omissions of material fact set forth herein, or recklessly disregarded the true facts that were available to them. Defendants

engaged in this misconduct to conceal BioMarin's true condition from the investing public and to support the artificially inflated prices of the Company's common stock.

180. Lead Plaintiff and the Class have suffered damages in that, in reliance on the integrity of the market, they paid artificially inflated prices for BioMarin's common stock. Lead Plaintiff and the Class would not have purchased the Company's common stock at the prices they paid, or at all, had they been aware that the market prices for BioMarin's common stock had been artificially inflated by Defendants' fraudulent course of conduct.

181. As a direct and proximate result of Defendants' wrongful conduct, Lead Plaintiff and the other members of the Class suffered damages in connection with their respective purchases of the Company's common stock during the Class Period.

182. By virtue of the foregoing, Defendants violated Section 10(b) of the Exchange Act and Rule 10b-5, promulgated thereunder.

COUNT II

For Violations of Section 20(a) of the Exchange Act Against Defendants Bienaimé and Fuchs

183. Lead Plaintiff repeats, incorporates, and realleges each and every allegation set forth above as if fully set forth herein.

184. The Individual Defendants acted as controlling persons of BioMarin within the meaning of Section 20(a) of the Exchange Act. By virtue of their high-level positions, participation in and/or awareness of the Company's operations, direct involvement in the day-to-day operations of the Company, and/or intimate knowledge of the Company's actual performance, and their power to control public statements about BioMarin, the Individual Defendants had the power and ability to control the actions of BioMarin and its employees. By reason of such conduct, the Individual Defendants are liable pursuant to Section 20(a) of the Exchange Act.

PRAYER FOR RELIEF

185. WHEREFORE, Lead Plaintiff prays for judgment as follows:

A. Determining that this action is a proper class action under Rule 23 of the Federal Rules of Civil Procedure;

B. Awarding compensatory damages in favor of Lead Plaintiff and other Class members against all Defendants, jointly and severally, for all damages sustained as a result of Defendants' wrongdoing, in an amount to be proven at trial, including interest thereon;

C. Awarding Lead Plaintiff and the Class their reasonable costs and expenses incurred in this action, including attorneys' fees and expert fees; and

D. Awarding such equitable/injunctive or other further relief as the Court may deem just and proper.

JURY DEMAND

186. Lead Plaintiff demands a trial by jury.

Dated: February 22, 2021

Respectfully submitted,

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