UNITED STATES DISTRICT COURT
NORTHERN DISTRICT OF CALIFORNIA

STEVEN BAILEY, Individually and on Behalf of All Others Similarly Situated, Plaintiff, v. ULTRAGENYX PHARMACEUTICAL INC., EMIL D. KAKKIS, and ERIC CROMBEZ, Defendants.	Case No. 26-cv-1097 CLASS ACTION **COMPLAINT FOR VIOLATIONS OF THE FEDERAL SECURITIES LAWS** DEMAND FOR JURY TRIAL

Plaintiff Steven Bailey ("Plaintiff"), individually and on behalf of all other persons similarly situated, by his undersigned attorneys, alleges in this Complaint for violations of the federal securities laws (the "Complaint") the following based upon knowledge with respect to his own acts, and upon facts obtained through an investigation conducted by his counsel, which included, *inter alia*: (a) review and analysis of relevant filings made by Ultragenyx Pharmaceutical Inc. ("Ultragenyx" or the "Company") with the United States Securities and Exchange Commission (the "SEC"); (b) review and analysis of Ultragenyx's public documents, conference calls, press releases, and stock chart; (c) review and analysis of securities analysts' reports and advisories concerning the Company; and (d) information readily obtainable on the internet.

Plaintiff believes that further substantial evidentiary support will exist for the allegations set forth herein after a reasonable opportunity for discovery. Most of the facts supporting the allegations contained herein are known only to the defendants or are exclusively within their control.

NATURE OF THE ACTION

1. This is a federal securities class action on behalf of all investors who purchased or otherwise acquired Ultragenyx common stock between August 3, 2023, to December 26, 2025, inclusive (the "Class Period"), seeking to recover damages caused by Defendants' violations of the federal securities laws (the "Class").

2. Defendants provided investors with material information concerning Ultragenyx's expected results for its Phase III Orbit and Cosmic Studies, which tested setrusumab (UX 143) in patients with Osteogenesis Imperfecta ("OI"). Defendants' statements included, among other things, confidence in setrusumab's ability to ultimately trigger a decrease in the OI patients' annualized fracture rate, alongside confidence in the study designs to demonstrate such ability and reduce testing variability that could interfere with such a result.

3. Defendants provided these overwhelmingly positive statements to investors while, at the same time, disseminating materially false and misleading statements and/or concealing material adverse facts concerning the true state of setrusumab's potential and the true risk inherent in the study protocols put forth; notably, that, while setrusumab does increase material bone

density, this increase does not correlate to a decrease in annualized fracture rates or otherwise the Phase III Orbit and Cosmic studies were much less likely to be able to demonstrate such a link than management claimed. Such statements absent these material facts caused Plaintiff and other shareholders to purchase Ultragenyx's securities at artificially inflated prices.

4. Investors began to question the veracity of Defendants' public statements on July 9, 2025, following Ultragenyx's press release which informed investors that the Phase III Orbit study failed to achieve statistical significance for the second interim analysis. In pertinent part, Defendants announced the Phase III Orbit and Cosmic studies would now be "progressing toward final analysis."

5. Investors and analysts reacted immediately to Ultragenyx's revelation. The price of Ultragenyx's common stock declined dramatically. From a closing market price of $41.44 per share on July 9, 2025, Ultragenyx's stock price fell to $31.03 per share on July 10, 2025, a decline of about 25.12% in the span of just a single day.

6. Notwithstanding the July 9 disclosures, Ultragenyx and the Individual Defendants continued to mislead investors. Defendants continued to create the false impression that they possessed reliable information pertaining to the success of the Phase III Orbit and Cosmic Studies, while also minimizing the risk from study variability. Defendants repeatedly insisted they were confident that setrusumab's ability to increase material bone density would necessarily translate to a reduction in the annualized fracture rate of the type 1, 3, or 4 OI patients and, further, remained confident in the study design created to facilitate the ability to detect the difference in fracture rate between the treatment and control populations.

7. On December 29, 2025, the full truth emerged. Ultragenyx announced that both its Phase III Orbit and Cosmic Studies had not "achieved statistical significance against the primary endpoints of reduction in annualized clinical fracture rate compared to placebo or bisphosphonates, respectively." The Company attributed the study failure to a "low fracture rate in the placebo group" of Orbit and a trend that fell shy of statistical significance in Cosmic.

8. Investors and analysts reacted immediately to Ultragenyx's revelation. The price of Ultragenyx's common stock declined dramatically. From a closing market price of $34.19 per

share on December 26, 2025, Ultragenyx's stock price fell to $19.72 per share on December 29, 2025, a decline of about 42.32% in the span of just a single day.

JURISDICTION AND VENUE

9. Plaintiff brings this action, on behalf of himself and other similarly situated investors, to recover losses sustained in connection with Defendants' fraud.

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

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

12. Venue is proper in this District pursuant to §27 of the Exchange Act and 28 U.S.C. §1391(b), as Defendant Ultragenyx is headquartered in this District and a significant portion of its business, actions, and the subsequent damages to Plaintiff and the Class, took place within this District.

13. In connection with the acts, conduct and other wrongs alleged in this Complaint, Defendants, directly or indirectly, used the means and instrumentalities of interstate commerce, including but not limited to, the United States mail, interstate telephone communications and the facilities of the national securities exchange.

THE PARTIES

14. Plaintiff purchased Ultragenyx common stock at artificially inflated prices during the Class Period and was damaged upon the revelation of the Defendants' fraud. Plaintiff's certification evidencing his transaction(s) in Ultragenyx is attached hereto.

15. Ultragenyx Pharmaceutical Inc. is a California corporation with its principal executive offices located at 60 Leveroni Court, Novato, CA 94949. During the Class Period, the Company's common stock traded on the NASDAQ Stock Market (the "NASDAQ") under the symbol "RARE."

16. Defendant Emil D. Kakkis ("Kakkis") was, at all relevant times, the Founder, President, Chief Executive Officer, and Director of Ultragenyx.

17. Defendant Eric Crombez ("Crombez") was, at all relevant times, the Chief Medical Officer and Executive Vice President of Ultragenyx.

18. Defendants Kakkis and Crombez are sometimes referred to herein as the "Individual Defendants." Ultragenyx together with the Individual Defendants are referred to herein as the "Defendants."

19. The Individual Defendants, because of their positions with the Company, possessed the power and authority to control the contents of Ultragenyx's reports to the SEC, press releases, and presentations to securities analysts, money and portfolio managers, and institutional investors, *i.e.*, the market. Each Individual Defendant was provided with copies of the Company's reports and press releases alleged herein to be misleading prior to, or shortly after, their issuance and had the ability and opportunity to prevent their issuance or cause them to be corrected. Because of their positions and access to material non-public information available to them, each of these Individual Defendants knew that the adverse facts specified herein had not been disclosed to, and were being concealed from, the public, and that the positive representations which were being made were then materially false and/or misleading. The Individual Defendants are liable for the false statements pleaded herein, as those statements were each "group-published" information, the result of the collective actions of the Individual Defendants.

20. Ultragenyx is liable for the acts of the Individual Defendants, and its employees under the doctrine of respondeat superior and common law principles of agency as all the wrongful acts complained of herein were carried out within the scope of their employment with authorization.

21. The scienter of the Individual Defendants, and other employees and agents of the Company are similarly imputed to Ultragenyx under respondeat superior and agency principles.

SUBSTANTIVE ALLEGATIONS

A. Company Background

22. Ultragenyx is a biopharmaceutical company focused on rare and ultrarare genetic disorders. The Company's product candidates are typically in-licensed from partnerships or academic institutions.

23. Pertinently, Ultragenyx is testing setrusumab a.k.a. UX143 for the treatment of Osteogenesis Imperfecta ("OI"). Ultragenyx has proceeded to Phase III analysis through its Orbit and Cosmic studies, evaluating setrusumab's ability to reduce patients' annualized fracture rate ("AFR").

B. The Defendants Materially Misled Investors Concerning the Phase III Orbit and Cosmic Studies for Setrusumab in Patients with Osteogenesis Imperfecta.

August 3, 2023

24. On August 3, 2023, Defendants conducted an earnings call corresponding to the release of their second quarter fiscal 2023 results. In pertinent part, Defendant Kakkis confidently claimed that if their drug, setrusumab or UX143, could continue to show bone density growth, it would resultantly correlate to reduced AFR, stating:

> I'll spend a few minutes discussing the osteogenesis imperfecta . . . program . . . In June, we reported exciting data from the Phase II dose-finding portion of the pivotal Orbit study, showing statistically significant increase in levels of serum P1NP, a sensitive marker bone formation.
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> The bone production response to these patients was extraordinary. This led to a rapid bone-building effect following just 3 months of treatment with setrusumab, resulting in nearly 10% in lumbar bone mineral density. At baseline, these patients had very limited bone mineral density, with an average Z-score in the 20 mg cohort of minus 2.12, which means the bone mineral density were 2 standard deviations below the mean of normal patients for their age.
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> After 3 months on therapy, the mean Z-score increased by plus 0.65 points, resolving nearly 1/3 of the deficit from normal in a relatively short period of time. As we've said before, ***patients are showing meaningful improvements in bone health***, and we are highly encouraged with how they're doing. ***Improved bone health refers to the instance of fractures***, bone pain and relative global health and activity of the patients.
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> . . .
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> In July, we announced that we initiated dosing patients in 2 Phase III studies evaluating setrusumab in 2 different age groups. ***The Phase III portion of the pivotal Orbit study is evaluating the effect of setrusumab compared to placebo on annualized clinical fracture rate in patients 5 to 25 years old.***
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> ***The newly initiated Phase III Cosmic study is an active controlled study evaluating setrusumab compared to IV bisphosphonate therapy on annualized total fracturated patients aged 2 to 5 years old***. Enrollment in both of these studies

> is going well so far in part because the Phase II data has generated a lot of excitement for the potential setrusumab for both the clinical sites and from the patient community.

(Emphasis added).

25. During the question-and-answer segment of the call, Defendant Kakkis spoke directly to Ultragenyx's confidence in the Phase III Orbit and Cosmic studies (the "Phase III OI Studies") during the following pertinent exchange:

> <Q: Liisa Ann Bayko – Evercore ISI Institutional Equities – Manging Director & Fundamental Research Analyst> Can you just give us a sense of kind of your level of confidence about what you're seeing in the OI program and how the changes in bone mineral density relate to potential changes you might see in fracture?
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> <A: Emil D. Kakkis> We have a ***high level of confidence that the magnitude of bone mineral density we saw at 3 months was already sufficient enough to improve the strength of bones and probably reduce fractures at that level we saw at 3 months in***. So we have high confidence in the fact that bone mineral density will be improved by this mechanism, anti-sclerostin mechanism, where you're getting anabolism or production of new bone will translate into fracture improvements.
>
> And we've talked about the nonclinical data in the past, but we'll be able to talk more about this at the October Analyst Day to provide that support, ***but we have a high level of confidence that the BMD produced by an anti-sclerostin like setrusumab will translate into fracture reduction***.
>
> <Q: Liisa Ann Bayko> Just as a follow-up on that. Can you explain to me the amount of sort of the bone mineral density levels of OI patients? How do they relate to those of osteoporosis patients? Because I'm just trying to kind of relate the 2 changes, and the amount of changes you're seeing to what we -- outcomes we've seen in osteoporosis. It seems to me maybe that bone mineral density levels are slightly different. Can you expand on that at all?
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> <A: Emil D. Kakkis> Well, sure, Liisa. What we said for this population, this study, is that the mean bone mineral density was minus 2.12, which means 2 standard relatable mean of normal people. Now osteoporosis patients have reduced bone mineral density. I don't have for you exact comparisons to put forth.
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> But I would say the mean of minus 2 standard deviations is pretty low on the bone scale. And if you look at the range, we had patients as low as minus 4 standard deviation. So these patients have, I think, a more severe on average bone mineral density problem than an average osteoporosis patient would, and therefore, have more need of bone production.

> ***What has been the misunderstanding is everyone thought that the defect in the collagen was why the bones are fracturing. What we're kind of trying to say is actually, while that may be a factor, it's in fact the effect of that mutation on bone production appears to be a bigger factor. And that's something we can change with setrusumab, and that's why we think we're going to have an important effect on OI.***

(Emphasis added).

November 2, 2023

26. On November 2, 2023, Defendants conducted an earnings call corresponding to their third quarter fiscal 2023 results. During the call, Defendant Crombez discussed Ultragenyx's ORBIT Phase II results and how such learnings could impact Phase III, stating, in pertinent part:

> Importantly, a subset of 5- to 12-year olds saw nearly a 20% increase in bone mineral density with a Z-score change of 1.19. ***These improvements in bone mineral density across the 24 patients treated in the ORBIT Phase II translated to a 67% reduction in the annualized fracture rate following treatment with setrusumab for at least 6 months***. 20 of the 24 patients did not experience any new fractures in the 6 months following treatment with setrusumab.
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> For the 4 who did have a radiographically confirmed fracture, many of them occurred early on in treatment or had a traumatic precipitating event. The data is all the more compelling because many of the patients in this study were previously treated with phosphonates over the 2 years prior to dosing with setrusumab. During this time, these patients continued to see a high annualized fracture rate with many fractures occurring with very minimal activity. These types of fractures are referred to as fragility fractures and examples include fractures occurring during sleep or when transferring out of a chair.
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> What we heard from 2 principal investigators who joined us at Analyst Day is that ***they are not seeing fragility fractures in these studies, patients treated with setrusumab and that many of these kids are now feeling strong enough to engage in more physical activities with friends and family.***

(Emphasis added).

27. A question-and-answer segment again followed the Company's prepared remarks, during which Defendant Kakkis highlighted the lack of concern investors should have over increased activity during and post-treatment on the analysis of setrusumab during the following pertinent exchange:

> <Q: Dae Gon Ha – Stifel, Nicolaus & Company, Inc. – Research Analyst> Two, maybe on GTX-102 and the setrusumab. Just wanted to clarify on setrusumab,

> Emil, did you say enrollment completion in 1Q '24? Is that for both ORBIT as well as COSMIC? And are you placing any protocol restrictions on strenuous activity? I mean it's encouraging their being more active and fearless but in terms of endpoints, I wonder if that could kind of create a confounder . . .
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> <A: Emil D. Kakkis> Very good, Dae Gon. So for setrusumab, we're talking about both ORBIT and COSMIC in terms of finishing enrollment. I think we're likely. But the main one we're talking about is ORBIT, which is the main driver. And I believe both of them should get done in that time frame. And in terms of this control of excise [sic] or the hazard risk, if someone is feeling better and exercising, well, that's already what's happening in Phase II. ***People were a lot more active and what was, actually, on the plus side is that they were active and a lot of them where they were falling and had fractures unnecessarily. So while there is some risk that they might be doing more, there was one person who played volleyball, that they hadn't been playing.***
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> We're actually -- ***overall feeling is that the pattern of having falls and fractures seems to be better***. And so our ***net effect overall, as we think even with increased activity, there will be a reduction in fractures***, which is really the best thing possible, that is, a kid is going to be active and to have a reduction of fractures while being active. So w***e're not so worried about the, let's say, the noise of having more fracture risk at this point. It looks like you still see the effect well, even if there is some risk there***.

(Emphasis added).

May 2, 2024

28. On May 2, 2024, Defendants published their first quarter fiscal 2024 results. During the corresponding earnings call, Defendant Kakkis briefly discussed the planned interim analysis for the Orbit and Cosmic studies, noting, in pertinent part, the following:

> For the UX143 Phase 3 portion of the Orbit study, there are 2 interim analyses planned with the first anticipated by the year-end or early 2025. The first analysis will have a stringent threshold of p less than or equal to 001. If the threshold is not met, a second interim analyses will occur a few months later, followed by a final analysis at 18 months.
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> Interim analyses will not report to the company by the Data Monitoring Committee unless they are positive. In the event of a positive interim analyses, we would share that outcome, but top line results will not be announced immediately as the study would require patients to complete a final visit and time to collect and prepare the data for a formal analysis.

29. Following the Company's prepared remarks, Defendants Kakkis and Crombez fielded a question concerning the risk factors of the Phase III OI Studies. Pertinently, Defendants

addressed how they planned the study to control for fracture rate in order to give the studies the optimal chance to achieve the designated AFR end points:

> <Q: Kristen Brianne Klusta – Cantor Fitzgerald & Co. – Analyst> We often get asked about setting expectations for the first interim readouts for setrusumab. Could you please help us frame what are some of the factors that are controllable that we can kind of help to predict in advance? And then some of the items where we're less sure about. And again, how to help frame these 2 expectations?
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> <A: Emil D. Kakkis> Well, I think our Phase 2 data kind of lay down what I think we're going to expect. I would expect it to be -- ***that a reduction would be very similar to what we've seen, if not better***. So we found there with only a minimum of 6 months, an average of 9 months exposure, 67% reduction. The patients we're enrolling are very comfortable with that. ***If anything, enrolled patients might have a higher fracture rate, I think. And so we would expect that reduction to be something that you'd expect to see***. Those are -- I don't know if you consider -- when you enroll patients with fractures, it's not exactly controllable. They are who they are. ***But because we have a threshold requirement to get in the trial, we're essentially eliminating patients who would have very low fracture rates and wouldn't necessarily be able to demonstrate benefit in that period of time.***
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> I think with the type of patients enrolled, the number of which type, ***I think we've set ourselves up to replicate what we saw before. And I really don't see any uncontrolled factors***. I don't know, Eric, if you have anything.
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> <A: Eric Crombez> Yes, definitely. I mean, ***I think the biggest controllable factor was really enrollment rate and the studies are fully enrolled***. Yes, we know the types of patients, and it was good to get a good mix of 1, 3 and 4 in there. I would say, yes, I would agree, ***the uncontrollable factor may be***, especially when you're first initiating treatment ***in the first couple of months, you may have some patients arriving with fractures before, setrusumab really takes effect there***. But there is a degree of unpredictability with fractures.
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> <A: Emil D. Kakkis> It's probably also the fact that some people -- ***some of the kids like feel really good and are getting more active. People are worried maybe that caused more fractures, but it didn't look like that was true or that they felt they didn't fracture***. So ***we actually are not concerned about the fact that his [sic] might feel good and start being more active. It doesn't look like it's going to cause a problem that looks like their bones are stronger and they're doing great.***
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> So the ***truth is that more activity probably strengthens the bone faster because the action actually puts strain in the bone, the bone actually are strengthened by that actual action.*** So thank you for the question.

(Emphasis added).

August 1, 2024

30. On August 1, 2024, Defendants presented their second quarter results for the year and again spoke to the issue of being able to show a reduction of AFR in their Phase III OI Studies. In pertinent part, the following exchanges occurred during the question-and-answer segment of the call:

> <Q: Salveen Jaswal Richter – Goldman Sachs Group, Inc. – Vice President> Could you help us understand how you look on OI with regard -- how you look at OI with regard to the Phase II data translating to Phase III here? And particularly, as the patients see improvements, how that kind of impacts the rate of fractures here for the population, and your assumptions around that in the Phase III trial?
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> <A: Emil D. Kakkis> Well, I think what we've shown at the 14-month data was, in fact, that the bone marrow density continues to increase dramatically. And the p-value got much smaller. So remember, that's looking at all the patients, not just the median, but it tells you -- ***the p-value declining substantially tells you that all the patients are moving toward a reduction in fractures***. So ***we feel that the effect is very large***.
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> In terms of translating to Phase III, we know from the data we had in a few placebos that they do not see bone marrow density improvement during this period of time. So there will be no placebo effect from that.
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> With regard to fractures, ***fractures are dependent on both disease severity and also environmental factors like what the patient is doing. Our expectation is that patients, when they feel better, could start doing more work, but what we have seen is patients that have gotten stronger and have been on treatment for a longer period of time will have falls and not have fractures. So we feel pretty confident that the strength of bones as such to even compensate for any change might occur because patients are more active***. But we do think that the way patients feel their activity will bode well for supportive clinical data and how the patients are doing, which I think will support the value of the product and its clinical meaning for this.
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> . . .
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> <Q: Kristen Brianne Klusta – Cantor Fitzgerald & Co. – Analyst> Congrats on a great quarter. On setrusumab, I wanted to ask if you think that there are any benefits this drug could potentially show as it relates to the pain these patients experience. Is there a reason to think that both reducing those fractures and putting down better bone has the potential to have an impact on pain?
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> <A: Emil D. Kakkis> Yes. Our impression from the Phase II patients, particularly with their increased activity, they're feeling better. They're having less pain. And while we look -- talk about fractures all the time, OI patients have weak bones. And what that means is lots of microfractures. So if they do some heavily strong activity,

they'll feel terrible the next day because they probably have induced a bunch of microfractures. So it's not a single point fracture.

What we can see from the patients treated at the 1-year point or beyond? Patients are having much more activity, not needing wheelchairs, not being as afraid of physical activity. So we have confidence that stronger bones will reduce microfractures and will improve pain. And so we are evaluating both pain, quality of life and other measures in the study. ***And it's a large enough study that it should help us power those endpoints***. So we think it's one of the ways that will make, I think, setrusumab a really important therapy for OI.

<Q: Kristen Brianne Klusta> And then just on that point, I know people sometimes ask, if you're feeling better and you're doing more activities, does that open the door for any potential fractures? But maybe on the other end of that spectrum, if people are exercising and doing more activity, could that help even further slowdown any type of bone loss or density loss?

<A: Emil D. Kakkis> Yes, it's a very good point. ***I think it certainly could increase fracture risk.*** We did have a patient who started doing sports again and did have a fracture, but I'm not the one to tell a patient, you feel great now, now don't do anything with that, right? It's just not rational to think that. ***What I will say is these patients, if you're sedentary, you or I sit in our bed and we don't do enough, our bones get weaker. So the exercise they do will actually stimulate their bones to lay down the bone where their bone is weakest. It will actually enhance their bone strength further***. So I think ***it'll have a beneficial effect*** for them to be more active and -- with sports or anything else. ***So we're not worried about the moral risk of getting more fractures. We think it's part of a healthy pattern towards more activity, stronger bone and better lives for these OI patients.***

(Emphasis added).

31. Further into the question-and-answer portion of the call, Defendant Kakkis discussed the potential for the varying types of OI, highlighting that Ultragenyx believed setrusumab to be able to reduce AFR regardless of OI subtype:

<Q: Michael H. Riad – Morgan Stanley – Research Associate> This is Michael Riad on for Jeff Hung. Going back to setrusumab and thinking about that cycle of fractures leading to bone deformation and then loss of activity. What factors do you think play a role -- bigger role in the treatment course? Is it age or OI type? I mean, if you think about like the profile of setrusumab, do you view it as like a broadly better option for most pediatric patients regardless of type, whereas for adults you'd expect more OI-type-dependent penetration?

<A: Emil D. Kakkis> Well, I think each patient is going to have a reason to be treated. It may be different. If you're a Type 3 patient or Type 4 with a really severe bone disease, and you're treated when you're 1 or 2 years old, our hope, and we will see what the Phase III data show, is that we could be transformed in terms of

> stopping fracture, stopping vertebral compression and not basically destroying your skeleton before you're 3 or 4 years of age and ending up in a wheelchair. So that would be what you could do when you're treating kids that are young.
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> However, when they're old, like even if you're in a wheelchair because you have deformed bones, you're still fracturing, you're still in pain all the time. ***Being able to stop being in pain by stopping fracturing***, even if you can't change deformation, it's still highly valuable in an adult with Type 3 or 4.
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> ***For Type 1, probably the superior half of that population will have enough fractures where at any age, young or old, it's going to be beneficial***. They don't have as much deformation, but being able to be comfortable, participating in sports or activities you might not have been doing before, I think will get Type 1s treated.
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> There may be some Type 1s who are milder, don't have as many fractures, and there might not be as an addressable -- as much addressable need in those patients. So we wouldn't expect all the Type 1s. ***What I can say from the data we've shown you though, the Type 1s do really well on the treatment as do the Type 3s and 4s. So we expect that we'd have a good penetration of all 3 types as well as in all ages because we think there's a reason to treat at any point in life in almost any of these diseases.***

(Emphasis added).

32. Defendant Kakkis additional fielded a question related to potential competition in the OI space, again highlighting how Ultragenyx will be able to differentiate itself by being "superior" in reducing fracture rates:

> <Q: Jingming Chen – Evercore ISI Institutional Equities – Research Analyst> This is Jingming on for Liisa. So we noticed that Amgen is running an open-label Phase III study for romosozumab in OI, and they have indicated that if the Phase III study is positive, they may have an opportunity to pursue approval and launch in OI. So I'm just wondering what implications do you think it would have for setrusumab if Amgen decides to pursue approval in OI?
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> <A: Emil D. Kakkis> Well, that's news to us. They've already given us the intellectual property access. So I don't think they've had that much interest in it. They -- it's a biologic for them. Osteoporosis is a huge indication. It's growing. There's a big shift toward anabolic agents in osteoporosis. I really think that's their focus.
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> With regard to OI, we've seen their Phase II data. We understand their dosing from the published comments in the clinicaltrials.gov, or the European version of it. Right now, they're getting substantially less bone mineral density at the dose levels they're using. ***So we're a superior treatment in terms of our bone mineral density improvement, and we will then be superior in fracture reduction.***

> So I think you should look at this as an unclear story. What they've done in their Phase III is not optimize the drug nor the presentation for OI. And so I really don't have concerns right now because we know our data. It's far superior for them to get to our data. They would have to change their dosing dramatically from Phase III, which is not likely to happen at this point. ***So at this point, I think they will be inferior to us, and I think that will be a factor.***

(Emphasis added).

November 5, 2024

33. On November 5, 2024, Defendants unveiled third quarter fiscal year 2024 results. During the earnings call that followed, Defendants engaged with analysts during the question-and-answer segment, providing further assurances of the rigor and methodology of the ongoing Phase III OI Studies, in pertinent part as follows:

> <Q: Christopher Josphe Raymond – Piper Sandler & Co. – MD & Senior Research Analyst> . . . And then maybe also a follow-up on setrusumab. Can you give a little bit more color on this negative binomial regression model that you're using just to explain a little bit about what that means, what you're doing there?
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> <A: Emil D. Kakkis> . . . With regard to setrusumab, P. K. Tandon, our Head of Biometrics, a highly experienced biometric statistician who was at Genzyme for 20 years and has done probably more rare disease programs than anyone, believes ***a negative binomial model is the best way to do an event-driven analysis, and it's a basic model that the FDA has agreed to***. We are -- for me to go through the math would be probably pretty difficult, but we probably can provide some explanation for investors on that model. ***But it's the best way to look at events and looking at event rates and being able to control in the model for things like baseline fracture rate or age or other factors that will be different between different patients***. So ***while I can't explain it, what I can say is the study is very well powered to succeed in the setrusumab Orbit study.***
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> <Q: Joseph Patrick Schwartz – Leerink Partners LLC – Senior MD of Rare Diseases & Senior Research Analyst> Great. I also have a couple of questions on setrusumab. I was wondering, first, on Orbit, if you could talk a little bit more about how you're calculating the effect size in Orbit, how that compares to how you did in Phase II and then the range of effect size separations that might be needed in order to hit stat sig at different interim analyses would be very helpful. And then I have a follow-up on Cosmic.
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> <A: Emil D. Kakkis> Well, we assumed a 50% reduction in fracture rate and a fracture rate of 0.7 for the powering estimate. However, for the interims and the choice of doing interims, that was based on the concept there could be more

> fractures events happening, not a higher fracture rate reduction. ***And so if there are more fractures, it improves the power to detect that result earlier, right, just because more events defined***.
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> So the effect size of 50% and the fracture rate, 0.7, was what was used in both the power and design. Given that the fracture rate reduction was closer to 67%, which could be similar or higher with the binomial, ***I think we feel pretty comfortable that we're in good position in how we've designed the study.*** So that's sort of what happened there with regard to the effect size.
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> <Q: Joseph Patrick Schwartz> Okay. So in terms of Cosmic, what kind of a treatment effect do you assume in your powering relative to bisphosphonates? What do you hope to see for the setrusumab arm? Are there any nuances in terms of how the end points in Cosmic are calculated versus Orbit?
>
> <A: Emil D. Kakkis> Well, keep in mind something about Orbit and the Phase II part Orbit is those patients were -- the vast majority of those patients had been on bisphosphonates. The bisphosphonates are in their bones. ***So when we're looking at the 67% reduction***, that's really like setrusumab on top of bisphosphonates, just to be clear, right? That's not -- so ***we'd expect that a similar differential occur even head to head with bisphosphonates, right? It is really like an add-on, if you will, in Orbit because they already have them in their bones.***
>
> There might be some tailing off of the bisphosphonate effect in Orbit. But in Cosmic, everyone had to be on bisphosphonates upfront, so our expectation is actually similar in terms of we went with the 50% reduction in fracture rate.
>
> ***Now the fracture rate in little kids can be much higher. It could be several fold higher, which is partly why the study is in the 60- to 70-patient range rather than 150. But that's our assumptions right now***.

(Emphasis added).

34. Defendant Kakkis, in fielding questions on the timing and potential interim analysis outcomes, further discussed the breakdown of patients in the Phase III OI Studies during the following exchanges:

> <Q: Huidong Wang – Barclays Bank PLC – Research Analyst> I have one question regarding setrusumab Phase III study. I mean you did actually provide a little bit more clarity regarding the time line. I remember last time was more likely beginning of 2025. Now is at year-end '24, beginning of 2025. And second interim, very definitive, is 1Q '25. Is that because the event's already picking up and you have more clarity regarding when this will happen?

And then also, will you share the baseline characteristics of Phase III trial at some point? If not, could you comment on patient baseline attack rate range and also the breakdown of the patients, specifically between age 5 to 12, 12 to 18 and 18 to 25?

<A: Emil D. Kakkis> With regard to the first interim timing, the clarity on the timing is not based on data we're collecting, so it's not based on fractures. We said from the beginning that would be end of the year, early 2025. And then a few months later, we're being a little more specific saying middle for 2025, but it was always a few months. ***So we weren't intending to change anything. It was just where the time line is.*** But we did change -- a long time ago, we talked about having fracture number as being the trigger, but because it was so operationally challenged, ***we just estimated when we hit a certain number of fractures. But none of the change in timings were related to the fractures.***

We haven't put out baseline characteristics yet, but we will at the appropriate time. Usually when we bring out the Phase III data, we'll bring in the characteristics, but we would not expect to put out that data until we're releasing our Phase III data. ***What we have said to date is that the population has more type III and type IV patients, closer to half or more as opposed to what was in Phase II where there was about 1/3, and so that's 1 difference. We'd expect those patients to have more fractures. We expect then the Phase III trend -- study to have a higher fracture rate than what we saw before.***

But right now, ***we haven't put in the breakout for age groups that are enrolled in the study either. It is spread across the age groups. It is primarily ped study with the majority of the patients in the peds age range***. We are stratifying in the randomization to make sure that we're -- we have similar populations in both groups. That's where we stand. Thanks for the good questions.

. . .

<Q: Maurice Thomas Raycroft – Jefferies LLC – Equity Analyst> Congrats on the progress. For setrusumab, just wondering what are key learnings from the Phase II 14-month data update at ASBMR that help you triangulate around fracture rates and chances of success for the first interim or second interim updates. And maybe just a quick follow-up, if you can clarify if you'll have new patients with less follow-up in the Angelman data updates that you have.

<A: Emil D. Kakkis> Okay. So I think what we learned from the 14-month update on setrusumab was, in fact, that ***these patients can have a very profound degree of separation, and that separation can lead to the majority of patients having no fractures over a significant period of time.***

The other thing we learned is that particularly ***the younger patients have a dramatic improvement in bone mineral density. So I think what we learned is that how strong the effect could be, and that gave us more confidence in putting in the interim in the first place*** because if they are separating very quickly within 2 or 3 months and if that effect size is large, then we would expect the groups could

> separate early. We just don't know for sure. We set a stringent threshold for the first one. The second one, less so.
>
> But that data gave us confidence that we can do that. ***It also gave us confidence we can lower the number of patients modestly and shorten the time line then to finish enrollment.*** So those are the things we learned and what we expect to know. And everything that we've seen so far tells us that we have a strong effect going on, and we want to reach that as promptly as we can.

(Emphasis added).

35. Defendant Kakkis went on to discuss in detail why Ultragenyx is confident in being able to demonstrate an improved fracture rate in the fragile population being studied during the following pertinent exchange:

> <Q: Kristen Brianne Klusta – Cantor Fitsgerald & Co – Analyst> On setrusumab, I was hoping to get a little bit more color around thoughts about the placebo arm. We know that the 5 bisphosphonate studies had diverse readouts. So can you give us some context about how you developed that 20% figure? And then is there any possibility in this trial that because patients are used to being quite inactive that we could see more fractures on placebo if the protocol requires them to go to the clinic?
>
> <A: Emil D. Kakkis> Yes. So there were -- we're aware of 5 randomized studies to look at bisphosphonates. Three of them failed and 2 of them were successful. And the 2 that were successful, there was an estimate that they had a reduction of 20% in fracture reduction. And they did make patients feel better, too, which is one of the reasons why people are using it, less about fracture reduction than feeling better, which is probably dealing with like micro fractures and something of that kind. So the data are not really that compelling, ***but if you look at our own Phase II data, the 67% reduction was on top of bisphosphonates, which were on the majority of those patients. So it's pretty clear what -- we should be able to see a substantial difference between the 2.***
>
> Now if you talk about the placebo arm in the study, ***they're not getting the bisphosphonates anymore during the study, so they will be weaning***, which might have some impact on their bones over the period of the year. But in addition, ***most of them would be normally staying at home***. And we know that b***y coming in the clinic alone, the incident of accidents and fractures goes up. It's one of the reasons patients are elected to come in a placebo-controlled study.***
>
> ***They know going back and forth the clinic every month opens them up to having fractures. So we'd expect actually the clinical activity to actually increase their fractures, which would give us more opportunity to detect the difference between them.*** So -- but because the data in the Phase II were so strong, the doctors, the patient decided they want to get in even if they got placebo because they realized they would cross over on the drug before anyone else, and they want that opportunity. ***So that's why we suddenly were able to get enrollment to crank up***

> ***and go real well as people felt like this is going to be too big a difference to not want to be part of it.***

(Emphasis added).

January 13, 2025

36. On January 13, 2025, Defendants issued a press release announcing preliminary fiscal 2024 results, guidance for fiscal 2025, and pertinently announced that "UX143 (setrusumab) Phase 3 Orbit study for osteogenesis imperfecta [would be] progressing to secondary interim analysis in mid-2025."

37. The same day, Ultragenyx presented at the 43rd Annual J.P. Morgan Healthcare Conference 2025 and provided additional clarity on the interim results. In pertinent part, Defendant Kakkis responded to an inquiry on the matter as follows:

> <Q: Anupam Rama – J.P. Morgan Chase & Co – Vice President and Analyst> . . . There's this thesis out there now that you've gone past the first interim to the second interim that somehow the probability of technical success is now different for the Orbit study. Do you agree with this? Where would you push back on that?
>
> <A: Emil D. Kakkis> No, no, I completely disagree. The idea of the first interim was simply try to see if we can accelerate further had nothing to do with that. I think the time frame and how much time of exposure was the question, how fast they can separate. They need a very extreme rapid separation. ***But the truth is that we know what will happens between 6 months and 1 year of exposure, we already have that data and we present it to people. So we feel really comfortable that this is going to be a successful product***.
>
> ***The question is how fast we can accelerate.*** Remember, it was originally a 2-year type design, and we've been pulling it up***. So it's really more about -- not about PTS, it's about how fast we can get to the success point***.
>
> ***So we feel very comfortable that either the mid or the end of the year, we'll be hitting the trial. We know the drug works very well. And so we're confident in it.***

(Emphasis added).

February 13, 2025

38. On February 13, 2025, Defendants provided their full year fiscal 2024 results and provided further points of confidence in the construction and ultimate success of their Phase III OI Studies.

39. In pertinent part, Defendant Kakkis discussed the decision and ramifications of increasing the prevalence of higher severity types of OI during the following exchanges:

> <Q: Yaron Benjamin Werber – TD Cowen – MD & Senior Biotechnology Analyst> Right. So I also, shockingly, have a 143 question. In the Orbit study, are you stratifying, just remind us, Type I, III and IV between the two arms? And then secondly, when you look at the primary of fracture rates, do you have a secondary looking fracture rates by type, underlying type?
>
> <A: Emil D. Kakkis> Yes. So in general, we do stratify, but it's mainly for -- it's overall fractures and its age. I'll let Eric talk about the way we're approaching.
>
> <A: Eric Crombez> Yes. So because the primary endpoint is annualized fracture rate, you want to stratify by fracture rate. ***So while that definitely will kind of encompass the different types there, the strict stratification is based on fracture rate coming into the study***.
>
> <A: Emil D. Kakkis> ***So Type [III and IVs] may have a higher fracture, but we're focusing on that -- doing it by the IIIs and IVs and Is, it didn't look like that was going to be the right way to go, as fracture rate was a better way***. So we are also looking at ages so there's an age balance between the groups. And regarding the other endpoints, ***we are looking at total fractures, not just the fractures minus fingers, toes, skull. Those total fractures are our endpoint***. And the subset between subtypes, I'm sure we'll do analysis sensitivities on that in there, but it's not a formal secondary endpoint.
>
> . . .
>
> <Q: Jeff Hung – Morgan Stanley – Equity Analyst> I just wanted to clarify, make sure I understood correctly. You talked about how the Phase II data and the 0.014. But just for setrusumab, would the Orbit Phase II portion have hit with the second interim analysis criteria, and if not, how were the baseline fracture rates different from the Phase II portion?
>
> <A: Emil D. Kakkis> Yes. So what I said was the Phase II data at 6 months, last patient in, we had 0.04, and then with the 14-month data we had 0.0014, right? So that was the difference. You're asking how close does that reflect what's going on? ***Well, the Phase II patients are fairly similar in terms of the entry criteria for fractures are the same***. They're made up of Type Is, IIIs, 4. ***Their Phase III has somewhat more 3s and IVs, but not a dramatic difference. So it's a very comparable population, age range, types included and baseline fracture requirements. So I think that those are reasonable ways to look at what Phase III should be happening.*** And so the only question has to do with how -- the variation in the population, how big is it and how much it moves in the timeframe. But I think the data from Phase II are a reasonable model for what's happening. Is that helpful?

(Emphasis added).

40. Further, Defendant Kakkis again spoke to Ultragenyx's ability to meet the interim analysis and why the inability to do so should not reduce investor confidence in the ultimate outcome of the studies during the following pertinent exchanges:

> <Q: Salveen Jaswal Richter – Goldman Sachs Group, Inc. – Vice President> Maybe help us understand, if it doesn't hit on the second interim, what would those reasons be, or what are the risks to that? And then how long would we have to wait for the final analysis?
>
> <A: Emil D. Kakkis> Well, we said the second interim will be midyear. ***For it not to hit at 12 months, it's usually in rare disease it would have to do with the amount of variation and the number of fractures***. If there's a lot of variation, there's a wide range of patient baseline fracture rates because we have some Type III, IV and then Type I patients. A large variation could create some -- a challenge. But I think that ***so far, we feel like the trial is proceeding as expected***. So if it doesn't hit in the second interim, we'd expect to release data by the end of the year on the final assessment for the trial.
>
> <Q: Joon So Lee – Truist Securities, Inc. – Vice President> If the OI study goes to completion in 4Q, does that imply that the magnitude of effect may not be as great as expected? And in that case, how competitive would setrusumab be compared to bisphosphonate?
>
> <A: Emil D. Kakkis> Actually, it would not mean it's not as great. If you remember, earlier when we had 6-month data and 14 months in Phase II, they both had 67% reduction in fractures. What it has to do with is the two lines have to separate. So the biggest [creation] if there's too much variation and those variations might cause a delay. But the actual rate separation could very well be 67%. ***It's just you have a lot of patients that may have 10 fractures a year or one fracture a year in the same study, and some of the ones may not have fractured, for example, and then -- for whatever reason. And so it's really more about separating the two groups***. But I don't think it necessarily tells you what the percent reduction is.
>
> We think if you listen to some of the KOLs, that 50% or greater reduction in fracture is considered really important. ***And frankly, when we look at patients after a year, 15, 16 months of therapy, we've had some -- or longer -- many of them are not fracturing at all at some point. So we feel very comfortable that the long-term outcome here is greatly reduced fractures***, whatever the number is. But I think the biggest issue is the variation in how much fractures are occurring in each group and that wide range that probably exists that will impact how the study reads out. ***We are using covariables to manage that variation, but that would be the #1 reason. So I don't think you can conclude the drug is not working well if we go to the end.***
>
> Remember, the original plan here was to do a 2-year study. ***The only reason we felt we could go sooner is because the percent reduction was higher than we thought and that the speed of response was faster***. Those are the things that give us

> confidence that we can go earlier. But we've been moving this up from 18 months to 2-year study, right, down to what we're talking about now to the 12- to 18-month time frame. ***So 18 months is still a win, and I feel confident, whichever one happens, that we have a drug that will be far better than bisphosphonate and certainly the best treatment for OI that's available.***

(Emphasis added).

May 6, 2025

41. On May 6, 2025, Defendant Crombez reiterated their plans for an interim announcement on positive results at the midyear point, stating, in pertinent part:

> The Orbit and Cosmic studies will both have an interim analysis midyear after all patients have been on therapy for at least 12 months. The data readouts will be led by Orbit, meaning that if Orbit clears the p-value threshold of less than 0.01, we will look to see if Cosmic has cleared the same p-value threshold of less than 0.01. If Orbit progresses to full study completion in the fourth quarter of this year, Cosmic will also continue to a data readout to align with the Orbit data readout without spending alpha at this interim assessment.

42. During the question-and-answer segment, Defendant Kakkis fielded a question on the likelihood of success should the Phase III OI Studies fail to reach the second interim date benchmark during the following exchange:

> <Q: Tazeen Ahmad – BofA Securities – MD in Equity Research & Research Analyst> . . . if the study moves to a third interim read, what's your view of the likelihood of success? You've talked now multiple times about confidence in the molecule overall, and we would agree that the drug is active. But if the study moves to the third interim, what would be a reason to be concerned that it would not work at the third interim?
>
> <A: Emil D. Kakkis> Right. Well, it won't be -- the next assessment is the final assessment for the study, and that p-value threshold will be 0.04. So it would be a lot easier to hit 0.04. ***So we think that we will hit one or the other based on our experience, what we've seen. I don't think we could miss the 0.04 at that point with 18 months of time***. But as always in rare disease programs, the other -- the thing you always are battling us is variation, variability in patients. ***But based on the profound difference in bone mineral density change that we see that happens within 2 to 3 months and the fracture rate effect happens within 2 to 3 months, we feel pretty good about IA2 hitting, but confident about overall the study hitting even at the end, if not at the IA2***.
>
> So I can't tell you a reason why, but variation is always the thing that can create complexities. But given that the patients -- ***the program is 159 patients, that's a pretty large study. And we were -- the data we're talking about before was 24. So I think we've got a lot of power in there***, but -- ***and we've done everything we can***

> ***to manage variations***. ***So I feel good about we'll hit it this year, either at 0.01 or 0.04 after 18 months.***

(Emphasis added).

43. Defendant Kakkis further spoke to the prevalence and impact of increased severe OI subtypes in the Phase III studies during the following pertinent exchanges:

> <Q: Yigal Dov Nochomovitz – Citigroup Inc. – Director and Smid Cap Biotech Analyst> Have you commented at all on the distribution of the types for OI for 1, 3 and 4 for the Phase II versus the Orbit trial?
>
> . . .
>
> <A: Emil D. Kakkis> So on the OI types, I think we've disclosed before that in the ***Phase II study, there were 7 type 3s and 4s and 17 type 1s***. And then because the doctors were then impressed with the results, then they were interested in bringing in their more severe 3 and 4 patients. ***So we ended up with more type 3s and about half the patients are type 3 and 4 approximately there in the study. So it's definitely an increase in Type 3s and 4s in the Phase III study than they were in the Phase II study, all right?***
>
> . . .
>
> <Q: Mahdi Goudarzi – Truist Securities, Inc. – Biotech Equity Research Associate> This is Mahdi on for Joon. So I go on OI and follow up Yigal's question on composition of OI types. So do you agree that setrusumab's MOA benefits the type 1 patients more than Type 4 and 3. This is the question.
>
> <A: Emil D. Kakkis> ***Well, I know there's been some academics saying that,*** but -- and I know some of them very good academics, ***but they're actually incorrect because we already have data***. So it's not -- the theory would be that in type 1 patients have deficient collagen, don't have abnormal collagen. ***Therefore, if we just make more bone, it will be okay.*** And the type 3s and 4s have abnormal collagen, therefore, it's not improved, but that's not actually what we saw. We see both of them have improved reduction in fractures. And in fact, the ones fractures we did see were in type 1 patients, I think were some of the ones not type 3s and 4s.
>
> So the truth is all of them are improved because while one is a deficiency collagen and one is abnormal collagen, whether deficient or abnormal, the net benefit of making more bone is bone, greater bone strength and reduced fractures. So it actually works in all three. ***And historical clinical view of OI is going to change because the truth is that even with abnormal collagen, the bones can be strengthened***, we believe, in these patients, and that's what we've seen, and that's in the data from Phase II. ***And so we're confident that the type will not matter***. You get the same bone mineral density effect and the strength improvement will be the same regardless of the collagen mutation.

(Emphasis added).

44. The above statements in Paragraphs 24 to 43 were false and/or materially misleading. Defendants created the false impression that they possessed reliable information pertaining to the effects of setrusumab on patients with variable types of OI, while also minimizing risk that patients in the Phase III Orbit study would fail to achieve a statistically significant reduction in AFR, such that the second interim analysis could be performed and presented to the investing public. In truth, Ultragenyx's optimism in the Phase III Orbit study's results and interim analysis benchmark were misplaced; Ultragenyx failed to convey the risk associated with basing such threshold figures on Phase II results that had no placebo control group for appropriate comparison and thus had not ruled out that the reduction in AFR from that study could merely be triggered by an increased standard of care and the placebo effect of being provided a novel treatment.

C. Ultragenyx Reveals the Phase III ORBIT Study Failed To Achieve the Second Interim Output

July 9, 2025

45. On July 9, 2025, Ultragenyx issued a press release and "announced that the randomized, placebo-controlled Phase 3 portion of the *Orbit* study evaluating UX143 (setrusumabg) in pediatric and young adult patients with [OI] is progressing toward a final analysis consistent with the original plan, around the end of the year."

46. The release further reminded investors of the easier-to-achieve threshold for the final analysis due at years end:

> Patients will continue dosing in the ongoing Phase 3 Orbit and Cosmic clinical studies with the final analyses to be conducted after patients have been on therapy for at least 18 months. The threshold for the Phase 3 Orbit final analysis is $p<0.04$ and for the Phase 3 Cosmic final analysis is $p<0.05$.

47. The aforementioned press releases and statements made by the Individual Defendants are in direct contrast to statements they made during the above-referenced earnings calls and shareholder presentations. On those calls, Defendants continually expressed confidence

in the ability of its Phase III Orbit study to achieve the second interim results threshold necessary to present results to the investing public, while also minimizing the risk that the study could fail to demonstrate that setrusumab results in a reduction in AFR for the OI patients tested.

48. Investors and analysts reacted immediately to Ultragenyx's revelation. The price of Ultragenyx's common stock declined dramatically. From a closing market price of $41.44 per share on July 9, 2025, Ultragenyx's stock price fell to $31.03 per share on July 10, 2025, a decline of about 25.12% in the span of just a single day.

49. A number of well-known analysts who had been following Ultragenyx lowered their price targets in response to Ultragenyx's disclosures. For example, Wells Fargo, while slashing their price target more than 26%, justified their cut on the failure to meet the second interim analysis thresholds. In pertinent part, the analyst highlighted that "most of our inbounds suggested high expectations that the study would hit this interim analysis; management commentary had also been bullish on IA2, with even recent comments around commercial readiness planning."

50. The fact that this analyst, and others, discussed both Ultragenyx's "bullish" commentary on the second interim readout and the failure to achieve the threshold requirements for the analysis suggest the public placed significant weight on Ultragenyx's prior optimistic and confident statements regarding the Phase III OI Studies. The frequent, in-depth discussion of such statements confirms that Defendants' statements during the Class Period were material.

51. Notwithstanding Defendants' disclosures, they continued to mislead investors by misrepresenting their understanding of the risk that the Phase III OI Studies would be unable to achieve their respect endpoints of reduced AFR. In doing so, the Defendants deceptively claimed confidence in the studies achieving positive results.

August 5, 2025

52. On August 5, 2025, Defendants issued a press release announcing their financial results for the second quarter of fiscal year 2025 and a "Corporate Update" on its existing studies.

53. Pertinently, the release reminded investors that the "randomized, placebo-controlled Phase 3 portion of the Orbit study was evaluated by the Data Monitoring Committee at

an interim analysis in July 2025 and they informed the company that UX143 demonstrated an acceptable safety profile and that the study should continue to the final analysis."

54. During the same-day earnings call, and despite missing the second interim window, Defendant Kakkis reiterated Ultragenyx's confidence in setrusumab's ability to demonstrate a statistically reduced fracture rate in the Phase III OI Studies, stating, in pertinent part:

> ***The Orbit and Cosmic studies are continuing to the final analysis that will occur around the end of the year***. While I hope the studies might have stopped early at the interim time point last month, ***we remain confident in completing a successful study.*** We're pleased the safety profile is as expected and that after looking at the data, the DMC recommended we continue to the final analysis. ***As we head to the final analysis, the continued treatment of Phase III should further strengthen bones of the treated patients. The additional 6 months of treatment for the treated subjects, along with the larger p-value threshold at 0.04, will help power the final assessment***. We look forward to unblinding the Phase III datasets and sharing results around the end of the year.
>
> Now based on all the data we've seen in Phase II, ***we are confident UX143 will be a transformational treatment for pediatric and adult patients with osteogenesis imperfecta***. The ***combination mechanism of building bone and reducing excess resorption is at exactly the sites in their body where they need more bone will increase bone strength and reduce fractures, while at the same time improving overall bone health***. In addition to reducing fractures, we are encouraged by the functional effect we are seeing on increasing physical activity and ability that speaks to the long-term potential for this treatment.

(Emphasis added).

55. Defendants Kakkis and Crombez further fielded several question pertaining to why the study was unable to meet its interim thresholds and why the Defendants remained confident in the study, pertinently as follows:

> <Q: Kristen Brianne Kluska – Cantor Fitzgerald & Co. – Analyst> For the Orbit study reading out later this year, I know you still have very high conviction in the trial being successful. But I wanted to talk about a hypothetical scenario where maybe the fracture data falls slightly under what you were hoping for, but you see really strong benefits on pain. Do you still think that there is a strong case to make for the FDA here? And could you argue that this will drive higher adoption for patients since they deal with this on a daily basis over the fracture aspects?
>
> <A: Emil D. Kakkis> Well, I think that your point is maybe there's some variation in fractures and you just missed that and you have other supportive data. I think the FDA will always look at the total -- totality of the data in our rare disease program. We've had that many times in many programs***. Our sense here is that we're seeing***

a fundamental mechanistic effect on bone mineral density, the effect it has on fractures depends on how many fractures that patient have in their particular condition.

We have a lot more Type IIIs and Type IVs in the study. They have a lot of complex problems. And so I'm sure that the support of other data would help us in any situation, whatever the statistical or treatment size is. And so that's just generally been the case. ***We feel confident what the fracture data will be, what it is. We're seeing what's going on in Phase II. We know that as time goes on, there's very few fractures among patients after they've gotten established on the treatment.***

So we feel good we'll be able to do that. But hypothetically, I think the data will always be more than just fractures in this disease state. And the body of data we have, we think, will support its use however we come out with on fractures.

. . .

<Q: Yigal Dov Nochomovitz – Citigroup Inc. – Director and SMidCap Biotech Analyst> Okay. I was going to say on OI, given the first 2 interims have passed and now we're looking at the final one, I'm just curious if you have any updated thoughts as far as what you believe the expected placebo AFR would be. Obviously, we've done some work, and there are a number of epidemiologic studies out there, both in Scandinavia as well as the United States, which point to various ranges for AFR.

I'm just wondering if you could comment on what you believe would be the most likely scenario at this point as well as on some of the more specific aspects of the statistics again regarding this concept of variance or overdispersion which, as we know, is a feature of this particular dataset given the way the fractures are distributed.

<A: Eric Crombez> Yes. So yes, we're aware of the annualized fracture rate available in the literature. And looking at natural history the principal investigators have on hand, we really use a lot of the data coming on for pretreatment for baseline for both Orbit and Cosmic to do our modeling. ***And we were really looking at those patients with the baseline AFR between 0.72 and 1 for our modeling to support both of the work for the interim analysis and obviously, the powering we did for the primary efficacy analysis period at 18 months.***

And with the dispersion, yes, I mean, I think while we did not change the entry criteria for Orbit Phase II going into Orbit Phase III, on the strength of the Phase II data, ***we had really what I consider to be a self-enrichment of patients with Type IIIs and Types IV. I think they needed to see that strong safety and efficacy data to take the risk to come into clinic because remember, they really are at risk just from traveling into sites to sign consents and begin studying participation. So I will say we did -- we have a greater number of patients with Type III and Type IV OI in the Phase III part of Orbit compared to Phase II.***

(Emphasis added).

November 4, 2025

56. On November 4, 2025, during Ultragenyx's final earnings call before the results Phase III OI Studies would be released, Defendant Kakkis remained confident they would demonstrate a reduction in AFR, pertinently stating the following during the call:

> As we move into the final analyses, we remain confident in setrusumab's mechanism of action, its ability to make more bone in the places that need more strength, which should reduce fractures. If successful, this will lead to a transformational treatment for pediatric and adult patients with osteogenesis imperfecta.
>
> . . .
>
> Now with regard to what we expect in Phase III***, we've said that anywhere between 40% and 70% reduction in fractures, in that range, is a very good fracture reduction level. And I don't think that the exact percentage within that range matters as much as how patients feel and how they're functioning***. And from the Phase II study, it's pretty clear that the way patients are functioning is quite important in terms of their ability to take on exercises, to walk better, get out of wheelchairs or using walkers, et cetera.
>
> ***So we think anywhere in that range -- and I think most KOLs have suggested something better than 40%***. We've seen 67% in the Phase II study. I think anywhere in that range is -- I think, would be a clinically meaningful change for these patients
>
> . . .
>
> So I think if we get one or the other study positive, we'll be able to work forward. And how we solve the issue of the age range and the indication, ***I don't expect there to be a difference in how the drug works. I think both studies should show a substantial bone mineral density benefit improvement and should show improvement in fractures***. So we're confident in the program, but I think we can make it work with either combination of results.

(Emphasis added).

57. The above statements in Paragraphs 52 to 56 were false and/or materially misleading. Defendants created the false impression that they possessed reliable information pertaining to the effects of setrusumab on patients with variable types of OI, while also minimizing risk from study variability and the potential outperformance of the control groups. In truth,

Ultragenyx's claims of changing the "historical clinical view of OI," increasing the control group's fracture rate to "give [Ultragenyx] more opportunity to detect the difference between them," and being able to demonstrate a reduction in AFR despite permitting increased activity among tested patients fell shy of reality as such claims relied far too heavily on assumptions Defendants had made on a smaller sample size and differing patient pool that populated the Phase II Orbit study. Ultimately, Defendants failed to convey the associated risk of such assumptions to Ultragenyx's investors. Ultragenyx was simply either unable to generate a study that could accurately show setrusumab's impact on annualized fracture rates or the drug simply does not have the impact that Defendants repeatedly and confidently claimed they could demonstrate.

D. The Full Truth Emerges When Ultragenyx Reports Results of the Phase III Orbit and Cosmic Studies

December 29, 2025

58. On the morning of December 29, 2025, Defendants announced the "results from the Phase 3 Orbit and Cosmic studies for setrusumab (UX143) in Osteogenesis Imperfecta" in a form 8-K filing.

59. The filing disclosed that both studies failed to achieve statistical significance "against the primary endpoints of reduction in annualized clinical fracture rate compared to placebo or bisphosphonates," respectively, despite achieving "secondary endpoints of improvements in bone mineral density ('BMD')."

60. In pertinent part, the release provided the following limited details, adding:

> In the Orbit study, participants experienced statistically significant and substantial improvements in BMD compared to placebo, at levels consistent with the treatment effect observed in the Phase 2 portion of the study. ***These BMD changes were not accompanied by a corresponding reduction in annualized fracture rates and there was a low fracture rate in the placebo group***.
>
> In the pediatric Cosmic study, ***patients had a substantially higher baseline fracture rate*** compared to the patients enrolled in Orbit. In this younger patient population, meaningful improvements in BMD were ***associated with a reduction*** in annualized fracture rate for setrusumab treated patients compared to bisphosphonate treated patients, though ***the reduction did not meet statistical significance.***

(Emphasis added).

61. Defendants further announced that, as a result of the failed studies, Ultragenyx "is evaluating its planned operations and ***will promptly define and implement significant expense reductions***" (emphasis added).

62. The aforementioned press releases and statements made by the Individual Defendants are in direct contrast to statements they made during the above-referenced earnings calls, shareholder presentations, and interim study updates. In those statements, Defendants continually claimed a high level of confidence that setrusumab's ability to increase material bone density would necessarily translate to a reduction in the annualized fracture rate of patients with type 1, 3, or 4 osteogenesis imperfecta. Defendants further claimed a lack of concern over whether minimal fractures in the control groups or increased fractures in the treatment groups could ultimately confound the results of the Phase III OI Studies. Indeed, in both instances, Defendants claimed their study design would facilitate the ability to detect the difference in fracture rate between the groups while minimizing the risk of either scenario triggering a significant negative impact on the results.

63. Investors and analysts reacted immediately to Ultragenyx's revelation. The price of Ultragenyx's common stock declined dramatically. From a closing market price of $34.19 per share on December 26, 2025, Ultragenyx's stock price fell to $19.72 per share on December 29, 2025, a decline of about 42.32% in the span of just a single day.

64. A number of well-known analysts who had been following Ultragenyx lowered their price targets in response to Ultragenyx's disclosures. For example, Baird, while slashing their price target by nearly 35%, justified their price cut as, "disappointingly, setrusumab did not show a benefit in AFR in the ~159-patient-placebo-controlled ORBIT study (despite a statistically-significant improvement in BMD, [Bone Mineral Density]." The analyst further highlighted Ultragenyx's position that a "higher-than-expected placebo response was a key headwind to the study," and pointed to anticipated "analyses, such as responses in Type 1s vs Type 3/4s."

65. Similarly, Barclays, while dropping their price target 12% to $44, noted that "while ORBIT's miss was ***somewhat a surprise***, COSMIC's failure ***was in line with our prediction*** …

While both studies showed low nominal p value for BMN improvement, it was not accompanied by a reduction of AFT in ORBIT and placebo had a low fracture rate" (emphasis added).

66. Additionally, Wells Fargo reduced their probability of success for FDA approval down to only 33%, noting the new difficulties from the Phase III study failure, in pertinent part:

> FDA's stance seems to be more about using BMD to avoid the need for large, long trials required to evaluate AFR. However, RARE has already done this & we think asking the FDA to ignore an already generated negative AFR result in favor of BMD will require flexibility beyond the spirit of the osteoporosis guidance. Secondly, the FDA explicitly calls out hip BMD as assessed by DXA, and not lumbar BMD which is what RARE assessed in ORBIT/COSMIC.

Notably, Wells Fargo reduced their price target by an additional $20 to $45.00, a reduction of nearly 49% from the $88 target leading into the second interim analysis.

E. Additional Remarks Following the Class Period

January 12, 2026

67. On January 12, 2026, Defendant Kakkis presented on behalf of Ultragenyx at the 44th Annual J.P. Morgan Healthcare Conference and provided additional color on the ORBIT and COSMIC results.

68. In pertinent part, Defendant Kakkis highlighted the breakdown of the presented variations of osteogenesis imperfecta in the studies, stating:

> If you look at the patients that we enrolled in the 2 designs, ***in Orbit, they were generally balanced, but there were more type 3s, which are severe in the setrusumab arm and more type 4s in the placebo arm. And if you look at Cosmic, they're relatively well balanced. So there were 64% of the more severe type vs 54***.
>
> . . .
>
> Now in Orbit, because it was placebo controlled, we had to include a rescue arm because patients did not want to enroll in a trial that could go as long as 2 years, be off their bisphosphonate treatments. So if they hit a certain number of fractures, they could exit the study; 19%, ***19.5% did exit the study***. ***The placebo, though exited with type 3, type 4 patients, essentially, as you would expect, at nearly twice the rate of what the treated patients. So there's clearly a differential between which patients came out.***

(Emphasis added).

69. Defendant Kakkis went on to highlight and discuss the details behind the failure of the studies to achieve their annualized fracture rate endpoints, providing, in pertinent part, the following:

> Now if we dive in the Orbit fracture story, this is where things started to change. If you look on the right, you can see the cumulative fracture distribution curves, and you can see the groups are not really separating whichever way you look at it without the primary endpoint, without vertebral fractures and fingers and skull and the others where you look at total fractures, very similar.
>
> And because the story comes really -- ***if you look at the placebo group, based on the negative binomial model, it was only a 0.55 fracture rate estimated. But the median, if you look above is 0, which means of 52 patients, 26 patients had no fractures in the study. So this gives you very little power now to tell the difference***. The fracture frequency in the UX143 group was actually fine, 0.71. It was not very high, considering what the pretreatment fractures were at.
>
> ***So both study arms were lower. The placebo is slightly favored, but that's only with the primary endpoint, looking at all fractures are about the same***. So the question is what's going on here in the study with fractures. This was the part that was a surprise. ***Now we know from the Phase II study patients felt better and got more active. So one of the question is did they get more active and cause more fractures, that we won't know for sure, but we did look at how they did, and Orbit is a placebo-controlled trial.***
>
> . . .
>
> So you might wonder, ***well, if the fractures aren't better, why are they doing better? I think it could be because they're doing better and they're running around. And we did see that in Phase II that some kids had fractures, but we still should have seen some differentiation, we didn't.*** But this is the Orbit story, we think this shows an activity, but we didn't show the fracture differential.
>
> So now let's talk about Cosmic. ***In Cosmic, the story is a little different***. The fracture rate was higher. If you look at the mean or median, you could see there was 2.6 was the mean for the bisphosphonate group. So several fold more fractures going on in the study. ***If you look at the cumulative distribution graphs on the right, you can see that they're separating quickly, right, after initiation of trial. There's a little turn up in the graph that happens to be when we do a 12-month skeletal survey***.
>
> That's why a bunch of fractures are added because we see the fractures on a survey that weren't seen before and they're added. But you can see the separation of the 2 lines gets even greater. ***So this is separating in the way we would have thought, right? This is what we were expecting. When you do the negative binomial model, it shows a 21% difference, but still not significant***, but it's -- the pattern looks more like what we would have expected to see.

(Emphasis added).

70. During the question-and-answer segment that followed, Defendant Kakkis further highlighted the Company's current lack of understanding as to the fracture rate results during the following exchange:

> <Q: Anupam Rama – JPMorgan Chase & Co – Vice President and Analyst> How do you think about next regulatory steps now that you have the totality of this data? Like is there a path forward, you believe, even if it's in a subset of patients such as pediatrics?
>
> <A: Emil D. Kakkis> I think right now, ***our most important thing is to really understand more about the data and why it is the way it is. Long bone did not appear better. Is that because kids were more active and they fractured? Or is there something else going on there?*** So we need to understand all of that to understand it better. And then we need to do that before we end up going. If we go to the agency, we'll want to do that with a firm understanding of what we have in front of us. And I think there's a little more work to do before we make that move.

(Emphasis added).

71. Additionally, William Blair, reporting after Ultragenyx provided more data on the results in mid-January, noted that "the updated data from the Orbit study showed patients treated with setrusumab had numerically more breaks than patients treated with placebo … the fracture rate in patients treated with setrusumab was 0.71, compared to 0.55 in the placebo group … management indicated that the ***study wasn't well powered to show a difference*** in the negative binomial model selected for the primary endpoint."

72. The fact that these analysts, and others, discussed the failure of Ultragenyx's Phase III OI Studies to demonstrate their primary endpoint of reduced AFR suggests the public placed significant weight on Ultragenyx's confidence in both the from of its Phase III OI Studies and their ability to demonstrate a reduction in fractures across all types of OI tested. The frequent in-depth discussions of the tests and their results confirm that Defendants' statements during the Class Period were material.

F. Additional Scienter Allegations

73. During the Class Period, Defendants acted with scienter in that they knew, should have known, or otherwise were deliberately reckless in not knowing that the public statements

disseminated on behalf of Ultragenyx were materially false and misleading at the time they were made. Defendants had actual knowledge of, or access to, non-public information concerning both the existing results of setrusumab and the intended study design and scope of the Phase III Orbit and Cosmic studies.

74. Notwithstanding such, Defendants repeatedly and affirmatively represented to investors that Ultragenyx's study designs for its Phase III Orbit and Cosmic studies were well positioned to demonstrate the primary endpoint of a reduction in AFR.

75. Defendants further repeatedly claimed there would be no significant negative impact from the inclusion of a greater proportion of Type 3 and 4 OI patients in the studies than were present in the Phase II Orbit study.

76. Yet, Defendants made selective and misleading disclosures in articulating the breakdown and potential impact of the OI type variances being studied. Notably, during the February 13, 2025, earnings call, Defendants indicated there was a "fairly similar" make up in patient entry criteria between Phase II and III with some increase in Type 3 and 4s, "but not a dramatic difference." By the following earnings call on May 6, 2025, Defendants conceded that about half of Phase III patients were type 3 or 4, compared to less than a third in Phase II. In August the narrative shifted to "a lot more Type IIIs and Type IVs in the study," blaming the failure to reach an interim analysis partially on their "complex problems."

77. Defendants' scienter was further evidenced by their claims that the control group patients would generate enough fractures to properly compare them to the treatment populations.

78. Defendants repeatedly claimed that by forcing the control group to merely come into the clinic, "the incident of accident and fractures [would go] up."

79. Further, and despite acknowledging that patients in the Phase II Orbit study who felt improved on treatment tended to engage in more physical activity and thus trigger more fractures, Defendants argued there was no reason to implement any activity limitations on the patient population. Defendants were aware of the risk but dismissed them to investors, claiming they "we're not so worried about the … noise of having more fracture risk at this point," and

arguing that setrusumab would increase the strength of the bones sufficiently "to even compensate for any change [that] might occur because patients are more active."

80. Defendants were aware of these risks yet continued to deliberately disregard or otherwise minimize them in their claims of confidence to investors. At one point Defendant Kakkis even claimed that he did not "see any uncontrolled factors" in the Phase III protocols.

81. Ultimately, these risks, which Defendants either knew or deliberately disregarded, came to fruition. The Phase III OI Studies failed to demonstrate a reduction in AFR as the control groups ultimately achieved similar reductions to the testing groups due (1) inflated pre-test bone breaks among the more severe OI type populations; (2) a failure to increase the fracture rate of control group patients by merely coming into the clinic; and (3) a smaller reduction in AFR among the treatment population because of their increased activity. Alternatively, the studies failed merely because setrusumab's ability to increase material bone density does not truly cause or even correlate to a reduction in annualized fracture rate among OI patients, suggesting Defendants' repeated statements of confidence in the drug and its potential was, at best deliberately reckless.

G. Loss Causation and Economic Loss

82. During the Class Period, as detailed herein, Defendants made materially false and misleading statements and engaged in a scheme to deceive the market and a course of conduct that artificially inflated the price of Ultragenyx's common stock and operated as a fraud or deceit on Class Period purchasers of Ultragenyx's common stock by materially misleading the investing public. Later, Defendants' prior misrepresentations and fraudulent conduct became apparent to the market, the price of Ultragenyx's common stock materially declined, as the prior artificial inflation came out of the price over time. As a result of their purchases of Ultragenyx's common stock during the Class Period, Plaintiff and other members of the Class suffered economic loss, *i.e.*, damages under federal securities laws.

83. Ultragenyx's stock price fell in response to the corrective events on July 9, 2025 and December 26, 2025, as alleged *supra*. On July 9, 2025, Ultragenyx revealed that it would be progressing with the study instead of ending it early. From a closing market price of $41.44 per

share on July 9, 2025, Ultragenyx's stock price fell to $31.03 per share on July 10, 2025, a decline of about 25.12% in the span of just a single day.

84. On December 26, 2025, Ultragenyx announced both its Phase III Orbit and Cosmic studies failed demonstrate that setrusumab triggered a statistically significant reduction in annualized fracture rates for patients with osteogenesis imperfecta. The price of Ultragenyx's common stock declined dramatically. From a closing market price of $34.19 per share on December 26, 2025, Ultragenyx's stock price fell to $19.72 per share on December 29, 2025, a decline of about 42.32% in the span of just a single day.

H. Presumption of Reliance; Fraud-On-The-Market

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

(a) Ultragenyx's common stock met the requirements for listing and was listed and actively traded on the NASDAQ during the Class Period, a highly efficient and automated market;

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

(c) Ultragenyx was followed by several securities analysts employed by major brokerage firms who wrote reports that were distributed to the sales force and certain customers of their respective brokerage firms during the Class Period. Each of these reports was publicly available and entered the public marketplace; and

(d) Unexpected material news about Ultragenyx was reflected in and incorporated into the Company's stock price during the Class Period.

86. As a result of the foregoing, the market for Ultragenyx's common stock promptly digested current information regarding the Company from all publicly available sources and reflected such information in Ultragenyx's stock price. Under these circumstances, all purchasers of Ultragenyx's common stock during the Class Period suffered similar injury through their

purchase of Ultragenyx's common stock at artificially inflated prices, and a presumption of reliance applies.

87. Alternatively, reliance need not be proven in this action because the action involves omissions and deficient disclosures. Positive proof of reliance is not a prerequisite to recovery pursuant to ruling of the United States Supreme Court in *Affiliated Ute Citizens of Utah v. United States*, 406 U.S. 128 (1972). All that is necessary is that the facts withheld be material in the sense that a reasonable investor might have considered the omitted information important in deciding whether to buy or sell the subject security.

I. No Safe Harbor; Inapplicability of Bespeaks Caution Doctrine

88. The statutory safe harbor provided for forward-looking statements under certain circumstances does not apply to any of the material misrepresentations and omissions alleged in this Complaint. As alleged above, Defendants' liability stems from the fact that they provided investors with revenue projections while at the same time failing to maintain adequate forecasting processes. Defendants provided the public with forecasts that failed to account for this decline in sales and/or adequately disclose the fact that the Company at the current time did not have adequate forecasting processes.

89. To the extent certain of the statements alleged to be misleading or inaccurate may be characterized as forward looking, they were not identified as "forward-looking statements" when made and there were no meaningful cautionary statements identifying important factors that could cause actual results to differ materially from those in the purportedly forward-looking statements.

90. Defendants are also liable for any false or misleading "forward-looking statements" pleaded because, at the time each "forward-looking statement" was made, the speaker knew the "forward-looking statement" was false or misleading and the "forward-looking statement" was authorized and/or approved by an executive officer of Ultragenyx who knew that the "forward-looking statement" was false. Alternatively, none of the historic or present-tense statements made by Defendants were assumptions underlying or relating to any plan, projection, or statement of future economic performance, as they were not stated to be such assumptions underlying or

relating to any projection or statement of future economic performance when made, nor were any of the projections or forecasts made by the defendants expressly related to or stated to be dependent on those historic or present-tense statements when made.

CLASS ACTION ALLEGATIONS

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

92. The members of the Class are so numerous that joinder of all members is impracticable. Throughout the Class Period, Ultragenyx's common stock were actively traded on the NASDAQ. While the exact number of Class members is unknown to Plaintiff at this time and can be ascertained only through appropriate discovery, Plaintiff believes that there are hundreds or thousands of members in the proposed Class. Record owners and other members of the Class may be identified from records maintained by Ultragenyx or its transfer agent and may be notified of the pendency of this action by mail, using the form of notice similar to that customarily used in securities class actions. As of October 30, 2025, there were 96.47 million shares of the Company's common stock outstanding. Upon information and belief, these shares are held by thousands, if not millions, of individuals located throughout the country and possibly the world. Joinder would be highly impracticable.

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

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

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

(a) whether the federal securities laws were violated by Defendants' acts as alleged herein;

(b) whether statements made by Defendants to the investing public during the Class Period misrepresented material facts about the business, operations and management of Ultragenyx;

(c) whether the Individual Defendants caused Ultragenyx to issue false and misleading financial statements during the Class Period;

(d) whether Defendants acted knowingly or recklessly in issuing false and misleading financial statements;

(e) whether the prices of Ultragenyx's common stock during the Class Period were artificially inflated because of the Defendants' conduct complained of herein; and

(f) whether the members of the Class have sustained damages and, if so, what is the proper measure of damages.

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

COUNT I

Against All Defendants for Violations of

Section 10(b) and Rule 10b-5 Promulgated Thereunder

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

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

99. During the Class Period, Defendants engaged in a plan, scheme, conspiracy and course of conduct, pursuant to which they knowingly or recklessly engaged in acts, transactions, practices and courses of business which operated as a fraud and deceit upon. Plaintiff and the other members of the Class; made various untrue statements of material facts and omitted to state material facts necessary in order to make the statements made, in light of the circumstances under which they were made, not misleading; and employed devices, schemes and artifices to defraud in connection with the purchase and sale of securities. Such scheme was intended to, and, throughout the Class Period, did: (i) deceive the investing public, including Plaintiff and other Class members, as alleged herein; (ii) artificially inflate and maintain the market price of Ultragenyx common stock; and (iii) cause Plaintiff and other members of the Class to purchase or otherwise acquire Ultragenyx's securities at artificially inflated prices. In furtherance of this unlawful scheme, plan and course of conduct, Defendants, and each of them, took the actions set forth herein.

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

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

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

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

104. During the Class Period, Ultragenyx's common stock was traded on an active and efficient market. Plaintiff and the other members of the Class, relying on the materially false and misleading statements described herein, which the defendants made, issued or caused to be disseminated, or relying upon the integrity of the market, purchased or otherwise acquired shares of Ultragenyx's common stock at prices artificially inflated by defendants' wrongful conduct. Had Plaintiff and the other members of the Class known the truth, they would not have purchased or otherwise acquired said common stock, or would not have purchased or otherwise acquired them at the inflated prices that were paid. At the time of the purchases and/or acquisitions by Plaintiff and the Class, the true value of Ultragenyx's common stock was substantially lower than the prices paid by Plaintiff and the other members of the Class. The market price of Ultragenyx's common

stock declined sharply upon public disclosure of the facts alleged herein to the injury of Plaintiff and Class members.

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

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

COUNT II

Against the Individual Defendants

for Violations of Section 20(a) of the Exchange Act

107. Plaintiff repeats and realleges each and every allegation contained in the foregoing paragraphs as if fully set forth herein.

108. During the Class Period, the Individual Defendants participated in the operation and management of the Company, and conducted and participated, directly and indirectly, in the conduct of the Company's business affairs. Because of their senior positions, they knew the adverse non-public information about Ultragenyx's misstatements.

109. As officers and/or directors of a publicly owned company, the Individual Defendants had a duty to disseminate accurate and truthful information, and to correct promptly any public statements issued by Ultragenyx which had become materially false or misleading.

110. Because of their positions of control and authority as senior officers, the Individual Defendants were able to, and did, control the contents of the various reports, press releases and public filings which Ultragenyx disseminated in the marketplace during the Class Period concerning the misrepresentations. Throughout the Class Period, the Individual Defendants exercised their power and authority to cause Ultragenyx to engage in the wrongful acts complained of herein. The Individual Defendants therefore, were "controlling persons" of the Company within

the meaning of Section 20(a) of the Exchange Act. In this capacity, they participated in the unlawful conduct alleged which artificially inflated the market price of Ultragenyx's common stock.

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

112. By reason of the above conduct, the Individual Defendants and/or Ultragenyx are liable pursuant to Section 20(a) of the Exchange Act for the violations committed by the Company.

PRAYER FOR RELIEF

WHEREFORE, Plaintiff demand judgment against defendants as follows:

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

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

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

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

DEMAND FOR TRIAL BY JURY

Plaintiff hereby demands a trial by jury.