

UNITED STATES DISTRICT COURT
DISTRICT OF NEW JERSEY

____, Individually and on Behalf of All
Others Similarly Situated,

Plaintiff,

v.

ROCKET PHARMACEUTICALS, INC.,
GAURAV SHAH, and AARON
ONDREY,

Defendants.

Case No.

CLASS ACTION COMPLAINT

JURY TRIAL DEMANDED

Plaintiff ____ (“Plaintiff”), individually and on behalf of all others similarly situated, by Plaintiff’s undersigned attorneys, for Plaintiff’s complaint against Defendants, alleges the following based upon personal knowledge as to Plaintiff and Plaintiff’s own acts, and information and belief as to all other matters, based upon, *inter alia*, the investigation conducted by and through Plaintiff’s

attorneys, which included, among other things, a review of the Defendants' public documents, conference calls and announcements made by Defendants, United States ("U.S.") Securities and Exchange Commission ("SEC") filings, wire and press releases published by and regarding Rocket Pharmaceuticals, Inc. ("Rocket" or the "Company"), analysts' reports and advisories about the Company, and information readily obtainable on the Internet. Plaintiff believes that substantial, additional evidentiary support will exist for the allegations set forth herein after a reasonable opportunity for discovery.

NATURE OF THE ACTION

1. This is a federal securities class action on behalf of a class consisting of all persons and entities other than Defendants that purchased or otherwise acquired Rocket securities between September 17, 2024 and May 26, 2025, both dates inclusive (the "Class Period"), seeking to recover damages caused by Defendants' violations of the federal securities laws and to pursue remedies under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 (the "Exchange Act") and Rule 10b-5 promulgated thereunder, against the Company and certain of its top officials.

2. Rocket, together with its subsidiaries, operates as a late-stage biotechnology company that focuses on developing gene therapies for rare and devastating diseases in the U.S. The Company develops in vivo adeno-associated

viral (“AAV”) programs, including, *inter alia*, RP-A501 for the treatment of Danon disease (“DD”), a multi-organ lysosomal-associated disorder leading to early death due to heart failure. RP-A501 is in Phase 2 clinical development.

3. Defendants provided investors with material information concerning RP-A501 including, among other things, confidence in the drug’s safety and efficacy, as well as the clinical trial’s detailed protocol and Rocket’s purported ability to meet the trial’s endpoints as per the Company’s ascribed timeline.

4. Throughout the Class Period, Defendants made materially false and misleading statements regarding the Company’s business, operations, and prospects. Specifically, Defendants made false and/or misleading statements and/or failed to disclose that: (i) RP-A501 was less effective than Defendants had led investors to believe; (ii) to increase its effectiveness, Rocket amended RP-A501’s clinical trial protocol by introducing a novel immunomodulatory agent; (iii) the foregoing increased the risk that patients would suffer from a Serious Adverse Event (“SAE”); (iv) accordingly, RP-A501’s safety, as well as its clinical, regulatory, and commercial prospects, were overstated; and (v) as a result, Defendants’ public statements were materially false and misleading at all relevant times.

5. On May 27, 2025, Rocket announced that the U.S. Food & Drug Administration (“FDA”) placed a clinical hold on the RP-A501 Phase 2 pivotal study after at least one patient suffered an SAE, ultimately, death, while enrolled in the

study following a substantive amendment to the protocol that the Company failed to disclose to investors at the time management made the revision. In fact, Rocket stated that, while the patient was dosed in May, the decision to amend the protocol was made “several months” earlier. Despite this, Rocket made no attempt to alert investors or the public to the change until after the SAE occurred.

6. On this news, Rocket’s stock price fell \$3.94 per share, or 62.84%, to close at \$2.33 per share on May 27, 2025.

7. Market analysts were quick to comment on the Company’s announcement. For example, on May 27, 2025, J.P. Morgan published a report entitled “Clinical Hold a Major Setback for Danon Pivotal and Shares,” which stated, in relevant part, that “[i]n our view, this morning’s announcement of a patient death in the pivotal phase 2 study of RP-A501 for Danon Disease and subsequent FDA placed clinical hold represents a major, perhaps insurmountable, setback for the program.”

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

JURISDICTION AND VENUE

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

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

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

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

PARTIES

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

14. Defendant Rocket is a Delaware corporation with principal executive offices located at 9 Cedarbrook Drive, Cranbury, New Jersey 08512. The

Company's common stock trades in an efficient market on the Nasdaq Global Market ("NASDAQ") under the ticker symbol "RCKT."

15. Defendant Gaurav Shah ("Shah") has served as Rocket's Chief Executive Officer and a Director of the Company at all relevant times.

16. Defendant Aaron Ondrey ("Ondrey") has served as Rocket's Chief Financial Officer at all relevant times.

17. Defendants Shah and Ondrey are collectively referred to herein as the "Individual Defendants."

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

19. Rocket and the Individual Defendants are collectively referred to herein as “Defendants.”

SUBSTANTIVE ALLEGATIONS

Background

20. Rocket, together with its subsidiaries, operates as a late-stage biotechnology company that focuses on developing gene therapies for rare and devastating diseases in the U.S. The Company develops *in vivo* AAV programs, including, *inter alia*, RP-A501 for the treatment of DD, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. RP-A501 is in Phase 2 clinical development.

Materially False and Misleading Statements Issued During the Class Period

21. The Class Period begins on September 17, 2024, when the Company issued a press release entitled “Rocket Pharmaceuticals Announces Completion of Enrollment in Phase 2 Pivotal Trial of RP-A501 for the Treatment of Danon Disease.” The press release stated, in relevant part:

Rocket [. . .] today announced that all patients have been enrolled in the global, pivotal Phase 2 clinical trial evaluating RP-A501 to treat male patients with Danon disease.

After the two-patient safety run-in, followed by harmonized global site activations, the remaining 10 patients were enrolled across the United States (U.S.) and European Union within three months. Given the prevalence of Danon disease across regions, the Company plans to pursue regulatory filings concomitantly in the U.S. and ex-U.S.

“From a clinical perspective, the important thing is that we are moving closer to the goal of having a treatment for patients with Danon disease,” said Barry H. Greenberg, MD, FHFSA, Director of the Advanced Heart Failure Treatment Program and Distinguished Professor of Medicine at UC San Diego Health. “I can attest to the excitement and anticipation within the Danon patient community for this novel, one-time treatment designed to improve cardiac abnormalities associated with Danon disease and help preserve normal cardiac function by delivering functional LAMP2B genes to the heart tissue. ***The rapid recruitment of the Phase 2 trial signifies the positive views of the study clinicians regarding this investigational therapy.***”¹

22. On November 7, 2024, Rocket issued a press release announcing the Company’s financial and operating results for the third quarter of 2024. The press release stated, in relevant part:

“Rocket made meaningful progress during the third quarter, notably with the completion of enrollment in the RP-A501 program for Danon disease, low dose cohort enrollment completion in the RP-A601 program for PKP2-ACM, and appointment of seasoned pharmaceutical executive, Mikael Dolsten to our Board of Directors,” said [Defendant] Shah[.] “As we continue to pursue our mission of seeking gene therapy cures for patients with rare and devastating diseases, we remain focused on expediently advancing our deep pipeline of cardiovascular and hematology programs.”

Recent Pipeline and Operational Updates

- Continued advancement of Phase 2 pivotal study of RP-A501 for [DD].
 - In September, Rocket announced completion of enrollment in the Phase 2 pivotal study of RP-A501 to treat Danon Disease.
 - Dosing in the Phase 2 pivotal study is ongoing.
 - Updated data from the Phase 1 study to be presented at the American Heart Association’s 2024 Late-Breaking Science sessions on November 18.

¹ All emphases included herein are added unless otherwise indicated.

23. On November 18, 2024, the Company issued a press release entitled “Rocket Pharmaceuticals Announces New England Journal of Medicine Publication of Phase 1 RP-A501 Long-Term Data and Presents at Late-Breaking Scientific Sessions at 2024 American Heart Association Conference.” The press release stated, in relevant part:

“Data presented today at AHA and published in The New England Journal of Medicine represents a critical milestone for the RP-A501 program and cardiac gene therapy in general, demonstrating for the first time that AAV conferred long-term efficacy in a cardiac indication. This program represents the most comprehensive investigational gene therapy dataset for any cardiac condition,” said [Defendant] Shah[.] ***“As is true for many other recent internal and peer company programs, when gene therapy works, it is life changing. RP-A501 is being developed as a potential one-time gene therapy and the results of the long-term Phase 1 study show the promise of gene therapy across cardiac diseases, including PKP2-ACM, BAG3-DCM and others.”***

“The long-term safety and efficacy results in the Phase 1 study are very encouraging for patients with Danon disease. In this study we found consistent, robust improvements and/or normalization across multiple quantifiable parameters that cardiologists use in clinical practice for assessing risk and making management decisions,” said Barry H. Greenberg, MD, FHFSA, Distinguished Professor of Medicine at University of California San Diego School of Medicine and Director of the Advanced Heart Failure Treatment Program at UC San Diego Health, primary investigator of the RP-A501 Phase 1 trial and primary author of the manuscript. ***“Currently, there are no other therapies that have been shown to demonstrate improvement of Danon disease-related cardiomyopathy, and while heart transplantation can prolong life, it is not curative and is associated with significant one-year mortality and complications. Data from this study shows promise for the Danon disease community.”***

24. On February 27, 2025, Rocket published fourth quarter and full year 2024 financial results and highlights regarding RP-A501. The press release stated, in relevant part:

“In 2024, we made strong progress in advancing our gene therapy pipeline, underscored by the *New England Journal of Medicine* publication of the Phase 1 study of RP-A501 for Danon disease and long-term data presented at AHA showing its safety and meaningful efficacy up to five years. Our momentum continues as we progress with the Phase 2 pivotal trial of RP-A501 and the Phase 1 trial of RP-A601 for PKP2-ACM, and we remain on track to submit the IND for BAG3-DCM in the first half of 2025,” said [Defendant] Shah[.] “Looking ahead to 2025, we will maintain our focus and resources on advancing our AAV cardiovascular programs while seeking to realize value in our full pipeline in a thoughtful manner, so we deliver the greatest value to our patients and shareholders.”

Recent Pipeline and Operational Updates

- **Dosing in the Phase 2 pivotal study of RP-A501 for Danon disease is ongoing.**
 - Details of the Phase 2 pivotal study can be found at www.ClinicalTrials.gov under NCT identifier NCT06092034.
 - Program update anticipated in the first half of 2025.
- **Long-term data from the Phase 1 study of RP-A501 for Danon disease published in *The New England Journal of Medicine* and new data presented at the American Heart Association’s 2024 Late-Breaking Science sessions.**
 - RP-A501 demonstrated safety and meaningful efficacy; all evaluable patients show cardiac LAMP2 expression and $\geq 10\%$ reduction in LV mass index at 12 months and sustained through most recent follow up (up to five years).
 - Evidence of sustained clinically meaningful improvement was observed in pediatric patients followed up to 24 months and adult/adolescent patients followed up to 60 months.

- All evaluable patients had reductions in NYHA heart failure (from Class II to Class I; no longer displaying symptoms of heart failure), improvements in KCCQ (median 27-point increase), and substantial improvements in troponin (median reduction 84%) and BNP (median reduction 57%) observed 24-54 months after treatment.

25. That same day, Rocket filed an Annual Report on Form 10-K with the SEC, reporting the Company's financial and operational results for the year ended December 31, 2024 (the "2024 10-K"). In providing an overview of the Company, the 2024 10-K stated, in relevant part, "[w]e are a fully integrated, late-stage biotechnology company focused on the development of first, *only and best in class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases.*"

26. Further, in discussing the Company's strategy, the 2024 10-K stated, in relevant part:

We seek to bring hope and relief to patients with devastating, undertreated and rare pediatric diseases through the development and commercialization of potentially curative first in class gene therapies. As a fully-integrated biotechnology company, we are well positioned to achieve these objectives. In the near and medium-term, we intend to develop our first-in-class product candidates, which target devastating diseases with substantial unmet need, develop proprietary in-house analytics and manufacturing capabilities and continue to conduct registration trials for our currently planned programs. In the medium and long-term, pending favorable data, we expect to submit BLAs for the rest of our suite of clinical programs, and establish our gene therapy platform and expand our pipeline to target additional indications that we believe to be potentially compatible with our gene therapy technologies. In addition, during that time, we believe that our currently planned programs will become eligible for priority review vouchers

from the FDA that provide expedited review. We have assembled a leadership and research team with expertise in cell and gene therapy, rare disease drug development, product approval and commercial launches.

We believe that our competitive advantage lies in our disease-based selection approach, a rigorous process to identify target diseases and ability to develop products to treat identified target diseases. We believe that this approach to asset development differentiates us as a gene therapy company and potentially provides us with a first-mover advantage and first-to-market of meaningful treatments for devastating, undertreated, and rare pediatric diseases.

27. Appended to the 2024 10-K as an exhibit was a signed certification pursuant to the Sarbanes-Oxley Act of 2002 by the Individual Defendants, attesting that “the information contained in the [2024 10-K] fairly presents, in all material respects, the financial condition and results of the Company.”

28. On May 8, 2025, Rocket published first quarter 2025 financial results and highlights regarding RP-A501, in relevant part:

Recent Pipeline and Operational Updates

- **Phase 2 pivotal study of RP-A501 for Danon disease is ongoing.**
 - Program update anticipated in mid-year 2025 and a clinical data readout expected in mid-year 2026. Details of the Phase 2 pivotal study can be found at www.ClinicalTrials.gov under NCT identifier NCT06092034.
 - In March, the largest longitudinal natural history study of Danon disease to date was published in the Journal of the American Heart Association (JAHA), revealing key insights into the distinct cardiac patterns of Danon disease patients, showing earlier, more severe heart issues in male patients, while also noting that many females develop

progressive cardiomyopathy and heart failure in adolescence or early adulthood.

29. The statements referenced in ¶¶ 21-28 were materially false and misleading because Defendants made false and/or misleading statements, as well as failed to disclose material adverse facts about Rocket’s business, operations, and prospects. Specifically, Defendants made false and/or misleading statements and/or failed to disclose that: (i) RP-A501 was less effective than Defendants had led investors to believe; (ii) to increase its effectiveness, Rocket amended RP-A501’s clinical trial protocol by introducing a novel immunomodulatory agent; (iii) the foregoing increased the risk that patients would suffer from a Serious Adverse Event (“SAE”)); (iv) accordingly, RP-A501’s safety, as well as its clinical, regulatory, and commercial prospects, were overstated; and (v) as a result, Defendants’ public statements were materially false and misleading at all relevant times.

The Truth Emerges

30. On May 27, 2025, Rocket published a press release providing an update on the Company’s Phase 2 Clinical Trial of RP-A501 for Danon Disease, including that a patient enrolled in the trial experienced an unexpected Serious Adverse Event (SAE). The press release stated, in relevant part:

A patient participating in the Phase 2 pivotal trial of RP-A501 experienced an unexpected Serious Adverse Event (SAE). The SAE involved clinical complications related to a capillary leak syndrome. Rocket is conducting a comprehensive root cause analysis and remains in active dialogue with the U.S. Food and Drug

Administration (FDA) and other key stakeholders, with the current focus being on the recent introduction of a novel immune suppression agent to the pre-treatment regimen that had been implemented to mitigate complement activation observed in some patients. This novel agent was specific to the AAV9-Danon program. Upon learning of the initial event, Rocket voluntarily paused further dosing in the study. On May 23, 2025, the FDA placed a clinical hold on the trial to allow for further evaluation. Rocket is deeply saddened to report that this patient has since passed away after an acute systemic infection.

Rocket is working with the FDA, the Independent Data Safety Monitoring Committee, clinical investigators, and scientific experts, and is committed to ensuring the safety of all study patients while resuming the trial as expeditiously as possible. ***While the clinical hold remains in place, the company is unable to provide guidance on the anticipated timing for completion of the Phase 2 trial.***

“We are heartbroken by this loss and are fully committed to our mission to develop gene therapies that address the underlying cause of devastating diseases like Danon. We are immensely grateful for the patients and families who participate in this important research,” said [Defendant] Shah[.]

31. The same day, Rocket hosted a special call to detail the RP-A501 updates, including the SAE that occurred during the Phase 2 trial (the “RP-A501 Call”). During the RP-A501 Call, Defendant Shah stated, in relevant part:

A patient enrolled in our Phase II pivotal trial experienced an unexpected serious adverse event and clinical complications related to capillary leak syndrome. Rocket is conducting a comprehensive root cause analysis and remains in active dialogue with the FDA and other key stakeholders with the current focus being on the recent introduction of a novel immune suppression agent to the pretreatment regimen that has been implemented to mitigate complement activation.

Now this novel agent was specific to the Danon program and not for PKP2, BAG3 or other programs. Following this initial SAE, Rocket proactively and voluntarily paused further dosing in the study out of an

abundance of caution. We immediately notified the U.S. FDA, and the FDA on May 23 placed the trial on clinical hold to allow for additional evaluation. ***Subsequently, the patient experienced additional medical and procedural complications during his hospital course and unfortunately passed away after a systemic infection.***

First and foremost, our thoughts are with the patient's family, caregivers and the treating clinical team. This is a deeply tragic loss, and we are committed to fully understanding their circumstances surrounding it objectively and neutrally. We are also immensely grateful to the family for their contribution to this important clinical research and their commitment to helping advance science for the broader Danon community.

Now as was shared in our press release and just now, there is an on-going and objective review to assess the root cause of the initial SAE. And as I mentioned, an area of focus is a recent protocol amendment that introduced a novel immunomodulatory agent to the pretreatment regimen. This change was implemented proactively to further enhance patient safety and was informed by the occurrence of complement activation earlier.

Rocket is carefully evaluating whether a mechanism related to the new agent may have influenced immune responses in an unexpected or paradoxical way. Again, this agent is specific to the Danon program and has not been used in PKP2, BAG3 or other programs. Also, these programs are not impacted by this clinical hold. We're working now with sites, external scientific experts and the FDA. And while the clinical hold remains in place, we're unable to provide guidance on the exact timing of completion of the Phase II trial.

32. As part of the RP-A501 Call, Rocket hosted a question-and-answer segment, wherein the Company's management responded to questions from analysts, in relevant part:

<Q: Joshua Elliott Schimmer - Cantor Fitzgerald & Co – Analyst>
Condolences to the family of the patient. I guess are you able to provide any additional details around this event in terms of when it occurred,

number one, what the specific immune or novel agent was that -- was added? I guess this is incremental to the steroid, sirolimus and rituximab.

And then are you able to provide any comments in terms of the number of patients treated in the program to date? And if the answer to any of those is no, when might we expect to hear?

<A: Defendant Shah> *Yes. So the patient was treated in early May. And the agent that was used was the C3 inhibitor, and it was introduced into this trial because there was ongoing evidence of complement activation in Danon disease. And we aim to try to completely eliminate any TMA risk altogether.* So at that time, this particular agent was coming into the market and also we had some experience in pediatric patients. So the timing was right to try to eradicate the risk of TMA altogether, not just for Danon, but potentially as a read-through to other AAV programs across our portfolio and others. So it was with that in mind that we introduced this novel agent.

Now I will say that we are considering that as one option, one thought, one idea for root cause. We're doing a comprehensive root cause analysis pretty neutral and objectively, and this is one idea. It's the current focus, just one idea. And in terms of the number of patients, we're not quite ready to comment on that, but as the protocol, it goes through the FDA, and we have discussions with them to resolve the hold, we'll be able to provide further guidance.

<Q: Mani Foroohar - Leerink Partners LLC – Analyst> I would add my condolences to the family of this patient. I want to follow up a little bit on Josh's question, which seem to be the most important. Can you give us a sense of when the decision was made to add this C3 inhibitor potentially to the protocol? Was this the only patient to receive this agent? And then I have a quick follow-up.

<A: Defendant Shah> *Yes. So the decision was made earlier several months ago to add this. And the -- there was one more additional patient who did receive this agent as well after this patient that we're talking about. And the second patient has had a much reduced*

course, has had evidence of capillary but has had a reduced course. What we were able to do here is learn from the first case and intervene so that we didn't see the same events happening in the second patient. So there are 2 cases like this now. And I will say that both of these cases are cases where this -- the only difference really was the introduction of this agent. So that's why that's one hypothesis that we're working with.

<Q: Mani Foroohar> Okay. And when you talk -- so is the right interpretation of that, that you had some number of patients, at least these 2, who have had capillary leak syndrome after dosing with the AAV, after receiving the other 3 agents and the immunomodulatory regimen that Josh [helpfully listed]. And then after that, at some point, they had capillary leak syndrome and then this novel agent was given, and then they had an acute infection? Or did the acute infection come prior to the CPS and the novel agent was given after? Could you clarify the order of events there?

<A: Defendant Shah> Sure. So yes, let's walk through the time line here in some granularity. *So these are the only 2 patients that have seen what we're calling a capillary leak syndrome, the ones that we're talking about here. Now this agent is given before the infusion. It's given a few doses after infusion as well. And it's given in conjunction with the other standard immunomodulatory regimen, including rituximab, sirolimus and steroids with hopefully rapid taper. So all of that is given together. And it was a full package that was intended to eradicate complement activation as well as any later T cell responses to really focus on the safety profile of these patients in the days and weeks after therapy.* Safety is, of course, our first priority here while we develop a full benefit-risk profile.

So in terms of the occurrence of the medical events, about -- so we did not see TMA. We did not see other gene therapy effects like myocarditis. *About a week after the infusion, that's when we started seeing some evidence of capillary leak.* There were other medical complications and procedural complications in the week or so afterwards. And actually the patient was, at that time, stable and doing potentially well enough that we were cautiously optimistic of recovery. And the capillary leak was improving. Unfortunately, over the weekend, over this past weekend, he developed an acute systemic

infection that accelerated his demise. That's the full sequence of events. And the clinical hold was placed on Friday -- the clinical hold was placed Friday just before this demise. So all of these -- the most severe events unfolded literally in the last 3 or 4 days.

<Q: Avraham Leib Novick - Morgan Stanley – Research Associate>
It's Avi Novick on the line for Mike. I guess are there any patients that have been enrolled in the study that have still not been infused with RP-A501? And I guess just as a quick follow-up, were there any previously dosed patients who have had complement-mediated adverse events?

<A: Defendant Shah> Yes. So there are patients who are still waiting to be treated. Our plan and the time line was such that we would have finished the treatment of these patients by midyear, which is when we were going to have the program update as we've guided to previously. So there were more patients left to be treated, all ready to go and were lined up to be treated shortly. Unfortunately, we had the setback that we're talking about today. So that's going to be paused.

Earlier in the trial, we did see complement activation and TMA. It was a gene therapy-associated effect that's part of trial safety benefit that we usually read out at the end of the trial. And those patients are actually now doing well from a safety and potentially even an efficacy viewpoint. But because it was part of routine explained side effects of gene therapy, we continue with the program. There was no clinical hold, and we modified the protocol in the way that I described earlier, to continue to further mitigate the risk.

<Q: Tyler Martin Van Buren - TD Cowen – Analyst> Since you discussed that this agent was introduced to reduce the incidence of TMAs, can you give more color on the incidence of TMAs that have been observed to date? Or any additional color on what you've seen so far in the trial?

<A: Defendant Shah> Yes. So there was an initial episode of TMA that was linked to a gene mutation, an additional gene mutation that confers

complement sensitivity in some patients. We modified the protocol with the 14 panel test to reduce the -- or to eliminate those patients from enrolling who would have those sorts of gene mutations that exacerbate complement.

There was another one that persisted. There was another TMA that we saw also. And TMA is a known risk of this therapy. We don't think that the risk is ever going to be zero. But in order to try to make it as close to zero as possible, we introduced this new inhibitor, and we're going to evaluate what finally happened here.

And I should also say that those patients who did have those early complement activations have completely recovered from the sequelae and are doing well right now.

<Q: Thibaut R. Pardo-García - LifeSci Capital, LLC, – Research Associate> This is Thibaut Pardo for Cory Jubinville. My condolences to the family of the patient. So we have -- we had up to 5 years of AEs data that were going pretty well. So this Phase I program, we had everything pretty controlled with -- once the update to include rituximab, sirolimus and prednisone combo. Why take that risk of introducing a novel agent?

<A: Defendant Shah> We always aim to provide the optimal experience for patients and really lean in on the benefit risk. And although at the time, we didn't know how rapidly these complement issues would and could resolve. So we worked with an abundance of caution putting patient safety first to make sure that other patients didn't necessarily have those experiences. Now since then, those patients, as I mentioned, have recovered fully from the complement activation issues. And I think that -- so we were trying to be extra careful with safety and trying to eliminate all risk altogether.

<Q: Gil Joseph Blum - Needham & Company, LLC, Research – Analyst> And allow me to add my condolences. So just to be perfectly, perfectly clear, the only event of capillary leak syndrome that we're

seeing were only when this agent was used? There was no evidence in any of the other patients?

<A: Defendant Shah> That's correct.

33. Market analysts were quick to comment on the Company's announcement. For example, in a report entitled "Clinical Hold a Major Setback for Danon Pivotal and Shares," J.P. Morgan stated, in relevant part:

In our view, this morning's announcement of a patient death in the pivotal phase 2 study of RP-A501 for Danon Disease and subsequent FDA placed clinical hold represents a major, perhaps insurmountable, setback for the program. With the Danon program being the primary focus for the Street, and little value being ascribed to the company's remaining pipeline, the pre-market reaction (shares down ~66% vs XBI up 1%) strikes us as appropriate. For reference, cash at the end of 1Q25 was ~\$3/share while our model forecasts a year end pro-forma ~\$2/share. While the company will seek to work with the FDA to understand what drove the patient's capillary leak syndrome (initially thought to be due to the novel immune pre-conditioning agent), we see a real possibility that the event changes the risk/ benefit dynamic for A501 such that the bar for registration is more conservative than the current pivotal design presently supports. While we expect better visibility on the path forward on the upcoming call at 8:30, we wouldn't be surprised by shares continuing to be under pressure at least through resolution of the clinical hold.

34. On this news, Rocket's stock price fell \$3.94 per share, or 62.84%, to close at \$2.33 per share on May 27, 2025.

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

SCIENTER ALLEGATIONS

36. During the Class Period, Defendants had both the motive and opportunity to commit fraud. They also had actual knowledge of the misleading nature of the statements they made, or acted in reckless disregard of the true information known to them at the time. In so doing, Defendants participated in a scheme to defraud and committed acts, practices, and participated in a course of business that operated as a fraud or deceit on purchasers of the Company's securities during the Class Period.

PLAINTIFF'S CLASS ACTION ALLEGATIONS

37. 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 Rocket securities during the Class Period (the "Class"); and were damaged upon the revelation of the alleged corrective disclosures. Excluded from the Class are Defendants herein, the officers and directors of the Company, at all relevant times, members of their immediate families and their legal representatives, heirs, successors or assigns and any entity in which Defendants have or had a controlling interest.

38. The members of the Class are so numerous that joinder of all members is impracticable. Throughout the Class Period, Rocket securities were actively traded on the NASDAQ. While the exact number of Class members is unknown to

Plaintiff at this time and can be ascertained only through appropriate discovery, Plaintiff believes that there are hundreds or thousands of members in the proposed Class. Record owners and other members of the Class may be identified from records maintained by Rocket 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.

39. 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.

40. 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.

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

- whether the federal securities laws were violated by Defendants' acts as alleged herein;
- whether statements made by Defendants to the investing public during the Class Period misrepresented material facts about the business, operations and management of Rocket;

- whether the Individual Defendants caused Rocket to issue false and misleading financial statements during the Class Period;
- whether Defendants acted knowingly or recklessly in issuing false and misleading financial statements;
- whether the prices of Rocket securities during the Class Period were artificially inflated because of the Defendants' conduct complained of herein; and
- whether the members of the Class have sustained damages and, if so, what is the proper measure of damages.

42. 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.

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

- Defendants made public misrepresentations or failed to disclose material facts during the Class Period;
- the omissions and misrepresentations were material;
- Rocket securities are traded in an efficient market;
- the Company's shares were liquid and traded with moderate to heavy volume during the Class Period;

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

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

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

COUNT I

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

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

47. 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.

48. 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 Rocket securities; and (iii) cause Plaintiff and other members of the Class to purchase or otherwise acquire Rocket securities and options at artificially inflated prices. In furtherance of this unlawful scheme, plan and course of conduct, Defendants, and each of them, took the actions set forth herein.

49. 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 Rocket 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 Rocket's finances and business prospects.

50. By virtue of their positions at Rocket, 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.

51. 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 Rocket, the Individual Defendants had knowledge of the details of Rocket's internal affairs.

52. 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 Rocket. 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 Rocket'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 Rocket securities was artificially inflated throughout the Class Period. In ignorance of the adverse facts concerning Rocket's business and financial condition which were concealed by Defendants, Plaintiff and the other members of the Class purchased or otherwise acquired Rocket securities at artificially inflated prices and relied upon the price of the securities, the integrity of the market for the securities and/or upon statements disseminated by Defendants, and were damaged thereby.

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

by Plaintiff and the Class, the true value of Rocket securities was substantially lower than the prices paid by Plaintiff and the other members of the Class. The market price of Rocket securities declined sharply upon public disclosure of the facts alleged herein to the injury of Plaintiff and Class members.

54. 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.

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

COUNT II

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

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

57. During the Class Period, the Individual Defendants participated in the operation and management of Rocket, and conducted and participated, directly and indirectly, in the conduct of Rocket's business affairs. Because of their senior

positions, they knew the adverse non-public information about Rocket's misstatement of income and expenses and false financial statements.

58. As officers and/or directors of a publicly owned company, the Individual Defendants had a duty to disseminate accurate and truthful information with respect to Rocket's financial condition and results of operations, and to correct promptly any public statements issued by Rocket which had become materially false or misleading.

59. 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 Rocket disseminated in the marketplace during the Class Period concerning Rocket's results of operations. Throughout the Class Period, the Individual Defendants exercised their power and authority to cause Rocket to engage in the wrongful acts complained of herein. The Individual Defendants, therefore, were "controlling persons" of Rocket 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 Rocket securities.

60. Each of the Individual Defendants, therefore, acted as a controlling person of Rocket. By reason of their senior management positions and/or being directors of Rocket, each of the Individual Defendants had the power to direct the

actions of, and exercised the same to cause, Rocket to engage in the unlawful acts and conduct complained of herein. Each of the Individual Defendants exercised control over the general operations of Rocket 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.

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

PRAYER FOR RELIEF

WHEREFORE, Plaintiff demands judgment against Defendants as follows:

- A. Determining that the instant action may be maintained as a class action under Rule 23 of the Federal Rules of Civil Procedure, and certifying Plaintiff as the Class representative;
- B. Requiring Defendants to pay damages sustained by Plaintiff and the Class by reason of the acts and transactions alleged herein;
- C. Awarding Plaintiff and the other members of the Class prejudgment and post-judgment interest, as well as their reasonable attorneys' fees, expert fees and other costs; and
- D. Awarding such other and further relief as this Court may deem just and proper.

DEMAND FOR TRIAL BY JURY

Plaintiff hereby demands a trial by jury.

Dated:
