UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of
The Securities Exchange Act of 1934

Date of Report (Date of earliest event reported)

February 24, 2022

Rocket Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**001-36829**	**04-3475813**
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

9 Cedarbrook Drive
Cranbury, NJ 08512
(Address of principal executive offices, including zip code)
(646) 440-9100
(Registrant's telephone number, including area code)
Not applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, $0.01 par value	RCKT	The Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02. Results of Operations and Financial Condition.

On February 24, 2022, Rocket Pharmaceuticals, Inc. (the "Company") announced its financial results for the quarter and year ended December 31, 2021. A copy of the press release issued in connection with the announcement is being furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Current Report on Form 8-K (including Exhibit 99.1 attached hereto) is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act") or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

99.1	Press Release, dated February 24, 2022
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Rocket Pharmaceuticals, Inc.

Date: February 24, 2022

By: /s/ Gaurav Shah, MD

Gaurav Shah, MD

Chief Executive Officer and Director

Exhibit 99.1

Rocket Pharmaceuticals Reports Fourth Quarter and Full Year 2021 Financial and Operational Results

— *Positive Phase 1 data from Danon Disease gene therapy trial demonstrated sustained benefit across clinical, functional and biomarker endpoints in all four adult patients with long-term follow up; pediatric cohort data expected Q3 2022* —

— *Reported engraftment in all nine severe LAD-I patients treated in pivotal Phase 2 trial and CD18 expression ranging from 26% to 87% of normal; top-line data expected Q2 2022*—

— *Demonstrated evidence of engraftment in six Fanconi Anemia patients with at least 12-months of follow-up with MMC resistance between 16% and 63% at a minimum of one timepoint in pivotal Phase 2 trial; top-line data expected Q3 2022* —

— Cash position of $388.7M; runway expected into 2H'23 —

CRANBURY, N.J. – **Feb. 24, 2022** – Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today reports financial and operational results for the fourth quarter and year ended December 31, 2021.

"2021 was a very productive year marked by clinical data supporting potential durable cures in three devastating bone marrow-derived diseases, and for the first time in history by the demonstration of potential durable clinical benefit in a cardiac disease using a gene therapy approach," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. "The stage is set for 2022 to be an important year for Rocket in which we anticipate progression of the Danon Disease program toward a registrational trial, as well as top-line data from our pivotal LAD-I and Fanconi Anemia clinical trials that move us toward registration. I am growing increasingly confident about our unique gene therapy approach at Rocket focused on addressing specific rare diseases using highly targeted, enabled technologies with a proven track record. Additionally, we anticipate in-house AAV cGMP manufacturing readiness in the second quarter of this year, as we rapidly scale our capabilities and work towards commercial readiness."

Dr. Shah continued, "I am extremely proud of the positive momentum witnessed in 2021 and our steadfast focus on superior execution. Notably, we announced positive data from our Phase 1 trial in Danon Disease showing clinical, functional and biomarker improvements at one year or beyond, and we initiated treatment in the pediatric patient cohort at the low-dose, with pediatric data anticipated in the third quarter of 2022. In our pivotal Phase 2 trials in LAD-I and Fanconi Anemia, the favorable safety and efficacy observed are very encouraging as we approach top-line data readouts. We were also pleased with the progress of our PKD program demonstrating sustained normal-range hemoglobin through 12-months post-treatment in two adult patients in the Phase 1 trial. As we continue accelerating our current first wave of clinical programs, we are also poised to grow our Wave 2 pipeline based on our core R&D strategy from this year onward. We are excited by the momentum and look forward to continuing on our mission of seeking gene therapy cures for young patients and their families facing such devastating diseases."

Key Pipeline and Operational Updates

- **Announced positive clinical data from ongoing Phase 1 trial of RP-A501 for Danon Disease.** In November 2021, the Company reported updated data from the Phase 1 trial evaluating a single intravenous infusion of RP-A501 for the treatment of Danon Disease, including interim safety and efficacy data from patients in the low-dose (6.7e13 vg/kg; n=3) and high-dose (1.1e14 vg/kg; n=2) adult and adolescent cohorts. Results demonstrated sustained benefit across clinical, functional and biomarker endpoints in all four patients with long-term follow up. RP-A501 was generally well tolerated at the low-dose. Data from the pediatric patient cohort of the Phase 1 trial are expected in the third quarter of 2022 and Phase 2 trial activities are expected to begin in the fourth quarter of 2022.

- **Announced positive clinical data from ongoing pivotal Phase 2 trial of RP-L201 for Leukocyte Adhesion Deficiency-I (LAD-I).** The Company provided an update in its presentation at the 40th Annual J.P. Morgan Healthcare Conference, indicating engraftment in all nine severe LAD-I patients who have received RP-L201 in the pivotal Phase 2 trial; all patients had at least three months of follow-up and demonstrated CD18 expression ranging from 26% to 87% of normal. The most recently treated patient has engrafted with neutrophil CD18 expression of 61% at three months. The Company previously presented preliminary data from eight of nine severe LAD-I patients who received RP-L201 treatment at the 63rd American Society of Hematology (ASH) Annual Meeting. Top-line data from the pivotal Phase 2 trial are expected in the second quarter of 2022.

- **Announced positive clinical data from ongoing pivotal Phase 2 trial of RP-L102 for Fanconi Anemia (FA).** Data presented at the 63rd ASH Annual Meeting included preliminary data from 11 pediatric FA patients who were treated with RP-L102 as of the Nov. 1, 2021, cut-off date. Evidence of engraftment was observed in six of eight patients with at least 12-months of follow-up with MMC resistance between 16% and 63% at a minimum of one timepoint. The tolerability profile of RP-L102 appeared favorable and all patients were treated without conditioning. Top-line data from the pivotal Phase 2 trial are expected in the third quarter of 2022.

- **Announced positive clinical data from ongoing Phase 1 trial of RP-L301 for Pyruvate Kinase Deficiency (PKD).** Data presented at the 63rd ASH Annual Meeting included preliminary data from two adult PKD patients with significant anemia and transfusion requirements who were treated with RP-L301 as of the Nov. 3, 2021, cut-off date. RP-L301 was well tolerated, with no drug product-related serious adverse events or infusion-related complications observed through 12-months post-treatment. Preliminary clinical activity was observed in both patients at 12-months post-RP-L301 infusion. Both patients reported improved quality of life following treatment. Preliminary Phase 1 data and Phase 2 trial initiation activities are expected in the fourth quarter of 2022.

- **Received Regenerative Medicine Advanced Therapy (RMAT) designation and Priority Medicines (PRIME) designation for RP-L201 gene therapy for the treatment of LAD-I.** RMAT designation allows companies to work closely with the U.S. Food and Drug Administration (FDA) on a program’s development and includes all the benefits of the FDA's Fast Track and Breakthrough Therapy designations. The European Medicine Agency’s (EMA) PRIME program aims to optimize development plans and speed up evaluation of medicines that may offer a major therapeutic advantage over existing treatments or benefit patients without treatment options. These medicines are considered priority medicines by the EMA and are intended to reach patients earlier. Rocket also holds Rare Pediatric (U.S.), Orphan Drug (U.S./EU), and Advanced Therapy Medicinal Product (EU) designations for the RP-L201 program.

- **Strengthened leadership team with new appointments.** The Company announced the appointment of Isabel Carmona, J.D., as Chief Human Resources Officer in September 2021. She has more than 25 years of experience in human resources and organizational leadership, including the last 15 years within the life sciences industry. Ms. Carmona joined Rocket from Ichnos Sciences, where she was Chief Human Resources Officer. Martin L. Wilson joined Rocket as General Counsel and Chief Compliance Officer in December 2021. Mr. Wilson has nearly 20 years of legal, compliance and executive experience and accomplishment within the life sciences industry. Before Rocket, Mr. Wilson was General Counsel and Chief Corporate Officer at Ichnos Sciences.

- **Continued buildout of R&D and manufacturing facility.** The Company expects to achieve in-house AAV current Good Manufacturing Practice (cGMP) manufacturing readiness in its approximately 100,000-square-foot facility in Cranbury, NJ in the second quarter of 2022.

- **Continued our commitment to the patient community.** In February 2021, the Company recognized Rare Disease Day by leading the "Light Up for Rare" campaign and hosting a month-long series of virtual expert panels to provide educational resources for patients with rare diseases and their families. On February 28, 2021, global landmarks including the Empire State Building and Niagara Falls were lit to drive awareness and foster solidarity among the global rare disease community.

- **Closed $26.4 million private placement.** In August 2021, the Company closed a private placement with a fund affiliated with RTW Investments, LP, the Company's largest shareholder, for which the net proceeds are expected to be used to continue to advance and expand Rocket's pipeline of product candidates, for research and development expenses and for working capital.

Anticipated 2022 Milestones

RP-A501 for Danon Disease (AAV)

- Report data from pediatric patient cohort of Phase 1 trial – Q3 2022

- Initiate pivotal Phase 2 trial activities – Q4 2022

RP-L201 for Leukocyte Adhesion Deficiency-I (LVV)

- Report top-line data from pivotal Phase 2 trial – Q2 2022

RP-L102 for Fanconi Anemia (LVV)

- Report top-line data from pivotal Phase 2 trial – Q3 2022

RP-L301 for Pyruvate Kinase Deficiency (LVV)

- Report preliminary Phase 1 data – Q4 2022

- Initiate pivotal Phase 2 trial activities – Q4 2022

Manufacturing Facility in Cranbury, New Jersey

- Achieve in-house AAV current Good Manufacturing Practice (cGMP) manufacturing readiness – Q2 2022

Upcoming Investor Conferences

- Cowen's 42nd Annual Health Care Conference – March 7-9, 2022
- Guggenheim Genomic Medicines & Rare Disease Day – March 31-April 1, 2022
- Needham 21st Annual Virtual Healthcare Conference – April 11-14, 2022

Fourth Quarter and Full Year 2021 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of December 31, 2021, were $388.7 million.
- **R&D expenses.** Research and development expenses were $32.2 million and $125.5 million for the three and twelve months ended December 31, 2021, compared to $50.1 million and $105.4 million for the three and twelve months ended December 31, 2020. The increase in research and development expenses for the three and twelve months ended December 31, 2021, was primarily driven by an increase in manufacturing and development costs, compensation and benefits expense due to increased R&D headcount and an increase in non-cash stock compensation expense, offset by a decrease in non-cash new research agreements expenses.
- **G&A expenses.** General and administrative expenses were $12.2 million and $41.8 million for the three and twelve months ended December 31, 2021, compared to $9.1 million and $28.9 million for the three and twelve months ended December 31, 2020. The increase in general and administrative expenses for three and twelve months ended December 31, 2021, was primarily driven by an increase in non-cash stock compensation expense, an increase in compensation and benefits expense due to increased G&A headcount, an increase in office and administrative costs, and an increase in commercial preparation expenses, offset by a decrease in debt conversion expense.
- **Net loss.** Net loss was $44.2 million and $169.1 million or $0.69 and $2.67 per share (basic and diluted) for the three and twelve months ended December 31, 2021, compared to $60.9 million and $139.7 million or $1.08 and $2.52 per share (basic and diluted) for the three and twelve months ended December 31, 2020.
- **Shares outstanding.** 64,505,889 shares of common stock were outstanding as of December 31, 2021.

Financial Guidance

- **Cash position.** As of December 31, 2021, we had cash, cash equivalents and investments of $388.7 million. Rocket expects such resources will be sufficient to fund its operations into the second half of 2023, including the continued buildout and initiation of AAV cGMP manufacturing capabilities at our Cranbury, New Jersey R&D and manufacturing facility and continued development of our four clinical programs.

About Rocket Pharmaceuticals, Inc.
Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of genetic therapies that correct the root cause of complex and rare childhood disorders. The Company's platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients afflicted with rare genetic diseases. Rocket's clinical programs using lentiviral vector (LVV)-based gene therapy are for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer, Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal, and Pyruvate Kinase Deficiency (PKD), a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia. Rocket's first clinical program using adeno-associated virus (AAV)-based gene therapy is for Danon Disease, a devastating, pediatric heart failure condition. For more information about Rocket, please visit www.rocketpharma.com.

Rocket Cautionary Statement Regarding Forward-Looking Statements
Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding its guidance for 2022 in light of COVID-19, the safety and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), and Danon Disease, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, Rocket's plans for the advancement of its Danon Disease program following the lifting of the FDA's clinical hold and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, our expectations regarding the delays and impact of COVID-19 on clinical sites, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, filed November 5, 2021 with the SEC. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

Selected Financial Information
Operating Results:
(amounts in thousands, except share and per share data)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 32,161	$ 50,093	$ 125,476	$ 105,438
General and administrative	12,171	9,144	41,772	28,865
Total operating expenses	44,332	59,237	167,248	134,303
Loss from operations	(44,332)	(59,237)	(167,248)	(134,303)
Research and development incentives	500	-	1,000	-
Interest expense	(463)	(1,640)	(2,977)	(6,967)
Interest and other income net	849	235	3,068	2,150
Amortization of premium on investments - net	(801)	(274)	(2,912)	(580)
Net loss	$ (44,247)	$ (60,916)	$ (169,069)	$ (139,700)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.69)	$ (1.08)	$ (2.67)	$ (2.52)
Weighted-average common shares outstanding - basic and diluted	64,470,930	56,284,599	63,235,417	55,380,740

	December 31, 2021	December 31, 2020
Cash, cash equivalents and investments	388,740	482,719
Total assets	497,020	590,824
Total liabilities	42,296	87,305
Total stockholders' equity	454,724	503,519

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