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)
August 9, 2021

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 August 9, 2021, Rocket Pharmaceuticals, Inc. (the "Company") announced its financial results for the quarter ended June 30, 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 August 9, 2021
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: August 9, 2021

By: /s/ Gaurav Shah, MD
Gaurav Shah, MD
Chief Executive Officer and Director

Exhibit 99.1

Rocket Pharmaceuticals Reports Second Quarter 2021 Financial Results and Highlights Recent Progress

— Working with FDA on Changes to Clinical Trial Protocol in Danon Disease Trial; Rocket Anticipates Trial Will Resume in 3Q—

— Increasing and Durable Benefit Observed in Low Dose Cohort (6.7e13 dose) in Danon Disease; Removing Higher Dose Cohort (1.1e14 dose) From Future Dosing Plans; Full Clinical Update Expected in 4Q –

— Positive Results from FA, LAD-I, PKD Trials Presented at 24th ASGCT Annual Meeting Show Preliminary Evidence of Activity and Favorable Tolerability —

— Clinical Updates in FA, LAD-I, PKD and IMO Also Expected in 4Q —

— Ending Balance Sheet with $426.8 Million in Cash; Cash Runway Expected into 2H'23 —

— Conference Call to Be Held at 4:30 p.m. ET Today—

CRANBURY, NJ – **Aug. 9, 2021** – 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 results for the quarter ending June 30, 2021 and updates on the Company's key pipeline developments, business operations, and upcoming milestones.

"We are grateful to the FDA for its support and for working with us on our Danon program toward resuming our trial, which we believe will occur in the third quarter," said Gaurav Shah, M.D., chief executive officer of Rocket Pharma. "Further, we have observed durable clinical benefit in the low dose adult cohort, which we believe is supportive of its potential as a viable Phase 2 dose. As of July 2021, we see improvement in two of three low dose patients in NYHA class. In these two patients, we also observed substantial improvement of a key marker of heart failure, BNP, which decreased from a pretreatment baseline by 75 percent in one patient and 79 percent in the other as well as improvement in cardiac output by 35 percent in one patient and 62 percent in the other as measured by invasive hemodynamics. The third patient has demonstrated stabilization of NYHA class and BNP. Given the positive benefit/risk profile in the low dose, and additionally to mitigate safety concerns observed at the higher dose, in agreement with FDA we will no longer treat Danon patients with the higher dose (1.1e14). With our full focus on the low dose, we look forward to progressing our trial on behalf of Danon patients devastated by this disease."

Dr. Shah continued, "We also presented positive data across three of our lentiviral-based gene therapy programs at ASGCT in May, which we believe support the growing potential of these programs to treat Fanconi Anemia (FA), LAD-1 and PKD patients. Based on these results, we continue our momentum toward advancing these programs. In the case of our FA program, two recent publications reinforce the natural history, clinical design and methods being utilized in our Phase 1/2 FA trial of RP-L102. We look forward to providing updates on all five of our programs in the fourth quarter of 2021."

Dr. Shah added, “Finally, and importantly, we are deeply saddened that the first patient dosed in our Phase 1 IMO trial, has passed way from pulmonary hemorrhage related to thrombocytopenia following conditioning therapy and also related to underlying osteopetrosis. This event was considered likely not related to RP-L401 gene therapy. Consistent with the trial protocol, enrollment has been temporarily paused pending a comprehensive evaluation in collaboration with the Independent Data Monitoring Committee, which will include a review of the conditioning regimen and other potential safety measures to mitigate the impact of underlying disease on treatment. This outcome underscores the need to find cures for this devastating disease and has furthered our commitment and dedication to patients and our mission to develop curative gene therapies for rare disease.”

Key Pipeline and Operational Updates

Danon Disease:

- **Progressed toward agreement with FDA on changes to the Phase 1 clinical trial protocol in Danon Disease. Rocket anticipates trial may resume this quarter.** The U.S. Food and Drug Administration (FDA) had previously requested Rocket pause patient dosing in the Phase 1 clinical trial of RP-A501 and modify the protocol and other supporting documents with revised guidelines for patient selection and management. No new drug-related safety events were observed in the low- or higher-dose adult cohorts; the previously disclosed SAE of thrombotic microangiopathy, which has since resolved, was reclassified as a SUSAR. All follow-up study activities continue. Longer-term results from the low (6.7e13) and higher dose (1.1e14) adult cohorts will be reported in the fourth quarter. In agreement with FDA, Rocket will no longer continue dosing patients at the higher dose (1.1e14).

Fanconi Anemia (FA):

- **Presented positive clinical updates from RP-L102 Fanconi Anemia (FA) program at ASGCT.** Preliminary results from the Phase 1 and 2 trials presented in a poster at ASGCT are from nine pediatric patients. For RP-L102, Rocket’s *ex vivo* lentiviral gene therapy candidate for FA, increasing evidence of engraftment was observed in at least six of the nine patients, including two patients with at least 15-months of follow-up and four patients with at least 6-months of follow-up. A highly favorable tolerability profile was also observed with all subjects being treated without conditioning and with no reports of dysplasia. One patient experienced a Grade 2 transient infusion-related reaction. The full data presented are available here.

- **Published two peer-reviewed studies supporting the natural history and clinical design of FA clinical trial.** “Natural gene therapy by reverse mosaicism leads to improved hematology in Fanconi anemia patients” was published in the *American Journal of Hematology*. Data strengthen the natural history of Fanconi Anemia and indicate that reverse mosaicism is a good prognostic factor in FA and is associated with more favorable long-term clinical outcomes. FA mosaicism in hematopoietic cells is a biologic and clinical proof-of principle for autologous gene therapy in FA patients and results provide a compelling rationale for continued clinical evaluation of autologous gene therapy. Additionally, “Improved Collection of Hematopoietic Stem Cells and Progenitors from Fanconi Anemia Patients for Gene Therapy Purposes” was published in *Molecular Therapy: Methods & Clinical Development*. Results demonstrate the safety and efficacy of filgrastim and plerixafor for mobilization of hematopoietic stem and progenitor cells (HSPCs) and collection by leukapheresis in FA patients, offering crucial information for the enrollment of FA patients for gene therapy studies.

Infantile Malignant Osteopetrosis (IMO):

- **First patient dosed in the RP-L401 Infantile Malignant Osteopetrosis (IMO) Phase 1 clinical trial passed away from likely non-RP-L401 gene therapy related pulmonary complications.** IMO is a bone marrow-derived disorder associated with severe bone and hematologic manifestations leading to death in the first decade of life, frequently within the first two years of life, without an allogenic hematopoietic stem cell transplant (HSCT).

  The first patient in the Phase 1 study, a six-year-old child with severe IMO-related anemia and bone abnormalities, was infused with RP-L401 without immediate complications. During the initial weeks after therapy, the patient died of pulmonary complications, most likely pulmonary hemorrhage related to thrombocytopenia following conditioning therapy and also related to underlying osteopetrosis. Pulmonary hemorrhage is a rare but documented complication of HSCT, and pulmonary complications, including life-threatening and fatal complications, have been observed to occur with high frequency in osteopetrosis patients undergoing allogeneic HSCT procedures.

  The patient death is not considered to be RP-L401-related by study investigators and as corroborated by autopsy findings. In accordance with the trial protocol, enrollment has been temporarily paused pending a comprehensive evaluation in collaboration with the Independent Data Monitoring Committee.

Leukocyte Adhesion Deficiency-I (LAD-I):

- **Presented positive clinical updates from RP-L201 Leukocyte Adhesion Deficiency-I (LAD-I) program at ASGCT.** Phase 1/2 data presented in an oral presentation at ASGCT are from four pediatric patients with severe LAD-I. RP-L201, Rocket's *ex-vivo* lentiviral gene therapy candidate showed preliminary activity in all four patients, including one patient with 18-months of follow-up and one patient with 9-months of follow-up. CD18 expression substantially exceeded the 4-10% threshold in all four patients, which is associated with survival into adulthood and consistent with the reversal of severe LAD-I phenotype. Most importantly, all four patients were able to leave the hospital in the weeks following RP-L201 therapy. The full data presented are available here.

Pyruvate Kinase Deficiency (PKD):

- **Presented positive clinical updates from RP-L301 Pyruvate Kinase Deficiency (PKD) program at ASGCT.** Updated preliminary Phase 1 data presented in an oral presentation at ASGCT are from two patients with significant anemia and transfusion requirements that showed sustained tolerability. Preliminary activity, measured by peripheral blood VCN levels, was observed in both patients during the initial 9-months and 3-months post-treatment, respectively. Durable normalization of hemoglobin levels were observed, from an average baseline of ~7.4 grams (g)/deciliter (dL) to 13.1 g/dL at 9-months post treatment in the first patient and from a baseline of ~7.0 g/dL to 14.4 g/dL at 6-months post treatment in the second patient. The Phase 1 trial continues to enroll patients with longer-term data expected in the fourth quarter. The full data presented are available here.

Anticipated Milestones

- Fanconi Anemia (RP-L102)
  - Updated “Process B” data (Q4 2021)
- LAD-I (RP-L201)
  - Longer-term Phase 2 data (Q4 2021)
- Danon Disease (RP-A501)
  - Longer-term Phase 1 data (Q4 2021)
- PKD (RP-L301)
  - Longer-term Phase 1 data (Q4 2021)
- IMO (RP-L401)
  - Phase 1 clinical update (Q4 2021)

Upcoming Investor Conference

- Citi’s 16th Annual BioPharma Virtual Conference, Sept. 8-10, 2021

Second Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of June 30, 2021 were $426.8 million.
- **R&D expenses.** Research and development expenses were $24.8 million for the three months ended June 30, 2021, compared to $16.7 million for the three months ended June 30, 2020, due to an increase in compensation and benefits expense resulting from increased R&D headcount, an increase in non-cash stock compensation expense, an increase in manufacturing and development costs, and an increase in clinical trials expense.
- **G&A expenses.** General and administrative expenses were $9.3 million for the three months ended June 30, 2021, compared to $6.8 million for the three months ended June 30, 2020, due to an increase in non-cash stock compensation expense, an increase in compensation and benefits expense due to increased G&A headcount and an increase in office and administrative costs.
- **Net loss.** Net loss was $34.5 million or $0.55 per share (basic and diluted) for the three months ended June 30, 2021, compared to $25.0 million or $0.45 per share (basic and diluted) for the three months ended June 30, 2020.
- **Shares outstanding.** 63,448,069 shares of common stock were outstanding as of June 30, 2021.

Financial Guidance

- Rocket expects its balance in cash, cash equivalents and investments of $426.8 million as of June 30, 2021 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 five clinical programs.

Conference Call Details
Rocket management will host a conference call today at 4:30 p.m. ET. To access the call and webcast, please visit the events section of the website. The webcast replay will be available on the Rocket website following the completion of the call.

Investors may access the conference call by dialing (866) 939-3921 from locations in the United States or +1 (678) 302-3550 from outside the United States. Please refer to conference ID number 50210581.

About Rocket Pharmaceuticals, Inc.
Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket") 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, Pyruvate Kinase Deficiency (PKD) a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia and Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. 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 2021 in light of COVID-19, the safety, effectiveness and timing of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, the actions of the FDA regarding the clinical hold on Rocket's Danon Disease program 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 Annual Report on Form 10-K for the year ended December 31, 2020, filed March 1, 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 June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 24,798	$ 16,731	$ 53,340	$ 33,687
General and administrative	9,250	6,828	19,930	13,990
Total operating expenses	34,048	23,559	73,270	47,677
Loss from operations	(34,048)	(23,559)	(73,270)	(47,677)
Research and development incentives	-	-	500	-
Interest expense	(251)	(1,786)	(1,980)	(3,360)
Interest and other income net	501	429	1,412	1,395
Amortization of premium on investments - net	(727)	(124)	(1,366)	(62)
Net loss	$ (34,525)	$ (25,040)	$ (74,704)	$ (49,704)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.55)	$ (0.45)	$ (1.20)	$ (0.90)
Weighted-average common shares outstanding - basic and diluted	63,061,232	55,158,459	62,321,926	55,020,789

	June 30, 2021	December 31, 2020
Cash, cash equivalents and investments	426,830	482,719
Total assets	535,154	590,824
Total liabilities	45,717	87,305
Total stockholders' equity	489,437	503,519

Contacts:

Media
Kevin Giordano
Director, Corporate Communications
kgiordano@rocketpharma.com

Investors
Mayur Kasetty, M.D.
Director, Business Development & Operations
investors@rocketpharma.com