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 27, 2023

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)	(Zip Code)

Registrant's telephone number, including area code: (646) 440-9100

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 27, 2023, Rocket Pharmaceuticals, Inc. announced its financial results for the quarter and year ended December 31, 2022. 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 27, 2023
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 27, 2022

By: /s/ Gaurav Shah, MD

Gaurav Shah, MD

Chief Executive Officer and Director

Rocket Pharmaceuticals Reports Fourth Quarter and Full Year 2022 Financial and Operational Results

Advanced pipeline of four clinical programs across AAV cardiology and LV hematology portfolios delivering strong results for Danon Disease, Fanconi Anemia, LAD-I and PKD

Expanded leadership position in AAV cardiac gene therapy, with significant opportunities for value creation across Danon Disease, PKP2-ACM and BAG3-DCM programs

Established in-house cGMP manufacturing for AAV, poised to support Danon pivotal trial in Q2'23 and subsequent commercialization

Cash, cash equivalents and investments of approximately $400M; expected operational runway through 2024

CRANBURY, N.J. – **Feb. 27, 2023** – Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare disorders with high unmet need, today reported financial and operational results for the fourth quarter and year ended December 31, 2022.

"Rocket closed 2022 with positive results across four clinical gene therapy programs spanning both our AAV cardiology and LV hematology therapeutic areas in an unprecedented fashion for a gene therapy company, validating our approach to strong science and R&D, carefully selected assets and smart execution. We are thrilled to begin 2023 with the FDA recently granting RMAT designation to RP-A501 for Danon Disease, validating the strength of our results and underscoring RP-A501's potential as a transformative therapy for Danon patients," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma.

Dr. Shah continued, "At the same time, we expanded our leadership position in AAV cardiac gene therapy with the recent unveiling of compelling preclinical proof of concept for RP-A601 for the treatment of PKP2 arrhythmogenic cardiomyopathy (PKP2-ACM) and addition of the BAG3-associated dilated cardiomyopathy (DCM) asset following the acquisition of Renovacor. Taken together, these two diseases along with Danon Disease affect more than 100,000 patients in the U.S. and EU. Further, our LV hematology portfolio delivered stellar results as we now advance towards our first regulatory filings for Leukocyte Adhesion Deficiency (LAD-I) in the second quarter of 2023 and Fanconi Anemia (FA) in the fourth quarter of 2023, and subsequent commercialization."

"I am also pleased that we brought in additional funds of $197.7 million in 2022. We begin this year in a strong financial position to execute on near and long-term milestones, anticipating that our cash runway of approximately $400 million will fund operations through 2024," said Dr. Shah. "I am grateful to our growing Rocket team of multi-disciplinary experts, scientific collaborators and the critical voices of the patient community who were essential to this progress and continue to help advance our mission of seeking gene therapy cures. I look forward to building off the successes of 2022 and continuing our progress in 2023."

Key Pipeline and Operational Updates

- **Announced positive clinical data from Phase 1 trial of RP-A501 for Danon Disease.** The Company provided an update at the 41st Annual J.P. Morgan Healthcare Conference that continued to demonstrate RP-A501 was generally well tolerated with evidence of restored expression of the deficient LAMP2 protein and durable improvement or stabilization of clinical parameters in the adult and pediatric Danon Disease patients treated in the Phase 1 study. The Company previously presented positive data from the study at the Heart Failure Society of America (HFSA) Annual Scientific Meeting 2022. The FDA recently granted Regenerative Medicine Advanced Therapy (RMAT) designation to RP-A501 that will provide the benefits of added intensive FDA guidance and expedited review through the program's development. The initiation of the Phase 2 pivotal trial is on track for the second quarter of 2023. As previously disclosed, the Company anticipates pursuing a single arm, open-label trial with a biomarker-based composite endpoint and a natural history comparator.

- **Successfully completed two in-house AAV production runs at state-of-the-art, Current Good Manufacturing Practice (cGMP) manufacturing and R&D facility in Cranbury, N.J.** In-house production runs of AAV drug product for the planned Phase 2 pivotal study in Danon Disease resulted in high-quality drug substance enabling an approximately threefold increase in the number of patients treatable per batch, a significantly improved full versus empty particle ratio, and promising product comparability data generated to date compared to the Phase 1 material manufactured externally.

- **Announced positive top-line data from Phase 2 pivotal trial of RP-L201 for Leukocyte Adhesion Deficiency-I (LAD-I).** Positive top-line data presented at the 2022 Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) and at the 64th American Society of Hematology (ASH) Annual Meeting demonstrated 100% overall survival at 12 months post-infusion via Kaplan Meier estimate and a statistically significant reduction in all hospitalizations, infection- and inflammatory-related hospitalizations and prolonged hospitalizations for all nine LAD-I patients with three to 24 months of available follow-up. Data also showed evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities. The Company recently presented data at the Tandem Meetings of the American Society for Transplantation and Cellular Therapy (ASTCT) and Center for International Blood & Marrow Transplant Research (CIBMTR) in February 2023 demonstrating all patients sustained neutrophil CD18 restoration and expression of more than 10% (range: 15%-89%, median: 51%), as of the November 2, 2022, cut-off date. At the congress, the RP-L201 program received first place in the abstract awards competition. Based on the data from the Phase 2 pivotal LAD-I trial, Rocket anticipates submitting a Biologics License Application (BLA) in the second quarter of 2023.

- **Announced positive top-line data from Phase 2 pivotal trial of RP-L102 for Fanconi Anemia (FA).** Data presented at the 64th ASH Annual Meeting (cut-off October 26, 2022), indicated that RP-L102 demonstrated phenotypic correction in at least six of 10 evaluable patients with ≥12 months of follow-up as demonstrated by increased resistance to mitomycin-C (MMC) in bone marrow (BM)-derived colony forming cells, concomitant genetic correction and hematologic stabilization. A seventh patient has displayed evidence of progressively increasing genetic correction as demonstrated by peripheral blood and BM vector copy numbers (VCNs), with recent development of MMC resistance and indicators of hematologic stability after 36 months of follow-up. Data demonstrated increased resistance to MMC in five patients at least at two timepoints, ranging from 74% to 94% at the most recently evaluated timepoint. The safety profile of RP-L102 was highly favorable, and the treatment, administered without any cytotoxic conditioning, was well tolerated. The Company recently presented these data at the Tandem Meetings of ASTCT and CIBMTR in February 2023 and the RP-L102 program received second place in the abstract awards competition. Based on the data from the Phase 2 pivotal Fanconi Anemia trial, Rocket anticipates submitting a BLA in the fourth quarter of 2023.

- **Announced positive clinical data from Phase 1 trial of RP-L301 for Pyruvate Kinase Deficiency (PKD).** Data presented at the 64th ASH Annual Meeting (cut-off October 26, 2022) demonstrated that both patients at 24 months post-RP-L301 infusion had robust and sustained efficacy demonstrated by normalized hemoglobin (from baseline levels in the 7.0-7.5 g/dL range), improved hemolysis parameters, independence from red blood cell transfusions and improved quality of life both reported anecdotally and as documented via formal quality of life assessments. The safety profile appears highly favorable, with no RP-L301-related serious adverse events through 24 months post-infusion in both adult patients. Adult and pediatric enrollment is completed in the Phase 1 study. The company recently presented these data at the Tandem Meetings of ASTCT and CIBMTR in February 2023. Phase 2 pivotal trial initiation is anticipated in the fourth quarter of 2023.

- **Raised $197.7M to develop full pipeline of assets.** During 2022, the Company completed a follow-on offering of common stock for net proceeds of $108.1 million, sold shares of common stock for net proceeds of $46.6 million pursuant to the at-the-market offering program and acquired cash and cash equivalents of $43.0 million as part of the acquisition of Renovacor.

- **Acquired Renovacor, extending leadership in AAV-based cardiac gene therapy.** In December 2022, Rocket completed the acquisition of Renovacor, bringing in $43.0 million of non-dilutive capital. The acquisition also provided Rocket access to an AAV-based gene therapy targeting BAG3-associated dilated cardiomyopathy (DCM), a severe form of heart failure. Additionally, Rocket gained access to world-class scientific collaborators, a robust intellectual property portfolio and personnel with expertise in BAG3-DCM.

- **Strengthened Board of Directors and leadership team with new appointments**. Broadened cardiovascular experience with the appointment of Fady Malik, M.D., Ph.D. to Rocket's Board of Directors. Dr. Malik brings nearly 25 years of experience as an internationally recognized cardiovascular physician-scientist and highly successful biopharmaceutical executive. In addition, Rocket appointed Mayo Pujols as Executive Vice President, Chief Technical Officer. Mr. Pujols has nearly 30 years of experience in leadership roles across technical operations, quality operations, validation, process development and cGMP manufacturing. Rocket also appointed Carlos Martin as Senior Vice President, Chief Commercial Officer. Mr. Martin brings over 20 years of global commercial leadership gained at Novartis, Schering Plough and Eli Lilly.

- **Recognized Rare Disease Day with an event at NASDAQ Tower in New York City**. On February 28, 2022, Rocket hosted its annual Rare Disease Day celebration highlighting the theme, “Rare, But Not Alone.” More than 250 members of the global rare disease community and Rocket team gathered in person and virtually to hear about the impact of rare disease and clinical research from patients, families, advocacy groups and scientific collaborators and innovators – including Dr. Moris Danon, who first identified Danon Disease. The event concluded with the lighting of the Empire State Building, as well as other global landmarks, in Rare Disease Day colors.

Upcoming Investor Conferences

- Cowen’s 43rd Annual Health Care Conference: March 6-8, 2023
- Needham 21st Annual Virtual Healthcare Conference: April 17-20, 2023

Fourth Quarter and Full Year 2022 Financial Results

- **Cash position**. Cash, cash equivalents and investments as of December 31, 2022, were $399.7 million.
- **R&D expenses**. Research and development expenses were $50.0 million and $165.6 million for the three and twelve months ended December 31, 2022, compared to $32.2 million and $125.5 million for the three and twelve months ended December 31, 2021. The increase in R&D expenses was primarily driven by increases in manufacturing and development costs of $26.3 million, laboratory supplies of $6.6 million, compensation and benefits expense of $11.5 million due to increased R&D headcount and direct materials of $3.6 million. Increases noted were offset by a decrease in license fees of $12.9 million attributable to the expense in connection with warrants to purchase shares of common stock recorded for the year ended December 31, 2021.
- **G&A expenses**. General and administrative expenses were $19.0 million and $58.8 million for the three and twelve months ended December 31, 2022, compared to $12.2 million and $41.8 million for the three and twelve months ended December 31, 2021. The increase in G&A expenses was primarily driven by increases in commercial preparation expenses which consists of commercial strategy, medical affairs, market development and pricing analysis of $4.9 million, compensation and benefits of $4.4 million due to increased G&A headcount and acquisition related expense of $3.2 million related to the Renovacor acquisition which closed on December 1, 2022.
- **Net loss**. Net loss was $66.7 million and $221.9 million or $0.92 and $3.26 per share (basic and diluted) for the three and twelve months ended December 31, 2022, compared to $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.

- **Shares outstanding**. 79,123,312 shares of common stock were outstanding as of December 31, 2022.

Financial Guidance

- **Cash position.** As of December 31, 2022, Rocket had cash, cash equivalents and investments of $399.7 million. Rocket expects such resources will be sufficient to fund its operations through 2024, including producing AAV cGMP batches at the Company's Cranbury, N.J. R&D and manufacturing facility and continued development of our six clinical and/or preclinical programs.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare 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 (LV)-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. Rocket also has preclinical AAV-based gene therapy programs in PKP2-arrhythmogenic cardiomyopathy (ACM) and BAG3-associated dilated cardiomyopathy (DCM). 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 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), Danon Disease (DD) and other diseases, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, Rocket's plans for the advancement of its Danon Disease program, including its planned pivotal trial, 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 "aim," "anticipate," "believe," "can," "continue," "design," "estimate," "expect," "intend," "may," "plan," "potential," "will give," "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, 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, our ability to submit regulatory filings with the U.S. Food and Drug Administration (FDA) and to obtain and maintain FDA or other regulatory authority approval of our product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, our integration of an acquired business, which involves a number of risks, including the possibility that the integration process could result in the loss of key employees, the disruption of our ongoing business, or inconsistencies in standards, controls, procedures, or policies, our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire and any 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, 2021, filed February 28, 2022 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. 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.

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 50,037	$ 32,161	$ 165,570	$ 125,476
General and administrative	19,044	12,171	58,773	41,772
Total operating expenses	69,081	44,332	224,343	167,248
Loss from operations	(69,081)	(44,332)	(224,343)	(167,248)
Research and development incentives	500	500	500	1,000
Interest expense	(467)	(463)	(1,862)	(2,977)
Interest and other income, net	1,245	849	3,889	3,068
Amortization of premium on investments - net	1,081	(801)	(47)	(2,912)
Total other income (expense), net	2,359	85	2,480	(1,821)
Net loss	$ (66,722)	$ (44,247)	$ (221,863)	$ (169,069)
Net loss per share attributable to common stockholders - basic and diluted	$ (0.92)	$ (0.69)	$ (3.26)	$ (2.67)
Weighted-average common shares outstanding - basic and diluted	72,889,548	64,470,930	68,148,925	63,235,417

	December 31, 2022	December 31, 2021
Cash, cash equivalents, and investments	$ 399,670	$ 388,740
Total assets	551,807	497,020
Total liabilities	62,121	42,296
Total stockholders' equity	489,686	454,724

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