



Rocket Pharmaceuticals Reports Third Quarter 2022 Financial Results and Highlights Recent Progress

November 3, 2022

- Positive updates from Phase 1 clinical trial in Danon Disease showed RP-A501 was well tolerated with evidence of durable treatment effect and disease improvement for both pediatric patients and adult patients with up to nine and 36 months of follow-up, respectively—
- Phase 2 pivotal study design and endpoint selection for Danon Disease on track with FDA feedback anticipated in Q4 2022; previously disclosed data to be presented at AHA 2022—
- LAD-I and Fanconi Anemia (FA) gene therapy programs remain on track, regulatory filings for LAD-I and FA anticipated 1H 2023 and 2H 2023, respectively; clinical data across LAD-I, FA and PKD lentiviral (LV) programs to be presented at the 64th ASH Annual Meeting—
- Announced acquisition of Renovacor extending Rocket's leadership and capabilities in adeno-associated virus (AAV)-based cardiac gene therapy; expected to close by Q1 2023—
- Enrollment completed in Phase 1 trial of RP-L301 for PKD; Phase 1 update now anticipated 1H 2023 and Phase 2 pivotal trial initiation expected in 2023—
- Cash, cash equivalents and investments of \$306.5M; with additional net proceeds of \$108.2M from recent equity offering, expected operational runway now extends into 2H 2024, pending close of Renovacor acquisition—

CRANBURY, N.J.--(BUSINESS WIRE)--Nov. 3, 2022-- [Rocket Pharmaceuticals, Inc.](#) (NASDAQ: RCKT), a leading late-stage, clinical biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders with high unmet need, today reports financial results for the quarter ending September 30, 2022, and updates from the Company's key pipeline developments, business operations and upcoming milestones.

"Rocket had a remarkable third quarter highlighted by significant progress in our Phase 1 clinical trial for Danon Disease, with positive early findings demonstrating RP-A501 was generally well tolerated with evidence of positive treatment effect and improvement of the natural course of the disease in pediatric patients with up to nine months follow-up. Additionally, we observed continued, durable robust activity in adults with up to 36 months follow-up," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. "As previously announced, we reached an understanding with the FDA on chemistry, manufacturing and controls (CMC) requirements to start AAV cGMP manufacturing at our in-house facility as well as potency assay plans for a Phase 2 pivotal study. We are now laser focused on advancing Phase 2 pivotal study design and endpoint selection with FDA feedback expected this quarter."

Dr. Shah continued, "In the third quarter, we also announced the expected acquisition of Renovacor which aims to further solidify our leadership in AAV-based cardiac gene therapy. By combining Rocket's clinical, regulatory, and chemistry, manufacturing and control expertise with Renovacor's compelling preclinical research in BAG3-associated dilated cardiomyopathy and early assets, we look forward to advancing precision gene therapies even further to address the significant unmet need of patients with genetically driven cardiac diseases."

"Based on the strong results presented across our Leukocyte Adhesion Deficiency-I (LAD-I) and Fanconi Anemia (FA) lentiviral gene therapy programs to date, we remain on track towards our first regulatory filings in 2023 – LAD-I in the first half and FA in the second half – and expect to present data on both programs at ASH in December," said Dr. Shah. "Regarding our Pyruvate Kinase Deficiency (PKD) program, we also expect to share updated data from both adult patients at ASH. Both pediatric patients in the study have been enrolled. We now anticipate a Phase 1 update in the first half of 2023 and initiation of the Phase 2 pivotal trial in 2023."

Dr. Shah continued, "Finally, our exceptional third quarter progress was capped off by an extension of our cash runway into the second half of 2024 following an equity raise with net proceeds of \$108.2 million. Taken together, I am very proud of our continued progress at Rocket and look forward to the final push towards another successful year in our pursuit of gene therapy cures for patients and their loved ones facing these devastating, life-threatening genetic diseases."

Key Pipeline and Operational Updates

- **Announced positive clinical data from ongoing Phase 1 trial of RP-A501 for Danon Disease.** Data presented at the Heart Failure Society of America (HFSA) Annual Scientific Meeting 2022 included updated safety and efficacy data from patients in the pediatric and adult cohorts. Results showed RP-A501 was generally well tolerated with evidence of treatment effect and improvement of Danon Disease, including for both pediatric patients with up to nine months of follow-up and four adult patients with up to 36 months of follow-up. Efficacy data from the pediatric patients are following similar or more favorable positive trends as in the adults at a similar timeframe. Based on the data available, the Company expects feedback from the FDA on Phase 2 pivotal study design and endpoints this quarter. Previously disclosed data will be presented in an oral presentation at the American Heart Association (AHA) Scientific Sessions 2022 taking place in Chicago, Illinois from November 5-7.
- **Announced plans to acquire Renovacor, extending leadership in AAV-based cardiac gene therapy.** The Company announced plans to acquire Renovacor in an all-stock transaction for an implied value of approximately \$2.60 per share, based on the volume weighted average trading price of Rocket shares of \$15.51 for the 30 trading days through and including Monday, September 19, 2022. The acquisition is expected to close by the first quarter of 2023.
- **Phase 2 pivotal studies of RP-L201 for LAD-I and RP-L102 for FA and first regulatory filings remain on track.** Based on positive top-line safety and efficacy data from Phase 2 pivotal studies, regulatory filings are anticipated for RP-L201 for LAD-I in the first half of 2023 and for RP-L102 for FA in the second half of 2023.
- **Clinical data to be presented across all three LV-based programs at ASH 2022.** The Company will present posters from its LV-based gene therapy programs in LAD-I, FA and PKD at the 64th American Society of Hematology (ASH) Annual Meeting taking place in New Orleans, Louisiana, from December 10-13, 2022. Updated safety and efficacy data will be presented from the Phase 2 pivotal trial of RP-L102 for FA and Phase 1 trial of RP-L301 for PKD. Previously disclosed data will be presented from the Phase 2 pivotal trial of RP-L201 for LAD-I.
- **Enrollment completed in Phase 1 trial of RP-L301 for PKD.** Both pediatric patients in the Phase 1 study have been enrolled. Updated safety and efficacy data will be presented from the two adult patients at ASH 2022. The Company now anticipates a Phase 1 update across both cohorts in the first half of 2023 and initiation of the Phase 2 pivotal trial is expected to occur in 2023.
- **Public offering.** On October 6, 2022, the Company completed a public offering of 7,820,000 shares of common stock at a public offering price of \$14.75 per share. The net proceeds to Rocket were \$108.2 million. All shares in the offering were sold by Rocket. Morgan Stanley, J.P. Morgan and SVB Securities acted as joint book-running managers, and BTIG acted as the lead manager for the offering.

Upcoming Investor Conferences

- Barclays Gene Editing & Gene Therapy Summit, November 14, 2022
- Stifel Healthcare Conference 2022, November 15-16, 2022
- 5th Annual Evercore ISI HealthCONx Conference 2022, November 29-December 1, 2022

Third Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of September 30, 2022, were \$306.5 million.
- **R&D expenses.** Research and development expenses were \$43.4 million for the three months ended September 30, 2022, compared to \$39.6 million for the three months ended September 30, 2021. The increase in research and development expenses was primarily driven by increases in manufacturing and development costs, direct materials, laboratory supplies and compensation and benefits expenses due to increased R&D headcount.
- **G&A expenses.** General and administrative expenses were \$15.1 million for the three months ended September 30, 2022, compared to \$10.0 million for the three months ended September 30, 2021. The increase in general and administrative expenses was primarily driven by increases in compensation and benefits expense due to increased G&A headcount, non-cash stock compensation expense, consulting expenses and pre-acquisition related expenses.
- **Net loss.** Net loss was \$57.8 million or \$0.87 per share (basic and diluted) for the three months ended September 30, 2022, compared to \$50.1 million or \$0.79 per share (basic and diluted) for the three months ended September 30, 2021.
- **Shares outstanding.** 67,838,803 shares of common stock were issued and outstanding as of September 30, 2022.

Financial Guidance

- **Cash position.** As of September 30, 2022, the Company had cash, cash equivalents and investments of \$306.5 million. Through September 30, 2022, the Company sold 3.3 million shares of common stock for net proceeds of \$46.6 million under its at-the-market facility. With the at-the-market facility proceeds and the October 2022 public offering proceeds, the Company expects that such resources will be sufficient to fund our operating expenses and capital expenditure requirements into the second half of 2024, including the continued buildout and initiation of AAV cGMP manufacturing

capabilities at our Cranbury, New Jersey R&D and manufacturing facility, and the continued development of our four clinical programs, as well as future pipeline 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 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 pending acquisition of Renovacor, 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, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, Rocket's plans for the advancement of its 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, 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 September 30, 2022		Nine Months Ended September 30, 2021	
	2022	2021	2022	2021
Revenue	\$ -	\$ -	\$ -	\$ -
Operating expenses:				
Research and development	43,383	39,621	115,533	92,459
General and administrative	15,105	10,025	39,728	30,456
Total operating expenses	58,488	49,646	155,261	122,915
Loss from operations	(58,488)	(49,646)	(155,261)	(122,915)
Research and development incentives	-	-	-	500
Interest expense	(465)	(534)	(1,395)	(2,514)
Interest and other income, net	1,353	806	2,644	2,218
Amortization of premium on investments - net	(156)	(744)	(1,128)	(2,111)
Net loss	\$ (57,756)	\$ (50,118)	\$ (155,140)	\$ (124,822)
Net loss per share - basic and diluted	\$ (0.87)	\$ (0.79)	\$ (2.37)	\$ (1.99)
Weighted-average common shares outstanding - basic and diluted	66,215,535	63,825,429	65,406,844	62,828,601

	September 30, December 31,	
	2022	2021
Cash, cash equivalents, and investments	\$ 306,534	\$ 388,740
Total assets	417,265	497,020
Total liabilities	49,952	42,296
Total stockholders' equity	367,313	454,724

Media

Kevin Giordano

Director, Corporate Communications

kgiordano@rocketpharma.com

Investors

Jessie Yeung, M.B.A.

Vice President, Investor Relations and Corporate Finance

investors@rocketpharma.com

Source: Rocket Pharmaceuticals, Inc.