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

May 10, 2021

—RP-A501 Phase 1 Trial for the Treatment of Danon Disease Placed on Clinical Hold To Address FDA Request For Additional Risk Mitigation Methods in Protocol; No New Safety Events Observed; Anticipate Delay in Enrollment by One Quarter —

—Updated Low Dose Results in RP-A501 Treatment for Danon Disease Demonstrate Stabilization or Improvements in Key Clinical Outcomes Including 6MWT and NYHA Class; Full Data to be presented in 4Q —

—Reported Hemoglobin Increases of Nearly 2x from Baseline Sustained Up to 6-Months Post RP-L301 Treatment in PKD —

— Announced Sustained Efficacy, Durability and Safety Up to 18-Months Post RP-L201 Treatment in LAD-I —

—Advanced Fifth Gene Therapy Program in Infantile Malignant Osteopetrosis into Patient Dosing —

—Updated Results from FA, LAD-I, PKD Trials to be Presented at 24th ASGCT Virtual Annual Meeting —

— Redemption of 2022 Convertible Notes Strengthens Balance Sheet; $466.4 Million in Cash Solidifies Runway into 2H 2023 —

—Webcast and Conference Call Regarding Danon Program to be Held at 4:30 PM EDT Today —

CRANBURY, N.J.--(BUSINESS WIRE)--May 10, 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 March 31, 2021 and updates on the Company's key pipeline developments, business operations, and upcoming milestones.

“I am pleased that we began 2021 with sustained momentum across clinical, regulatory and manufacturing activities as reflected in our exciting PKD and LAD-I results, advancement of the pipeline toward regulatory submissions, and continued progress of our GMP manufacturing facility,” said Gaurav Shah, M.D., Chief Executive Officer of Rocket. “New data readouts and designations from regulatory agencies continue to validate and reinforce the transformative potential of our gene therapies for patients suffering from rare diseases with no current drug treatments. We continued to strengthen our world class operations and capabilities as we added to our leadership team and fortified our balance sheet.”

Dr. Shah continued, “We are encouraged by the positive low dose RP-A501 Phase 1 trial results in Danon Disease disclosed today. We anticipate a one quarter delay in enrollment to address FDA requests for risk mitigation methods in our protocol. We have successfully treated five patients in the low- and high-dose adult cohorts. Today, we announce that new longer-term low-dose data demonstrate durable expression and ongoing improvements in biomarkers, and evidence of a positive risk/benefit profile out to 18-months. In the two patients followed to 18-months, we see improvement in 6MWT in one patient and stabilization/improvement the other, and improvement in NYHA Class from II to I in one patient with stabilization in the other. These updated results increase our confidence in the low dose as a potentially viable dose for patients with Danon Disease. Safety is our top priority as we progress our gene therapy trials. We are diligently working with the agency prior to initiating our low-dose pediatric cohort, which we believe has high potential as a Phase 2 dose given the durable results noted in adult patients. “

Dr. Shah continued, “The balance of the year is shaping up to be a productive period, starting next week with updates from our FA, LAD-I, and PKD programs at ASGCT, followed by initial IMO data in the third quarter of 2021. We are focused on strong clinical and operational execution as we continue our development of total cures at the genetic level.”

Key Pipeline and Operational Updates

- **RP-A501 Danon Disease program paused for additional risk mitigation.** No new drug-related safety events have been observed in the low- or high-dose adult cohorts of the Phase 1 trial. The U.S. Food and Drug Administration (FDA) has requested the Company to pause patient dosing and modify the protocol and other supporting documents with revised guidelines for patient selection and management. All follow-up study activities will continue and no additional data are requested. Rocket is continuing its dialogue with the agency to ensure safety measures are updated and harmonized

adequately and anticipates additional patient treatment by 3Q2021.

- **Low Dose ($6.7x10^{13}$) RP-A501 Treatment Demonstrates Durable Expression and Improvements in Biomarkers.** New Data Include:
  - Patient 1002: 78% IHC (revised from previous 67% due to improved technique), BNP continues to decrease to 200 (baseline 943)
  - Functional outcomes
    - Patient 1001: NYHA Class stable (II), 6-minute walk test (MWT) improved at 18-months
    - Patient 1002: NYHA Class Improved (II to I), 6MWT stable to modestly improved at 18 months
    - Patient 1005: NYHA Class Stable (II), 6MWT pending at 12 months
  - Higher Dose ($1.1x10^{14}$) data will be updated in the fourth quarter
  - Rocket does not anticipate pursuing doses higher than $1.1x10^{14}$ moving forward.

- **Presented positive clinical updates from RP-L201 Leukocyte Adhesion Deficiency-I (LAD-I) program at the Clinical Immunology Society (CIS) Annual Meeting.** The Phase 1/2 data presented in a poster at CIS 2021 are from four pediatric patients with severe LAD-I. RP-L201 was well tolerated with no safety issues reported with treatment or post-treatment. All four patients achieved hematopoietic reconstitution within 5-weeks and demonstrated CD18 expression substantially exceeding the 4-10% threshold associated with survival into adulthood. The first patient with 18-months follow up demonstrated durable CD18 expression of ~40%, peripheral blood vector copy number (VCN) levels of 1.2 at 12-months post-treatment and resolution of skin lesions with no new lesions. The second patient with 9-months of follow up demonstrated CD18 expression of ~28% and peripheral blood VCN levels of 0.75 at 6-months post-treatment with kinetics consistent with those of the first patient. The third and fourth patients demonstrated high CD18 expression of ~70% and ~51%, respectively at 3-months post treatment, and peripheral blood VCN kinetics consistent with those of the first two patients. A link to the full data disclosed is available here: https://rocketpharma.com/CIS. More comprehensive Phase 2 results will be presented at ASGCT.

- **Announced updated positive preliminary clinical data from Phase 1 trial of RP-L301 for the treatment of Pyruvate Kinase Deficiency (PKD).** The updated preliminary Phase 1 RP-L301 data are from two patients that showed sustained safety and tolerability 6- and 3-months after treatment, respectively. The two patients demonstrated durable normalization of hemoglobin levels from an average baseline of ~7.4 grams (g)/deciliter (dL) to 13.9 g/dL at 6-months post treatment in the first patient and from a baseline of ~7.0 g/dL to 13.8 g/dL at 3-months post treatment in the second patient. The two patients both demonstrated significant improvements in bilirubin 6- and 3-months after treatment, which had been substantially elevated prior to study enrollment. The Phase 1 trial continues to enroll patients with longer-term data on track for the fourth quarter of 2021, with a near-term update at ASGCT.

- **Dosed first patient with RP-L401 in Infantile Malignant Osteopetrosis Phase I trial.** Patient treatment has commenced in the Phase I trial of RP-L401, Rocket's LVV-based gene therapy for infantile malignant osteopetrosis(IMO). The first patient is being treated at UCLA Children's Hospital. With this significant milestone patients are now being treated in all five gene therapy programs across the company's pipeline. Preliminary results are expected in the third quarter of this year.

- **Received regulatory designations for LAD-I program from the FDA and European Medicines Agency (EMA).** The LAD-I program received Regenerative Medicine Advanced Therapy (RMAT) designation from the FDA and Priority Medicines (PRIME) designation from the EMA, completing the full complement of all U.S. and EU accelerated regulatory designations for the program.

- **Further strengthened Rocket leadership.** In March 2021, Gayatri Rao, MD, JD was promoted to Senior Vice President, Chief Development Officer of Rocket's LVV pipeline. Since joining Rocket from the FDA in 2018, Dr. Rao has led global product development of the Company's four clinical-stage LVV programs, as well as Regulatory Policy and Patient Advocacy. Going forward, she will be responsible for the integrated development of Rocket's LVV pipeline and LVV Global Product Teams. In late 2020, Jose Trevejo, M.D., Ph.D. was appointed Senior Vice President, Chief Development Officer of Rocket's AVV pipeline. Dr. Trevejo brings significant clinical development leadership to Rocket from roles at Genentech, Vertex Pharmaceuticals, and others, most recently serving as Chief Executive Officer of non-viral gene therapy company SmartPharm. The Company's establishing of two CDOs across AAV/LVV reflect the company's commitment to developing world-class capabilities and treatments across the spectrum of gene therapy.

- **Strengthened balance sheet with redemption of existing convertible notes.** On April 26, 2021, Rocket completed a redemption of its outstanding 6.25% Convertible Senior Notes due 2022. The company redeemed at an aggregate redemption price equal to 100% for each $1,000 principal amount of such notes, plus accrued and unpaid interest, removing the $38.35 million of the 6.25% Convertible Senior Notes from the consolidated balance sheet. Approximately $5.15 million aggregate principal amount of the 5.75% Convertible Senior Notes due 2021 remain outstanding.

Anticipated Milestones

- Fanconi Anemia (RP-L102)
  - Updated “Process B” data (Q2 2021)
- LAD-I (RP-L201)
  - Longer-term Phase 2 data (Q3 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)
  - Initial Phase 1 data (Q3 2021)

Upcoming Investor Conferences

- BofA Securities’ 2021 Virtual Health Care Conference, May 13, 2021
- UBS Global Healthcare Virtual Conference, May 26, 2021

First Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of March 31, 2021 were $466.4 million.
- **R&D expenses.** Research and development expenses were $28.5 million for the three months ended March 31, 2021, compared to $17.0 million for the three months ended March 31, 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 $10.7 million for the three months ended March 31, 2021, compared to $7.2 million for the three months ended March 31, 2020, due to an increase in non-cash stock compensation expense and an increase in compensation and benefits expense due to increased G&A headcount.
- **Net loss.** Net loss was $40.2 million or $0.65 per share (basic and diluted) for the three months ended March 31, 2021, compared to $24.7 million or $0.45 per share (basic and diluted) for the three months ended March 31, 2020.
- **Shares outstanding.** 61,987,799 shares of common stock were outstanding as of March 31, 2021.

Financial Guidance

- Rocket expects its balance in cash, cash equivalents and investments of $466.4 million as of March 31, 2021 to fund its operations into the second half of 2023, including the buildout and initiation of AAV cGMP manufacturing capabilities at our Cranbury, New Jersey R&D and manufacturing facility and advancement of our five clinical programs.

Conference Call Details

Rocket management will host a conference call today at 4:30 PM EDT. 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 listen to the 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 50162863.

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 may develop, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, 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.

	Three Months Ended March 31,	
	2021	**2020**
Operating expenses:		
Research and development	$ 28,542	$ 16,957
General and administrative	10,680	7,163
Total operating expenses	39,222	24,120
Loss from operations	(39,222)	(24,120)
Research and development incentives	500	-
Interest expense	(1,729)	(1,573)
Interest and other income net	911	967
(Amortization of premium) accretion of discount on investments - net	(639)	62
Total other expense, net	(957)	(544)
Net loss	$ (40,179)	$ (24,664)
Net loss per share attributable to common stockholders - basic and diluted	$ (0.65)	$ (0.45)
Weighted-average common shares outstanding - basic and diluted	61,574,405	54,883,120

	March 31, 2021	**December 31, 2020**
Cash, cash equivalents and investments	466,353	482,719
Total assets	576,484	590,824
Total liabilities	96,485	87,305
Total stockholders' equity	479,999	503,519

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Claudine Prowse, Ph.D.
SVP, Strategy & Corporate Development
investors@rocketpharma.com

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