Rocket Pharmaceuticals Reports Second Quarter 2020 Financial Results and Highlights Recent Progress

August 5, 2020

—"Process B" Demonstrates Safety and Sustained Efficacy for FA (RP-L102) and LAD-I (RP-L201)—

—First Patient Treated in Global Phase 1 Study of RP-L301 for PKD—

—FDA Clearance of IND for IMC (RP-L401) —

—Preliminary Data Readouts in Danon and PKD and Additional "Process B" Data in FA and LAD-I on Track for Fourth Quarter—

—Strong Balance Sheet with $250 Million in Cash; Capitalized into 2Q 2022—

NEW YORK--(BUSINESS WIRE)--Aug. 5, 2020-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today reports financial results for the quarter that ended June 30, 2020, along with an update on the Company's key pipeline developments, business operations and upcoming milestones.

"During this quarter, I am pleased to announce we continued to build pipeline momentum with positive data readouts and significant clinical trial progress," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We continued to enroll patients in our FA, LAD-I and PKD trials, and received FDA clearance of our IND for IMO. In addition, we presented positive data demonstrating sustained efficacy for both RP-L102 for FA and RP-L201 for LAD-I at ASGCT. With the continued buildout of our manufacturing facility, we anticipate GMP clinical material produced in 2021. With steady commitment and dedication, we remain focused on establishing a truly integrated and industry-leading multi-platform gene therapy approach aimed at improving and saving lives."

Dr. Shah continued, "Our progress comes amidst the backdrop of COVID-19, which continues to affect the lives of millions. As members of the healthcare community, we are committed to continuing to operate with a sense of responsibility to our employees and patients as we advance our pipeline aimed at addressing so many devastating rare genetic disorders. We want to also applaud the relentless work of our partners, patients and healthcare communities who skillfully navigate and respond to this pandemic with passion and care."

Key Pipelines and Operational Updates

- **Phase 1 clinical trial of RP-A501 for the treatment of Danon Disease continues to progress.** Rocket has been cleared by the U.S. Food and Drug Administration (FDA) and the Independent Data Safety Monitoring Committee (IDSMC) to move to the higher dose cohort of the study. The first patient in this cohort is expected to be treated in the third quarter, and preliminary Danon data is anticipated in the fourth quarter.
- **Positive clinical updates for the Company's Fanconi Anemia (FA) and Leukocyte Adhesion Deficiency-I (LAD-I) programs presented at the American Society of Gene & Cellular Therapy (ASGCT) meeting.** At the virtual ASGCT conference, Rocket presented longer-term data from the FANCOLEN-I study of RP-L102 "Process A" for FA. Patients followed for a year or more after treatment with RP-L102 "Process A" continued to demonstrate durable engraftment and hematologic correction, without the use of pre-treatment conditioning regimens. Updates on FA "Process B" data are expected in the fourth quarter. In Rocket's Phase 1/2 study of RP-L201 for the treatment of severe LAD-I, the first patient treated with RP-L201 demonstrated an increase in CD18 expression from less than 1% to 45%, sustained over six months. These results lend further support to the applicability of "Process B" across the lentiviral portfolio. Enrollment is now complete in the Phase 1 portion of the study, and Rocket expects to move to Phase 2 in the fourth quarter. Additional LAD-I data are on track for the fourth quarter.
- **First patient treated in the Phase 1 Trial of RP-L301 for Pyruvate Kinase Deficiency (PKD).** PKD represents the Company's largest lentiviral program. The trial is designed to assess safety, tolerability and preliminary efficacy of RP-L301 for the treatment of PKD. Preliminary data is anticipated in the fourth quarter.

- **Global Phase 1 trial of RP-L401 for Infantile Malignant Osteopetrosis (IMO) initiates enrollment.** The Company received Investigational New Drug (IND) application approval of RP-L401 at the end of the second quarter. The trial is designed to assess the safety, tolerability and preliminary efficacy of RP-L401.
- **Continued focus on supporting the patient community by serving as a pivotal resource for patient education during the COVID-19 pandemic.** Throughout the quarter, as patients were faced with significant difficulties presented by the pandemic, the Rocket team made it a priority to connect with the community, share updates and host a variety of programs and events to support patients through this critical time. In addition to hosting multiple community Q&A sessions, the Rocket team and its partners participated in the 2020 Fanconi Anemia Family Meeting, hosted by the Fanconi Anemia Research Fund (FARF) where the latest FA data was presented, and a Q&A session was held for the patient community. More recently, Rocket organized two virtual sessions for patients with Danon Disease and their families. The first virtual event was focused on addressing the community's concerns around COVID-19. The second event brought together patients with Danon Disease, their families and experts in the field to discuss current treatment options, the present state of research, the development of new treatments and ongoing clinical trials.
- **Rocket strengthens its balance sheet by extending the maturity of the existing convertible notes.** Rocket extended the maturity of the 5.75% convertible notes due 2021. The Company conducted a second exchange offering and successfully exchanged an additional $7.5 million of the outstanding $12.65 million convertible notes. The new notes, with an aggregate principal amount of $46.85 million, will mature on August 1, 2022 and have an interest payment of 6.25% per annum. The remainder of the notes will retain the existing maturity date and interest payment.

Anticipated Milestones

- **FA (RP-L102)**
  - Preliminary "Process B" data (4Q)
  - Phase 2 "Process B" data (2021)
- **Danon Disease (RP-A501)**
  - First patient treatment in higher dose (3Q)
  - Preliminary Phase 1 data (4Q)
  - Phase 1 longer-term data (2021)
  - Phase 2 study initiation (2021)
- **LAD-I (RP-L201)**
  - Initiate Phase 2 study (4Q)
  - Phase 1 data update (4Q)
  - Phase 2 data (2021)
- **PKD (RP-L301)**
  - Preliminary Phase 1 data (4Q)
  - Longer-term Phase 1 data (2021)
  - Phase 2 study initiation (2021)
- **IMO (RP-L401)**
  - Initiation of clinical study (4Q)
  - Phase 1 data (2021)
  - Phase 2 study initiation (2021)

Upcoming Investor Conferences

- Citi's 15 th Annual BioPharma Virtual Conference, September 9-10, 2020
- Morgan Stanley 18th Annual Global Healthcare Virtual Conference, September 14-18, 2020

Second Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of June 30, 2020 were $249.8 million.
- **Debt.** Our balance sheet includes $52.0 million of fully convertible notes.
- **R&D expenses.** Research and development expenses were $16.7 million for the three months ended June 30, 2020, compared to $14.0 million for the three months ended June 30, 2019, due to an increase in compensation and benefits expense due to increased R&D headcount, and an increase in manufacturing and development costs.
- **G&A expenses.** General and administrative expenses were $6.8 million for the three months ended June 30, 2020, compared to $4.4 million for the three months ended June 30, 2019, 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 $25.0 million or $0.45 per share (basic and diluted) for the three months ended June 30, 2020, compared to $18.7 million or $0.38 per share (basic and diluted) for the three months ended June 30, 2019.
- **Shares outstanding.** 55,170,003 shares of common stock were outstanding as of June 30, 2020.

Financial Guidance

- **Cash position.** As of June 30, 2020, we had cash, cash equivalents and investments of $249.8 million. Rocket expects such resources will be sufficient to fund its operations into the second quarter of 2022.

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 2020 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 when clinical trial sites will resume normal business operations, 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-Q for the quarter ended June 30, 2020, filed May 8, 2020 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,	
	2020	2019	2020	2019
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	16,731	13,989	33,687	29,126
General and administrative	6,828	4,403	13,990	8,211
Total operating expenses	23,559	18,392	47,677	37,337
Loss from operations	(23,559)	(18,392)	(47,677)	(37,337)
Research and development incentives	-	-	-	250
Interest expense	(1,786)	(1,544)	(3,360)	(3,148)
Interest and other income net	429	941	1,395	1,542
Accretion of discount on investments	(124)	315	(62)	562
Net loss	$ (25,040)	$ (18,680)	$ (49,704)	$ (38,131)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.45)	$ (0.38)	$ (0.90)	$ (0.81)
Weighted-average common shares outstanding - basic and diluted	55,158,459	49,267,247	55,020,789	47,206,480

Selected Balance Sheet Information

(amounts in thousands)

	June 30, 2020	December 31, 2019
Cash, cash equivalents and investments	249,803	304,115
Total assets	353,065	372,121
Total liabilities	86,542	64,824
Total stockholders' equity	266,523	307,297

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

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