Rocket Pharmaceuticals Reports Third Quarter 2019 Financial Results and Operational Highlights

November 7, 2019

–First Evidence of Long-Term Improvement and Stabilization in Blood Counts and Durable Mosaicism in RP-L102 “Process A” for Fanconi Anemia–

–Four Gene Therapies Now in the Clinic–

–Initial Data from FA “Process B” and LAD-I Phase 1 Studies Anticipated by Year-End–

NEW YORK--(BUSINESS WIRE)--Nov. 7, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”), a leading U.S.-based multi-platform clinical-stage gene therapy company, reports financial results for the quarter ended September 30, 2019, and provides an update on the Company's recent pipeline developments, as well as upcoming milestones.

"In the third quarter, we made meaningful progress towards our clinical and regulatory milestones,” said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. “We received IMPD and IND clearance for our global Phase 1 trial for PKD, treated our first patient for LAD-I and continued enrollment of our Phase 1 trial of RP-A501 for Danon disease. We also announced promising long-term data from our Phase 1/2 trial of first-generation RP-L102 for Fanconi Anemia supporting sustained clinical improvement with robust engraftment exceeding the 10% threshold agreed to by regulators for the upcoming global Phase 2 trial. We hope to further optimize these responses with our ‘Process B’ of RP-L102 designed to enable consistent results with commercial-grade product without cytotoxic conditioning. We look forward to data from ‘Process B’ later this year, along with initial Phase 1 data from our RP-L201 trial for LAD-I.”

Dr. Shah continued, “We now have four gene therapy candidates in the clinic, meeting one of our 2019 corporate goals and reinforcing our multi-platform strategy. As we advance our first candidate into late-stage development, we remain dedicated to our pursuit of potentially curative treatments for patients contending with rare genetic diseases.”

Recent Pipeline Developments

- **Commencement of registration-enabling Phase 2 study for Fanconi Anemia (FA)**. Patient enrollment is ongoing in the global Phase 2 trial. The study initiation follows recent alignment from the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) on the trial design and the primary endpoint. Resistance to mitomycin-C, a DNA damaging agent, in bone marrow stem cells at a minimum time point of one year will serve as the primary endpoint and potentially as a surrogate endpoint for accelerated approval. Patient dosing is anticipated to begin in the fourth quarter with preliminary Phase 2 data anticipated in 2020.
- **Long-term Phase 1/2 clinical data of RP-L102 for FA “Process A” presented at the European Society of Gene and Cell Therapy (ESGCT) Annual Congress.** Long-term patient follow-up data demonstrate evidence of increasing and durable engraftment leading to bone marrow restoration exceeding the 10% threshold agreed to by the FDA and EMA for the ongoing registration-enabling Phase 2 trial. In patient 02002, who received what we consider adequate drug product, hemoglobin levels are now similar to those in the first year after birth, suggesting hematologic correction over the long term. Preliminary results from the first two patients receiving "Process B" of RP-L102 are anticipated in the fourth quarter.
- **Global Phase 1 trial of RP-L301 for Pyruvate Kinase Deficiency (PKD) initiates enrollment.** Rocket received Investigational Medicinal Product Dossier (IMPD) clearance and Investigational New Drug (IND) application approval of RP-L301 during the quarter. The open-label, single-arm, clinical trial will enroll six adult and pediatric transfusion-dependent PKD patients in the U.S. and Europe. The trial is designed to assess the safety, tolerability and preliminary efficacy of RP-L301. Preliminary data are anticipated in 2020.
- **Patient dosing continues in Phase 1/2 registrational trial of RP-L201 for Leukocyte Adhesion Deficiency-I (LAD-I).** The Phase 1 portion of the trial is expected to enroll two patients and will assess the safety and tolerability of RP-L201. The Phase 2 portion of the trial will evaluate overall survival. Preliminary Phase 1 data will be presented in the fourth quarter.

- **Patient dosing continues in first cohort in the Phase 1 clinical trial of RP-A501 for the treatment of Danon disease**. Rocket continues to enroll patients in the trial and anticipates reporting Phase 1 data in 2020. The study is designed to assess the safety and tolerability of a single infusion of RP-A501. Pediatric dosing will initiate pending determination of safety in a patient population comprised of older adolescents and young adults.

Anticipated Milestones

- **FA (RP-L102)**
  - Global Phase 2 trial first patient treatment (4Q19)
  - Initial Phase 1 data for “Process B” (4Q19)
  - Additional Phase 1 “Process B” data (1H20)
  - Preliminary Phase 2 data (2H20)
- **Danon Disease (RP-A501)**
  - Phase 1 data (2020)
- **LAD-I (RP-L201)**
  - Initial Phase 1 data (4Q19)
  - Phase 1 data update (1H20)
  - Commence enrollment of Phase 2 study (2H20)
- **PKD (RP-L301)**
  - Preliminary Phase 1 data (2H20)
- **IMO (RP-L401)**
  - Initiation of clinical study (2H20)

Upcoming Investor Conferences

- Barclays Gene Editing & Gene Therapy Summit—November 13, 2019 in New York, N.Y.
- Evercore ISI 2nd Annual HealthCONx Conference—December 3, 2019 in Boston, M.A.
- Piper Jaffray's 31st Annual Healthcare Conference—December 5, 2019 in New York, N.Y.

Third Quarter 2019 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of September 30, 2019, were $240.6 million.
- **Debt.** Our balance sheet includes a $52.0 million fully convertible debenture which matures in August of 2021.
- **R&D expenses.** Research and development expenses were $14.8 million for the three months ended September 30, 2019, compared to $13.1 million for the three months ended September 30, 2018. The increase was primarily driven by an increase in clinical trial costs of $1.3 million.
- **G&A expenses.** General and administrative expenses were $4.3 million for the three months ended September 30, 2019, compared to $2.3 million for the three months ended September 30, 2018. The increase was primarily driven by an increase in non-cash stock compensation expense of $1.4 million and an increase in compensation and benefits expense of $0.3 million as a result of increased headcount.
- **Net loss.** Net loss was $19.3 million or $0.38 per share (basic and diluted) for the three months ended September 30, 2019, compared to $16.1 million or $0.40 per share (basic and diluted) for the three months ended September 30, 2018.
- **Shares outstanding.** 50,376,030 shares of common stock were outstanding as of September 30, 2019.

Financial Guidance

- **Cash position.** As of September 30, 2019, we had cash, cash equivalents and investments of $240.6 million. Rocket expects such resources will be sufficient to fund its operations into the first half of 2021.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”) is an emerging, clinical-stage biotechnology company focused on developing first-in-class gene therapy treatment options for rare, devastating diseases. Rocket’s multi-platform development approach applies the well-established lentiviral vector (LVV) and adeno-associated viral vector (AAV) gene therapy platforms. Rocket's clinical programs using 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 AAV-based gene therapy is for Danon disease, a devastating, pediatric heart failure condition. Rocket’s pre-clinical pipeline program is for Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. 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, 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 successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the preclinical and clinical results for its product candidates, which may not support further development and marketing approval, the potential advantages of Rocket's product candidates, 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 and its licensors' ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, 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, 2018. 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 September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	14,829	13,065	43,955	29,590
General and administrative	4,336	2,268	12,547	15,021
Total operating expenses	19,165	15,333	56,502	44,611
Loss from operations	(19,165)	(15,333)	(56,502)	(44,611)
Research and development incentives	-	-	250	186
Interest expense	(1,466)	(1,176)	(4,615)	(4,010)
Interest and other income net	979	420	2,522	1,236
Accretion of discount on investments	368	-	930	-
Net loss	$ (19,284)	$ (16,089)	$ (57,415)	$ (47,199)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.38)	$ (0.40)	$ (1.19)	$ (1.22)
Weighted-average common shares outstanding - basic and diluted	50,364,649	39,900,551	48,270,771	38,598,304

Selected Balance Sheet Information

(amounts in thousands)

	September 30,	December 31,
	2019	**2018**
Cash, cash equivalents and investments	240,561	213,132

Total assets	299,910	251,313
Total liabilities	67,992	57,276
Total stockholders' equity	231,918	194,037

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