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

November 3, 2021

— Treatment initiated in pediatric patient cohort in Danon Disease trial of RP-A501—

—Treatment completed in nine of nine patients in LAD-1 Phase 1/2 trial; favorable safety profile and initial clinical benefit reported in all seven patients with at least 3 months of follow up —

—Clinical updates anticipated in Danon Disease at AHA 2021 Scientific Sessions and in FA, LAD-I and PKD at 63rd ASH Annual Meeting —

— Ending Balance Sheet with $421.5 Million in Cash; Cash Runway Expected into 2H'23 —

CRANBURY, N.J.--(BUSINESS WIRE)--Nov. 3, 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 September 30, 2021 and updates on the Company's key pipeline developments, business operations, and upcoming milestones.

"We are excited about the strong progress we made in the third quarter as we initiated treatment in our trial of RP-A501 for Danon Disease in the low-dose (6.7e13 vg/kg) pediatric patient cohort," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. "I am proud of our team's collaboration with the FDA, through which we were able to swiftly resolve the clinical hold on the Danon trial and resume this highly important work on behalf of Danon patients. We are gearing up for the remainder of 2021 and look forward to reporting a comprehensive clinical update on our Phase I trial in Danon Disease this month at the AHA Scientific Sessions as well as key updates on all five of our first-in-class gene therapy programs throughout the fourth quarter."

Dr. Shah continued, "We are equally excited about the progress of our LAD-I program, where we completed treatment for all nine patients in the RP-L201 Phase 1/2 clinical trial and presented positive interim data updates on the initial seven patients at the ESGCT Congress in October. Based on the data presented from these seven patients, RP-L201 continues to demonstrate a favorable safety profile and preliminary clinical benefit in patients with severe LAD-I. We will share additional clinical data from the LAD-I trial at the 63rd ASH Annual Meeting in December, where we will also report clinical updates on our Fanconi Anemia and PKD programs. I am proud of the Rocket team's unwavering dedication to developing and bringing life-changing curative therapies to patients with rare diseases."

Key Pipeline and Operational Updates

Danon Disease:

- **Initiated pediatric patient treatment in Phase 1 trial of RP-A501 for the treatment of Danon Disease.** Rocket has resumed patient enrollment and initiated treatment in the low-dose (6.7e13 vg/kg) pediatric patient cohort. A comprehensive clinical update is anticipated at the American Heart Association (AHA) Scientific Sessions 2021 being held virtually November 13-15, 2021.
- **Presented previously disclosed data from ongoing RP-A501 Phase 1 trial in Danon Disease at HFSA.** The late-breaking oral presentation at the Heart Failure Society of America (HFSA) Annual Scientific Meeting 2021 overviewed data from the low-dose (6.7e13 vg/kg) adult cohort which demonstrated RP-A501 was well tolerated and showed progressive and durable clinical benefit.

Leukocyte Adhesion Deficiency-I (LAD-I):

- **Presented positive interim data updates from RP-L201 LAD-I trial at ESGCT.** The oral presentation included data from the initial seven patients with severe LAD-I who were treated with RP-L201 in the Phase 1/2 trial. The safety profile of RP-L201 appears favorable with all infusions well tolerated and no drug product-related serious adverse events. Preliminary efficacy was evident in all seven patients, including two patients with at least 12 months of follow-up. All seven

patients demonstrated durable neutrophil CD18 expression that exceeded the 4-10% threshold associated with survival into adulthood and consistent with reversal of the severe LAD-I phenotype. Peripheral blood vector copy number (VCN) levels have been stable and in the 0.5 – 2.5 copy per genome range. No patients have had LAD-I related infections requiring hospitalization subsequent to hematopoietic reconstitution post RP-L201. Additional clinical data are anticipated at the 63rd American Society of Hematology (ASH) Annual Meeting being held December 11-14, 2021.

Fanconi Anemia (FA):

- **Presented previously disclosed clinical data from RP-L102 Fanconi Anemia (FA) program at ESGCT.** The oral presentation included data for nine pediatric patients treated with RP-L102, Rocket's *ex vivo* lentiviral gene therapy candidate, in the ongoing clinical trials. RP-L102 demonstrated a highly favorable safety profile with all subjects being treated without conditioning and with no sign of dysplasia or other concerning features. RP-L102 showed evidence of preliminary engraftment in at least six of nine patients. A clinical update is anticipated at the 63rd ASH Annual Meeting being held December 11-14, 2021.

Pyruvate Kinase Deficiency (PKD):

- **Presented incremental updates from RP-L301 Pyruvate Kinase Deficiency (PKD) program at ESGCT.** The oral presentation included data from two adult patients treated with RP-L301, Rocket's *ex vivo* lentiviral gene therapy candidate, in the ongoing Phase 1 trial. The safety profile of RP-L301 appears favorable with no infusion-related serious adverse events at up to 9 months post-infusion. Both patients have normalized hemoglobin, improved hemolysis markers and no red blood cell transfusion requirements post-engraftment, as well as no hospitalizations post-hospital discharge. A clinical update is anticipated at the 63rd ASH Annual Meeting being held December 11-14, 2021.
- **Published peer-reviewed studies supporting scientific rationale for clinical results observed to date in RP-L301 trial.** "Preclinical studies of efficacy thresholds and tolerability of a clinically ready lentiviral vector for pyruvate kinase deficiency treatment" was published in *Molecular Therapy: Methods & Clinical Development*. The studies demonstrate that in the murine model of PKD, reversion of the phenotype is seen when at least 20% of hematopoietic stem/progenitors are corrected, with comprehensive reversion seen when at least 30% of these progenitors are corrected. This provides meaningful scientific rationale for the clinical results that have been observed to date for the first two patients who have received RP-L301.

Infantile Malignant Osteopetrosis (IMO):

- **Presented preclinical data supporting ongoing RP-L401 Infantile Malignant Osteopetrosis (IMO) Phase 1 trial at ESGCT.** The Phase 1 trial is designed to assess safety and tolerability, as well as preliminary efficacy, of RP-L401, Rocket's *ex vivo* lentiviral gene therapy candidate. A clinical update on the Phase 1 trial is anticipated later in the fourth quarter.

Anticipated Milestones

- Fanconi Anemia (RP-L102)
  - Updated "Process B" data (Q4 2021)
- LAD-I (RP-L201)
  - Longer-term Phase 2 data (Q4 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)
  - Phase 1 clinical update (Q4 2021)

Third Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of September 30, 2021, were $421.5 million.
- **R&D expenses.** Research and development expenses were $40.0 million for the three months ended September 30, 2021, compared to $21.7 million for the three months ended September 30, 2020, due to increase in manufacturing and development costs, an increase in new research agreements of $7.6 million in non-cash expenses, increases in compensation and benefits due to increased R&D headcount, and an increase in non-cash stock compensation expense.
- **G&A expenses.** General and administrative expenses were $9.7 million for the three months ended September 30, 2021, compared to $5.7 million for the three months ended September 30, 2020, due to an increase in non-cash stock compensation expense, an increase in compensation and benefits expense due to increased G&A headcount and an increase in commercial preparation expenses.
- **Net loss.** Net loss was $50.1 million or $0.79 per share (basic and diluted) for the three months ended September 30,

2021, compared to $29.1 million or $0.53 per share (basic and diluted) for the three months ended September 30, 2020.

- **Shares outstanding.** 64,442,601 shares of common stock were outstanding as of September 30, 2021.

Financial Guidance

Rocket expects its balance in cash, cash equivalents and investments of $421.5 million as of September 30, 2021 to fund its operations into the second half of 2023, including the continued buildout and initiation of AAV cGMP manufacturing capabilities at our Cranbury, New Jersey R&D and manufacturing facility and continued development of our five clinical programs.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) 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 and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, Rocket's plans for the advancement of its Danon Disease program following the lifting of the FDA's clinical hold 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.

Selected Financial Information

Operating Results:
(amounts in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
Operating expenses:				
Research and development	$ 39,975	$ 21,657	$ 93,315	$ 55,345
General and administrative	9,671	5,730	29,600	19,720
Total operating expenses	49,646	27,387	122,915	75,065
Loss from operations	(49,646)	(27,387)	(122,915)	(75,065)
Research and development incentives	-	-	500	-
Interest expense	(534)	(1,967)	(2,514)	(5,326)
Interest and other income net	806	518	2,218	1,913
Amortization of premium on investments - net	(744)	(244)	(2,111)	(306)
Net loss	$ (50,118)	$ (29,080)	$ (124,822)	$ (78,784)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.79)	$ (0.53)	$ (1.99)	$ (1.43)
Weighted-average common shares outstanding - basic and diluted	63,825,461	55,188,956	62,828,612	55,077,254

	September 30, 2021	December 31, 2020
Cash, cash equivalents and investments	421,459	482,719
Total assets	530,241	590,824
Total liabilities	44,583	87,305

Total stockholders' equity	485,658	503,519

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