Rocket Pharmaceuticals Reports Fourth Quarter and Full Year 2020 Financial and Operational Results

February 25, 2021

— Achieved Favorable Clinical Results Across Four First-in-Class Gene Therapy Programs for Danon Disease, FA,LAD-I and PKD —

— Announced >50% Protein Expression with Stabilization and Biomarker Improvement Including Cardiac Output in Low Dose Cohort for Danon Disease Heart Failure Program —

— Longer-term Data from FA and LAD-I Registration-Enabling Trials Anticipated in 2Q 2021, Danon and PKD Programs in 2H 2021; Preliminary Data in IMO Anticipated 2H 2021 —

— Clinical GMP Manufacturing to be Initiated in 2021 —

—~$300 Million Equity Offering Strengthens Balance Sheet and Extends Cash Runway into 2H 2023 —

CRANBURY, N.J.--(BUSINESS WIRE)--Feb. 25, 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 and operational results for the fourth quarter and year ended December 31, as well as guidance for anticipated 2021 milestones.

“2021 is poised to be another exciting year for Rocket,” said Gaurav Shah, M.D., Chief Executive Officer of Rocket. “We anticipate clinical data from all five of our gene therapy programs, initiation of our first in-house AAV cGMP production from our new manufacturing facility, and continued strengthening of our organization, manufacturing capabilities and infrastructure. We look forward to continued validation of our gene therapy platforms with updated results from registrational studies in FA and LAD-I, longer term findings in Danon Disease and PKD, and clinical proof-of-concept for IMO. Under strengthened leadership, we will complete buildout of Rocket’s new R&D and manufacturing facility leading to our first in-house cGMP production and work toward commercial readiness. Achieving these milestones will advance our mission to help improve the lives of patients facing rare and devastating childhood disorders.”

“This new year comes on the heels of a seminal period in which we achieved transformational results across all four clinical programs including the near doubling of hemoglobin in our PKD trial, bone marrow normalization in our FA program, and reversal of disease phenotype in our Danon Disease study. These results demonstrated the significant potential of our platforms to change the lives of patients across many rare disease indications who currently have limited or no treatment options. We also expanded our organizational, R&D, and manufacturing capabilities toward commercial preparedness, and substantially strengthened our balance sheet. This work was achieved during a disruptive global pandemic, thanks to our employees, patients, partners, and the scientific community as a whole who met every challenge with determination and care. We look forward to continuing our steady progress by building on these results with hard work and perseverance as we move forward in 2021.”

Key Pipeline and Operational Updates

- **Announced positive preliminary clinical data from RP-A501 trial for Danon Disease.** The Company reported positive gene expression, clinical biomarker and preliminary functional data from the Phase 1 trial. Preliminary results from the three patients treated at the low dose demonstrated that RP-A501 was generally well tolerated with a manageable safety profile. In the higher-dose cohort, one patient experienced a drug-related severe adverse event related to complement activation. The patient’s risk was enhanced by high weight, vector dose, and pre-existing adeno-associated virus (AAV) immunity. All patients have fully recovered from immune-related sequelae. All three low dose participants demonstrated evidence of cardiac *LAMP2B* expression by Western blot and/or immunohistochemistry, and two who were compliant with the immunosuppressive regimen showed >50% expression and clinical biomarker improvements consistent with improved cardiac function. Brain natriuretic peptide and autophagic vacuoles improved in all three patients, while creatine kinase myocardial band either improved or stabilized in the immunosuppressive-compliant patients. Benefit observed in all three patients serves as clinical proof-of-concept. The Phase 1 trial continues to enroll patients with updated data anticipated in the second half of 2021.

- **Announced positive longer-term updates from the RP-L102 Fanconi Anemia (FA) and RP-L201 Leukocyte Adhesion Deficiency-I (LAD-I) programs supporting potential registration path using "Process B".** The RP-L102 data presented at the 62nd American Society of Hematology Annual Meeting (ASH) are from seven of the nine patients treated in the U.S. Phase 1 and global Phase 2 studies. RP-L102 was generally well tolerated with no significant safety issues reported with infusion or post-treatment. Evidence of preliminary engraftment was observed in five out of seven patients. Two of three patients with greater than 12-months follow-up showed evidence of increasing engraftment, mitomycin-C resistance and stable blood counts. One patient's course was complicated by *Influenza B* resulting in progressive bone marrow failure. The patient received a successful bone marrow transplant. The RP-L201 data presented are from three pediatric patients with severe LAD-I. RP-L201 was well tolerated with no safety issues reported with infusion or post-treatment. All patients achieved hematopoietic reconstitution within 5-weeks. The two patients with greater than 6-months follow-up demonstrated sustained CD18 expression of 23% to 40%, far exceeding the 4-10% threshold associated with survival into adulthood. Further updates from the RP-L102 and RP-L201 programs are anticipated in the second quarter of 2021.

- **Announced preliminary clinical data from the RP-L301 Pyruvate Kinase Deficiency (PKD) program demonstrating its potential to address the root cause of the disease for the first time at the genetic level.** The data presented at ASH are from two adult patients with significant anemia and transfusion requirement. RP-L301 was well tolerated in the first patient treated, with no serious safety issues or infusion-related complications observed 3-months post treatment. This patient nearly doubled hemoglobin levels to a normal range and normalized additional hemolysis markers. The second patient was recently treated with RP-L301. Additional Phase 1 data are anticipated in the second half of 2021.

- **Continued buildout of new Research and Development (R&D) and Manufacturing facility**. In January, Rocket announced plans for its new R&D and Chemistry, Manufacturing and Controls (CMC) operation which will also serve as the Company's new headquarters in Cranbury, New Jersey. This new 103,720 ft^2 facility will support clinical development of Rocket's growing pipeline of lentivirus and AAV gene therapies, with space scaled for AAV Current Good Manufacturing Practice (cGMP) production. The other half features state-of-the-art R&D labs to support the expanding pipeline and Quality Control laboratories to support CMC development for process and analytics. The first cGMP production at this facility will be initiated in 2021.

- **Announced FDA clearance of Investigational New Drug (IND) application for RP-L401 gene therapy for Infantile Malignant Osteopetrosis (IMO).** RP-L401 is Rocket's lentiviral vector-based gene therapy for the treatment of IMO, a rare, severe monogenic bone resorption disorder characterized by skeletal deformities, neurologic abnormalities, and bone marrow failure. Preliminary data are anticipated in the second half of 2021.

- **Expanded clinical sites for FA, Danon, LAD-I and IMO trials, adding to global centers of excellence.** The newly added centers, which include some of the leading gene therapy research programs in the world, include: Great Ormond Street Hospital in London, Children's Hospital of Philadelphia (CHOP), the University of Minnesota, the University of California, Los Angeles (UCLA), and the University of Colorado. These additional sites will expand patient access to Rocket's clinical trials worldwide.

- **Received Fast Track Designation for IMO and Rare Pediatric Disease Designation for Danon Disease programs from FDA.** The FDA's Fast Track program facilitates the development of products intended to treat serious conditions that have the potential to address unmet medical needs and enables greater access to the FDA for the purpose of expediting the product's development, review, and potential approval. The FDA grants Rare Pediatric Disease Designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the U.S.

- **The California Institute for Regenerative Medicine (CIRM) awarded Rocket a $3.7 million CLIN2 grant award to support the clinical development of RP-L401 for IMO.** Proceeds from the grant will help fund clinical trial costs, as well as provide manufactured drug product for Phase 1 patients enrolled at the U.S. clinical trial site at UCLA.

- **Strengthened organization with new Officer appointments.** In January 2021, Carlos Garcia Parada joined Rocket as Chief Financial Officer (CFO). Prior to joining Rocket, Carlos held several roles of increasing responsibility over 30 years at Novartis where he led finance organizations in the US and other major pharmaceutical markets. He most recently served as the Vice President & Finance Head Oncology USA, a position he held since January 2011. In February 2021, Kinnari Patel, PharmD, MBA was appointed President and Chief Operating Officer (COO). Since joining Rocket in 2015, Dr. Patel has overseen pipeline development and execution, IT, HR, regulatory, clinical execution, alliance management, CMC and quality organizations.

- **Continued focus on supporting the patient community by serving as a pivotal resource for patient education during the COVID-19 pandemic, and by recognizing Rare Disease Day through "Lighting up for Rare."** As part of

Rocket's mission to be a patient-centric gene therapy leader, throughout 2020 as patients faced significant difficulties presented by the pandemic, Rocket hosted a variety of programs and events to support patients through this critical time. Events included 1) virtual sessions for patients with Danon Disease and their families; 2) Rocket's third PKD day; and 3) participation in the 2020 Fanconi Anemia Family Meeting, hosted by the Fanconi Anemia Research Fund. In continuation of Rocket's commitment to Patients, Rocket is recognizing 2021 Rare Disease Day by leading the "Lighting up for Rare" campaign hosting a month of expert panel series driving awareness of rare diseases, culminating on February 28, 2021 as iconic landmarks across the U.S., including the Empire State Building, NASDAQ Tower, and Niagara Falls, are expected to be lit to drive awareness and foster solidarity among the global rare disease community.

- **Strengthened balance sheet, with ~$300 million equity offering and extension and partial conversion of existing convertible notes.** On December 14, 2020, Rocket closed an upsized underwritten public offering of 5,339,286 shares of its common stock, inclusive of greenshoe, at the public offering price of $56.00 per share. This capital raise extends Rocket's cash runway to the second half of 2023. In the first half of 2020, Rocket entered into two privately negotiated agreements with certain holders of its outstanding 5.75% Convertible Senior Notes due 2021. Rocket exchanged approximately $46.85 million aggregate principal amount of the Old Notes for new 6.25% Convertible Senior Notes due 2022. Approximately $5.15 million aggregate principal amount of the 2021 Notes will remain outstanding. Additionally, $8.5 million of the 2022 Notes have been converted into shares by certain holders. At year-end $38.35 million of the 2022 Notes remain outstanding.

Anticipated Milestones

- Fanconi Anemia (RP-L102)
  - Updated "Process B" data (2Q21)
- Danon Disease (RP-A501)
  - Longer-term Phase 1 data (2H21)
- LAD-I (RP-L201)
  - Initial Phase 2 data (2Q21)
- PKD (RP-L301)
  - Longer-term Phase 1 data (2H21)
- IMO (RP-L401)
  - Initial Phase 1 data (2H21)

Upcoming Investor Conferences

- 10th Annual Global SVB Leerink Healthcare Conference—February 25, 2021
- Cowen's 41 st Annual Healthcare Conference—March 2, 2021
- Oppenheimer's 31 st Annual Healthcare Conference—March 16, 2021

Fourth Quarter and Full Year 2020 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of December 31, 2020, were $482.7 million.
- **Debt.** Our balance sheet includes $39.9 million of fully convertible notes.
- **R&D expenses.** Research and development expenses were $51.0 million and $106.4 million for the three and twelve months ended December 31, 2020, compared to $14.7 million and $58.6 million for the three and twelve months ended December 31, 2019. The increase in research and development expenses for the three and twelve months ended December 31, 2020, was primarily driven by an increase in new research agreements totaling $26.5 million in non cash expenses, clinical trial expenses, manufacturing expenses, compensation expense due to increased headcount and amortization expense related to the Cranbury, NJ facility lease.
- **G&A expenses.** General and administrative expenses were $8.2 million and $27.9 million for the three and twelve months ended December 31, 2020, compared to $5.0 million and $17.5 million for the three and twelve months ended December 31, 2019. The increase in general and administrative expenses for three and twelve months ended December 31, 2020, was primarily driven by fees incurred in connection with the convertible note exchange, an increase in compensation expense and non-cash stock-based compensation expense due to increased headcount, and an increase in office and administrative expenses.
- **Net loss.** Net loss was $60.9 million and $139.7 million or $1.08 and $2.52 per share (basic and diluted) for the three and twelve months ended December 31, 2020, compared to $19.9 million and $77.3 million or $0.39 and $1.58 per share (basic and diluted) for the three and twelve months ended December 31, 2019.
- **Shares outstanding.** 60,996,367 shares of common stock were outstanding as of December 31, 2020.

Financial Guidance

- **Cash position.** As of December 31, 2020, we had cash, cash equivalents and investments of $482.7 million. Rocket

expects such resources will be sufficient to fund its operations into the second half of 2023.

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 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 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, filed November 6, 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

	Three Months Ended December 31,		Year Ended December 31,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 51,036	$ 14,663	$ 106,382	$ 58,623
General and administrative	8,201	4,981	27,921	17,528
Total operating expenses	59,237	19,644	134,303	76,151
Loss from operations	(59,237)	(19,644)	(134,303)	(76,151)
Research and development incentives	-	-	-	250
Interest expense	(1,640)	(1,344)	(6,967)	(5,958)
Interest and other income net	235	889	2,150	3,414
(Amortization of premium) accretion of discount on investments - net	(274)	244	(580)	1,175
Total other expense, net	(1,679)	(211)	(5,397)	(1,119)
Net loss	$ (60,916)	$ (19,855)	$ (139,700)	$ (77,270)

	December 31, 2020	December 31, 2019
Cash, cash equivalents and investments	482,719	304,115
Total assets	590,824	372,121
Total liabilities	87,305	64,824
Total stockholders' equity	503,519	307,297

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