Rocket Pharmaceuticals Reports Second Quarter 2022 Financial Results and Highlights Recent Progress

August 8, 2022

—Reported positive safety data at ASGCT from pediatric cohort of Phase 1 Danon Disease study that demonstrated RP-A501 was well-tolerated; efficacy update across pediatric as well as adult cohorts on track for late Q3 2022 —

—Announced top-line data at ASGCT from pivotal Phase 2 trial for severe LAD-I that showed RP-L201 was well-tolerated with 100% overall survival at one year; regulatory filings anticipated in first half of 2023 —

—Primary endpoint met for Fanconi Anemia pivotal Phase 2 trial, FDA dialogue initiated for BLA planning; continued data readouts for Fanconi Anemia and Pyruvate Kinase Deficiency programs expected in Q4 2022 —

—Achieved Current Good Manufacturing Practice (cGMP) readiness milestone for state-of-the-art AAV manufacturing facility in Cranbury, N.J.; continuing on the path toward commercialization —

—Appointed proven gene therapy technical operations expert and manufacturing leader Mayo Pujols as first Chief Technical Officer —

— Cash, cash equivalents and investments of $321.4M; expected operational runway into first half of 2024 —

CRANBURY, N.J.--(BUSINESS WIRE)--Aug. 8, 2022-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage, clinical biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders with high unmet need, today reports financial results for the quarter ending June 30, 2022, and updates from the Company's key pipeline developments, business operations and upcoming milestones.

"Rocket continues on an excellent trajectory following a strong and highly productive quarter that featured positive results across all four clinical programs, concrete steps taken toward manufacturing readiness and leadership, and filing preparedness for our first two gene therapies," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. "Positive safety data from our Phase 1 study for Danon Disease demonstrated RP-A501 was well-tolerated in both pediatric patients. We now look forward to presenting early efficacy data from the pediatric cohort with three to six months of follow-up in late Q3. If early signals of efficacy and ongoing tolerability in the pediatric cohort are demonstrated along with evidence of longer-term safety and efficacy in the adult cohort, we expect to begin Phase 2 pivotal trial planning activities in Q4, including FDA alignment on study design and endpoints."

Dr. Shah continued, "In parallel, we reached an understanding with the FDA on chemistry, manufacturing and controls (CMC) requirements to start AAV cGMP manufacturing at our in-house facility as well as potency assay plans for a Phase 2 pivotal trial in Danon Disease. To further strengthen our manufacturing and commercial capabilities, we appointed Mayo Pujols, one of the most seasoned cell and gene therapy technical operations and manufacturing leaders in the industry, as our Chief Technical Officer."

"This quarter, we also shared positive top-line data from our pivotal Phase 2 trial for severe LAD-I showing that RP-L201 was well-tolerated and associated with 100% overall survival at one year," said Dr. Shah. "We have initiated work towards regulatory filings planned for the first half of 2023. In addition, based on achievement of the primary endpoint in our pivotal Phase 2 study for Fanconi Anemia, we have initiated FDA dialogue around BLA planning activities."

"These steps deliver on our effort to best leverage our strong cash position to create value as we embark on transitioning from a clinical to a commercial-stage company. Importantly, we continue to maintain a healthy operational cash runway into the first half of 2024," concluded Dr. Shah. "Taken together, our positive data updates and steady progress this quarter continue to motivate us to push the boundaries of science and deliver on our mission to seek gene therapy cures for patients and families facing devastating, life-threatening diseases."

Key Pipeline and Operational Updates

- **Danon, FA, LAD-I and PKD trials remain on track.** All 2022 milestones remain on track, including pediatric efficacy data readout from the Phase 1 Danon Disease trial in late Q3, updated results for FA and preliminary Phase 1 data readout for PKD in Q4. The originally planned Q3 topline readout for FA was achieved earlier than anticipated in Q2 when the trial met

its primary endpoint.

- **Announced positive clinical data from ongoing Phase 1 trial of RP-A501 for Danon Disease.** Data presented at the 2022 Annual Meeting of the ASGCT included new initial safety data from the low-dose (6.7 x 10^{13}GC/kg; n=2) pediatric cohort as of April 30, 2022, cut-off date. Results demonstrated RP-A501 was well-tolerated in both patients. The patients were observed to have normal-range platelets, diminished complement activation and no complement-related adverse events. Early efficacy and safety data with three to six months of follow up from the pediatric patient cohort of the Phase 1 trial are expected in late Q3 2022; longer-term safety and efficacy for adults will also be presented. Pending health authority interactions, Phase 2 trial planning activities are expected to begin in Q4 of 2022.

- **Announced positive clinical data from ongoing pivotal Phase 2 trial of RP-L102 for Fanconi Anemia (FA).** Data presented at the 2022 Annual Meeting of the ASGCT included updated data from the initial nine of 12 FA patients who received RP-L102 as of the April 4, 2022, cut-off date. Five of nine evaluable patients had increased resistance to mitomycin-C in bone marrow-derived colony forming cells, ranging from 21% to 42% at 12 to 18 months, increasing to 51% to 94% at 18 to 21 months. The primary endpoint has been achieved, based on a trial protocol in which statistical and clinical significance requires a minimum of five patients to attain increased MMC resistance at least 10% above baseline at two or more timepoints and concomitant evidence of genetic correction and clinical stabilization. The safety profile of RP-L102 appears favorable with no signs of dysplasia, clonal dominance or oncogenic integrations; as previously reported, one patient experienced a Grade 2 transient infusion related reaction, which resolved. Based on these results, the Company has initiated FDA dialogue in anticipation of BLA filing activities.

- **Announced positive clinical data from ongoing pivotal Phase 2 trial of RP-L201 for Leukocyte Adhesion Deficiency-I (LAD-I).** Data presented at the 2022 Annual Meeting of the ASGCT included efficacy and safety data at three to 24 months of follow-up after RP-L201 infusion for all nine patients as of the March 9, 2022, cut-off date and overall survival data for the seven patients with at least 12 months after infusion. All patients demonstrated sustained CD18 restoration and expression on more than 10% of neutrophils (range: 20%-87%, median: 56%), as well as a statistically significant reduction in the rate of all-cause hospitalizations and severe infections, relative to pre-treatment. At one year, the overall survival without allogeneic hematopoietic stem cell transplantation across the cohort was 100% based on the Kaplan-Meier estimate. RP-L201 was well-tolerated with no drug product-related serious adverse events as of the cut-off date. Based on the data presented at ASGCT, Rocket has initiated discussions with health authorities on filing plans for RP-L201 for the treatment of severe LAD-I and anticipates filings in the first half of 2023.

- **Announced positive clinical data from ongoing Phase 1 trial of RP-L301 for Pyruvate Kinase Deficiency (PKD).** Data presented at the 2022 Annual Meeting of the ASGCT included interim data from two adult PKD patients with severe and transfusion dependent anemia who were treated with RP-L301 as of the April 13, 2022, cut-off date. At 18 months post-infusion, both patients had sustained transgene expression, normalized hemoglobin, improved hemolysis, no red blood cell transfusion requirements post-engraftment and improved quality of life, both reported anecdotally and as documented via formal quality of life assessments. RP-L301 appears favorable with no drug product-related serious adverse events through 18 months post-infusion. Transient transaminase elevation was seen in both patients post-therapy/conditioning, with no clinical stigmata of liver injury and subsequent resolution without clinical sequelae. Enrollment in the pediatric cohort is ongoing, and additional Phase 1 data are expected in Q4 2022.

- **Achieved in-house AAV cGMP manufacturing readiness.** The Company's state-of-the-art, 103,720 ft ^{2} manufacturing facility in Cranbury, New Jersey has been scaled up to manufacture AAV drug product for a planned Phase 2 pivotal study in Danon Disease. The facility also houses lab space for research and development and quality.

- **Appointed Chief Technical Officer and expanded leadership team**. In July 2022, Mayo Pujols joined the Company as its first Chief Technical Officer and Executive Vice President. Mr. Pujols brings nearly three decades of experience from leadership roles across technical operations, quality operations, validation, process development and Good Manufacturing Practice (cGMP) manufacturing. He most recently served as Chief Executive Officer of Andelyn Biosciences, a leading gene therapy contract development and manufacturing organization (CDMO), and prior to Andelyn was the Head of Global Cell and Gene Technical Development and Manufacturing for Novartis Pharmaceuticals. Mr. Pujols has also served in key technical operations and manufacturing roles at Celgene, Merck, Advaxis, MedImmune and Schering-Plough. In his new role, Mr. Pujols leads the technical operations function and chemistry, manufacturing and controls (CMC) for all lentiviral programs. Additionally, he leads the Company's state-of-the-art adeno-associated virus (AAV) manufacturing facility.

Upcoming Investor Conferences

- BTIG Biotechnology Conference – August 8-9, 2022
- Citi 17th Annual BioPharma Conference – September 7-8, 2022
- Morgan Stanley 20th Annual Global Healthcare Conference – September 12-14, 2022

Second Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of June 30, 2022, were $321.4 million.
- **R&D expenses.** Research and development expenses were $41.4 million for the three months ended June 30, 2022, compared to $24.5 million for the three months ended June 30, 2021. The increase in research and development expense was primarily driven by an increase in manufacturing and development costs, an increase in compensation and benefits expense due to increased R&D headcount and an increase in laboratory supplies.
- **G&A expenses.** General and administrative expenses were $12.9 million for the three months ended June 30, 2022, compared to $9.5 million for the three months ended June 30, 2021. The increase in general and administrative expenses was primarily driven by an increase in commercial preparation expenses, an increase in compensation and benefits expense due to increased G&A headcount, and an increase in legal expenses.
- **Net loss.** Net loss was $54.4 million or $0.83 per share (basic and diluted) for the three months ended June 30, 2022, compared to $34.5 million or $0.55 per share (basic and diluted) for the three months ended June 30, 2021.
- **Shares outstanding.** 65,837,894 shares of common stock were outstanding as of June 30, 2022.

Financial Guidance

- **Cash position.** As of June 30, 2022, the Company had cash, cash equivalents and investments of $321.4 million. As of June 30, 2022, the Company sold 1.3 million shares of common stock for net proceeds of $17.3 million under its at-the-market facility. With the at-the-market facility proceeds, the Company expects such resources will be sufficient to fund its operating expenses and capital expenditure requirements into the first half of 2024, 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 four clinical programs as well as future pipeline 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, 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 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 2022 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), and Danon Disease, the expected timing and data readouts of Rocket’s ongoing and planned clinical trials, the expected timing and outcome of Rocket’s regulatory interactions and planned submissions, Rocket’s plans for the advancement of its Danon Disease program 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, 2021, filed February 28, 2022 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. 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 June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	41,356	24,530	72,150	52,839
General and administrative	12,854	9,518	24,624	20,431
Total operating expenses	54,210	34,048	96,774	73,270
Loss from operations	(54,210)	(34,048)	(96,774)	(73,270)

Research and development incentives	-	-	-	500
Interest expense	(465)	(251)	(928)	(1,980)
Interest and other income, net	669	501	1,291	1,412
Amortization of premium on investments - net	(396)	(727)	(973)	(1,366)
Net loss	$ (54,402)	$ (34,525)	$ (97,384)	$ (74,704)
Net loss per share attributable to common stockholders - basic and diluted	$ (0.83)	$ (0.55)	$ (1.50)	$ (1.20)
Weighted-average common shares outstanding - basic and diluted	65,476,531	63,061,232	64,995,797	62,321,926

	June 30, 2022	December 31, 2021
Cash, cash equivalents, and investments	$ 321,368	$ 388,740
Total assets	431,852	497,020
Total Liabilities	44,156	42,296
Total stockholders' equity	387,696	454,724

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