Rocket Pharmaceuticals Reports Second Quarter 2018 Financial Results and Operational Highlights

August 8, 2018

- Achieved Regulatory Designations for Fanconi Anemia (FA) in the U.S. and Europe; Registrational Trial on Track for 2019 -

- Presented Promising Fanconi Anemia Data at ASGCT -

- Adeno-associated Viral Vector Program Disclosure on Track for Fourth Quarter of 2018 -

NEW YORK--(BUSINESS WIRE)--Aug. 8, 2018-- Rocket Pharmaceuticals, Inc. (NASDAQ:RCKT) ("Rocket"), a leading U.S.-based multi-platform gene therapy company, today reported financial results for the quarter ended June 30, 2018, and provided an update on the Company's recent achievements, as well as upcoming milestones.

"Rocket made significant progress on our clinical, regulatory and corporate initiatives in the second quarter," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We are pleased with the positive clinical data from our FA program that were presented at ASGCT and look forward to additional data over the next 12-18 months. The momentum has continued with recent regulatory designations for FA, including Rare Pediatric Disease from the U.S. Food and Drug Administration (FDA) and Advanced Therapy Medicinal Product (ATMP) by the European Medicines Agency (EMA). These positive steps set the stage nicely for a global registrational study in 2019."

"Our additional gene therapy pipeline programs for devastating rare diseases remain on track. These include our products for Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and our undisclosed adeno-associated viral vector (AAV) program. We expect to disclose the indication and share preclinical data from our AAV program later this year, and clinical data on up two programs in 2019. Our progress to date has been a true collaboration between the Rocket team, our partners, physicians, and the patients we serve. We look forward to meeting the milestones ahead."

Recent Pipeline and Corporate Updates

- **Rare Pediatric Disease Designation for FA**. In July 2018, the Company was notified that it received Rare Pediatric Disease designation from the FDA for RP-L102 for the treatment of FA Type A. The FDA defines a "rare pediatric disease" as a serious and life-threatening disease that affects less than 200,000 people in the U.S. that are aged between birth to 18 years. The Rare Pediatric Disease designation program allows for a Sponsor who receives an approval for a product to potentially qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.
- **Advanced Therapy Medicinal Product Classification for FA**. In June 2018, the Company was notified that the EMA classified RP-L102 as an ATMP. The ATMP classification recognizes and defines medicines for human use that are considered gene-, tissue- or cell-based therapies. The key benefit of ATMP classification is the early involvement and guidance from the EMA's Committee of Advanced Therapies, which is the regulatory reviewing body for gene therapies.
- **Phase 1/2 data of RP-L102 in FA shows promising engraftment and chromosomal stability leading to improved bone marrow functionality.** At the ASGCT Annual Meeting in May 2018, updated data from the ongoing Phase 1/2 clinical trial of RP-L102 was presented and included data from four patients that have been followed for 12-24 months and a fifth patient, treated with transduction-enhanced RP-L102, that was followed for two months. All patients demonstrated continued improvement in engraftment following administration of RP-L102 with sustained phenotypic reversals and earlier evidence of gene correction seen in higher-dosed patients. The progressive increases of corrected versus non-corrected peripheral blood leukocytes indicate the potential of RP-L102 to restore the functionality of bone marrow hematopoietic stem cells. The one patient that received transduction enhanced RP-L102 showed the highest transduction efficiency seen to date in all five patients treated, with a preliminary drug product vector copy number (VCN) of ~2.5 – 3, and a cell dose considered below the threshold level of 500,000 CD34+/kg. Rocket plans to engage with regulatory authorities to progress RP-L102 towards a potential global registrational study in 2019.

- **Stanford University research collaboration**. In May 2018, Rocket and the Stanford University School of Medicine announced a strategic collaboration to support the advancement of FA and PKD gene therapy research. Under the terms of the collaboration agreement, Stanford will serve as a lead clinical trial research center in the U.S. for the planned FA registrational trial and would also be the lead site for PKD clinical trials. The project will also separately evaluate the potential for non-myeloablative, non-genotoxic antibody-based conditioning regimens as a future development possibility that may be applied across bone marrow-derived disorders.
- **Strengthened management team with addition of former FDA Director of the Office of Orphan Products Development (OOPD)**. Gayatri R. Rao, M.D., J.D., was appointed Vice President, Regulatory Policy and Patient Advocacy, in May 2018. Dr. Rao most recently served as Director of the OOPD within the FDA for the last five years where she was responsible for implementing statutory programs focused on promoting the development of medical products for rare diseases. In her new role at Rocket, Dr. Rao will support the development of global regulatory policies and strategies, patient advocacy initiatives, and rare disease natural history studies.

Anticipated Milestones

- Preclinical data and disclosure of the AAV-based gene therapy program (4Q18)
- Investigational Medicinal Product Dossier (IMPD) filing in Spain for the LAD-I program (4Q18)
- IMPD filing in Spain for the PKD program (Early 2019)
- Additional FA patient data (Next 12-18 months)
- Investigational New Drug (IND) application filing in the U.S. for the AAV-based program (2019)
- IND application filing in the U.S. for the FA program (2019)

September Conferences

- Citi's 13th Annual Biotech Conference – September 5-6, 2018 in Boston, MA
- Morgan Stanley 16th Annual Global Healthcare Conference – September 12-14, 2018 in New York, NY
- Oppenheimer Specialty Pharma & Rare Disease Fall Summit – September 25-26 in New York, NY
- Jefferies Gene Therapy and Editing Summit – September 27, 2018 in New York, NY

Second Quarter 2018 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of June 30, 2018, were $171.5 million, which includes a $52.0 million fully convertible debenture which expires in 2021.
- **R&D expenses.** Research and development expenses were $10.8 million and $16.5 million for the three and six months ended June 30, 2018, compared to $2.8 million and $5.1 million for the three and six months ended June 30, 2017.
- **G&A expenses.** General and administrative expenses were $4.1 million and $12.8 million for the three and six months ended June 30, 2018, compared to $0.7 million and $1.3 million for the three and six months ended June 30, 2017.
- **Net loss.** Net loss was $15.8 million and $31.1 million or $(0.40) and $(0.82) per share (basic and diluted) for the three and six months ended June 30, 2018, compared to $3.3 million and $6.2 million or $(0.49) and $(0.91) per share (basic and diluted) for the three and six months ended June 30, 2017.
- **Shares outstanding.** Approximately 39.5 million shares of common stock were outstanding as of June 30, 2018.

Financial Guidance

- **Cash position**. Based on its current operating plan, Rocket expects its cash, cash equivalents and investments as of June 30, 2018, will be sufficient to run its operations into 2020.

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 lead clinical program is a LVV-based gene therapy for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer. Preclinical studies of additional bone marrow-derived disorders are ongoing and target Pyruvate Kinase Deficiency (PKD), Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO). Rocket is also developing an AAV-based gene therapy program for an undisclosed rare pediatric disease. For more information about Rocket, please visit www.rocketpharma.com.

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, including in collaboration with academic partners, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO), 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, Rocket's ability to commence a registrational study in FA within the projected time periods, 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, 2017. 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,	
	2018	**2017**	**2018**	**2017**
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	10,772	$ 2,819	16,525	5,104
General and administrative	4,100	702	12,752	1,287
Total operating expenses	14,872	3,521	29,277	6,391
Loss from operations	(14,872)	(3,521)	(29,277)	(6,391)
Research and development incentives	-	192	186	192
Interest expense	(1,363)	-	(2,834)	-
Interest income	473	-	805	-
Other income	(5)	-	10	-
Net loss	$ (15,767)	$ (3,329)	$ (31,110)	$ (6,199)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.40)	$ (0.49)	$ (0.82)	$ (0.91)
Weighted-average common shares outstanding - basic and diluted	39,483,006	6,795,627	37,954,972	6,795,627

Selected Balance Sheet Information

(amounts in thousands)

	June 30, 2018	**December 31, 2017**
Cash, cash equivalents and investments	171,466	18,142
Total assets	207,574	20,147
Total liabilities	50,498	4,628
Total shareholders' equity	157,076	15,519

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