



Rocket Pharmaceuticals Reports First Quarter 2018 Financial Results and Operational Highlights

May 11, 2018

Four Gene Therapy Programs Potentially in Clinic in 2019 with Clinical Data from up to Two Programs

Regulatory Submissions on Schedule for LAD-I, PKD and AAV Programs Over the Next 12 Months

Development of a Long-term Sustainable Growth Company Leveraging a Multi-Platform Approach

NEW YORK--(BUSINESS WIRE)--May 11, 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 March 31, 2018, and provided an update on the Company's recent achievements, as well as upcoming milestones.

"2018 is off to a great start for Rocket. During the first quarter, we continued to make excellent progress advancing our pipeline of lentiviral vector (LVV) and adeno-associated viral vector (AAV) gene therapy programs towards key value inflection points. We look forward to presenting updated patient data from the Fanconi Anemia program later this month, and to disclosing our AAV-based gene therapy program by the end of the year," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We remain on track for multiple regulatory filings across our pipeline over the next 12 months. As a result, there is the potential for four programs to be in the clinic in 2019, with clinical data for up to two programs."

Dr. Shah continued, "While near-term milestones are certainly important, Rocket's long-term strategy is based on a sustainable and integrated multi-platform approach. We remain focused on our mission of continued innovation to deliver best-in-class curative cell and gene therapies to patients living with devastating diseases."

Anticipated Milestones Over the Next 12 -18 Months

- **Fanconi Anemia (FA):** Updated patient data from the ongoing Phase 1/2 study of RP-L102 conducted with CIEMAT is expected over the next 12 months. The next update will be provided at the American Society of Gene and Cell Therapy's (ASGCT) 21st Annual Meeting in Chicago. The oral presentation is scheduled during the Presidential Symposium on Friday, May 18, 2018, at 2:45 p.m. Central Time.
- **Leukocyte Adhesion Deficiency-I (LAD-I):** An Investigational Medicinal Product Dossier (IMPD) is expected to be filed in the fourth quarter of 2018. Clinical data is expected over the next 12-18 months.
- **Pyruvate Kinase Deficiency (PKD):** An IMPD application is expected to be filed in early 2019.
- **Infantile Malignant Osteopetrosis (IMO):** Preclinical studies continue to advance in support of filing a first-in-human clinical study.
- **AAV:** Disclosure of the disease indication and preclinical data is planned for the second half of 2018.

Upcoming Conferences

- **Bank of America Merrill Lynch Health Care Conference 2018.** Rocket is scheduled to present on Tuesday, May 15, at 9:20 a.m. Pacific Time.
- **UBS Global Healthcare Conference.** Rocket is scheduled to present on Wednesday, May 23, at 9:30 a.m. Eastern Time.
- **Jefferies Global Healthcare Conference.** Rocket is scheduled to present on Thursday, June 7, at 2:00 p.m. Eastern Time.

First Quarter 2018 Financial Results

- **Cash position.** Cash, cash equivalents and short-term investments as of March 31, 2018, were \$182.7 million, which includes a \$52.0 million fully convertible debenture which expires in 2021.
- **R&D expenses.** Research and development expenses were \$5.7 million for the quarter ended March 31, 2018, compared

to \$2.3 million for the quarter ended March 31, 2017.

- **G&A expenses.** General and administrative expenses were \$8.7 million for the quarter ended March 31, 2018, compared to \$0.6 million for the quarter ended March 31, 2017. The increase in G&A expenses was primarily due to one-time merger related expenses of \$5.3 million.
- **Net loss.** Net loss was \$15.3 million or \$(0.42) per share (basic and diluted) for the quarter ended March 31, 2018, compared to \$2.7 million or \$(0.39) per share (basic and diluted) for the quarter ended March 31, 2017.
- **Shares outstanding.** Approximately 39.4 million shares of common stock were outstanding as of March 31, 2018.

Financial Guidance

- **Cash position.** Based on its current operating plan, Rocket expects its cash, cash equivalents and short-term investments as of March 31, 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 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 products 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, 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 March 31,	
	2018	2017
Revenue	\$ -	\$ -
Operating expenses:		
Research and development	5,743	2,285
General and administrative	8,662	585
Total operating expenses	14,405	2,870
Loss from operations	(14,405)	(2,870)
Research and development incentives	186	192
Interest expense	(1,427)	-
Interest income	288	-
Other income	15	-
Net loss	\$ (15,343)	\$ (2,678)
Net loss per share attributable to common shareholders - basic and diluted	\$ (0.42)	\$ (0.39)
Weighted-average common shares outstanding - basic and diluted	36,137,120	6,795,627

Selected Balance Sheet Information

(amounts in thousands)

	March 31, 2018	December 31, 2017
Cash, cash equivalents and short-term investments	182,697	18,142
Total assets	217,165	20,147
Total liabilities	47,019	4,628
Total shareholders' equity	170,146	15,519

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Source: Rocket Pharmaceuticals, Inc.

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