Rocket Pharmaceuticals Reports Full Year 2018 Financial Results and Operational Highlights

March 7, 2019

- Three Rocket-sponsored Gene Therapy INDs Across Two Platforms Cleared in Three Months -

- Clinical Trials to Commence in 2019 for Danon Disease, Fanconi Anemia, Leukocyte Adhesion Deficiency-I and Pyruvate Kinase Deficiency -

- Proof of Concept Data from Two Programs Expected in 2019 -

NEW YORK--(BUSINESS WIRE)--Mar. 7, 2019-- Rocket Pharmaceuticals, Inc. (Nasdaq:RCKT) ("Rocket"), a leading U.S.-based multi-platform gene therapy company, reports financial results for the year ended December 31, 2018, and provides an update on the Company's recent achievements, as well as upcoming milestones.

"2018 was a great year for Rocket," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We successfully established ourselves as a public gene therapy company, funded our pipeline with more than $250 million in capital, achieved clinical proof of concept for our lead lentiviral gene therapy program in FA, expanded our pipeline with a new AAV gene therapy program in Danon disease, and received FDA clearance of three Rocket-sponsored INDs in only three months. These accomplishments are a testament to our ability to execute on our goals and commitment to bring new therapies to patients who desperately need them."

Dr. Shah added, "Looking ahead, Rocket is entering an exciting period in our evolution as we anticipate treating more patients with our gene therapy products across our pipeline. We look forward to at least two clinical readouts this year that, if successful, may support advancing our gene therapies that have the potential to transform the treatment paradigm in diseases that are limited to toxic bone marrow or organ transplant regimens. We believe we are well-positioned and prepared to meet all of these exciting challenges and opportunities."

Full Year 2018 and Recent Highlights

- **Expanded Pipeline with First AAV Gene Therapy Program Targeting a Monogenic Heart Failure Syndrome.**In November 2018, the Company presented preclinical proof of concept data from its first adeno-associated viral vector (AAV)-based gene therapy, RP-A501, targeting Danon disease. RP-A501 is being developed in collaboration with Dr. Eric Adler, Director of Cardiac Transplant and Mechanical Circulatory Support at UC San Diego Health and Professor of Medicine at University of California San Diego School of Medicine. Preclinical studies show RP-A501 improves animal survival and corrects the disease phenotype with dose-dependent improvements in molecular, structural, and functional endpoints with a clean safety and tolerability profile. The Company simultaneously announced an exclusive, worldwide license agreement with REGENXBIO Inc. for Rocket to develop and commercialize gene therapy treatments for Danon disease using REGENXBIO's NAV AAV9 vector, as well as exclusive options for two additional NAV AAV vectors for the treatment of Danon disease. A Phase 1 clinical trial of RP-A501 is planned to commence in the second quarter of 2019.
- **Initial Clinical Proof of Concept Achieved in FA.** Long-term clinical data from the ongoing Phase 1/2 clinical trial of RP-L102 for Fanconi Anemia (FA) utilizing "Process A", the first-generation non-optimized process, were presented at ESGCT in November 2018. Results showed durable engraftment at 30 months post treatment, and stabilization of previously-declining blood counts and progressive increases in corrected versus non-corrected peripheral blood leukocytes. Moving forward, the Company plans to initiate a Phase 1 clinical trial of RP-L102 at Stanford University utilizing "Process B" which incorporates higher cell doses, transduction enhancers, and commercial-grade vector manufacturing and cell processing.
- **Three Gene Therapy INDs Cleared by the FDA.** In a three-month period, the FDA cleared three Rocket-sponsored Investigational New Drug (IND) applications, paving the way for Phase 1 clinical trials to begin in FA, Danon disease along with a Phase 1/2 adaptive clinical study in Leukocyte Adhesion Deficiency-I (LAD-I).
- **Nine Regulatory Designations Across Pipeline – RMAT, Fast Track, ATMP, Orphan Drug and Rare Pediatric Disease.**

    - The Company received notification that the following programs received regulatory designations:
        - RP-L102 for FA received Regenerative Medicine Advanced Therapy (RMAT), Fast Track, and Rare Pediatric Disease designations from the FDA, as well as Advanced Therapy Medicinal Product (ATMP) classification from the European Medicines Agency (EMA).
        - RP-L201 for LAD-I received Fast Track and Rare Pediatric Disease designations from the FDA, as well as ATMP classification from the EMA.
        - RP-L401 for Infantile Malignant Osteopetrosis received Orphan Drug designation from the FDA.
        - RP-A501 for Danon disease received Orphan Drug designation from the FDA.
    - Each designation provides numerous incentives to support the development of Rocket's programs, including prescription drug user fee waivers, increased access to the regulatory authorities, expedited review timelines and tax credits towards the cost of clinical trials.
- **Approximately $150 Million Secured in Two Oversubscribed Public Offerings.** Following the successful completion of its reverse merger with Inotek Pharmaceuticals in January 2018 that capitalized the Company with $97.6 million in cash, Rocket completed two oversubscribed underwritten public offerings of its common stock for gross proceeds of approximately $150 million over the course of the year.

Anticipated Milestones

- **FA (RP-L102)**
    - Initiation of Phase 1 clinical trial of RP-L102 utilizing "Process B" (2Q19)
    - Additional data from RP-L102 utilizing "Process A" (2H19)
    - Initial data from Phase 1 clinical trial of RP-L102 utilizing "Process B" (2H19)
    - Regulatory alignment on final endpoints for registration (2H19)
- **LAD-I (RP-L201)**
    - Initiation of Phase 1/2 clinical trial of RP-L201 in support of registration (2Q19)
    - Initial data from Phase 1 portion of clinical trial of RP-L201 (2H19)
- **PKD (RP-L301)**
    - Initiation of Phase 1 clinical trial of RP-L301 (2H19)
- **Danon (RP-A501)**
    - Initiation of Phase 1 clinical trial of RP-A501 (2Q19)

Upcoming Investor Conferences

- **Cowen and Company 39th Annual Health Care Conference.** Rocket is scheduled to participate in a fireside chat on Tuesday, March 12, 2019, at 10:40 a.m. Eastern Time.
- **Alliance for Regenerative Medicine Cell & Gene Investor Day.** Rocket is scheduled to present on Thursday, March 21, 2019, at 9:55 a.m. Eastern Time.

Fourth Quarter and Full Year 2018 Financial Results

- **Cash position.** Cash, cash equivalents and investments as of December 31, 2018, were $213.1 million.
- **Debt.** Our cash position includes a $52.0 million fully convertible debenture which matures in August of 2021.
- **R&D expenses.** Research and development expenses were $23.7 million and $53.3 million for the three and twelve months ended December 31, 2018, compared to $4.9 million and $14.9 million for the three and twelve months ended December 30, 2017.
- **G&A expenses.** General and administrative expenses were $2.9 million and $17.9 million for the three and twelve months ended December 31, 2018, compared to $1.7 million and $4.9 million for the three and twelve months ended December 31, 2017.
- **Net loss.** Net loss was $27.3 million and $74.5 million or $(0.66) and $(1.89) per share (basic and diluted) for the three and twelve months ended December 31, 2018, compared to $6.7 million and $19.6 million or $(0.98) and $(2.88) per share (basic and diluted) for the three and twelve months ended December 31, 2017.
- **Shares outstanding.** Approximately 45.2 million shares of common stock were outstanding as of December 31, 2018.

Financial Guidance

- Cash position. Based on its current operating plan, Rocket expects its cash, cash equivalents and investments as of December 31, 2018, will be sufficient to run its operations into the second half of 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. Rocket's additional

pipeline programs for bone marrow-derived disorders are for 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 a devastating, pediatric heart failure indication, Danon disease. 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 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), 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 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, 2018. 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 December 31,		Year Ended December 31,	
	2018	2017	2018	2017
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	23,666	4,918	53,270	14,917
General and administrative	2,880	1,737	17,886	4,855
Total operating expenses	26,546	6,655	71,156	19,772
Loss from operations	(26,546)	(6,655)	(71,156)	(19,772)
Research and development incentives	-	-	186	192
Interest expense	(1,573)	-	(6,039)	-
Interest and other income net	500	1	1,690	2
Accretion of discount on investments	300	-	801	-
Net loss	$ (27,319)	$ (6,654)	$ (74,518)	$ (19,578)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.66)	$ (0.98)	$ (1.89)	$ (2.88)
Weighted-average common shares outstanding - basic and diluted	41,690,337	6,795,627	39,377,666	6,795,627

Selected Balance Sheet Information
(amounts in thousands)

	December 31, 2018	December 31, 2017
Cash, cash equivalents and investments	213,132	18,142
Total assets	251,313	20,147
Total liabilities	57,276	4,628
Total shareholders' equity	194,037	15,519

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Source: Rocket Pharmaceuticals, Inc.

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