



Rocket Pharmaceuticals Reports Full Year 2017 Financial Results and Operational Highlights

March 6, 2018

- Enters 2018 as a Public Company with Solid Financial Position Following Successful Completion of Merger with Inotek Pharmaceuticals and Subsequent Follow-on Offering -

- Multiple Milestones Expected in 2018, with Additional Data in Fanconi Anemia, Disclosure of AAV Program, and Several Programs Advancing Toward the Clinic -

NEW YORK--(BUSINESS WIRE)--Mar. 6, 2018-- [Rocket Pharmaceuticals, Inc.](#) (NASDAQ:RCKT) ("Rocket"), a leading U.S.-based multi-platform gene therapy company, today reported financial results and operational highlights for the full year ended December 31, 2017.

"2017 was an important year for Rocket. We advanced our pipeline of gene therapy programs focused on life-threatening and devastating pediatric diseases, and we implemented strategic initiatives that have strengthened our organization," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We believe Rocket's multi-platform development approach uniquely positions us to aggressively pursue a broad range of best-in-class indications, enabling first-mover advantage."

"With the successful closing of our merger with Inotek Pharmaceuticals and recent public offering in January, we enter 2018 with a solid financial position to accomplish our key clinical and operational goals," continued Dr. Shah. "In addition to more clinical data on our lead program in Fanconi Anemia (FA), we plan to announce the disease indication for our adeno-associated viral vector (AAV) gene therapy program in the second half of this year. We also expect to file Investigational Medicinal Product Dossier (IMPD) applications for our Leukocyte Adhesion Deficiency-I (LAD-I) and Pyruvate Kinase Deficiency (PKD) programs, as well as an Investigational New Drug (IND) application for our AAV program over the next 12 months. With multiple programs moving forward in parallel, we believe we have the key elements in place to build a transformative, multi-platform gene therapy company delivering sustained, long-term growth for our shareholders."

Recent Pipeline and Corporate Updates

- **Promising preliminary patient data reported for RP-L102, a Phase 1/2 lentiviral vector (LVV)-based gene therapy for FA.** At the European Society of Gene and Cell Therapy's Annual Meeting in October 2017, Rocket's collaboration partners at the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT) in Spain presented interim results from the ongoing Phase 1/2 trial of RP-L102 in FA. Data presented in FA patients, without the use of conditioning, showed promising *in vivo* engraftment and stabilization of blood counts despite declining counts in the months to years preceding therapy.
- **Studies of FA gene therapy product, RP-L101, utilizing new gene modified cell product manufacturing process underway.** At the American Society of Hematology Annual Meeting in December 2017, Rocket's collaboration partners at the Fred Hutchinson Cancer Research Center presented data from a pilot clinical trial assessing the feasibility of a novel lineage depletion protocol for hematopoietic stem cell (HSC) enrichment and subsequent transduction with the LVV-based gene therapy, RP-L101. Collection, lentiviral transduction and re-infusion of gene modified cells was shown to effectively produce long-term engraftment in mice. Patient treatment with this novel protocol is underway.
- **Three additional LVV-based gene therapy pipeline programs in rare bone marrow disorders advance towards the clinic.** LAD-I and PKD programs continue to move forward in preclinical IND-enabling studies. An additional program targeting Infantile Malignant Osteopetrosis (IMO) is also in preclinical development. Similar to FA, these diseases can lead to serious complications and have very limited long-term safe and effective treatment options.
- **Rocket commenced trading on the NASDAQ Global Market under the symbol "RCKT", following the successful closing of its merger with Inotek Pharmaceuticals Corporation (Inotek) in January.** In conjunction with the completion of the merger, the Company announced its executive management team will be led by Dr. Shah and will consist of: Jonathan Schwartz, M.D., Chief Medical Officer, and Kinnari Patel, Pharm.D., MBA, Chief Operating Officer.
- **Rocket completed an oversubscribed underwritten public offering of 6,325,000 shares of its common stock in**

January 2018. Net proceeds to Rocket from the offering were approximately \$78.8 million after deducting underwriting discounts and commissions.

Anticipated Milestones

- Updated FA patient data (2Q18)
- Preclinical data and disclosure of the AAV-based gene therapy program (2H18)
- Target rolling IMPD filing for the LAD-I program (4Q18)
- Target IND filing for the AAV-based program (next 12 months)
- Target rolling IMPD filing for the PKD program (next 12 months)

Full Year Rocket Pharmaceuticals Ltd. 2017 Financial Results

- The foregoing financial results presented for the year ended December 31, 2017 represent Rocket Pharmaceuticals Ltd. as a standalone private company, as the merger with Inotek closed on January 4, 2018. The financial statements for the first quarter 2018 will reflect the financial results as a merged public company.
- Research and development expenses were \$14.9 million for the year ended December 31, 2017, compared to \$6.0 million for the year ended December 31, 2016. General and administrative expenses were \$4.9 million for the year ended December 31, 2017, compared to \$1.6 million for the year ended December 31, 2016.
- Net loss was \$19.6 million or \$(219.49) per share (basic and diluted) for the year ended December 31, 2017, compared to \$7.6 million or \$(84.43) per share (basic and diluted) for the year ended December 31, 2016.
- Cash as of December 31, 2017 for Rocket Pharmaceuticals Ltd. was \$18.1 million. Following the merger with Inotek and the January 2018 offering of common stock, cash, cash equivalents and short-term investments as of February 28, 2018 totaled approximately \$185 million, which includes a \$52 million fully convertible debenture which expires in 2021.
- The standalone historical financial statements of Inotek for the year ended December 31, 2017 are presented in Rocket's Annual Report on Form 10-K for the year ended December 31, 2017.

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 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.

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Source: Rocket Pharmaceuticals, Inc.

Claudine Prowse, Ph.D.
SVP Corporate Development and IRO
Rocket Pharmaceuticals, Inc.
The Alexandria Center for Life Science

430 East 29 Street, Suite 1040
New York, NY 10016
cp@rocketpharma.com