



## **Rocket Pharmaceuticals and REGENXBIO Announce New License Agreement for the Treatment of Danon Disease Using NAV AAV9 Vector**

November 26, 2018

*- REGENXBIO grants Rocket exclusive, worldwide rights to NAV AAV9 for the development and commercialization of treatments for Danon Disease -  
- Investigational New Drug Application Expected to be Filed in 2019; Clinical Trial to Follow -*

NEW YORK & ROCKVILLE, Md.--(BUSINESS WIRE)--Nov. 26, 2018-- Rocket Pharmaceuticals, Inc. (Nasdaq:RCKT), a leading U.S.-based multi-platform gene therapy company, and REGENXBIO Inc. (Nasdaq:RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV<sup>®</sup> Technology Platform, today announced an exclusive, worldwide license agreement 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.

Under the terms of the agreement, REGENXBIO has granted Rocket an exclusive, worldwide license, with rights to sublicense, to REGENXBIO's NAV AAV9 vector for the development and commercialization of gene therapy treatments for Danon disease. REGENXBIO has also granted Rocket exclusive options to two additional undisclosed NAV AAV vectors for the treatment of Danon disease for up to four years. In return for these rights, REGENXBIO will receive a \$7 million upfront payment, ongoing fees, milestone payments and high-single to low-double digit royalties on net sales of products incorporating the licensed intellectual property.

"Rocket is delighted to partner with REGENXBIO to advance our first gene therapy product derived from the NAV Technology Platform, reinforcing the potential of our disease-focused, multi-platform development approach allowing for the potential first-mover advantage without being limited by vector type," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "Danon disease represents a significant area of unmet medical need as no specific therapies are available for the patients and families suffering from the disease. We believe the well-understood disease biology and severe patient need makes Danon disease an ideal target for gene therapy and we expect to enter the clinic next year."

"This license agreement provides further validation of the potential for the diverse application of our NAV Technology Platform for the treatment of severe diseases with high unmet medical," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are pleased to initiate our partnership with Rocket, an emerging leader in gene therapy for rare diseases, via development of a gene therapy treatment for Danon disease."

### **About Danon Disease**

Danon disease is a rare neuromuscular and cardiovascular disease characterized by profound cardiomyopathy, skeletal myopathies, and mild cognitive impairment. It is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and the European Union. Danon disease is caused by mutations in the gene encoding lysosome-associated membrane protein 2 (LAMP-2), an important mediator of autophagy. The LAMP-2 protein has three distinct variants generated by alternative splicing: LAMP-2A, LAMP 2B and LAMP-2C. Mutations resulting in LAMP-2B dysfunction are associated with severe cardiac disease features. The disease is often fatal in patients in the second or third decade of life due to progressive heart failure unless treated with a cardiac transplantation, which is nonetheless not considered curative. There are no specific therapies available for the treatment of Danon disease.

### **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's first AAV-based gene therapy program targets Danon disease, a rare neuromuscular and cardiovascular disease. For more information about Rocket, please visit [www.rocketpharma.com](http://www.rocketpharma.com).

### **About REGENXBIO Inc.**

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy.

REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

### **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) 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.

### **REGENXBIO Forward-Looking Statements**

This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's future operations and clinical trials. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2017 and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at [www.sec.gov](http://www.sec.gov). All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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