REGENXBIO Announces Exclusive Worldwide License with Ultragenyx for the Treatment of CDKL5 Deficiency Disorder Using NAV Vectors

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ROCKVILLE, Md., Oct. 22, 2018 /PRNewswire/ -- 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® Technology Platform, today announced that Ultragenyx Pharmaceutical Inc. has exercised an option for an exclusive worldwide license to REGENXBIO's NAV Vectors, including NAV AAV9, for the treatment of CDKL5 Deficiency Disorder (CDD).

Under the terms of the license, REGENXBIO will receive an upfront payment, ongoing fees, milestone payments and royalties on net sales of products incorporating NAV Vectors. The option exercised by Ultragenyx is the final remaining disease indication option under the 2015 license agreement between REGENXBIO and Dimension Therapeutics, Inc., a wholly owned subsidiary of Ultragenyx. CDD is a severe and debilitating neurological disorder that shares many features of Rett Syndrome, though the two disorders are now considered distinct from each other.

"We are looking forward to initiating this program in CDD, a disease with increasing awareness and diagnosis," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "AAV9 has been shown to be effective for gene therapy delivery to the central nervous system, and we believe it is well-suited for this neurological indication."

Dr. Kakkis, will present the Keynote Address on Tuesday, October 23 at the 2018 CDKL5 Forum hosted by the Loulou Foundation.

"This license for CDD further validates the NAV Technology Platform for use in development of treatments for disorders of the central nervous system," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are pleased to expand our partnership with Ultragenyx with this new license, and to continue to enable their commitment to develop gene therapy treatments for serious rare and ultra-rare genetic diseases."

About CDD
CDD is an X-linked genetic disorder that results in seizures that typically begin in the first few months of life and severe intellectual and gross motor impairment. The CDKL5 gene provides instructions for making a protein that is essential for normal brain development, with mutations causing a deficiency in the protein level. CDD is caused by a deficiency in an enzyme that may only be needed in small quantities for the brain to function normally. In the past, CDD was commonly diagnosed as an atypical Rett Syndrome until genetic diagnostics changed this view and improved accurate diagnosis.

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.

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, for which there are no approved therapies.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

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 cash flow. 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. 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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