REGENXBIO and Ultragenyx Announce New License Agreement for Use of NAV® Technology Platform for the Treatment of Rare Metabolic Disorder

March 31, 2020

- REGENXBIO grants Ultragenyx exclusive, worldwide rights to NAV AAV8 and AAV9 Vectors for the development and commercialization of gene therapy treatments for a rare metabolic disorder

- Agreement for use of NAV Vectors expands ongoing relationship between Ultragenyx and REGENXBIO

ROCKVILLE, Md. and NOVATO, Calif., March 31, 2020 (GLOBE NEWSWIRE) -- 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, and Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare diseases, today announced a new exclusive, worldwide license agreement, extending the companies' existing gene therapy partnership.

Under the terms of the agreement, REGENXBIO has granted Ultragenyx an exclusive, worldwide license, with rights to sublicense, to REGENXBIO's NAV AAV8 and AAV9 Vectors for the development and commercialization of gene therapy treatments for a rare metabolic disorder. In return for these rights, REGENXBIO will receive an upfront payment of $7 million, ongoing fees, milestone payments, and royalties on net sales of products incorporating the licensed intellectual property.

"We are pleased to expand our long-standing relationship with Ultragenyx through this new license agreement, which will enable Ultragenyx to apply our proprietary NAV Vectors to the development of a new gene therapy for rare diseases," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Today's announcement provides further validation of the breadth and depth of our intellectual property portfolio, and we remain committed to working closely with leading companies to enable the development of important gene therapy programs."

"This latest license agreement reinforces Ultragenyx's commitment to developing gene therapies for multiple rare diseases and adds a new indication to our relationship with REGENXBIO," said Emil D. Kakkis, MD, PhD, Chief Executive Officer and President of Ultragenyx.

Ultragenyx is a longstanding licensee partner of REGENXBIO. In addition to today's agreement, Ultragenyx has in-licensed NAV Vectors for the development of gene therapies to treat Ornithine Transcarbamylase (OTC) Deficiency, Glycogen Storage Disease Type Ia (GSDIa), CDKL5 Deficiency, Hemophilia A, and Wilson Disease.

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

Ultragenyx is a biopharmaceutical company committed to bringing 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 typically no approved therapies treating the underlying disease.

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.

For more information on Ultragenyx, please visit the Company's website at www.ultragenyx.com.

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, 2019, 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.

Ultragenyx Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations regarding plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, collaboration with third parties, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of regulatory filings and approvals (including whether such approvals can be obtained), and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our products and drug candidates. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Annual Report filed on Form 10-K with the Securities and Exchange Commission on February 14, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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