REGENXBIO Expands Pipeline Using NAV Vectors to Deliver Therapeutic Antibodies for the Treatment of Hereditary Angioedema and Neurodegenerative Diseases

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- REGENXBIO adds new AAV-mediated antibody delivery gene therapy program for the treatment of hereditary angioedema to its pipeline

- REGENXBIO and Neurimmune announce partnership to develop novel AAV-mediated human antibody gene therapies for chronic neurodegenerative diseases, including tauopathies

- New programs establish REGENXBIO's leading capabilities using NAV Vectors to potentially deliver therapeutic antibodies to the retina, brain and liver to treat diseases with significant unmet need

ROCKVILLE, Md., July 24, 2019 /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 an expansion of its pipeline to include novel treatments for hereditary angioedema (HAE) and neurodegenerative diseases, including tauopathies, using NAV Vectors to deliver therapeutic antibodies.

REGENXBIO's new gene therapy product candidate for the treatment of HAE utilizes NAV Vectors that are designed to deliver a gene encoding a therapeutic antibody that targets and binds to plasma kallikrein, a key protein left unregulated in patients with HAE.

In addition, REGENXBIO and Neurimmune AG announced earlier today an exclusive license, development and commercialization agreement to discover and develop novel AAV gene therapies using NAV Vectors to deliver human antibodies against targets implicated in chronic neurodegenerative diseases, including tauopathies.

"Using NAV Vectors to deliver therapeutic antibodies has enormous potential for patients who lack treatments or who are currently underserved by existing therapies, and provides a significant opportunity to expand our pipeline through the application of our AAV-mediated antibody delivery capabilities and expertise to a number of validated and new targets in multiple therapeutic areas and tissues," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are thrilled to announce our research program for the treatment of HAE, a chronic, life-threatening disease characterized by recurring severe swelling, most commonly in the face, airway, intestines and limbs, for which a one-time AAV-mediated antibody delivery approach may provide significant benefit for patients. In addition, we are excited to partner with Neurimmune and we believe the combination of Neurimmune's human-derived antibodies with REGENXBIO's AAV expertise creates a unique opportunity to develop significant therapies for chronic neurodegenerative diseases."

"REGENXBIO is at the forefront of research and development of AAV-mediated antibody gene therapies," said Olivier Danos, Ph.D., Chief Scientific Officer of REGENXBIO. "Our pipeline expansion through our research for the treatment of HAE and our previously announced partnership with Neurimmune builds on promising results with our clinical-stage RGX-314 program, where we are using our NAV Vectors to deliver a therapeutic antibody for the treatment of wet AMD."

NAV Vectors for AAV-Mediated Antibody Delivery

REGENXBIO has established a new treatment modality to treat serious and chronic diseases based on foundational technology and intellectual property, as well as scientific, clinical and manufacturing expertise for the development of one-time treatments in multiple disease areas based on AAV-mediated antibody delivery using NAV Vectors.

These AAV-mediated antibody delivery treatments share similar gene therapy delivery technology and manufacturing processes, and may potentially share similar pharmacology profiles, including, safety and tolerability, and medicinal properties. Product candidates in this modality will be designed to modify some of the patients' own cells and may result in the sustained production of therapeutic antibodies *in vivo*.

REGENXBIO's lead product candidate, RGX-314, consists of the NAV AAV8 vector encoding a gene for an antibody fragment that binds vascular endothelial growth factor (VEGF). RGX-314 is being developed as a novel, one-time subretinal treatment for wet age-related macular degeneration (wet AMD) and diabetic retinopathy.

Existing standard of care for many diseases involves frequent administration of therapeutic antibodies. A single administration AAV gene therapy approach using NAV Vectors may provide improved treatment options for patients with these diseases by reducing their treatment burden or enabling treatments in tissues where it is difficult to deliver sufficient amounts of therapeutic antibodies via traditional delivery methods, such as in the central nervous system.

REGENXBIO Program for the Treatment of Hereditary Angioedema

HAE is a chronic and severe disease that results from C1-inhibitor (C1-INH) deficiency. HAE is characterized by recurring severe swelling (angioedema), most commonly in the face, airway, intestines and limbs. Antibodies to plasma kallikrein, a key protein left unregulated in patients with HAE, have been shown to reduce the swelling and pain associated with HAE. These antibodies, however, require frequent administration to reduce the occurrence of angioedema events.

REGENXBIO's HAE program is focused on developing a novel, one-time treatment utilizing a NAV Vector to deliver a gene encoding for a therapeutic antibody that targets and binds to plasma kallikrein. Following a single intravenous administration, REGENXBIO's HAE product candidate is designed to allow liver cells to produce therapeutic antibodies that are secreted into the blood. In preclinical animal models, REGENXBIO has used NAV Vectors to express therapeutic antibodies that target and bind to plasma kallikrein. REGENXBIO expects to provide an update in early 2020 on the HAE preclinical studies, as well as plans for entering clinical trials.

REGENXBIO and Neurimmune Collaboration

As previously announced, REGENXBIO and Neurimmune will jointly develop and commercialize novel therapies using AAV vectors to deliver human antibodies with an initial focus on diseases associated with the accumulation and deposition of the microtubule-associated protein tau (tauopathies). The companies have initiated their first exclusive collaboration program for the treatment of tauopathies and will provide updates on program progress as the collaboration advances.

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.

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, research and development activities, preclinical studies 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, 2018, 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.

CONTACTS:

Investors:
Heather Savelle, 212-600-1902
heather@argotpartners.com

Media:
David Rosen, 212-600-1902
david.rosen@argotpartners.com

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