REGENXBIO and Neurimmune Announce Collaboration to Develop Vectorized Antibodies for the Treatment of Neurodegenerative Diseases

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- REGENXBIO and Neurimmune to develop novel vectorized human antibodies for chronic neurodegenerative diseases, including tauopathies, by combining antibodies identified using Neurimmune's Reverse Translational Medicine™ platform with REGENXBIO's NAV® Technology Platform

ROCKVILLE, Md. and ZURICH, 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 and Neurimmune AG, a leading clinical-stage Swiss biotech company translating human immune memory into antibody therapeutics, today announced 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.

"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. "We are thrilled to initiate our partnership with Neurimmune, which builds on promising results to date with our RGX-314 clinical-stage program, where we are using our NAV Vectors to deliver a therapeutic antibody for the treatment of wet AMD."

"Neurimmune's Reverse Translational Medicine technology decodes the genetic information of immune cells obtained from healthy aged people to identify next generation antibody therapeutics," said Jan Grimm, Ph.D., Chief Scientific Officer of Neurimmune. "We are excited to partner with REGENXBIO and combine our human antibody technology with REGENXBIO's AAV expertise to develop vectorized human antibodies as a novel treatment modality. We believe the potential to deliver sustained levels of therapeutic human antibodies to the brain with AAV vectors may have a significant impact on chronic neurodegenerative conditions."

Under the exclusive license, development and commercialization agreement, REGENXBIO and Neurimmune will jointly develop and commercialize novel therapies using AAV vectors to deliver human antibodies. Initially, the companies will focus on diseases associated with the accumulation and deposition of the microtubule-associated protein tau (tauopathies). REGENXBIO and Neurimmune will be jointly responsible for the design and development of vectorized antibody therapies and will share associated development costs equally. Following an initial research phase, on a target-by-target basis, each party will have the option to continue as a co-development and co-commercialization partner in the collaboration or to elect to receive a phase-based worldwide royalty in lieu of continued development investment. 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.

Tau is an abundant protein with a primary function to stabilize microtubules, a structural component of healthy neurons. Abnormal forms of tau can misfold and accumulate in neurofibrillary tangles that are hallmarks of several neurodegenerative diseases, including Alzheimer's disease, corticobasal degeneration, frontotemporal dementia, Pick's disease and progressive supranuclear palsy, referred to collectively as tauopathies. Delivery of human antibodies using AAV vectors has the potential to provide sustained brain exposure of antibodies for the clearance of abnormal tau via a one-time CNS administration.

REGENXBIO NAV Vectors for AAV-Mediated Antibody Delivery

REGENXBIO has established foundational technology, intellectual property, 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 to treat serious and chronic diseases.

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 making treatments possible in tissues where it is difficult to deliver sufficient amounts of therapeutic antibodies via traditional delivery methods, such as the central nervous system.

Neurimmune's Reverse Translational Medicine Platform

Reverse Translational Medicine (RTM ™) is Neurimmune's proprietary technology platform. It is based on the scientific understanding and high-throughput analyses of human immune responses to disease-related proteins in selected populations including elderly with the capability to stay healthy during the aging process. The technology translates the genetic information obtained from human white blood cells into selective high affinity antibody candidates. Neurimmune's human monoclonal antibody therapeutics combine the full range of advantages provided by human affinity maturation and tolerance selection. These processes were optimized through human evolution over millions of years for unsurpassed efficacy and safety.

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 (NAV Vectors). 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 Neurimmune AG

Neurimmune is a clinical-stage Swiss biotech company translating human immune memory into therapeutics. Neurimmune's pipeline comprises high-potential drug candidates at both clinical and advanced preclinical development stages. Rights in antibodies BIIB054 for Parkinson's disease and BIIB076 for Alzheimer's disease were acquired by Biogen. Neurimmune has partnered with Biogen, TVM and Eli Lilly's Chorus unit and Ono Pharmaceutical to discover and develop human monoclonal antibodies for neurodegenerative diseases. Neurimmune's pipeline includes human antibody programs for neurodegenerative diseases, cardiomyopathy, type-2 diabetes as well as a small molecule program for cognitive dysfunction.

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.

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