REGENXBIO Announces Exclusive Worldwide Option and License Agreement with Clearside Biomedical for Evaluation of In-Office Delivery Platform for RGX-314

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- REGENXBIO expands RGX-314 gene therapy program to evaluate Clearside's proprietary, in-office SCS Microinjector™ platform targeting suprachoroidal space delivery

- Recent publication in The Journal of Clinical Investigation highlights the potential of suprachoroidal space delivery for RGX-314 in preclinical studies

- In-office delivery of RGX-314 could allow for treatment of expanded population of patients with wet AMD and diabetic retinopathy with one-time gene therapy

ROCKVILLE, Md. and ALPHARETTA, Ga., Sept. 4, 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 Clearside Biomedical, Inc. (Nasdaq: CLSD), a biopharmaceutical company dedicated to developing and delivering treatments that restore and preserve vision for people with serious back of the eye diseases, today announced an option and license agreement for exclusive worldwide rights to Clearside's proprietary, in-office SCS Microinjector ™for the delivery of RGX-314 to the suprachoroidal space to treat wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other conditions for which anti-vascular endothelial growth factor (anti-VEGF) treatment is currently the standard of care. REGENXBIO plans to evaluate RGX-314 using Clearside's SCS Microinjector for in-office, non-surgical delivery into the suprachoroidal space, while continuing to advance its RGX-314 subretinal delivery program currently in development for wet AMD and DR.

Delivery of NAV AAV8-based gene therapy through the suprachoroidal space can potentially provide a targeted, in-office, non-surgical approach to obtaining widespread transgene expression in the retina without exposing the vitreous and the anterior segment of the eye to the injected drug. Clearside's patented SCS Microinjector is specifically designed to allow for consistent injection into the suprachoroidal space and has been tested in over 1,000 injections in clinical trials to date.

"We are pleased to partner with Clearside in evaluating the use of the SCS Microinjector to deliver RGX-314 to the suprachoroidal space in the eye. We believe this approach can potentially allow for the treatment of an expanded population of patients with wet AMD and DR by providing access to gene therapy treatment in all settings of patient care," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "Delivery into the suprachoroidal space, which in clinical trials has demonstrated minimal associated procedural risks, may allow physicians to treat patients with diseases like DR earlier in the disease course with RGX-314. We will continue to advance the RGX-314 subretinal delivery clinical program, with a Phase IIb clinical trial in wet AMD and a Phase II clinical trial in DR expected to begin by the end of 2019, and we look forward to moving both delivery approaches through development as part of our deep commitment to patients."

"REGENXBIO is the ideal partner for us given its leadership, expertise and pioneering work in the development and manufacturing of AAV vectors for the delivery of anti-VEGF therapeutic antibodies in the ophthalmology space," said George Lasezkay, Pharm.D., J.D., Chief Executive Officer of Clearside. "We are excited by the recent positive interim data reported by REGENXBIO from the current RGX-314 Phase I/IIa trial in wet AMD. This is an exciting time for us to collaborate with REGENXBIO to evaluate the potential application of our proprietary in-office SCS Microinjector for AAV gene therapy."

A recent research collaboration with Johns Hopkins School of Medicine published in The Journal of Clinical Investigation[1] highlights the potential of RGX-314 gene therapy delivered into the suprachoroidal space. In these preclinical studies, delivery of RGX-314 into the suprachoroidal space resulted in similar expression of anti-VEGF Fab and suppression of VEGF-induced vascular leakage as subretinal delivery.

"Intravitreal delivery of AAV gene therapy can be limited by ocular inflammation, immune responses in the vitreous and restrictive physical transduction barriers due to the inner limiting membrane on the retina. These hurdles can potentially be overcome with alternative in-office delivery approaches such as delivery to the suprachoroidal space," said Olivier Danos, Ph.D., Chief Scientific Officer of REGENXBIO. "Preclinical suprachoroidal delivery studies conducted with the Johns Hopkins University School of Medicine demonstrated encouraging initial results in multiple animal species, and we look forward to further evaluation of this route of administration for RGX-314."

"Following on the positive long-term clinical results from RGX-314 delivered subretinally, it is exciting to see that preliminary preclinical studies with suprachoroidal delivery of RGX-314 achieved widespread transgene expression in the retina, similar to what was observed in RGX-314 preclinical studies using subretinal delivery. Suprachoroidal delivery of gene therapy has the opportunity for broad transduction of the retina and has the potential to be the preferred choice for in-office treatment of wet AMD, DR and other chronic retinal diseases," said Robert Avery, M.D., study investigator from California Retina Consultants in Santa Barbara, CA. "I am thrilled to see REGENXBIO evaluating an in-office procedure in addition to the on-going RGX-314 subretinal delivery program, which has the potential to provide multiple treatment options to patients with significant unmet needs."

Under the terms of the agreement, Clearside has granted REGENXBIO an option to receive an exclusive, worldwide commercial license, with rights to sublicense, to Clearside's SCS Microinjector for the delivery of AAV gene therapies for the treatment of wet AMD, DR, and other conditions for which chronic anti-VEGF treatment is currently the standard of care. In return for these rights, Clearside will receive a fee upon REGENXBIO's exercise of its option, as well as up to $34 million in total development milestones across multiple indications, up to $102 million in sales milestones and mid-single digit royalties on net sales of products using the SCS Microinjector. REGENXBIO will be responsible for all development and commercialization activities for gene therapy product candidates. Clearside will be responsible for supplying the SCS Microinjector and supporting REGENXBIO's preclinical studies, clinical studies and commercial use.

About RGX-314

RGX-314 is being developed as a potential one-time treatment for wet AMD, DR and other additional chronic retinal conditions treated with anti-VEGF. RGX-314 consists of the NAV AAV8 vector encoding an antibody fragment which inhibits VEGF, modifying the pathway for formation of new leaky blood vessels which lead to retinal fluid accumulation and vision loss. In REGENXBIO's on-going clinical trial in subjects with wet AMD, dose-dependent increases in protein expression levels across five cohorts have been seen, and 50% of Cohort 3 subjects continued to be free of anti-VEGF injections at 18 months following a single subretinal administration of RGX-314.

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. For more information, visit http://www.regenxbio.com.

About Clearside Biomedical, Inc.

Clearside Biomedical, Inc. is a biopharmaceutical company dedicated to developing and delivering treatments that restore and preserve vision for people with serious back of the eye diseases. Clearside's proprietary SCS Microinjector ™targeting the suprachoroidal space (SCS) offers unique access to the macula, retina and choroid where sight-threatening disease often occurs. The Company's SCS injection platform is an inherently flexible, in-office, non-surgical procedure, intended to provide targeted delivery to the site of the disease and to work with both established and new formulations of medications, as well as future therapeutic innovations such as gene therapy. For more information, please visit www.clearsidebio.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, preclinical studies, clinical trials 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, 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.

Clearside Forward-Looking Statements

Any statements contained in this press release that do not describe historical facts may constitute forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995. These statements may be identified by words such as "believe", "expect", "may", "plan", "potential", "will", and similar expressions, and are based on Clearside's current beliefs and expectations. These forward-looking statements include statements regarding the potential application of the SCS Microinjector for AAV gene therapy and the potential benefits of the SCS injection platform. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements. Risks and uncertainties that may cause actual results to differ materially include uncertainties inherent in the conduct of clinical trials, Clearside's reliance on third parties over which it may not always have full control, and other risks and uncertainties that are described in Clearside's Annual Report on Form 10-K for the year ended December 31, 2018, filed with the U.S. Securities and Exchange Commission ("SEC") on March 15, 2019, Clearside's Quarterly Report on Form 10-Q for the quarter ended June 30, 2019, filed with the SEC on August 8, 2019, and Clearside's other Periodic Reports filed with the SEC. Any forward-looking statements speak only as of the date of this press release and are based on information available to Clearside as of the date of this release, and Clearside assumes no obligation to, and does not intend to, update any forward-looking statements, whether as a result of new information, future events or otherwise.

1. **Ding, K., Shen, J., Hafiz, Z., Hackett, S. F., Silva, R. L. E., Khan, M., … Campochiaro, P. A. (2019). AAV8-vectored suprachoroidal gene transfer produces widespread ocular transgene expression. *Journal of Clinical Investigation*. doi: 10.1172/jci129085**

Contacts:

For REGENXBIO

Tricia Truehart
Investor Relations and Corporate Communications
347-926-7709
ttruehart@regenxbio.com

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

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

For CLEARSIDE

Jenny Kobin
Remy Bernarda
ir@clearsidebio.com
(678) 430-8206

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