# REGENXBIO Reports Second Quarter 2018 Financial and Operating Results and Interim Data from Ongoing Clinical Trials for Wet Age-Related Macular Degeneration and Homozygous Familial Hypercholesterolemia

August 8, 2018 11:30 AM EDT

**- Announces positive interim update from RGX-314 Phase I clinical trial for wet AMD**

**- Provides preliminary interim update from RGX-501 Phase I/II clinical trial for HoFH**

**- Announces appointment of Dr. Ram Palanki as SVP of Commercial Strategy and Operations**

**- $306 million in cash, cash equivalents and marketable securities as of June 30, 2018**

**- Conference call and webcast Wednesday, August 8th at 8:00 a.m. ET**

ROCKVILLE, Md., Aug. 8, 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 financial results for the quarter ended June 30, 2018 and recent operational highlights, including updates to interim data from its ongoing clinical trials.

"We are very encouraged by the positive interim data for RGX-314 and the potential of NAV gene therapy as a one-time treatment for wet AMD, particularly as this is a non-rare patient population with a significant treatment burden," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "REGENXBIO looks forward to applying what we are learning from this trial to expand the RGX-314 clinical program into a Phase II trial and bring this novel therapy to patients as quickly as possible."

**Interim Phase I Trial Update for RGX-314 for the Treatment of Wet Age-Related Macular Degeneration**

- *Well-tolerated at all doses*
- *Dose-dependent protein expression levels*
- *Dose-dependent reduction in anti-VEGF injections, along with maintenance of retinal thickness and vision*
- *50% of subjects treated in Cohort 3 are free of anti-VEGF injections at six months*
- *Company plans to proceed to Phase II clinical trial as soon as possible*
- *Current clinical trial expanded to include new Cohort 4 dose and the first subject has been dosed*
- *Additional data expected to be presented at AAO in October 2018*

REGENXBIO announced today an update to its Phase I trial of RGX-314 for the treatment of wet age-related macular degeneration (wet AMD), including updated results on the three dose cohorts since its previously reported interim results.

REGENXBIO also announced that these data and additional study results for all completed cohorts are expected to be presented at the American Association of Ophthalmology (AAO) annual meeting being held in October 2018 in Chicago, Illinois.

In the Phase I trial, 18 subjects with wet AMD received a single administration of RGX-314 across three dose cohorts (six participants in each cohort). To qualify for inclusion in the trial, participants were required to have a history of frequent anti-VEGF treatment (including at least four anti-VEGF injections in the eight months preceding trial enrollment) and a documented history of response to anti-VEGF therapy. The trial design included doses of 3 x 10^9 (Cohort 1), 1 x 10^10 (Cohort 2) and 6 x 10^10 (Cohort 3) genome copies (GC)/eye. Subjects have been assessed every month to the six-month primary endpoint, with long-term follow-up continuing for 24 months.

The interim data update announced today includes safety and efficacy assessments as of July 27, 2018, and subjects have been followed for an average of 11 months for Cohort 1, nine months for Cohort 2 and six months for Cohort 3. Dose-dependent protein expression levels, dose-dependent reduction in anti-vascular endothelial growth factor (VEGF) injections and maintenance of central retinal thickness (CRT) by spectral domain optical coherence tomography (SD-OCT) have been reported across all cohorts. Additionally, through month six, Best Corrected Visual Acuity (BCVA) assessments for subjects in Cohort 3 have a mean improvement in visual acuity. Three subjects from Cohort 3 have been free of anti-VEGF injections since the administration of RGX-314.

The interim data for each cohort are summarized below based on data through July 27, 2018.

***Safety***

RGX-314 was well-tolerated by all subjects with no reported drug-related adverse events (AEs) or drug-related serious adverse events (SAEs). The most common AEs in all dose cohorts were assessed as mild (Grade 1, 83 percent), and there have been no observed immune responses, drug-related ocular inflammation or any post-surgical inflammation beyond what is expected following routine vitrectomy. Five SAEs that were not drug-related were reported among three subjects.[1]

***Intraocular Protein Levels***

RGX-314 protein expression has been detected in all subjects treated to date. Dose-dependent increases in RGX-314 protein expression levels, as measured from aqueous samples by enzyme-linked immunosorbent assay (ELISA) (Protein Levels) at approximately one month after administration of RGX-314, have been observed (see Table 1).

**Table 1: RGX-314 Protein Levels (ng/ml) of All Doses at One Month (N=18)**

| Dose | Cohort 1 | Cohort 2 | Cohort 3 |
|---|---|---|---|
| N | 6 | 6 | 6 |
| Mean Protein Level (ng/ml) | 2.4 | 12.8 | 160.2 |
| Median Protein Level (ng/ml) | 1.2 | 9.3 | 93.1 |

***Anti-VEGF Injection Rate***

Subjects in the study had received on average more than 35 anti-VEGF injections since diagnosis. The number of anti-VEGF injections required following the administration of RGX-314 through six months was lowest in Cohort 3 (see Table 2). We compared the number of anti-VEGF injections each subject had received during the six months prior to their most recent anti-VEGF injection preceding their enrollment in the study to the number of anti-VEGF injections received in the six months following RGX-314 administration to understand how the frequency of anti-VEGF injections changed among subjects within each cohort. In Cohort 3, the mean number of anti-VEGF injections received following RGX-314 administration was reduced by 53 percent when compared to this prior history (see Table 2). Additionally, 50 percent of subjects treated in Cohort 3 have been free of anti-VEGF injections since the administration of RGX-314.

**Table 2: Summary and Comparison of Mean Anti-VEGF Injections (N=18)**

| | | Before RGX-314 Administration* | After RGX-314 dministration** | |
|---|---|---|---|---|
| | | | | |
| Dose | N | Mean (median, SD) | Mean (median, SD) | Percent Change in Number of Injections (%) |
| Cohort 1 | 6*** | 3.3 (3.0, 0.8) | 4.7 (4.5, 1.2) | +40 |
| Cohort 2 | 6 | 4.2 (4.0, 0.8) | 3.8 (5.0, 2.6) | -8 |
| Cohort 3 | 6 | 2.8 (2.5, 1.0) | 1.3 (1.0, 1.6) | -53 |

** Number of anti-VEGF injections reported between a day before the first anti-VEGF injection preceding Day 1 visit and six months prior.*
*** Number of anti-VEGF injections reported between RGX-314 administration date and six-month visit.*
**** One subject in Cohort 1 discontinued from the study at four months with four injections and was imputed as requiring six injections through six months.*

***Retinal Thickness***

Maintenance of CRT, as measured by SD-OCT, from baseline to six months has been observed in the three dosing cohorts. In Cohort 3, the mean CRT decreased by -14 µm from baseline to six months (see Table 3), with this cohort also receiving fewer anti-VEGF injections on average than Cohorts 1 and 2.

**Table 3: Change from Baseline in Mean CRT at Six Months (N=17)**

| Dose | Cohort 1 | Cohort 2 | Cohort 3 |
|---|---|---|---|
| N | 5* | 6 | 6 |
| Mean Change from Baseline in CRT (µm) | -14 | +26 | -14 |
| Median Change from Baseline in CRT (µm) | +22 | +23 | -16 |
| Range (low, high) | (-181, +92) | (-7, +62) | (-27, +7) |

** One subject in Cohort 1 discontinued from the study at four months.*

***Best Corrected Visual Acuity***

BCVA (as measured by Early Treatment Diabetic Retinopathy Study (ETDRS) letters) from baseline to six months was maintained across all cohorts. Subjects in Cohort 3 experienced a mean gain of +8 ETDRS letters (see Table 4), with this cohort also receiving fewer anti-VEGF injections on average than Cohorts 1 and 2.

**Table 4: Change from Baseline in Mean BCVA (ETDRS Letters) at Six Months (N=17)**

| Dose | Cohort 1 | Cohort 2 | Cohort 3 |
|---|---|---|---|
| N | 5* | 6 | 6 |
| Mean Change from Baseline in BCVA (ETDRS Letters) | -2 | +7 | +8 |

| | | | |
|---|---|---|---|
| Median Change from Baseline in BCVA (ETDRS Letters) | -3 | +9 | +4 |
| Range (low, high) | (-8, +10) | (-4, +15) | (0, +21) |

** One subject in Cohort 1 discontinued from the study at four months.*

***Summary of Cohort 3***

Positive signals from clinical measures in Cohort 3 at six months indicate that this may be a clinically meaningful dose, which REGENXBIO expects to use in a Phase II trial for wet AMD. Protein Levels at one month after administration of RGX-314 have been highest in Cohort 3, with a mean level over 100 ng/ml. Through six months, the majority of subjects treated in Cohort 3 have required minimal or no anti-VEGF injections, with maintenance of CRT and BCVA assessments showing maintenance or improvements in visual acuity.

Through six months, 50 percent of subjects treated from Cohort 3 were free of anti-VEGF intravitreal injections and have evidence of clinically meaningful measures in mean CRT (-21 µm) and mean BCVA (+8 ETDRS letters) at six months versus baseline and generally higher Protein Levels, as measured at one month.

"This is the first reported wet AMD gene therapy study to detect intraocular protein being made in the eyes of all subjects. I am encouraged by the dose-dependent increases in protein and biological effect seen in the study that correlated with signals of efficacy," said Dr. Jeffrey Heier, Co-President and Director of Retina Research at Ophthalmic Consultants of Boston and primary investigator for the trial.

REGENXBIO believes that the current results support further study of the Cohort 3 dose based on the potential to get subjects safely into a range of sustained visual acuity while significantly reducing frequent anti-VEGF injections. REGENXBIO plans to engage with regulatory agencies, including the U.S. Food and Drug Administration (FDA), before the end of 2018 to evaluate a Phase II clinical trial design with the Cohort 3 dose data in order to bring this novel therapy to patients as quickly as possible.

"This study enrolled the hardest to treat wet AMD patients and the promising signals of safety and efficacy released today indicate the potential of a gene therapy to maintain the outcomes of frequent anti-VEGF injections with a one-time intervention," said Dr. Allen Ho, Retina Surgeon and Director of Retina Research at Wills Eye Hospital and study investigator.

***Phase I Trial Dose Expansion***

Based on RGX-314 being well-tolerated and the dose-dependent effects observed on protein expression, reductions in anti-VEGF injections and improvements in visual acuity, REGENXBIO plans to evaluate the potential benefits of a higher dose of RGX-314.

REGENXBIO announced today that, based on an amendment filed with the FDA, it is cleared to proceed under the current Investigational New Drug (IND) application with the initiation of dosing of an additional cohort (six participants) at a dose of 1.6 x 10^11 GC/eye (Cohort 4).

Additionally, an independent Data Safety and Monitoring Board (DSMB) granted clearance to proceed to dosing a fourth cohort based on their assessment of the safety and tolerability data of the first three cohorts. Based on this clearance, the first subject in Cohort 4 was dosed this week.

***Phase II Study and Regulatory Status***

REGENXBIO plans to initiate a Phase II trial for RGX-314 in 2019. Final determination of the study design is under way and REGENXBIO expects to engage with regulatory agencies before the end of 2018. REGENXBIO plans to provide further information regarding the expected trial design and overall plans for the RGX-314 clinical program in early 2019.

"We are encouraged by the positive signals we are seeing in Cohort 3 in these hard-to-treat wet AMD subjects with long-standing disease. We are excited to have initiated dosing at a higher dose in Cohort 4 to further evaluate the dose response and look forward to starting a Phase II trial next year," said Dr. Stephen Yoo, Senior Vice President and Chief Medical Officer of REGENXBIO.

***Manufacturing***

All investigational drug product required to complete dosing of the Phase I clinical trial has been produced and released, inclusive of investigational drug product to support dosing of subjects in Cohort 4. Manufacturing of Phase II product in support of the continuing clinical development program is on track, with bulk drug successfully produced. REGENXBIO expects that the current manufacturing process is capable of meeting future clinical program demand and anticipated potential commercial demand.

**Interim Phase I/II Trial Update for RGX-501 for the Treatment of Homozygous Familial Hypercholesterolemia**

- *Transaminase elevations observed in Cohort II*
- *Administration of steroid appears to mitigate transaminase elevations and related effects*
- *Clinical trial protocol expected to be amended to enroll additional subjects using steroid prophylaxis*
- *Additional data expected to be presented in oral presentation in the fourth quarter of 2018*

REGENXBIO announced today an update to the Phase I/II trial of RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH) being conducted at the University of Pennsylvania and funded by REGENXBIO, including updated safety and efficacy measures on the two dose cohorts.

REGENXBIO also announced that it expects further results for both cohorts will be presented by Dr. Marina Cuchel, Research Associate Professor of

Medicine in the Institute for Translational Medicine and Therapeutics at the University of Pennsylvania Perelman School of Medicine, and primary investigator for the RGX-501 Phase I/II clinical trial, at a scientific meeting in the fourth quarter of 2018.

In the Phase I/II trial, a total of six subjects with HoFH received a single administration of RGX-501 across two dose cohorts (three participants in each) at doses of 2.5 x 10^12 (Cohort I) and 7.5 x 10^12 (Cohort II) GC/kg body weight, respectively.

The interim data update announced today includes safety and efficacy assessments as of July 27, 2018 for the two dose cohorts. Subjects have been followed for an average of 63 weeks for Cohort I and 23 weeks for Cohort II.

***Safety***

As of July 27, 2018, there have been four SAEs reported during the 52-week active study period, two of which were reported as drug-related. As previously reported in January 2018, one subject in Cohort I experienced a mild transitory activation of the innate immune system accompanied by hypotension and elevation in transaminases approximately 22 hours post dosing that resolved within a day. A second subject in Cohort II experienced transient, asymptomatic elevation in transaminases with a peak ALT of 1469 IU/L. This subject was briefly hospitalized to manage the transaminases and to perform additional assessments.

All three subjects in Cohort II experienced an elevation in transaminases 4-6 weeks post-dosing. The peak ALTs were 165, 388 and 1469 IU/L in the three subjects. All three subjects were asymptomatic and responded rapidly to the initiation of prednisone followed by a slow taper, with normalization of the transaminases.

Transaminase elevations have been observed in other AAV-mediated gene transfer trials (e.g., hemophilia) and were attributed to a T-cell response. In these trials, transgene expression was preserved by the administration of steroids after mild to moderate increases in transaminases and contributed to sustained transgene expression. Similarly, a T-cell response may be the cause of the transaminase elevations observed in the three subjects in Cohort II of the RGX-501 trial.

***LDL-C Levels***

At 12 weeks, the three subjects in Cohort I did not show a clinically meaningful change in LDL cholesterol (LDL-C) levels.

REGENXBIO believes that the ability to assess LDL-C levels at 12 weeks in the three subjects in Cohort II may be confounded by the potential effects on the liver and the resulting steroid therapy.

***Plans for Phase I/II Trial Protocol Amendment and Dose Expansion***

After review of the interim data from both cohorts with an independent DSMB, a protocol amendment is expected to be submitted to regulatory agencies that includes changes such as introduction of steroid prophylaxis and the primary evaluation of LDL-C at a later time point, such as 26 weeks, to ensure that steroid therapy has been discontinued. Based on the protocol amendment, REGENXBIO expects Cohort II to be expanded with additional subjects and include steroid prophylaxis. A third dose cohort is planned, also including steroid prophylaxis, and will be initiated after the additional subjects in Cohort II reach approximately 12 weeks and an independent DSMB review is completed. REGENXBIO expects to provide an update on these plans in the fourth quarter of 2018.

***Manufacturing***

REGENXBIO has completed manufacturing of new investigational product to support dose expansion and received feedback from the FDA that this material is acceptable for use. Additionally, REGENXBIO has initiated the transfer of an optimized suspension process to our manufacturing partner, FUJIFILM Diosynth Biotechnologies, to prepare for advancing the RGX-501 clinical program. This optimized suspension process is also expected to be used to support other current lead product candidates and future pipeline candidates through clinical development and commercialization.

**Other Recent Operational Highlights**

REGENXBIO announced today that it has further strengthened its management team with the appointment of Ram Palanki, Pharm.D., as Senior Vice President of Commercial Strategy and Operations. Dr. Palanki brings more than 15 years of experience in ophthalmology across pharmaceutical strategy and development, marketing, market access, sales, pricing, reimbursement, supply chain, distribution, business development, medical affairs and portfolio planning leadership. Prior to joining REGENXBIO, he was Senior Vice President of Commercial for all of Americas at Santen Inc. Preceding Santen, he was Global Head of Marketing and Sales at Thrombogenics, Inc. where he led the commercial launch for Jetrea, a novel proteolytic enzyme therapy for symptomatic vitreomacular adhesion. Previously, Dr. Palanki served as Director of Global Marketing and Sales at NeoVista, Inc. and earlier in his career, held several positions at Genentech as part of the team that developed and launched Lucentis. Prior to Genentech, he worked at Eyetech Pharmaceuticals as part of the initial team submitting the New Drug Application for Macugen and at Novartis Pharmaceuticals in the New Product Commercialization group. Dr. Palanki earned his Pharm.D. at Albany College of Pharmacy in New York and completed his postdoctoral fellowship at Rutgers University in New Jersey.

Subject recruitment has started in the Phase I clinical trial evaluating RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I). Dosing of the first subject in the clinical trial is now expected in the fourth quarter of 2018.

Subject recruitment has started in the Phase I/II clinical trial evaluating RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II). Dosing of the first subject in the clinical trial is now expected in the fourth quarter of 2018.

REGENXBIO's NAV Technology Platform is currently being applied in the development of more than 20 partnered product candidates by our NAV Technology Licensees. Twelve of these partnered product candidates are in active clinical development. Recent highlights include:

- In May 2018, Novartis AG announced the closing of its $8.7 billion acquisition of AveXis, Inc. As a result of the change of control of AveXis, REGENXBIO received accelerated license payments of $100 million.

- In July 2018, Novartis announced that after a successful second quarter 2018 pre-Biologics License Application (BLA) meeting with the FDA, it remained on track to file the BLA for AVXS-101 in the third quarter of 2018. The Phase I data in spinal muscular atrophy (SMA)

Type 1 will be the basis for the BLA submission with some data from the on-going Phase III STR1VE study. AVXS-101 uses the NAV AAV9 vector.

- In July 2018, Ultragenyx Pharmaceutical Inc. announced that the first subject was dosed in the Phase I/II clinical trial for DTX401 for the treatment of glycogen storage disease type 1a. DTX401 uses the NAV AAV8 vector.

- In August 2018, Audentes Therapeutics announced an update to its positive interim data from its Phase I/II clinical trial for AT132 for the treatment of X-linked myotubular myopathy and that it expects to proceed with dose escalation per protocol in the coming weeks. AT132 uses the NAV AAV8 vector.

- In August 2018, Ultragenyx Pharmaceutical announced that the IND for DTX201 for the treatment of Hemophilia A, which is being developed in partnership with Bayer AG, is active. DTX201 uses the NAV AAVhu37 vector.

"We are pleased to enhance and expand our capabilities with the appointment of Dr. Ram Palanki as Senior Vice President of Commercial Strategy and Operations. We also expect several key developments in the second half of the year, including presenting data from our ongoing HoFH study, providing additional clarity on the development path for the RGX-501 program and initiating dosing in our first clinical trials in RGX-111 for MPS I and RGX-121 for MPS II," said Mr. Mills.

**Financial Results**

Cash, cash equivalents and marketable securities were $306.3 million as of June 30, 2018, compared to $176.4 million as of December 31, 2017. Cash, cash equivalents and marketable securities as of June 30, 2018 include $180.0 million received in 2018 in connection with the amendment to our license agreement with AveXis for the development and commercialization of treatments for SMA.

Revenues were $40.0 million for the three months ended June 30, 2018, compared to $6.6 million for the three months ended June 30, 2017. The increase is primarily attributable to $40.0 million of license revenue recognized during the three months ended June 30, 2018 under the amended license agreement with AveXis. The May 2018 acquisition of AveXis by Novartis triggered the acceleration of $100.0 million in license payments to REGENXBIO, of which $40.0 million, related to an accelerated sale-based milestone payment, was recognized as revenue during the three months ended June 30, 2018. Due to the non-recurring license revenue recognized under the amended license agreement with AveXis during the three and six months ended June 30, 2018, REGENXBIO expects license revenue for the remainder of 2018 to be substantially lower than the first half of 2018.

Research and development expenses were $21.5 million for the three months ended June 30, 2018, compared to $13.9 million for the three months ended June 30, 2017. The increase was primarily attributable to personnel costs as a result of increased headcount, laboratory and facilities costs and expenses associated with conducting clinical trials and externally sourced manufacturing-related services.

General and administrative expenses were $8.3 million for the three months ended June 30, 2018, compared to $6.4 million for the three months ended June 30, 2017. The increase was primarily attributable to personnel costs as a result of increased headcount and professional fees for advisory services.

Net income was $10.6 million, or $0.33 basic and $0.30 diluted net income per share, for the three months ended June 30, 2018, compared to a net loss of $14.5 million, or $0.47 basic and diluted net loss per share, for the three months ended June 30, 2017. Net income for the three months ended June 30, 2018 was primarily driven by the non-recurring license revenue recognized under the amended license agreement with AveXis.

**Financial Guidance**

Based on its current operating plan, REGENXBIO expects that its balance in cash, cash equivalents and marketable securities will be between $250 million and $260 million as of December 31, 2018, which will be used to support the continued development of its lead product candidate programs.

**Conference Call and Webcast Information**

In connection with this announcement, REGENXBIO will host a conference call and webcast today at 8:00 a.m. ET. To access the live call by phone, dial (855) 422-8964 (domestic) or (210) 229-8819 (international), and enter the passcode 3154969. To access a live or recorded webcast of the call and accompanying slides, please visit the "Investors" section of the REGENXBIO website at www.regenxbio.com. The recorded webcast will be available for approximately 30 days following the call.

**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 RGX-314**

RGX-314 is being developed as a one-time subretinal treatment for wet AMD. It includes 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 preclinical animal models with conditions similar to macular degeneration, significant and dose-dependent reduction of blood vessel growth and prevention of disease progression was observed after a single subretinal dose of RGX-314.

**About Wet AMD**

Wet AMD is characterized by loss of vision due to new leaky blood vessel formation in the retina. This results in fluid leakage that can manifest in physical changes in the structure of the retina and loss of vision. Wet AMD is a significant cause of vision loss in the United States, Europe and Japan. There may be more than 2 million people living with wet AMD in these geographies alone.

Current anti-VEGF therapies have significantly changed the landscape for treatment of wet AMD, becoming the standard of care due to their ability to improve vision and retinal fluid in the majority of patients. These therapies, however, require repetitive and inconvenient intraocular injections, typically ranging from every four to eight weeks in frequency, to maintain efficacy. Patients often experience a decline in the initial vision gain from therapy with reduced frequency of treatment over time.

**About RGX-501**

RGX-501 is being developed as a novel, one-time intravenous treatment for HoFH. RGX-501 is designed to use the NAV AAV8 vector to deliver a functional copy of the LDLR gene to liver cells. This may enable liver cells to make the LDLR protein they need to process elevated levels of LDL-C. The liver is a preferred target for HoFH gene therapy as it is the most important organ for expressing LDLRs, and contributes to greater than 90 percent of the capture and breakdown of LDL-C. RGX-501 has received orphan drug product designation from the FDA.

**About HoFH**

HoFH occurs when people inherit an abnormal copy of the LDLR gene from each of their parents. Individuals with HoFH have very low levels of – or are completely missing – LDLR, resulting in high levels of LDL-C in the blood. High levels of LDL-C (or "bad" cholesterol) are associated with premature and aggressive buildup of plaque in arteries, life-threatening coronary artery disease and an increase in the risk of a heart attack or stroke. If untreated, individuals with HoFH can suffer serious cardiac events before the age of 30. Current treatments do not provide a cure and may not lower cholesterol to optimal levels.

**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, clinical trials, costs 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.

[1] Three SAEs are attributed to one subject in Cohort 1 and the events were assessed as not related to RGX-314. Five months after the administration of RGX-314, this subject was hospitalized after developing symptoms related to a pre-existing condition that led to the subject's death. One SAE was a procedure-related peripheral retinal detachment that occurred, was repaired with a scleral buckle and resolved without significant sequelae. The other SAE was assessed as mild in severity with no relationship to RGX-314.

**REGENXBIO INC.**
**CONSOLIDATED BALANCE SHEETS**
**(unaudited)**
**(in thousands, except per share data)**

| | June 30, 2018 | December 31, 2017 |
|---|---|---|
| **Assets** | | |
| Current assets | | |
| Cash and cash equivalents | $ 106,889 | $ 46,656 |
| Marketable securities | 179,605 | 114,122 |
| Accounts receivable | 739 | 473 |
| Prepaid expenses | 3,690 | 5,334 |

| | | |
|---|---|---|
| Other current assets | 2,347 | 1,412 |
| Total current assets | 293,270 | 167,997 |
| Marketable securities | 19,795 | 15,616 |
| Accounts receivable | 4,485 | — |
| Property and equipment, net | 16,698 | 13,977 |
| Restricted cash | 225 | 225 |
| Other assets | 1,514 | 862 |
| Total assets | $ 335,987 | $ 198,677 |
| **Liabilities and Stockholders' Equity** | | |
| Current liabilities | | |
| Accounts payable | $ 4,230 | $ 4,832 |
| Accrued expenses and other current liabilities | 12,023 | 9,605 |
| Deferred revenue | 600 | — |
| Total current liabilities | 16,853 | 14,437 |
| Deferred rent, net of current portion | 1,192 | 1,211 |
| Other liabilities | 720 | — |
| Total liabilities | 18,765 | 15,648 |
| Stockholders' equity | | |
| Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at June 30, 2018 and December 31, 2017 | — | — |
| Common stock; $0.0001 par value; 100,000 shares authorized at June 30, 2018 and December 31, 2017; 32,275 and 31,295 shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively | 3 | 3 |
| Additional paid-in capital | 386,110 | 371,497 |
| Accumulated other comprehensive loss | (771) | (715) |
| Accumulated deficit | (68,120) | (187,756) |
| Total stockholders' equity | 317,222 | 183,029 |
| Total liabilities and stockholders' equity | $ 335,987 | $ 198,677 |

**REGENXBIO INC.**
**CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)**
**(unaudited)**
**(in thousands, except per share data)**

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2018 | 2017 | 2018 | 2017 |
| **Revenues** | | | | |
| License revenue | $ 40,031 | $ 6,555 | $ 172,422 | $ 7,010 |
| Other revenues | — | 7 | — | 7 |
| Total revenues | 40,031 | 6,562 | 172,422 | 7,017 |
| **Expenses** | | | | |

| | | | | |
|---|---|---|---|---|
| Costs of revenues | | | | |
| Licensing costs | 3,872 | 1,311 | 6,280 | 1,402 |
| Other | — | 6 | — | 6 |
| Research and development | 21,486 | 13,917 | 41,036 | 30,536 |
| General and administrative | 8,318 | 6,355 | 16,698 | 12,977 |
| Other operating expenses | 5 | 29 | 33 | 74 |
| Total operating expenses | 33,681 | 21,618 | 64,047 | 44,995 |
| Income (loss) from operations | 6,350 | (15,056) | 108,375 | (37,978) |
| **Other Income** | | | | |
| Interest income from licensing | 6,898 | — | 8,253 | — |
| Investment income | 1,196 | 583 | 2,055 | 1,512 |
| Total other income | 8,094 | 583 | 10,308 | 1,512 |
| Income (loss) before income taxes | 14,444 | (14,473) | 118,683 | (36,466) |
| **Income Tax Expense** | (3,850) | — | (3,850) | — |
| Net income (loss) | $ 10,594 | $ (14,473) | $ 114,833 | $ (36,466) |
| **Other Comprehensive Income (Loss)** | | | | |
| Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense | 132 | (74) | (56) | (613) |
| Total other comprehensive income (loss) | 132 | (74) | (56) | (613) |
| Comprehensive income (loss) | $ 10,726 | $ (14,547) | $ 114,777 | $ (37,079) |
| Net income (loss) applicable to common stockholders | $ 10,594 | $ (14,473) | $ 114,833 | $ (36,466) |
| Net income (loss) per share: | | | | |
| Basic | $ 0.33 | $ (0.47) | $ 3.60 | $ (1.27) |
| Diluted | $ 0.30 | $ (0.47) | $ 3.29 | $ (1.27) |
| Weighted-average common shares outstanding: | | | | |
| Basic | 32,082 | 30,662 | 31,858 | 28,678 |
| Diluted | 35,272 | 30,662 | 34,884 | 28,678 |

**CONTACT:**
**Investors**
Natalie Wildenradt, 646-681-8192
natalie@argotpartners.com

**Media**
Adam Pawluk, 202-591-4063
apawluk@jpa.com

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