REGENXBIO Provides Year-End 2017 Corporate Update

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- Initiated third cohort dosing of RGX-314 Phase I clinical trial for wet AMD -

- Initiated second cohort dosing of RGX-501 Phase I/II clinical trial for HoFH -

- Anticipate completing dosing and presenting topline data from RGX-314 and RGX-501 trials in late 2018 -

- Anticipate initiating dosing in clinical trials for MPS I and MPS II in first half 2018 -

- Ended 2017 with greater than $175 million in cash, cash equivalents and marketable securities -

ROCKVILLE, Md., Jan. 04, 2018 (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, today provided a year-end 2017 corporate update.

"In 2017, REGENXBIO achieved our key development goals for our lead product candidate programs and expanded our research and development organization and capabilities. We expect to build upon this success in 2018 as we work toward advancing a robust clinical pipeline of product candidates that hold the potential to improve treatment in many diseases," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "This coming year, we anticipate substantial clinical data reporting from programs using our NAV Technology Platform, including topline data from our trials of RGX-314 for the treatment of wet age-related macular degeneration, RGX-501 for the treatment of homozygous familial hypercholesterolemia, and trials from our licensees Audentes, Shire and Ultragenyx. In addition, we expect several new INDs for NAV Technology Licensee programs to be filed in 2018, as well as progress updates from AveXis on the continued enrollment of their pivotal trial for SMA Type 1, which uses the NAV AAV9 vector."

Lead Product Candidate Clinical Trial Updates

- RGX-314 for the treatment of wet age-related macular degeneration (wet AMD)
  -- As of December 31, 2017, 13 participants had been treated with a single administration of RGX-314 in the dose-escalation clinical trial, with no reported drug-related serious adverse events (SAEs).
  -- An independent Data Safety and Monitoring Board (DSMB) granted clearance to proceed to dosing a third cohort based on their assessment of the safety and tolerability data of the first and second cohorts.
  -- As of December 1, 2017, the first and second cohorts (six participants in each) had received a single administration of RGX-314 at doses of 3 x 10^9 and 1 x 10^10 genome copies (GC)/eye, respectively, and had been followed for an average of 20 weeks and six weeks, respectively. Additionally, the first patient in the third cohort was subsequently treated at a dose of 6 x 10^10 GC/eye.
  -- Treatments have been administered at four sites in the United States. The procedure has been completed via automated delivery by each of the trained surgeons using a vitrectomy machine within an hour or less in most cases.
  -- The primary endpoint for our ongoing Phase I clinical trial of RGX-314 is safety. Based on the 12 patients dosed as of December 1, 2017, we have observed RGX-314 to be generally well-tolerated. There were no drug-related SAEs, and all drug-related adverse events (AEs) were assessed as mild or moderate.
  -- There have been no observed immune responses, drug-related ocular inflammation, or any post-surgical inflammation beyond what is expected following routine vitrectomy.
  -- Evidence of dose-dependent RGX-314 protein expression, as measured by enzyme-linked immunosorbent assay (ELISA) at approximately four weeks after administration of RGX-314, has been noted in subjects treated to date in the first and second dosing cohorts.
  -- For further details on the RGX-314 Phase I trial, enrollment criteria and eligibility, please

contact patientadvocacy@regenxbio.com or visit https://clinicaltrials.gov/ct2/show/NCT03066258.

- RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH)
  -- As of December 31, 2017, three participants had been treated with a single administration of RGX-501 in the dose-escalation clinical trial, with no dose limiting toxicities reported, and a fourth participant was recently treated.
  -- An independent DSMB granted clearance to proceed to dosing a second cohort based on their assessment of the safety and tolerability data from the first dosing cohort.
  -- As of December 1, 2017, the first three participants had received a single administration of RGX-501 at an initial dose of 2.5 x 10^12 genome copies (GC)/kg body weight and had been followed for an average of 29 weeks. A fourth patient was recently infused at a dose of 7.5 x 10^12 GC/kg body weight.
  -- All treatments have been administered at the University of Pennsylvania and infusion time has averaged less than two hours.
  -- The primary endpoint for our ongoing Phase I/II clinical trial of RGX-501 is safety. Based on the three patients dosed as of December 1, 2017, we have observed RGX-501 to be generally well-tolerated.
  -- One subject in the first cohort experienced an SAE within 24 hours of dosing of hypotension associated with a mild inflammatory response, which resolved within a few hours of onset. The nature and time frame of the SAE is distinct from expected and known immune responses to AAV therapy. The subject recovered quickly from the event without significant sequalae. All other AEs to date have been mild to moderate with no determined relationship to drug product.
  -- For further details on the RGX-501 Phase I/II trial, enrollment criteria and eligibility, please contact patientadvocacy@regenxbio.com or visit http://www.clinicaltrials.gov/ct2/show/NCT02651675.
- RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I)
  -- Site activation is continuing in the Phase I clinical trial evaluating RGX-111 for the treatment of MPS I.
  -- Patient recruitment is anticipated to begin in the first quarter of 2018, with the first patient expected to be dosed in the first half of 2018.
- RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II)
  -- In December 2017, REGENXBIO announced that the Investigational New Drug (IND) application for the Phase I/II clinical trial of RGX-121 for the treatment of MPS II is active.
  -- Site activation in this planned multi-center, open-label, multiple-cohort, dose-escalation trial is underway to support recruitment and patient enrollment, with the first patient expected to be dosed in the first half of 2018.

Other Operational Highlights

In 2017, REGENXBIO invested in internal capabilities, expanded and enhanced its research and development organization – including the addition of Olivier Danos, Ph.D., as Chief Scientific Officer – and initiated the build-out of a state-of-the-art research and development facility, which is expected to be completed in the first half of 2018.

Anticipated 2018 Milestones

REGENXBIO expects to meet the following anticipated milestones related to the clinical development of internal lead product candidate programs:

- RGX-314 for the treatment of wet AMD
  -- Complete enrollment in the Phase I clinical trial in the first half of 2018.
  -- Present topline data from the Phase I clinical trial in late 2018.
- RGX-501 for the treatment of HoFH
  -- Complete enrollment in the Phase I/II clinical trial in mid-2018.
  -- Present topline data from the Phase I/II clinical trial in late 2018.
- RGX-111 for the treatment of MPS I

  - -- Begin enrollment in a Phase I clinical trial in the first half of 2018.
  - -- Present interim update from the Phase I clinical trial in late 2018.
- RGX-121 for the treatment of MPS II
  - -- Begin enrollment in a Phase I/II clinical trial in the first half of 2018.
  - -- Present interim update from the Phase I/II clinical trial in late 2018.

In addition, REGENXBIO expects to announce the designation of a new pipeline lead program candidate in the second half of 2018.

NAV Technology Licensee Program Highlights

As of December 31, 2017, REGENXBIO's NAV Technology Platform was being applied in more than 20 partnered product candidates developed by 10 NAV Technology Platform Licensees (NAV Technology Licensees). There were a number of NAV Technology Licensee advancements announced in 2017, which included:

Clinical Development of NAV Technology Platform

- AVXS-101 (AveXis, Inc.), which uses the NAV AAV9 vector for the treatment of spinal muscular atrophy (SMA):
  -- Announced the first patient has been dosed in the pivotal trial of AVXS-101 for the treatment of SMA Type 1.
  -- Announced plans to immediately initiate a Phase I clinical trial of AVXS-101 for the treatment of SMA Type 2 via the intrathecal (IT) route of administration.
- AT132 (Audentes Therapeutics, Inc.), which uses the NAV AAV8 vector for the treatment of X-linked myotubular myopathy:
  -- Announced completed enrollment of the first cohort in the Phase I/II clinical trial evaluating AT132 for the treatment of X-linked myotubular myopathy.
  -- On January 4, 2018, Audentes announced positive interim data from the first cohort.
- DTX301 (Ultragenyx Pharmaceutical Inc.), which uses the NAV AAV8 vector for the treatment of ornithine transcarbamylase (OTC) deficiency:
  -- Announced completed enrollment of the first cohort in the Phase I/II clinical trial evaluating DTX301 for the treatment of OTC deficiency.
- SHP654 (Shire plc), which uses the NAV AAV8 vector for the treatment of hemophilia A
  -- Announced the FDA had awarded Orphan Drug Designation to its gene therapy candidate SHP654 for the treatment of hemophilia A.

Expansion of NAV Technology Platform

- In August 2017, we granted Prevail Therapeutics Inc. a license for the use of the NAV AAV9 vector to develop gene therapy product candidates for the treatment of Parkinson's disease and other related neurodegenerative disorders.
- In June 2017, we granted AveXis, Inc. a new license for the use of the NAV AAV9 vector for the development and commercialization of gene therapies for Rett syndrome and amyotrophic lateral sclerosis (ALS) caused by mutations in the SOD1 gene.

Financial Guidance

As of December 31, 2017, REGENXBIO had more than $175 million in cash, cash equivalents and marketable securities. REGENXBIO's cash burn in 2017 was approximately $64 million, which is below the previously updated cash burn guidance range of between $75 million and $80 million, excluding the effect of REGENXBIO's underwritten public offering of common stock in March 2017, which resulted in aggregate net proceeds to REGENXBIO of approximately $81.5 million after deducting underwriting discounts and commissions and offering expenses. REGENXBIO expects full-year 2018 cash burn to be between $85 million and $95 million, which will support the continued development of its lead product candidate programs.

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.

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 of REGENXBIO’s clinical trials; the timing and success of preclinical studies and clinical trials 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, 2016 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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