REGENXBIO ANNOUNCES NEW POSITIVE DATA FROM AFFINITY DUCHENNE® TRIAL OF RGX-202

August 01, 2024 07:03 AM EDT

- *Robust microdystrophin expression observed in new data from pivotal dose*
  - *Patients aged 5.8 and 8.5 years at dosing had expression levels at 77.2% and 46.5% of control, respectively*
- *Consistent high expression of microdystrophin across treated patients in all age groups continues to support plans for accelerated approval*
- *Expects to initiate pivotal trial in Q4 2024*

ROCKVILLE, Md., Aug. 1, 2024 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX) today reported new, positive interim safety and efficacy data in the Phase I/II AFFINITY DUCHENNE® trial of RGX-202 in patients with Duchenne muscular dystrophy (Duchenne) ages 1 to 11 years old.

RGX-202 is an investigational one-time AAV Therapeutic designed to deliver a novel microdystrophin gene that includes key regions of the naturally occurring dystrophin gene. RGX-202 is the only gene therapy approved or in development for Duchenne that encodes for the C-Terminal (CT) domain to produce a microdystrophin that is closer to naturally occurring dystrophin. In preclinical studies, the CT domain has been shown to protect the muscle from contraction-induced stress and improve its ability to repair itself.

"With today's new, positive data, we are seeing a clear dose response and consistent, robust microdystrophin expression levels across all treated patients in the AFFINITY DUCHENNE trial, and, notably, among the highest levels of microdystrophin expression reported in older ambulatory patients," said Curran M. Simpson, President and CEO, REGENXBIO. "This data continues to build on the totality of evidence supporting the potential for RGX-202 to be a differentiated, best-in-class treatment for Duchenne. RGX-202 is well positioned to be the next potential gene therapy approved for Duchenne, and, with our commercial-ready, suspension-based manufacturing platform process, we believe we have the capacity to serve the entire market."

"I remain encouraged by the biomarker data from the AFFINITY DUCHENNE trial of RGX-202 and am eagerly anticipating the initial functional data from this program," said Aravindhan Veerapandiyan, M.D., Arkansas Children's Hospital. "This update is also encouraging for the Duchenne community, which is exploring various treatment options that could influence disease progression."

Data Update
In patients aged 5.8 and 8.5 who received RGX-202 at dose level 2, RGX-202 microdystrophin expression was measured to be 77.2%, and 46.5%, respectively, compared to control at three months.

As of July 8, 2024, RGX-202 has been well tolerated with no serious adverse events. All seven patients who completed three-month trial assessments indicate meaningful increases in expression of RGX-202 microdystrophin and reduction from baseline in serum creatinine kinase levels, supporting evidence of clinical improvement.

Microdystrophin expression results to date by dose and age in the AFFINITY DUCHENNE trial are shown in the table below.

Age range at screening	Dose Level 1 % RGX-202 microdystrophin (n = 3)	Dose Level 2 (Pivotal Dose) % RGX-202 microdystrophin (n = 4)
4 to 7 years	38.8, 83.4	**77.2**
8 to 11 years	11.1	**20.9, 46.5, 75.7**

Clinical Program Updates
RGX-202 in the AFFINITY DUCHENNE trial is manufactured at the REGENXBIO Manufacturing Innovation Center using the Company's proprietary, high-yielding NAVXpress™ platform process. This suspension-based manufacturing process has demonstrated scalability up to 2,000L with consistent yield and product purity and is suitable for product commercialization. The REGENXBIO Manufacturing Innovation Center has the capacity and yields to produce up to 2,500 doses of RGX-202 per year.

REGENXBIO expects to complete enrollment in the dose level 2 expansion cohort early in the third quarter of 2024 and has initiated enrollment in the cohort for boys aged 1-3. Initiation of the pivotal trial is expected in the fourth quarter of 2024.

REGENXBIO expects to share initial strength and functional assessment data for both dose levels of the AFFINITY DUCHENNE trial in the second half of 2024.

AFFINITY DUCHENNE Trial Design
The Phase I/II AFFINITY DUCHENNE trial is a multicenter, open-label dose escalation and dose expansion clinical study to evaluate the safety, tolerability and clinical efficacy of a one-time intravenous (IV) dose of RGX-202 in patients with Duchenne aged 1-11.

The trial design was informed by the Duchenne community and engagement with key opinion leaders, including a comprehensive, short-term, prophylactic immunosuppression regimen to proactively mitigate potential complement-mediated immunologic responses, and inclusion criteria based on dystrophin gene mutation status, including DMD gene mutations in exons 18 and above. Trial endpoints include safety, immunogenicity assessments, pharmacodynamic and pharmacokinetic measures of RGX-202, including microdystrophin protein levels in muscle, and strength and functional assessments, including the North Star Ambulatory Assessment (NSAA) and timed function tests.

About RGX-202
RGX-202 is a next-generation investigational gene therapy designed for improved function and outcomes in Duchenne. RGX-202 is the only gene therapy approved or in late-stage development for Duchenne with a differentiated microdystrophin construct that encodes key regions of naturally occurring dystrophin, including the C-Terminal (CT) domain. In preclinical studies, the CT domain has been shown to protect the muscle from contraction-induced stress and improve its ability to repair itself. Additional design features, including codon optimization and reduction of CpG content, may potentially improve gene expression, increase protein translation efficiency and reduce immunogenicity. RGX-202 is designed to support the delivery and targeted expression of genes throughout skeletal and heart muscle using the NAV® AAV8 vector and a well-characterized muscle-specific promoter (Spc5-12). RGX-202 is manufactured using REGENXBIO's proprietary, high-yielding NAVXpress™ suspension-based platform process.

About Duchenne Muscular Dystrophy
Duchenne is a severe, progressive, degenerative muscle disease, affecting 1 in 3,500 to 5,000 boys born each year worldwide. Duchenne is caused by mutations in the Duchenne gene which encodes for dystrophin, a protein involved in muscle cell structure and signaling pathways. Without dystrophin, muscles throughout the body degenerate and become weak, eventually leading to loss of movement and independence, required support for breathing, cardiomyopathy and premature death.

ABOUT REGENXBIO Inc.
REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Since its founding in 2009, REGENXBIO has pioneered the development of AAV Therapeutics, an innovative class of gene therapy medicines. REGENXBIO is advancing a pipeline of AAV Therapeutics for retinal and rare diseases, including ABBV-RGX-314 for the treatment of wet AMD and diabetic retinopathy, being developed in collaboration with AbbVie, RGX-202 for the treatment of Duchenne and RGX-121 for the treatment of MPS II. Thousands of patients have been treated with REGENXBIO's AAV Therapeutic platform, including Novartis' ZOLGENSMA for children with spinal muscular atrophy. Designed to be one-time treatments, AAV Therapeutics have the potential to change the way healthcare is delivered for millions of people. For more information, please visit www.regenxbio.com.

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," "assume," "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, regulatory plans, 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, 2023, 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. Except as required by law, 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:
Dana Cormack
Corporate Communications
dcormack@regenxbio.com

Investors:
Chris Brinzey
ICR Westwicke
339-970-2843
chris.brinzey@westwicke.com

View original content to download multimedia:https://www.prnewswire.com/news-releases/regenxbio-announces-new-positive-data-from-affinity-duchenne-trial-of-rgx-202-302212086.html

SOURCE REGENXBIO Inc.