Annexon Presented Additional Positive Phase 3 Results for ANX005 C1q-Targeted Immunotherapy in Guillain-Barré Syndrome at the 2024 PNS Annual Meeting

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ANX005-Treated Patients Demonstrated Faster and More Complete Recovery from Week 1 through Week 26 on Primary and Multiple Pre-Specified Endpoints

Two and a Half Times More ANX005-Treated Patients Returned to a Normal / Pre-Disease State of Health Over Placebo on GBS-DS by Week 26, Increasing Over Time

ANX005 Beneficial Impact Larger in Patients with North American and European Baseline Characteristics Across Key Measures of Disability and Muscle Strength

Single Infusion of ANX005 was Generally Well-Tolerated with Safety Profile Similar to Placebo

Data Reinforce Potential of ANX005 to be First Targeted Immunotherapy Treatment for GBS

BRISBANE, Calif., June 25, 2024 (GLOBE NEWSWIRE) -- Annexon, Inc. (Nasdaq: ANNX), today announced positive results from the completed pivotal Phase 3 trial of C1q-targeted immunotherapy, ANX005, in Guillain-Barré Syndrome (GBS) at the 2024 Peripheral Nerve Society (PNS) Annual Meeting in Montréal, Canada. Leading global experts in the GBS field highlighted the significant unmet need and opportunity to transform the GBS treatment landscape with a targeted immunotherapy approach, as well as additional Phase 3 analyses of early and durable treatment effects important to patients and the medical community.

“ANX005 rapidly suppressed neuroinflammation and validated the role of C1q inhibition in GBS during the active phase of disease, leading to highly statistically significant improvements across multiple endpoints and over multiple timepoints versus placebo,” said Douglas Love, president and chief executive officer of Annexon. “Having successfully completed the first placebo-controlled GBS trial in decades, we were honored to present the pivotal Phase 3 results showing accelerated and durable recovery of GBS patients treated with ANX005 compared to placebo in the plenary Symposium at PNS. With these favorable results, we are laser focused on bringing ANX005 to GBS patients worldwide as quickly as possible.”

Dr. Quazi Deen Mohammad, Principal Investigator of the trial and Founding Director of the National Institute of Neurosciences and Hospital (NINS), Bangladesh added, “This well-designed and well-executed study demonstrated that acute suppression of C1q with ANX005 enabled patients to get better sooner, which translated into continued long-term benefits and a significantly higher likelihood of full recovery versus placebo. Notably, patients came off ventilation and walked one month earlier, regained their independence faster, and got back to a normal way of life sooner. In addition, Phase 3 patients with baseline characteristics consistent with North American and European GBS patients had a more pronounced treatment effect, being three times more likely versus placebo to be in a good state of health with ANX005 treatment. These compelling data reinforce the therapeutic potential of ANX005 to be the first targeted immunotherapy treatment for GBS.”

Summary of Phase 3 Data with ANX005 30 mg/kg Treatment

GBS-Disability Scale (GBS-DS)

- Primary endpoint at Week 8: 2.41-fold higher likelihood of being in a better state of health with ANX005 vs. placebo (p = 0.0058)
- Week 1: 7.22-fold higher likelihood of being in a better state of health with ANX005 vs. placebo (*p < 0.0001)
- Week 4: 2.49-fold higher likelihood of being in a better state of health with ANX005 vs. placebo (*p = 0.0073)
- Week 26: 2.5 times more patients had fully recovered to a normal / pre-disease state of health (GBS-DS = 0) with ANX005 (21.5%) vs. placebo (8.6%) (OR 4.14, *p = 0.0092)
- Week 8 responder analysis (pre-specified sensitivity analysis): 2-times more patients improved 3 points or more with ANX005 (28.2%) vs. placebo (13.6%) (*p = 0.0309)
- Week 8 dichotomy analysis (pre-specified sensitivity analysis): 2.5-times more patients were able to run or better ANX005 (29%) vs. placebo (12%) (OR 3.34, *p = 0.0065)

Functional Measures

- Walking 31 days earlier with ANX005 treatment (56 days) vs. placebo (87 days) (*p = 0.0211)
- Off ventilation 28 days earlier with ANX005 treatment (20 days) vs. placebo (48 days) (*p = 0.0356)

Patients with North American and European baseline characteristics

- Week 1: 8.8-point improvement in muscle strength measured by Medical Research Council (MRC) sumscore with ANX005 vs. placebo (*p <0.0001)
- Week 8: 3 times more likely to be in a better state of health on the GBS-DS scale with ANX005 vs. placebo (*p = 0.0102)

Key Findings from the Phase 3 GBS Trial of ANX005 30 mg/kg Treatment

- Phase 3 trial informed by dose-ranging Phase 1b trial, replicating earlier results
- Demonstrated a highly statistically significant effect on primary endpoint of GBS-DS, further supported by multiple prespecified sensitivity analyses
- Defined the effective treatment window during the active phase of GBS, an acute disease
- Early, robust and durable treatment effects expedited patient recovery
- Single dose administration of ANX005 was generally well-tolerated with mostly mild to moderate adverse events, no increased infection rate while not requiring vaccination or prophylactic antibiotics, and a profile similar to placebo

ANX005 Comparability Analyses Flash Presentation and Poster Sessions

- The indirect comparison of ANX005-treated patients from the Phase 1b GBS trial with a separate cohort of matched intravenous immunoglobulin (IVIg)-treated patients was presented as a flash oral and poster presentation at PNS, showing early and significant improvements in muscle strength and overall functional outcomes including reduced mechanical ventilation in patients treated with ANX005 versus IVIg
- The methodology for patient-matching based on prognostic factors for the real-world evidence (RWE) study was presented in a separate poster presentation at PNS. Data from the RWE study will compare the Phase 3 outcomes with patients from the International GBS Outcome Study (IGOS).

*Nominal *p* value

About ANX005
Annexon's lead investigational therapy, ANX005, is a first-of-its kind selective, targeted and rapid-acting agent designed to reduce inflammation and nerve damage by fully stopping C1q activity in the peripheral and central nervous systems. In GBS, ANX005 seeks out C1q and selectively blocks it from binding to its target on peripheral nerves. ANX005 is administered intravenously and has been observed to act almost immediately. In GBS, the aim is to rapidly stop the autoimmune damage of nerve cells, allowing the patient to regain their muscle strength sooner to regain independence and return to pre-illness activities. ANX005 has received both fast track and orphan drug designations from the Food and Drug Administration as well as orphan drug designation by the European Medicines Agency for the treatment of GBS.

About Guillain-Barré Syndrome (GBS)
GBS is a severe disease resulting from an acute autoantibody attack on peripheral nerves that generally occurs post-infection in otherwise healthy persons following activation of C1q and the classical complement cascade. It is a rapid and acute neurological disease with a narrow therapeutic window that results in hospitalization of over 22,000 people annually in the U.S. and Europe. The peripheral nerve damage progresses rapidly, causing acute neuromuscular paralysis, and may lead to significant morbidity, disability and mortality. Currently, there are no approved treatments for GBS in the U.S. The long-term disease burden associated with GBS has led to a multi-billion-dollar annual economic cost to the U.S. healthcare system alone.

About Annexon
Annexon Biosciences (Nasdaq: ANNX) is a biopharmaceutical company advancing a late-stage clinical platform of novel therapies for people living with devastating classical complement-mediated neuroinflammatory diseases of the body, brain, and eye. Annexon's novel scientific approach targets upstream C1q to block the classical complement inflammatory cascade before it starts, and its therapeutic candidates are designed to provide meaningful benefits across multiple autoimmune, neurodegenerative and ophthalmic diseases. With proof-of concept data in Guillain-Barré syndrome, Huntington's disease and geographic atrophy, Annexon is rigorously advancing its mid-to late-stage clinical trials to bring their potential treatments to patients as quickly as possible. To learn more visit annexonbio.com.

Forward Looking Statements
This press release contains 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. In some cases, you can identify forward-looking statements by terminology such as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "design," "due," "estimate," "expect," "goal," "intend," "may," "objective," "plan," "positioned," "potential," "predict," "seek," "should," "suggest," "target," "on track," "will," "would" and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. All statements other than statements of historical facts contained in this press release are forward-looking statements. These forward-looking statements include, but are not limited to, statements about: ability of ANX005 to stop C1q activity; ability to bring ANX005 to patients worldwide as soon as possible; the clinical and regulatory status of ANX005; the potential of ANX005 to be the first approved C1q-targeted treatment for GBS; the timing of completion of RWE study and potential submission of a BLA with the FDA; the potential therapeutic benefit of ANX005 or any other product candidates on GBS, Huntington's disease or geographic atrophy; potential benefit of ANX005, if approved, compared to existing therapies; market size; the potential benefits from treatment with anti-C1q therapy; and Annexon's ability to rigorously advance mid-to late-stage clinical trials and continue development of the company's portfolio. Forward-looking statements are not guarantees of future performance and are subject to risks and uncertainties that could cause actual results and events to differ materially from those anticipated, including, but not limited to, risks and uncertainties related to: the potential for any final clinical trial results to differ from preliminary or topline results; the company's history of net operating losses; the company's ability to obtain necessary capital to fund its clinical programs; the early stages of clinical development of the company's product candidates; the effects of public health crises on the company's clinical programs and business operations; the company's ability to obtain regulatory approval of and successfully commercialize its product candidates; any undesirable side effects or other properties of the company's product candidates; the company's reliance on third-party suppliers and manufacturers; the outcomes of any future collaboration agreements; and the company's ability to adequately maintain intellectual property rights for its product candidates. These and other risks are described in greater detail under the section titled "Risk Factors" contained in the company's Annual Report on Form 10-K and Quarterly Reports on Form 10-Q and the company's other filings with the SEC. Any forward-looking statements that the company makes in this press release are made pursuant to the Private Securities Litigation Reform Act of 1995, as amended, and speak only as of the date of this press release. Except as required by law, the company undertakes no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise.

Investor Contact:
Joyce Allaire

LifeSci Advisors, LLC
jallaire@lifesciadvisors.com

Media Contact:
Sheryl Seapy
Real Chemistry
949-903-4750
sseapy@realchemistry.com