Annexon Announces Clinical and Regulatory Progress for ANX005 Pivotal Program in Guillain-Barré Syndrome (GBS)

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ANX005 Granted Orphan Drug Designation by the European Medicines Agency for the Treatment of GBS

Target Enrollment Achieved in Phase 3 Pivotal Study of ANX005 in GBS Representing the Company's Third Successfully Executed Clinical Study in GBS

Company on Track to Report Pivotal Data in the First Half of 2024

BRISBANE, Calif., Oct. 10, 2023 (GLOBE NEWSWIRE) -- Annexon, Inc. (Nasdaq: ANNX) a clinical-stage biopharmaceutical company developing a new class of complement-based medicines for patients with classical complement-mediated autoimmune, neurodegenerative and ophthalmic disorders, today announced that the European Medicines Agency (EMA) granted orphan drug designation to ANX005 for the treatment of Guillain-Barré Syndrome (GBS). The U.S. Food and Drug Administration (FDA) previously granted orphan drug designation to ANX005 for the treatment of GBS. **For the EMA Committee for Orphan Medicinal Products (COMP) meeting minutes, see here**.

ANX005, a humanized monoclonal antibody, inhibits C1q, the initiator molecule of the classical complement pathway, and is designed to stop complement mediated inflammation and neuronal damage early in GBS. The recent EMA orphan drug designation was based on an indirect comparison between ANX005 and intravenous immunoglobulin (IVIg) that demonstrated a notable and early improvement in muscle strength with ANX005 versus patients treated with IVIg, which translated into observable gains in health status, including a reduction in the need of mechanical ventilation. In granting the designation, EMA stated that preliminary clinical data with ANX005 constitutes "a clinically relevant advantage" over IVIg for patients affected by GBS.

Importantly, Annexon has also achieved target enrollment of 225 patients in the randomized, double-blind, placebo-controlled Phase 3 trial of ANX005 in patients with GBS. This key milestone enables the company to deliver topline Phase 3 data in the first half of 2024.

"GBS is a terrifying and underappreciated life-threatening condition that causes sudden onset of weakness in previously healthy people, leading to significant acute and long-lasting disability and, in some cases, death despite standard of care. This rare disease affects approximately 12,000 people in the U.S. and Europe each year, and the financial burden to the healthcare system is over $2 billion annually in the U.S. alone," says Douglas Love, president and CEO of Annexon. "With European regulatory acknowledgement of the potential for meaningful clinical benefit of ANX005 over standard of care and the achievement of target enrollment in our Phase 3 study, we've taken another important step to deliver this potential therapy to patients."

Advantages of the EMA's orphan drug designation include protocol assistance, reduced fees for EMA procedures, a centralized EU approval process and ten (10) years of market exclusivity. The designation is designed to encourage the development of new treatments for rare conditions. To qualify, an investigational medicine must target a seriously debilitating or life-threatening condition and show sufficient non-clinical or clinical data to suggest it may be of significant benefit over approved products for those affected by the condition.

About the ANX005 Phase 3 Study in GBS
The randomized, double-blind, placebo-controlled, multi-center Phase 3 trial aims to evaluate the efficacy, safety, pharmacokinetics and pharmacodynamics of multiple doses of ANX005 administered by a single intravenous (IV) infusion in recently diagnosed patients with GBS. A single infusion of ANX005 may be important in the early management of this acute disease, in contrast to the five days of IVIg dosing. The study enrolled patients in Southeast Asia diagnosed with GBS according to the National Institute of Neurological Disorders and Stroke Diagnostic Criteria for Guillain-Barré Syndrome at the onset of GBS-related weakness ≤10 days prior to the start of treatment. ANX005 is being developed as a first-line monotherapy treatment option for GBS, and this is the third clinical trial conducted by Annexon in GBS.

The primary endpoint of the Phase 3 study is GBS Disability Score at Week 8, and secondary endpoints include safety, duration of ventilation support, duration of ICU stays, muscle strength, mortality, and patient global impression of change scores. The primary endpoint will utilize a proportional odds methodology to assess the proportion of patients who shift to better outcomes on the GBS Disability Score with ANX005 treatment compared to placebo at week 8.

More information about the study is available at ClinicalTrials.gov (NCT Number: NCT04701164).

The company expects to report data from this pivotal study in the first half of 2024.

About Guillain-Barré Syndrome
GBS is a serious and urgent autoimmune condition of the nervous system that can lead to sudden paralysis and even death in otherwise healthy individuals. GBS is caused by antibodies generated in response to a seemingly routine illness, such as flu or diarrhea, that cross-react with components of a patient's own peripheral nerves. After the infection resolves, the antibodies quietly continue to build until they trigger the immune protein C1q to unleash a powerful inflammatory response that damages peripheral nerves and nerve roots coming from the spinal cord. Within days of feeling normal, patients are sent to the emergency room with weakness or paralysis and, in 1 in 4 cases, need ventilator support with IVIg treatment because of severe muscle weakness that impacts their ability to breathe. GBS impacts approximately 12,000 people annually in the U.S. and EU, and there are currently no approved therapies for GBS in the U.S. The development of targeted treatments for GBS is crucial to improve outcomes and quality of life for those affected by this debilitating condition.

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 nerves in the arms and legs, while allowing other complement system pathways and the rest of the immune system to function normally. ANX005 is administered intravenously and has been observed to act almost immediately – with the aim in GBS to rapidly stop the autoimmune damage of nerve cells, allowing the patient to regain their muscle strength more quickly with greater ability to return to pre-illness activities.

ANX005 is being evaluated in clinical trials for the treatment of GBS, Huntington's disease and ALS. It has received both fast track and orphan drug designations from the FDA as well as orphan drug designation by the EMA for the treatment of GBS.

About Annexon
Annexon Biosciences (Nasdaq: ANNX) is a clinical-stage biopharmaceutical company utilizing a distinctive scientific approach to stop the initiator of classical complement-mediated inflammation, C1q, before it starts. As the only company solely focused on shutting down C1q, Annexon is developing a purposeful pipeline of investigational medicines designed to provide meaningful benefits across multiple diseases of the body, brain and eye. With proof-of concept data in both Guillain-Barré syndrome and geographic atrophy, Annexon is rigorously advancing mid-to late-stage clinical trials to bring first-of-their kind therapies to millions of people living with devastating inflammatory-related diseases. 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; the timing of completion of Phase 3 trial of ANX005 in patients with GBS; the potential therapeutic benefit of ANX005 or any other product candidates on GBS or geographic atrophy; potential benefit of ANX005, if approved, compared to existing therapies; market size; plans to report final results following study conclusion; the potential benefits from treatment with anti-C1q therapy; and continuing advancement 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 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.

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