Annexon Biosciences Reports Top-line Phase 1b Results for Novel C1q Inhibitor ANX007 in Glaucoma

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- Intravitreal ANX007 was well-tolerated and demonstrated full target engagement and inhibition of C1q in the eye for at least four weeks -

- Annexon plans to advance the clinical development of ANX007 in geographic atrophy -

SOUTH SAN FRANCISCO, Calif., October 9, 2019 – – Annexon Biosciences, a clinical-stage biopharmaceutical company developing a pipeline of novel therapies for patients with classical complement-mediated disorders of the body, eye and brain, today announced encouraging results from a Phase 1b dose-ranging clinical trial to evaluate its anti-C1q antibody, ANX007, in patients with glaucoma. ANX007 is an investigational monoclonal antibody antigen-binding fragment (Fab) formulated for intravitreal administration. In the Phase 1b trial, ANX007 was well-tolerated and resulted in full target engagement and inhibition of C1q in the eye for at least four weeks following a single intravitreal treatment.

"Inhibition of C1q may provide neuroprotective benefit by preventing the aberrant loss of functioning synapses in the retina in a variety of ophthalmic diseases," said Jeffrey L. Goldberg, M.D., professor and chair of ophthalmology at the Byers Eye Institute at Stanford University and member of Annexon's Scientific Advisory Board. "There is a significant need to develop novel neuroprotective therapies that can slow damage to the optic nerve and prevent vision loss for patients with glaucoma and other ophthalmic diseases."

Phase 1b Clinical Trial of ANX007

In a Phase 1b clinical trial of ANX007, glaucoma patients (n=17) were treated with multiple doses of intravitreal ANX007. The trial objectives included safety, tolerability, pharmacokinetics (PK), and target engagement. Top-line results demonstrated:

- ANX007 was well-tolerated at all dose levels
- At the two higher dose levels, a single intravitreal injection of ANX007 achieved complete suppression of the C1q target for at least four weeks, as measured in ocular fluid from aqueous humor taps.
- These results were consistent with preclinical models.

Sanjay Keswani, M.D., chief medical officer of Annexon added, "We're encouraged by ANX007's safety, tolerability and PK profile since this agent fully inhibited C1q in the eye at both the low and high doses evaluated. Based on the promising data from this clinical trial, our preclinical data to date, and our knowledge of C1q biology in this setting, we are excited to advance ANX007 into later-stage clinical trials for geographic atrophy and other ophthalmic diseases."

The classical complement pathway is implicated in geographic atrophy (GA) by human genetics, and C1q appears to play a potential dual role in GA. C1q accumulates on photoreceptor cell synapses with normal age or disease and may lead to aberrant synapse removal and neuronal loss in disease. C1q also accumulates in the retina below photoreceptor cells on extracellular deposits called drusen, and may contribute to the localized tissue damage unique to the specialized compartment of the outer retina in GA. C1q is produced locally in the eye by infiltrating immune cells and, in contrast to other complement factors, may be more amenable to local inhibition by intravitreal administration of ANX007. In a preclinical model of photoreceptor cell loss, C1q inhibition was shown to be protective against cell loss and functional decline, and in a pharmacokinetic analysis, ANX007 achieved complete C1q suppression for at least four weeks following intravitreal administration.

About ANX007

ANX007 is clinical-stage investigational monoclonal antibody antigen-binding fragment (Fab) for the treatment of patients with neurodegenerative ophthalmic diseases. Formulated for intravitreal administration, ANX007 is designed to potently bind and inhibit C1q and all downstream components of the classical complement cascade, including C3 and C5, but to not interfere with the normal function of C3 and C5 as part of other complement pathways. Annexon has completed a Phase 1b clinical trial of ANX007 in glaucoma and plans to advance ANX007 into later-stage clinical trials for geographic atrophy and other ophthalmic diseases.

About Geographic Atrophy

Geographic Atrophy (GA) is an advanced, vision threatening form of age-related macular degeneration (AMD) and is a chronic, progressive neurological disease of the central retina, or macula, that results in loss of central vision. Dry AMD is the most common form of AMD, representing approximately 85% to 90% of all AMD cases. In its advanced stages, dry AMD leads to geographic atrophy (GA), characterized by progressive atrophy of retinal pigment epithelial cells, overlying photoreceptors and underlying choriocapillaries. GA accounts for about ten percent of legal blindness related to AMD. Approximately one million individuals in the United States suffer from GA and the prevalence increases with age. There are no approved therapies to prevent either the onset or progression of GA.

About Annexon Biosciences

Annexon is a clinical-stage biopharmaceutical company developing a pipeline of novel therapies for patients with classical complement-mediated disorders of the body, eye and brain. The Company's pipeline is based on its platform technology addressing well-researched classical complement-mediated autoimmune and neurodegenerative disease processes, both of which are triggered by aberrant activation of C1q, the initiating molecule of the classical complement pathway. The Company's first product candidate, ANX005, is a full-length monoclonal antibody formulated for intravenous administration in autoimmune and neurodegenerative disorders. The Company's second product candidate, ANX007, is a monoclonal antibody antigen-binding fragment (Fab) formulated for intravitreal administration for the treatment of neurodegenerative ophthalmic disorders. Based on

learnings from its initial trials, Annexon is advancing its current programs while evaluating additional orphan and large market indications. Annexon is deploying a disciplined, biomarker-driven development strategy designed to establish that each of its product candidates is engaging the specific target at a well-tolerated therapeutic dose in the intended patient tissue. For more information, visit www.annexonbio.com.

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