

## **RGX-314**

**RGX-314 is our product candidate for the treatment of wet age-related macular degeneration (AMD).**

RGX-314 is being developed as a novel, one-time subretinal treatment for wet AMD that includes the NAV AAV8 vector containing a gene encoding for a monoclonal antibody fragment. The expressed protein is designed to neutralize vascular endothelial growth factor (VEGF) activity, modifying the pathway for formation of new leaky blood vessels and retinal fluid accumulation.

Wet AMD is characterized by loss of vision due to excess blood vessel formation between two layers of cells in the retina. This excess blood vessel formation results in fluid leakage that can result in physical changes in the structure of the retina and changes in vision. As this process becomes more severe, blindness can result from scar formation due to hemorrhaging.

Current anti-VEGF therapies require repetitive and inconvenient intraocular injections, typically ranging from every four to eight weeks in frequency, to maintain efficacy. Due to a variety of factors, including inconvenience and discomfort associated with frequent injections in the eye, patient compliance is a significant concern with anti-VEGF therapies. Patients often experience vision loss with reduced frequency of treatment.

Subjects are currently being enrolled in our Phase I clinical trial of subretinally delivered RGX-314.