Exegenesis Bio Announces FDA Clearance of Investigational New Drug (IND) Application for EXG102-031:

A Novel Gene Therapy for the Treatment of neovascular Age-Related Macular Degeneration (nAMD)

- Phase 1 clinical trial to be initiated in Q1 2023 –

- The first clinical stage gene therapy for treatment of nAMD that targets all known subtypes of VEGF and ANG2 -

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PHILADELPHIA--(BUSINESS WIRE) -- Exegenesis Bio, a rapidly growing global gene therapy company is pleased to announce that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for EXG102-031, a recombinant adeno-associated virus (rAAV) based gene therapy that is being studied for the treatment of neovascular Age Related Macular Degeneration (nAMD), the leading cause of severe vision loss and irreversible blindness worldwide.

"We are excited by the progress that our company has made and pleased to have reached this critical milestone; this is our second IND approval and the first in North America since the inception of Exegenesis Bio 3 years ago," stated Zhenhua Wu, CEO of Exegenesis Bio. "This is a strong validation of the world-class R&D, CMC, quality and regulatory capabilities that we have built. We look forward to accelerating development of our innovative gene therapy pipeline in areas with high unmet medical needs and bringing these innovative treatments to patients worldwide."

About AMD and nAMD

Nearly 20 million people in the US are living with Age-Related Macular Degeneration (AMD), a progressive eye disease caused by damage to the macula in the center of the retina that can lead to blurred vision and blindness. Almost 2 million of these individuals have a more severe form of AMD, neovascular Age-related macular degeneration (nAMD), also called wet AMD (wAMD). nAMD is caused by abnormal blood vessel growth and blood or fluid leakage into the macula, leading to scarring and rapid loss of central vision. nAMD accounts for approximately 90 percent of all AMD-related blindness worldwide.

About EXG102-031

EXG102-031 intraocular injection is a rAAV-based gene therapy expressing a therapeutic fusion protein that is able to bind/neutralize all known subtypes of Vascular Endothelial Growth Factor (VEGF) and Angiopoietin-2 (ANG2), which are known to stimulate abnormal blood vessel formation and vascular leakage in the retina. The EXG102-031 Phase 1 clinical trial will evaluate safety and tolerability, as well as visual acuity and central retinal thickness in patients with nAMD.

About Exegenesis Bio

Exegenesis Bio is a clinical stage global gene therapy company with operations in Philadelphia, Boston and China. The company's innovative gene therapy pipeline is based on proprietary capsids, promoters and unique protein engineering designs. Two programs have advanced to the clinical stage: (1) Type I Spinal Muscular Atrophy (SMA) AAV gene therapy in China and, (2) nAMD in USA. The company has built state-of-the-art cGMP manufacturing facilities that include 500L and 2,000L disposable bioreactors for viral vectors and 30L disposable fermenters for plasmids. Exegenesis Bio has raised over $150 M since inception in 2019 and currently employs over 200 scientific and operations staff worldwide.

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Sources: Rein DB, Wittenborn JS, Burke-Conte Z, et al. Prevalence of Age-Related Macular Degeneration in the US in 2019. *JAMA Ophthalmol.* 2022;140(12):1202–1208. doi:10.1001/jamaophthalmol.2022.4401