Stoke Therapeutics to Present at The Association for Research in Vision and Ophthalmology (ARVO) 2022 Annual Meeting

April 7, 2022

– New preclinical data support the ongoing preclinical development of STK-002 for the treatment of autosomal dominant optic atrophy (ADOA) –

– ADOA is the most common inherited optic nerve disorder –

BEDFORD, Mass.--(BUSINESS WIRE)--Apr. 7, 2022-- Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company dedicated to addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines, announced today the upcoming presentation of new preclinical data on STK-002 at The Association for Research in Vision and Ophthalmology (ARVO) 2022 Annual Meeting being held May 1-4, 2022 in Denver, Colorado. STK-002 is a proprietary antisense oligonucleotide (ASO) in preclinical development for the treatment of autosomal dominant optic atrophy (ADOA).

The presentation details are as follows:

Title: STK-002, an Antisense Oligonucleotide (ASO) for the Treatment of Autosomal Dominant Optic Atrophy (ADOA), is Taken Up by Retinal Ganglion Cells (RGC) and Upregulates OPA-1 Protein Expression After Intravitreal Administration to Non-human Primates (NHP)
Presenter: Karen Anderson
Session: Gene therapy and other novel therapeutics in ophthalmic diseases 1
Date and Time: May 2, 2022 from 10:00 AM to 12:00 PM MDT
Presentation Number: 1111

Title: Models of Autosomal Dominant Optic Atrophy (ADOA) using *OPA1* haploinsufficient iPSCs and response to Targeted Augmentation of Nuclear Gene Output (TANGO) Antisense Oligonucleotides (ASOs) Treatment
Presenter: Ray Oh
Session: Animal models of human ocular disease
Date and Time: May 2, 2022 from 3:00 PM to 5:00 PM MDT
Poster Number: 1895 – A0041

About TANGO

TANGO (Targeted Augmentation of Nuclear Gene Output) is Stoke's proprietary research platform. Stoke's initial application for this technology are diseases in which one copy of a gene functions normally and the other is mutated, also called haploinsufficiencies. In these cases, the mutated gene does not produce its share of protein, so the body does not function normally. Using the TANGO approach and a deep understanding of RNA science, Stoke researchers design antisense oligonucleotides (ASOs) that bind to pre-mRNA and help the target genes produce more protein. TANGO aims to restore missing proteins by increasing – or stoking – protein output from healthy genes, thus compensating for the non-functioning copy of the gene.

About Autosomal Dominant Optic Atrophy (ADOA)

Autosomal dominant optic atrophy is the most common inherited optic nerve disorder. It is a rare disease that causes progressive and irreversible vision loss in both eyes starting in the first decade of life. Severity can vary and the rate of vision loss can be difficult to predict. Roughly half of people with ADOA fail driving standards and up to 46% are registered as legally blind. ADOA is considered a haploinsufficiency disease, as most people living with ADOA have genetic mutations in the *OPA1* gene that result in only half the necessary OPA1 protein being produced. More than 400 *OPA1* mutations have been reported in people diagnosed with ADOA. Currently there is no approved treatment for people living with ADOA. ADOA affects approximately one in 30,000 people globally with a higher incidence in Denmark of one in 10,000 due to a founder effect. An estimated 65% to 90% of cases are caused by mutations in the *OPA1* gene, most of which lead to a haploinsufficiency resulting in 50% OPA1 protein expression and disease manifestation.

About STK-002

STK-002 is a proprietary antisense oligonucleotide (ASO) in preclinical development for the treatment of Autosomal Dominant Optic Atrophy (ADOA). Stoke believes that STK-002 has the potential to be the first disease-modifying therapy for people living with ADOA. STK-002 is designed to upregulate OPA1 protein expression by leveraging the non-mutant (wild-type) copy of the OPA1 gene to restore OPA1 protein expression with the aim to stop or reverse vision loss in patients with ADOA. Stoke has generated preclinical data demonstrating proof-of-mechanism and proof-of-concept for STK-002.

About Stoke Therapeutics

Stoke Therapeutics (Nasdaq: STOK), is a biotechnology company dedicated to addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines. Using Stoke's proprietary TANGO (Targeted Augmentation of Nuclear Gene Output) approach, Stoke is developing antisense oligonucleotides (ASOs) to selectively restore protein levels. Stoke's first compound, STK-001, is in clinical testing for the treatment of Dravet syndrome, a severe and progressive genetic epilepsy. Dravet syndrome is one of many diseases caused by a haploinsufficiency, in which a loss of ~50% of normal protein levels leads to disease. Stoke is pursuing the development of STK-002 for the treatment of autosomal dominant optic atrophy (ADOA), the most common inherited optic nerve disorder. Stoke's initial focus is haploinsufficiencies and diseases of the

central nervous system and the eye, although proof of concept has been demonstrated in other organs, tissues, and systems, supporting its belief in the broad potential for its proprietary approach. Stoke is headquartered in Bedford, Massachusetts with offices in Cambridge, Massachusetts. For more information, visit https://www.stoketherapeutics.com/ or follow Stoke on Twitter at @StokeTx.

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