

Stoke Therapeutics Presents Early Research on its Novel ASO Pipeline for Severe Genetic Diseases at OPT Boston

Bedford, Mass., March 26, 2018 — Stoke Therapeutics, Inc., a biotechnology company developing therapeutics that increase gene expression to treat a wide array of diseases caused by genetic insufficiency, will present research on its first-in-class medicines today at the Oligonucleotide & Peptide Therapeutics Boston Conference in Cambridge, Mass.

Charles R. Allerson, Ph.D., vice president of chemistry at Stoke Therapeutics, will present preclinical data on the company's pipeline of novel antisense oligonucleotide (ASO) therapeutics, which are designed to upregulate gene expression and treat an array of severe genetic diseases. As an example, Stoke has been able to increase productive *Scn1a* mRNA and resulting Na_v1.1 protein levels in mouse models without affecting closely related ion channels. Inherited deficiency of *Scn1a* gene dosage is known to cause Dravet Syndrome, a severe form of epilepsy. By directly and specifically increasing *Scn1a* gene expression, Stoke's program represents a potential first-in-class disease-modifying strategy to treat Dravet Syndrome.

"We're excited to share our early data as we work to develop a pipeline of innovative ASO therapeutics," Allerson said. "Many human genetic diseases are due to loss or reduction of function of a single gene, yet there are no drugs on the market to address the underlying genetic causes of most of these conditions. At Stoke, we're developing strategies to treat these monogenic diseases by directly increasing protein expression — without relying on viral or nanoparticle vectors."

Allerson's presentation, called "Targeted Augmentation of Nuclear Gene Output," will be held today at 4:45 p.m. For the full conference agenda, please see <http://optcongress.com/Oligonucleotide-Discovery/>

About Stoke Therapeutics

Stoke Therapeutics is a biotechnology company working to increase gene expression to treat a wide array of severe genetic diseases, including genetic conditions affecting the central nervous system, eye and liver. Stoke was launched in 2018 with a \$40 million Series A investment funded by Apple Tree Partners. For more information visit www.stoketherapeutics.com.

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