



## **Stoke Therapeutics to Present Research Supporting Its Novel Approach to Oligonucleotide Mediated Gene Up- Regulation at TIDES and ASGCT Conferences**

May 8, 2018

**Bedford, Mass., May 8** — Stoke Therapeutics, Inc., will present research at two upcoming conferences supporting its development of first-in-class therapeutics to treat monogenic diseases by leveraging antisense oligonucleotides to increase target protein expression.

Huw M. Nash, Ph.D., Stoke's chief operating officer and chief business officer, will present on antisense control of RNA splicing to treat genetic disease at the TIDES Oligonucleotide and Peptide Therapeutics conference in Boston. His presentation is scheduled for Thursday, May 10 from 11:15 a.m. to 11:45 a.m.

Gene Liau, Ph.D., Stoke's executive vice president and head of research and preclinical development, will present a poster describing Stoke's TANGO (Targeted Augmentation of Nuclear Gene Output) technology at the American Society of Gene and Cell Therapy annual meeting May 16-19 in Chicago. His poster (#304) will be presented during the Oligonucleotide Therapeutics I session on Wednesday, May 16 at 5:30 p.m. in Stevens Salon C, D.

Stoke is targeting a diverse set of monogenic diseases, with an initial focus on haploinsufficiencies that affect the central nervous system, eye and liver. One of the company's lead programs, which is moving into formal preclinical development, targets Dravet Syndrome, an epileptic encephalopathy characterized by severe and high frequency seizures and cognitive impairment. By addressing the root cause of these seizures – haploinsufficiency of the sodium channel Nav 1.1 – Stoke's investigational therapy aims to both prevent seizures and address developmental deficits. In the upcoming presentations, Stoke will present proof of target engagement and durable response in both wild-type and Dravet Syndrome model mice.

"TANGO enables us to precisely modulate splicing to increase a target protein's expression by exploiting naturally occurring non-productive splicing events," Dr. Liau said. "We've identified hundreds of genes associated with monogenic loss-of-function diseases that we may be able to up-regulate with our approach. As we move forward with preclinical development, we're excited to present some of our early research at these conferences."

### **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](http://www.stoketherapeutics.com) and follow Stoke on Twitter [@StokeTx](https://twitter.com/StokeTx).

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