

Stoke Therapeutics to Present on First Disease-Modifying Therapies for Genetic Epilepsies at Cowen and Company Annual Health Care Conference

BEDFORD, Mass., March 7, 2019 – Stoke Therapeutics will present next week at the Cowen and Company 39th Annual Health Care Conference in Boston on its work to develop the first gene-specific, disease-modifying therapies for treatment of Dravet syndrome and other severe genetic diseases.

Huw M. Nash, Ph.D., Stoke's chief operating officer and chief business officer, will present on Stoke's novel antisense oligonucleotide (ASO) medicines, which target pre-mRNA splicing to increase protein expression to near normal levels. Stoke's ASOs utilize the Company's proprietary technology platform, Targeted Augmentation of Nuclear Gene Output, or TANGO, and are designed to increase the expression of protein by individual genes in a patient.

Stoke is working to advance its lead product candidate for treatment of Dravet syndrome, a severe and progressive genetic epilepsy. Dravet syndrome affects approximately 35,000 patients across the U.S., Canada, Japan, Germany, France and the United Kingdom and is primarily caused by loss-of-function mutations in the *SCN1A* gene which encodes the Na_v1.1 protein. Dr. Nash will present preclinical data showing the impact of Stoke's TANGO technology on Na_v1.1 protein expression in mouse models.

Stoke is also developing ASO therapies for other genetic epilepsies, as well as genetic diseases of the central nervous system, eye, liver and kidney.

Stoke's presentation details:

Date: Monday, March 11, 2019

Time: 8:00 – 8:25 a.m. EST

Location: Boston Marriott Copley Place, MIT Conference Room, 3rd Floor

About Stoke Therapeutics

Launched in 2018, Stoke Therapeutics is pioneering a new way to treat the underlying causes of severe genetic diseases by precisely upregulating protein expression. Stoke is targeting a wide array of genetic disorders affecting the central nervous system, eye, liver and kidney. Its lead program is the potential first disease-modifying therapy for Dravet syndrome, a severe and progressive genetic epilepsy. To date, Stoke has raised \$130 million in funding from two rounds of financing; investors include Apple Tree Partners, RTW Investments, RA Capital Management, Cormorant Asset Management, Perceptive Advisors, funds managed by Janus Henderson Investors, Redmile Group, Sphera Funds Management and Alexandria Venture Investments.

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