

Stoke Therapeutics Announces \$40 Million Series A Financing to Create Pioneering New Medicines that Restore Gene Expression in Severe Genetic Disease

Edward M. Kaye, M.D., industry leader in rare disease drug development, named Chief Executive Officer

Company leveraging groundbreaking work on RNA splicing from Scientific Founder, Cold Spring Harbor Laboratory Professor Adrian Krainer, Ph.D.

Bedford, Mass., January 4, 2018 — Stoke Therapeutics, Inc, a new company working to increase gene expression to treat a wide array of diseases caused by genetic insufficiency, today announced that it has completed a \$40 million Series A financing to support the further advancement of multiple pre-clinical development programs. The Series A round was provided by founding investor Apple Tree Partners.

Edward M. Kaye, M.D., an industry leader in the development of gene-based medicines for rare diseases and most recently the Chief Executive Officer of Sarepta Therapeutics, has joined Stoke as Chief Executive Officer. He leads a deeply experienced management team with a track record of successfully translating novel biology into groundbreaking new therapies for patients.

Stoke is pioneering a unique therapeutic approach using antisense oligonucleotides to increase the expression of proteins whose function is reduced in genetic diseases. The company is focused specifically on modulating RNA splicing, a critical step in gene expression, to increase the production of messenger RNA that can be translated into protein.

“Stoke Therapeutics represents a bold step forward in opening up a vast new area of drug development focused on up-regulation of gene expression,” said Edward M. Kaye, Stoke Therapeutics Chief Executive Officer. “By restoring gene dosage using target-specific antisense approaches, we have the opportunity to create a new way of treating diseases that are not amenable to enzyme replacement, gene therapy or other existing modalities.”

The technological foundation of Stoke’s approach was developed in close collaboration with Scientific Founder Adrian Krainer, Ph.D., of Cold Spring Harbor Laboratory, an expert in RNA splicing and an inventor of the recently approved antisense therapy SPINRAZA® (nusinersen), a life-saving drug for children with Spinal Muscular Atrophy (SMA).

Since its inception, Stoke has validated hundreds of disease target genes that could be up-regulated with its proprietary TANGO (Targeted Augmentation of Nuclear Gene Output) platform and is prioritizing therapeutic programs that target antisense-addressable tissues including the central nervous system, eye, and liver. While Stoke’s initial focus is on inherited diseases caused by the reduced function of a single mutated gene, the TANGO approach is also applicable to diseases whereby targeted augmentation of a non-mutated gene could reverse or prevent disease progression.

“We are tremendously impressed by the broad potential of this approach to address so many debilitating diseases and have assembled the team, platform and funding to thoroughly exploit this opportunity,” said Sam Hall, Ph.D., Principal of Apple Tree Partners and member of the Stoke Board of Directors.

Stoke Therapeutics is led by a team with strong scientific and clinical experience in the identification and development of novel therapies for patients. In addition to Dr. Kaye, the company’s leadership includes:

- Huw M. Nash, Ph.D., is Chief Operating Officer and Chief Business Officer and was Founding CEO of Stoke; an Entrepreneur-in-Residence at Apple Tree Partners with 20 years' experience establishing, funding and growing start-up biotechnology companies.
- Barry Ticho, M.D., Ph.D., FACC, is Chief Medical Officer; he previously held senior positions at Moderna Therapeutics, Pfizer and
- Isabel Aznarez, Ph.D., is co-founder and VP, Head of Biology; she was previously a member of the Krainer lab and has extensive experience in human genetics, RNA metabolism, and modulation of RNA processes using antisense oligonucleotides.
- Charles R. Allerson, Ph.D., is VP, Head of Chemistry; he has extensive expertise in oligonucleotide therapeutics and previously held senior positions at Regulus Therapeutics and Ionis Pharmaceuticals.

In addition to Dr. Kaye, the company's Board of Directors includes:

- Adrian R. Krainer, Ph.D., is co-founder of Stoke and is the St Giles Professor of Molecular Genetics and Program Chair of Cancer & Molecular Biology at Cold Spring Harbor Laboratory.
- Arthur A. Levin, Ph.D., is currently Executive Vice President, R&D at Avidity Biosciences; previously he held that position at miRagen Therapeutics, and prior to that he held senior drug development roles at Ionis Pharmaceuticals and Santaris Pharma.
- Seth L. Harrison, M.D., is the founder and Managing Partner of Apple Tree Partners and is Chairman of the Board; he is currently Chairman of Braeburn Pharmaceuticals, Elstar Therapeutics, Limelight Bio, and Syntimmune, and is a Director of Corvidia Therapeutics.
- Sam Hall, Ph.D., is a Principal at Apple Tree Partners; he was previously a member of the investment team at Symphony Capital and prior to that a member of the healthcare investment banking team at Citigroup.

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.

About Apple Tree Partners

Apple Tree Partners (ATP) is a New York-based venture capital firm dedicated to building transformative life sciences businesses. The firm is actively investing its fourth fund, with \$1.5 billion in capital commitments. ATP considers therapeutics and medical device investments at all stages, from discovery research through to commercialization and takes a long-term view to create sustainable value. For more information visit www.appletreepartners.com.

About Cold Spring Harbor Laboratory

Founded in 1890, Cold Spring Harbor Laboratory has shaped contemporary biomedical research and education with programs in cancer, neuroscience, plant biology and quantitative biology. Home to eight Nobel Prize winners, the private, not-for-profit Laboratory employs 1,100 people including 600 scientists, students and technicians. For more information, visit www.cshl.edu.

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