# Stoke Therapeutics Reports Fourth Quarter and Full Year 2022 Financial Results and Provides Business Updates

March 6, 2023

*– Company expects to complete Phase 1/2a studies of STK-001 in children and adolescents with Dravet syndrome in 2023 in order to initiate a Phase 3 program in 2024 –*

*– Company on track to provide additional safety and seizure frequency data from patients who received multiple 45mg doses of STK-001 in mid-2023 –*

*– Data from multiple 70mg doses of STK-001 anticipated in the second half of 2023 –*

*– Strong cash position with runway extended to the end of 2025 –*

BEDFORD, Mass.--(BUSINESS WIRE)--Mar. 6, 2023-- 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, today reported financial results for the full year ended December 31, 2022 and provided business updates.

"In 2022 we continued to advance the first potential new medicine to treat the underlying cause of Dravet syndrome, generating data that showed that upregulating protein expression with STK-001 reduces seizure frequency and demonstrated an early trend toward improvement in some non-seizure aspects of this disease," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "These data support our belief that STK-001 represents a turning point in treatment of Dravet syndrome by addressing the syndrome, not just the seizures associated with this devastating disease. In the coming months, we look forward to sharing more clinical data from our studies of STK-001."

"By extending our cash runway to the end of 2025 with additional proceeds raised since December, Stoke is well-positioned to execute on our near-term priority of wrapping up the Phase 1/2a studies and generating the data necessary to advance STK-001 to a pivotal program in 2024," continued Kaye. "Beyond Dravet, we continue to make meaningful progress with our pipeline of first-in-class disease-modifying medicines for severe diseases including STK-002 for autosomal dominant optic atrophy, which we anticipate will enter the clinic next year as well as several other promising programs for diseases of the central nervous system and the eye."

**Fourth Quarter 2022 Business Highlights and Recent Developments**

- Today, the Company provided the following updates from the Phase 1/2a studies of STK-001 in children and adolescents with Dravet syndrome:
  - In the U.S., MONARCH enrollment is nearing completion. Dosing is ongoing in the expanded 45mg multiple dose cohort.
  - In the U.K., enrollment is now complete in ADMIRAL. Dosing is ongoing in the 70mg multiple dose cohort.
- In January, the Company submitted a Clinical Trial Application (CTA) in the U.K. for the planned Phase 1/2 study of STK-002 in patients with autosomal dominant optic atrophy (ADOA), the most common inherited optic nerve disorder.
- In the fourth quarter, the Company shared positive topline data from a combined interim analysis of six patients treated with three doses of 45mg in the Phase 1/2a MONARCH and ADMIRAL studies that showed:
  - Multiple doses of STK-001 up to 45mg were well tolerated.
  - A 55% median reduction from baseline in convulsive seizure frequency from Day 29 after the first dose to three months after receiving the last dose.
  - Reductions from baseline in convulsive seizure frequency in 5/6 (83%) patients.
  - A greater than 50% reduction in convulsive seizure frequency in 4/6 (67%) patients.
  - Reductions in seizure frequency began one month after the first dose and continued with additional treatment, consistent with the anticipated mechanism of action of STK-001.
- In the fourth quarter, the Company shared preliminary data from a small cohort of patients treated with multiple 30mg doses of STK-001 in SWALLOWTAIL, an open-label extension study, that showed:
  - Reductions in convulsive seizure frequency that were observed in MONARCH were maintained with ongoing treatment.
  - A trend toward improvement in non-seizure comorbidities as measured by the BRIEF-P, an assessment of executive function.

**Upcoming Anticipated Milestones**

- Data from patients treated with multiple 45mg doses of STK-001 is anticipated in mid-2023.
- Data from patients treated with multiple 70mg doses of STK-001 in the ADMIRAL study is anticipated in the second half of

2023.
- The Company expects to complete Phase 1/2a studies of STK-001 in 2023 in order to initiate a Phase 3 program in 2024.

**Year End 2022 Financial Results**

- Revenue recognized for upfront license fees and services provided from a License and Collaboration Agreement for the year ended December 31, 2022 was $12.4 million. There was no revenue in the year ended December 31, 2021.
- Net loss for the year ended December 31, 2022 was $101.1 million, or $2.60 per share compared to $85.8 million or $2.34 per share for 2021.
- Research and development expenses for the year ended December 31, 2022 were $77.8 million, compared to $54.2 million for 2021.
- General and administrative expenses for the year ended December 31, 2022 were $38.9 million, compared to $31.9 million for 2021.
- The increase in expenses for the year ended December 31, 2022 as compared to the same period in 2021 primarily relate to increases in costs associated with personnel, third party contracts, consulting, facilities and other costs associated with development activities for STK-001 and STK-002, research on additional therapeutics and growing a public corporation.
- As of December 31, 2022, Stoke had $230.2 million in cash, cash equivalents, marketable securities, and restricted cash. Stoke expects that these resources, together with the proceeds since December 31, 2022 from its Controlled Equity Offering Sales Agreement of $44.7 million, will be sufficient to fund its operations to the end of 2025.

**Fourth Quarter 2022 Financial Results**

- Revenue recognized for upfront license fees and services provided from a License and Collaboration Agreement for the three months ended December 31, 2022 was $3.3 million. There was no revenue in the same period in 2021.
- Net loss for the three months ended December 31, 2022 was $25.7 million, or $0.65 per share, compared to $24.4 million, or $0.66 per share, for the same period in 2021.
- Research and development expenses for the three months ended December 31, 2022 were $21.1 million, compared to $15.8 million for the same period in 2021.
- General and administrative expenses for the three months ended December 31, 2022 were $9.4 million, compared to $8.7 million for the same period in 2021.
- The increase in expenses for the three months ended December 31, 2022 as compared to the same period in 2021 primarily relate to increases in costs associated with personnel, third party contracts, consulting, facilities and other costs associated with development activities for STK-001 and STK-002, research on additional therapeutics and growing a public corporation.

**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, resulting in disease. 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 functional (or wild-type) genes produce more protein. TANGO aims to restore missing proteins by increasing – or stoking – protein output from healthy genes, thus compensating for the mutant copy of the gene.

**About Dravet Syndrome**

Dravet syndrome is a severe and progressive genetic epilepsy characterized by frequent, prolonged and refractory seizures, beginning within the first year of life. Dravet syndrome is difficult to treat and has a poor long-term prognosis. Complications of the disease often contribute to a poor quality of life for patients and their caregivers. The effects of the disease go beyond seizures and often include intellectual disability, developmental delays, movement and balance issues, language and speech disturbances, growth defects, sleep abnormalities, disruptions of the autonomic nervous system and mood disorders. The disease is classified as a developmental and epileptic encephalopathy due to the developmental delays and cognitive impairment associated with the disease. Compared with the general epilepsy population, people living with Dravet syndrome have a higher risk of sudden unexpected death in epilepsy, or SUDEP. There are no approved disease-modifying therapies for people living with Dravet syndrome. One out of 16,000 babies are born with Dravet syndrome, which is not concentrated in a particular geographic area or ethnic group.

**About STK-001**

STK-001 is an investigational new medicine for the treatment of Dravet syndrome currently being evaluated in ongoing clinical trials. Stoke believes that STK-001, a proprietary antisense oligonucleotide (ASO), has the potential to be the first disease-modifying therapy to address the genetic cause of Dravet syndrome. STK-001 is designed to upregulate NaV1.1 protein expression by leveraging the non-mutant (wild-type) copy of the *SCN1A* gene to restore physiological NaV1.1 levels, thereby reducing both occurrence of seizures and significant non-seizure comorbidities. STK-001 has been granted orphan drug designation by the FDA and the EMA, and rare pediatric disease designation by the FDA as a potential new treatment for Dravet syndrome.

**About Phase 1/2a MONARCH Study (United States)**

The MONARCH study is a Phase 1/2a open-label study of children and adolescents ages 2 to 18 who have an established diagnosis of Dravet syndrome and have evidence of a genetic mutation in the *SCN1A* gene. The primary objectives for the study are to assess the safety and tolerability of STK-001, as well as to determine the pharmacokinetics in plasma and exposure in cerebrospinal fluid. A secondary objective is to assess the efficacy

as an adjunctive antiepileptic treatment with respect to the percentage change from baseline in convulsive seizure frequency. Stoke also intends to measure non-seizure aspects of the disease, such as quality of life, as secondary endpoints. Additional information about the MONARCH study can be found at https://www.monarchstudy.com/.

Patients who participated in the MONARCH study and meet study entry criteria are eligible to continue treatment in SWALLOWTAIL, an open-label extension (OLE) study designed to evaluate the long-term safety and tolerability of repeat doses of STK-001. We expect that SWALLOWTAIL will also provide valuable information on the preliminary effects of STK-001 on seizures along with non-seizure aspects of the disease, such as quality of life and cognition.

Enrollment and dosing in SWALLOWTAIL are underway.

**About Phase 1/2a ADMIRAL Study (United Kingdom)**

The ADMIRAL study is a Phase 1/2a open-label study of children and adolescents ages 2 to <18 who have an established diagnosis of Dravet syndrome and have evidence of a genetic mutation in the *SCN1A* gene. The primary objectives for the study are to assess the safety and tolerability of multiple doses of STK-001, as well as to determine the pharmacokinetics in plasma and exposure in cerebrospinal fluid. A secondary objective is to assess the effect of multiple doses of STK-001 as an adjunctive antiepileptic treatment with respect to the percentage change from baseline in convulsive seizure frequency. Stoke also intends to measure non-seizure aspects of the disease, such as overall clinical status and quality of life, as secondary endpoints. Additional information about the ADMIRAL study can be found at https://www.admiralstudy.com.

Patients who participated in the ADMIRAL study and meet study entry criteria are eligible to continue treatment in LONGWING, an open-label extension (OLE) study designed to evaluate the long-term safety and tolerability of repeat doses of STK-001. We expect that LONGWING will also provide valuable information on the preliminary effects of STK-001 on seizures along with non-seizure aspects of the disease, such as quality of life and cognition.

Enrollment and dosing in LONGWING are underway.

**About Autosomal Dominant Optic Atrophy (ADOA)**

Autosomal dominant optic atrophy (ADOA) 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. 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.

**About STK-002**

STK-002 is a proprietary antisense oligonucleotide (ASO) in preclinical development for the treatment of Autosomal Dominant Optic Atrophy (ADOA). Approximately 80% of individuals with ADOA experience symptoms before age 10, typically beginning between the ages of 4 and 6. Stoke believes that STK-002 has the potential to be the first disease-modifying therapy for people living with ADOA. 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. 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 slow vision loss in patients with ADOA. Stoke has generated preclinical data demonstrating proof-of-mechanism and proof-of-concept for STK-002. STK-002 has been granted orphan drug designation by the FDA as a potential new treatment for ADOA.

**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.

**Cautionary Note Regarding Forward-Looking Statements**

This press release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to: the Company's year-end results and cash runway; its future operating results, financial position and liquidity; the ability of STK-001 to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in non-seizure comorbidities; the ability of STK-002 to treat the underlying causes of ADOA; the timing and expected progress of clinical trials, data readouts and presentations; the timing or receipt of regulatory approvals; the ability of TANGO to design medicines to increase protein production and the expected benefits thereof. Statements including words such as "plan," "will," "continue," "expect," or "ongoing" and statements in the future tense are forward-looking statements. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they prove incorrect or do not fully materialize, could cause our results to differ materially from those expressed or implied by such forward-looking statements, including, but not limited to, risks and uncertainties related to: the Company's ability to advance its product candidates, obtain regulatory approval of and ultimately commercialize its product candidates; the timing and results of preclinical and clinical trials; positive results in a clinical trial may not be replicated in subsequent trials or successes in early stage clinical trials may not be predictive of results in later stage trials; preliminary interim data readouts of ongoing trials may show results that change when such trials are completed; the Company's ability to fund development activities and achieve development goals to the end of 2025; the Company's ability to protect its intellectual property; the direct and indirect impacts of the ongoing COVID-19 pandemic and its variants on the Company's business; and other risks and uncertainties described under the heading "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2022, its quarterly reports on Form 10-Q, and the other documents the Company files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release, and the Company

undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

**Financial Tables Follow**

**Stoke Therapeutics, Inc.**
**Consolidated balance sheets**
**(in thousands, except share and per share amounts)**

| | As of December 31, | |
|---|---|---|
| | **2022** | **2021** |
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 113,556 | $ 144,895 |
| Marketable securities | 116,039 | 74,915 |
| Prepaid expenses | 10,932 | 6,943 |
| Other current assets | 2,955 | 2,216 |
| Deferred financing costs | — | 117 |
| Interest receivable | 588 | 132 |
| Total current assets | $ 244,070 | $ 229,218 |
| Restricted cash | 569 | 569 |
| Operating lease right-of-use assets | 4,753 | 4,939 |
| Property and equipment, net | 6,675 | 4,139 |
| Total assets | $ 256,067 | $ 238,865 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 766 | $ 2,385 |
| Accrued and other current liabilities | 15,748 | 14,754 |
| Deferred revenue - current portion | 14,880 | — |
| Total current liabilities | $ 31,394 | $ 17,139 |
| Deferred revenue - net of current portion | 36,856 | — |
| Other long term liabilities | 2,968 | 3,949 |
| Total long term liabilities | $ 39,824 | $ 3,949 |
| Total liabilities | $ 71,218 | $ 21,088 |
| Commitments and contingencies | | |
| Stockholders' equity | | |
| Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 39,439,575 and 36,902,499 shares issued and outstanding as of December 31, 2022 and 2021, respectively | 4 | 4 |
| Additional paid-in capital | 483,170 | 414,024 |
| Accumulated other comprehensive loss | (1,175) | (168) |
| Accumulated deficit | (297,150) | (196,083) |
| Total stockholders' equity | $ 184,849 | $ 217,777 |
| Total liabilities and stockholders' equity | $ 256,067 | $ 238,865 |

**Stoke Therapeutics, Inc.**
**Consolidated statements of operations and comprehensive loss**
**(in thousands, except share and per share amounts)**

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | **2022** | **2021** | **2022** | **2021** |
| Revenue | $ 3,269 | $ — | $ 12,405 | $ — |
| Operating expenses: | | | | |
| Research and development | 21,061 | 15,802 | 77,837 | 54,168 |
| General and administrative | 9,383 | 8,724 | 38,924 | 31,897 |
| Total operating expenses | 30,444 | 24,526 | 116,761 | 86,065 |
| Loss from operations | (27,175) | (24,526) | (104,356) | (86,065) |
| Other income (expense): | | | | |
| Interest income (expense), net | 1,479 | 36 | 3,122 | 120 |
| Other income (expense), net | 41 | 42 | 167 | 140 |
| Total other income (expense) | 1,520 | 78 | 3,289 | 260 |
| Net loss | $ (25,655) | $ (24,448) | $ (101,067) | $ (85,805) |

| | | | | |
|---|---|---|---|---|
| Net loss per share—basic and diluted | $ (0.65) | $ (0.66) | $ (2.60) | $ (2.34) |
| Weighted average common shares outstanding—basic and diluted | 39,434,027 | 36,836,072 | 38,897,442 | 36,739,269 |
| Comprehensive loss: | | | | |
| Net loss | $ (25,655) | $ (24,448) | $ (101,067) | $ (85,805) |
| Other comprehensive loss: | | | | |
| Unrealized gain (loss) on marketable securities | 528 | (145) | (1,007) | (168) |
| Total other comprehensive loss | $ 528 | $ (145) | $ (1,007) | $ (168) |
| Comprehensive loss | $ (25,127) | $ (24,593) | $ (102,074) | $ (85,973) |

View source version on businesswire.com: https://www.businesswire.com/news/home/20230306005219/en/

**Stoke Media & Investor Contacts:**
Dawn Kalmar
Chief Communications Officer
dkalmar@stoketherapeutics.com
781-303-8302

Eric Rojas
Vice President, Investor Relations
IR@stoketherapeutics.com
617-312-2754

Source: Stoke Therapeutics, Inc.