

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

**FORM 8-K**

**CURRENT REPORT  
Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): March 15, 2023**

**Stoke Therapeutics, Inc.**

(Exact Name of Registrant as Specified in its Charter)

**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**001-38938**  
(Commission  
File Number)

**47-1144582**  
(I.R.S. Employer  
Identification No.)

**45 Wiggins Ave**  
**Bedford, Massachusetts**  
(Address of principal executive offices)

**01730**  
(Zip Code)

**Registrant's telephone number, including area code: (781) 430-8200**

**Not Applicable**

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.0001 par value per share	STOK	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 7.01. Regulation FD.**

On March 15, 2023, Stoke Therapeutics, Inc., a Delaware corporation (the “Company”), issued a press release announcing the approval of a higher single dose of STK-001 (70mg) by the U.S. Food and Drug Administration (the “FDA”). A copy of the press release is attached hereto as Exhibit 99.1 to this report.

The information furnished under this Item 7.01, including Exhibit 99.1, shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference into any other filing under the Exchange Act or the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such a filing.

**Item 8.01 Other Events.**

On March 15, 2023, the Company announced that the FDA will allow the administration of a higher single dose of STK-001 (70mg) in its ongoing Phase 1/2a MONARCH study of children and adolescents with Dravet syndrome. This new 70mg single dose cohort in MONARCH will complement the ongoing evaluation of multiple doses of 70mg in the Company’s ADMIRAL study.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

<u>Exhibit Number</u>	<u>Description</u>
99.1	<a href="#">Press release issued by Stoke Therapeutics, Inc., dated March 15, 2023.</a>
104	Cover Page Interactive Data File (the cover page XBRL tags are embedded within the inline XBRL document).

**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**STOKE THERAPEUTICS, INC.**

Date: March 15, 2023

By: /s/ Stephen J. Tulipano

**Stephen J. Tulipano**  
**Chief Financial Officer**

Stoke Therapeutics Announces That FDA Will Allow the Administration of a Higher Single Dose of STK-001 (70mg) in the Ongoing Phase 1/2a MONARCH Study of Children and Adolescents with Dravet Syndrome

*– Dosing anticipated to start in the coming weeks –*

*– Company remains on track for its planned data readouts from the Phase 1/2a MONARCH and ADMIRAL studies in 2023 –*

**BEDFORD, Mass., March 15, 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 announced that the FDA will allow the administration of a higher single dose of STK-001 (70mg) in its ongoing Phase 1/2a MONARCH study of children and adolescents with Dravet syndrome. This new 70mg single dose cohort in MONARCH will complement the ongoing evaluation of multiple doses of 70mg in the ADMIRAL study.

Dravet syndrome is a severe and progressive genetic epilepsy characterized by frequent, prolonged and refractory seizures beginning within the first year of life. Complications of the disease often contribute to poor quality of life for patients and their caregivers. Despite available anti-seizure medicines, seizures are not adequately controlled for more than 90% of patients. STK-001 is a proprietary antisense oligonucleotide (ASO) being developed by Stoke as the first potential new medicine to address the genetic cause of Dravet syndrome.

“There is an urgent need for a disease modifying approach to treat Dravet syndrome, a devastating disease with effects that go beyond seizures and impact quality of life for patients and their families,” said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. “We thank the FDA for its timely review of the clinical data from our ongoing studies. We expect data from this new dose cohort to provide information about the potential effects and durability of a single dose of 70mg. We look forward to dosing the first patient in the coming weeks and to be able to include preliminary findings in our anticipated data readout in the second half of 2023.”

#### **Upcoming Anticipated Milestones**

- Data from patients treated with multiple 45mg doses of STK-001 are anticipated in mid-2023.
- Data from patients treated with 70mg of STK-001 in MONARCH (single dose) and ADMIRAL (multiple doses) are anticipated in the second half of 2023.
- The Company expects to complete the Phase 1/2a studies of STK-001 in 2023 in order to initiate a Phase 3 program in 2024.

The MONARCH study remains on partial clinical hold for multiple doses of STK-001 above 45mg.

## 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 the 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 the 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 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 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 ability of STK-001 to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in non-seizure comorbidities at the indicated dosing levels or at all, and the timing and expected progress of clinical trials, data readouts and presentations for STK-001. 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, obtain regulatory approval of, and ultimately commercialize its product candidates; the timing of data readouts and interim and final 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 into 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.