UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended June 30, 2024

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 001-38938

Stoke Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**47-1144582**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
45 Wiggins Ave Bedford, Massachusetts	**01730**
(Address of principal executive offices)	**(Zip Code)**

(781) 430-8200

(Registrant's telephone number, including area code)

Not applicable

(Former name, former address and former fiscal year, if changed since last report)

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 (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		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. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of July 31, 2024 the registrant had 52,647,799 shares of common stock, $0.0001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended (the "Securities Act") and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements other than statements of present and historical facts contained in this Quarterly Report on Form 10-Q, including, but not limited to, statements regarding the ability of zorevunersen (STK-001) to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in behavior or cognition at the indicated dosing levels or at all, the timing and expected progress of clinical trials, our future results of operations and financial position, business strategy, prospective products, planned preclinical studies and clinical or field trials, regulatory approvals, research and development costs, and timing and likelihood of success, as well as plans and objectives of management for future operations, may be forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words.

Forward-looking statements are based on our management's beliefs and assumptions and on information currently available to us. Such statements are subject to a number of known and unknown risks, uncertainties and assumptions, and actual results may differ materially from those expressed or implied in the forward-looking statements due to various factors, including, but not limited to, those identified in Part I. Item 2. "Management's Discussion and Analysis of Financial Condition and Results of Operations" and Part II. Item 1A "Risk Factors." These risks and uncertainties include, but are not limited to:

- our ability to become profitable;
- our ability to procure sufficient funding;
- our limited operating history;
- the direct and indirect impact of inflation, interest rates, foreign currency exchange rates, instability in the global banking system, geopolitical conflict and macroeconomic conditions, including as a result of a potential temporary federal government shutdown, on our business, financial condition and operations, including on our expenses, supply chain, strategic partners, research and development costs, clinical trials and employees;
- our ability to develop, obtain regulatory approval for and commercialize zorevunersen, STK-002 and our future product candidates;
- our success in early preclinical studies or clinical trials, which may not be indicative of results obtained in later studies or trials;
- the success of our collaboration with Acadia Pharmaceuticals and our ability to enter into successful collaborations in the future;
- the availability of coverage and adequate reimbursement from third party payors for zorevunersen, STK-002 and our future product candidates, if such products are approved;
- our ability to identify patients with the diseases treated by zorevunersen, STK-002 or our future product candidates, and to enroll patients in trials;
- the success of our efforts to use TANGO (Targeted Augmentation of Nuclear Gene Output) to expand our pipeline of product candidates and develop marketable products;
- our ability to obtain, maintain and protect our intellectual property;
- our reliance upon intellectual property licensed from third parties;
- our ability to identify, recruit and retain key personnel;
- our financial performance; and
- developments or projections relating to our competitors or our industry.

You should read this Quarterly Report on Form 10-Q and the documents that we reference herein completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

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

	June 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 193,476	$ 191,442
Marketable securities	88,506	9,952
Prepaid expenses	10,345	11,320
Restricted cash - short-term	75	—
Interest receivable	305	64
Other current assets	4,287	2,561
Total current assets	$ 296,994	$ 215,339
Restricted cash - long-term	494	569
Operating lease right-of-use assets	5,499	6,611
Property and equipment, net	4,770	5,823
Total Assets	$ 307,757	$ 228,342
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,945	$ 1,695
Accrued and other current liabilities	14,500	13,815
Deferred revenue - current portion	26,051	15,309
Total current liabilities	$ 44,496	$ 30,819
Deferred revenue - net of current portion	16,946	33,074
Other long term liabilities	3,606	4,884
Total long term liabilities	20,552	37,958
Total liabilities	$ 65,048	$ 68,777
Commitments and contingencies (Note 6)		
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 52,305,641 and 45,918,233 shares issued and outstanding as of June 30, 2024 and December 31, 2023, respectively	5	5
Additional paid-in capital	696,637	561,433
Accumulated other comprehensive loss	(15)	(24)
Accumulated deficit	(453,918)	(401,849)
Total stockholders' equity	$ 242,709	$ 159,565
Total liabilities and stockholders' equity	$ 307,757	$ 228,342

The accompanying notes are an integral part of these unaudited consolidated financial statements.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Revenue	$ 4,831	$ (2,481)	$ 9,048	$ 2,671
Operating expenses:				
Research and development	21,136	20,551	43,504	40,182
General and administrative	13,037	10,230	23,258	20,442
Total operating expenses	34,173	30,781	66,762	60,624
Loss from operations	(29,342)	(33,262)	(57,714)	(57,953)
Other income (expense):				
Interest income (expense), net	3,695	2,567	6,121	4,670
Other income (expense), net	(48)	41	(476)	84
Total other income (expense)	3,647	2,608	5,645	4,754
Net loss	$ (25,695)	$ (30,654)	$ (52,069)	$ (53,199)
Net loss per share, basic and diluted	$ (0.46)	$ (0.69)	$ (1.02)	$ (1.23)
Weighted-average common shares outstanding, basic and diluted	55,765,948	44,188,464	51,288,222	43,367,032
Comprehensive loss:				
Net loss	$ (25,695)	$ (30,654)	$ (52,069)	$ (53,199)
Other comprehensive gain (loss):				
Unrealized gain (loss) on marketable securities	(15)	219	9	796
Total other comprehensive gain (loss)	$ (15)	$ 219	$ 9	$ 796
Comprehensive loss	$ (25,710)	$ (30,435)	$ (52,060)	$ (52,403)

The accompanying notes are an integral part of these unaudited consolidated financial statements.

Stoke Therapeutics, Inc. and subsidiary
Consolidated statements of stockholders' equity
(in thousands, except share and per share amounts)
(unaudited)

	Common Stock		Additional paid-in capital	Accumulated other comprehensive gain (loss)	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance as of December 31, 2022	**39,439,575**	**$ 4**	**$ 483,170**	**$ (1,175)**	**$ (297,150)**	**$ 184,849**
Net loss	—	—	—	—	(22,545)	(22,545)
Unrealized gain on marketable securities	—	—	—	577	—	577
Stock-based compensation	—	—	5,887	—	—	5,887
Issuance of common stock upon exercise of stock options	80,611	—	158	—	—	158
Issuance of common stock related to employee stock purchase plan	19,550	—	153	—	—	153
Shares sold as part of controlled equity offering sales agreement	4,635,353	—	44,743	—	—	44,743
Balance as of March 31, 2023	**44,175,089**	**$ 4**	**$ 534,111**	**$ (598)**	**$ (319,695)**	**$ 213,822**
Net loss	—	—	—	—	(30,654)	(30,654)
Unrealized gain on marketable securities	—	—	—	219	—	219
Stock-based compensation	—	—	6,760	—	—	6,760
Issuance of common stock upon exercise of stock options	27,908	—	48	—	—	48
Balance as of June 30, 2023	**44,202,997**	**$ 4**	**$ 540,919**	**$ (379)**	**$ (350,349)**	**$ 190,195**
Balance as of December 31, 2023	**45,918,233**	**$ 5**	**$ 561,433**	**$ (24)**	**$ (401,849)**	**$ 159,565**
Net loss	—	—	—	—	(26,374)	(26,374)
Unrealized gain on marketable securities	—	—	—	24	—	24
Stock-based compensation	—	—	5,410	—	—	5,410
Issuance of common stock upon exercise of stock options	270,032	—	250	—	—	250
Issuance of common stock related to employee stock purchase plan	37,542	—	168	—	—	168
Shares sold as part of controlled equity offering sales agreement	272,270	—	1,299	—	—	1,299
Balance as of March 31, 2024	**46,498,077**	**$ 5**	**$ 568,560**	**$ —**	**$ (428,223)**	**$ 140,342**
Net loss	—	—	—	—	(25,695)	(25,695)
Unrealized gain on marketable securities	—	—	—	(15)	—	(15)
Stock-based compensation	—	—	7,427	—	—	7,427
Issuance of common stock upon exercise of stock options	252,007	—	766	—	—	766
Issuance of common stock and warrants upon follow-on offering, net of underwriting discounts, commissions and offering costs	5,555,557	—	119,884	—	—	119,884
Balance as of June 30, 2024	**52,305,641**	**$ 5**	**$ 696,637**	**$ (15)**	**$ (453,918)**	**$ 242,709**

The accompanying notes are an integral part of these unaudited consolidated financial statements.

Stoke Therapeutics, Inc. and subsidiary
Consolidated statements of cash flows
(in thousands)
(unaudited)

	Six Months Ended June 30,	
	2024	2023
Cash flows from operating activities:		
Net loss	$ (52,069)	$ (53,199)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	1,111	1,208
Amortization and accretion of marketable securities	(313)	(114)
Stock-based compensation	12,837	12,647
Reduction in the carrying amount of right of use assets	1,112	1,106
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(993)	(310)
Accounts payable, accrued liabilities and lease liabilities	1,658	(4,076)
Deferred revenue	(5,386)	(418)
Net cash used in operating activities	(42,043)	(43,156)
Cash flows from investing activities:		
Purchases of marketable securities	(88,232)	—
Purchases of property and equipment	(58)	(1,004)
Sales of marketable securities	10,000	77,562
Net cash (used in) provided by investing activities	(78,290)	76,558
Cash flows from financing activities:		
Proceeds from Employee Stock Purchase Plan	168	206
Proceeds from issuance of common stock upon exercise of stock options	1,016	153
Proceeds from issuance of common stock in controlled equity offering sales agreements	1,299	44,743
Proceeds from follow-on offering, net of underwriting discounts, commissions and offering costs	119,884	—
Net cash provided by financing activities	122,367	45,102
Net increase in cash, cash equivalents and restricted cash	2,034	78,504
Cash, cash equivalents and restricted cash—beginning of period	192,011	114,125
Cash, cash equivalents and restricted cash—end of period	$ 194,045	$ 192,629

The accompanying notes are an integral part of these unaudited consolidated financial statements.

1. Nature of the business

Organization

Stoke Therapeutics, Inc. (the "Company") was founded in June 2014 and was incorporated under the laws of the State of Delaware. The Company is a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine.

Shelf Registration

In May 2022, the Company filed a universal Shelf Registration statement on Form S-3 (the "Registration Statement") with the SEC. The Registration Statement was declared effective by the SEC on May 31, 2022, and contains two prospectuses: a base prospectus, which covers the offering, issuance and sale by the Company of up to a maximum aggregate offering price of $400.0 million of its common stock, preferred stock, debt securities, warrants to purchase common stock, preferred stock or debt securities, subscription rights to purchase common stock, preferred stock or debt securities and/or units consisting of some or all of these securities; and a sales agreement prospectus covering the offering, issuance and sale by the Company of up to a maximum aggregate offering price of $150.0 million of its common stock that may be issued and sold under a Controlled Equity Offering Sales Agreement ("Sales Agreement"). The specific terms of any securities to be offered pursuant to the base prospectus will be specified in a prospectus supplement to the base prospectus. The $150.0 million of common stock that may be offered, issued and sold under the sales agreement prospectus is included in the $400.0 million of securities that may be offered, issued and sold by the Company under the base prospectus. As of June 30, 2024, the Company had issued approximately 6.5 million shares of common stock pursuant to the Sales Agreement for net proceeds of $53.4 million. Since June 30, 2024, the Company has issued approximately 0.3 million shares of common stock pursuant to the Sales Agreement for net proceeds of $4.0 million. The Company may terminate this at-the-market program at any time, pursuant to its terms. On April 2, 2024, the Company completed an underwritten public offering of its common stock and issued and sold 5,555,557 shares of common stock at a public offering price of $13.50 per share and issued pre-funded warrants to purchase 3,703,730 shares of common stock at a public offering price of $13.499 per share subject to an exercise price equal to $0.0001. The common stock and pre-funded warrants sold resulted in net proceeds of $119.9 million after deducting underwriting discounts, commissions and offering costs.

Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Liquidity

The Company expects that its operating losses and negative cash flows will continue for the foreseeable future. As of the issuance date of these unaudited consolidated financial statements, the Company expects that its cash, cash equivalents and marketable securities will be sufficient to fund its operating expenses and capital expenditure requirements through at least twelve months from the issuance date of these unaudited consolidated financial statements.

2. Summary of significant accounting policies and recent accounting pronouncements

Basis of presentation and consolidation

The accompanying unaudited consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP") and include the accounts of the Company and its wholly-owned subsidiary. Any reference in these notes to applicable guidance is meant to refer to GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB"). All intercompany transactions between and among the Company and its consolidated subsidiary have been eliminated.

Unaudited interim financial information

The accompanying interim unaudited consolidated financial statements and related disclosures are unaudited and have been prepared in accordance with GAAP for interim financial information and the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all the information and footnotes required by GAAP for complete financial statements and should be read in conjunction with the Company's consolidated financial statements and related notes as of and for the year ended December 31, 2023, which was filed with the SEC on March 25, 2024. The Company's financial information as of June 30, 2024 and for the three and six months ended June 30, 2024 and 2023 is unaudited, but in the opinion of management, all adjustments, consisting only of normal recurring adjustments, considered necessary for a fair presentation of the financial position, results of operations and cash flows at the dates and for the periods presented of the results of these interim periods have been included. The balance sheet information as of December 31, 2023 was derived from audited consolidated financial statements. The results of the Company's operations for any interim period are not necessarily indicative of the results that may be expected for any other interim period or for a full fiscal year.

Use of estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenue, expenses and disclosure of contingent assets and liabilities. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Actual results could differ from those estimates.

Cash, cash equivalents and restricted cash

The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents. The Company deposits its cash in checking, sweep and money market accounts.

At June 30, 2024, restricted cash consisted of money market accounts collateralizing letters of credit issued as security deposits in connection with the Company's leases of its corporate facilities.

Cash and cash equivalents, and restricted cash in the consolidated statements of cash flows consists of the following (in thousands):

	As of June 30,	
	2024	2023
Cash and cash equivalents	$ 193,476	$ 192,060
Restricted cash - short-term	75	—
Restricted cash - long-term	494	569
Total cash, cash equivalents and restricted cash	$ 194,045	$ 192,629

Emerging growth company and smaller reporting company status

The Company is an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act, until such time as those standards apply to private companies.

The Company has elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that it is (i) no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, the Company's consolidated financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

The Company will remain an emerging growth company until the earliest of (i) the last day of the first fiscal year (a) following the fifth anniversary of the completion of the Company's IPO, (b) in which the Company has total annual gross revenue of at least $1.235 billion or (c) in which the Company is deemed to be a large accelerated filer, which means the market value of the Company's common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th, and (ii) the date on which the Company has issued more than $1.0 billion in non-convertible debt securities during the prior three- year period. The Company anticipates ceasing to be an emerging growth company as of December 31, 2024, which is the last day of the fiscal year following the fifth anniversary of the completion of the Company's IPO.

The Company is also a "smaller reporting company," meaning that in the event of an IPO the market value of its stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to the Company as a result of such offering is less than $700.0 million and its annual revenue is less than $100.0 million during the most recently completed fiscal year. The Company may continue to be a

smaller reporting company as long as either (i) the market value of its stock held by non-affiliates is less than $250.0 million or (ii) its annual revenue is less than $100.0 million during the most recently completed fiscal year and the market value of its stock held by non-affiliates is less than $700.0 million. If the Company is a smaller reporting company at the time it ceases to be an emerging growth company, the Company may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, the Company may choose to present only the two most recent fiscal years of audited financial statements in its Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

3. Fair value measurements

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values (in thousands):

	Fair value measurements as of June 30, 2024			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 186,819	$ —	$ —	$ 186,819
Total	$ 186,819	$ —	$ —	$ 186,819
Marketable Securities:				
US Government debt securities	$ —	$ 88,506	$ —	$ 88,506
Total	$ —	$ 88,506	$ —	$ 88,506

	Fair value measurements as of December 31, 2023			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 186,186	$ —	$ —	$ 186,186
Total	$ 186,186	$ —	$ —	$ 186,186
Marketable Securities:				
US Government debt securities	$ —	$ 9,952	$ —	$ 9,952
Total	$ —	$ 9,952	$ —	$ 9,952

The Company's cash equivalents and marketable securities are carried at fair value, determined according to the fair value hierarchy. The carrying value of the Company's accounts payable and accrued expenses approximate their fair values due to the short-term nature of these liabilities.

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds. Money market funds are publicly traded mutual funds and are presented as cash equivalents on the consolidated balance sheets as of June 30, 2024 and December 31, 2023.

The Company measures its marketable securities at fair value on a recurring basis and classifies those instruments within Level 2 of the fair value hierarchy. Marketable securities are valued using models or other valuation methodologies that use Level 2 inputs. These models are primarily industry-standard models that consider various assumptions, including time value, yield curve, volatility factors, default rates, current market and contractual prices for the underlying financial instruments, as well as other economic measures. Substantially all of these assumptions are observable in the marketplace, can be derived from observable data or are supported by observable levels at which transactions are executed in the marketplace.

There were no transfers to Level 3 in the periods presented.

4. Marketable securities

The following table summarizes the Company's marketable securities as of June 30, 2024 (in thousands):

	June 30, 2024			
	Amortized Cost	Unrealized Gains	Unrealized Loss	Fair Value
Marketable securities:				
US Government debt securities	$ 88,521	$ —	$ (15)	$ 88,506
Total	$ 88,521	$ —	$ (15)	$ 88,506

The following table summarizes the Company's marketable securities as of December 31, 2023 (in thousands):

	December 31, 2023			
	Amortized Cost	Unrealized Gains	Unrealized Loss	Fair Value
Marketable securities:				
US Government debt securities	$ 9,976	$ —	$ (24)	$ 9,952
Total	$ 9,976	$ —	$ (24)	$ 9,952

As of June 30, 2024, the weighted average maturity of the Company's marketable securities ranges from approximately 0.13 years to 0.83 years.

The Company did not record an allowance for credit losses as of June 30, 2024 related to its marketable securities. Further, given the lack of significant change in the credit risk of these investments, the Company did not recognize any other-than-temporary impairment losses.

5. Accrued and other current liabilities

Accrued and other current liabilities consisted of the following (in thousands):

	June 30, 2024	December 31, 2023
Accrued employee compensation costs	$ 3,540	$ 5,611
Accrued professional costs	2,149	651
Accrued research and development costs	5,497	4,634
Current portion of operating lease liabilities	2,277	2,062
Other current liabilities	1,037	857
	$ 14,500	$ 13,815

6. Commitments and contingencies

Operating lease

The Company determines whether an arrangement is a lease at inception. The Company accounts for a lease when it has the right to control the leased asset for a period of time while obtaining substantially all of the assets' economic benefits. Operating lease right-of-use assets and operating lease liabilities are recognized based on the present value of the future minimum lease payments over the lease term at the lease commencement date. The discount rate used to determine the present value of the lease payments is the Company's incremental borrowing rate based on the information available at lease inception, as the Company did not have information to determine the rate implicit in the leases. Lease expense for operating leases is recognized on a straight-line basis over the reasonably assured lease term based on the total lease payments (which include initial direct costs and lease incentives). The expense is included in operating expenses in the consolidated statements of operations and comprehensive loss. The Company's lease agreements also contain variable payments, primarily maintenance-related costs, which are expensed as incurred and not included in the measurement of the right-of-use assets and lease liabilities.

In August 2018, the Company entered into an agreement to lease approximately 23,000 square feet of space for a term of three years. Lease terms are triple net lease commencing at $0.9 million per year, then with 3% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The lease commencement date was December 10, 2018.

In September 2021, the Company entered into an agreement to extend the initial term of the 23,000 square foot lease for a period of three years commencing on December 15, 2021 and ending December 31, 2024. In addition, this lease provides for the lease of an additional 15,000 square feet of rentable space beginning on April 1, 2022 and ending on December 31, 2024. In December 2021, the

Company recognized a right-of-use asset and operating lease liability of $3.5 million for the 23,000 square feet. On April 1, 2022, the Company recognized a right-of-use asset and operating lease liability of $1.8 million for the 15,000 square feet.

In December 2023, the Company entered into an agreement to extend the term of the 38,000 square foot lease for a period of two years commencing on January 1, 2025 and ending on December 31, 2026. In December 2023, the Company recognized a right-of-use asset and operating lease liability of $4.1 million.

In December 2018, the Company entered into an agreement to lease 2,485 square feet of space for an initial term of three years. The lease includes one renewal option for an additional two years; however, any time after the initial term the landlord may relocate the Company from the premises to a space reasonably comparable in size and utility. As the Company does not have the right to control the use of the identified asset after the initial term, the renewal option was excluded from the lease liability calculation. Lease terms commence at $0.2 million per annum, with 2.5% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The lease commencement date was May 1, 2019.

In June 2021, the Company amended the agreement to extend the initial term of the 2,485 square foot lease for a period of three years commencing May 1, 2022 and ending April 30, 2025. In addition, the amendment provided for the lease of an additional 2,357 square feet of rentable space beginning on July 6, 2021 and ending on April 30, 2025. The amended lease provides the Company with the option to extend the term of the lease for an additional two years. In 2021, the Company recognized a right-of-use asset and operating lease liabilities of $0.7 million for the extension of the lease to April 30, 2025 and a right-of-use asset and operating lease liabilities of $0.8 million for the additional 2,357 square feet of rentable space.

Future minimum lease payments under non-cancellable leases as of June 30, 2024 were as follows (in thousands):

2024	$	1,307
2025		2,661
2026		2,564
Total lease payments		6,532
Less imputed interest		(704)
Present value of lease liabilities	$	5,828

Lease balances as of June 30, 2024 were as follows (in thousands):

Operating right-of-use assets	$	5,499
Current Portion of operating lease liabilities	$	2,277
Non-current portion of operating lease liabilities		3,551
Total operating lease liabilities	$	5,828

The weighted average remaining lease term and weighted average discount rate of the Company's operating leases as of June 30, 2024 were as follows:

Weighted average remaining lease term in years	2.4
Weighted average discount rate	10.03%

Lease expense incurred under operating leases was $0.7 million for the three months ended June 30, 2024and was $0.7 million for the three months ended June 30, 2023. Lease expense incurred under operating leases was $1.4 million for the six months ended June 30, 2024 and was $1.2 million for the six months ended June 30, 2023.

License and research agreements

In April 2016, the Company entered into an exclusive, worldwide license agreement with the University of Southampton (the "Southampton Agreement"), whereby the Company acquired rights to foundational technologies related to the Company's TANGO technology. Under the Southampton Agreement, the Company receives an exclusive, worldwide license under certain licensed patents and applications relating to TANGO. Under the Southampton Agreement, the Company may be obligated to make additional payments that are contingent upon certain milestones being achieved, as well as royalties on future product sales. These royalty obligations survive until the latest of (i) the expiration of the last valid claim of a licensed patent covering a subject product or (ii) the expiration of any regulatory exclusivity for the subject product in a country. In addition, if the Company sublicenses its rights under the Southampton Agreement, the Company is required to pay a mid-single digit percentage of the sublicense revenue to the University of Southampton. As of June 30, 2024, the Company had paid $0.70 million under the Southampton Agreement as a result of entering into the Acadia Pharmaceuticals Inc. license and collaboration agreement in January 2022 (see Note 7). Additionally, certain licenses under the Southampton Agreement require the Company to reimburse the University of Southampton for certain past and ongoing patent related expenses. For the three months ended June 30, 2024 these expenses were $0.04 million compared to $0.10 million for

the three months ended June 30, 2023, and for the six months ended June 30, 2024 these expenses were $0.07 million compared to $0.14 million for the six months ended June 30, 2023.

7. License and collaboration agreement with Acadia Pharmaceuticals Inc.

In January 2022, the Company entered into a license and collaboration agreement with Acadia Pharmaceuticals Inc. (“Acadia”) for the discovery, development and commercialization of novel RNA-based medicines for the treatment of severe and rare genetic neurodevelopmental diseases of the central nervous system. The agreement focuses on the targets SYNGAP1, MECP2 (Rett syndrome), and an undisclosed neurodevelopmental target of mutual interest. In connection with each target, the Company will collaborate with Acadia to identify potential treatments for further development and commercialization as licensed products. With respect to SYNGAP1, the Company has agreed with Acadia to co-develop and co-commercialize licensed products for such target globally, and in connection therewith the Company granted to Acadia worldwide, co-exclusive (with the Company) licenses for such licensed products. With respect to MECP2 and the neurodevelopmental target, the Company granted to Acadia worldwide, exclusive licenses to develop and commercialize licensed products for such targets.

Pursuant to the terms of the agreement, the Company received an upfront payment of $60.0 million from Acadia. Acadia agreed to fund the research to identify potential licensed products for MECP2 and the neurodevelopmental target, and the Company will equally fund with Acadia the research to identify potential licensed products for SYNGAP1. The Company is eligible to receive up to $907.5 million in potential total milestone payments based upon the achievement of certain development, regulatory, first commercial sales and sales milestone events across the programs for the three targets, assuming each milestone were achieved at least once. With respect to licensed products for MECP2 and the neurodevelopmental target, the Company is also eligible to receive tiered royalties at percentages ranging from the mid-single digits to the mid-teens on future net sales by Acadia of licensed products worldwide. Royalties payable under the agreement are subject to standard royalty reductions. For SYNGAP1 licensed products that the Company is co-developing and co-commercializing, the Company will be responsible for 50% of the development and commercialization costs and will receive 50% of the profits from global commercialization. The Company is provided with a co-development and co-commercialization opt out option relating to the SYNGAP1 target indication at the Company’s discretion. Such opt-out would reduce development and commercialization milestones but would provide the Company with royalties on an escalating basis attributable to net sales milestones.

Acadia agreement accounting

At the commencement of the Acadia agreement, the Company identified three performance obligations consisting of pre-clinical research activities for each of the three targets, SYNGAP1, MECP2, and the undisclosed neurodevelopmental target. The exclusive or co-exclusive licenses granted to Acadia to conduct pre-clinical research activities on each of the three targets, and participation on each of the respective joint research committees were identified as promised services. However, the licenses granted to Acadia and the research activities were determined to be not distinct from each other, and therefore are considered a combined performance obligation for each of the three targets. Participation on each of the joint research committees was determined to be quantitatively and qualitatively immaterial in the context of the arrangement with Acadia.

The Company is recognizing the transaction price for the pre-clinical research activities for each of the three targets over time as the research services are provided. The transfer of control to Acadia occurs over this time period, and in management’s judgment, is the best measure of progress towards satisfying the performance obligation. An input method is used that measures the cost incurred to date in satisfying each of the three research activities in relation to the estimated total projected cost of each of the research activities to fulfill the respective obligations. The cumulative effect of revisions to estimated costs and/or the transaction price to complete the research performance obligations will be recorded in the period in which changes are identified and amounts can be reasonably estimated.

Milestone payments that are not within the control of the Company, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. For other milestones, the Company evaluated factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone in making this assessment. Milestones that are outside of the Company’s or Acadia’s control will not be recognized until such milestones are achieved. As to the other milestones, to date, no milestone payments have been included in the transaction price due to the uncertainty as to whether these milestones will be achieved. The Company will at the end of each reporting period reevaluate the probability of achievement of all milestones subject to constraint and, if necessary, adjust its estimate of the overall transaction price for each of the research activities on the three targets. Any such adjustments will be recorded on a cumulative catch-up basis.

As of June 30, 2024, the Company had $43.0 million in upfront consideration associated with the Acadia agreement relating to performance obligations that are unsatisfied or partially unsatisfied.

8. Equity incentive plans

In June 2019, the Company's board of directors and stockholders approved the 2019 Equity Incentive Plan (the "2019 Plan") which became effective on June 17, 2019 and replaced the Company's 2014 Equity Incentive Plan (the "2014 Plan"). In addition to the shares of common stock reserved for future issuance under the 2014 Plan that were added to the 2019 Plan upon its effective date, the Company initially reserved 2,200,000 shares of common stock for issuance under the 2019 Plan. The number of shares reserved for issuance under the 2019 Plan will increase automatically on January 1 of each of 2020 through 2029 by the number of shares equal to 4% of the aggregate number of outstanding shares of the Company's common stock as of the immediately preceding December 31, or a lesser number as may be determined by the Company's board of directors.

In April 2023, the Company's board of directors adopted the Stoke Therapeutics, Inc. 2023 Inducement Plan (the "2023 Plan"). As permitted by Nasdaq stock market rules, the Company's stockholders were not required to approve the 2023 Plan. The 2023 Plan provides for up to 1,000,000 shares of the Company's common stock under awards granted to newly hired employees. An "award" is any right to receive common stock of the Company through nonstatutory stock options or restricted stock units ("RSUs").

As of June 30, 2024, there were no shares available for future issuance under the 2014 Plan, 2,362,405 shares were available under the 2019 Plan and 190,400 shares were available under the 2023 Plan.

During the three months ended June 30, 2024, the Company granted options to purchase 897,712 shares of common stock to certain of its employees. The options vest over a period of up to four years and are exercisable at a per share price equal to the fair value of the common stock on the grant date. During the three months ended June 30, 2024, the Company did not grant RSUs or performance stock units ("PSUs") to its employees.

Stock-based compensation

As of June 30, 2024, there was $30.9 million of unrecognized compensation cost related to unvested stock options granted under the 2019 and 2023 Plans. Compensation expense is expected to be recognized over a weighted average period of 3.0 years as of June 30, 2024. As of June 30, 2024, there was $29.3 million of unrecognized stock-based compensation related to RSUs and is expected to be recognized over a weighted average period of 2.4 years. As of June 30, 2024 there was $4.2 million of unrecognized stock-based compensation related to PSUs.

Stock-based compensation expense recorded as research and development and general and administrative expenses in the accompanying consolidated statements of operations and comprehensive loss is as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Research and development	$ 2,924	$ 2,776	$ 5,015	$ 5,031
General and administrative	4,503	3,984	7,822	7,616
Total	$ 7,427	$ 6,760	$ 12,837	$ 12,647

2019 Employee stock purchase plan

In June 2019, the Company adopted the 2019 Employee Stock Purchase Plan (the "ESPP"), which became effective on June 18, 2019. The Company initially reserved 315,000 shares of common stock for sale under the ESPP. At June 30, 2024, the Company had 1,631,069 shares available for issuance under the ESPP. The average grant date fair value per share under the ESPP was $5.86 for 2024. The total ESPP stock-based compensation expense for the three and six months ended June 30, 2024 was $0.1 million and $0.2 million, respectively, and for the three and six months ended June 30, 2023 was $0.1 million and $0.2 million, respectively. The number of shares reserved for issuance under the ESPP will increase automatically on January 1 of each of the first ten calendar years following the first offering date by the number of shares equal to the lesser of 1% of the total outstanding shares of the Company's common stock as of the immediately preceding December 31 or a lower amount determined by the Company's board of directors. The aggregate number of shares issued over the term of the ESPP will not exceed 3,150,000 shares of the Company's common stock.

9. Net loss per share

The following table summarizes the computation of basic and diluted net loss per share of the Company (in thousands except share and per share amounts):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Numerator:				
Net loss	$ (25,695)	$ (30,654)	$ (52,069)	$ (53,199)
Denominator:				
Weighted-average number of common shares, basic and diluted	55,765,948	44,188,464	51,288,222	43,367,032
Net loss per share, basic and diluted	$ (0.46)	$ (0.69)	$ (1.02)	$ (1.23)

The Company has included the outstanding pre-funded warrants from the April 2024 offering in the number of total outstanding shares used for the computation of basic and diluted net loss per share for the period ending June 30, 2024, since they have a de minimis exercise price. The Company's potential dilutive securities, which include common stock options, RSUs, PSUs, and ESPP purchase rights, have been excluded from the computation of diluted net loss per share as the effect would be anti-dilutive. Therefore, the weighted average number of shares of the Company's common stock outstanding, including warrants, used to calculate both basic and diluted net loss per share is the same.

The Company excluded the following potential common shares, presented based on amounts outstanding at period end, from the computation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	June 30,	
	2024	2023
Outstanding options to purchase common stock	8,064,604	9,441,904
Restricted stock units	2,390,957	783,600
Total	10,455,561	10,225,504

10. Income taxes

The Company did not record an income tax benefit in its consolidated statements of operations and comprehensive loss for the three and six months ended June 30, 2024 and 2023 as it is more likely than not that the Company will not recognize the federal and state deferred tax benefits generated by its losses. The Company has provided a valuation allowance for the full amount of its net deferred tax assets as of June 30, 2024 and December 31, 2023, as management has determined it is more likely than not that any future benefit from deductible temporary differences and net operating loss and tax credit carryforwards would not be realized.

The Company did not record any amounts for unrecognized tax benefits as of June 30, 2024 or December 31, 2023.

11. Subsequent events

Since June 30, 2024, through the date of the issuance of these consolidated financial statements, the Company sold 0.3 million shares of our common stock and received $4.0 million after deducting commissions related to the Sales Agreement.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis of our financial condition and consolidated results of operations together with the section entitled "Risk Factors" and our interim consolidated financial statements and related notes appearing elsewhere in this Quarterly Report. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should carefully read the sections entitled "Special Note Regarding Forward-Looking Statements" and "Risk Factors" to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements.

Overview

We are a clinical-stage company dedicated to addressing the underlying causes of severe diseases by upregulating protein expression with RNA-based medicines. Using our proprietary TANGO (Targeted Augmentation of Nuclear Gene Output) approach, we are developing antisense oligonucleotides ("ASOs") to selectively restore protein levels.

Our first compound, zorevunersen (STK-001), is in clinical testing for the treatment of Dravet syndrome, a severe and progressive genetic epilepsy. Dravet syndrome is characterized by frequent, prolonged and refractory seizures beginning within the first year of life. The disease is classified as a developmental and epileptic encephalopathy due to the developmental delays and cognitive impairment associated with it. Dravet syndrome is one of many diseases caused by a haploinsufficiency, in which a loss of approximately 50% of normal protein levels leads to disease. We have completed two Phase 1/2a open-label studies of zorevunersen, MONARCH in the United States and ADMIRAL in the United Kingdom. We also have two ongoing Open Label Extension ("OLE") studies of zorevunersen for children and adolescents with Dravet syndrome, SWALLOWTAIL in the U.S. and LONGWING in the United Kingdom. Patients who participated in the MONARCH study in the United States or the ADMIRAL study in the United Kingdom and met study entry criteria were eligible to continue treatment in SWALLOWTAIL or LONGWING, respectively, both of which are designed to evaluate the long-term safety and tolerability of repeat doses of zorevunersen.

We are also pursuing treatment for a second haploinsufficient disease, autosomal dominant optic atrophy ("ADOA"), the most common inherited optic nerve disorder. STK-002 is our lead clinical candidate for the treatment of ADOA. 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. We have received authorization in the United Kingdom to proceed with a Phase 1 open-label study (OSPREY) of children and adults ages 6 to 55 who have an established diagnosis of ADOA and have evidence of a genetic mutation in the OPA1 gene. We expect the OSPREY study to start later this year.

In May 2022, we filed a universal Shelf Registration statement on Form S-3 (the "Registration Statement") with the SEC. The Registration Statement was declared effective by the SEC on May 31, 2022, and contains two prospectuses: a base prospectus, which covers the offering, issuance and sale by us of up to a maximum aggregate offering price of $400.0 million of our common stock, preferred stock, debt securities, warrants to purchase common stock, preferred stock or debt securities, subscription rights to purchase common stock, preferred stock or debt securities and/or units consisting of some or all of these securities; and a sales agreement prospectus covering the offering, issuance and sale by us of up to a maximum aggregate offering price of $150.0 million of common stock that may be issued and sold under a Controlled Equity Offering Sales Agreement ("Sales Agreement"). The specific terms of any securities to be offered pursuant to the base prospectus will be specified in a prospectus supplement to the base prospectus. The $150.0 million of common stock that may be offered, issued and sold under the sales agreement prospectus is included in the $400.0 million of securities that may be offered, issued and sold by us under the base prospectus.

As of June 30, 2024, we had issued approximately 6.5 million shares of common stock pursuant to the Sales Agreement for net proceeds of $53.4 million. Since June 30, 2024, the Company has issued approximately 0.3 million shares of common stock pursuant to the Sales Agreement for net proceeds of $4.0 million. We may terminate this at-the-market program at any time, pursuant to its terms. On April 2, 2024, we completed an underwritten public offering of our common stock and issued and sold 5,555,557 shares of common stock at a public offering price of $13.50 per share and issued pre-funded warrants to purchase 3,703,730 shares of common stock at a public offering price of $13.499 per share subject to an exercise price equal to $0.0001. The common stock and pre-funded warrants sold resulted in net proceeds of $119.9 million after deducting underwriting discounts, commissions and offering costs. No pre-funded warrants have been exercised as of June 30, 2024.

As of June 30, 2024 and December 31, 2023 we had $282.0 million and $201.4 million, respectively, in cash, cash equivalents and marketable securities.

Since inception, we have had operating losses, the majority of which are attributable to research and development activities. Our net losses were $25.7 million and $30.7 million for the three months ended June 30, 2024 and 2023, respectively and $52.1 million and $53.2 million for the six months ended June 30, 2024 and 2023, respectively. As of June 30, 2024, we had an accumulated deficit of $453.9 million.

Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and to a lesser extent, general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses. We expect to continue to

incur net losses for the foreseeable future, and we expect our research and development expenses, general and administrative expenses, and capital expenditures will continue to increase. In particular, we expect our expenses and losses to increase as we continue our development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products, as well as hire additional personnel, develop commercial infrastructure, pay fees to outside consultants, lawyers and accountants, and incur increased costs associated with being a public company such as expenses related to services associated with maintaining compliance with Nasdaq listing rules and SEC requirements, insurance and investor relations costs. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities.

Based upon our current operating plan, we believe that our existing cash, cash equivalents and marketable securities as of June 30, 2024 will enable us to fund our operating expenses and capital expenditure requirements at least to the end of 2025. To date, we have not had any products approved for sale and have not generated any product sales. We do not expect to generate any revenues from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. As a result, until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Any failure to raise capital as and when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise capital, we will need to delay, reduce or terminate planned activities to reduce costs.

Program Update

Dravet Syndrome Program – zorevunersen

In August 2024 we announced that the U.S. Food and Drug Administration removed the partial clinical hold on higher doses of zorevunersen in the MONARCH and SWALLOWTAIL studies in the United States. While the MONARCH study has been completed, patients in the SWALLOWTAIL OLE study in the United States currently receive chronic dosing every four months of 45mg of zorevunersen. In the United Kingdom, Patients in the LONGWING OLE study receive chronic dosing every four months of 45mg of zorevunersen as well.

Discussions with global regulatory agencies are underway and we are on track to provide a regulatory update on Phase 3 registrational plans for zorevunersen in the second half of 2024.

Financial operations overview

Revenue

We currently do not have any products approved for sale and have not generated any revenue from product sales since inception through June 2024. If we are able to successfully develop, receive regulatory approval for and commercialize any of our current or future product candidates alone or in collaboration with third parties, we may generate revenue from the sales of these product candidates.

In January 2022, we entered into a license and collaboration agreement with Acadia Pharmaceuticals Inc. (“Acadia”) for the discovery, development and commercialization of novel RNA-based medicines for the treatment of severe and rare genetic neurodevelopmental diseases of the central nervous system. The agreement focuses on the targets SYNGAP1, MECP2 (Rett syndrome), and an undisclosed neurodevelopmental target of mutual interest. In connection with each target, we will collaborate with Acadia to identify potential treatments for further development and commercialization as licensed products. With respect to SYNGAP1, we have agreed with Acadia to co-develop and co-commercialize licensed products for such target globally, and in connection therewith we granted to Acadia worldwide, co-exclusive (with us) licenses for such licensed products. With respect to MECP2 and the neurodevelopmental target, we granted to Acadia worldwide, exclusive licenses to develop and commercialize licensed products for such targets.

Pursuant to the terms of the agreement, we received an upfront payment of $60.0 million from Acadia. Acadia agreed to fund the research to identify potential licensed products for MECP2 and the neurodevelopmental target, and we will equally fund with Acadia the research to identify potential licensed products for SYNGAP1. We are eligible to receive up to $907.5 million in potential total milestone payments based upon the achievement of certain development, regulatory, first commercial sales and sales milestone events across the programs for the three targets, assuming each milestone were achieved at least once. With respect to licensed products for MECP2 and the neurodevelopmental target, we are also eligible to receive tiered royalties at percentages ranging from the mid-single digits to the mid-teens on future net sales by Acadia of licensed products worldwide. Royalties payable under the agreement are subject to standard royalty reductions. For SYNGAP1 licensed products that we are co-developing and co-commercializing, we will be responsible for 50% of the development and commercialization costs and will receive 50% of the profits from global commercialization. We are provided with a co-development and co-commercialization opt out option relating to the SYNGAP1 target

indication at our discretion. Such opt-out would reduce development and commercialization milestones but would provide us with royalties on an escalating basis attributable to net sales milestones.

See *Note 7—License and Collaboration Agreement with Acadia Pharmaceuticals, Inc.* of the notes to our unaudited consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

Operating expenses

Research and development

Research and development expenses consist primarily of costs incurred for the development of our discovery work and preclinical programs, which include:

- personnel costs, which include salaries, benefits and stock-based compensation expense;
- expenses incurred under agreements with consultants, third-party contract organizations that conduct research and development activities on our behalf, costs related to production of preclinical material and laboratory and vendor expenses related to the execution of preclinical studies;
- scientific consulting, collaboration and licensing fees;
- laboratory equipment and supplies; and
- facilities costs, depreciation and other expenses related to internal research and development activities.

We use our personnel and infrastructure resources across multiple research and development programs directed toward identifying and developing product candidates. Our direct research and development expenses are tracked on a program-by-program basis from the point a program becomes a clinical candidate for us and consists primarily of external costs, such as fees paid to consultants, central laboratories and contractors in connection with our preclinical activities. We do not allocate employee costs, costs associated with our technology or facility expenses, including depreciation or other indirect costs, to specific programs because these costs are currently deployed across multiple product development programs and, as such, are not separately classified. We use internal resources to manage our development activities and our employees work across multiple development programs and, therefore, we do not track their costs by program.

The table below summarizes our research and development expenses incurred by development program (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Zorevunersen (STK-001)	$ 6,543	$ 7,055	$ 15,076	$ 13,132
STK-002	1,509	1,552	$ 3,215	3,689
SYNGAP1	239	25	$ 523	101
MECP2	254	277	$ 497	408
Non-program specific and unallocated research and development expenses	12,591	11,642	24,193	22,852
Total research and development expenses	$ 21,136	$ 20,551	$ 43,504	$ 40,182

We expense all research and development costs in the periods in which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and third-party service providers.

We expect that our expenses will increase substantially in connection with our planned discovery work, preclinical and clinical development activities in the near term and our planned clinical trials in the future. At this time, we cannot reasonably estimate the costs for completing the preclinical and clinical development of any of our other product candidates. We expect our research and development expenses to increase substantially for the foreseeable future as we continue to invest in research and development activities related to developing our product candidates, including investments in manufacturing, as our programs advance into later stages of development and we conduct clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

Because of the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration and completion costs of the current or future preclinical studies and clinical trials or if, when, or to what extent we will generate revenues from the commercialization and sale of our product candidates. We may never succeed in achieving regulatory

approval for our product candidates. The duration, costs and timing of preclinical studies and clinical trials and development of our product candidates will depend on a variety of factors, including:

- successful completion of preclinical studies and investigational new drug-enabling studies;
- successful enrollment in, and completion of, clinical trials;
- receipt of regulatory approvals from applicable regulatory authorities;
- furthering our commercial manufacturing capabilities and arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity;
- launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety profile following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

A change in the outcome of any of these factors could mean a significant change in the costs and timing associated with the development of our current and future preclinical and clinical product candidates. For example, if the FDA, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development, or if we experience significant delays in execution of or enrollment in any of our preclinical studies or clinical trials, we could be required to expend significant additional financial resources and time on the completion of preclinical and clinical development. We expect our research and development expenses to increase for the foreseeable future as we continue the development of product candidates.

General and administrative expenses

General and administrative expenses consist primarily of personnel costs, costs related to maintenance and filing of intellectual property, expenses for outside professional services, including legal, human resources, information technology, audit and accounting services, and facilities and other expenses. Personnel costs consist of salaries, benefits and stock-based compensation expense. We expect our general and administrative expenses to increase over the next several years to support our continued research and development activities, manufacturing activities, increased costs of operating as a public company and the potential commercialization of our product candidates. These increases are anticipated to include increased costs related to the hiring of additional personnel, developing commercial infrastructure, fees to outside consultants, lawyers and accountants, and increased costs associated with being a public company such as expenses related to services associated with maintaining compliance with Nasdaq listing rules and Securities and Exchange Commission (“SEC”) requirements, insurance and investor relations costs.

Other income (expense)

Our other income (expense) includes (i) interest income earned on cash reserves in our operating, money market fund, investment accounts and on our marketable securities investments and (ii) other items of income (expense), net.

Results of operations for the three months ended June 30, 2024 and 2023

The following table sets forth our results of operations:

	Three Months Ended June 30,	
	2024	2023
	(in thousands)	
Consolidated statements of operations:		
Revenue	$ 4,831	$ (2,481)
Operating expenses:		
Research and development	21,136	20,551
General and administrative	13,037	10,230
Total operating expenses	34,173	30,781
Loss from operations	(29,342)	$ (33,262)
Other income (expense):		
Interest income (expense), net	3,695	2,567
Other income (expense), net	(48)	41
Total other income (expense)	3,647	2,608
Net loss	$ (25,695)	$ (30,654)

Revenue

Revenue for the three months ended June 30, 2024 was $4.8 million compared to $(2.5) million for the three months ended June 30, 2023, an increase of $7.3 million. Revenue is generated from satisfying contractual obligations of the collaboration and licensing agreement with Acadia Pharmaceuticals, Inc.

Research and development expenses

Research and development expenses were $21.1 million for the three months ended June 30, 2024 as compared to $20.6 million for the three months ended June 30, 2023, an increase of $0.5 million. The table below summarizes our research and development expenses (in thousands):

	Three Months Ended June 30,	
	2024	2023
Zorevunersen	$ 6,543	$ 7,055
STK-002	1,509	1,552
SYNGAP1	239	25
MECP2	254	277
Personnel-related expenses	8,868	8,274
Third-party services	296	604
Scientific consulting	503	146
Facilities and other research and development expenses	2,924	2,618
Total research and development expenses	$ 21,136	$ 20,551

The increase in research and development expenses was primarily attributable to an increase of $0.6 million in personnel-related expenses, $0.2 million in facilities and other research and development costs, an increase of $0.2 million in external third-party expenses related to SYNGAP1 and MECP2 offset by a decrease of $0.5 million in expenses related to zorevunersen and an immaterial change in expense related to our STK-002 program, both of which are comprised of third-party services and scientific consulting fees.

General and administrative expenses

General and administrative expenses were $13.0 million for the three months ended June 30, 2024 as compared to $10.2 million for the three months ended June 30, 2023. The increase of $2.8 million in general and administrative expenses was primarily attributable to an increase of $1.3 million in professional fees, $0.8 million in personnel related expenses, and $0.7 million in facilities and other general and administrative costs.

Other income (expense)

Other income (expense) was $3.6 million for the three months ended June 30, 2024 as compared to $2.6 million for the three months ended June 30, 2023.

Results of operations for the six months ended June 30, 2024 and 2023

The following table sets forth our results of operations:

	Six Months Ended June 30,	
	2024	2023
	(in thousands)	
Consolidated statements of operations:		
Revenue	$ 9,048	$ 2,671
Operating expenses:		
Research and development	43,504	40,182
General and administrative	23,258	20,442
Total operating expenses	66,762	60,624
Loss from operations	(57,714)	(57,953)
Other income:		
Interest income (expense), net	6,121	4,670
Other income (expense), net	(476)	84
Total other income	5,645	4,754
Net loss	$ (52,069)	$ (53,199)

Revenue

Revenue for the six months ended June 30, 2024 was $9.0 million compared to $2.7 million for the six months ended June 30, 2023, an increase of $6.3 million. Revenue is generated from satisfying contractual obligations of the collaboration and licensing agreement with Acadia Pharmaceuticals, Inc.

Research and development expenses

Research and development expenses were $43.5 million for the six months ended June 30, 2024 as compared to $40.2 million for the six months ended June 30, 2023, an increase of $3.3 million. The table below summarizes our research and development expenses (in thousands):

	Six Months Ended June 30,	
	2024	2023
Zorevunersen	$ 15,076	$ 13,132
STK-002	3,215	3,689
SYNGAP1	523	101
MECP2	497	408
Personnel-related expenses	16,824	16,289
Third-party services	464	1,135
Scientific consulting	743	298
Facilities and other research and development expenses	6,162	5,130
Total research and development expenses	$ 43,504	$ 40,182

The increase in research and development expenses was primarily attributable to an increase of $1.9 million in expenses related to zorevunersen, which is comprised of third-party services and scientific consulting fees, an increase of $0.9 million in facilities and other research and development costs, an increase of $0.5 million in personnel-related expenses, an increase of $0.5 million in external third-party expenses related to SYNGAP1 and MECP2, offset by a decrease of $0.5 million in expenses related to our STK-002 program, which is comprised of third-party services and scientific consulting fees.

General and administrative expenses

General and administrative expenses were $23.3 million for the six months ended June 30, 2024 as compared to $20.4 million for the six months ended June 30, 2023. The increase of $2.9 million in general and administrative expenses was primarily attributable to an

increase of $1.1 million in professional fees, $1.3 million in personnel related expenses, and $0.5 million in facilities and other general and administrative costs.

Other income (expense)

Other income (expense) was $5.6 million for the six months ended June 30, 2024 as compared to $4.8 million for the six months ended June 30, 2023.

Liquidity and capital resources

Since our inception through June 30, 2024, our operations have been financed by net proceeds of $663.7 million from the sale of convertible notes payable and our convertible preferred stock, our initial public offering ("IPO"), public offerings of common stock and pre-funded warrants, proceeds from the Sales Agreement and the upfront payment from Acadia. As of June 30, 2024, we had $282.0 million in cash, cash equivalents and marketable securities. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. On April 2, 2024, we completed an underwritten public offering of our common stock and issued and sold 5,555,557 shares of common stock at a public offering price of $13.50 per share and issued pre-funded warrants to purchase 3,703,730 shares of common stock at a public offering price of $13.499 per share subject to an exercise price equal to $0.0001. The common stock and warrants sold resulted in net proceeds of $119.9 million after deducting underwriting discounts, commissions and offering costs. No pre-funded warrants have been exercised.

We have incurred losses since our inception in June 2014 and, as of June 30, 2024, we had an accumulated deficit of $453.9 million. Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and to a lesser extent, general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

Based upon our current operating plan, we believe that our existing cash, cash equivalents and marketable securities as of June 30, 2024 will enable us to fund our operating expenses and capital expenditure requirements at least to the end of 2025. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. We will continue to require additional financing to advance our current product candidates through clinical development, to develop, acquire or in-license other potential product candidates and to fund operations for the foreseeable future. We will continue to seek funds through equity offerings, debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our ability to raise additional funds may be adversely impacted by potential worsening global macroeconomic conditions, including inflation, changing interest rates and instability in the global banking system, and disruptions to and volatility in the credit and financial markets in the United States and worldwide. If we do raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Any failure to raise capital as and when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise capital, we will need to delay, reduce or terminate planned activities to reduce costs.

Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the scope, progress, results and costs of researching and developing our lead product candidates or any future product candidates, and conducting nonclinical studies and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals or clearances for our lead product candidates or any future product candidates;
- the number and characteristics of any additional product candidates we develop or acquire;
- the timing of any cash milestone payments if we successfully achieve certain predetermined milestones;
- the cost of manufacturing our lead product candidates or any future product candidates and any products we successfully commercialize, including costs associated with building-out our manufacturing capabilities;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of any such agreements that we may enter into;
- the expenses needed to attract and retain skilled personnel;

- the costs associated with being a public company; and
- the timing, receipt and amount of sales of any future approved or cleared products, if any.

Further, our operating plans may change, and we may need additional funds to meet operational needs and capital requirements for clinical trials and other research and development activities. We currently have no credit facility or committed sources of capital. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated product development programs.

Cash flows

The following table summarizes our cash flows:

	Six Months Ended June 30,	
	2024	2023
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ (42,043)	$ (43,156)
Investing activities	(78,290)	76,558
Financing activities	122,367	45,102
Net increase in cash, cash equivalents and restricted cash	$ 2,034	$ 78,504

Operating activities

During the six months ended June 30, 2024, cash used in operating activities was $42.0 million. This was primarily attributable to a net loss of $52.1 million, a net change of $4.6 million in our net operating assets and liabilities, offset by non-cash charges of $14.7 million for stock-based compensation, depreciation, amortization and accretion of marketable securities, and a reduction in the carrying amount of right of use assets.

During the six months ended June 30, 2023, cash used in operating activities was $43.2 million. This was primarily attributable to a net loss of $53.2 million, a net change of $4.8 million in our net operating assets and liabilities, offset by non-cash charges of $14.8 million for share-based compensation, depreciation, amortization and accretion of marketable securities, and a reduction in the carrying amount of right of use assets.

Investing activities

Our investing activities during the six months ended June 30, 2024 consisted of $88.2 million of purchases of marketable securities offset by $10.0 million from the sales of marketable securities.

Our investing activities during the six months ended June 30, 2023 consisted of $77.6 million from the sales of marketable securities partially offset by purchases of property and equipment.

Financing activities

Our financing activities during the six months ended June 30, 2024 consisted of $119.9 million of net proceeds from a follow-on offering, $1.3 million of net proceeds from the controlled equity offering sales agreement, $1.0 million from the exercise of stock options and $0.2 million in proceeds from our Employee Stock Purchase Plan (the “ESPP”).

Our financing activities during the six months ended June 30, 2023 consisted of $44.7 million of net proceeds from the controlled equity offering sales agreement, $0.2 million from the exercise of stock options and $0.2 million in proceeds from the ESPP.

Contractual obligations and commitments

The following table summarizes our contractual obligations as of June 30, 2024 and the effects that such obligations are expected to have on our liquidity and cash flows in future fiscal periods:

	Payments Due by Fiscal Period				
	Total	Less Than 1 Year	1 to 3 Years	4 to 5 Years	More than 5 Years
			(in thousands)		
Operating lease obligations	$ 6,532	$ 1,307	$ 5,225	$ —	$ —
Total	$ 6,532	$ 1,307	$ 5,225	$ —	$ —

In August 2018, we entered into an agreement to lease approximately 23,000 square feet of space for a term of three years. Lease terms are triple net lease commencing at $0.9 million per year, then with 3% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The lease commencement date was December 10, 2018.

In September 2021, we entered into an agreement to extend the initial term of the 23,000 square foot lease for a period of three years ending December 31, 2024. In addition, this agreement provides for the lease of an additional 15,000 square feet of rentable space beginning on April 1, 2022 and ending on December 31, 2024. Initial monthly lease payments are approximately $0.1 million with respect to the 23,000 square feet space, and $0.1 million with respect to the 15,000 square feet space, and in each case subject to annual rent escalations.

In December 2023, we entered into an agreement to extend the term of the 38,000 square foot lease for a period of two years commencing on January 1, 2025 and ending on December 31, 2026. In December 2023, we recognized a right-of-use asset and operating lease liability of $4.1 million.

In December 2018, we entered into an agreement to lease 2,485 square feet of space for a term of three years. The lease includes one renewal option for an additional two years. Lease terms commence at $0.2 million per year, with 2.5% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. We occupied this space in May 2019.

In June 2021, we amended the agreement to extend the initial term of the 2,485 square foot lease for a period of three years ending April 30, 2025. In addition, the amendment provided for the lease of an additional 2,357 square feet of rentable space beginning on July 6, 2021 and ending on April 30, 2025. The amended lease provides us with the option to extend the term of the lease for an additional two years with a base annual rent increase of 3%.

Commitments

Our commitments primarily consist of obligations under our agreement with the University of Southampton. As of June 30, 2024, we were unable to estimate the timing or likelihood of achieving the milestones or making future product sales. For additional information regarding our agreements, see *Note 6—Commitments and Contingencies* of the notes to our consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

Additionally, we have entered into agreements with third-party contract manufacturers for the manufacture and processing of certain of our product candidates for preclinical testing purposes, and we have entered and will enter into other contracts in the normal course of business with contract research organizations for clinical trials and other vendors for other services and products for operating purposes. These agreements generally provide for termination or cancellation, other than for costs already incurred.

Off-balance sheet arrangements

During the periods presented, we did not have, nor do we currently have, any off-balance sheet arrangements as defined under SEC rules.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles ("GAAP"). The preparation of these consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

There have been no significant changes in our critical accounting policies and estimates as compared to the critical accounting policies and estimates disclosed in the section titled “Management’s Discussion and Analysis of Financial Condition and Operations” included in our Annual Report on Form 10-K filed with the SEC on March 25, 2024.

Emerging growth company and smaller reporting company status

We are an “emerging growth company,” as defined in the Jumpstart our Business Startups Act of 2012 (the “JOBS Act”). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies.

We have elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date we (i) are no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act.

As a result, our consolidated financial statements may not be comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates.

We will remain an emerging growth company until the earliest of (i) the last day of our first fiscal year (a) following the fifth anniversary of the completion of our IPO, (b) in which we have total annual gross revenues of at least $1.235 billion, or (c) when we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th, and (ii) the date on which we have issued more than $1.0 billion in non-convertible debt securities during the prior three-year period. We anticipate ceasing to be an emerging growth company as of December 31, 2024, which is the last day of our fiscal year following the fifth anniversary of the completion of our IPO.

We are also a “smaller reporting company,” meaning that the market value of our stock held by non-affiliates was less than $700.0 million and its annual revenue is less than $100.0 million during the most recently completed fiscal year. We may continue to be a smaller reporting company as long as either (i) the market value of our stock held by non-affiliates is less than $250.0 million or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700.0 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Recently Issued Accounting Pronouncements

In November 2023, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update (“ASU”) 2023-07, “*Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures*” (“ASU 2023-07”), which expands disclosures about a public entity’s reportable segments and requires more enhanced information about a reportable segment’s expenses, interim segment profit or loss, and how a public entity’s chief operating decision maker uses reported segment profit or loss information in assessing segment performance and allocating resources. The standard is effective for annual reporting periods beginning after December 15, 2023, and interim periods within years beginning after December 15, 2024, with early adoption permitted. The Company is currently assessing the impact that the adoption will have on its consolidated financial statements.

In December 2023, the FASB issued ASU 2023-09, “*Income Taxes (Topic 740): Improvements to Income Tax Disclosures*” (“ASU 2023-09”). ASU 2023-09 requires that an entity disclose specific categories in the effective tax rate reconciliation as well as provide additional information for reconciling items that meet a quantitative threshold and certain disclosures of state versus federal income tax expenses and taxes paid. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024. The Company does not expect the adoption of ASU 2023-09 to have a material impact on its consolidated financial statements and will adopt the standard effective January 1, 2025.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest rate risk

We are exposed to market risks in the ordinary course, primarily including interest sensitivities. As of June 30, 2024 and December 31, 2023, we had cash, cash equivalents and marketable securities of $282.0 million and $201.4 million respectively. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Because of the short-term maturities of our cash and cash equivalents, we do not believe that an immediate 10% increase in interest rates would have any significant impact on the realized value of our investments. Accordingly, we do not believe we are exposed to material market risk with respect to our cash and cash equivalents.

Inflation Risk

Inflation generally affects us by increasing our clinical trial costs. We do not believe that inflation has had a material effect on our business, financial condition or results of operations during the periods ended June 30, 2024 and 2023.

Item 4. Controls and Procedures.

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Under the supervision and with the participation of our management, including our Chief Financial Officer and Chief Executive Officer, we evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934 (the “Exchange Act”)) as of June 30, 2024. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate, to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on our management’s evaluation (with the participation of our Chief Executive Officer and our Chief Financial Officer), as of the end of the period covered by this report, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There has been no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act) during the period covered by this report that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Inherent Limitations on Effectiveness of Controls

Internal control over financial reporting may not prevent or detect all errors and all fraud. Any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objective and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may be involved in legal proceedings arising in the ordinary course of our business. We are not presently a party to any legal proceedings that, in the opinion of management, would have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity and reputation harm, and other factors.

Item 1A. Risk Factors.

Summary of Risk Factors

An investment in our common stock involves various risks, and prospective investors are urged to carefully consider the matters discussed in the section titled “Risk Factors” prior to making an investment in our common stock. These risks include, but are not limited to, the following:

- We are early in our development efforts. If we or our collaborators are unable to develop, obtain regulatory approval for and commercialize zorevunersen (STK-001), STK-002 and our future product candidates, or if we experience significant delays in doing so, our business will be materially harmed.
- Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials, including in our Dravet syndrome program or our Autosomal Dominant Optic Atrophy (“ADOA”) program.
- Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a narrower indication than we seek.
- Certain of the diseases we seek to treat have low prevalence, and it may be difficult to identify patients with these diseases, which may lead to delays in enrollment for our trials or slower commercial revenue growth if zorevunersen, STK-002 or our future product candidates are approved.
- If clinical trials of zorevunersen, STK-002 or any other product candidate that we develop fail to demonstrate safety and efficacy to the satisfaction of the United States Food and Drug Administration (the “FDA”) or foreign regulatory authorities or do not otherwise produce favorable results, we may incur additional costs or experience delays in completing, or ultimately may be unable to complete, the development and commercialization of such product candidate.
- We may not be successful in our efforts to use our Targeted Augmentation of Nuclear Gene Output (“TANGO”) technology to expand our pipeline of product candidates and develop marketable products.
- Any product candidate for which we obtain marketing approval will be subject to extensive post-marketing regulatory requirements and could be subject to post-marketing restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them are approved.
- Our failure to obtain regulatory approval in international jurisdictions would prevent us from marketing our product candidates outside the United States.
- Zorevunersen, STK-002 or our future product candidates may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.
- A Rare Pediatric Disease designation by the FDA does not guarantee that the new drug application (“NDA”) for the product will qualify for a priority review voucher upon approval, and it does not lead to a faster development or regulatory review process, or increase the likelihood that zorevunersen, STK-002 or our future product candidates will receive marketing approval.
- A Fast Track Designation by the FDA, even if granted for zorevunersen, STK-002 or our future product candidates, may not lead to a faster development or regulatory review or approval process, and would not increase the likelihood that our product candidates will receive marketing approval.
- A Breakthrough Therapy Designation by the FDA, even if granted for zorevunersen, STK-002 or our future product candidates may not lead to a faster development or regulatory review or approval process, and it would not increase the likelihood that the product candidates will receive marketing approval.

•Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

•The commercial success of our product candidates, including zorevunersen and STK-002 will depend upon their degree of market acceptance by providers, patients, patient advocacy groups, third-party payors and the general medical community.

•The pricing, insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

•Current and potential future healthcare reforms may adversely impact pricing, insurance coverage and reimbursement status of newly approved products.

•We have a history of operating losses, and we may not achieve or sustain profitability. We anticipate that we will continue to incur losses for the foreseeable future. If we fail to obtain additional funding to conduct our planned research and development effort, we could be forced to delay, reduce or eliminate our product development programs or commercial development efforts.

•We expect that we will need to raise additional funding before we can expect to become profitable from any potential future sales of zorevunersen, STK-002 or our future product candidates. This additional financing may not be available on acceptable terms or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

•Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

•Our success depends in part on our ability to obtain, maintain and protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.

•The market price of our stock may be volatile, and you could lose all or part of your investment.

Risks Related to Product Development and Regulatory Approval

We are early in our development efforts. If we are unable to develop, obtain regulatory approval for and commercialize zorevunersen (STK-001), STK-002 and our future product candidates, or if we experience significant delays in doing so, our business will be materially harmed.

We have invested substantially all of our efforts and financial resources in the development of TANGO and our current lead product candidate, zorevunersen, for the treatment of Dravet syndrome. We submitted an investigational new drug application (“IND”) for zorevunersen to the FDA in late 2019. In August 2020, we dosed the first patient with zorevunersen in the single ascending dose portion of the MONARCH Phase 1/2a Study at the 10mg dose level.

In addition, in November 2020, we announced the nomination of OPA1 as our next target for preclinical development to treat ADOA. In November 2021, we announced the nomination of STK-002 as the lead product candidate for the treatment of ADOA and intend to invest significant efforts and financial resources in its development. We submitted a Clinical Trial Authorization (“CTA”) application for STK-002 to the United Kingdom Medicines and Healthcare Products Regulatory Agency (the “MHRA”) in early 2023, and the MHRA authorized such CTA in April 2023, but enrollment and dosing of patients has not yet commenced. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of TANGO and our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

Each of our programs and product candidates will require preclinical and clinical development, regulatory approval in multiple jurisdictions, obtaining preclinical, clinical and commercial manufacturing supply, capacity and expertise, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenue from product sales. Zorevunersen, STK-002 and our future product candidates must be authorized for marketing by the FDA or certain other foreign regulatory agencies, such as the European Medicines Agency (the “EMA”) or the MHRA, before we may commercialize any of our product candidates.

The success of zorevunersen, STK-002 and our future product candidates depends on multiple factors, including:

•effective INDs and CTAs that allow commencement of our planned clinical trials or future clinical trials for our product candidates in relevant territories;

•our ability to obtain approval from institutional review boards (“IRBs”) or ethics committees to conduct clinical trials at their respective sites;

•potential delays in enrollment, site visits, evaluations, or dosing of patients participating in clinical trials as hospitals face staffing shortages, whether due to labor relations or otherwise, or patients decide not to enroll in the study as a result of such staffing shortages;

•the direct and indirect impact of general economic, industry and market conditions, including fluctuating interest rates, inflation, market volatility, potential recessions, a potential federal government shutdown, and any health pandemic on our business and operations, third party vendors, supply chain, and regulatory approvals;

•successful completion of preclinical studies, including those compliant with Good Laboratory Practices toxicology studies, biodistribution studies and minimum effective dose studies in animals;

•our ability to reach agreements on acceptable terms with prospective third-party contract research organizations ("CROs") and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among CROs and trial sites;

•successful enrollment and completion of clinical trials compliant with current Good Clinical Practices;

•positive results from our clinical programs that demonstrate safety and efficacy and provide an acceptable risk-benefit profile for our product candidates in the intended patient populations;

•receipt of regulatory approvals from applicable regulatory authorities;

•establishment of arrangements with third-party contract manufacturing organizations ("CMOs") for key materials used in our manufacturing processes and to establish backup sources for clinical and large-scale commercial supply;

•establishment and maintenance of patent and trade secret protection and regulatory exclusivity for our product candidates;

•commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others;

•acceptance of our product candidates, if and when approved, by patients, patient advocacy groups, third-party payors and the general medical community;

•our effective competition against other therapies available in the market;

•establishment and maintenance of adequate reimbursement from third-party payors for our product candidates;

•our ability to acquire or in-license additional product candidates;

•prosecution, maintenance, enforcement and defense of intellectual property rights and claims; and

•maintenance of a continued acceptable safety profile of our product candidates following approval.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials, including in our Dravet syndrome program or our ADOA program.

Zorevunersen is currently being evaluated in human clinical trials, and we may experience unexpected or negative results in the future. We will be required to demonstrate through adequate and well-controlled clinical trials that our product candidates are safe and effective, with a favorable benefit-risk profile, for use in their target indications before we can seek regulatory approvals for their commercial sale. The positive results we have observed for our product candidates in preclinical animal models may not be predictive of our future clinical trials in humans, as mouse models carry inherent limitations relevant to all preclinical studies. In particular, the Dravet syndrome mouse model is more severe than the human disease and provides a shorter post-symptomatic observation period. Trial designs and results from early-phase trials are not necessarily predictive of future clinical trial designs or results, and initial positive results we may observe may not be confirmed in later-phase clinical trials. For example, although we recently reported end of study data from our Phase 1/2a open-label studies of zorevunersen demonstrating a reduction in median convulsive seizure frequency compared to baseline, these results were based on pooling data from the Phase 1/2a open-label studies of zorevunersen in the United States (MONARCH) and in the United Kingdom (ADMIRAL) and additional trials may not confirm these results. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development even if they successfully advance through initial clinical trials, and preliminary interim data readouts of ongoing trials may show results that change when such trials are completed. We may not be able to demonstrate a disease-modifying effect of zorevunersen in our clinical trials in Dravet syndrome patients, even if we are able to demonstrate efficacy on seizure reduction, and we may be similarly unable to demonstrate the efficacy of STK-002 in our ADOA program or other future programs. In addition, our clinical trials to date have necessarily involved relatively small numbers of participants. Therefore, conclusions we draw based upon trial results to date may not be repeatable across larger cohorts of participants or patients with different characteristics. Moreover, even if our clinical trials

demonstrate acceptable safety and efficacy of zorevunersen, STK-002 or our future product candidates, the labeling we obtain through negotiations with the FDA or foreign regulatory authorities may not include data on secondary endpoints and may not provide us with a competitive advantage over other products approved for the same or similar indications.

Many companies in the biotechnology industry have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and there is a high failure rate for product candidates proceeding through clinical trials. In addition, different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Preclinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent regulatory approval. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, including zorevunersen for Dravet syndrome or STK-002 for ADOA, then the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld or withdrawn. Regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development. We cannot be certain that we will not face similar setbacks.

If clinical trials of zorevunersen, STK-002 or any other product candidate that we develop fail to demonstrate safety and efficacy to the satisfaction of FDA or foreign regulatory authorities or do not otherwise produce favorable results, we may incur additional costs or experience delays in completing, or ultimately may be unable to complete, the development and commercialization of such product candidate.

Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, including zorevunersen and STK-002, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing.

Clinical trials may be placed on a full or partial clinical hold by the FDA, foreign regulatory authorities, or us for various reasons, including but not limited to: deficiencies in the conduct of the clinical trials, including failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols; deficiencies in the clinical trial operations or trial sites; deficiencies in the trial designs necessary to demonstrate efficacy; fatalities or other adverse effects arising during a clinical trial due to medical problems that may or may not be related to clinical trial treatments; the product candidates may not appear to be more effective than current therapies; the quality or stability of the product candidates may fall below acceptable standards; or the data from animal studies are not sufficient to support the anticipated exposure (dose, route of administration, and duration) for the proposed clinical trial. For example, the FDA previously placed a partial clinical hold on certain doses of zorevunersen pending additional preclinical testing. Even though the FDA removed the partial clinical hold on zorevunersen, our current and future product candidates may be subject to other clinical holds in the future.

In addition, we, the FDA, foreign regulatory authorities, or an IRB or similar foreign review board or committee, may delay initiation of, suspend or limit dose escalation of clinical trials of a product candidate at any time for various reasons, including if we or they believe the healthy volunteer subjects or patients participating in such trials are being exposed to unacceptable health risks. Among other reasons, adverse side effects of a product candidate or a related product in preclinical trials or on healthy volunteer subjects or patients in a clinical trial could result in such a decision. For example, in November 2022, we announced our decision to limit chronic dosing in the open-label extension studies to 30mg in SWALLOWTAIL in the U.S. and 45mg in LONGWING in the U.K. Our decision at that time was based on interactions with regulatory agencies and a review of interim chronic toxicology data from a study in non-human primates (“NHPs”) in which the total drug administered to NHPs over a 1-year period was substantially higher than what we would anticipate giving to participants in clinical trials.

Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a narrower indication than we seek.

Prior to commercialization, zorevunersen, STK-002, and our other future product candidates must be approved by the FDA pursuant to an NDA in the United States and pursuant to similar marketing applications by the EMA and similar regulatory authorities outside the United States. The process of obtaining marketing approvals, both in the United States and abroad, is expensive and takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market zorevunersen, STK-002 or any of our other future product candidates from regulatory authorities in any jurisdiction. We have no experience in submitting and supporting the applications necessary to gain marketing approvals, and, in the event regulatory authorities indicate that we may submit such applications, we may be unable to do so as quickly and efficiently as desired. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate’s safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Our product candidates

may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use. Regulatory authorities have substantial discretion in the approval process and may refuse to accept or file any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate.

Approval of zorevunersen, STK-002 and our other future product candidates may be delayed or refused for many reasons, including:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate, to the satisfaction of the FDA or comparable foreign regulatory authorities, that our product candidates are safe and effective for any of their proposed indications;
- the results of clinical trials may not meet the level of statistical significance or clinical meaningfulness required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical programs or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an NDA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- the facilities of third-party manufacturers with which we contract or procure certain service or raw materials may not be adequate to support approval of our product candidates;
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval; and
- potential delays in enrollment, site visits, evaluations, or dosing of patients participating in the clinical trial as hospitals face staffing shortages, whether due to labor relations or otherwise, or patients decide to not enroll in the study as a result of or such staffing shortages.

Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials, a potential temporary federal government shutdown and the review process.

Further, in June 2024, the U.S. Supreme Court reversed its longstanding approach under the Chevron doctrine, which provided for judicial deference to regulatory agencies, including the FDA. As a result of this decision, we cannot be sure whether there will be increased challenges to existing agency regulations or how lower courts will apply the decision in the context of other regulatory schemes without more specific guidance from the U.S. Supreme Court. For example, this decision may result in more companies bringing lawsuits against the FDA to challenge longstanding decisions and policies of the FDA, which could undermine the FDA's authority, lead to uncertainties in the industry, and disrupt the FDA's normal operations, which could impact the timely review of any regulatory filings or applications we submit to the FDA.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a risk evaluation and mitigation strategy ("REMS"). These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and adversely affect our business, financial condition, results of operations and prospects. While currently we are not experiencing any significant delays or disruptions to our clinical trials as a result of hospital staffing shortages or global macroeconomic conditions, we take into consideration such shortages and conditions may directly or indirectly impact our clinical trial enrollment, dosing, and regulatory approval timelines.

Certain of the diseases we seek to treat have low prevalence, and it may be difficult to identify patients with these diseases, which may lead to delays in enrollment for our trials or slower commercial revenue growth if zorevunersen, STK-002 or our future product candidates are approved.

Genetically defined diseases generally, and especially those for which our product candidates are targeted, have low incidence and prevalence. We estimate that the worldwide incidence of Dravet syndrome is approximately one in 16,000 births, and the incidence of

ADOA is approximately one in 30,000 births. This could pose obstacles to the timely recruitment and enrollment of a sufficient number of eligible patients into our trials or limit a product candidate's commercial potential. Patient enrollment may be affected by other factors including:

- the ability to identify and enroll patients that meet study eligibility criteria in a timely manner for clinical trials;
- the severity of the disease under investigation;
- design of the study protocol;
- the perceived risks, benefits and convenience of administration of the product candidate being studied;
- the patient referral practices of providers; and
- the proximity and availability of clinical trial sites to prospective patients.

Any inability to enroll a sufficient number of patients with these diseases for our planned clinical trials would result in significant delays and could cause us to not initiate or abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidate, which would cause the value of our company to decline and limit our ability to obtain additional financing.

Additionally, our projections of both the number of people who have Dravet syndrome or ADOA, as well as the people with this disease who have the potential to benefit from treatment with our product candidates, are based on estimates derived from a market research study that we commissioned, which may not accurately identify the size of the market for our product candidates. The total addressable market opportunity for zorevunersen, STK-002 and our future product candidates will ultimately depend upon, among other things, the final labeling for our product candidates, if our product candidates are approved for sale in our target indications, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients globally may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our product candidates, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

Moreover, in light of the limited number of potential patients impacted by Dravet syndrome and ADOA, our per-patient therapy pricing of zorevunersen, STK-002 and our future product candidates, if approved, must be high in order to recover our development and manufacturing costs, fund additional research and achieve profitability. We may also need to fund patient support programs upon the marketing of a product candidate, which would negatively affect our product revenue. We may be unable to maintain or obtain sufficient therapy sales volumes at a price high enough to justify our development efforts and our sales, marketing and manufacturing expenses.

We may not be successful in our efforts to use TANGO to expand our pipeline of product candidates and develop marketable products.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. Our business depends on our successful development and commercialization of the limited number of internal product candidates we are researching or have in preclinical development. Even if we are successful in continuing to build our pipeline, development of the potential product candidates that we identify will require substantial investment in additional clinical development, management of clinical, preclinical and manufacturing activities, regulatory approval in multiple jurisdictions, obtaining manufacturing supply capability, building a commercial organization, and significant marketing efforts before we generate any revenue from product sales. Furthermore, such product candidates may not be suitable for clinical development, including as a result of their harmful side effects, limited efficacy or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance. If we cannot validate TANGO by successfully developing and commercializing product candidates based upon our technological approach, we may not be able to obtain product revenue in future periods, which would adversely affect our business, prospects, financial condition and results of operations.

In November 2021, we announced the nomination of STK-002 as our lead product candidate for in the treatment of ADOA; however, we are primarily focused on our lead product candidate for Dravet syndrome, zorevunersen, and we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. Our understanding and evaluation of biological targets for the discovery and development of new product candidates may fail to identify challenges encountered in subsequent preclinical and clinical development. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights.

Any product candidate for which we obtain marketing approval will be subject to extensive post-marketing regulatory requirements and could be subject to post-marketing restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them are approved.

Our product candidates and the activities associated with their development and potential commercialization, including their testing, manufacturing, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other U.S. and international regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, including current Good Manufacturing Practices ("CGMPs"), quality control, quality assurance and corresponding maintenance of records and documents, including periodic inspections by the FDA and other regulatory authorities and requirements regarding the distribution of samples to providers and recordkeeping.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of any approved product. The FDA closely regulates the post-approval marketing and promotion of drugs and biologics to ensure they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding use of their products. If we promote our product candidates in a manner inconsistent with FDA-approved labeling or otherwise not in compliance with FDA regulations, we may be subject to enforcement action. Moreover, while we believe our product candidates may provide improved safety profiles over existing products, unless we conduct head-to-head studies, we will not be able to make comparative claims for products, if approved.

Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws and similar laws in international jurisdictions.

In addition, later discovery of previously unknown adverse events or other problems with our product candidates, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such product candidates, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of any approved product from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of product candidates;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our product candidates;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with European requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with Europe's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Our failure to obtain regulatory approval in international jurisdictions would prevent us from marketing our product candidates outside the United States.

To market and sell zorevunersen, STK-002 and our future product candidates, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, we must secure product reimbursement approvals before regulatory authorities will approve the product for sale in that country. Failure to obtain foreign regulatory approvals or non-compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries. The United Kingdom's exit from the European Union (the "EU"), which is referred to as

“Brexit,” became fully effective on December 31, 2020. Brexit continues to create political and economic uncertainty, particularly in the United Kingdom and the EU. Prior to Brexit, a significant proportion of the regulatory framework in the United Kingdom was derived from EU directives and regulations. Following Brexit, the United Kingdom retained the EU regulatory regime with certain modifications as standalone U.K. legislation. Therefore, the U.K. regulatory regime is currently similar to EU regulations, but the United Kingdom has enacted new legislation, the Medicines and Medical Devices Act. Under this legislation, the U.K. may adopt changed regulations that may diverge from the EU legislative regime for medicines, including their research, development and commercialization and has issued a consultation document with respect to future changes. Brexit may lead to additional regulatory costs and could materially impact the regulatory regime with respect to the approval of our product candidates in the United Kingdom or the EU.

If we fail to comply with the regulatory requirements in international markets and receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business will be adversely affected. We may not obtain foreign regulatory approvals on a timely basis, if at all. Our failure to obtain approval of any of our product candidates by regulatory authorities in another country may significantly diminish the commercial prospects of that product candidate and our business prospects could decline.

Zorevunersen, STK-002 or our future product candidates may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.

Although other antisense oligonucleotides (“ASOs”) have received regulatory approval, our method of seeking to upregulate protein expression by targeting the underlying genetic causes of haploinsufficiencies presents a new approach to disease treatment, which means there is uncertainty associated with the safety profile of zorevunersen, STK-002 or our future product candidates and drugs in the antisense oligonucleotide class.

In addition to side effects caused by our product candidates, the intrathecal or intravitreal administration process or related procedures also can cause adverse side effects. If any such adverse events occur, our clinical trials could be suspended or terminated. If we are unable to demonstrate that any adverse events were caused by the administration process or related procedures, the FDA, the European Commission, the EMA, the U.K. MHRA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we can demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may adversely affect our business, financial condition, results of operations and prospects significantly. Finally, SPINRAZA, which is produced by Biogen Inc., is an ASO therapy utilizing intrathecal delivery, and if SPINRAZA is found to cause undesirable side effects or to be unsafe due to a potential class effect, it may adversely affect demand for zorevunersen and our other future product candidates. Other ASOs in clinical development utilizing intrathecal delivery could also generate data that could adversely affect the clinical, regulatory or commercial perception of zorevunersen and our other future product candidates.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a REMS to ensure that the benefits of the product outweigh its risks, which may include, for example, a Medication Guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners, or other elements to assure safe use of the product. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings in the labeling;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these occurrences may harm our business, financial condition, results of operations and prospects significantly.

A Rare Pediatric Disease designation by the FDA does not guarantee that the NDA for the product will qualify for a priority review voucher upon approval, and it does not lead to a faster development or regulatory review process, or increase the likelihood that zorevunersen, STK-002 or our future product candidates will receive marketing approval.

Under the Rare Pediatric Disease Priority Review Voucher program, upon the approval of a qualifying NDA for the treatment of a rare pediatric disease, the sponsor of such an application would be eligible for a rare pediatric disease priority review voucher that can be used to obtain priority review for a subsequent Biologics License Application or NDA. As part of our business strategy for

zorevunersen, we received Rare Pediatric Disease Designation in October 2022. We may also seek Rare Pediatric Disease designations for any other future product candidates. If a product candidate is designated before September 30, 2024, it is eligible to receive a voucher if it is approved before September 30, 2026. However, there is no expectation that zorevunersen, STK-002 or our future product candidates will be designated, other than zorevunersen, or approved by those dates, or at all, or that the program will be further extended, and, therefore, we may not be in a position to obtain any priority review vouchers. Additionally, designation of a drug for a rare pediatric disease does not guarantee that an NDA will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Finally, a Rare Pediatric Disease Designation does not lead to faster development or regulatory review of the product or increase the likelihood that it will receive marketing approval.

A Fast Track Designation by the FDA, even if granted for zorevunersen, STK-002 or any of our future product candidates, or any use of the accelerated approval pathway, may not lead to a faster development or regulatory review or approval process, and would not increase the likelihood that our product candidates will receive marketing approval.

If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply to the FDA for Fast Track Designation. The FDA has broad discretion whether to grant this designation. Even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation for any of our product candidates, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program. Many drugs that have received Fast Track Designation have failed to obtain approval.

We may also seek accelerated approval for our product candidates. Under the FDA's accelerated approval program, the FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Full approval of another product for the same indication as any of our product candidates for which we are seeking accelerated approval may make accelerated approval of our product candidates more difficult. For drugs granted accelerated approval, post-marketing confirmatory trials are required to describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. These confirmatory trials must be completed with due diligence and in general the FDA may require that the trial be designed and/or initiated prior to approval. The Food and Drug Omnibus Reform Act ("FDORA") was recently enacted, which included provisions related to the accelerated approval pathway. Pursuant to FDORA, the FDA is authorized to require a post-approval study to be underway prior to approval or within a specified time period following approval. FDORA also requires the FDA to specify conditions of any required post-approval study, which may include milestones and requires sponsors to submit progress reports for required post-approval studies and any conditions required by the FDA. FDORA enables the FDA to initiate enforcement action for the failure to conduct with due diligence a required post-approval study, including a failure to meet any required conditions specified by the FDA or to submit timely reports. All promotional materials for product candidates approved via accelerated approval are subject to prior review by the FDA. Moreover, the FDA may withdraw approval of any product candidate or indication approved under the accelerated approval pathway if, for example:

- the trial or trials required to verify the predicted clinical benefit of the product candidate fail to verify such benefit or do not demonstrate sufficient clinical benefit to justify the risks associated with the drug;
- other evidence demonstrates that the product candidate is not shown to be safe or effective under the conditions of use;
- we fail to conduct any required post-approval trial of the product candidate with due diligence; or
- we disseminate false or misleading promotional materials relating to the product candidate.

A Breakthrough Therapy Designation by the FDA, even if granted for zorevunersen, STK-002 or any of our future product candidates, may not lead to a faster development or regulatory review or approval process, and it would not increase the likelihood that the product candidate will receive marketing approval.

We may seek a Breakthrough Therapy Designation for zorevunersen, STK-002 or one or more of our future product candidates. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for priority review if supported by clinical data at the time of the submission of the NDA.

Designation as a breakthrough therapy is at the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such

designation. In any event, the receipt of a Breakthrough Therapy Designation for a drug may not result in a faster development process, review, or approval compared to drugs considered for approval under conventional FDA procedures and it would not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product candidate no longer meets the conditions for qualification or it may decide that the time period for FDA review or approval will not be shortened.

Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

Existing regulatory policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may not obtain or may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

In addition, in the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. The pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. Previously, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "ACA") was enacted, which was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Healthcare reform initiatives recently culminated in the enactment of the Inflation Reduction Act ("IRA") in August 2022, which, among other things, allows U.S. Department of Health and Human Services ("HHS") to directly negotiate the selling price of a statutorily specified number of drugs and biologics each year that the Centers for Medicare & Medicaid Services ("CMS") reimburses under Medicare Part B and Part D. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for single-source biologics) are eligible be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. Negotiations for Medicare Part D products take place in 2024 with the negotiated price taking effect in 2026, and negotiations for Medicare Part B products will begin in 2026 with the negotiated price taking effect in 2028. In August 2023, HHS announced the ten Medicare Part D drugs and biologics that it selected for negotiations. HHS will announce the negotiated maximum fair prices by September 1, 2024, and this price cap, which cannot exceed a statutory ceiling price, will go into effect on January 1, 2026. A drug or biological product that has an orphan drug designation for only one rare disease or condition will be excluded from the IRA's price negotiation requirements, but will lose that exclusion if it receives designations for more than one rare disease or condition, or if is approved for an indication that is not within that single designated rare disease or condition, unless such additional designation or such disqualifying approvals are withdrawn by the time CMS evaluates the drug for selection for negotiation. The negotiated prices will represent a significant discount from average prices to wholesalers and direct purchasers. The law also imposes rebates on Medicare Part D and Part B drugs whose prices have increased at a rate greater than the rate of inflation. In addition, the law eliminates the "donut hole" under Medicare Part D beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost through a newly established manufacturer discount program which requires manufacturers to subsidize 10% of Part D enrollees' prescription costs for brand drugs below the out-of-pocket maximum, and 20% once the out-of-pocket maximum has been reached. The IRA also extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. Manufacturers that fail to comply with the IRA may be subject to various penalties, including civil monetary penalties. These provisions may be subject to legal challenges. For example, the provisions related to the negotiation of selling prices of high-expenditure single-source drugs and biologics have been challenged in multiple lawsuits brought by pharmaceutical manufacturers. Thus, while it is unclear how the IRA will be implemented, it will likely have a significant impact on the pharmaceutical industry.

In addition, other legislative changes have been proposed and adopted. These changes included aggregate reductions of Medicare payments to providers of up to 2% per fiscal year, which went into effect in 2013, and will remain in effect through 2031, with the exception of a temporary suspension implemented under various COVID-19 relief legislation from May 1, 2020 through March 31, 2022, unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and accordingly, our financial operations. Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA authorization under an FDA expanded access program; however, manufacturers are not obligated to

provide investigational new drug products under the current federal right to try law. We may choose to seek an expanded access program for our product candidates, or to utilize comparable rules in other countries that allow the use of a drug, on a named patient basis or under a compassionate use program.

Furthermore, there have been, and continue to be, a number of other initiatives at the United States federal and state levels that seek to reduce healthcare costs. For example, in December 2020, CMS issued a final rule implementing significant manufacturer price reporting changes under the Medicaid Drug Rebate Program, including an alternative rebate calculation for line extensions that is tied to the price increases of the original drug, and Best Price reporting related to certain value-based purchasing arrangements. Additionally, under the American Rescue Plan Act of 2021, effective January 1, 2024, the statutory cap on Medicaid Drug Rebate Program rebates that manufacturers pay to state Medicaid programs on a unit of drug is eliminated. Elimination of this cap may, in some cases, require pharmaceutical manufacturers to pay more in rebates than they receive on the sale of products. Further, the Infrastructure Investment and Jobs Act added a requirement, effective January 1, 2023, for manufacturers of certain single-source drugs separately paid for under Medicare Part B for at least 18 months and marketed in single-dose containers or packages (known as refundable single-dose containers or single-use package drugs) to provide annual refunds for any portions of the dispensed drug that are unused and discarded if those unused or discarded portions exceed an applicable percentage defined by statute or regulation. Manufacturers are subject to periodic audits and those that fail to pay refunds for their refundable single-dose containers or single-use package drugs shall be subject to civil monetary penalties. Healthcare reforms that have been adopted, and that may be adopted in the future, could result in further reductions in coverage and levels of reimbursement for pharmaceutical products, increases in rebates payable under U.S. government rebate programs and additional downward pressure on pharmaceutical product prices.

We expect that the ACA, the IRA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product candidates.

At the state level in the United States, legislatures are increasingly enacting laws and implementing regulations designed to control pharmaceutical and biologic product pricing, including price constraints, restrictions on certain product access, reporting on price increases and the introduction of high-cost drugs.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

We may be unsuccessful in obtaining Orphan Drug Designation or transfer of designations obtained by others for future product candidates. And, even if we obtain such designation, we may be unable to maintain the benefits associated with Orphan Drug Designation, including the potential for market exclusivity, for zorevunersen, STK-002 or our future product candidates.

As part of our business strategy for zorevunersen, we received Orphan Drug Designation for the treatment of Dravet syndrome in the United States in 2019 and also in the EU in 2022. As part of our business strategy for STK-002, we received Orphan Drug Designation for the treatment of ADOA in the United States in 2022. We may seek such designations for our product candidates in other countries as well. However, Orphan Drug Designation does not guarantee future orphan drug marketing exclusivity, and there is no guarantee that we will be successful in obtaining such designation for our future product candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs intended to treat relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is defined as a patient population of fewer than 200,000 individuals in the United States. In the United States, Orphan Drug Designation entitles a party to financial incentives such as opportunities for tax credits for qualified clinical research costs and exemption from prescription drug user fees. Similarly, in the EU, the European Commission grants Orphan Drug Designation after receiving the opinion of the EMA's Committee for Orphan Medicinal Products on an Orphan Drug Designation application. In the EU, Orphan Drug Designation is intended to promote the development of drug that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or the product would be a significant benefit to those affected). In the EU, Orphan Drug Designation entitles a party to financial incentives such as reduction of fees or fee waivers.

Generally, if a drug with an Orphan Drug Designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes EMA or the FDA from approving another marketing application for the same drug and indication for that time period, except in limited circumstances. If a competitor is able to obtain orphan drug exclusivity prior to us for a product that constitutes the same active moiety and treats the same indications

as our product candidates, we may not be able to obtain approval of our drug by the applicable regulatory authority for a significant period of time unless we are able to show that our drug is clinically superior to the approved drug. The applicable period is seven years in the United States and ten years in the EU. The EU exclusivity period can be reduced to six years if a drug no longer meets the criteria for Orphan Drug Designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

Even after an orphan drug is approved, the FDA can also subsequently approve a later application for the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan Drug Designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

The orphan drug exclusivity contained in the Orphan Drug Act has been the subject of recent scrutiny from the press, from some members of Congress and from some in the medical community. Furthermore, the FDA's interpretations of the Orphan Drug Act have not been successfully challenged in court and future court decisions could continue that trend. There can be no assurances that the exclusivity granted to orphan drugs approved by the FDA will not be modified in the future, or as to how any such changes might affect our products, if approved.

The FDA's and the MHRA's ability to review and approve new products may be hindered by a variety of factors, including budget and funding levels, government shutdowns, ability to hire and retain key personnel, and statutory, regulatory and policy changes.

The ability of the FDA and the MHRA to review and approve new products can be affected by a variety of factors, including budget and funding levels, government shutdowns, ability to hire and retain key personnel, and statutory, regulatory, and policy changes. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

The ability of the FDA, the MHRA and other government agencies to properly administer their functions is highly dependent on the levels of government funding and the ability to fill key leadership appointments, among various factors. Delays in filling or replacing key positions could significantly impact the ability of the FDA, the MHRA and other agencies to fulfill their functions and could greatly impact healthcare and the pharmaceutical industry.

In December 2016, the 21st Century Cures Act was signed into law, and was designed to advance medical innovation and empower the FDA with the authority to directly hire positions related to drug and device development and review. In the past, the FDA was often unable to offer key leadership candidates (including scientists) competitive compensation packages as compared to those offered by private industry. The 21st Century Cures Act is designed to streamline the agency's hiring process and enable the FDA to compete for leadership talent by expanding the narrow ranges that are provided in the existing compensation structures.

Disruptions at the FDA, the MHRA and other governmental agencies may also slow the time necessary for new drugs to be reviewed or approved by necessary government agencies, which would adversely affect our operating results and business.

Our operations and relationships with future customers, providers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties including criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with providers, third-party payors and customers will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any product candidates for which we obtain marketing approval.

Restrictions under applicable U.S. federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under government healthcare programs such as Medicare and Medicaid, and a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- federal false claims laws, including the federal False Claims Act, imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent (including claims for items and services resulting from a violation of the federal Anti-Kickback Statute) or making a false statement to avoid, decrease or conceal

an obligation to pay money to the federal government, and certain marketing practices, including off-label promotion, may also violate false claims laws;

•the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA") imposes criminal and civil liability for, among other things, knowingly and willfully executing or attempting to execute a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

•HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of people and entities with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

•the federal Physician Payment Sunshine Act requires applicable manufacturers of covered drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually payments and other transfers of value to physicians, physician assistants, certain types of advance practice nurses and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, such providers, and to report annually certain ownership and investment interests held by physicians and their immediate family, which includes annual data collection and reporting obligations; and

•analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

Some state and local laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers. Other state laws require pharmaceutical companies to report marketing expenditures or price increases that exceed a statutory threshold, as well as information on the reasons for the price increase, or to report the introduction into the market of costly drugs. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of product candidates from government-funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs.

Risks Related to Commercialization and Manufacturing

The commercial success of our product candidates, including zorevunersen and STK-002, will depend upon their degree of market acceptance by providers, patients, patient advocacy groups, third-party payors and the general medical community.

Ethical, social and legal concerns about genetic treatments generally could result in additional regulations restricting or prohibiting our product candidates. Even with the requisite approvals from the FDA, the MHRA, the EMA and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of providers, patients and third-party payors of drugs designed to increase protein expression in general, and our product candidates in particular, as medically necessary, cost-effective and safe. In addition, we may face challenges in seeking to establish and grow sales of zorevunersen, STK-002 and any future product candidates, including acceptance of intravitreal injection, the lumbar puncture and intrathecal administration, which carries risks of infection or other complications. Any product that we commercialize may not gain acceptance by providers, patients, patient advocacy groups, third-party payors and the general medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of genetic medicines and, in particular, zorevunersen, STK-002 and our future product candidates, if approved for commercial sale, will depend on several factors, including:

•the efficacy, durability and safety of such product candidates as demonstrated in clinical trials;

•the potential and perceived advantages of product candidates over alternative treatments;

•the cost of treatment relative to alternative treatments;

•the clinical indications for which the product candidate is approved by the FDA, the MHRA or the European Commission;

- the willingness of providers to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, MHRA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- the willingness of providers to prescribe, and of patients to receive, intrathecal injections;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the quality of our relationships with patient advocacy groups;
- publicity concerning our product candidates or competing products and treatments; and
- sufficient third-party payor coverage and adequate reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

The pricing, insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

Our target indications, including Dravet syndrome and ADOA, are indications with small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payors, the adoption of those product candidates and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect that coverage and reimbursement by third-party payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of zorevunersen, STK-002 and our future product candidates will depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement by government authorities for new products are typically made by the CMS since it decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. However, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. Further, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the EU, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payors, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of certain third-party payors, such as health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market. Recently there have been instances in which third-party payors have refused to reimburse treatments for patients for whom the treatment is indicated in the FDA-approved product label. Even if we are successful in obtaining FDA approvals to commercialize our product candidates, we cannot guarantee that we will be able to secure reimbursement for all patients for whom treatment with our product candidates is indicated.

In addition to CMS and private payors, professional organizations such as the American Medical Association can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payors contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more product candidates for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

If third parties on which we depend to conduct our planned preclinical studies, any future clinical trials, or manufacturing of our product candidates do not perform as contractually required, fail to satisfy regulatory or legal requirements or miss expected deadlines, our development program could be delayed with adverse effects on our business, financial condition, results of operations and prospects.

We rely on third parties for genetic testing, and on third-party CROs, CMOs, consultants and others to design, conduct, supervise and monitor key activities relating to, discovery, manufacturing, preclinical studies and clinical trials of our product candidates, and we intend to do the same for future activities relating to existing and future programs. Because we rely on third parties and do not have the ability to conduct all required testing, discovery, manufacturing, preclinical studies or clinical trials independently, we have less control over the timing, quality and other aspects of discovery, manufacturing, preclinical studies and clinical trials than we would if we conducted them on our own. These investigators, CROs, CMOs and consultants are not our employees and we have limited control over the amount of time and resources that they dedicate to our programs. These third parties may have contractual relationships with other entities, some of which may be our competitors, which may draw time and resources from our programs. The third parties we contract with might not be diligent, careful or timely in conducting our discovery, manufacturing, preclinical studies or clinical trials, resulting in testing, discovery, manufacturing, preclinical studies or clinical trials being delayed or unsuccessful, in whole or in part. In addition, these third parties may be subject to macroeconomic conditions, such as staffing shortages and supply chain or inflationary pressures that limit their ability to achieve anticipated timelines or result in a greater cost to us. For example, we are aware of a shortage of NHPs available for preclinical studies and although that is not expected to impact our current business, if we begin new product development programs we could be subject to longer development times or difficulty completing necessary research.

If we cannot contract with acceptable third parties on commercially reasonable terms, or at all, or if these third parties do not carry out their contractual duties, satisfy legal and regulatory requirements for the conduct of preclinical studies or clinical trials or meet expected deadlines, our clinical development programs could be delayed and otherwise adversely affected. In all events, we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the general investigational plan and protocols for the trial as well as regulatory requirements. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Any such event could have an adverse effect on our business, financial condition, results of operations and prospects.

We face significant competition in an environment of rapid technological change and it is possible that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business, financial condition and our ability to successfully market or commercialize zorevunersen, STK-002 and our future product candidates.

The biotechnology and pharmaceutical industries, including the genetic medicine and antisense oligonucleotide fields, are characterized by rapidly changing technologies, competition and a strong emphasis on intellectual property. We are aware of several companies focused on developing RNA-based treatments in various indications as well as several companies addressing other methods for modifying genes and regulating protein expression. We also expect to face competition from large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions.

Numerous treatments for epilepsy exist, including 5-HT agonists, such as UCB's Fintepla, cannabidiols, such as Jazz Pharmaceuticals' Epidiolex, GABA receptor agonists, such as clobazam and stiripentol, and glutamate blockers, which is one of the mechanisms of action of topiramate. In addition, numerous compounds are in clinical development for treatment of epilepsy. We believe the clinical

development pipeline includes cannabinoids, 5-HT release stimulants, cholesterol 24-hydroxylase inhibitors, potassium channel openers, and sodium channel agonists from a variety of companies. In addition to competition from these small molecule drugs, any products we may develop may also face competition from other types of therapies, such as gene therapy, gene editing, tRNA therapies, modified mRNA therapies or other ASO approaches. For example, one company (Encoded Therapeutics) has announced a clinical development plan for a gene regulation therapy in Dravet syndrome that may address the underlying genetic cause of the disease.

Although there are no approved treatments for ADOA at this time, we may also face potential competition in our ADOA program. For example, one company (PYC Therapeutics) has announced a clinical development plan for an RNA-based therapy in ADOA that may address the underlying genetic cause of the disease.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources than we do, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any product candidates that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market, if ever. Additionally, new or advanced technologies developed by our competitors may render our current or future product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

To become and remain profitable, we must develop and eventually commercialize product candidates with significant market potential, which will require us to be successful in a range of challenging activities. These activities include, among other things, completing preclinical studies and initiating and completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products that are approved and satisfying any post marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

The manufacture of drugs is complex and our third-party manufacturers may encounter difficulties in production. If any of our third-party manufacturers encounter such difficulties, our ability to provide supply of zorevunersen, STK-002 or our future product candidates for clinical trials, our ability to obtain marketing approval, or our ability to provide supply of our product candidates for patients, if approved, could be delayed or stopped.

We have established manufacturing relationships with a limited number of suppliers to manufacture raw materials and the drug substance of any product candidate for which we are responsible for preclinical or clinical development. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain. As part of any marketing approval, a manufacturer and its processes are required to be qualified by the FDA prior to commercialization. If supply from the approved vendor is interrupted, there could be a significant disruption in commercial supply. An alternative vendor would need to be qualified through an NDA supplement which could result in further delay. The FDA or other regulatory agencies outside of the United States may also require additional studies if a new supplier is relied upon for the manufacture of clinical trial materials or commercial production. Switching vendors may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

The process of manufacturing drugs is complex, highly-regulated and subject to multiple risks. Manufacturing drugs is highly susceptible to product loss due to contamination, equipment failure, improper installation or operation of equipment, vendor or operator error, inconsistency in yields, variability in product characteristics and difficulties in scaling the production process. Even minor deviations from normal manufacturing processes could result in reduced production yields, product defects and other supply disruptions. If microbial, viral or other contaminations are discovered at the facilities of our manufacturers, such facilities may need to be closed for an extended period of time to investigate and remedy the contamination, which could delay clinical trials and adversely harm our business. Moreover, if the FDA determines that our manufacturers are not in compliance with FDA laws and regulations, including those governing CGMPs, the FDA may deny NDA approval until the deficiencies are corrected or we replace the manufacturer in our NDA with a manufacturer that is in compliance.

In addition, there are risks associated with large scale manufacturing for clinical trials or commercial scale including, among others, cost overruns, potential problems with process scale-up, process reproducibility, stability issues, compliance with good manufacturing practices, lot consistency and timely availability of raw materials. Even if we or our collaborators obtain regulatory approval for any of our product candidates, there is no assurance that manufacturers will be able to manufacture the approved product to specifications acceptable to the FDA or other regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential

launch of the product or to meet potential future demand. If our manufacturers are unable to produce sufficient quantities for clinical trials or for commercialization, research and commercialization efforts would be impaired, which would have an adverse effect on our business, financial condition, results of operations and prospects.

Our reliance on a limited number of manufacturers, the complexity of drug manufacturing and the difficulty of scaling up a manufacturing process could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our product candidates successfully. Furthermore, if our suppliers fail to deliver the required commercial quantities of materials on a timely basis and at commercially reasonable prices, and we are unable to secure one or more replacement suppliers capable of production in a timely manner at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell zorevunersen, STK-002 and our future product candidates, we may be unable to generate any revenues.

We currently do not have an organization for the sales, marketing and distribution of zorevunersen, STK-002 and our future product candidates and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. To market any products that may be approved, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. With respect to certain of our current programs as well as future programs, we may rely completely on an alliance partner for sales and marketing. In addition, although we intend to establish a sales organization if we are able to obtain approval to market any product candidates, we may enter into strategic alliances with third parties to develop and commercialize zorevunersen, STK-002 and other future product candidates, including in markets outside of the United States or for other large markets that are beyond our resources. This will reduce the revenue generated from the sales of these products.

Any future strategic alliance partners may not dedicate sufficient resources to the commercialization of our product candidates or may otherwise fail in their commercialization due to factors beyond our control. If we are unable to establish effective alliances to enable the sale of our product candidates to healthcare professionals and in geographical regions, including the United States, that will not be covered by our own marketing and sales force, or if our potential future strategic alliance partners do not successfully commercialize the product candidates, our ability to generate revenues from product sales will be adversely affected.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate sufficient product revenue and may not become profitable. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to timely capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We have entered into a collaboration with Acadia Pharmaceuticals and may, in the future, seek to enter into collaborations with other third parties for the discovery, development and commercialization of our product candidates. If our collaborators cease development efforts under our collaboration agreements, or if any of those agreements are terminated, these collaborations may fail to lead to commercial products and we may never receive milestone payments or future royalties under these agreements.

We have entered into a collaboration with Acadia Pharmaceuticals to discover or develop certain novel RNA-based medicines for the potential treatment of severe and rare genetic neurodevelopmental diseases of the central nervous system. The collaboration includes SYNGAP1 syndrome, Rett syndrome (MECP2), and an undisclosed neurodevelopmental target of mutual interest, and such collaboration could represent a significant portion of our product pipeline. We may derive a significant portion of our future revenue from these agreements or other similar agreements into which we may enter in the future. Revenue from research and development collaborations depends upon continuation of the collaborations, payments for research and development services and resulting options to acquire any licenses of successful product candidates, and the achievement of milestones, contingent payments and royalties, if any, derived from future products developed from our research.

Collaborations involving our product candidates currently pose, and will continue to pose, the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;

•collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on preclinical studies or clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors such as an acquisition that diverts resources or creates competing priorities;

•collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;

•collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

•collaborators with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;

•collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation or potential liability;

•collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;

•disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and

•collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

As a result of the foregoing, our current and any future collaboration agreements may not lead to development or commercialization of our product candidates in the most efficient manner or at all. If a collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated. Any failure to successfully develop or commercialize our product candidates pursuant to our current or any future collaboration agreements could have a material and adverse effect on our business, financial condition, results of operations and prospects.

Moreover, to the extent that any of our existing or future collaborators were to terminate a collaboration agreement, we may be forced to independently develop these product candidates, including funding preclinical studies or clinical trials, assuming marketing and distribution costs and defending intellectual property rights, or, in certain instances, abandon product candidates altogether, any of which could result in a change to our business plan and have a material adverse effect on our business, financial condition, results of operations and prospects.

We may not be successful in finding strategic collaborators for continuing development of certain of our future product candidates or successfully commercializing or competing in the market for certain indications.

In the future, we may decide to collaborate with non-profit organizations, universities, pharmaceutical and biotechnology companies for the development and potential commercialization of existing and new product candidates. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing drugs, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or

marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

The success of any potential collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of such collaboration arrangements. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

Risks Related to our Financial Position

We have a history of operating losses, and we may not achieve or sustain profitability. We anticipate that we will continue to incur losses for the foreseeable future. If we fail to obtain additional funding to conduct our planned research and development effort, we could be forced to delay, reduce or eliminate our product development programs or commercial development efforts.

We are an early-stage biotechnology company with a limited operating history on which to base your investment decision. Biotechnology product development is a highly speculative undertaking and involves a substantial degree of risk. Our operations to date have been limited primarily to organizing and staffing our company, business planning, raising capital, acquiring and developing product and technology rights, manufacturing, and conducting research and development activities for our product candidates. We have never generated any revenue from product sales. We have not obtained regulatory approvals for any of our product candidates, and have funded our operations to date through proceeds from sales of our preferred stock and common stock.

We have incurred net losses in each year since our inception. We incurred net losses of $25.7 million and $30.7 million, for the three months ended June 30, 2024 and 2023, respectively. As of June 30, 2024, we had accumulated deficits of $453.9 million. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant expenses and operating losses over the next several years and for the foreseeable future as we intend to continue to conduct research and development, clinical testing, regulatory compliance activities, manufacturing activities, and, if any of our product candidates is approved, sales and marketing activities that, together with anticipated general and administrative expenses, will likely result in us incurring significant losses for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We expect that we will need to raise additional funding before we can expect to become profitable from any potential future sales of zorevunersen, STK-002 or our future product candidates. This additional financing may not be available on acceptable terms or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We will require substantial future capital in order to complete planned and future preclinical and clinical development for zorevunersen, STK-002 and other future product candidates, if any, and potentially commercialize these product candidates. Based upon our current operating plan, we believe that our cash, cash equivalents and marketable securities of $282.0 million as of June 30, 2024 will enable us to fund our operating expenses and capital expenditure requirements at least to the end of 2025. We expect our spending levels to increase in connection with our preclinical studies and clinical trials of our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to commercial launch, product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Additional capital might not be available when we need it and our actual cash requirements might be greater than anticipated. If we require additional capital at a time when investment in our industry or in the marketplace in general is limited, we might not be able to raise funding on favorable terms if at all. If we are not able to obtain financing on terms favorable to us, we may need to cease or reduce development or commercialization activities, sell some or all of our assets or merge with another entity, which could result in a loss of all or part of your investment.

Our future capital requirements will depend on many factors, including:

- the costs associated with the scope, progress and results of discovery, preclinical development, laboratory testing and clinical trials for our product candidates;

- the costs associated with the development of our internal manufacturing facility and processes;
- the costs related to the extent to which we enter into partnerships or other arrangements with third parties to further develop our product candidates;
- the costs and fees associated with the discovery, acquisition or in-license of product candidates or technologies;
- our ability to establish collaborations on favorable terms, if at all;
- the costs of future commercialization activities, if any, including product sales, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval; and
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims.

Our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of product candidates that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives, which may not be available to us on acceptable terms, or at all.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are a clinical stage biotechnology company formed in June 2014. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring our technology, identifying potential product candidates, undertaking research, preclinical and clinical development of our product candidates, manufacturing, and establishing licensing arrangements. We have not yet demonstrated the ability to complete clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial scale product or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a licensing and research focus to a company that is also capable of supporting clinical development and commercial activities. We may not be successful in such a transition.

Our ability to utilize our net operating loss carryforwards may be subject to limitations.

We have incurred substantial losses during our history. We do not expect to be profitable soon and may never achieve profitability. As of December 31, 2023, we had federal and state net operating loss carryforwards, or NOLs, of approximately $189.5 million and $199.4 million, respectively, and as of December 31, 2022, we had federal and state NOLs of approximately $210.9 million and $212.8 million, respectively. Our pre-2018 NOLs expire at various dates beginning in 2034. In general, NOLs generated in and after 2018 have no expiration. To the extent that we continue to generate NOLs, unused NOLs carry forward to offset future taxable income until such NOLs expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986 (“IRC“), as amended, or the Code, if a corporation undergoes an “ownership change,” generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change NOLs and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. The Company recently performed an IRC 382 study and identified ownership changes in prior years. Based on existing Section 382 limitations, $0.9 million of the existing federal NOL will not be utilizable due to restrictive limitations. We may experience additional ownership changes in the future because of subsequent shifts in our stock ownership. As a result, our ability to use our pre-change NOLs to offset U.S. federal taxable income, if any, is subject to limitations, which could potentially result in increased future tax liability. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

U.S. federal income tax reform and changes in other tax laws could adversely affect us.

In December 2017, U.S. federal tax legislation, commonly referred to as the Tax Cuts and Jobs Act (the “TCJA”) was signed into law, significantly reforming the Code. The TCJA, among other things, includes changes to U.S. federal tax rates, imposes significant additional limitations on the deductibility of business interest, allows for the expensing of capital expenditures, puts into effect the migration from a “worldwide” system of taxation to a partial “territorial” system, and modifies or repeals many business deductions and credits. Beginning in 2022, the TCJA also eliminated the option to immediately deduct research and development expenditures and required taxpayers to amortize domestic expenditures over five years and foreign expenditures over fifteen years.

We continue to examine the impact the TCJA may have on our business. The TCJA is a far-reaching and complex revision to the U.S. federal income tax laws with disparate and, in some cases, countervailing impacts on different categories of taxpayers and industries,

and will require subsequent rulemaking and interpretation in a number of areas. The long-term impact of the TCJA on the overall economy, the industries in which we operate and our and our partners' businesses cannot be reliably predicted at this early stage of the new law's implementation. There can be no assurance that the TCJA will not negatively impact our operating results, financial condition, and future business operations. The estimated impact of the TCJA is based on our management's current knowledge and assumptions, following consultation with our tax advisors.

Because of our valuation allowance in the U.S., ongoing tax effects of the TCJA are not expected to materially change our effective tax rate in future periods.

Risks Related to our Intellectual Property

Our success depends in part on our ability to obtain, maintain and protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.

Our commercial success will depend in large part on obtaining and maintaining patent, trademark, trade secret and other intellectual property protection of our proprietary technologies and product candidates, which include TANGO, zorevunersen, STK-002 and the additional gene targets identified by TANGO, their respective components, formulations, combination therapies, methods used to manufacture them and methods of treatment, as well as successfully defending our patents and other intellectual property rights against third-party challenges. Our ability to stop unauthorized third parties from making, using, selling, offering to sell, importing or otherwise commercializing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. If we are unable to secure and maintain patent protection for any product or technology we develop, or if the scope of the patent protection secured is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to commercialize any product candidates we may develop may be adversely affected. The patenting process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not pursue or obtain patent protection in all relevant markets. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from or license to third parties and are reliant on our licensors or licensees to do so. Our pending and future patent applications may not result in issued patents. Even if patent applications we license or own currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we hold or in-license may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our platform advances and product candidates will be protectable or remain protected by valid and enforceable patents. In addition, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technologies.

We depend on intellectual property licensed from third parties, and our licensors may not always act in our best interest. If we fail to comply with our obligations under our intellectual property licenses, if the licenses are terminated, or if disputes regarding these licenses arise, we could lose significant rights that are important to our business.

We are dependent on patents, know-how and proprietary technology licensed from others. Our licenses to such patents, know-how and proprietary technology may not provide exclusive rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our products in the future. The agreements under which we license patents, know-how and proprietary technology from others are complex, and certain provisions in such agreements may be susceptible to multiple interpretations.

For example, we are a party to a license agreement with the University of Southampton, pursuant to which we in-license key patents and patent applications for our TANGO platform, zorevunersen, STK-002 and our future product candidates. For more information regarding the agreement, please see "Business—License and research agreements." The agreement imposes various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, our licensor may have the right to terminate our license, in which event we would not be able to develop or market our TANGO platform, zorevunersen, STK-002 or any other technology or product candidates covered by the intellectual property licensed under the agreement. In addition, we may need to obtain additional licenses from our existing licensor and others to advance our research or allow commercialization of product candidates we may develop. It is possible that we may be unable to obtain any additional licenses at a reasonable cost or on reasonable terms, if at all. In either event, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected technology or product candidates.

If we or our existing or future licensors fail to adequately protect our licensed intellectual property, our ability to commercialize product candidates could suffer. We do not have complete control over the maintenance, prosecution and litigation of our in-licensed patents and patent applications and may have limited control over future intellectual property that may be in-licensed. For example, we cannot be certain that activities such as the maintenance and prosecution by our existing or future licensors have been or will be

conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. It is possible that our existing or future licensors' infringement proceedings or defense activities may be less vigorous than had we conducted them ourselves or may not be conducted in accordance with our best interests.

Furthermore, inventions contained within some of our existing or future in-licensed patents and patent applications may be made using U.S. government funding or other non-governmental funding. We rely on our existing or future licensors to ensure compliance with applicable obligations arising from such funding, such as timely reporting, an obligation associated with in-licensed patents and patent applications. The failure of our existing or future licensors to meet their obligations may lead to a loss of rights or the unenforceability of relevant patents. For example, the government could have certain rights in such in-licensed patents, including a non-exclusive license authorizing the government to use the invention or to have others use the invention on its behalf for non-commercial purposes. If the U.S. government then decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. These rights may also permit the government to exercise march-in rights to use or allow third parties to use the technology covered by such in-licensed patents. The government may also exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such in-licensed government-funded inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations, and prospects significantly.

In addition, the resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant patents, know-how and proprietary technology, or increase what we believe to be our financial or other obligations under the relevant agreement. Disputes that may arise between us and our existing or future licensors regarding intellectual property subject to a license agreement could include disputes regarding:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected technology or product candidates. As a result, any termination of or disputes over our intellectual property licenses could result in the loss of our ability to develop and commercialize our TANGO platform, zorevunersen, or STK-002, or we could lose other significant rights, any of which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Moreover, our agreements with certain of our third-party research partners provide that improvements developed in the course of our relationship may be owned solely by either us or our third-party research partner, or jointly between us and the third party. If we determine that rights to such improvements owned solely by a research partner or other third party with whom we collaborate are necessary to commercialize our drug candidates or maintain our competitive advantage, we may need to obtain a license from such third party in order to use the improvements and continue developing, manufacturing or marketing our drug candidates. We may not be able to obtain such a license on an exclusive basis, on commercially reasonable terms, or at all, which could prevent us from commercializing our drug candidates or allow our competitors or others the chance to access technology that is important to our business. We also may need the cooperation of any co-owners of our intellectual property in order to enforce such intellectual property against third parties, and such cooperation may not be provided to us.

Our owned and in-licensed patents and patent applications may not provide sufficient protection of our TANGO platform, our zorevunersen and STK-002 product candidates, and our future product candidates or result in any competitive advantage.

We own multiple issued U.S. and foreign patents covering zorevunersen and related compositions, the mechanism of action and use of zorevunersen for treating diseases, as well as multiple pending U.S., PCT international, and foreign patent applications covering zorevunersen and related compositions, and the mechanism of action and use of zorevunersen for treating diseases. We have also in-licensed multiple issued U.S. and foreign patents that cover the mechanism of action of zorevunersen, use of the mechanism for treating diseases, and related compositions. With respect to STK-002, we have applied for and are currently pursuing patent protection for the mechanism of action of STK-002, compositions related to STK-002, and uses of those compositions in several economically significant countries. We own multiple U.S. and foreign patents covering STK-002 and related compositions. We also own a pending PCT international application and numerous pending U.S. and foreign patent applications covering STK-002 and related

compositions, mechanism of action and use of STK-002 for treating diseases. Furthermore, our in-licensed issued U.S. and foreign patents (mentioned above) cover the mechanism of action of STK-002. We cannot be certain that any of the issued patents we currently own or in-license will adequately protect zorevunersen, STK-002 and other programs or that they will not be challenged, narrowed, circumvented, invalidated or held unenforceable. We also cannot be certain that any of the pending patent applications will issue as patents, and if they do, that such patents will cover or adequately protect zorevunersen, STK-002 and other programs or that such patents will not be challenged, narrowed, circumvented, invalidated or held unenforceable.

In addition to claims directed toward the technology underlying our TANGO platform, our owned and in-licensed patents and patent applications contain claims directed to compositions of matter on the active pharmaceutical ingredients ("APIs") in our product candidates, as well as methods-of-use directed to the use of an API for a specified treatment. Composition-of-matter patents on the active pharmaceutical ingredient in prescription drug products provide protection without regard to any particular method of use of the API used. Method-of-use patents do not prevent a competitor or other third party from developing or marketing an identical product for an indication that is outside the scope of the patented method. Moreover, with respect to method-of-use patents, even if competitors or other third parties do not actively promote their product for our targeted indications or uses for which we may obtain patents, providers may recommend that patients use these products off-label, or patients may do so themselves. Although off-label use may infringe or contribute to the infringement of method-of-use patents, the practice is common, and this type of infringement is difficult to prevent or prosecute.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries. For example, while our patent applications are pending, we may be subject to a third party preissuance submission of prior art to the United States Patent and Trademark Office (the "USPTO") or become involved in interference or derivation proceedings, or equivalent proceedings in foreign jurisdictions. Even if patents do successfully issue, third parties may challenge their inventorship, validity, enforceability or scope, including through opposition, revocation, reexamination, post-grant and *inter partes* review proceedings. An adverse determination in any such submission, proceeding or litigation may result in loss of patent rights, loss of exclusivity, or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property or prevent others from designing around our claims. Moreover, some of our owned and in-licensed patents and patent applications may be co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us. If the breadth or strength of protection provided by the patent applications we hold with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in development, testing, and regulatory review of new product candidates, the period of time during which we could market our product candidates under patent protection would be reduced.

Since patent applications in the United States and other countries are confidential for a period of time after filing, at any moment in time, we cannot be certain that we were in the past or will be in the future the first to file any patent application related to our product candidates. In addition, some patent applications in the United States may be maintained in secrecy until the patents are issued. As a result, there may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim, and we may be subject to priority disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that, if challenged, our patents would be declared by a court, patent office or other governmental authority to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, that block our efforts or potentially result in our product candidates or our activities infringing such claims. It is possible that our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Those patent applications may have priority over our owned and in-licensed patent applications or patents, which could require us to obtain rights to issued patents covering such technologies. The possibility also exists that others will develop products that have the same effect as our product candidates on an independent basis that do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our product candidates.

Likewise, our currently owned and in-licensed patents and patent applications, if issued as patents, directed to our proprietary technologies and our product candidates are expected to expire from 2035 through 2045, without taking into account any possible patent term adjustments or extensions. Our earliest in-licensed patents may expire before, or soon after, our first product achieves

marketing approval in the United States or foreign jurisdictions. Additionally, we cannot be assured that the USPTO or relevant foreign patent offices will grant any of the pending patent applications we own or in-license currently or in the future. Upon the expiration of our current patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a similar material adverse effect on our business, financial condition, results of operations and prospects.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make or use compounds that are similar to the active compositions of our product candidates but that are not covered by the claims of our patents;
- the active pharmaceutical ingredients in our current product candidates will eventually become commercially available in generic drug products, and no patent protection may be available with regard to formulation or method of use;
- we or our licensors, as the case may be, may fail to meet our obligations to the U.S. government regarding any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss or unenforceability of patent rights;
- we or our licensors, as the case may be, might not have been the first to file patent applications for certain inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our owned or in-licensed patents, as the case may be, or parts of our owned or in-licensed patents;
- it is possible that others may circumvent our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our product candidates or technology similar to ours;
- the laws of foreign countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- our owned or in-licensed issued patents may not provide us with any competitive advantages, may be narrowed in scope, or be held invalid or unenforceable as a result of legal challenges by third parties;
- the inventors of our owned or in-licensed patents or patent applications may become involved with competitors, develop products or processes that design around our patents, or become hostile to us or the patents or patent applications on which they are named as inventors;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) that should be listed as inventor(s) or include individual(s) that should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- we have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or diagnostic tests we develop may be covered by third parties' patents or other exclusive rights; or
- the patents of others may have an adverse effect on our business.

Any of the foregoing could have a material adverse effect on our business, financial conditions, results of operations and prospects.

Our strategy of obtaining rights to key technologies through in-licenses may not be successful.

We seek to expand our product candidate pipeline in part by in-licensing the rights to key technologies, including those related to specific gene targets which may be upregulated by TANGO. The future growth of our business will depend in part on our ability to in-license or otherwise acquire the rights to additional product candidates and technologies. Although we have succeeded in licensing technologies from the University of Southampton in the past, we cannot assure you that we will be able to in-license or acquire the rights to any product candidates or technologies from third parties on acceptable terms or at all.

For example, our agreements with certain of our third-party research partners provide that improvements developed in the course of our relationship may be owned solely by either us or our third-party research partner, or jointly between us and the third party. If we determine that exclusive rights to such improvements owned solely by a research partner or other third party with whom we collaborate are necessary to commercialize our drug candidates or maintain our competitive advantage, we may need to obtain an exclusive license from such third party in order to use the improvements and continue developing, manufacturing or marketing our drug candidates. We may not be able to obtain such a license on an exclusive basis, on commercially reasonable terms, or at all, which could prevent us from commercializing our drug candidates or allow our competitors or others the opportunity to access technology that is important to our business. We also may need the cooperation of any co-owners of our intellectual property in order to enforce such intellectual property against third parties, and such cooperation may not be provided to us.

In addition, the in-licensing and acquisition of these technologies is a highly competitive area, and a number of more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our area of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business and prospects could be materially and adversely affected.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to patent protection, we rely upon know-how and trade secret protection, as well as non-disclosure agreements and invention assignment agreements with our employees, consultants and third-parties, to protect our confidential and proprietary information, especially where we do not believe patent protection is appropriate or obtainable.

It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties, except in certain specified circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and that are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In the case of consultants and other third parties, the agreements provide that all inventions conceived in connection with the services provided are our exclusive property. However, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We have also adopted policies and conduct training that provides guidance on our expectations, and our advice for best practices, in protecting our trade secrets. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches.

In addition to contractual measures, we try to protect the confidential nature of our proprietary information through other appropriate precautions, such as physical and technological security measures. However, trade secrets and know-how can be difficult to protect. These measures may not, for example, in the case of misappropriation of a trade secret by an employee or third party with authorized access, provide adequate protection for our proprietary information. Our security measures may not prevent an employee or consultant from misappropriating our trade secrets and providing them to a competitor, and any recourse we might take against this type of misconduct may not provide an adequate remedy to protect our interests fully. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, trade secrets may be independently developed by others in a manner that could prevent us from receiving legal recourse. If any of our confidential or proprietary information, such as our trade secrets, were to be disclosed or misappropriated, or if any of that information was independently developed by a competitor, our competitive position could be harmed.

In addition, courts outside the United States are sometimes less willing to protect trade secrets. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. Even if we are successful, these types of lawsuits may consume our time and other resources. Although we take steps to protect our proprietary information and trade secrets, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. As a result, we may not be able to meaningfully protect our trade secrets. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Third-party claims of intellectual property infringement may prevent, delay or otherwise interfere with our product discovery and development efforts.

Our commercial success depends in part on our ability to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property or proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation, *inter*

partes review, post grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates and/or proprietary technologies infringe, misappropriate or otherwise violate their intellectual property rights. Numerous U.S. and foreign issued patents and pending patent applications that are owned by third parties, such as Ionis Pharmaceuticals, exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our field, third parties may allege they have patent rights encompassing our product candidates, technologies or methods.

If a third party claims that we infringe, misappropriate or otherwise violate its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property claims that, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business;
- substantial damages for infringement, which we may have to pay if a court decides that the product candidate or technology at issue infringes on or violates the third party's rights, and, if the court finds that the infringement was willful, we could be ordered to pay treble damages plus the patent owner's attorneys' fees;
- a court prohibiting us from developing, manufacturing, marketing or selling our product candidates, or from using our proprietary technologies, unless the third party licenses its product rights to us, which it is not required to do, on commercially reasonable terms or at all;
- if a license is available from a third party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our product candidates;
- the requirement that we redesign our product candidates or processes so they do not infringe, which may not be possible or may require substantial monetary expenditures and time; and
- there could be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, financial condition, results of operations and prospects.

Third parties may assert that we are employing their proprietary technology without authorization, including by enforcing its patents against us by filing a patent infringement lawsuit against us. In this regard, patents issued in the United States by law enjoy a presumption of validity that can be rebutted only with evidence that is "clear and convincing," a heightened standard of proof.

There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents.

If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, or materials used in or formed during the manufacturing process, or any final product itself, the holders of those patents may be able to block our ability to commercialize our product candidate unless we obtain a license under the applicable patents, or until those patents were to expire or those patents are finally determined to be invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy or patient selection methods, the holders of that patent may be able to block our ability to develop and commercialize the product candidate unless we obtain a license or until such patent expires or is finally determined to be invalid or unenforceable. In either case, a license may not be available on commercially reasonable terms, or at all, particularly if such patent is owned or controlled by one of our primary competitors. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could significantly harm our business. Even if we obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve

substantial litigation expense and would be a substantial diversion of employee time and resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any license of this nature would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates and we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could significantly harm our business.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful and could result in a finding that such patents are unenforceable or invalid.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that one or more of our patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question.

In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. These types of mechanisms include re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). These types of proceedings could result in revocation or amendment to our patents such that they no longer cover our product candidates. The outcome for any particular patent following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, or if we are otherwise unable to adequately protect our rights, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Defense of these types of claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business.

Conversely, we may choose to challenge the patentability of claims in a third party's U.S. patent by requesting that the USPTO review the patent claims in re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings), or we may choose to challenge a third party's patent in patent opposition proceedings in the European Patent Office (the "EPO") or another foreign patent office. Even if successful, the costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, the EPO or other patent office then we may be exposed to litigation by a third party alleging that the patent may be infringed by our product candidates or proprietary technologies.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, that perception could have a substantial adverse effect on the price of our common stock. Any of the foregoing could have a material adverse effect on our business financial condition, results of operations and prospects.

We have limited foreign intellectual property rights and may not be able to protect our intellectual property rights throughout the world.

We have limited intellectual property rights outside the United States. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as that in the United States. These products may compete with our product candidates in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biopharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products against third parties in violation of our proprietary rights generally. The initiation of proceedings by third parties to challenge the scope or validity of our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other

aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Geopolitical actions in the United States and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors. For example, the United States and foreign government actions related to Russia's invasion of Ukraine may limit or prevent filing, prosecution and maintenance of patent applications in Russia. Government actions may also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in Russia. In addition, a decree was adopted by the Russian government in March 2022, allowing Russian companies and individuals to exploit inventions owned by patentees that have citizenship or nationality in, are registered in, or have predominately primary place of business or profit-making activities in the United States and other countries that Russia has deemed unfriendly without consent or compensation. Consequently, we would not be able to prevent third parties from practicing our inventions in Russia or from selling or importing products made using our inventions in and into Russia. Similarly, the ongoing conflict in Israel could result in regulatory delays or the inability to secure intellectual property or commercialize our products there. Accordingly, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Our use of open source software could impose limitations on our ability to commercialize our product candidates.

Our use of open source software could impose limitations on our ability to commercialize our product candidates. Our technology utilizes open source software that contains modules licensed for use from third-party authors under open source licenses. In particular, some of the software that powers TANGO may be provided under license arrangements that allow use of the software for research or other non-commercial purposes. As a result, in the future, as we seek to use our platform in connection with commercially available products, we may be required to license that software under different license terms, which may not be possible on commercially reasonable terms, if at all. If we are unable to license software components on terms that permit its use for commercial purposes, we may be required to replace those software components, which could result in delays, additional cost and additional regulatory approvals.

Use and distribution of open source software may entail greater risks than use of third-party commercial software, as open source licensors generally do not provide warranties or other contractual protections regarding infringement claims or the quality of the software code. Some open source licenses contain requirements that we make available source code for modifications or derivative works we create based upon the type of open source software we use. If we combine our proprietary software with open source software in a certain manner, we could, under certain of the open source licenses, be required to release the source code of our proprietary software to the public. This could allow our competitors to create similar products with lower development effort and time, and ultimately could result in a loss of product sales for us. Although we monitor our use of open source software, the terms of many open source licenses have not been interpreted by U.S. courts, and there is a risk that those licenses could be construed in a manner that could impose unanticipated conditions or restrictions on our ability to commercialize our product candidates. We could be required to seek licenses from third parties in order to continue offering our product candidates, to re-engineer our product candidates or to discontinue the sale of our product candidates in the event re-engineering cannot be accomplished on a timely basis, any of which could materially and adversely affect our business, financial condition, results of operations and prospects.

Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets.

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at universities or other biopharmaceutical or pharmaceutical companies, including our competitors or potential competitors. Although no misappropriation or improper disclosure claims against us are currently pending, and although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. We may then have to pursue litigation to defend against these claims. If we fail in defending any claims of this nature in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against these types of claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, that perception could have a substantial adverse effect on the price of our common stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities, and we may not have sufficient financial or other resources to adequately conduct this type of litigation or proceedings. For example, some of our competitors may be able to sustain the costs of this type of litigation or proceedings more effectively than we can because of their substantially greater financial resources. In any case, uncertainties resulting from the initiation and continuation of intellectual property litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

The growth of our business may depend in part on our ability to acquire, in-license or use third-party proprietary rights. For example, our product candidates may require specific formulations to work effectively and efficiently, we may develop product candidates containing our compounds and pre-existing pharmaceutical compounds, or we may be required by the FDA or comparable foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates, any of which could require us to obtain rights to use intellectual property held by third parties. In addition, with respect to any patents we may co-own with third parties, we may require licenses to such co-owners interest to such patents. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary or important to our business operations. In addition, we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. Were that to happen, we may need to cease use of the compositions or methods covered by those third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on those intellectual property rights, which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license, it may be non-exclusive, which means that our competitors may also receive access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Additionally, we sometimes collaborate with academic institutions to accelerate our preclinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Even if we hold such an option, we may be unable to negotiate a license from the institution within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program.

The licensing and acquisition of third-party intellectual property rights is a competitive area, and companies that may be more established or have greater resources than we do may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. There can be no assurance that we will be able to successfully complete these types of negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to develop or market. If we are unable to successfully obtain rights to required third-party intellectual property or to maintain the existing intellectual property rights we have, we may have to abandon development of certain programs and our business financial condition, results of operations and prospects could suffer.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign patent agencies also require compliance with a number of procedural, documentary, fee payment and other provisions during the patent application process and following the issuance of a patent. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. Were a noncompliance event to occur, our competitors might be able to enter the market, which would have a material adverse effect on our business financial condition, results of operations and prospects.

Changes in patent law in the United States and in non-U.S. jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain.

Past or future patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. For example, in March 2013, under the Leahy-Smith America Invents Act (the "America Invents Act") the United States moved from a "first to invent" to a "first-to-file" patent system. Under a "first-to-file" system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. The America Invents Act includes a number of other significant changes to U.S. patent law, including provisions that affect the way patent applications are prosecuted, redefine prior art and establish a new post-grant review system. The effects of these changes are currently

unclear as the USPTO continues to promulgate new regulations and procedures in connection with the America Invents Act and many of the substantive changes to patent law, including the “first-to-file” provisions, only became effective in March 2013. In addition, the courts have yet to address many of these provisions and the applicability of the act and new regulations on the specific patents discussed in this filing have not been determined and would need to be reviewed. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

Additionally, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the case, Assoc. for Molecular Pathology v. Myriad Genetics, Inc., the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of our owned or in-licensed patents will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents. Any similar adverse changes in the patent laws of other jurisdictions could also have a material adverse effect on our business, financial condition, results of operations and prospects.

Additionally, starting from June 1, 2023, European applications have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unitary Patent Court (“UPC”). This is a significant change in European patent practice. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty of any litigation.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting our product candidates might expire before or shortly after we or our partners commercialize those candidates. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984 (the “Hatch-Waxman Amendments”). The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent per product may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, even if we were to seek a patent term extension, it may not be granted because of, for example, the failure to exercise due diligence during the testing phase or regulatory review process, the failure to apply within applicable deadlines, the failure to apply prior to expiration of relevant patents, or any other failure to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

We are subject to a variety of privacy and data security laws, and our failure to comply with them could harm our business.

We maintain a large quantity of sensitive information, including confidential business and patient health information in connection with our preclinical studies, and are subject to laws and regulations governing the privacy and security of such information. In the United States, there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure and protection of personal information, including federal and state health information privacy laws, federal and state security breach notification laws, and federal and state consumer protection laws. Each of these laws is subject to varying interpretations and constantly evolving. In May 2018, a new privacy regime, the General Data Protection Regulation (the “GDPR”) took effect in the European Economic Area (the “EEA”) and the United Kingdom. The GDPR governs the collection, use, disclosure, transfer or other processing of personal data of European and United Kingdom persons. The GDPR continues to form part of law in the United Kingdom with some amendments following Brexit (“UK GDPR”), although there is a risk of divergence in the future which may increase our overall data protection compliance cost. Among other things, the GDPR and UK GDPR impose new requirements regarding the security of personal data and notification of data processing obligations to the competent national data processing authorities, changes the lawful bases on which personal data can be processed, expands the definition of personal data and requires changes to informed consent practices, as well as more detailed notices for clinical trial subjects and investigators. In addition, the GDPR and UK GDPR increase the scrutiny of transfers of personal data from clinical trial sites located in the EEA and the United Kingdom to the United States and other jurisdictions that the European Commission or the United Kingdom do not recognize as having “adequate” data protection laws, and imposes substantial fines for breaches and violations (up to the greater of €20 million or 4% of our consolidated annual worldwide gross revenue). The GDPR and UK GDPR also confer a private right of

action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR or UK GDPR.

More recently, the SEC has enacted regulations requiring companies to disclose or otherwise provide notifications regarding data security breaches. For example, the SEC recently adopted cybersecurity risk management and disclosure rules, which require the disclosure of information pertaining to cybersecurity incidents and cybersecurity risk management, strategy and governance. Compliance with these and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. If we fail to comply with any such laws or regulations, we may face significant fines and penalties that could adversely affect our business, financial condition and results of operations. Compliance with these and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. If we fail to comply with any such laws or regulations, we may face significant fines and penalties that could adversely affect our business, financial condition and results of operations.

Risks Related to Employee Matters, Managing Growth and Other Risks Related to our Business

We expect to expand our development and regulatory capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of product candidate development and growing our capability to conduct clinical trials. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

We must attract and retain highly skilled employees to succeed.

To succeed, we must recruit, retain, manage and motivate qualified clinical, scientific, technical and management personnel, and we face significant competition for experienced personnel. If we do not succeed in attracting and retaining qualified personnel, particularly at the management level, it could adversely affect our ability to execute our business plan, harm our results of operations and increase our capabilities to successfully commercialize zorevunersen, STK-002 and our future product candidates. In particular, we believe that our future success is highly dependent upon the contributions of our senior management, including Edward M. Kaye, our Chief Executive Officer, as well as our senior scientists and other members of our senior management team. The loss of services of one or more of these individuals, who all have at-will employment arrangements with us, could delay or prevent the successful development of our product pipeline, completion of our planned clinical trials or the commercialization of our product candidates, if approved. The competition for qualified personnel in the biotechnology field is intense and as a result, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel.

Many of the other biotechnology companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates than what we have to offer. If we are unable to continue to attract and retain high-quality personnel, the rate and success at which we can discover and develop product candidates and our business will be limited.

Future acquisitions or strategic alliances could disrupt our business and harm our financial condition and results of operations.

We may acquire additional businesses or drugs, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new drugs resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with strategic partners as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into our organization;

•the need to implement or improve controls, procedures, and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;

•liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities, and other known liabilities;

•unanticipated write-offs or charges; and

•litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or results of operations.

If we fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We will become subject to numerous environmental, health, and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment, and disposal of hazardous materials and wastes. Our operations will involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also may produce hazardous waste products. We generally anticipate contracting with third parties for the disposal of these materials and wastes. We will not be able to eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from any use by us of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities.

In addition, we may incur substantial costs in order to comply with current or future environmental, health, and safety laws and regulations. These current or future laws and regulations may impair our research, development, or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Unfavorable global economic conditions could adversely affect our business, financial condition, stock price and results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the global financial crisis of 2008 caused extreme volatility and disruptions in the capital and credit markets. Similarly, the global economy has been impacted by fluctuating interest rates and inflation, as well as the possibility of a recession or further economic downturn. Moreover, adverse developments that affect financial institutions, such as events involving liquidity that are rumored or actual, have in the past and may in the future lead to market-wide liquidity problems. For example, on March 10, 2023, Silicon Valley Bank ("SVB"), one of our banking partners, was closed by the California Department of Financial Protection and Innovation, which appointed the Federal Deposit Insurance Corporation (the "FDIC") as receiver. While we only had a minimal amount of our cash directly at SVB and, since that date, the FDIC has stated that all depositors of SVB will be made whole, there is no guarantee that the federal government would guarantee all depositors in the event of future bank closures, and continued instability in the banking system may adversely impact our business and financial condition. Likewise, the capital and credit markets may be adversely affected by the ongoing conflicts in Israel and Ukraine, and the possibility of a wider Middle Eastern, European or global conflict, global sanctions imposed in response thereto, an energy crisis and potential recessions. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive such difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget. Also, hospitals and other medical facilitates face staffing shortages, whether due to labor relations or otherwise, which could potentially cause delays in enrollment, site visits, evaluations or other activities important to our research and development efforts. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial

market conditions could adversely impact our business. Furthermore, our stock price may decline due in part to the volatility of the stock market and any general economic downturn.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, fire, hurricane, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our suppliers' manufacturing facilities, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Our internal computer and information systems, or those used by our CROs, CMOs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our development programs.

Despite the implementation of appropriate security measures, our internal computer and information systems and those of our current and any future CROs, CMOs and other contractors or consultants may become vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, or accident, and are unaware of any security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of data from completed or future preclinical studies or clinical trials could result in significant delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be significantly delayed.

A breakdown or breach of our technology systems could subject us to liability or interrupt the operation of our business.

We are increasingly dependent upon technology systems and data to operate our business. In particular, the COVID-19 pandemic caused us to modify our business practices, including increasing the prevalence of employees working remotely. As a result, we are increasingly dependent upon our technology systems to operate our business and our ability to effectively manage our business depends on the security, reliability and adequacy of our technology systems and data, which includes use of cloud technologies, including Software as a Service (SaaS), Platform as a Service (PaaS) and Infrastructure as a Service (IaaS). A breakdown, invasion, corruption, destruction or breach of our technology systems, including the cloud technologies that we utilize, and/or unauthorized access to our data and information could subject us to liability or negatively impact the operation of our business. Our technology systems, including the cloud technologies that we utilize, continue to increase in multitude and complexity, making them potentially vulnerable to breakdown, malicious intrusion and random attack. Likewise, data privacy or security breaches by individuals authorized to access our technology systems, including the cloud technologies that we utilize, may pose a risk that sensitive data, including intellectual property, trade secrets or personal information belonging to us, our patients or other business partners, may be exposed to unauthorized persons or to the public.

Cyber-attacks and other cybersecurity incidents are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. They are often carried out by motivated, well-resourced, skilled and persistent actors, including nation states, organized crime groups, "hacktivists" and employees or contractors acting with malicious intent. Cyber-attacks could include the deployment of harmful malware and key loggers, ransomware, a denial-of-service attack, a malicious website, the use of social engineering and other means to affect the confidentiality, integrity and availability of our technology systems and data. Cyber-attacks could also include supply chain attacks, which could cause a delay in the manufacturing of our products or products produced for contract manufacturing. Our key business partners face similar risks and any security breach of their systems could adversely affect our security posture. Cyber-attacks could include wrongful conduct by hostile foreign governments, industrial espionage, wire fraud and other forms of cyber fraud, the deployment of harmful malware, denial-of-service, social engineering fraud or other means to threaten data confidentiality, integrity and availability. A successful cyber-attack could cause serious negative consequences for us, including, without limitation, the disruption of operations, the misappropriation of confidential business information, including financial information, trade secrets, financial loss and the disclosure of corporate strategic plans. To date, we have not experienced a material compromise of our data or information systems. However, although we devote resources to protect our information systems, we realize that cyber-attacks are a threat, and there can be no assurance that our efforts will prevent information security breaches that would result in business, legal, financial or reputational harm to us, or would have a material adverse effect on our results of operations and financial condition.

In addition, the computer systems of various third parties on which we rely, including our CROs, CMOs and other contractors, consultants and law and accounting firms, may sustain damage from computer viruses, unauthorized access, data breaches, phishing attacks, cybercriminals, natural disasters (including hurricanes and earthquakes), terrorism, war and telecommunication and electrical failures. We rely on our third-party providers to implement effective security measures and identify and correct for any such failures, deficiencies or breaches.

Moreover, our increased use of cloud technologies and remote working arrangements could heighten these and other operational risks, and any failure by cloud technology service providers to adequately safeguard their systems and prevent cyber-attacks could disrupt our operations and result in misappropriation, corruption or loss of confidential or propriety information. Despite the implementation of appropriate security measures, our internal computer and information systems and those of our current and any future CROs, CMOs and other contractors or consultants may become vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, or accident, and are unaware of any security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of data from completed or future preclinical studies or clinical trials could result in significant delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be significantly delayed. While we continue to build and improve our systems and infrastructure, including our business continuity plans, there can be no assurance that our efforts will prevent breakdowns or breaches in our systems that could adversely affect our business and operations and/or result in the loss of critical or sensitive information, which could result in financial, legal, business, operational or reputational harm to us, or loss of competitive advantage. In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to security breaches, cyber-attacks and other related breaches.

Our employees, principal investigators, CROs, CMOs and consultants may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical studies, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Our business entails a significant risk of product liability and our ability to obtain sufficient insurance coverage could have a material and adverse effect on our business, financial condition, results of operations and prospects.

We will face an inherent risk of product liability exposure related to the testing of zorevunersen, STK-002 and our future product candidates in clinical trials and will face an even greater risk if we commercialize any of our product candidates. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant time and costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any product candidates that we may develop.

While we currently have product liability insurance that we believe is appropriate for our stage of development, we may need to obtain higher levels prior to clinical development or marketing zorevunersen, STK-002 or any of our future product candidates. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance at a reasonable cost to protect us against losses caused by product liability claims that could have a material and adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Ownership of our Common Stock

The market price of our stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock may be highly volatile and subject to wide fluctuations in response to various factors, some of which we cannot control. The market price for our common stock may be influenced by many factors, including the other risks described in this section and elsewhere in this report and the following:

- results of preclinical studies and clinical trials of our product candidates, or those of our competitors or our existing or future collaborators;
- regulatory or legal developments in the United States and other countries, especially changes in laws or regulations applicable to our product candidates;
- the success of competitive products or technologies;
- introductions and announcements of new products by us, our future commercialization partners, or our competitors, and the timing of these introductions or announcements;
- actions taken by regulatory agencies with respect to our product candidates, clinical studies, manufacturing process or sales and marketing terms;
- actual or anticipated variations in our financial results or those of companies that are perceived to be similar to us;
- the success of our efforts to acquire or in-license additional technologies, products or product candidates;
- developments concerning any future collaborations, including but not limited to those with our sources of manufacturing supply and our commercialization partners;
- market conditions in the pharmaceutical and biotechnology sectors;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures or capital commitments;
- developments or disputes concerning patents or other proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates and products;
- our ability or inability to raise additional capital and the terms on which we raise it;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- actual or anticipated changes in earnings estimates or changes in stock market analyst recommendations regarding our common stock, other comparable companies or our industry generally;
- our failure or the failure of our competitors to meet analysts' projections or guidance that we or our competitors may give to the market;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- announcement and expectation of additional financing efforts;
- speculation in the press or investment community;
- trading volume of our common stock;
- sales of our common stock by us or our stockholders;
- the concentrated ownership of our common stock;
- changes in accounting principles;

•terrorist acts, acts of war or periods of widespread civil unrest, including the conflict in Ukraine and actions taken by third parties in response to such conflict;

•natural disasters and other calamities; and

•general economic, industry and market conditions including interest rate increases and inflation.

In addition, the stock market in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme price and volume fluctuations that have been often unrelated or disproportionate to the operating performance of the issuer, including as a result of general economic conditions. These broad market and industry factors may seriously harm the market price of our common stock, regardless of our actual operating performance. The realization of any of the above risks or any of a broad range of other risks, including those described in this "Risk factors" section, could have a dramatic and adverse impact on the market price of our common stock.

If securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our stock, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. We do not have any control over the analysts, or the content and opinions included in their reports. If any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if our preclinical studies and clinical trials and results of operations fail to meet the expectations of analysts, our stock price would likely decline. If one or more of such analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause a decline in our stock price or trading volume.

We are an "emerging growth company" and a "smaller reporting company" and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies or smaller reporting companies will make our common stock less attractive to investors.

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act"). For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including (i) not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended (the "Sarbanes-Oxley Act"), (ii) reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and (iii) exemptions from the requirements of holding nonbinding advisory stockholder votes on executive compensation and stockholder approval of any golden parachute payments not approved previously.

We will remain an emerging growth company until the earliest of (i) the last day of the fiscal year (a) in which we have total annual gross revenue of at least $1.235 billion or (b) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th, (ii) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period and (iii) December 31, 2024. We anticipate ceasing to be an emerging growth company as of December 31, 2024, which is the last day of our fiscal year following the fifth anniversary of the completion of our IPO.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to take advantage of the benefits of this extended transition period. Our consolidated financial statements may therefore not be comparable to those of companies that comply with such new or revised accounting standards. Until the date that we are no longer an "emerging growth company" or affirmatively and irrevocably opt out of the exemption provided by Section 7(a)(2)(B) of the Securities Act, upon issuance of a new or revised accounting standard that applies to our consolidated financial statements and that has a different effective date for public and private companies, we will disclose the date on which adoption is required for non-emerging growth companies and the date on which we will adopt the recently issued accounting standard.

We are also a "smaller reporting company," meaning that the market value of our stock held by non-affiliates was less than $700.0 million and our annual revenue was less than $100.0 million during the most recently completed fiscal year. We may continue to be a smaller reporting company as long as either (i) the market value of our stock held by non-affiliates is less than $250.0 million or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700.0 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our share price may be more volatile.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Our restated certificate of incorporation and our restated bylaws contain provisions that could delay or prevent a change in control of our company. These provisions could also make it difficult for stockholders to elect directors who are not nominated by current members of our board of directors or take other corporate actions, including effecting changes in our management. These provisions:

- establish a classified board of directors so that not all members of our board of directors are elected at one time;
- permit only our board of directors to establish the number of directors and fill vacancies on our board of directors;
- provide that directors may only be removed "for cause" and only with the approval of two-thirds of our stockholders;
- require super-majority voting to amend some provisions in our restated certificate of incorporation and restated bylaws;
- authorize the issuance of "blank check" preferred stock that our board of directors could use to implement a stockholder rights plan;
- eliminate the ability of our stockholders to call special meetings of stockholders;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;
- prohibit cumulative voting; and
- establish advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

The exclusive forum provision in our restated certificate of incorporation may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, or other employees, which may discourage lawsuits with respect to such claims.

Our restated certificate of incorporation, to the fullest extent permitted by law, provides that the Court of Chancery of the State of Delaware is the exclusive forum for: any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the Delaware General Corporation Law (the "DGCL"), our restated certificate of incorporation, or our restated bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine. This exclusive forum provision does not apply to suits brought to enforce a duty or liability created by the Exchange Act. It could apply, however, to a suit that falls within one or more of the categories enumerated in the exclusive forum provision and asserts claims under the Securities Act, inasmuch as Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rule and regulations thereunder. There is uncertainty as to whether a court would enforce such provision with respect to claims under the Securities Act, and our stockholders will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder.

This choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, or other employees, which may discourage lawsuits with respect to such claims. Alternatively, if a court were to find the choice of forum provisions contained in our restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our business, results of operations and financial condition.

In addition, Section 203 of the DGCL may discourage, delay or prevent a change in control of our company. Section 203 imposes certain restrictions on mergers, business combinations and other transactions between us and holders of 15% or more of our common stock.

Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all claims brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. In April 2020, we amended and restated our restated bylaws to provide that the federal district courts of the United States will, to the fullest extent permitted by law, be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act (such provision, a "Federal Forum Provision"). Our decision to adopt a Federal Forum Provision followed a decision by the Supreme Court of the State of Delaware holding that such provisions are facially valid under Delaware law. While there can be no assurance that federal or state courts will follow the holding of the Delaware Supreme Court or determine that the Federal Forum Provision should be enforced in a particular case, application of the Federal Forum Provision means that suits brought by our stockholders to enforce any duty or liability created by the Securities Act must be brought in federal court and cannot be brought in state court.

Section 27 of the Exchange Act creates exclusive federal jurisdiction over all claims brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder. In addition, neither the exclusive forum provision nor the Federal Forum Provision applies to suits brought to enforce any duty or liability created by the Exchange Act. Accordingly, actions by our stockholders to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder must be brought in federal court.

Our stockholders will not be deemed to have waived our compliance with the federal securities laws and the regulations promulgated thereunder.

Any person or entity purchasing or otherwise acquiring or holding any interest in any of our securities shall be deemed to have notice of and consented to our exclusive forum provisions, including the Federal Forum Provision. These provisions may limit a stockholder's ability to bring a claim in a judicial forum of their choosing for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers, and other employees.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Select Market ("Nasdaq") and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, we expect these rules and regulations to substantially increase our legal and financial compliance costs and to make some activities more time consuming and costly. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers. Moreover, these rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

If we fail to maintain proper and effective internal control over financial reporting in the future, our ability to produce accurate and timely financial statements could be impaired, which could harm our operating results, investors' views of us and, as a result, the value of our common stock.

We previously were not required to independently comply with Section 404(a) of the Sarbanes-Oxley Act. Section 404(a) of the Sarbanes-Oxley Act requires annual management assessments of the effectiveness of our internal control over financial reporting, starting with the second annual report that we file with the SEC. We were required to meet these standards in the course of preparing our financial statements as of and for the year ended December 31, 2023, and our management is required to report on the effectiveness of our internal control over financial reporting for such year and annually thereafter. Additionally, once we are no longer an "emerging growth company," our independent registered public accounting firm will be required pursuant to Section 404(b) of the Sarbanes-Oxley Act to attest to the effectiveness of our internal control over financial reporting on an annual basis. The rules governing the standards that must be met for our management to assess our internal control over financial reporting are complex and require significant documentation, testing, and possible remediation.

To achieve compliance with Section 404(b) within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our consolidated financial statements. In addition, if we are not able to continue to meet these requirements, we may not be able to remain listed on Nasdaq.

As we grow, we expect to hire additional personnel and may utilize external temporary resources to implement, document and modify policies and procedures to maintain effective internal controls. However, it is possible that we may identify deficiencies and weaknesses in our internal controls. If material weaknesses or deficiencies in our internal controls exist and go undetected or unremediated, our consolidated financial statements could contain material misstatements that, when discovered in the future, could cause us to fail to meet our future reporting obligations and cause the price of our common stock to decline.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock may be volatile and, in the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Item 2. Unregistered Sales of Equity Securities, Use of Proceeds and Issuer Purchases of Equity Securities.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

During the six months ended June 30, 2024, none of our directors or officers, as defined in Rule 16a-1(f), informed us of the adoption, modification or termination of a "Rule 10b5-1 trading agreement" or "non-Rule 10b-51 trading agreement," as those terms are defined in Regulations S-K, Item 408.

Item 6. Exhibits.

The exhibits filed or furnished as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index below.

Exhibit Number	Description	Form	File No.	Exhibit Filing Date	Exhibit No.	Filed/Furnished Herewith
10.1**	Executive Employment Agreement, by and between the Registrant and Thomas Leggett, effective as of April 23, 2024.					X
10.2**	Change of Control Severance Agreement, by and between the Registrant and Thomas Leggett, effective as of April 23, 2024.					X
10.3†**	Separation and Release Agreement, by and between the Registrant and Stephen J. Tulipano, dated May 2, 2024.					X
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	Inline XBRL Instance Document (the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document).					X
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents					X
104	Inline Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101).					X

* This certification is deemed not filed for purposes of section 18 of the Exchange Act or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act of the Exchange Act.

** Indicates a management contract or compensatory plan or arrangement in which directors or executive officers are eligible to participate.

† Registrant has omitted certain portions of this exhibit pursuant to Item 601(b)(10) of Regulation S-K.

SIGNATURES

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 thereunto duly authorized.

STOKE THERAPEUTICS, INC.

Date: August 7, 2024

By: /s/ Edward M. Kaye, M.D.
Edward M. Kaye, M.D.
Chief Executive Officer
(Principal Executive Officer)

Date: August 7, 2024

By: /s/ Thomas E. Leggett
Thomas E. Leggett
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 10.1

EXECUTIVE EMPLOYMENT AGREEMENT

This Executive Employment Agreement (the "Agreement"), is made and entered into this 23rd day of April, 2024 (the "Effective Date"), by and between Stoke Therapeutics, Inc. ("Stoke"), and Thomas Leggett ("Executive").

WHEREAS, Stoke wishes to employ Executive to serve as its Chief Financial Officer;

WHEREAS, Executive represents that Executive possesses the necessary skills to perform the duties of this position and that Executive has no obligation to any other person or entity which would prevent, limit or interfere with Executive's ability to do so; and

WHEREAS, Executive and Stoke desire to enter into a formal Executive Employment Agreement to assure the harmonious performance of the affairs of Stoke.

NOW, THEREFORE, in consideration of the mutual promises, covenants, terms, provisions, and conditions contained herein and other good and valuable consideration, the receipt and sufficiency of which are hereby mutually acknowledged, the parties agree as follows:

1.Title and Duties. Subject to the terms and conditions of this Agreement, Executive's position with Stoke shall be Chief Financial Officer ("CFO") reporting to Stoke's Chief Executive Officer. Executive shall provide the services described hereunder in Stoke's office in Bedford, MA, provided that Executive shall be permitted to provide services in other locations as agreed upon by Executive and Stoke. Executive accepts such employment upon the terms and conditions set forth herein, and agrees to perform to the best of Executive's ability the duties normally associated with such position and as reasonably determined by Stoke in its sole discretion. While serving as CFO hereunder, Executive shall devote all of Executive's business time and energies to the business and affairs of Stoke, provided that nothing contained in this Section 1 shall prevent or limit: (a) Executive's right to manage Executive's personal investments on Executive's own personal time, including, without limitation the right to make passive investments in the securities of (i) any entity which Executive does not control, directly or indirectly, and which does not compete with Stoke, or (ii) any publicly held entity, so long as Executive's aggregate direct and indirect interest does not exceed two percent (2%) of the issued and outstanding securities of any class of securities of such publicly held entity; and (b) Executive's participation in civic and charitable activities, including as a member of a board of a civic or charitable organization, so long as such activities do not interfere with Executive's performance of Executive's duties hereunder.

2.Term; Termination.

(a)Term. Subject to the terms hereof, Executive's employment hereunder shall commence on May 7, 2024 (the "Commencement Date") and shall continue until terminated hereunder by either party (such term of employment shall be referred to herein as the "Term"). Executive's employment with Stoke shall be at-will and may be terminated by Executive or Stoke at any time, subject to the terms and conditions in this Agreement.

(b)Termination by Stoke. Notwithstanding anything else contained in this Agreement, Stoke may terminate Executive's employment hereunder as follows:

(i)For Cause. Stoke may terminate Executive's employment for Cause (as defined below) by written notice by Stoke to Executive that Executive's employment is being terminated for Cause, which termination shall be effective on the date of such notice or such later date as specified in writing by Stoke, provided that if Executive has cured the circumstances giving rise to Cause (as such cure right may be applicable pursuant to the terms and conditions set forth below) then such termination shall not be effective.

(ii)Without Cause. Stoke may terminate Executive's employment without Cause, by written notice by Stoke to Executive that Executive's employment is being terminated without Cause, which termination shall be effective on the date of such notice or such later date as specified in writing by Stoke.

For the purposes of this Agreement, "Cause" shall mean: (A) fraud, embezzlement, or illegal misconduct in connection with Executive's duties under this Agreement; (B) commission of a felony involving fraud, dishonesty or breach of trust; (C) willful misconduct or gross negligence in the performance of the duties delegated to Executive; (D) breach of this Agreement; or (E) material breach of Executive's Non-Competition, Non-Solicitation, Non-Disclosure, and Intellectual Property Agreement (as described below); provided that "Cause" shall not be deemed to have occurred pursuant to subsection (D) hereof unless Executive has first received written notice specifying in reasonable detail the particulars of such ground and that Stoke intends to terminate Executive's employment hereunder for such ground, and if such ground is curable, Executive has failed to cure such ground within a period of thirty (30) days from the date of his receipt of such notice.

(c)Termination by Executive. Notwithstanding anything else contained in this Agreement, Executive may terminate Executive's employment hereunder as follows:

(i)For Good Reason. Executive may terminate Executive's employment for Good Reason (as defined below) by written notice by Executive to Stoke that Executive is terminating Executive's employment for Good Reason, which termination shall be effective thirty (30) days after the date of such notice; provided that if Stoke has cured the circumstances giving rise to Good Reason then such termination shall not be effective; or

(ii)Without Good Reason. Executive may terminate Executive's employment without Good Reason by written notice by Executive to Stoke that Executive is terminating Executive's employment, which termination shall be effective ninety (90) days after the date of such notice.

For the purposes of this Agreement, "Good Reason" shall mean: (A) a material reduction in Executive's then-current Base Salary, provided that such material reduction is unique to Executive and not to all similarly situated executives of Stoke; (B) Executive does not report to the Chief Executive Officer of the parent company following any Change

in Control (as that term is defined in Executive's Change of Control Severance Agreement; (C) a material change in the geographic location at which the Executive provides services to Stoke outside of a fifty (50) mile radius from the then-current location; or (D) any action or inaction by Stoke that constitutes a material breach of this Agreement; provided that "Good Reason" shall not be deemed to have occurred unless: (1) Executive provides Stoke with written notice that Executive intends to terminate Executive's employment hereunder for one of the grounds set forth above within thirty (30) days of such ground first occurring, (2) if such ground is capable of being cured, Stoke has failed to cure such ground within a period of thirty (30) days from the date of such written notice, and (3) Executive terminates Executive's employment within seventy five (75) days from the date that Good Reason first occurs. For purposes of clarification, the above-listed conditions shall apply separately to each occurrence of Good Reason and failure to adhere to such conditions in the event of Good Reason shall not disqualify Executive from asserting Good Reason for any subsequent occurrence of Good Reason.

(d) Termination Due to Executive's Death or Disability. Notwithstanding anything else contained in this Agreement, Stoke may terminate Executive's employment immediately upon Executive's death, or due to Executive's Disability (as defined below) by written notice to Executive that Executive's employment is being terminated as a result of Executive's Disability, which termination shall be effective on the date of such notice or such later date as specified in writing by Stoke. For the purposes of this Agreement, "Disability" shall mean Executive's incapacity or inability to perform Executive's duties and responsibilities as contemplated herein for one hundred twenty (120) days or more within any one (1) year period (cumulative or consecutive), because Executive's physical or mental health has become so impaired as to make it impossible or impractical for Executive to perform the duties and responsibilities contemplated hereunder. Determination of Executive's physical or mental health shall be determined by Stoke after consultation with a medical expert appointed by mutual agreement between Stoke and Executive who has examined Executive. Executive hereby consents to such examination and consultation regarding Executive's health and ability to perform as aforesaid.

3.Compensation.

(a)Base Salary. While Executive is employed hereunder, Executive shall earn a base salary at the annual rate of $475,000.00 (the "Base Salary"). The Base Salary shall be payable in substantially equal periodic installments, in accordance with Stoke's payroll practices as in effect from time to time. Stoke shall deduct from each such installment all amounts required to be deducted or withheld under applicable law or under any employee benefit plan in which Executive participates.

(b)Annual Bonus. Executive shall be eligible to receive an annual cash bonus with a target amount equal to 40% of Executive's Base Salary, (the "Annual Bonus"). The amount of the Annual Bonus shall be based on factors such as Executive's work performance, Stoke's financial performance, Stoke's business forecasts, Stoke's determination of Executive's achievement of milestones for the applicable year, and economic conditions generally. The actual amount of the Annual Bonus shall be determined by the Board in its sole discretion. Executive's Annual Bonus shall be paid by the fifteenth day of the third month following Executive's or Stoke's taxable year in which it is earned, whichever is later. Executive must be employed by Stoke on the last day of

such fiscal year to which such Annual Bonus relates, to be deemed as having earned the Annual Bonus. Stoke shall deduct from the Annual Bonus all amounts required to be deducted or withheld under applicable law or under any employee benefit plan in which Executive participates.

(c)Equity. On the Commencement Date or as soon as practicable thereafter, and subject to the Board's approval, the Stoke shall grant you an option to purchase 396,200 shares of Stoke's common stock (the "Equity Award") under Stoke's 2023 Inducement Plan (the "Plan"). The Equity Award shall have an exercise price equal to the fair market value of Stoke's common stock on the date of grant. The Equity Award shall be an "incentive stock option" to the extent permitted under Section 409A the Internal Revenue Code ("Section 409A"). Subject to the Board's approval as described above and Executive's continued employment by Stoke, the Equity Award shall be vested as to one fourth (1/4th) of the total number of shares subject to the Equity Award on the one-year anniversary of the Commencement Date, and one forty-eighth (1/48th) of the total number of shares subject to the Equity Award shall vest in monthly installments thereafter on the same day of the month as the Commencement Date (and if there is no corresponding day, on the last day of the month), with all shares subject to the Equity Award being fully vested on the four-year anniversary of the Commencement Date. Vesting is contingent on Executive's continued employment with Stoke and shall be subject to the terms and conditions of the Plan and the written agreement governing the Equity Award except as explicitly set forth below.

(d)Fringe Benefits. Executive shall be entitled to participate in all benefit/welfare plans and fringe benefits provided to employees at the same level as Executive. Executive understands that, except when prohibited by applicable law, Stoke's benefit plans and fringe benefits may be amended by Stoke from time to time in its sole discretion.

(e)Vacation. Executive shall be eligible for Stoke's unlimited vacation time policy, subject to its terms and conditions, as established or modified from time to time by Stoke.

(f)Reimbursement of Expenses. Stoke shall reimburse Executive for all ordinary and reasonable out-of-pocket business expenses incurred by Executive in furtherance of Stoke's business in accordance with Stoke's policies with respect thereto as in effect from time to time. All reimbursements provided under this Agreement shall be made or provided in accordance with the requirements of Section 409A and the rules and regulations thereunder, including, where applicable, the requirement that (i) any reimbursement is for expenses incurred during Executive's lifetime (or during a shorter period of time specified in this Agreement); (ii) the amount of expenses eligible for reimbursement during a calendar year may not affect the expenses eligible for reimbursement in any other calendar year; (iii) the reimbursement of an eligible expense shall be made no later than the last day of the calendar year following the year in which the expense is incurred; and (iv) the right to reimbursement or in kind benefits is not subject to liquidation or exchange for another benefit.

(g)Indemnification. Executive shall be eligible for coverage under Stoke Directors' and Officers' ("D&O") insurance policies to the same extent and in the same manner to which Stoke's similarly situated executives are entitled to coverage under Stoke D&O insurance policies, subject to the terms and conditions of any such Stoke D&O insurance policies.

4.Termination Payments; Severance Benefit.

(a)Payment of Accrued Obligations. Regardless of the reason for any employment termination hereunder, Stoke shall pay to Executive: (i) the portion of Executive's Base Salary that has accrued prior to any termination of Executive's employment and has not yet been paid; (ii) except in the event of Executive's termination for Cause, the amount of any Annual Bonus determined by the Board and payable from a prior year which remains unpaid by Stoke as of the date of the termination of employment; (iii) the amount of any expenses properly incurred by Executive on behalf of Stoke prior to any such termination and has not yet been reimbursed (together, the "Accrued Obligations") promptly following the effective date of termination, and otherwise within any timeframe required by law. Executive's entitlement to other compensation or benefits under any Stoke plan or policy shall be governed by and determined in accordance with the terms of such plan or policy, except as otherwise specified in this Agreement. In the event of Stoke's termination of Executive's employment for Cause or Executive's termination of Executive's employment without Good Reason, Executive shall be eligible for the Accrued Obligations and shall not be eligible for any severance or severance-type payments, other than as expressly set forth herein.

(b)Severance in the Event of Termination Without Cause or Resignation for Good Reason. Subject to the terms and conditions of Section 4(c), in the event that Executive's employment hereunder is terminated by Stoke without Cause or terminated by Executive for Good Reason, then, in addition to the Accrued Obligations:

(i)Stoke shall continue to pay the Executive's Base Salary for a nine (9) month period following the termination date of Executive's employment (the "Separation Date"), with such payments to be made in accordance with Stoke's normal payroll practices and schedules, less all customary and required taxes and employment-related deductions.

(ii)In the event that Executive is eligible for coverage under a Stoke health insurance plan and Executive has elected to have coverage thereunder and was covered thereunder prior to termination, and in the event that Executive chooses to exercise Executive's right under the Consolidated Omnibus Budget Reconciliation Act of 1985 ("COBRA") to continue Executive's participation in such plan, Stoke shall pay its normal share of the costs for such coverage for a period of up to nine (9) months from the Separation Date, to the same extent that such insurance is provided to persons then currently employed by Stoke. Stoke shall deduct from each of the installments due under Section 4(b)(i) the portion of the monthly premium due from Executive in accordance with the terms of such coverage. Notwithstanding any other provision of this Agreement, this obligation shall cease on the date Executive becomes eligible to receive health insurance benefits through any other employer, and Executive agrees to provide Stoke with written notice immediately upon becoming eligible for such benefits. Executive's acceptance of any payment on Executive's behalf or coverage provided hereunder shall be an express representation to Stoke that Executive has no such eligibility.

Subsections (i) and (ii) are referred to as the Severance Benefit. The Severance Benefit is expressly subject to the conditions described above and in Section 4(c) below. Any payment or

benefit made as part of such Severance Benefit shall be paid less all customary and required taxes and employment-related deductions.

(c)Conditions. Stoke shall not be obligated to provide Executive with the Severance Benefit described in Section 4(b) unless and until Executive has executed without revocation a separation agreement in a form acceptable to Stoke, which must be signed by Executive, returned to Stoke and be enforceable and irrevocable no later than sixty (60) days following Executive's Separation Date (the "Review Period"), and which shall include, at a minimum, the provision of the Severance Benefit due from Stoke to Executive, a complete general release of claims against Stoke and its affiliated entities and each of their officers, directors and employees, and terms relating to non-disparagement, non-competition, confidentiality, cooperation and other customary terms determined by Stoke. If Executive executes and does not revoke such agreement within the time provided in the Separation Agreement, then provision of the Severance Benefit shall commence on the first (1st) regularly scheduled payroll date following the Review Period, provided that if the last day of the Review Period occurs in the calendar year following the year of termination, then the payment shall not commence until January 2 of such subsequent calendar year, and further provided that, as applied to Section 4(b), the first payments/benefits shall include in a lump sum all amounts that were otherwise payable to Executive from the Separation Date through such first payment. As stated in Stoke's Non-Competition, Non-Solicitation, Non-Disclosure, and Intellectual Property Agreement (described below), in the event Executive is eligible for garden leave or analogous payments in support of non-competition obligations, then Stoke reserves the right to offset the Severance Benefit with such garden leave or analogous payments to the extent permitted by applicable law (*i.e.* Stoke shall pay the greater of the severance or the garden leave but not both).

(d)COBRA. If the payment of any COBRA or health insurance premiums by Stoke on behalf of Executive as described herein would otherwise violate any applicable nondiscrimination rules or cause the reimbursement of claims to be taxable under the Patient Protection and Affordable Care Act of 2010, together with the Health Care and Education Reconciliation Act of 2010 (collectively, the "Act") or Section 105(h) of the Code, the COBRA premiums paid by Stoke shall be treated as taxable payments (subject to customary and required taxes and employment-related deductions) and be subject to imputed income tax treatment to the extent necessary to eliminate any discriminatory treatment or taxation under the Act or Section 105(h) of the Code. If Stoke determines in its sole discretion that it cannot provide the COBRA benefits described herein under Stoke's health insurance plan without potentially violating applicable law (including, without limitation, Section 2716 of the Public Health Service Act), Stoke shall in lieu thereof provide to Executive a taxable lump-sum payment in an amount equal to the sum of the monthly (or then remaining) COBRA premiums that Executive would be required to pay to maintain Executive's group health insurance coverage in effect on the Separation Date for the remaining portion of the period for which Executive shall receive the payments described in Section 4(b) above.

(e)No Other Payments or Benefits Owing. The payments and benefits set forth in this Section 4 shall be the sole amounts owing to Executive upon termination of Executive's employment for the reasons set forth above and Executive shall not be eligible for any other payments or other forms of compensation or benefits. The payments and benefits set forth in this Section shall be the sole remedy, if any, available to Executive in the event that Executive brings

any claim against Stoke relating to the termination of Executive's employment under this Agreement.

5.Non-Competition, Non-Solicitation, Non-Disclosure Agreement. In light of the competitive and proprietary aspects of the business of Stoke, and as a condition of Executive's employment hereunder, Executive agrees to sign and abide by Stoke's Non-Competition, Non-Solicitation, Non-Disclosure, and Intellectual Property Agreement.

6.Code Section 409A.

(a)In the event that the payments or benefits set forth in Section 4 constitute "non-qualified deferred compensation" subject to Section 409A, then the following conditions apply to such payments or benefits:

(i)Any termination of Executive's employment triggering payment of benefits under Section 4 must constitute a "separation from service" under Section 409A(a)(2)(A)(i) of the Code and Treas. Reg. §1.409A-1(h) before distribution of such benefits can commence. To the extent that the termination of Executive's employment does not constitute a separation of service under Section 409A(a)(2)(A)(i) of the Code and Treas. Reg. §1.409A-1(h) (as the result of further services that are reasonably anticipated to be provided by Executive to Stoke at the time Executive's employment terminates), any such payments under Section 4 that constitute deferred compensation under Section 409A shall be delayed until after the date of a subsequent event constituting a separation of service under Section 409A(a)(2)(A)(i) of the Code and Treas. Reg. §1.409A-1(h). For purposes of clarification, this Section 6(a) shall not cause any forfeiture of benefits on Executive's part, but shall only act as a delay until such time as a "separation from service" occurs.

(ii)Notwithstanding any other provision with respect to the timing of payments under Section 4 if, at the time of Executive's termination, Executive is deemed to be a "specified employee" of Stoke (within the meaning of Section 409A(a)(2)(B)(i) of the Code), then limited only to the extent necessary to comply with the requirements of Section 409A, any payments to which Executive may become entitled under Section 4 which are subject to Section 409A (and not otherwise exempt from its application) shall be withheld until the first (1st) business day of the seventh (7th) month following the termination of Executive's employment, at which time Executive shall be paid an aggregate amount equal to the accumulated, but unpaid, payments otherwise due to Executive under the terms of Section 4.

(b)It is intended that each installment of the payments and benefits provided under Section 4 shall be treated as a separate "payment" for purposes of Section 409A. Neither Stoke nor Executive shall have the right to accelerate or defer the delivery of any such payments or benefits except to the extent specifically permitted or required by Section 409A.

(c)Notwithstanding any other provision of this Agreement to the contrary, this Agreement shall be interpreted and at all times administered in a manner that avoids the inclusion of compensation in income under Section 409A, or the payment of increased taxes, excise taxes or other penalties under Section 409A. The parties intend this Agreement to be in compliance with

Section 409A. Executive acknowledges and agrees that Stoke does not guarantee the tax treatment or tax consequences associated with any payment or benefit arising under this Agreement, including but not limited to consequences related to Section 409A.

7.General.

(a)Notices. Except as otherwise specifically provided herein, any notice required or permitted by this Agreement shall be in writing and shall be delivered as follows with notice deemed given as indicated: (i) by personal delivery when delivered personally; (ii) by overnight courier upon written verification of receipt; (iii) by certified or registered mail, return receipt requested, upon verification of receipt; or (iv) by e-mail, upon acknowledgement of receipt from the receiving party.

Notices to Executive shall be sent to the address specified below, or the last known address in Stoke's records.

Notices to Stoke shall be sent to:

Jonathan Allan, General Counsel
Stoke Therapeutics, Inc.
45 Wiggins Ave
Bedford, MA 01730

Wendy Baccari, Senior Director, HR Operations
Stoke Therapeutics, Inc.
45 Wiggins Ave
Bedford, MA 01730

with a copy to:

Mintz, Levin, Cohn, Ferris, Glovsky & Popeo, P.C.
701 Pennsylvania Avenue NW #900
Washington, DC 20004
Fax:
Attn: David Barmak, Esq.

(b)Modifications and Amendments. The terms and provisions of this Agreement may be modified or amended only by written agreement executed by the parties hereto.

(c)Waivers and Consents. The terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

(d)Assignment. Stoke may assign its rights and obligations hereunder to any person or entity that succeeds to all or substantially all of Stoke's business or that aspect of Stoke's business in which Executive is principally involved. Executive may not assign Executive's rights and obligations under this Agreement without the prior written consent of Stoke.

(e)Governing Law; Jury Waiver. This Agreement and the rights and obligations of the parties hereunder shall be construed in accordance with and governed by the law of the Commonwealth of Massachusetts without giving effect to the conflict of law principles thereof. Any legal action or proceeding with respect to this Agreement shall be brought in the courts of the Commonwealth of Massachusetts or the United States of America for the District of Massachusetts. By execution and delivery of this Agreement, each of the parties hereto accepts for itself and in respect of its property, generally and unconditionally, the exclusive jurisdiction of the aforesaid courts. ANY ACTION, DEMAND, CLAIM OR COUNTERCLAIM ARISING UNDER OR RELATING TO THIS AGREEMENT SHALL BE RESOLVED BY A JUDGE ALONE AND EACH OF STOKE AND EXECUTIVE WAIVES ANY RIGHT TO A JURY TRIAL THEREOF.

(f)Headings and Captions. The headings and captions of the various subdivisions of this Agreement are for convenience of reference only and shall in no way modify or affect the meaning or construction of any of the terms or provisions hereof.

(g)Entire Agreement. This Agreement, together with the other agreements expressly referenced herein, embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof. No statement, representation, warranty, covenant or agreement of any kind not expressly set forth in this Agreement shall affect, or be used to interpret, change or restrict, the express terms and provisions of this Agreement.

(h)Counterparts. This Agreement may be executed in two or more counterparts, and by different parties hereto on separate counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. For all purposes signed counterparts delivered by fax or other digital means (e.g., .pdf) shall be treated as an original.

(i)Satisfactory Background Check. Executive understands and acknowledges that this Agreement and Stoke's offer of employment hereunder is contingent upon Executive's satisfactory completion of a background check, in accordance with applicable law.

[Signature Page to Follow]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first written above.

Thomas Leggett

/s/ Thomas Leggett_____
Printed name

/s/ Thomas Leggett_____
Signature

Address:

Date: April 23, 2024_____

STOKE THERAPEUTICS, INC.

/s/ Joan Wood_____
By: Joan Wood
Title: Chief Human Resources Officer

Exhibit 10.2

CHANGE OF CONTROL SEVERANCE AGREEMENT

This Change of Control Severance Agreement (the "Agreement"), is made and entered into this 23rd day of April, 2024 (the "Effective Date"), by and between Stoke Therapeutics, Inc. ("Stoke"), and Thomas Leggett ("Executive").

Recitals:

R-1. Stoke and Executive are parties to an Employment Agreement dated April 23, 2024 (the "Employment Agreement," which term shall include the Employment Agreement as it may be amended from time to time hereafter).

R-2. Stoke wishes to provide Executive with certain promises and benefits in the event Stoke terminates Executive's employment without Cause or Executive terminates Executive's employment for Good Reason, within ninety (90) days prior to, or one (1) year following a Change of Control.

NOW, THEREFORE, in consideration of the mutual promises, terms and conditions in this Agreement and other good and valuable consideration, the receipt and sufficiency of which are hereby mutually acknowledged, the parties agree as follows:

1.Definitions.

(a)All capitalized terms used in this Agreement and not otherwise defined herein shall have the meaning ascribed to them in Executive's Employment Agreement.

(b)Change of Control. As used in this Agreement, a "Change of Control" shall mean the occurrence of any of the following events:

(i) Ownership. Any "Person" (as such term is used in Sections 13(d) and 14(d) of the Securities Exchange Act of 1934, as amended) becomes the "Beneficial Owner" (as defined in Rule 13d-3 under said Act), directly or indirectly, of securities of Stoke representing fifty percent (50%) or more of the total voting power represented by Stoke's then outstanding voting securities (excluding for this purpose any such voting securities held by Stoke, or any affiliate, parent or subsidiary of Stoke, or by any employee benefit plan of Stoke) pursuant to a transaction or a series of related transactions which Stoke's Board of Directors (the "Board") does not approve; or

(ii) Merger/Sale of Assets. (A) A merger or consolidation of Stoke whether or not approved by the Board, other than a merger or consolidation which would result in the voting securities of Stoke outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or the parent of such corporation) at least fifty percent (50%) of the total voting power represented by the voting securities of Stoke or such surviving entity or parent of such corporation, as the case may be, outstanding immediately after such merger or consolidation; or (B) the sale or disposition by Stoke of all or substantially all of Stoke's assets.

2.Change of Control Severance Benefit.

(a)In the event that Executive's employment with Stoke is terminated by Stoke without Cause or terminated by Executive for Good Reason within ninety (90) days prior to, or within one (1) year following a Change of Control, then, in addition to any Accrued Obligations and in lieu of any Severance Benefit otherwise payable to the Executive under Executive's Employment Agreement and subject to the Executive signing and not revoking the separation agreement and release of claims provided for in Section 4 of this Agreement:

(i)Stoke shall provide Executive with a payment in an amount equal to Executive's Base Salary for a twelve (12) month period, with such payment to be made either in a lump sum, or in substantially equal installments in accordance with Stoke's normal payroll practices and schedules (such method of payment shall be determined by Stoke in its sole discretion), less all customary and required taxes and employment-related deductions.

(ii)Stoke shall provide Executive with a payment in an amount equal to one hundred percent (100%) of Executive's then-current target amount of Annual Bonus for the year in which Executive's termination date occurs (the "Separation Date"), paid in one lump sum amount within sixty (60) days following the Separation Date, less customary and required taxes and employment-related deductions.

(iii)In the event that Executive is eligible for coverage under a Stoke health insurance plan, Executive was covered thereunder prior to termination, and Executive chooses to exercise Executive's rights under the Consolidated Omnibus Budget Reconciliation Act of 1985 ("COBRA") to continue Executive's participation in such plan, Stoke shall pay the premiums charged to continue Executive's health insurance coverage pursuant to COBRA for a period of up to twelve (12) months from termination, to the same extent that such insurance is provided to persons then currently employed by Stoke. Notwithstanding any other provision of this Agreement, this obligation shall cease on the date Executive becomes eligible to receive health insurance benefits through any other employer, and Executive agrees to provide Stoke with written notice immediately upon becoming eligible for such benefits. Executive's acceptance of any payment on Executive's own behalf or coverage provided hereunder shall be an express representation to Stoke that Executive has no such eligibility.

(iv)All equity awards granted to Executive and outstanding as of the Separation Date shall fully accelerate and vest, subject to the terms of any applicable equity plan and equity agreements.

Section 2(a)(i)-2(a)(iv) are referred to as the "Change of Control Severance Benefit." The Change of Control Severance Benefit is expressly subject to the conditions described in this Agreement, including but not limited to Section 4.

3.Exclusions. In the event that Executive is eligible for the Change of Control Severance Benefit described in Section 2 of this Agreement, Executive shall not be eligible for, and shall not receive any severance payments or benefits under any other severance or separation agreement or policy, including but not limited to the Severance Benefit under Executive's Employment Agreement. In the event that Executive's employment is terminated for any reason

other than those outlined in Section 2, then Executive shall not be eligible for, and shall not receive the Change of Control Severance Benefit.

4.Separation Agreement, Release of Claims. Stoke shall not be obligated to provide Executive with the Change of Control Severance Benefit unless and until (a) the consummation of the Change of Control; and (b) Executive has executed, without revocation a separation agreement in a form acceptable to Stoke, which must be signed by Executive, returned to Stoke and be enforceable and irrevocable no later than sixty (60) days following Executive's Separation Date (the "Review Period"), and which shall include, at a minimum, the provision of the Change of Control Severance Benefit due from Stoke to Executive, a complete general release of claims against Stoke and its affiliated entities and each of their officers, directors and employees, and terms relating to non-disparagement, non-competition (if applicable), confidentiality, cooperation and other customary terms determined by Stoke. If Executive executes and does not revoke such agreement within the time provided in the separation agreement, then the Change of Control Severance Benefit shall be paid either: (i) in one lump sum amount on the first (1st) regularly scheduled payroll date following the Review Period; or (ii) in substantially equal installments commencing on the first (1st) regularly scheduled payroll date following the Review Period, provided that, as applied to Section 2(a)(i)-2(a)(iv), the first payments/benefits shall include in a lump sum all amounts that were otherwise payable to Executive from the Separation Date through such first payment.

5.Sections 409A and 280G of the Code.

(a)In the event that the payments or benefits set forth in Section 2 constitute "non-qualified deferred compensation" subject to Section 409A, then the following conditions apply to such payments or benefits:

(i)Any termination of Executive's employment triggering payment of Change of Control Severance Benefit under Section 2 must constitute a "separation from service" under Section 409A(a)(2)(A)(i) of the Code and Treas. Reg. §1.409A-1(h) before distribution of such benefits can commence. To the extent that the termination of Executive's employment does not constitute a separation of service under Section 409A(a)(2)(A)(i) of the Code and Treas. Reg. §1.409A-1(h) (as the result of further services that are reasonably anticipated to be provided by Executive to Stoke at the time Executive's employment terminates), any such payments under Section 2 that constitute deferred compensation under Section 409A shall be delayed until after the date of a subsequent event constituting a separation of service under Section 409A(a)(2)(A)(i) of the Code and Treas. Reg. §1.409A-1(h). For purposes of clarification, this Section 5(a) shall not cause any forfeiture of benefits on Executive's part, but shall only act as a delay until such time as a "separation from service" occurs.

(ii)Notwithstanding any other provision with respect to the timing of payments under Section 2 if, at the time of Executive's termination, Executive is deemed to be a "specified employee" of Stoke (within the meaning of Section 409A(a)(2)(B)(i) of the Code), then limited only to the extent necessary to comply with the requirements of Section 409A, any payments to which Executive may become entitled under Section 2 which are subject to Section 409A (and not otherwise exempt from its application) shall be withheld

until the first (1st) business day of the seventh (7th) month following the termination of Executive's employment, at which time Executive shall be paid an aggregate amount equal to the accumulated, but unpaid, payments otherwise due to Executive under the terms of Section 2.

(b)It is intended that each installment of the payments and benefits provided under Section 2 shall be treated as a separate "payment" for purposes of Section 409A. Neither Stoke nor Executive shall have the right to accelerate or defer the delivery of any such payments or benefits except to the extent specifically permitted or required by Section 409A.

(c)Notwithstanding any other provision of this Agreement to the contrary, this Agreement shall be interpreted and at all times administered in a manner that avoids the inclusion of compensation in income under Section 409A, or the payment of increased taxes, excise taxes or other penalties under Section 409A. The parties intend this Agreement to be in compliance with Section 409A. Executive acknowledges and agrees that Stoke does not guarantee the tax treatment or tax consequences associated with any payment or benefit arising under this Agreement, including but not limited to consequences related to Section 409A.

(d)If any payment or benefit that Executive would receive under this Agreement, when combined with any other payment or benefit Executive receives pursuant to a Change of Control (for purposes of this section, a "Payment") would: (i) constitute a "parachute payment" within the meaning of Section 280G the Code; and (ii) but for this sentence, be subject to the excise tax imposed by Section 4999 of the Code (the "Excise Tax"), then such Payment shall be either: (A) the full amount of such Payment; or (B) such lesser amount as would result in no portion of the Payment being subject to the Excise Tax, whichever of the foregoing amounts, taking into account the applicable federal, state and local employments taxes, income taxes and the Excise Tax, results in Executive's receipt, on an after-tax basis, of the greater amount of the Payment notwithstanding that all or some portion of the Payment may be subject to the Excise Tax. With respect to subsection (B), if there is more than one method of reducing the payment as would result in no portion of the Payment being subject to the Excise Tax, then Executive shall determine which method shall be followed, provided that if Executive fails to make such determination within thirty (30) days after Stoke has sent Executive written notice of the need for such reduction, Stoke may determine the amount of such reduction in its sole discretion.

6.No Impact on Employment Status. This Agreement is not intended to confer, and shall not be interpreted as conferring, any additional employment rights on Executive and has no impact on either party's right to terminate Executive's at-will employment.

7.General.

(a)Modifications and Amendments. The terms and provisions of this Agreement may be modified or amended only by written agreement executed by the parties hereto.

(b)Waivers and Consents. The terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions

of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

(c)Assignment. Stoke may assign its rights and obligations hereunder to any person or entity that succeeds to all or substantially all of Stoke's business or that aspect of Stoke's business in which Executive is principally involved. Executive may not assign Executive's rights and obligations under this Agreement without the prior written consent of Stoke.

(d)Governing Law; Jury Waiver. This Agreement and the rights and obligations of the parties hereunder shall be construed in accordance with and governed by the law of the Commonwealth of Massachusetts without giving effect to the conflict of law principles thereof. Any legal action or proceeding with respect to this Agreement shall be brought in the courts of the Commonwealth of Massachusetts or the United States of America for the District of Massachusetts. By execution and delivery of this Agreement, each of the parties hereto accepts for itself and in respect of its property, generally and unconditionally, the exclusive jurisdiction of the aforesaid courts. ANY ACTION, DEMAND, CLAIM OR COUNTERCLAIM ARISING UNDER OR RELATING TO THIS AGREEMENT SHALL BE RESOLVED BY A JUDGE ALONE AND EACH OF STOKE AND EXECUTIVE WAIVES ANY RIGHT TO A JURY TRIAL THEREOF.

(e)Headings and Captions. The headings and captions of the various subdivisions of this Agreement are for convenience of reference only and shall in no way modify or affect the meaning or construction of any of the terms or provisions hereof.

(f)Entire Agreement. This Agreement, together with the other agreements expressly referenced herein, embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof. The terms of this Agreement shall replace any agreement, policy or practice which otherwise would obligate Stoke to provide any severance compensation and/or benefits to Executive in connection with a Change of Control, provided that it shall not be construed to otherwise limit Executive's rights to payments or benefits provided under any pension plan (as defined in ERISA), deferred compensation, stock, stock option or similar plan sponsored by Stoke. Notwithstanding the foregoing and for the avoidance of doubt, any applicable stock option award agreement, other award agreement or equity incentive plan existing between Stoke and Executive, and any existing indemnity agreement or non-competition, non-solicitation, confidentiality or similar agreement between Stoke and Executive shall remain in full force and effect and shall not be superseded by this Agreement.

(g)Counterparts. This Agreement may be executed in two or more counterparts, and by different parties hereto on separate counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. For all purposes signed counterparts delivered by fax or other digital means (e.g., .pdf) shall be treated as an original.

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first written above.

Thomas Leggett

Thomas Leggett_____
Printed name

/s/ Thomas Legget_____
Signature

Address:

STOKE THERAPEUTICS, INC.

/s/ Joan Wood_____
By: Joan Wood
Title: Chief Human Resources Officer

Exhibit 10.3

May 2, 2024

Stephen Tulipano
Email:

Re: Terms of Separation

Dear Stephen:

This letter confirms the agreement ("***Agreement***") between you and Stoke Therapeutics, Inc. (the "***Company***") concerning the terms of your separation and offers you the separation compensation we discussed in exchange for a general release of claims and covenant not to sue.

1.Separation Date; Resignation from Officer Positions: May 7, 2024 is your last day of employment with the Company (the "***Separation Date***"). Pursuant to your Amended and Restated Executive Employment Agreement with the Company dated October 21, 2020 (the "***Employment Agreement***"), your separation constitutes a termination without Cause (as defined in the Employment Agreement). By signing below, you hereby resign, effective as of the Separation Date, from all officer positions of the Company that you may hold, including, without limitation, Chief Financial Officer.

2.Acknowledgment of Payment of Wages: By your signature below, you acknowledge that on May 7, 2024, we provided you one or more final paychecks for all wages, salary, bonuses, reimbursable expenses previously submitted by you, accrued vacation (if applicable) and any similar payments due you from the Company as of the Separation Date. By signing below, you acknowledge that the Company does not owe you any other amounts. Please promptly submit for reimbursement all final outstanding expenses, if any.

3.Separation Compensation: In exchange for your agreement to the general release and waiver of claims and covenant not to sue set forth below and your other promises herein, the Company agrees to provide you with the following:

a.Severance: Pursuant to the Employment Agreement, the Company agrees to pay you the gross amount of $357,739.50, less applicable state and federal payroll deductions (the "***Severance Payment***"), which equals nine (9) months of your base salary. The Severance Payment will be paid in equal installments in accordance with the Company's regular payroll schedule for a period of nine (9) months following the Separation Date, with the first installment to be paid on the Company's first regular payroll date following the Effective Date (as defined below) of this Agreement.

b.Outplacement Services: The Company agrees to provide you with comprehensive career services through Keystone Partners for up to nine (9) months. The cost of theses outplacement services, up to a total of $15,000, will be paid directly to Keystone Partners by the Company.

c.Advisory Services: The Company agrees to engage you as an advisor pursuant to the Advisor Agreement (the "***Advisor Agreement***") attached hereto as Exhibit A.

By signing below, you acknowledge that you are receiving the separation compensation outlined in this section in consideration for waiving your rights to claims referred to in this Agreement and that you would not otherwise be entitled to the separation compensation.

4.Return of Company Property: You hereby warrant to the Company that you have returned to the Company all property or data of the Company of any type whatsoever that has been in your possession or control.

5.Post-Employment Obligations: You hereby acknowledge that: (a) you continue to be bound by the attached Invention Assignment, Confidentiality and Non-Competition Agreement (Exhibit B hereto); (b) as a result of your employment with the Company, you have had access to the Company's proprietary and/or confidential information, and you will continue to hold all such information in strictest confidence and not make use of it on behalf of anyone; and (c) you must, and by your signature below confirm that you shall, deliver to the Company, no later than the Separation Date, all documents and data of any nature containing or pertaining to such information, and not take with you, or otherwise retain in any respect, any such documents or data or any reproduction thereof.

6.Equity:

a.Stock Options. Pursuant to your Stock Option Agreements with the Company and the Company's 2014 Equity Incentive Plan and the Company's 2019 Equity Incentive Plan (the "***2019 Plan***," and such agreements hereinafter collectively referred to as the "***Stock Option Agreements***") you were granted options to purchase an aggregate of 480,597 shares of the Company's Common Stock (the "***Options***"). From the Effective Date until the Advisory Period End Date (as defined in the Advisor Agreement), the Options will continue to vest in accordance with the terms of the applicable Stock Option Agreement; *however*, vesting will cease as of the Advisory Period End Date (as defined in the Advisor Agreement) (assuming you continue to provide services to the Company through such date). As of the Advisory Period End Date, the Options will have vested as to 356,634 shares (the "***Vested Option Shares***") and there will remain 123,963 unvested shares. Your rights concerning the Options will continue to be governed by the Stock Option Agreements. The post-termination exercise period during which you may continue to exercise any unexercised Vested Option Shares following the termination of your services (the "***Post-Termination Exercise Period***") will be determined as set forth in the Stock Option Agreements. After the Post-Termination Exercise Period, you will no longer have a right to exercise the Options as to any shares.

b.Restricted Stock Units. Pursuant to your Restricted Stock Unit Award Agreements with the Company and the 2019 Plan (such agreements hereinafter collectively referred to as the "***RSU Agreements***," and together with the Stock Option Agreements, the "***Equity Agreements***"), you were granted restricted stock unit awards settleable for 153,173 shares of Common Stock (the "***RSUs***"). From the Effective Date until the Advisory Period End Date, the RSUs will continue to vest in accordance with the terms of the applicable RSU Agreement; *however*, vesting will cease as of the Advisory Period End Date (assuming you continue to provide services to the Company through such date). As of the Advisory Period End Date, the RSUs will have vested as to 61,892 shares and there will remain 91,281 unvested shares. Your rights concerning the RSUs will continue to be governed by the RSU Agreements.

c.Performance Stock Units. Pursuant to your Performance Stock Unit Award Agreements with the Company and the 2019 Plan (such agreements hereinafter collectively referred to as the "***PSU Agreements***," and together with the Stock Option Agreements and RSU Agreements, the "***Equity Agreements***"), you were granted performance stock unit awards settleable for up to 40,000 shares of Common Stock (the "***PSUs***," and together with the Options and RSUs, the "***Equity Awards***"). From the Effective Date until the Advisory Period End Date, the PSUs will continue to vest in accordance with the terms of the applicable PSU Agreement; *however*, vesting will cease as of the Advisory Period End Date (assuming you continue to provide services to the Company through such date). Your rights concerning the PSUs will continue to be governed by the PSU Agreements.

7.General Release and Waiver of Claims:

a.The payments and promises set forth in this Agreement are in full satisfaction of all accrued salary, vacation pay, bonus and commission pay, profit-sharing, stock, stock options or other ownership interest in the Company, termination benefits or other compensation to which you may be entitled by virtue of your employment with the Company or your separation from the Company. To the fullest extent permitted by law, you hereby release and waive any other claims you may have against the Company and its owners, agents, officers, shareholders, employees, directors, attorneys, subscribers, subsidiaries, affiliates, successors and assigns (collectively "***Releasees***"), whether known or not known, including, without limitation, claims under any employment laws, including, but not limited to, claims of unlawful discharge, breach of contract, breach of the covenant of good faith and fair dealing, fraud, violation of public policy, defamation, physical injury, emotional distress, claims for additional compensation or benefits arising out of your employment or your separation of employment, claims under Title VII of the 1964 Civil Rights Act, as amended, the Massachusetts Fair Employment Practices Law, the Massachusetts Wage Act (the "***MA Wage Act***"), and any other laws and/or regulations relating to employment or employment discrimination, including, without limitation, claims based on age or under the Age Discrimination in Employment Act or Older Workers Benefit Protection Act, and/or claims based on disability or under the Americans with Disabilities Act.

b.**THIS RELEASE CONTAINS A WAIVER OF RIGHTS UNDER THE MA WAGE ACT**: You acknowledge, agree and understand that employees have certain rights under the MA Wage Act regarding when, how, and how much they must be paid, including but not limited to the right to be paid wages earned within timeframes provided in the MA Wage Act; that wages include amounts payable to employee for hours worked, which may include salaries, determined and due commissions, overtime pay, tips, and earned vacation or holiday payments due to employees under oral or written agreements; and that employees have the right to bring private lawsuits for violation of the MA Wage Act.

c.You hereby acknowledge that you are aware of the principle that a general release does not extend to claims that the releasor does not know or suspect to exist in his or her favor at the time of executing the release, which, if known by him or her, must have materially affected his or her settlement with the releasee. With knowledge of this principle, you hereby agree to expressly waive any rights you may have to that effect.

d. You and the Company do not intend to release claims that you may not release as a matter of law, including but not limited to any claims for enforcement of this Agreement. To

the fullest extent permitted by law, any dispute regarding the scope of this general release shall be determined by an arbitrator under the procedures set forth in the arbitration clause below.

8.Covenant Not to Sue:

a.To the fullest extent permitted by law, at no time subsequent to the execution of this Agreement will you pursue, or cause or knowingly permit the prosecution, in any state, federal or foreign court, or before any local, state, federal or foreign administrative agency, or any other tribunal, of any charge, claim or action of any kind, nature and character whatsoever, known or unknown, which you may now have, have ever had, or may in the future have against Releasees, which is based in whole or in part on any matter released by this Agreement.

b.Nothing in this section shall prohibit or impair you or the Company from complying with all applicable laws, nor shall this Agreement be construed to obligate either party to commit (or aid or abet in the commission of) any unlawful act.

9.Protected Rights: You understand that nothing in this Agreement, including the General Release and Waiver of Claims, Covenant Not to Sue, Non-disparagement and Confidentiality sections contained herein, limits, impedes or restricts your ability to file a charge or complaint with the Equal Employment Opportunity Commission, the National Labor Relations Board, the Occupational Safety and Health Administration, the Securities and Exchange Commission or any other federal, state or local government agency or commission ("***Government Agencies***"). You further understand that this Agreement does not limit your ability to communicate with any Government Agencies or otherwise participate and/or assist in any investigation or proceeding that may be conducted by any Government Agency, including providing documents or other information, without notice to the Company. This Agreement does not limit your right to receive an award for information provided to any Government Agencies or prohibit you from providing truthful information in response to a subpoena or other legal process.

10.Non-disparagement: Subject to the Protected Rights section above, and otherwise to the fullest extent permitted by applicable law, you agree that you will not, directly or indirectly, disparage or make negative remarks regarding Releasees or their products, services, agents, representatives, directors, officers, shareholders, attorneys, employees, vendors, affiliates, successors or assigns, or any person acting by, through, under or in concert with any of them, with any written or oral statement, including, but not limited to, any statement posted on social media (including online company review sites) or otherwise on the Internet, whether or not made anonymously or with attribution.

11.Arbitration: Except for any claim for injunctive relief arising out of a breach of a party's obligations to protect the other's proprietary information, the parties agree to arbitrate, in Boston, Massachusetts through JAMS, any and all disputes or claims arising out of or related to the validity, enforceability, interpretation, performance or breach of this Agreement, whether sounding in tort, contract, statutory violation or otherwise, or involving the construction or application or any of the terms, provisions, or conditions of this Agreement. Any arbitration may be initiated by a written demand to the other party. The arbitrator's decision shall be final, binding, and conclusive. The parties further agree that this Agreement is intended to be strictly construed to provide for arbitration as the sole and exclusive means for resolution of all disputes

hereunder to the fullest extent permitted by law. The parties expressly waive any entitlement to have such controversies decided by a court or a jury.

12.Attorneys' Fees: If any action is brought to enforce the terms of this Agreement, the prevailing party will be entitled to recover its reasonable attorneys' fees, costs and expenses from the other party, in addition to any other relief to which the prevailing party may be entitled.

13.Confidentiality: Subject to the Protected Rights section above, and otherwise to the fullest extent permitted by applicable law, the contents, terms and conditions of this Agreement must be kept confidential by you and may not be disclosed except to your immediate family, accountant or attorneys or pursuant to subpoena or court order. You agree that if you are asked for information concerning this Agreement, you will state only that you and the Company reached an amicable resolution of any disputes concerning your separation from the Company. Any breach of this confidentiality provision shall be deemed a material breach of this Agreement.

14.No Admission of Liability: This Agreement is not and shall not be construed or contended by you to be an admission or evidence of any wrongdoing or liability on the part of Releasees, their representatives, heirs, executors, attorneys, agents, partners, officers, shareholders, directors, employees, subsidiaries, affiliates, divisions, successors or assigns. This Agreement shall be afforded the maximum protection allowable under the Federal Rules of Evidence 408 and/or any other state or federal provisions of similar effect.

15.Complete and Voluntary Agreement: This Agreement, together with the exhibits hereto and the agreements referenced herein, constitute the entire agreement between you and Releasees with respect to the subject matter hereof and supersedes all prior negotiations and agreements, whether written or oral, relating to such subject matter. You acknowledge that neither Releasees nor their agents or attorneys have made any promise, representation or warranty whatsoever, either express or implied, written or oral, which is not contained in this Agreement for the purpose of inducing you to execute the Agreement, and you acknowledge that you have executed this Agreement in reliance only upon such promises, representations and warranties as are contained herein, and that you are executing this Agreement voluntarily, free of any duress or coercion.

16.Severability: The provisions of this Agreement are severable, and if any part of it is found to be invalid or unenforceable, including, without limitation, any part of the General Release, Covenant Not to Sue, Non-disparagement and/or Confidentiality sections above, the other parts shall remain fully valid and enforceable. Specifically, should a court, arbitrator, or government agency conclude that a particular claim may not be released as a matter of law, it is the intention of the parties that the general release, the waiver of unknown claims and the covenant not to sue above shall otherwise remain effective to release any and all other claims.

17.Modification; Counterparts; Electronic/PDF Signatures: It is expressly agreed that this Agreement may not be altered, amended, modified, or otherwise changed in any respect except by another written agreement that specifically refers to this Agreement, executed by authorized representatives of each of the parties to this Agreement. This Agreement may be executed in any number of counterparts, each of which shall constitute an original and all of which together shall constitute one and the same instrument. Execution of an electronic or PDF

copy shall have the same force and effect as execution of an original, and a copy of a signature will be admissible in any legal proceeding as if an original.

18.Review of Separation Agreement; Expiration of Offer: You understand that you may take up to twenty-one (21) days to consider this Agreement (the "***Consideration Period***"). The offer set forth in this Agreement, if not accepted by you before the end of the Consideration Period, will automatically expire. By signing below, you affirm that you were advised to consult with an attorney prior to signing this Agreement. You also understand you may revoke this Agreement within seven (7) days of signing this document and that the separation compensation to be provided to you pursuant to Section 3 will be provided only after the expiration of that seven (7) day revocation period.

19.Effective Date: This Agreement is effective on the eighth (8th) day after you sign it and without revocation by you (the "***Effective Date***").

20.Governing Law: This Agreement shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts.

If you agree to abide by the terms outlined in this Agreement, please sign this and return it to me. I wish you the best in your future endeavors.

Sincerely,

Stoke Therapeutics, Inc.

By: /s/ Edward M. Kaye
Edward M. Kaye, Chief Executive Officer

READ, UNDERSTOOD AND AGREED

/s/ Stephen Tulipano Date: May 7, 2024
Stephen Tulipano

EXHIBIT A

ADVISOR AGREEMENT

Stoke Therapeutics, Inc.

May 7, 2024

Via Email

Stephen Tulipano

Re: Advisory Services to Stoke Therapeutics, Inc.

Dear Stephen:

This letter agreement is to confirm our understanding with respect to your role as an advisor to Stoke Therapeutics, Inc. (the "***Company***"). The Company looks forward to a continued mutually beneficial association with you on the following terms, which are hereby made effective as of May 8, 2024, the date on which you first started providing advisory services to the Company (the "***Effective Date***"):

1.Informal Management Consultations. From time to time, I and possibly other members of the Company's management may contact you informally to provide advice relating to the Company's business. You agree to be available to the Company's management for consultations by telephone, email or in person, as your time and other business activities permit. You also agree to use reasonable efforts to attend meetings, if any, of the Company's advisors, which we anticipate will occur infrequently.

2.Compensation. As the sole consideration for the advisory services, the Company agrees to continue vesting of the Equity Awards (as defined in the separation agreement between you and the Company dated May 2, 2024 (the "***Separation Agreement***"), to which this letter agreement is attached as Exhibit A) for so long as you are providing services under this letter agreement and pursuant to the terms of the Equity Agreements (as defined in the Separation Agreement).

3.Reimbursement of Expenses. The Company will reimburse you for reasonable out-of-pocket expenses that you incur in connection with your services under this letter agreement, including conference, travel, and lodging expenses, provided that the Chief Executive Officer of the Company approves any such expenses in advance.

4.Independent Contractor. Your relationship with the Company will be that of an independent contractor, and you will not be an agent, employee or representative of the Company. You understand that you will have no authority to enter into contracts or create obligations on behalf of the Company. Accordingly, you acknowledge that you will not be eligible for any employee benefits, and that the Company will not make any tax withholdings on your behalf. In the event you receive compensation from the Company in connection with your services under this agreement, you agree that you are obligated to report any such compensation as income, and you agree to pay all withholding taxes, social security, workers' compensation, unemployment and disability insurance or similar items required by any government agency. You agree to indemnify

and hold the Company harmless from and against all damages, liabilities, losses, penalties, fines, expenses and costs (including reasonable fees and expenses of attorneys and other professionals) arising out of or relating to any obligation imposed by law on the Company to pay any withholding taxes, social security, unemployment or disability insurance or similar items in connection with any compensation received by you pursuant to this Agreement.

5.Property of the Company. For purposes of this letter agreement, "***Designs and Materials***" shall mean all designs, discoveries, inventions, products, computer programs, procedures, improvements, developments, drawings, notes, documents, information and materials made, conceived or developed by you alone or with others that result from or that are made, conceived or developed in connection with the services you provide to the Company pursuant to this letter agreement. You hereby irrevocably transfer and assign to the Company any and all of your right, title and interest in and to Designs and Materials, including but not limited to all copyrights, patent rights, trade secrets, trademarks and moral rights. You agree: (a) to disclose promptly in writing to the Company all Designs and Materials; (b) to cooperate with and assist the Company to apply for, and to execute any applications and/or assignments to obtain, any patent, copyright, trademark or other legal protection for Designs and Materials in the Company's name as the Company deems appropriate; and (c) to otherwise treat all Designs and Materials as "***Confidential Information***," as defined below.

6.Confidential Information. You recognize that, in the course of performing your services under this letter agreement, you will acquire information and materials from the Company and knowledge about information of a confidential or secret nature concerning the Company, including without limitation, knowledge about the Company's business, products and planned products, marketing plans, financial information, forecasts, personnel, customers, clients, suppliers, experimental work and programming techniques. All such knowledge, information and materials acquired, the existence, terms and conditions of this letter agreement, and all Designs and Materials, are and will be the trade secrets and confidential and proprietary information of the Company (collectively, the "***Confidential Information***"). Confidential Information will not include, however, any information which is or becomes part of the public domain through no fault of yours or that the Company regularly gives to third parties without restriction on use or disclosure. You agree to hold all such Confidential Information in strict confidence, not to disclose it to others or use it in any way, commercially or otherwise (including without limitation lecturing upon or publishing articles concerning Confidential Information), except in performing your obligations under this letter agreement, and not to allow any unauthorized person access to it. You agree to return to the Company promptly upon request, and in any event after termination or expiration of this letter agreement, any and all records, paper, media or other embodiment containing any Confidential Information. Nothing in this Section 6 or otherwise in this agreement shall limit or restrict in any way your immunity from liability for disclosing Company's trade secrets as specifically permitted by 18 U.S. Code Section 1833, the pertinent provisions of which are attached hereto as Exhibit 1.

7.Conflicts of Interest. You hereby represent that the obligations contemplated hereby do not, in any way, conflict with any other agreement and/or commitment on your part. You agree to inform the Company promptly and in writing if any such conflict arises. You agree that you will not disclose to the Company any proprietary information that you currently have obtained, or may obtain in the future, from any other individual or organization.

8.Non-Solicitation. During the term in which you provide services to the Company pursuant to this letter agreement and for a one (1) year period following the termination of this letter agreement, you will not directly or indirectly solicit away any employees or consultants of the Company for your benefit or for the benefit of any other person or entity.

9.Term; Termination. This letter agreement will commence on the Effective Date and, unless terminated earlier in accordance with the terms herein, will remain in force and effect until December 31, 2024 (the "***Advisory Period End Date***"). Either party may terminate this letter agreement for any reason upon five (5) days written notice. The provisions of Sections 4, 5, 6, 7, 8, 9 and 10 of this letter agreement will survive any expiration or termination of this letter agreement.

10.Interpretation. The terms contained in this letter agreement are subject to interpretation under the laws of the Commonwealth of Massachusetts, without giving effect to that body of laws pertaining to conflict of laws, and can be amended only in writing and by joint agreement of both you and the Company. If any provision of this letter agreement is determined by any court or arbitrator of competent jurisdiction to be invalid, illegal or unenforceable in any respect, such provision will be enforced to the maximum extent possible given the intent of the parties hereto. If such provision cannot be so enforced, such provision shall be stricken from this letter agreement and the remainder of this letter agreement shall be enforced as if such invalid, illegal or unenforceable provision had (to the extent not enforceable) never been contained in the letter agreement. This letter agreement constitutes the complete and exclusive understanding and agreement of you and the Company and supersedes all prior understanding and agreements, whether written or oral, with respect to the subject matter hereof. This letter agreement may be executed in two or more counterparts, including by electronic signature transmission, with the same force and effect as if each of the signatories had executed the same instrument.

If the foregoing represents your understanding of your role as an advisor to the Company, please sign below and return the executed letter agreement to me.

Very truly yours,

Stoke Therapeutics, Inc.

By: /s/ Edward M. Kaye
Edward Kaye,
Chief Executive Officer

AGREED AND CONSENTED TO:

/s/ Stephen Tulipano
Stephen Tulipano

EXHIBIT 1 TO ADVISOR AGREEMENT

DEFEND TRADE SECRETS ACT, 18 U.S. CODE § 1833 NOTICE:

18 U.S. Code Section 1833 provides as follows:

Immunity From Liability For Confidential Disclosure Of A Trade Secret To The Government Or In A Court Filing. An individual shall not be held criminally or civilly liable under any Federal or State trade secret law for the disclosure of a trade secret that (A) is made, (i) in confidence to a Federal, State, or local government official, either directly or indirectly, or to an attorney; and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (B) is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal.

Use of Trade Secret Information in Anti-Retaliation Lawsuit. An individual who files a lawsuit for retaliation by an employer for reporting a suspected violation of law may disclose the trade secret to the attorney of the individual and use the trade secret information in the court proceeding, if the individual (A) files any document containing the trade secret under seal; and (B) does not disclose the trade secret, except pursuant to court order.

EXHIBIT B

INVENTION ASSIGNMENT, CONFIDENTIALITY AND NON-COMPETITION AGREEMENT

Exhibit 31.1

CERTIFICATION PURSUANT TO RULE 13a-14(a) OR 15d-14(a) OF THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Edward M. Kaye, certify that:

1.I have reviewed this Quarterly Report on Form 10-Q of Stoke Therapeutics, Inc.;

2.Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3.Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4.The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a.Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b.Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c.Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d.Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5.The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a.All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b.Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2024

/s/ Edward M. Kaye, M.D.
Edward M. Kaye, M.D.
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO RULE 13a-14(a) OR 15d-14(a) OF THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Thomas E. Leggett, certify that:

1.I have reviewed this Quarterly Report on Form 10-Q of Stoke Therapeutics, Inc.;

2.Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3.Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4.The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a.Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b.Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c.Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d.Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5.The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a.All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b.Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2024

/s/ Thomas E. Leggett
Thomas E. Leggett
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Edward M. Kaye, Chief Executive Officer of Stoke Therapeutics, Inc. (the "Company"), do hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

1.the Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended June 30, 2024 (the "Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2.the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 7, 2024

/s/ Edward M. Kaye, M.D.
Edward M. Kaye, M.D.
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Thomas E. Leggett, Chief Financial Officer of Stoke Therapeutics, Inc. (the "Company"), do hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

1.the Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended June 30, 2024 (the "Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2.the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 7, 2024

/s/ Thomas E. Leggett
Thomas E. Leggett
Chief Financial Officer
(Principal Financial Officer and
Principal Accounting Officer)