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 March 31, 2025

☐ **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 April 30, 2025 the registrant had 54,596,924 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 receipt of upfront payments, potential milestone or royalty payments or the exercise of options under our collaboration with Biogen International GmbH ("Biogen"), 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, tariffs, 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 success of our collaboration with Biogen;
- 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)

	March 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 274,817	$ 127,983
Marketable securities - current	82,507	88,916
Prepaid expenses	12,694	11,117
Restricted cash - current	75	75
Interest receivable	606	700
Other current assets	5,037	3,965
Total current assets	$ 375,736	$ 232,756
Marketable securities - long-term	22,989	29,824
Restricted cash - long-term	721	721
Operating lease right-of-use assets	3,831	4,345
Property and equipment, net	3,611	3,909
Total assets	$ 406,888	$ 271,555
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,186	$ 2,498
Accrued and other current liabilities	24,335	18,567
Deferred revenue - current portion	18,164	18,991
Total current liabilities	$ 44,685	$ 40,056
Deferred revenue - net of current portion	10,255	—
Other long term liabilities	1,874	2,478
Total long term liabilities	12,129	2,478
Total liabilities	$ 56,814	$ 42,534
Commitments and contingencies (Note 6)		
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 54,595,714 and 54,032,826 shares issued and outstanding as of March 31, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	728,124	719,997
Accumulated other comprehensive loss	(104)	(151)
Accumulated deficit	(377,951)	(490,830)
Total stockholders' equity	$ 350,074	$ 229,021
Total liabilities and stockholders' equity	$ 406,888	$ 271,555

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

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

	Three Months Ended March 31,	
	2025	2024
Revenue	$ 158,569	$ 4,216
Operating expenses:		
Research and development	32,676	22,368
General and administrative	14,653	10,220
Total operating expenses	47,329	32,588
Income (loss) from operations	111,240	(28,372)
Other income (expense):		
Interest income (expense), net	2,889	2,426
Other income (expense), net	28	(428)
Total other income (expense)	2,917	1,998
Income (loss) before income taxes	$ 114,157	$ (26,374)
Provision for income taxes	1,278	—
Net income (loss)	$ 112,879	$ (26,374)
Net income (loss) per share:		
Basic	$ 1.95	$ (0.57)
Diluted	1.90	(0.57)
Weighted-average common shares outstanding:		
Basic	57,862,674	46,246,889
Diluted	59,398,600	46,246,889
Comprehensive income (loss):		
Net income (loss)	$ 112,879	$ (26,374)
Other comprehensive gain:		
Unrealized gain on marketable securities	47	24
Total other comprehensive gain	$ 47	$ 24
Comprehensive income (loss)	$ 112,926	$ (26,350)

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, 2023	**45,918,233**	**$ 5**	**$ 561,433**	**$ (24)**	**$ (401,849)**	**$ 159,565**
Unrealized gain on marketable securities	—	—	—	24	—	24
Stock-based compensation	—	—	5,410	—	—	5,410
Issuance of common stock upon exercise of stock options	79,682	—	250	—	—	250
Issuance of common stock related to employee stock purchase plan	37,542	—	168	—	—	168
Issuance of vested restricted stock units and performance stock units	190,350	—	—	—	—	—
Shares sold as part of controlled equity offering sales agreement, net of commissions	272,270	—	1,299	—	—	1,299
Net loss	—	—	—	—	(26,374)	(26,374)
Balance as of March 31, 2024	**46,498,077**	**$ 5**	**$ 568,560**	**$ —**	**$ (428,223)**	**$ 140,342**
Balance as of December 31, 2024	**54,032,826**	**$ 5**	**$ 719,997**	**$ (151)**	**$ (490,830)**	**$ 229,021**
Unrealized gain on marketable securities	—	—	—	47	—	47
Stock-based compensation	—	—	6,753	—	—	6,753
Issuance of common stock upon exercise of stock options	214,996	—	1,004	—	—	1,004
Issuance of common stock related to employee stock purchase plan	39,467	—	370	—	—	370
Issuance of vested restricted stock units and performance stock units	308,425	—	—	—	—	—
Net income	—	—	—	—	112,879	112,879
Balance as of March 31, 2025	**54,595,714**	**5**	**728,124**	**(104)**	**(377,951)**	**350,074**

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)

	Three Months Ended March 31,	
	2025	2024
Cash flows from operating activities:		
Net income (loss)	$ 112,879	$ (26,374)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Depreciation	477	563
Amortization and accretion of marketable securities	(494)	(24)
Stock-based compensation	6,753	5,410
Reduction in the carrying amount of right of use assets	599	550
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(2,555)	(349)
Accounts payable, accrued liabilities and lease liabilities	4,741	(1,919)
Deferred revenue	9,427	(2,423)
Net cash provided by (used in) operating activities	131,827	(24,566)
Cash flows from investing activities:		
Purchases of marketable securities	(19,016)	—
Purchases of property and equipment	(152)	(12)
Sales of marketable securities	32,800	10,000
Net cash provided by investing activities	13,632	9,988
Cash flows from financing activities:		
Proceeds from Employee Stock Purchase Plan	370	168
Proceeds from issuance of common stock upon exercise of stock options	1,004	250
Proceeds from issuance of common stock in controlled equity offering sales agreements	—	1,299
Net cash provided by financing activities	1,374	1,717
Net increase (decrease) in cash, cash equivalents and restricted cash	146,833	(12,861)
Cash, cash equivalents and restricted cash—beginning of period	128,780	192,011
Cash, cash equivalents and restricted cash—end of period	$ 275,613	$ 179,150
Supplemental Disclosure of Non-Cash Investing and Financing Activities:		
Property and equipment included in accrued expense and accounts payable	$ 27	$ 6
Deferred offering costs not yet paid	$ —	$ 402

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 an early-stage biopharmaceutical company pioneering a new way to treat the underlying causes of severe genetic diseases by precisely upregulating protein expression.

Shelf Registration

In May 2022, the Company filed a universal Shelf Registration statement on Form S-3 (the "Registration Statement") with the Securities and Exchange Commission (the "SEC"). The Registration Statement was declared effective by the SEC on May 31, 2022, and contained two prospectuses: a base prospectus, which covered 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 were specified in a prospectus supplement to the base prospectus. The $150.0 million of common stock that could have been offered, issued and sold under the Sales Agreement prospectus was included in the $400.0 million of securities that could have been offered, issued and sold by the Company under the base prospectus. As of March 31, 2025, the Company had issued approximately 7.0 million shares of common stock pursuant to the Sales Agreement for net proceeds of $61.0 million.

In April 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 approximately $119.9 million after deducting underwriting discounts, commissions and offering costs.

In October 2024, the Company filed an automatic universal Shelf Registration statement on Form S-3 (the "2024 Registration Statement") with the SEC. The 2024 Registration Statement contains two prospectuses: a base prospectus, which covers the offering, issuance and sale by the Company 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 the 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. Following the filing of the Company's Annual Report on Form 10-K for the year ended December 31, 2024 in March 2025, the 2024 Registration Statement is no longer effective. In addition, as of March 31, 2025, the Company had not issued or sold any shares pursuant to the 2024 Registration Statement.

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, 2024, which was filed with the SEC on March 18, 2025. The Company's financial information as of March 31, 2025 and for the three months ended March 31, 2025 and 2024 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, 2024 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 March 31, 2025, 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 March 31,	
	2025	2024
Cash and cash equivalents	$ 274,817	$ 178,581
Restricted cash - current	75	—
Restricted cash - long-term	721	569
Total cash, cash equivalents and restricted cash	$ 275,613	$ 179,150

Smaller reporting company status

The Company is a "smaller reporting company," meaning that the market value of its stock held by non-affiliates was less than $700.0 million and its annual revenue is less than $100.0 million as of the last business day of its most recently completed fiscal year. The Company will continue to be a smaller reporting company for so 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. In addition, 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 smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Revenue Recognition under ASC 606

For revenue arrangements accounted for under ASC 606, the Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, the Company performs the following five steps: (i) identification of the contract; (ii) determination of whether the promised goods or services are performance obligations; (iii) measurement of the transaction price, including evaluating the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. The Company applies the five-step model to all contracts, including transactions with collaborators or partners, if they are a transaction with a customer.

Performance obligations are promises to transfer distinct goods or services to the customer. Promised goods or services are considered distinct when (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised good or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the customer to develop the intellectual property on their own, or whether the required expertise is readily available.

The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include both fixed consideration and variable consideration. At the inception of an arrangement that includes variable consideration and at each reporting period, the Company evaluates the amount of potential payment and the likelihood that the payments will be received. The Company utilizes either the most likely amount method or expected amount method to estimate the amount to be received based on which method better predicts the amount expected to be received. If it is probable that a significant reversal of cumulative revenue would not occur, the variable consideration is included in the transaction price. The Company will assess its revenue generating arrangements in order to determine whether a significant financing component exists and conclude that a significant financing component does not exist in any of its arrangements if: (a) the promised consideration approximates the cash selling price of the promised goods and services or any significant difference is due to factors other than financing; and (b) timing of payment approximates the transfer of goods and services and performance is over a relatively short period of time within the context of the entire term of the contract.

Contracts may include pre-commercial milestone payments. At contract inception and at each reporting period, the Company assesses whether achievement of the milestone was most likely and whether it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur. If it is probable that a significant reversal of cumulative revenue would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's control or the customer's control are constrained and not included in the transaction price. At the end of each subsequent reporting period, the Company re-evaluates the probability of achievement of such milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment.

For arrangements that may include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of the Company's collaboration arrangements.

The Company allocates the transaction price based on the estimated standalone selling price of the underlying performance obligations or in the case of certain variable consideration to one or more performance obligations. The Company must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. The Company utilizes a range of key assumptions to determine the stand-alone selling price, which may include prices observed in comparable market transactions, pricing considered in negotiating the transaction, the estimated costs, plus a reasonable margin, forecasted revenue, development timelines, reimbursement rates for personnel costs, discount rates and probabilities of success in achieving various pre-commercial and commercial milestones, to complete the respective performance obligation. Certain variable consideration is allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated to each performance obligation are consistent with the amounts the Company would expect to receive for each performance obligation.

For performance obligations consisting of licenses, research and development activities, and other promises, the Company utilizes judgment to assess the nature of the performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. If the license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company will recognize revenue when the license is transferred to the customer and the customer is able to use and benefit from the license. Upfront payments and fees are recorded as deferred revenue upon receipt or when due until the Company performs its obligations under these arrangements. Amounts are recorded as accounts receivable when the right to consideration is unconditional, including unbilled amounts.

Payment terms and conditions vary by contract type, although terms typically require payment within 30 to 60 days. In instances where the timing of revenue recognition differs from that of invoicing, the Company has determined that its contracts generally do not include a significant financing component. The primary purpose of invoicing terms is to provide customers with simplified and predictable ways of purchasing the Company's products and services, not to facilitate financing arrangements.

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 March 31, 2025			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 270,159	$ —	$ —	$ 270,159
Total	$ 270,159	$ —	$ —	$ 270,159
Marketable Securities:				
Corporate bonds	$ —	$ 24,446	$ —	$ 24,446
Commercial paper	—	14,775	—	14,775
US Government debt securities	—	66,275	—	66,275
Total	$ —	$ 105,496	$ —	$ 105,496

	Fair value measurements as of December 31, 2024			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 122,446	$ —	$ —	$ 122,446
Total	$ 122,446	$ —	$ —	$ 122,446
Marketable Securities:				
Corporate bonds	$ —	$ 18,668	$ —	$ 18,668
Commercial paper	—	6,944	—	6,944
US Government debt securities	—	93,128	—	93,128
Total	$ —	$ 118,740	$ —	$ 118,740

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 March 31, 2025 and December 31, 2024.

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 March 31, 2025 (in thousands):

	March 31, 2025			
	Amortized Cost	Unrealized Gains	Unrealized Loss	Fair Value
Marketable securities:				
Corporate bonds	$ 24,493	$ 5	$ (52)	$ 24,446
Commercial paper	14,776	2	(3)	14,775
US Government debt securities	66,330	16	(71)	66,275
Total	$ 105,599	$ 23	$ (126)	$ 105,496

The following table summarizes the Company's marketable securities as of December 31, 2024 (in thousands):

	December 31, 2024			
	Amortized Cost	Unrealized Gains	Unrealized Loss	Fair Value
Marketable securities:				
Corporate bonds	$ 18,786	$ —	$ (118)	$ 18,668
Commercial paper	6,947	—	(3)	6,944
US Government debt securities	93,158	94	(124)	93,128
Total	$ 118,891	$ 94	$ (245)	$ 118,740

As of March 31, 2025, the weighted average maturity of the Company's marketable securities is 0.63 years.

The Company did not record an allowance for credit losses as of March 31, 2025 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):

	March 31, 2025	December 31, 2024
Accrued employee compensation costs	$ 2,938	$ 7,686
Accrued professional costs	1,424	1,108
Accrued research and development costs	15,548	6,698
Current portion of operating lease liabilities	2,351	2,322
Other current liabilities	2,074	753
	$ 24,335	$ 18,567

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. In March 2025, the Company further amended this lease to extend the term from April 30, 2025 to June 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.

In January 2025, the Company entered into an agreement to lease 7,581 square feet of space for an initial term of three years and three months. The lease includes two renewal options for an additional three years each. Lease terms commence at $0.7 million per annum, with 2.0% annual base rent increases plus operating expenses, real estate taxes, and utilities. The lease commencement date is expected to be July 1, 2025. As the Company did not have access to the space, a right-of-use asset was not recognized as of March 31, 2025.

Future minimum lease payments under non-cancellable leases as of March 31, 2025 were as follows (in thousands):

2025	$	2,167
2026		3,250
2027		699
2028		532
Total lease payments		6,648
Less imputed interest		(698)
Present value of lease liabilities	$	5,950

Lease balances as of March 31, 2025 were as follows (in thousands):

Operating right-of-use assets	$	3,831
Current Portion of operating lease liabilities	$	2,351
Non-current portion of operating lease liabilities		1,861
Total operating lease liabilities	$	4,212

The weighted average remaining lease term and weighted average discount rate of the Company’s operating leases as of March 31, 2025 were as follows:

Weighted average remaining lease term in years	2.3
Weighted average discount rate	10.25%

Lease expense incurred under operating leases was $0.7 million for the three months ended March 31, 2025 and was $0.7 million for the three months ended March 31, 2024.

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 March 31, 2025, the Company had paid $0.7 million under the Southampton Agreement as a result of entering into the Acadia Pharmaceuticals Inc. license and collaboration agreement in January 2022 and had recorded a liability of $8.2 million, for a sublicense fee expense, due to the Biogen license and collaboration agreement (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 March 31, 2025 these expenses were $0.05 million compared to $0.03 million for the three months ended March 31, 2024.

7. License and collaboration agreements

Acadia Pharmaceuticals Inc.

In January 2022, the Company entered into a License and Collaboration Agreement (the "Acadia 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 Acadia 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 Acadia 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 Acadia 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 March 31, 2025, the Company had $14.4 million in upfront consideration associated with the Acadia Agreement relating to performance obligations that are unsatisfied or partially unsatisfied.

Biogen International GmbH License and Collaboration Agreement

On February 14, 2025 (the "Effective Date"), the Company entered into a License and Collaboration Agreement (the "Biogen Agreement") with Biogen International GmbH ("Biogen") for the joint development and commercialization of zorevunersen and other compounds targeting the SCN1A gene (the "Licensed Product"). Under the terms of the Biogen Agreement, the Company granted Biogen an exclusive, royalty-bearing license to develop, manufacture, and export licensed products in all territories outside the U.S., Canada, and Mexico (the "Biogen Territory"). In addition, Biogen has the option to exercise an exclusive, royalty-bearing, sublicensable and transferable license for certain future follow-on ASOs directed to SCN1A controlled by the Company.

Under the Biogen Agreement, the parties are jointly developing zorevunersen, with the Company leading global development. The Company and Biogen share global development costs based on an agreed budget. The Company is responsible for 70.0% of these development costs and Biogen is responsible for the remaining 30.0% of these development costs.

In connection with the closing of this transaction in February 2025, the Company received an upfront payment of $165.0 million and is eligible to receive development and commercial milestone payments that could total up to approximately $50.0 million and $335.0 million, respectively, if all the specified milestones set forth in this collaboration are achieved. In addition, the Company is eligible to receive tiered, double-digit royalties ranging from the low double-digit to high teen percentages of future net sales on the licensed products. Royalties payable under the Agreement are subject to standard royalty reductions.

Biogen agreement accounting

At the commencement of the Agreement, the Company evaluated the Biogen Agreement and determined that it was reflective of a vendor-customer relationship and comprised of the following performance obligations within the scope of ASC 606.

As of the Effective Date, the Company identified the following performance obligations in the Biogen Agreement that were evaluated under the scope of ASC 606: (i) an exclusive royalty-bearing sublicensable, transferable license under the licensed intellectual property to exploit Licensed Products in the Biogen Territory, and non-exclusive, royalty-free, sublicensable, transferable license under the licensed intellectual property to develop, manufacture and export any Licensed Products in the Biogen Territory (collectively "IP License" and considered as one performance obligation), and (ii) development services, that are expected to generate data necessary for regulatory approval in the Biogen Territory for the licensed products ("Global Development Activities").

The IP License is considered functional intellectual property and distinct from the Global Development Activities, as Biogen can benefit from the license on its own or together with other readily available resources. Global Development Activities are considered distinct as they are not expected to significantly modify or customize the initial intellectual property as the Licensed Product is in Phase 3 of clinical development at contract inception.

The Company determined that the initial transaction price under ASC 606 was $243.4 million and is comprised of the non-refundable upfront payment of $165 million and $78.4 million of estimated variable consideration attributed to cost reimbursement in reimbursement for Global Development Activities. The Company utilized the expected value method to estimate the variable consideration for the future reimbursement of the Global Development Activities, and any subsequent adjustments arising from changes in estimates will be recorded on a cumulative catch-up basis in the period of such adjustment.

The Company determined that royalties and sales milestones relate solely to the licenses of intellectual property and are therefore excluded from the transaction price under the sales- or usage-based royalty exception of ASC 606. In addition, the Company estimated the probability, likelihood, and magnitude of a reversal of revenue for the commercial milestone payments and determined that any variable consideration related to commercial milestones was deemed to be fully constrained at inception and therefore excluded from

the initial transaction price due to high degree of uncertainty and risks associated with these potential payments as the Company can not assert that it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur.

The Company developed the estimated standalone selling price, at inception, for each of the performance obligations with the objective of determining the price at which we would sell each item if it were to be sold regularly on a standalone basis. The Company developed the estimated standalone selling price for the IP License primarily based on the probability-weighted present value of expected future cash flows associated with the underlying license or activity. In developing such estimates, the Company applied judgment in determining the forecasted revenues, taking into consideration the applicable market conditions and relevant entity-specific factors, the probability of success and the discount rate. The estimated standalone selling price for the Global Development Activities is primarily based on the costs necessary to perform the service plus a reasonable margin. In management's judgment the above inputs provide a faithful estimate and reflects a consideration that would be received to transfer the promised goods or services in an orderly transaction between unrelated market participants. As such, the estimated standalone selling price reflects the best estimate of the fair value that market participants would pay for each performance obligation on a standalone basis.

The Company allocated the transaction price to the performance obligations on a relative stand-alone selling price basis consistent with the allocation objective of ASC 606.

The IP License performance obligation was satisfied at a point in time upon transfer of the IP License to Biogen. Control of the IP License was transferred on the Effective Date as Biogen could begin to use and benefit from the IP License immediately. For the Global Development Activities performance obligation, the Company measures proportional performance over time using an input method based on costs incurred relative to the total estimated costs of the obligation by determining the proportion of costs incurred as a percentage of total costs the Company expects to expend. This ratio is applied to the transaction price allocated to this performance obligation to arrive at the cumulative revenue that should be recognized at each reporting date. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

The following table provides a summary of the transaction price allocated to each performance obligation, in addition to revenue activity during the period:

	As of March 31, 2025	
	Transaction Price Allocated	Revenue Recognized
Performance obligations		
IP License	$ 150,779	$ 150,779
Global Development Activity	92,644	1,642
	$ 243,423	$ 152,421

As of March 31, 2025 the Company had $14.0 million in upfront consideration associated with the Biogen 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 March 31, 2025, there were no shares available for future issuance under the 2014 Plan, 1,875,367 shares were available under the 2019 Plan and 31,800 shares were available under the 2023 Plan.

During the three months ended March 31, 2025, the Company granted options to purchase 1,959,171 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 March 31, 2025, the Company granted 1,007,842 RSUs and did not grant any performance stock units ("PSUs") to its employees. The RSUs vest over a period of up to four years.

Stock-based compensation

As of March 31, 2025, there was $38.6 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 2.8 years as of March 31, 2025. As of March 31, 2025, there was $23.7 million of unrecognized stock-based compensation related to RSUs and $1.5 million of unrecognized stock-based compensation related to PSUs. The compensation expense for RSUs and PSUs is expected to be recognized over a weighted average period of 2.9 years.

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 March 31,	
	2025	2024
Research and development	$ 2,799	$ 2,091
General and administrative	3,954	3,319
Total	$ 6,753	$ 5,410

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 March 31, 2025, the Company had 2,131,930 shares available for issuance under the ESPP. The average grant date fair value per share under the ESPP was $11.27 for 2025. The total ESPP stock-based compensation expense for the three months ended March 31, 2025 was $0.2 million, and for the three months ended March 31, 2024 was $0.1 million. 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 income (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 March 31,	
	2025	2024
Numerator:		
Net income (loss)	$ 112,879	$ (26,374)
Denominator:		
Weighted-average number of common shares, basic	57,862,674	46,246,889
Weighted-average number of common shares, diluted	59,398,600	46,246,889
Net loss per share, basic	$ 1.95	$ (0.57)
Net loss per share, diluted	$ 1.90	$ (0.57)

As the Company had net income during the three months ended March 31, 2025, the Company's potential dilutive securities, which include stock options, restricted stock units and performance stock units, were included in the calculation of diluted net income per share attributable to common stockholders. For the three months ended March 31, 2024, the Company's potential dilutive securities, which include stock options, restricted stock units and performance stock units have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share. Therefore, the weighted-average number of common stock

outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same for the three months ended March 31, 2024.

The Company excluded the following potential common stock, presented based on amounts outstanding at March 31, 2025 and 2024 from the computation of diluted net loss per share attributable to common stockholders because including them would have had an anti-dilutive effect.

	March 31,	
	2025	2024
Outstanding options to purchase common stock	7,214,876	7,439,396
Restricted stock units	589,219	2,410,752
Performance stock units	73,000	—
Total	7,877,095	9,850,148

10. Income taxes

For the three months ended March 31, 2025, the Company recorded federal and state tax expense of $1.1 million and $0.1 million, respectively due to recognizing income from the Biogen collaboration, offset by net operating loss carryforwards (NOLs) that were able to be utilized to the extent allowable by tax law. The Company did not record an income tax expense in its consolidated statements of operations and comprehensive loss for the three months ended March 31, 2024. The Company has provided a valuation allowance for the full amount of its net deferred tax assets as of March 31, 2025 and December 31, 2024, 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.

11. Segment Information

The Company comprises of one reportable segment, Stoke. As of March 31, 2025, this segment has not generated any product revenue since inception, as it does not yet have approved products for sale.

The Company primarily generates revenue in North America, with its long-lived assets also concentrated in this region, and manages its business activities on a consolidated basis. Decisions concerning the allocation of the Company’s resources are made by the Company’s Chief Operating Decision Maker (“CODM”), which is the Company’s Interim Chief Executive Officer. The CODM views the Company’s operations as a single operating segment which is the business of discovering and developing RNA-based medicines, using the Targeted Augmentation of Nuclear Gene Output (“TANGO”) approach and developing antisense oligonucleotides (“ASOs”) to selectively restore protein levels.

The CODM assesses performance for the Company's operations segment and decides how to allocate resources based on net income or loss that is also reported on the income statement as consolidated net income or loss and the measure of segment assets as reported on the balance sheet as total consolidated assets. Net income or loss is used to monitor budget vs actual results as well as assess the general performance of the segment in a given period.

The following table reconciles segment direct profit or loss to the Company’s consolidated results (in thousands):

	Three Months Ended March 31,	
	2025	2024
Revenue	$ 158,569	$ 4,216
Zorevunersen[1]	17,674	8,780
ADOA[1]	1,201	1,722
SYNGAP1[1]	342	284
MECP2[1]	147	242
Other external program costs	1,829	2,043
Payroll and other personnel costs - R&D [2]	11,012	8,163
Payroll and other personnel costs - G&A [2]	8,929	6,850
Other non-program costs - R&D [3]	1,407	1,460
Other non-program costs - G&A [3]	4,788	3,044
Subtotal	(47,329)	(32,588)
Other segment items		
Interest expense	(1)	(22)
Interest income	2,890	2,448
Other income (expense)	28	(428)
Provision for income taxes	(1,278)	—
Total other segment items	1,639	1,998
Segment net income (loss)	$ 112,879	$ (26,374)

(1) Includes external research and development and general and administrative expenses associated with programs.
(2) Includes salaries, benefits and other personnel related expenses, including stock based compensation expense, of relevant personnel.
(3) Includes costs incurred for conducting non-program activities related to Company activities.

12. Subsequent events

In May 2025, the Company received notice from Acadia that it had elected to discontinue two of the three research programs under the Acadia Agreement. Under the Acadia Agreement, the licenses granted to Acadia for the discontinued programs, which are the MECP2 program for Rett syndrome and the program for an undisclosed neurodevelopmental target, will terminate and the parties will wind down Acadia's funding for the two programs.

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 late-stage 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 investigational new medicine in development, zorevunersen (STK-001), is a potential disease modifying medicine that is in late-stage clinical testing for the treatment of Dravet syndrome. 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 (DEE) 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.

Following regulatory alignment with the U.S. Food and Drug Administration ("FDA"), European Medicines Agency ("EMA"), and Japan's Pharmaceuticals and Medical Devices Agency ("PMDA"), a Phase 3 study, EMPEROR, is expected to begin in the second quarter of 2025 to further evaluate efficacy and safety of zorevunersen in children and adolescents ages 2 to up to 18 with Dravet syndrome. We have completed Phase 1/2a clinical trials for zorevunersen in patients with Dravet syndrome. The positive results from the Phase 1/2a and open-label extension ("OLE") studies showed substantial and durable reductions in convulsive seizure frequency when administered on top of standard of care anti-seizure medicines. In the Ph1/2a studies, 85% of patients were taking at least three and 54% were taking at least four medicines to control seizures. Half the patients in the studies were taking concomitant fenfluramine. Additionally, ongoing treatment has led to continuous improvements in cognition and behavior through two years. Additional improvements were indicated within the first nine months of treatment among patients in the Phase 1/2a ADMIRAL study. These improvements were observed across multiple domains of theVineland-3 (Vineland Adaptive Behavior Scale, Third Edition), a standardized assessment of behavioral outcomes. Zorevunersen has been generally well tolerated across the studies.

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. There are currently no approved treatments for ADOA. We have received authorization in the UK 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. Since then, we have identified additional important pre-clinical activities as we seek to optimize our clinical plans for STK-002 and better understand the potential for targeting OPA1 beyond ADOA. We anticipate that these data, along with research on the commercial landscape, will provide a more complete picture of the opportunity for a potential disease-modifying medicine for ADOA.

In May 2022, we filed a universal Shelf Registration statement on Form S-3 (the "2022 Registration Statement") with the SEC. The 2022 Registration Statement was declared effective by the SEC on May 31, 2022, and contained two prospectuses: a base prospectus, which covered 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 (the "Sales Agreement").

On April 2, 2024, we completed an underwritten public offering, pursuant to the 2022 Registration Statement, of 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 approximately $119.9 million after deducting underwriting discounts, commissions and offering costs. No pre-funded warrants have been exercised as of March 31, 2025.

In October 2024, we filed an automatic universal Shelf Registration statement on Form S-3 (the "2024 Registration Statement") with the SEC. The 2024 Registration Statement contains two prospectuses: a base prospectus, which covers the offering, issuance and sale by us 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 the 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. Following the filing of our Annual Report on Form 10-K for the year ended December 31, 2024 in March 2025, the 2024 Registrations Statement is no longer effective.

As of March 31, 2025, we had issued approximately 7.0 million shares of common stock pursuant to the Sales Agreement for net proceeds of $61.0 million pursuant to the 2022 Registration Statement. As of March 31, 2025, we had not issued or sold any shares pursuant to the 2024 Registration Statement. We may terminate this at-the-market program at any time, pursuant to its terms.

As of March 31, 2025 and December 31, 2024 we had $380.3 million and $246.7 million, respectively, in cash, cash equivalents and marketable securities.

Since inception, we have primarily had operating losses, the majority of which are attributable to research and development activities. We had a net income of $112.9 million for the three months ended March 31, 2025 and our net loss was $26.4 million for the three months ended March 31, 2024. As of March 31, 2025, we had an accumulated deficit of $378.0 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 cash, cash equivalents, and marketable securities of approximately $380.3 million as of March 31, 2025, will fund operations to mid-2028. 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.

Biogen International GmbH License and Collaboration Agreement

On February 14, 2025, we entered into a License and Collaboration Agreement (the "Biogen Agreement") with Biogen International GmbH ("Biogen") for the joint development and commercialization of zorevunersen and other compounds targeting the SCN1A gene (the "Licensed Product"). Under the terms of the Biogen Agreement, we granted Biogen an exclusive, royalty-bearing license to develop, manufacture, and export licensed products in all territories outside the U.S., Canada, and Mexico (the "Biogen Territory"). In addition, Biogen has the option to exercise an exclusive, royalty-bearing, sublicensable and transferable license for certain future follow-on ASOs directed to SCN1A controlled by us.

Under the Biogen Agreement, the parties are jointly developing zorevunersen, and we are leading global development. Together with Biogen, we share global development costs based on an agreed budget. We are responsible for 70.0% of these development costs and Biogen is responsible for the remaining 30.0% of these development costs.

In connection with the closing of this transaction in February 2025, we received an upfront payment of $165.0 million and are eligible to receive development and commercial milestone payments that could total up to approximately $50.0 million and $335.0 million, respectively, if all the specified milestones set forth in this collaboration are achieved. In addition, we are eligible to receive tiered, double-digit royalties ranging from the low double-digit to high teen percentages of future net sales on the licensed products. Royalties payable under the Agreement are subject to standard royalty reductions.

For the three months ended, March 31, 2025, we recognized revenue related to the Biogen collaboration of $152.4 million.

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

Program Update

Dravet Syndrome Program – zorevunersen

We announced in December 2024 that the FDA had granted zorevunersen Breakthrough Therapy Designation for the treatment of Dravet syndrome with a confirmed mutation, not associated with gain-of-function, in the *SCN1A* gene. Breakthrough Therapy designation is a process designed to expedite the development and review of drugs that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically-significant endpoint(s). This designation grants zorevunersen access to all Fast Track designation features, intensive guidance on an efficient drug development program and an organizational commitment involving senior FDA managers. In January 2025, we announced alignment with the FDA, EMA and PMDA on the design of the Phase 3 EMPEROR study of zorevunersen. The pivotal Phase 3 study will be a global, randomized, double-blind, sham controlled trial and will be conducted at approximately 60 sites across the UK, US, EU, and Japan. The study duration of 60 weeks includes an 8-week baseline period and a 52-week treatment period consisting of two loading doses of 70mg followed by two maintenance doses of 45mg compared to sham in approximately 150 children and adolescents ages 2 to up to 18 with Dravet syndrome.

The primary endpoint will be percent change from baseline in major motor seizure frequency in patients receiving zorevunersen as compared to sham, measured at 28 weeks. Key secondary endpoints will include durability of effect on major motor seizure frequency at week 52 along with improvements in cognition and behavior as measured by Vineland-3 subdomains, including expressive communications, receptive communication, interpersonal relationships, coping skills and personal skills. Additional endpoints include safety, Clinician Global Impression of Change (CGI-C), Caregiver Global Impression of Change (CaGI-C), and the Bayley Scales of Infant Development (BSID-IV).

We plan to initiate the EMPEROR study in the second quarter of 2025, and pivotal data are anticipated in the second half of 2027, pending enrollment and study timelines. Patients who are eligible will be offered ongoing treatment with zorevunersen as part of an open-label extension (OLE) study.

Financial operations overview

Revenue

We are comprised of one reportable segment and as of March 31, 2025 and 2024, have not generated any product revenue since inception, as we do not yet have approved products for sale. 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 (the "Acadia 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. In May 2025, we received notice that Acadia had elected to discontinue the research programs for MECP2 (Rett Syndrome) and the undisclosed neurodevelopmental target. Under the Acadia Agreement, the licenses granted to Acadia for the discontinued programs will terminate and Acadia's funding for such programs will wind down. The collaboration with Acadia with respect to SYNGAP1 remains ongoing.

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.

For the three months ended, March 31, 2025, we recognized revenue related to the Acadia collaboration of $6.1 million.

See *Note 7—License and Collaboration Agreements* 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 March 31,	
	2025	2024
Zorevunersen (STK-001)	$ 16,930	$ 8,534
ADOA	1,056	1,706
SYNGAP1	342	284
MECP2	147	243
Non-program specific and unallocated research and development expenses	14,201	11,601
Total research and development expenses	$ 32,676	$ 22,368

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 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.

Other income (expense)

Our other income (expense), net 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 March 31, 2025 and 2024

The following table sets forth our results of operations:

	Three Months Ended March 31,	
	2025	2024
	(in thousands)	
Consolidated statements of operations:		
Revenue	$ 158,569	$ 4,216
Operating expenses:		
Research and development	32,676	22,368
General and administrative	14,653	10,220
Total operating expenses	47,329	32,588
Income (loss) from operations	$ 111,240	$ (28,372)
Other income (expense):		
Interest income (expense), net	2,889	2,426
Other income (expense), net	28	(428)
Income (loss) before income taxes	$ 114,157	$ (26,374)
Provision for income taxes	1,278	—
Net income (loss)	$ 112,879	$ (26,374)

Revenue

Revenue for the three months ended March 31, 2025 was $158.6 million compared to $4.2 million for the three months ended March 31, 2024, an increase of $154.4 million. Revenue is generated from satisfying contractual obligations of the collaboration and licensing agreements with Acadia and Biogen. The increase of $154.4 million is primarily driven by $150.8 million related to the IP license performance obligation as part of the Biogen agreement.

Research and development expenses

Research and development expenses were $32.7 million for the three months ended March 31, 2025 as compared to $22.4 million for the three months ended March 31, 2024, an increase of $10.3 million. The table below summarizes our research and development expenses (in thousands):

	Three Months Ended March 31,	
	2025	2024
Zorevunersen	$ 16,930	$ 8,534
ADOA	1,056	1,706
SYNGAP1	342	284
MECP2	147	243
Personnel-related expenses	11,012	7,956
Third-party services	526	168
Scientific consulting	317	240
Facilities and other research and development expenses	2,346	3,237
Total research and development expenses	$ 32,676	$ 22,368

The increase in research and development expenses was primarily attributable to an increase of $3.1 million in personnel-related expenses, $8.4 million in expenses related to our zorevunersen program, which is comprised of third-party services and scientific consulting fees, an increase of $0.4 million in external third-party expenses related to SYNGAP1 and MECP2, offset by a decrease of $0.3 million in expenses related to our ADOA program, which is comprised of third-party services and scientific consulting fees, and a decrease of $0.3 million in facilities and other non-program related costs.

General and administrative expenses

General and administrative expenses were $14.7 million for the three months ended March 31, 2025 as compared to $10.2 million for the three months ended March 31, 2024. The increase of $4.5 million in general and administrative expenses was primarily attributable to an increase of $1.9 million in personnel expenses, $2.3 million in professional fees, and an increase $0.3 million in facilities and other general and administrative costs.

Other income (expense)

Other income (expense) was $2.9 million for the three months ended March 31, 2025 as compared to $2.0 million for the three months ended March 31, 2024.

Liquidity and capital resources

Since our inception through March 31, 2025, our operations have been financed by net proceeds of $836.6 million from the sale of convertible notes payable and our convertible preferred stock, our initial public offering, public offerings of common stock and pre-funded warrants, proceeds from the Sales Agreement and the upfront payments from Acadia and Biogen. As of March 31, 2025, we had $380.3 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 primarily incurred losses since our inception in June 2014 and, as of March 31, 2025, we had an accumulated deficit of $378.0 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 cash, cash equivalents, and marketable securities of approximately $380.3 million as of March 31, 2025, will fund operations to mid-2028. 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, tariffs, 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:

	Three Months Ended March 31,	
	2025	2024
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ 131,827	$ (24,566)
Investing activities	13,632	9,988
Financing activities	1,374	1,717
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 146,833	$ (12,861)

Operating activities

During the three months ended March 31, 2025, cash provided by operating activities was $131.8 million. This was primarily attributable to a net income of $112.9 million, a net change of $11.5 million in our net operating assets and liabilities to non-cash charges of $7.3 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 three months ended March 31, 2024, cash used in operating activities was $24.6 million. This was primarily attributable to a net loss of $26.4 million, a net change of $4.7 million in our net operating assets and liabilities, offset by non-cash charges of $6.5 million for stock-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 three months ended March 31, 2025 consisted primarily of $32.8 million from the sales of marketable securities offset by $19.0 million of purchases of marketable securities.

Our investing activities during the three months ended March 31, 2024 consisted of $10.0 million from the sales of marketable securities partially offset by purchases of property and equipment.

Financing activities

Our financing activities during the three months ended March 31, 2025 consisted of $1.0 million from the exercise of stock options and $0.4 million in proceeds from our Employee Stock Purchase Plan (the “ESPP”).

Our financing activities during the three months ended March 31, 2024 consisted of $1.3 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 our Employee Stock Purchase Plan (the "ESPP").

Contractual obligations and commitments

The following table summarizes our contractual obligations as of March 31, 2025 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	$ 5,950	$ 2,167	$ 3,783	$ —	$ —
Total	$ 5,950	$ 2,167	$ 3,783	$ —	$ —

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

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%. In March 2025, we further amended this lease to extend the term from April 30, 2025 to June 30, 2025. In 2021, we 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.

In January 2025, we entered into an agreement to lease 7,581 square feet of space for an initial term of three years and three months. The lease includes two renewal options for an additional three years each. Lease terms commence at $0.7 million per annum, with 2.0% annual base rent increases plus operating expenses, real estate taxes, and utilities. The lease commencement date is expected to be July 1, 2025.

Commitments

Our commitments primarily consist of obligations under our agreement with the University of Southampton. As of March 31, 2025, 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 18, 2025, other than revenue recognition discussed below.

Revenue Recognition

Smaller reporting company status

We are 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 is less than $100.0 million as of the last business day of our most recently completed fiscal year. We will continue to be a smaller reporting company for so 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. In addition, 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 smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Recently Issued Accounting Pronouncements

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 and it will be reflected in the 2025 Annual Report on Form 10-K.

In November 2024, the FASB issued ASU 2024-03 "*Income Statement— Reporting Comprehensive Income—Expense Disaggregation Disclosures*" ("ASU 2024-03"). ASU 2024-03 requires disaggregated disclosure of income statement expenses for public business entities. The ASU does not change the expense captions an entity presents on the face of the income statement; rather, it requires disaggregation of certain expense captions into specified categories in disclosures within the footnotes to the financial statements. ASU 2024-03 is effective for fiscal years beginning after December 15, 2026. The Company does not expect the adoption of ASU 2024-03 to have a material impact on its consolidated financial statements.

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 March 31, 2025 and December 31, 2024, we had cash, cash equivalents and marketable securities of $380.3 million and $246.7 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 March 31, 2025 and 2024.

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 Interim 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 March 31, 2025. 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 Interim Chief Executive Officer and our Chief Financial Officer), as of the end of the period covered by this report, our Interim 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 or the failure of our collaborators to obtain regulatory approval in international jurisdictions would prevent us or our collaborators 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 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.
- 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 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.
- 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 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.

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, and in January 2025 we announced plans to initiate the EMPEROR Phase 3 study.

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, tariffs, 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 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 15,600 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 these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates derived from market research studies 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 or the failure of our collaborators to obtain regulatory approval in international jurisdictions would prevent us or our collaborators from marketing our product candidates outside the United States.

To market and sell zorevunersen, STK-002 and our future product candidates, we or our collaborators 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 or our collaborators 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 or our collaborators fail to comply with the regulatory requirements in international markets and receive applicable marketing approvals, the 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. Foreign regulatory approvals may not be obtained on a timely basis, if at all. 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, if commercially available ASO therapies utilizing intrathecal delivery, including SPINRAZA and QALSODY, both of which are produced by Biogen Inc., are 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. If the law is extended, we may also seek Rare Pediatric Disease designations for any other future product candidates. If a product candidate is designated before December 20, 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 candidates will receive marketing approval.

In December 2024 we announced that the FDA had granted zorevunersen Breakthrough Therapy Designation for the treatment of Dravet syndrome with a confirmed mutation, not associated with gain-of-function, in the SCN1A gene, and we may seek a Breakthrough Therapy Designation for 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 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. The negotiated price may not exceed a statutory ceiling price. 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. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product has been announced. CMS has selected 15 additional Medicare Part D drugs for negotiated maximum fair pricing in 2027. For 2028, an additional 15 drugs, which may be covered under either Medicare Part B or Part D, will be selected, and for 2029 and subsequent years, 20 Part B or Part D drugs will be selected. 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, and in November 2024, CMS finalized regulations pertaining to these inflation rebates. 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, in order for their brand drugs to be reimbursed, to subsidize 10% of Part D enrollees' prescription costs for brand drugs below the out-of-pocket limit, and 20% once the out-of-pocket limit 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 have included statutory aggregate reductions of Medicare payments to providers. More recently, in November 2024, CMS issued a final rule that decreased Medicare reimbursement for physician services by 2.8%, effective January 1, 2025. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Other laws or regulations 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 the statutory cap on Medicaid Drug Rebate Program rebates that manufacturers pay to state Medicaid programs on a unit of drug was 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.

Disruptions at the FDA may slow the time necessary for new products to be reviewed and/or approved, which would adversely affect our business. In addition, there is substantial uncertainty regarding new initiatives under the new Administration and how these might impact the FDA, its implementation of laws, regulations, policies and guidance and its personnel. Similar initiatives may also be directed toward other government agencies. These initiatives could prevent, limit or delay development and regulatory approval of our product candidates, which would adversely affect our business.

Disruptions at the FDA may slow the time necessary for new products to be reviewed and/or approved, which would adversely affect our business. Changes in FDA staffing could result in delays in the FDA's responsiveness or in its ability to review submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all. If any legislation, executive orders, or lapses in agency funding impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Similar consequences would also result in the event of another significant shutdown of the federal government. For example, in 2024, the U.S. government was on the verge of a shutdown and has previously shut down several times, and certain regulatory agencies, such as the FDA, had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, or if geopolitical or global health concerns prevent the FDA from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns or delays could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

FDA-regulated industries, such as ours, face uncertainty with regard to the regulatory environment we will face under the new Administration as we proceed with research and development and potential future commercialization. Some of these efforts have manifested to date as efforts to reduce the size of the federal government, including large-scale reductions in force at FDA. The loss of key personnel at the FDA, including those in leadership positions, is likely to impact operations at the FDA, which could result in, among other things, delays or limitations on our ability to obtain guidance from the FDA on our product candidates in development, longer review times, and delays in obtaining regulatory approvals for our product candidates. Moreover, the new Administration has recently proposed action to freeze or reduce the budget of the National Institutes of Health (the "NIH") as related to its funding for medical research, which could decrease the ability of facilities that rely on NIH funding to enroll and conduct clinical trials or increase the costs to us of conducting clinical trials. There remains general uncertainty regarding future activities. New executive orders, regulations, policies or guidance could be issued or promulgated that adversely affects us or creates a more challenging or costly environment to pursue the development of new therapeutic products. Alternatively, state governments may attempt to address or react to changes at the federal level with changes to their own regulatory frameworks in a manner that is adverse to our operations. If we become negatively impacted by future governmental orders, regulations, policies or guidance, there could be a material adverse effect on us and our 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 initiated 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.

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 have entered into a strategic alliance with Biogen to develop and commercialize zorevunersen, and we may enter into strategic alliances with third parties, such as Acadia Pharmaceuticals, to develop and commercialize 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.

Our current collaborators, such as Acadia Pharmaceuticals and Biogen, and 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 Biogen 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 have also entered into the Agreement with Biogen for the development and commercialization of zorevunersen and other potential products directed to SCN1A.

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 had a net income of $112.9 million for the three months ended March 31, 2025 and our net loss was $26.4 million for the three months ended March 31, 2024. As of March 31, 2025, we had accumulated deficits of $378.0 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 approximately $380.3 million as of March 31, 2025, will fund operations to mid-2028. 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 incur significant 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 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, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a research and clinical development focus to a company that is also capable of marketing 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, 2024, we had federal and state net operating loss carryforwards ("NOLs") of approximately $242.1 million and $261.5 million, respectively, and as of December 31, 2023, we had federal and state NOLs of approximately $189.5 million and $199.4 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, 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. We 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 IRC. 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. For instance, pursuant to the Agreement with Biogen, Biogen has certain rights to prepare, file, prosecute, and maintain certain patents and patent applications licensed to Biogen in the Biogen Territory. 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.

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

The agreements under which we license patents, know-how and proprietary technology to 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 Biogen, pursuant to which we out-license key patents and patent applications related to zorevunersen and certain related products. The agreement imposes various diligence, development and other obligations on both parties. If we or our licensee fail to comply with these respective obligations, either party may have the right to terminate the license, in which event our development or marketing of zorevunersen and related products may be negatively impacted and our business may suffer significantly. Moreover, if we and Biogen fail to adequately protect certain intellectual property covering zorevunersen or related products in the Biogen Territory, the ability to commercialize zorevunersen and related products in the Biogen Territory could suffer and our business may be negatively impacted.

We do not have complete control over the maintenance, prosecution and litigation of certain out-licensed patents and patent applications. For example, we cannot be certain that activities such as the maintenance and prosecution by our existing or future licensees 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 licensees' infringement proceedings, defense

activities, or enforcement actions may be less vigorous than had we conducted them ourselves or may not be conducted in accordance with our best interests.

In addition, the resolution of any contract interpretation disagreement that may arise could broaden what we believe to be the scope of our existing or future licensees' rights to the relevant patents, know-how and proprietary technology, or decrease what we believe to be our licensees' financial or other obligations under the relevant agreement. Disputes that may arise between us and our existing or future licensees regarding intellectual property subject to a license agreement may negatively impact our business.

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 issued 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.

Additionally, reforms at government agencies of the United States and those of non-U.S. jurisdictions, including applicable non-U.S. patent authorities, could also 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 ability of the USPTO and other applicable patent authorities to properly administer their functions is highly dependent on the levels of funding available to the agency and their ability to retain key personnel and fill key leadership appointments, among various factors. Firing of employees or delays in filling or replacing key positions could significantly impact the ability of the USPTO and other applicable patent authorities to fulfill their functions and could greatly impact our ability to adequately prosecute or maintain our patent applications or those of any current or future licensors, or our ability to adequately maintain, enforce, or defend our issued patents or those of any current or future licensors.

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 as well as our senior scientists and other members of our 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.

Further, we are currently undergoing a leadership transition, which may be viewed negatively by employees, investors, and/or our strategic partners. Moreover, any attrition associated with this transition could significantly delay or prevent the achievement of product development and commercialization, along with other business objectives, and adversely impact our stock price. Until we

integrate new personnel, and unless they are able to success in their positions, we may be unable to successfully manage and grow our business.

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, inflation and actual and threatened tariffs, as well as the possibility of a recession or further economic downturn. Supply chain disruptions and delays as a result of any new tariff policies or trade restrictions could also negatively impact our cost of materials and production processes. For example, on February 1, 2025, the United States imposed a 25% tariff on imports from Canada and Mexico, which were subsequently suspended for a period of one month, and a 10% additional tariff on imports from China, and on April 2, 2025, the United States announced a baseline 10% tariff on all foreign goods, with goods imported from specified nations, including China and those in the European Union, taxed at higher rates. In addition, there are currently headlines and discussions concerning potential increased tariffs for pharmaceutical products, which may impact our supply chain and create uncertainty in the broader pharmaceutical industry.

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 and actual or threatened tariffs 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 our technology systems and data 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 a "smaller reporting company" and we cannot be certain if the reduced reporting requirements applicable to smaller reporting companies will make our common stock less attractive to investors.

We are 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 as of the last business day of our 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. In addition, 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 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 incur significant costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, we incur significant legal, accounting and other expenses. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the continued 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 need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations substantially contribute to our legal and financial compliance costs and 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 three months ended March 31, 2025, 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 a "smaller reporting 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 three months ended March 31, 2025, 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**	2025 Non-Employee Director Compensation Policy					X
10.2	License and Collaboration Agreement, dated as of February 14, 2025, by and between Biogen International GmbH and the Registrant					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: May 13, 2025

By: /s/ Ian F. Smith

Ian F. Smith
Interim Chief Executive Officer
(Principal Executive Officer)

Date: May 13, 2025

By: /s/ Thomas E. Leggett

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

Exhibit 10.1

STOKE THERAPEUTICS, INC. ("*Company*")
Non-Employee Director Compensation Policy
Approved as of September 9, 2024 (the "*Approval Date*")
Amended as of February 12, 2025

Each member of the Board of Directors (the "***Board***") of Stoke Therapeutics, Inc. (the "***Company***") who is a non-employee director of the Company (each such member, a "***Non-Employee Director***") will receive the compensation described in this Non-Employee Director Compensation Policy (the "***Policy***") for his or her Board service. The Board and its Compensation Committee shall adhere to the Policy in approving and awarding Non-Employee Director compensation. The Company's Compensation Consultant (defined below) shall be provided a copy of the Policy.

This Policy may be further amended or terminated at any time in the sole discretion of the Board, except for certain requirements that may not be amended or terminated before December 31, 2029 (the "***Restriction Period***"), as indicated herein.

A Non-Employee Director may decline all or any portion of his or her compensation by giving notice to the Company prior to the date cash is to be paid or equity awards are to be granted, as the case may be.

Role of Compensation Consultant

The Board or the Compensation Committee of the Board (the "***Compensation Committee***") shall retain an independent compensation consultant (the "***Compensation Consultant***") on an annual basis to conduct an analysis of Non-Employee Director compensation at peer companies and annually review the Company's peer group for purposes of assessing Non-Employee Director compensation (the "***Peer Group***"). The Compensation Consultant will make recommendations to the Board or Compensation Committee concerning adjustments to the Peer Group and the levels of compensation paid to the Company's Non-Employee Directors. The consultant shall assess the Peer Group annually and will select companies as peers which are: (a) operating in the same industry as the Company (by reference to GICS code or similar reasonable identities); (b) similar in size to the Company based on market capitalization, revenues, or employees, as determined based on the advice of the Compensation Consultant and recognizing that similarity in size and industry may include a range in order to accurately capture the market for directors; (c) have a market capitalization between .33 and 3 times the market capitalization of the Company as of the date of selection. If the Compensation Consultant recommends removal of a company from the Peer Group for any reason, including if a company falls outside of the required market capitalization range, that company shall be excluded from the Peer Group at such time the Peer Group is approved for that year. The requirements set forth in this "Role of Compensation Consultant" section shall apply through the Restriction Period.

Cash Compensation

For the calendar year 2025, cash compensation payable to each Non-Employee Director shall consist of the following annual fees, which shall be paid quarterly and shall be pro-rated for partial quarters served.

- General Board Service Fee: $40,000
- Non-Executive Chairman Fee: $35,000
- Committee Chair Service Fee (in addition to General Board Service Fee; in lieu of

Non- Chair Committee Member Service Fee set forth below):

- Audit Committee chair: $20,000
- Compensation Committee chair: $15,000
- Nominating and Governance Committee chair: $10,000

- Non-Chair Committee Member Service Fee (in addition to General Board Service Fee; not in addition to Committee Chair Service Fee):
  - Audit Committee member: $10,000
  - Compensation Committee member: $7,500
  - Nominating and Governance Committee member: $5,000

During the Restriction Period, the cash compensation paid to the Non-Employee Directors shall each not exceed the 65th percentile of Company's Peer Group for the year in which the compensation is approved and determined at the time the Board approves annual Non-Employee Director compensation.

Equity Compensation

Each Non-Employee Director will be eligible to receive either stock options to purchase shares of the Company's common stock ("***Options***") or restricted stock units to acquire shares of the Company's common stock ("***RSUs***" and together with Options, "***Awards***") under the Company's 2019 Equity Incentive Plan (the "***Equity Plan***") or any successor equity incentive plan as described below for service on the Board, with the amounts and terms of such equity compensation to be determined by the Board on an annual basis. During the Restriction Period, the Awards shall be calculated at the time of the grant and granted in terms of a designated Value (as defined below) and not a fixed number of shares.

Equity Compensation – Initial Award

Any Non-Employee Director of the Board being newly appointed to the Board will receive an Award to acquire a number of shares determined based on a Value (as defined below) designated by the Board (such award, the "***Initial Award***") under the Equity Plan (unless the Board affirmatively determines that any Non-Employee Directors shall not receive an Initial Award under this Policy).

As of the Approval Date, the Initial Award shall consist of an Option to acquire such number of shares of the Company's common stock calculated based on a Value equal to 2x the Value of the Annual Award (defined below), on the date the Initial Award is granted (the "***Initial Award Grant Date***").

"***Value***" (for the purposes of determining the number shares that will be subject to an Award under the Policy) means the grant date fair value of the Award determined in accordance with ASC 718.

As of the Approval Date, the Initial Award shall vest as to 1/12th of the total shares on each quarterly anniversary of the Initial Award Grant Date, such that the grant will become fully vested and exercisable on the three-year anniversary of the Initial Award Grant Date, so long as the Non-Employee Director continues to provide Service (as defined in the Equity Plan) to the Company through any given vesting date. If a Non-Employee Director's Service ends on the date of vesting, then the vesting shall be deemed to have occurred.

The Initial Award shall accelerate in full upon the consummation of a Corporate Transaction (as defined in the Equity Plan), subject to the applicable Non-Employee Director's continued Service to the Company as of immediately prior to such Corporate Transaction.

Equity Compensation – Annual Award

On the date of each annual meeting of the Company's stockholders (the "***Annual Meeting***"), each Non-Employee Director who is serving on the Board prior to, and will continue to serve on the Board following, the Annual Meeting will receive an Award to acquire a number of shares determined based on a Value (as defined below) designated by the Board (such award, the "***Annual Award***") under the Equity Plan. During the Restriction Period, the Annual Award shall not exceed the 65th percentile of the Company's Peer Group for the year in which the compensation is approved and determined at the time the Board approves annual Non-Employee Director compensation.

For the calendar year 2025, the Annual Award shall consist of an Option to acquire such number of shares of the Company's common stock calculated based on a Value of $224,000 on the Annual Award Grant Date (as defined below).

The Annual Award will automatically be granted on the date of the Annual Meeting of the Company's stockholders (the "***Annual Award Grant Date***").

As of the Approval Date, the Annual Award shall vest on the earlier of (i) the one-year anniversary of the Annual Award Grant Date and (ii) the date of the next Annual Meeting, in each case, so long as the Non-Employee Director continues to provide Service to the Company through such date. If a Non-Employee Director's Service ends on the date of vesting, then the vesting shall be deemed to have occurred.

The Annual Award shall accelerate in full upon the consummation of a Corporate Transaction (as defined in the Equity Plan), subject to the applicable Non-Employee Director's continued Service to the Company as-of immediately prior to such Corporate Transaction.

Compensation Limit

Notwithstanding any other provision of this Policy to the contrary, in no event will the total amount of compensation payable to any Non-Employee Director exceed the limits set forth in Section 13(a) of the Equity Plan.

Exhibit 10.2

CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [***], HAS BEEN OMITTED BECAUSE IT IS NOT MATERIAL AND WOULD LIKELY CAUSE COMPETITIVE HARM TO THE COMPANY IF PUBLICLY DISCLOSED

LICENSE AND COLLABORATION AGREEMENT

by and between

STOKE THERAPEUTICS, INC.

and

BIOGEN INTERNATIONAL GMBH

Dated as of February 14, 2025

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Schedule 2.2.2: Option Data Package Contents

LICENSE AND COLLABORATION AGREEMENT

This License and Collaboration Agreement (this "**Agreement**") is entered into as of February 14, 2025 (the "**Effective Date**") by and between Stoke Therapeutics, Inc., a Delaware corporation ("**Stoke**") and Biogen International GmbH, a Swiss limited liability company ("**BIG**"). Stoke and BIG are each hereafter referred to individually as a "**Party**" and together as the "**Parties**".

WHEREAS, Stoke is a clinical stage biopharmaceutical company that develops therapies based on ASOs (as defined below) for rare diseases;

WHEREAS, BIG is a commercial stage biopharmaceutical company with expertise in research, development, manufacturing and commercialization of pharmaceutical and biologics products; and

WHEREAS, BIG and Stoke desire to collaborate to develop and commercialize zorevunersen (STK-001), an ASO directed to SCN1A (as defined below), as well as potentially other products directed to SCN1A, in accordance with the terms and conditions of this Agreement.

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and for other good and valuable consideration, the receipt and adequacy of which are hereby acknowledged, the Parties hereby agree as follows:

ARTICLE 1DEFINITIONS

All references to particular Exhibits, Articles or Sections mean the Exhibits to, and Articles and Sections of, this Agreement, unless otherwise specified. For the purposes of this Agreement and the Exhibits and Appendices hereto, the following words and phrases have the following meanings:

Section 1.1"**Accounting Standards**" means, U.S. Generally Accepted Accounting Principles (GAAP) as generally and consistently applied through each Party's and its Affiliates' organization.

Section 1.2"**Additional Compound**" has the meaning set forth in Section 1.3.

Section 1.3"**Additional Licensed Product**" means any pharmaceutical product that (a) comprises or contains, as an active pharmaceutical ingredient, an ASO Directed To SCN1A Controlled by Stoke or its Affiliate as of the Effective Date or at any time during the Term other than the Initial Compound (such other compound, "**Additional Compound**"), [***], and (b) is designed to be delivered intrathecally.

Section 1.4"**Affiliate**" means, with respect to any Person, any other Person that controls, is controlled by or is under common control with such Person, for as long as such control exists. For purposes of this definition, "control" means the direct or indirect ownership of more than fifty percent (50%) of the voting or economic interest of a Person, or the power, whether pursuant to contract, ownership of securities or otherwise, to direct the management and policies of a Person. For clarity, once a Person ceases to be an Affiliate of a Party, then, without any further

action, such Person shall cease to have any rights, including license and sublicense rights, under this Agreement by reason of being an Affiliate of such Party.

Section 1.5"**Agreement**" has the meaning set forth in the Preamble.

Section 1.6"**Alliance Manager**" has the meaning set forth in Section 3.6.

Section 1.7"**Annual Net Sales**" means cumulative Net Sales over a single Calendar Year.

Section 1.8"**Anti-Corruption Laws**" means the U.S. Foreign Corrupt Practices Act of 1977, as amended, the U.K. Bribery Act 2010, any laws or regulations implementing the OECD Convention Against Bribery of Foreign Public Officials in International Business Transactions, any anti-bribery or anti-corruption laws and other similar anti-corruption laws, prohibiting bribery, kickbacks or other unlawful or improper means of obtaining business or commercial advantages, including applicable local and international anti-bribery and anti-corruption laws and regulations applicable to the Licensed Products or Option Products (after Option Exercise Effective Date) in either Party's territory.

Section 1.9"**Antitrust Clearance**" has the meaning set forth in Section 2.2.4(c).

Section 1.10"**Antitrust Consents**" has the meaning set forth in Section 2.2.4(c).

Section 1.11"**Antitrust Laws**" means the Sherman Antitrust Act of 1890, the Clayton Antitrust Act of 1914, the HSR Act and the Federal Trade Commission Act of 1914, each as amended, and all other applicable Law, whether federal, state, or foreign, that is designed or intended to prohibit, restrict or regulate actions having the purpose or effect of monopolization or restraint of trade, lessening of competition, or abuse of dominance through merger or acquisition or any structured collaboration.

Section 1.12"**Arising Know-How**" means any and all Know-How discovered, invented, developed, or otherwise made by or on behalf of a Party or its Affiliates or its or their Sublicensees, whether singly or jointly with another, in connection with the performance of such Party's activities or exercise of rights under this Agreement.

Section 1.13"**ASO**" means a single-stranded antisense oligonucleotide (including any analog, variant, mimic, or mimetic thereof), in unconjugated form, that modulates the expression of a gene target (a) directly via hybridization to an RNA of such gene target, thus affecting its transcription, splicing, stability or translation, or (b) indirectly via hybridization to an RNA that regulates such gene target.

Section 1.14"**BIG**" has the meaning set forth in the Preamble.

Section 1.15"**BIG Collaboration IP**" means BIG Collaboration Know-How, BIG Collaboration Patent Rights and BIG or its Affiliate's interest in any Joint Know-How and Joint Patent Rights.

Section 1.16"**BIG Collaboration Know-How**" means all Arising Know-How

discovered, invented, developed or otherwise made by or on behalf of BIG or its Affiliates or its or their Sublicensees in the performance of activities or exercise of rights under this Agreement, excluding any Stoke Platform Improvement Know-How and Joint Know-How.

Section 1.17"**BIG Collaboration Patent Rights**" means all Patent Rights Covering BIG Collaboration Know-How.

Section 1.18"**BIG Indemnified Parties**" has the meaning set forth in Section 11.1.

Section 1.19"**BIG Privacy and Security Obligations**" has the meaning set forth in Section 10.3.7.

Section 1.20"**BIG Territory**" means worldwide except for the Stoke Territory.

Section 1.21"**BIG Territory Development Plan**" has the meaning set forth in Section 4.2.

Section 1.22"**Bring-Down Date**" has the meaning set forth in Section 2.2.4(d).

Section 1.23"**Bring-Down Disclosure Schedule**" has the meaning set forth in Section 10.2.

Section 1.24"**Business Day**" means a day other than a Saturday or Sunday or a day on which banking institutions in the Commonwealth of Massachusetts or in Baar, Switzerland is permitted or required to be closed.

Section 1.25"**Calendar Quarter**" means each of the three (3) month periods ending March 31, June 30, September 30 and December 31; *provided*, *however*, that: (a) the first Calendar Quarter of the Term shall extend from the Effective Date to the end of the first complete Calendar Quarter thereafter; and (b) the last Calendar Quarter shall extend from the beginning of the Calendar Quarter in which this Agreement expires or terminates until the effective date of such expiration or termination.

Section 1.26"**Calendar Year**" means each of the twelve (12) month periods ending December 31; *provided*, *however*, that: (a) the first Calendar Year of the Term shall extend from the Effective Date to the end of the first complete Calendar Year thereafter; and (b) the last Calendar Year shall extend from the beginning of the Calendar Year in which this Agreement expires or terminates until the effective date of such expiration or termination.

Section 1.27"**Centralized Approval Procedure**" means the procedure through which a MAA filed with the EMA results in a single marketing authorization valid in all European Union member states, Iceland, Norway and Liechtenstein.

Section 1.28"**Clinical Supply Agreement**" has the meaning set forth in Section 6.2.

Section 1.29"**Clinical Trial Application**" or "**CTA**" means an application filed with a Regulatory Authority for authorization to commence clinical studies, including (a) an IND as defined in the FFDCA or any successor application or procedure filed with the FDA, EMA, or any

equivalent in other countries or regulatory jurisdictions, and (b) all supplements, amendments, variations, extensions and renewals thereof that may be filed with respect to the foregoing.

Section 1.30"**CMC**" means chemistry, manufacturing and controls activities in connection with a pharmaceutical product.

Section 1.31"CMO" means contract manufacturing organization.

Section 1.32"**Combination Product**" means a Licensed Product that [***].

Section 1.33"**Commercialization**" means any and all processes and activities conducted to establish and maintain sales for a Licensed Product or Option Product (after Option Exercise Effective Date), including processes and activities to market, advertise, promote, store, transport, distribute, import, export, offer to sell (including pricing and reimbursement and value and access activities as well as observational research and evidence generation including economic value), detail, sell a pharmaceutical product, conduct other commercialization activities, including activities conducted in connection with commercial launch, such as branding (including activities to obtain international nonproprietary names and other nonproprietary names for a Licensed Product or Option Product (after Option Exercise Effective Date), and research relating to product naming), establishing a sales force, forecasting mechanisms, drug pricing analyses and launch strategies, including in support of any of the foregoing (including training, materials, public relations and market research), and "**Commercialize**" shall have the correlative meaning with respect to such activities; *provided,* that Commercialization shall exclude Development and Manufacturing.

Section 1.34"**Commercially Reasonable Efforts**" means, with respect to the efforts and resources to be expended by a Party ("**Obligated Party**") with respect to any objective, activity or decision to be undertaken with respect to the Development, Manufacture or Commercialization of a Licensed Product or Option Product (after Option Exercise Effective Date), the efforts and resources to accomplish such objective, activity or decision that would be comparable with the efforts and resources that such Party would normally use to accomplish a similar objective, activity or decision with respect to its other pharmaceutical compounds or products that are at a similar stage of development or product life, are in a similar therapeutic and disease area and are of similar market potential taking into account all relevant legal, medical, scientific, technical and commercial and other factors including: [***]. Without limiting the foregoing, Commercially Reasonable Efforts requires that a Party at a minimum: [***]. In addition, with regard to BIG's obligations relating to the Development, Manufacture and Commercialization of Licensed Product(s) or Option Product(s) (after Option Exercise Effective Date) hereunder, it is anticipated that the level of effort may change over time, including to reflect changes in the status of the product in particular countries or markets. For the avoidance of doubt, where a Party has an obligation to use Commercially Reasonable Efforts, the efforts of such Party and its Affiliates and its and their subcontractors and (sub)licensees/Sublicensees shall be considered in determining whether such Party has satisfied such obligation.

Section 1.35"**Competing Product**" means any pharmaceutical product that a Party or its Affiliates is prohibited from Exploiting pursuant to Section 2.7.

Section 1.36"**Competitive Infringement**" has the meaning set forth in Section 9.3.2.

Section 1.37"**Confidential Disclosure Agreement**" means the Mutual Confidential Disclosure Agreement entered into by and between Stoke and Biogen MA Inc., a BIG Affiliate, dated March 26, 2024.

Section 1.38"**Confidential Information**" has the meaning set forth in Section 13.1.1.

Section 1.39"**Control**" or "**Controlled**" means, with respect to any Know-How, Patent Right, Regulatory Filings, or other Intellectual Property Rights, the possession (whether by ownership or license, other than by a license or sublicense granted pursuant to this Agreement) by a Party or its Affiliate of the ability to grant to the other Party a license or access as provided herein to such Know-How, Patent Right, Regulatory Filing or other Intellectual Property Rights, without violating the terms of any agreement or other arrangement of such Party or its Affiliate with any Third Party, in existence as of the time such Party or its Affiliates would first be required hereunder to grant the other Party such license or access, *provided* that a Party or its Affiliate will not be deemed to Control any Know-How, Patent Right, Regulatory Filings, or other Intellectual Property Rights under an agreement with a Third Party entered into by such Party or its Affiliate after the Effective Date unless the Parties agree to an allocation of payments thereunder in accordance with Section 8.5. Notwithstanding the foregoing, in the event of a Sale Transaction of a Party, such Party shall not be deemed to Control: (a) any Know-How, Patent Right, Regulatory Filings, or other Intellectual Property Rights owned or licensed by the acquirer (or any of its Affiliates) of such Party immediately prior to the closing of such a Sale Transaction; or (b) any Know-How, Patent Right, Regulatory Filings, or other Intellectual Property Rights that the acquirer (or any of its Affiliates) of such Party subsequently develops without accessing or practicing any Licensed IP, Option IP, BIG Collaboration IP or any Confidential Information of the other Party (or any Confidential Information of both Parties), in each case ((a) and (b)), except to the extent that such Know-How, Patent Right, Regulatory Filings, or other Intellectual Property Rights (i) is actually used by such Party or its Affiliates, or the acquirer of such Party, to Exploit a Licensed Product or Option Product following the closing of such Sale Transaction or (ii) was Controlled by such Party or its Affiliates prior to the closing of such Sale Transaction.

Section 1.40"**Convicted Individual**" or "**Convicted Entity**" is an individual or entity, as applicable, who has been convicted of a criminal offense that falls within the ambit of 21 U.S.C. §335a (a) or 42 U.S.C. §1320a – 7(a), but has not yet been excluded, debarred, suspended, or otherwise declared ineligible.

Section 1.41"**Costs**" means both internal expenses and actual external costs and expenses incurred.

Section 1.42"**Cover**" means (a) with respect to Know-How, that the Exploitation of a given molecule, pharmaceutical product, or item uses or would require the use such Know-How and (b) with respect to a Patent Right, that the Exploitation of a given molecule, pharmaceutical product, or item would infringe a claim of such Patent Right, or, as to a claim that is included in a pending Patent Right, would infringe such pending claim if such pending claim were to issue without modification (in each case in the absence of ownership of, or a license under, such Patent Right). Cognates of the word "**Cover**" have correlative meanings.

Section 1.43"**Covered Results**" has the meaning set forth in Section 13.3.

Section 1.44"**Data**" means any and all data, including preclinical data, safety and efficacy data, pharmacology data, chemistry data (including analytical, product characterization, Manufacturing, and stability data), toxicology data, data arising from any clinical trial (including investigator reports (both preliminary and final), statistical analyses, expert opinions and reports, safety and other electronic databases), together with supporting data, medical affairs data and data generated from early patient access programs or compassionate access programs.

Section 1.45"**Data Security and Privacy Laws**" mean all applicable Laws relating to the privacy, Processing and security of Personal Data.

Section 1.46"**Debarred Entity**" is a corporation, partnership, or association that has been debarred by the FDA pursuant to 21 U.S.C. §335a (a) or (b) from submitting or assisting in the submission of any abbreviated drug application, or a subsidiary or affiliate of such a corporation, partnership, or association.

Section 1.47"**Debarred Individual**" is an individual who has been debarred by the FDA pursuant to 21 U.S.C. §335a (a) or (b) from providing services in any capacity to a Person that has an approved or pending drug or biological product application.

Section 1.48"**Defense Proceeding**" has the meaning set forth in Section 9.2.1.

Section 1.49"**Development**" means those activities required or useful to obtain and maintain Regulatory Approval(s) of a Licensed Product or Option Product (after Option Exercise Effective Date), including test and diagnostic method development and stability testing, assay development and audit development, pre-clinical/non-clinical trials (including toxicology, pharmacokinetic, metabolism and excretion studies), formulation development, CMC development, manufacturing process development including commercial scale-up development, pharmacodynamics, quality assurance/quality control development, statistical analysis, clinical trials, medical affairs, packaging development, regulatory affairs, biomarker strategy and development, report writing and statistical analysis, the preparation, filing and prosecution of Regulatory Approval applications, and "**Develop**" shall have the correlative meaning with respect to such activities; *provided,* that Development shall exclude Commercialization. For clarity, the term Development shall include the conduct of a clinical study after receipt of a Regulatory Approval.

Section 1.50"**Directed To**" means, with respect to an ASO and a target, that such ASO is designed or known to bind to or otherwise modulate such target for therapeutic benefit.

Section 1.51"**Disclosing Party**" has the meaning set forth in Section 13.1.1.

Section 1.52"**Dispute**" has the meaning set forth in Section 15.5.1.

Section 1.53"**Divestiture**" means, with respect to a Competing Product, (a) an outright sale or assignment of all material rights in such Competing Product to a Third Party, or (b) an exclusive out-license of all Development and Commercialization rights with respect to such Competing Product with no further material role, influence or authority of such Party or any of its

Affiliates with respect to such Competing Product (for clarity, the right of such divesting Party or its Affiliates to receive reports, royalties, milestones or other payments in connection with an exclusive licensee's Development or Commercialization of such Competing Product shall not be deemed a material role, influence or authority for purposes of this definition). When used as a verb, "**Divest**" and "**Divested**" means to cause a Divestiture.

Section 1.54"**Dollars**" or "**$**" means United States Dollars.

Section 1.55"**Effective Date**" has the meaning set forth in the preamble.

Section 1.56"**EMA**" means the European Medicines Agency or any successor entity thereto.

Section 1.57"**Emperor Study**" means the phase 3 clinical trial having the protocol entitled "EMPEROR: A Multicenter, Randomized, Double-blind, Sham-controlled, Parallel Group, Phase 3 study Evaluating the Efficacy, Safety, and Tolerability of Zorevunersen (STK-001) in Patients With Dravet Syndrome".

Section 1.58"**European Union**" or "**EU**" means the European Union, as its membership may be altered from time to time, and any successor thereto.

Section 1.59"**EU5 Countries**" means France, Germany, Italy, Spain, and the United Kingdom.

Section 1.60"**Excluded Individual**" or "**Excluded Entity**" is (i) an individual or entity, as applicable, who has been excluded, debarred, suspended, or is otherwise ineligible to participate in federal health care programs such as Medicare or Medicaid by the Office of the Inspector General (OIG/HHS) of the U.S. Department of Health and Human Services; or (ii) is an individual or entity, as applicable, who has been excluded, debarred, suspended, or is otherwise ineligible to participate in federal procurement and non-procurement programs, including those produced by the U.S. General Services Administration (GSA).

Section 1.61"**Executive Officers**" means (a) with respect to Stoke, [***], or any other person that such officer designates from time to time, and (b) with respect to BIG, [***], or any other person that such officer designates from time to time.

Section 1.62"**Existing Agreement**" has the meaning set forth in Section 10.2.7.

Section 1.63"**Existing Patent Rights**" has the meaning set forth in Section 10.2.1.

Section 1.64"**Exploit**" means to make, have made, use, distribute, sell, offer for sale, sell and import, including to research, develop, modify, enhance, improve, register, distribute, commercialize, export or otherwise dispose of, and, with respect to any Licensed Product or Option Product (after Option Exercise Effective Date), to Develop, Manufacture or Commercialize. Cognates of the word "**Exploit**" shall have correlative meanings.

Section 1.65"**FDA**" means the United States Food and Drug Administration or any successor entity thereto.

Section 1.66"**FDA's Disqualified/Restricted List**" is the list of clinical investigators restricted from receiving investigational drugs, biologics, or devices if the FDA has determined that the investigators have repeatedly or deliberately failed to comply with regulatory requirements for studies or have submitted false information to the study sponsor or the FDA.

Section 1.67"**Field**" means all human and non-human diagnostic, prophylactic and therapeutic uses.

Section 1.68"**First Commercial Sale**" means, with respect to a Licensed Product or Option Product (after Option Exercise Effective Date) and a country or other jurisdiction in the BIG Territory, the first sale for monetary value for use or consumption by the end user of such Licensed Product or Option Product in such country or other jurisdiction after Regulatory Approval has been obtained in such country or other jurisdiction. Sales prior to receipt of Regulatory Approval for such Licensed Product or Option Product in such country or other jurisdiction, such as so-called "treatment IND sales", "expanded early access programs", "named patient programs" or "named patient sales", and "compassionate use programs" or "compassionate use sales", shall not be construed as a First Commercial Sale.

Section 1.69"Force Majeure Event" has the meaning set forth in Section 15.13.

Section 1.70"**FTE**" means the equivalent of the work of one (1) employee full time for one (1) Calendar Year consisting of a total of [***] hours per Calendar Year of work.

Section 1.71"**FTE Costs**" means, with respect to a Party for any period, the applicable FTE Rate multiplied by the applicable number of FTE hours of such Party performing the applicable activity described hereunder during such period.

Section 1.72"**FTE Rate**" means an initial rate of [***] per FTE, which shall apply through December 31, 2025. Thereafter, the FTE Rate shall be changed annually by the JSC on a Calendar Year basis to reflect any year-to-year percentage increase or decrease (as the case may be), consistent with the Consumer Price Index for All Urban Consumers (CPI U) for the U.S. City Average, calculated by the Bureau of Labor Statistics during the immediately preceding Calendar Year.

Section 1.73"**GCP**" means good clinical practices as such standard is defined in accordance with the applicable guidance and regulations including the International Conference of Harmonization (ICH).

Section 1.74"**Generic Product**" means, with respect to a Licensed Product or Option Product (after Option Exercise Effective Date), any product that is approved in reliance, in whole or in part, on the prior Regulatory Approval (or on safety or efficacy data submitted in support of the prior Regulatory Approval) of such Licensed Product or Option Product as determined by the applicable Regulatory Authority, including any product authorized for sale (i) in the U.S. pursuant to Section 505(b)(2) or Section 505(j) of the FFDCA (21 U.S.C. §355(b)(2) and 21 U.S.C. §355(j), respectively), (ii) in the EU pursuant to a provision of Articles 10, 10a or 10b of Parliament and Council Directive 2001/83/EC as amended (including an application under Article 6.1 of Parliament and Council Regulation (EC) No 726/2004 that relies for its content on any such provision), or (iii) in any other country or jurisdiction pursuant to all equivalents of such

provisions, including any amendments and successor statutes with respect to any of the foregoing. For clarity, a product licensed, marketed, sold, manufactured, or produced by BIG, its Affiliates, or Sublicensees under the same NDA as a Licensed Product or Option Product will not constitute a Generic Product.

Section 1.75"**Global Development Activities**" has the meaning set forth in Section 4.1.

Section 1.76"**Global Development Budget**" has the meaning set forth in Section 4.1.

Section 1.77"**Global Development Plan**" has the meaning set forth in Section 4.1.

Section 1.78"**Global Study Opt-In Notice**" has the meaning set forth in Section 4.6.2.

Section 1.79"**Governmental Authority**" means any court, agency, department, bureaus, commissions, councils, authority or other instrumentality of any supra-national, federal, national, state, county, regional, provincial, local city or other political subdivision.

Section 1.80"**ICC**" has the meaning set forth in Section 15.5.2.

Section 1.81"**Immaterial Changes**" means a change or series of related changes to a Global Development Plan that [***].

Section 1.82"**IND**" means (a) an investigational new drug application, clinical trial application or similar application for authorization to commence clinical studies filed with a Regulatory Authority, and (b) all supplements and amendments that may be filed with respect to the foregoing.

Section 1.83"**Indemnitee**" has the meaning set forth in Section 11.3.

Section 1.84"**Indemnitor**" has the meaning set forth in Section 11.3.

Section 1.85"**Infringement**" has the meaning set forth in Section 9.3.1.

Section 1.86"**Initial Compound**" means zorevunersen (STK-001).

Section 1.87"**Initial Disclosure Schedule**" has the meaning set forth in Section 10.2.

Section 1.88"**Initial Licensed Product**" means a pharmaceutical product that (a) comprises or contains, as an active pharmaceutical ingredient, the Initial Compound and (b) is designed to be delivered intrathecally.

Section 1.89"**Intellectual Property Rights**" means all Know-How, trade secrets, copyrights, Patent Rights, trademarks, service marks, goodwill, moral rights, and any and all other intellectual property or proprietary rights (including applications relating thereto), whether or not now known or hereafter recognized in any jurisdiction.

Section 1.90"**Intrathecal SCN1A Product**" means any pharmaceutical product that (a) comprises or contains, as an active pharmaceutical ingredient, an ASO Directed To SCN1A [***], and (b) is designed to be delivered intrathecally.

Section 1.91"**Issuing Party**" has the meaning set forth in Section 13.2.2.

Section 1.92"**Joint Commercialization Committee**" or "**JCC**" has the meaning set forth in Section 3.2.1.

Section 1.93"**Joint Development Committee**" or "**JDC**" has the meaning set forth in Section 3.2.1.

Section 1.94"**Joint IP**" means the Joint Know-How and Joint Patent Rights.

Section 1.95"**Joint Know-How**" means all Arising Know-How discovered, invented, developed or otherwise made jointly by or on behalf of (a) BIG or its Affiliates or its or their Sublicensees and (b) Stoke or its Affiliates or its or their Sublicensees, in each case in the performance of activities or exercise of rights under this Agreement, excluding any Stoke Platform Improvement Know-How.

Section 1.96"**Joint Patent Rights**" means any Patent Rights that claim Joint Know-How.

Section 1.97"**Joint Steering Committee**" or "**JSC**" has the meaning set forth in Section 3.1.1.

Section 1.98"**Know-How**" means proprietary techniques, technology, trade secrets, inventions (whether patentable or not), methods, know-how, Data and results (including pharmacological, toxicological and clinical data and results), and analytical and quality control data and results, regulatory documents, and other information regarding Materials.

Section 1.99"**Labeling**" means any and all (a) labels and other written, printed or graphic matter, including artwork, upon a product or any container utilized with a product; (b) product packaging; or (c) the product package inserts.

Section 1.100"**Law**" means, individually and collectively, any and all laws, ordinances, rules, orders, directives, administrative circulars and regulations of any kind whatsoever of any Governmental Authority within the applicable jurisdiction.

Section 1.101"**Lead Development Party**" has the meaning set forth in Section 4.1(a).

Section 1.102"**Licensed IP**" means (a) Licensed Patent Rights, (b) Licensed Know-How, and (c) Stoke and its Affiliate's interest in any Joint Know-How and Joint Patent Rights.

Section 1.103"**Licensed Know-How**" means all Know-How Controlled by Stoke or its Affiliates as of the Effective Date or during the Term (including Stoke Platform Improvement Know-How) that is necessary or reasonably useful for (a) Exploitation of a Licensed Product in the Field in the BIG Territory or (b) Development or Manufacture of a Licensed Product in the Field in the Stoke Territory for Exploitation of a Licensed Product in the Field in the BIG Territory.

Section 1.104"**Licensed Patent Rights**" means all Patent Rights Controlled by Stoke or its Affiliates as of the Effective Date or during the Term that Cover the Licensed Know-How, or

are otherwise necessary or reasonably useful for (a) Exploitation of a Licensed Product in the Field in the BIG Territory, or (b) Development or Manufacture of a Licensed Product in the Field in the Stoke Territory for Exploitation of a Licensed Product in the Field in the BIG Territory. Licensed Patent Rights existing as of the Effective Date are listed in Exhibit 1.104.

Section 1.105"**Licensed Product**" means, as applicable, (a) an Initial Licensed Product or (b) an Additional Licensed Product.

Section 1.106"**Licensed Product-Specific Know-How**" means any Licensed Know-How that Covers the composition of matter, formulation, or method of use or making, of a Licensed Product and is not Stoke Platform Know-How.

Section 1.107"**Licensed Product-Specific Patent Rights**" means any Licensed Patent Rights that (a) Cover the composition of matter, formulation, or method of use or making a Licensed Product, Initial Compound, or Additional Compound, and (b) are not Stoke Platform Patent Rights. All Licensed Product-Specific Patent Rights existing as of the Effective Date are set forth in Exhibit 1.104(b).

Section 1.108"**Losses**" has the meaning set forth in Section 11.1.

Section 1.109"**Manufacture**" or "**Manufacturing**" means, with respect to a Licensed Product or Option Product (after Option Exercise Effective Date), any and all processes and activities directed to producing, manufacturing, processing, sourcing of materials, filling, finishing, packaging, Labeling, inspecting, quality controls testing, quality assurance and release, receiving, holding, shipping or storage of a Licensed Product or Option Product (after Option Exercise Effective Date) or any raw materials or packaging materials with respect thereto, or any intermediate of any of the foregoing, including process and cost optimization, process qualification and validation, commercial manufacture, stability and release testing, and quality control.

Section 1.110"**Manufacturing Process**" has the meaning set forth in Section 6.1.

Section 1.111"**Marketing Authorization Application**" or "**MAA**" means a new drug application or product license application or its equivalent filed with and accepted by the FDA or EMA after completion of human clinical trials to obtain marketing approval for a pharmaceutical product, or any comparable application filed with and accepted by the regulatory authorities of a country or region.

Section 1.112"**Materials**" means any proprietary biological, chemical, or other physical substance, including cells, cell lines, reagents, plasmids, proteins, assays, compositions of matter and compounds, and excluding off-the-shelf or commercially available materials.

Section 1.113"**MHLW**" means the Ministry of Health, Labour and Welfare of Japan, working together with the Pharmaceuticals and Medical Devices Agency, and any successor agency or administration thereto.

Section 1.114"**Net Sales**" means, with respect to a Licensed Product or Option Product (after Option Exercise Effective Date) during the Royalty Term, the gross amount invoiced by or on behalf of BIG or its Affiliates or its or their Sublicensees (excluding distributors) (each of the

foregoing Persons, a "**Selling Party**") for the sale or other disposition of such Licensed Product or Option Product to a Third Party (including Third Party distributors), in bona fide arms' length transactions in the BIG Territory, less the following deductions (from the gross amount invoiced), in each case, with respect to such Licensed Product or Option Product: [***].

Such amounts will be determined consistent with a Selling Party's customary practices and in accordance with Accounting Standards. It is understood that any accruals for individual items reflected in Net Sales are periodically (at least quarterly) trued up and adjusted by each Selling Party consistent with its customary practices and in accordance with Accounting Standards.

Notwithstanding any provision to the contrary set forth in this Agreement, Net Sales will not be imputed to transfers of such Licensed Product or Option Product to Third Parties as bona fide samples, as donations, for clinical trial purposes, any early access program or expanded access program, any compassionate sales or use program (including named patient program or single patient program) or any indigent program (*provided* that in each case sales made pursuant to any such program after Regulatory Approval and during the Royalty Term shall be included in Net Sales) or for similar bona fide business purposes in accordance with applicable Law.

Sale or transfer of such Licensed Products or Option Products between any of the Selling Parties will not result in any Net Sales unless a Selling Party is an end user, with such other Net Sales to be based only on any subsequent sales or dispositions to a non-Selling Party. To the extent that any Selling Party receives consideration other than or in addition to cash upon the sale or disposition of such Licensed Product or Option Product to a non-Selling Party in a given country, Net Sales will be calculated based on [***].

For purposes of calculating Net Sales, all Net Sales shall be converted into Dollars in accordance with Section 8.7.

In the case of any Combination Product sold in a given country and reporting period, Net Sales for the purpose of determining royalties and Sales Milestones of the Combination Product in such country will be calculated by [***].

If, on a country-by-country basis in a particular reporting period, such Licensed Product or Option Product that contains the same active pharmaceutical ingredient as included in such Combination Product as its sole active pharmaceutical ingredient(s) is sold separately in the same indication in a country, but the product that contains the Other Components in the Combination Product as its sole active pharmaceutical ingredient(s) is not sold separately in the same indication in such country, then Net Sales for the purpose of determining royalties and Sales Milestones of the Combination Product for such country will be calculated by [***].

If, on a country-by-country basis in a particular reporting period, such Licensed Product or Option Product that contains the same active pharmaceutical ingredient as included in such Combination Product as its sole active pharmaceutical ingredient(s) is not sold separately in the same indication in such country, but the product that contains the Other Components in the Combination Product as its sole active pharmaceutical ingredient(s) is sold separately in the same indication in such country, then Net Sales for the purpose of determining royalties and Sales Milestones of the Combination Product for such country will be calculated by [***].

If neither such Licensed Product or Option Product that contains the same active pharmaceutical ingredient as included in such Combination Product as its sole active pharmaceutical ingredient(s) nor the product that contains the Other Components in the Combination Product as its sole active pharmaceutical ingredient(s) are sold separately in the same indication in a given country during a particular reporting period, then Net Sales for such Combination Product in such country will be determined in good faith by the Parties based on the relative fair market values of such Licensed Product or Option Product and such product for such indication in such country.

Section 1.115"**NMPA**" means the National Medical Products Administration of the PRC and any successor agency or administration thereto.

Section 1.116"**Non-Intrathecal SCN1A Product**" means any pharmaceutical product that (a) comprises or contains, as an active pharmaceutical ingredient, an ASO Directed To SCN1A [***] and (b) is not designed to be delivered intrathecally.

Section 1.117"**Non-Proposing Party**" has the meaning set forth in Section 4.6.1.

Section 1.118"**Non-Publishing Party**" has the meaning set forth in Section 13.3.1.

Section 1.119"**Obligated Party**" has the meaning set forth in Section 1.34.

Section 1.120"**Option**" has the meaning set forth in Section 2.2.1.

Section 1.121"**Option Compound**" has the meaning set forth in Section 1.130.

Section 1.122"**Option Data Package**" has the meaning set forth in Section 2.2.2.

Section 1.123"**Option Exercise Effective Date**" has the meaning set forth in Section 2.2.3.

Section 1.124"**Option Exercise Fee**" has the meaning set forth in Section 8.2.

Section 1.125"**Option Exercise Notice**" has the meaning set forth in Section 2.2.3.

Section 1.126"**Option IP**" means (a) Option Patent Rights, (b) Option Know-How and (c) and Stoke and its Affiliate's interest in any Joint Know-How and Joint Patent Rights.

Section 1.127"**Option Know-How**" means, with respect to each Option Product, all Know-How Controlled by Stoke or any of its Affiliates as of the Effective Date or at any time during the Term (including Stoke Platform Improvement Know-How), that is necessary or reasonably useful for (i) Exploitation of the applicable Option Product in the Field in the BIG Territory and (ii) Development or Manufacture of the applicable Option Product in the Field in the Stoke Territory for Exploitation of such Option Product in the Field in the BIG Territory.

Section 1.128"**Option Patent Rights**" means, with respect to each Option Product, all Patent Rights Controlled by Stoke or any of its Affiliates as of the Effective Date or at any time during the Term that Cover the Option Know-How, or are otherwise necessary or reasonably useful for (a) Exploitation of the applicable Option Product in the Field in the BIG Territory, or (b)

Development or Manufacture of the applicable Option Product in the Field in the Stoke Territory for Exploitation of such Option Product in the Field in the BIG Territory.

Section 1.129"**Option Period**" has the meaning set forth in Section 2.2.3.

Section 1.130"**Option Product**" means (a) any pharmaceutical product that (i) comprises or contains, as an active pharmaceutical ingredient, an ASO Directed To SCN1A Controlled by Stoke or its Affiliate as of the Effective Date or at any time during the Term (such compound, "**Option Compound**"), [***], and (ii) is not designed to be delivered intrathecally, and (b) any improvements, modifications or derivatives of the foregoing product in clause (a) made by or on behalf of either Party after the Option Exercise Effective Date to the extent comprising or containing, as an active pharmaceutical ingredient, an ASO Directed To SCN1A and not designed to be delivered intrathecally. For clarity, an Option Product includes any pharmaceutical product comprising or containing the Initial Compound or an Additional Licensed Compound and not designed to be delivered intrathecally.

Section 1.131"**Option Product License**" has the meaning set forth in Section 2.2.4.

Section 1.132"**Option Product-Specific Know-How**" means any Option Know-How that Covers the composition of matter, formulation, or method of use or making, of an Option Product and is not Stoke Platform Know-How.

Section 1.133"**Option Product-Specific Patent Rights**" means any Option Patent Rights that Cover the composition of matter, formulation, or method of use or making an Option Product and are not Stoke Platform Patent Rights.

Section 1.134"**Option Product Third Party Agreement**" has the meaning set forth in Section 2.2.2.

Section 1.135"**Other Components**" has the meaning set forth in Section 1.32.

Section 1.136"**Out-of-Pocket Costs**" mean costs and expenses paid to Third Parties (or payable to Third Parties and accrued in accordance with Accounting Standards) by a Party or its Affiliates and incurred in the performance of the relevant activity under this Agreement, including the cost of materials and services (including Third Party consultants) and any taxes and duties thereon. Out-of-Pocket Costs exclude (a) capital expenditures and financing costs, (b) internal costs of a Party or its Affiliates, including FTE Costs and financial and legal costs, and (c) any costs or expenses of a Party or its Affiliates to the extent caused by such Party or its Affiliate's breach of this Agreement.

Section 1.137"**Owed Party**" has the meaning set forth in Section 8.6.

Section 1.138"**Owing Party**" has the meaning set forth in Section 8.6.

Section 1.139"**Party**" and "**Parties**" has the meaning set forth in the Preamble.

Section 1.140"**Patent Rights**" means (a) all national, regional and international patents and patent applications in any country or jurisdiction worldwide, including provisional patent

applications; (b) all patent applications filed either from such patents, patent applications or provisional applications or from an application claiming priority from either of these, including divisionals, continuations, continuations-in-part, provisionals, converted provisionals and continued prosecution applications; (c) any and all patents that have issued or in the future issue from the foregoing patent applications ((a) and (b)), including utility models, petty patents, design patents and certificates of invention; (d) any and all extensions or restorations by existing or future extension or restoration mechanisms, including revalidations, reissues, re-examinations and extensions (including any supplementary protection certificates and the like) of the foregoing patents or patent applications ((a), (b) and (c)); and (e) any similar rights, including so-called pipeline protection or any importation, revalidation, confirmation or introduction patent or registration patent or patent of additions to any of such foregoing patent applications and patents.

Section 1.141"**Patent Term Extension**" has the meaning set forth in Section 9.2.4.

Section 1.142"**Payee**" has the meaning set forth in Section 8.8.2.

Section 1.143"**Payment**" has the meaning set forth in Section 8.8.2.

Section 1.144"**Payor**" has the meaning set forth in Section 8.8.2.

Section 1.145"**Person**" means any corporation, limited or general partnership, limited liability company, joint venture, trust, unincorporated association, governmental body, authority, bureau or agency, any other entity or body, or an individual.

Section 1.146"**Personal Data**" means (a) all information identifying, or in combination with other information, identifiable to an individual, including pseudonymized (key-coded) clinical data containing such information, and (b) any other information that is governed, regulated, or protected by one or more Data Security and Privacy Laws.

Section 1.147"**Phase 3 Clinical Trial**" means a human clinical trial of a pharmaceutical product (whether a standalone trial or a stage of a "Phase 2/3" clinical trial described in the protocol as the "Phase 3 portion"): (a) (1) with a defined dose or a set of defined doses of such product designed to establish statistically significant efficacy and the safety of such product for the purpose of enabling the preparation and submission of an MAA to the competent Regulatory Authorities in a country or jurisdiction, or (2) where the results of such clinical trial are intended (if successful) to be used to establish both safety and efficacy of such product in patients which are the subject of such trial and serve as the basis for initial or supplemental Regulatory Approval of such product, and (b) that satisfies the requirements of 21 CFR § 312.21(c) or its non-U.S. equivalents.

Section 1.148"**PRC**" means the People's Republic of China, excluding, for purposes of this Agreement only, Taiwan and the Special Administrative Regions of Hong Kong and Macao.

Section 1.149"**Pre-Commercial Milestone Event**" means achievement of [***].

Section 1.150"**Pricing Authority**" means any Governmental Authority with the authority to control, approve, recommend or otherwise determine pricing and of pharmaceutical products, including those with authority to enter into risk sharing schemes and/or to impose retroactive price reductions, discounts, or rebates.

Section 1.151"**Pricing and Reimbursement Approval**" means with respect to a Licensed Product or Option Product (after Option Exercise Effective Date), as applicable, either: (a) the approval, agreement, determination or decision establishing prices that can be charged for such product in national-level regulatory jurisdictions where the applicable Governmental Authorities (including Pricing Authorities) approve or determine the price of pharmaceutical products, or (b) the approval, agreement, determination or decision establishing the prices at which such product will be reimbursed at the national-level in regulatory jurisdictions where the applicable Governmental Authority (including Pricing Authority) approves, determines or recommends the reimbursement of pharmaceutical products.

Section 1.152"**Processing**" (or its conjugates) means any operation or set of operations that is performed upon Personal Data, whether or not by automatic means, such as collection, recording, organization, storage, adaptation or alternation, retrieval, consultation, use, disclosure by transmission, dissemination, or otherwise making available, alignment or combination, blocking, erasure, or destruction.

Section 1.153"**Proposed Global Study**" has the meaning set forth in Section 4.6.1.

Section 1.154"**Proposing Party**" has the meaning set forth in Section 4.6.1.

Section 1.155"**Publication**" has the meaning set forth in Section 13.3.

Section 1.156"**Publication Strategy**" has the meaning set forth in Section 3.2.2(g).

Section 1.157"**Publishing Party**" has the meaning set forth in Section 13.3.

Section 1.158"**Receiving Party**" has the meaning set forth in Section 13.1.1.

Section 1.159"**Regulatory Approval**" means, with respect to a country or other jurisdiction, any and all approvals, licenses, registrations or authorizations of any Regulatory Authority (including such approvals, licenses, registrations or authorizations obtained through the Centralized Approval Procedure for the European Union), necessary to manufacture, use, storage, import, commercially distribute, sell or market a pharmaceutical product in such country, including, where applicable, (a) Pricing and Reimbursement Approval in such country or jurisdiction if such Pricing and Reimbursement Approval is required to market in such country or jurisdiction, and (b) pre-approval marketing authorizations (including any prerequisite Manufacturing approval or authorization related thereto).

Section 1.160"**Regulatory Authority**" means any Governmental Authority or other authority responsible for granting Regulatory Approvals for a pharmaceutical product, including the FDA, EMA, NMPA, MHLW and any corresponding national or regional regulatory authorities.

Section 1.161"**Regulatory Exclusivity**" means, with respect to a pharmaceutical product in any country or other jurisdiction in the BIG Territory, a market protection (including any such rights that would satisfy the requirements of Article 10 of Directive 2001/83/EC, Article 14(11) of Parliament and Council Regulation (EC) No. 726/2004, Parliament and Council Regulation (EC) No. 141/2000 on orphan medicines, Parliament and Council Regulation (EC) No. 1901/2006 on

medicinal products for pediatric use and all international equivalents of any of the foregoing), other than Patent-related protection, granted by a Regulatory Authority in such country or other jurisdiction that (a) confers an exclusive Commercialization period during which BIG or its Affiliates or Sublicensees have the exclusive right to market and sell such pharmaceutical product in such country or other jurisdiction, or (b) prohibits a Person from (i) relying on safety or efficacy data generated by or on behalf of a Party with respect to such pharmaceutical product in an application for Regulatory Approval of a Generic Product or (ii) otherwise submitting such an application or obtaining such Regulatory Approval.

Section 1.162"**Regulatory Filing**" means any documentation comprising or relating to or supporting any filing or application with any Regulatory Authority with respect to a pharmaceutical product, including any documents submitted to any Regulatory Authority, including CTAs, MAAs, Labeling changes, any submission to a regulatory advisory board, and any supplement or amendment thereto, and all correspondence with any Regulatory Authority with respect to any pharmaceutical product (including minutes of any meetings, telephone conferences or discussions with any Regulatory Authority).

Section 1.163"**Release**" has the meaning set forth in Section 13.2.2.

Section 1.164"**Reversion Product**" has the meaning set forth in Section 14.7.7.

Section 1.165"**Review Period"** has the meaning set forth in Section 13.3.2.

Section 1.166"**Reviewing Party**" has the meaning set forth in Section 13.2.2.

Section 1.167"**Royalty Term**" means, with respect to each Licensed Product or Option Product (after Option Exercise Effective Date) in each country in the BIG Territory, the period beginning on the date of the First Commercial Sale of such Licensed Product or Option Product in such country and ending on the latest to occur of: (a) the expiration of the last-to-expire Valid Claim of the Licensed Patent Rights or Option Patent Rights, as applicable, in such country that Covers the composition of matter of the active pharmaceutical ingredient of such Licensed Product or Option Product, (b) the expiration of the last-to-expire Valid Claim of the Licensed Patent Rights or Option Patent Rights, as applicable, in such country that Covers the method of use of such Licensed Product or Option Product as indicated on the approved label for such Licensed Product or Option Product in such country, (c) the [***] anniversary of the First Commercial Sale of such Licensed Product or Option Product in such country, and (d) the expiration of Regulatory Exclusivity for such Licensed Product or Option Product in such country.

Section 1.168"**Sales Milestone**" has the meaning set forth in Section 8.3.3.

Section 1.169"**Sale Transaction**" has the meaning set forth in Section 15.10.

Section 1.170"**SCN1A**" means sodium voltage-gated channel alpha subunit 1, which encodes a sodium channel alpha subunit.

Section 1.171"**Segregates**" has the meaning set forth in Section 2.7.2.

Section 1.172"**Selling Party**" has the meaning set forth in Section 1.114.

Section 1.173"**Shared Global Development Costs**" has the meaning set forth in Section 4.5.1.

Section 1.174"**Stoke**" has the meaning set forth in the Preamble.

Section 1.175[***].

Section 1.176"**Stoke Indemnified Parties**" has the meaning set forth in Section 11.2.

Section 1.177"**Stoke Platform**" means [***].

Section 1.178"**Stoke Platform Improvement Know-How**" means all Arising Know-How discovered, invented, developed or otherwise made by or on behalf of either Party or its Affiliates or its or their licensees or Sublicensees (whether solely or jointly with the other Party or its Affiliates or its or their Sublicensees) in the performance of activities or exercise of rights under this Agreement that relates to the Stoke Platform and is not specific to a Licensed Product or Option Product (after the Option Exercise Effective Date).

Section 1.179"**Stoke Platform Improvement Patent Rights**" means all Patent Rights that Cover Stoke Platform Improvement Know-How.

Section 1.180"**Stoke Platform Know-How**" means all (a) with respect to Licensed Products, all Licensed Know-How, or with respect to Option Products (if the Option is exercised), all Option Know-How, in each case that relates to or has general applicability to the Stoke Platform, or otherwise has general applicability, and is not specific to a Licensed Product or Option Product (if the Option is exercised), as applicable, and (b) Stoke Platform Improvement Know-How.

Section 1.181"**Stoke Platform Patent Rights**" means all Licensed Patent Rights (with respect to Licensed Products) or Option Patent Rights (with respect to Option Products if the Option is exercised) that Cover Stoke Platform Know-How for the Licensed Products or Option Products, respectively, including Stoke Platform Improvement Patent Rights. All Stoke Platform Patent Rights existing as of the Effective Date are set forth in Exhibit 1.104(a).

Section 1.182"**Stoke Privacy and Security Obligations**" has the meaning set forth in Section 10.2.19.

Section 1.183"**Stoke Territory**" means the U.S., Canada and Mexico.

Section 1.184"**Stoke Territory Development Plan**" has the meaning set forth in Section 4.2.

Section 1.185"**Sublicensee(s)**" means a Third Party, other than a Third Party subcontractor or distributor, that has been granted a sublicense under the rights granted to a Party pursuant to Sections 2.1.1, 2.1.2 or 2.2.4, in accordance with Section 2.3. Notwithstanding the foregoing, for the purpose of the Net Sales definition, the term "Sublicensee" will not include distributors.

Section 1.186"**Term**" has the meaning set forth in Section 14.1.

Section 1.187"**Territory Development Plan**" has the meaning set forth in Section 4.2.

Section 1.188"**Third Party**" means a Person other than (a) BIG or any of its Affiliates or (b) Stoke or any of its Affiliates.

Section 1.189"**Third Party Claim**" has the meaning set forth in Section 11.1.

Section 1.190"**Third Party Infringement Claim**" has the meaning set forth in Section 9.4.1.

Section 1.191"**Third Party IP**" has the meaning set forth in Section 8.5.

Section 1.192"**Third Party Payments**" has the meaning set forth in Section 8.4.2(c).

Section 1.193"Trademark" means any mark, word, name, symbol, color, shape, designation or any combination thereof, including any trademark, service mark, trade name, brand name, sub-brand name, trade dress, product configuration, program name, delivery form name, certification mark, collective mark, logo, tagline, slogan, design or business symbol, that functions as an identifier of source or origin, whether or not registered, and all statutory and common law rights therein, and all registrations and applications therefor, together with all goodwill associated with, or symbolized by, any of the foregoing.

Section 1.194"**U.S.**" means the United States of America and its territories and possessions.

Section 1.195"**Valid Claim**" means a claim of (a) any issued and unexpired Patent Right whose validity, enforceability or patentability has not been affected by (i) irretrievable lapse, abandonment, revocation, dedication to the public or disclaimer, or (ii) a holding, finding, or decision of invalidity, unenforceability or non-patentability by a court, governmental agency, national or regional patent office or other appropriate body that has competent jurisdiction, such holding, finding or decision being final and unappealable or unappealed within the time allowed for appeal, or (b) a pending patent application that has not been finally cancelled, finally rejected, withdrawn, expired, or abandoned, without the opportunity for appeal, *provided* that Valid Claim shall exclude any such pending claim that (A) has not been granted within [***] after the earliest priority filing date for such application, or (B) has not been or is not being prosecuted in good faith.

Section 1.196"**VAT**" means any value added tax imposed under Council Directive 2006/112/EC (or under any rules, regulation, orders or instruments authorized by that Directive) and any similar value added tax pursuant to the laws of any jurisdiction which is not a member of the European Union.

ARTICLE 2LICENSE GRANT

Section 2.1Licensed Product.

2.1.1 Licenses to BIG. Subject to the terms and conditions of this Agreement, Stoke hereby grants to BIG and its Affiliates:

(a) an exclusive (even as to Stoke and its Affiliates), royalty-bearing, sublicensable (through multiple tiers in accordance with Section 2.3), transferable (in accordance with Section 15.10) license under the Licensed IP to Exploit any and all Licensed Products in the Field in the BIG Territory; and

(b) a non-exclusive, royalty-free, sublicensable (through multiple tiers in accordance with Section 2.3), transferable (in accordance with Section 15.10) license under the Licensed IP to Develop (solely in accordance with the Global Development Plan as set forth in Section 4.1 or BIG Territory Development Plan and subject to and solely in accordance with Section 4.3.2), Manufacture, have Manufactured and export (but for clarity, not Commercialize) any and all Licensed Products in the Field in the Stoke Territory, solely for the purposes of Exploiting such Licensed Products in the Field in the BIG Territory.

For clarity, the foregoing license grant excludes a license or right to Intellectual Property Rights owned or controlled by Stoke or its Affiliates to Exploit a [***]. Notwithstanding the exclusive license grant under clause (a) above, Stoke retains the right under the Licensed IP to Develop (subject to and solely in accordance with Section 4.3.2), Manufacture, have Manufactured and export (but for clarity, not Commercialize) any and all Licensed Products in the Field in the BIG Territory, solely for the purposes of performing its obligations under a Global Development Plan or Exploiting such Licensed Products in the Field in the Stoke Territory.

2.1.2 Licenses to Stoke. Subject to the terms and conditions of this Agreement, BIG hereby grants to Stoke and its Affiliates:

(a) an exclusive (even as to BIG and its Affiliates), royalty-free, sublicensable (through multiple tiers in accordance with Section 2.3), transferable (in accordance with Section 15.10) license under the BIG Collaboration IP to Exploit any and all Licensed Products in the Field in the Stoke Territory; and

(b) a non-exclusive, royalty-free, sublicensable (through multiple tiers in accordance with Section 2.3), transferable (in accordance with Section 15.10) license under the BIG Collaboration IP to (i) perform Stoke's obligations or rights under the Global Development Plan or Stoke Territory Development Plan for any and all Licensed Products anywhere in the world, or (ii) Manufacture, have Manufactured and export (but for clarity, not Commercialize) any and all Licensed Products in the Field in the BIG Territory solely for the purposes of Exploiting such Licensed Products in the Field in the Stoke Territory.

Notwithstanding the exclusive license grant under clause (a) above, BIG retains the right under the BIG Collaboration IP to Develop (subject to and solely in accordance with Section 4.3.2), Manufacture, have Manufactured and export (but for clarity, not Commercialize) any and all Licensed Products in the Field in the Stoke Territory, solely for the purposes of performing its obligations under a Global Development Plan or Exploiting such Licensed Products in the Field in the BIG Territory.

Section 2.2Option Product.

2.2.1 Option. Subject to the terms and conditions of this Agreement, Stoke hereby grants to BIG, a fully paid-up, irrevocable and exclusive (even as to Stoke and its Affiliates), transferable (in accordance with Section 15.10) option ("**Option**") during the Option Period to obtain the Option Product License.

2.2.2 Option Data Package. [***].

2.2.3 Option Exercise. BIG shall have the right, but not the obligation, to exercise the Option by giving Stoke a written notice of BIG's exercise of the Option ("**Option Exercise Notice**") within [***] after receipt of the Option Data Package (such period, the "**Option Period**"). With the exercise of the Option, BIG shall pay Stoke a one-time Option Exercise Fee in accordance with Section 8.2. If an Option Product Third Party Agreement is applicable to such Option, any upfront, royalty or milestone payments that have been or would be payable under an Option Product Third Party Agreement will be [***]. The terms and provisions (other than this Section 2.2) relating to Option Products, including the Option Product License and the exclusivity provisions of Section 2.7 as applicable to an Option Product, shall not become effective until the later of (a) the date on which BIG pays the Option Exercise Fee and (b) completion of the Antitrust Clearance ("**Option Exercise Effective Date**"). [***]

2.2.4 Option Product Licenses.

(a) Subject to the terms and conditions of this Agreement, upon the Option Exercise Effective Date, Stoke hereby grants to BIG and its Affiliates:

(i) an exclusive (even as to Stoke and its Affiliates), royalty-bearing, sublicensable (through multiple tiers in accordance with Section 2.3), transferable (in accordance with Section 15.10) license under the Option IP to Exploit any and all Option Products in the Field in the BIG Territory; and

(ii) a non-exclusive, royalty-free, sublicensable (through multiple tiers in accordance with Section 2.3), transferable (in accordance with Section 15.10) license under the Option IP to Develop (solely in accordance with the Global Development Plan as set forth in Section 4.1 or BIG Territory Development Plan and subject to and in accordance with Section 4.3.2), Manufacture, have Manufactured and export (but for clarity, not Commercialize) any and all Option Products in the Field in the Stoke Territory, solely for the purposes of Exploiting such Option Products in the Field in the BIG Territory (such licenses in clauses (a) and (b) of this Section 2.2.4 together, an "**Option Product License**").

For clarity, the foregoing license grant excludes a license or right to Intellectual Property Rights owned or controlled by Stoke or its Affiliates to Exploit any [***]. Notwithstanding the exclusive license grant under clause (a) above, Stoke retains the right under the Option IP to Develop (subject to and solely in accordance with Section 4.3.2), Manufacture, have Manufactured and export (but for clarity, not Commercialize) any and all Option Products in the Field in the BIG Territory, solely for the purposes of performing its obligations under a Global Development Plan or Exploiting such Option Products in the Field in the Stoke Territory.

(b) **Option Product Licenses to Stoke**. Subject to the terms and conditions of this Agreement, upon the Option Exercise Effective Date, BIG hereby grants to Stoke

and its Affiliates:

(i) an exclusive (even as to BIG and its Affiliates), royalty-free, sublicensable (through multiple tiers in accordance with Section 2.3), transferable (in accordance with Section 15.10) license under the BIG Collaboration IP to Exploit any and all Option Products in the Field in the Stoke Territory; and

(ii) a non-exclusive, royalty-free, sublicensable (through multiple tiers in accordance with Section 2.3), transferable (in accordance with Section 15.10) license under the BIG Collaboration IP to (i) perform Stoke's obligations or rights under the Global Development Plan or Stoke Territory Development Plan for any and all Option Products in the world, or (ii) Manufacture, have Manufactured and export (but for clarity, not Commercialize) any and all Option Products in the Field in the BIG Territory solely for the purposes of Exploiting such Option Products in the Field in the Stoke Territory.

Notwithstanding the exclusive license grant under clause (a) above, BIG retains the right under the BIG Collaboration IP to Develop (subject to and solely in accordance with Section 4.3.2), Manufacture, have Manufactured and export (but for clarity, not Commercialize) any Option Product in the Field in the Stoke Territory, solely for the purposes of performing its obligations under a Global Development Plan or Exploiting such Option Product in the Field in the BIG Territory.

(c) **Antitrust Consents**. Prior to BIG's exercise of the Option, if either Party reasonably determines that a filing or consent with any Governmental Authority pursuant to applicable Antitrust Laws (the "**Antitrust Consents**") is required before the licenses contemplated by an Option may become effective, then each Party shall cooperate with the other Party to prepare and submit any necessary filing(s) and obtain such consents from the applicable Governmental Authority. During the pendency of any waiting period or decision from a Governmental Authority with respect to an Antitrust Consent, the Option Period thus affected shall be tolled, *provided* that in no event shall the Option Period extend beyond [***] without Stoke's written consent, such consent [***]. Notwithstanding the foregoing, either Party may terminate an Option if [***]. Each Party shall bear its own costs and expenses incurred in connection with this Section 2.2.4(c). The date on which all Antitrust Consents are obtained shall be the "**Antitrust Clearance**".

(d) **Bring-Down of Representations, Warranties and Covenants**. As part of the Option Data Package, Stoke shall provide BIG with an updated disclosure schedule of the representations and warranties in Section 10.2 with respect to the Option Product(s) only (the date of the Option Data Package the "**Bring-Down Date**").

Section 2.3Sublicenses. Each Party shall have the right to grant sublicenses through multiple tiers under the licenses granted to it under Sections 2.1.1, 2.1.2 or 2.2.4, by means of written agreement, to its Affiliates or Third Parties; *provided, however*, that as a condition precedent to and requirement of any such sublicense: (a) any such sublicense shall be consistent with and subject to the terms and conditions of this Agreement (including Article 9 and Article 13), and (b) such Party will continue to be responsible for full performance of its obligations under this Agreement and will be responsible for all actions of such Sublicensee as if such Sublicensee were the sublicensing Party hereunder. Notwithstanding the foregoing, neither Party nor its

Affiliates shall have the right to sublicense or delegate any of its rights or obligations under the Global Development Plan to a Third Party without the other Party's prior written consent except that each Party may, without the other Party's prior written consent, delegate any of its rights or obligations under the Global Development Plan to (i) any Third Party contractor specified in a Global Development Plan (such Third Party contractor list may be updated from time to time through the JSC or JDC, as applicable) and (ii) its Affiliates.

Section 2.4Existing Agreement. [***].

Section 2.5No Other Rights. No right or license under any Patent Rights or other Intellectual Property Rights of a Party is granted or shall be granted by implication to the other Party, and each Party covenants not to practice or use any Patent Rights or other Intellectual Property Rights of the other Party except pursuant to the licenses expressly granted in this Agreement. All such rights or licenses are or shall be granted only as expressly provided in this Agreement.

Section 2.6Transfer of Know-How and Technical Assistance. Stoke shall, and shall cause its Affiliates to, [***] disclose and make available to BIG, in a form mutually agreed to by the Parties (*e.g.*, by providing hard copies thereof or electronic access thereto), (a) with respect to each Licensed Product, (i) all necessary or reasonably useful (in Stoke's reasonable judgment) Licensed Know-How that is in existence as of the Effective Date promptly (and in no event later than [***]), in each case to the extent not already provided to BIG, and (ii) subject to Section 4.4.2, all necessary or reasonably useful (in Stoke's reasonable judgment) Licensed Know-How that comes into existence after the Effective Date reasonably promptly after the earliest of the conception, discovery, development or other making of such Licensed Know-How; and (b) with respect to each Option Product, (i) all necessary or reasonably useful (in Stoke's reasonable judgment) Option Know-How that is in existence as of the Option Exercise Effective Date promptly (and in no event later than [***]), and (ii) subject to Section 4.4.2, all necessary or reasonably useful (in Stoke's reasonable judgment) Option Know-How that comes into existence after the Option Exercise Effective Date promptly after the earliest of the conception, discovery, development or other making of such Option Know-How. Each Party shall reasonably cooperate with the other in connection with the transfers contemplated by this Section 2.6 so as not to delay the timelines set forth in the applicable Global Development Plan for such Licensed Product or Option Product. Stoke shall provide BIG with reasonable consultation and assistance regarding such initial transfer of Licensed Know-How and Option Know-How (i.e., the transfers under clauses (a)(i) and (b)(i)) for up to [***] FTE hours per month free-of-charge, and at Stoke's FTE Rate thereafter, in each case for a maximum of [***]. Stoke shall invoice BIG for such FTE Costs on a Calendar Quarter basis, and BIG shall pay to Stoke the invoiced amount within [***] after the receipt of such invoice. During the Term, subject to Section 4.4.2, BIG shall, and shall cause its Affiliates to, at BIG's cost and expense, disclose and make available to Stoke, in a form mutually agreed to by the Parties (*e.g.*, by providing copies thereof or electronic access thereto), all necessary or reasonably useful (in BIG's reasonable judgment) BIG Collaboration Know-How that comes into existence after the Effective Date reasonably promptly after the earliest of the conception, discovery, development or other making thereof. If either Party believes that Know-How Controlled by the other Party that is necessary or reasonably useful (in its own reasonable judgment) for the enjoyment of its rights or performance of its obligations under this Agreement has not been transferred in accordance with this Section 2.6, it shall promptly notify the other Party

and the other Party shall promptly consider such request to transfer or initiate resolution of any disagreement in accordance with Section 15.5. All Know-How to be disclosed or made available by a Party pursuant to this Section 2.6 will be provided in the language in which it was created, provided that if such language is not English, then such Party shall, at its cost and expense, also provide a copy translated into English.

Section 2.7Exclusivity.

2.7.1 Non-Compete.

(a) **Stoke**.

(i) **Licensed Product**. On and after the Effective Date and until the expiration or earlier termination of this Agreement, Stoke shall not and shall cause its Affiliates to not, (a) directly or indirectly, Exploit or (b) license, authorize, appoint or otherwise enable any Third Party to directly or indirectly, Exploit, in each case ((a) and (b)), any Intrathecal SCN1A Product in the Field anywhere in the world, except for activities permitted under this Agreement.

(ii) **Option Product**. On and after the Option Exercise Effective Date and until the expiration or earlier termination of this Agreement, Stoke shall not and shall cause its Affiliates to not, (a) directly or indirectly, Exploit or (b) license, authorize, appoint or otherwise enable any Third Party to directly or indirectly, Exploit, in each case ((a) and (b)), any Non-Intrathecal SCN1A Product in the Field anywhere in the BIG Territory, except for activities permitted under this Agreement.

(b) **BIG**.

(i) **Licensed Product**. On and after the Effective Date and until the expiration or earlier termination of this Agreement, BIG shall not and shall cause its Affiliates to not, (a) directly or indirectly, Exploit or (b) license, authorize, appoint or otherwise enable any Third Party to directly or indirectly, Exploit, in each case ((a) and (b)), any Intrathecal SCN1A Product in the Field anywhere in the world, except for activities permitted under this Agreement.

(ii) **Option Product**. On and after the Option Exercise Effective Date and until the expiration or earlier termination of this Agreement, BIG shall not and shall cause its Affiliates to not, (a) directly or indirectly, Exploit or (b) license, authorize, appoint or otherwise enable any Third Party to directly or indirectly, Exploit, in each case ((a) and (b)), any Non-Intrathecal SCN1A Product in the Field anywhere in the BIG Territory, except for activities permitted under this Agreement.

2.7.2 Exceptions.

(a) In the event a Party (a) acquires any asset of a Third Party that is deemed a Competing Product, or (b) merges or consolidates with, or acquires a Third Party through a transaction or series of transactions whereby such Party does not undergo a Sale Transaction, and such Third Party or any of its Affiliates prior to such transaction or series of transactions is Exploiting any Competing Product as of the effective date of such transaction, then such Party will not be deemed in breach of its obligations under Section 2.7.1 so long as such Party [***].

(b) In the event a Party undergoes a Sale Transaction in which the Third Party acquirer or any of its Affiliates prior to such Sale Transaction is Exploiting any Competing Product as of the effective date of such Sale Transaction or thereafter, then such Party will not be deemed in breach of its obligations under Section 2.7.1 so long as [***].

2.7.3 Acknowledgement. Each Party acknowledges and agrees that (x) this Section 2.7 has been negotiated by the Parties without duress, (y) the geographical and time limitations on activities set forth in this Section 2.7 are reasonable, valid and necessary in light of the Parties' circumstances and necessary for the adequate protection of the business of the Licensed Products and Option Products (after the Option Exercise Effective Date), as applicable, in the Party's respective territory, and (z) neither Party would have entered into this Agreement without the protection afforded it by this Section 2.7. If, notwithstanding the foregoing, a court of competent jurisdiction determines that the restrictions set forth in this Section 2.7 are too broad or otherwise unreasonable (for example, due to a change in circumstance) under applicable Law, including with respect to duration, geographic scope or space, the court is hereby requested and authorized by the Parties to revise this Section 2.7 to include the maximum restrictions allowable under applicable Law.

ARTICLE 3GOVERNANCE

Section 3.1Joint Steering Committee.

3.1.1 Formation. Within [***], the Parties shall establish a joint steering committee (the "**Joint Steering Committee**" or "**JSC**") to serve as the oversight and decision-making body (as set forth herein) for the activities to be conducted by each Party pursuant to the Global Development Plans and Territory Development Plans and for coordinating and sharing information regarding the Parties' activities in their respective territories with respect to the Licensed Product(s) and Option Product(s) (after the Option Exercise Effective Date).

3.1.2 Composition. Unless otherwise agreed to by the Parties, the JSC shall consist of three (3) representatives from each Party, each with the requisite experience and seniority to enable such representative to make decisions on behalf of the applicable Party with respect to the issues falling within the jurisdiction of the JSC. From time to time, each Party may substitute one (1) or more of its representatives to the JSC on written notice to the other Party, which notice may be given by e-mail sent to the other Party's members of the JSC. Each Party shall select from its representatives a chairperson for the JSC to chair meetings on an alternating basis, with the Stoke representative acting as chairperson for the first meeting. From time to time, either Party may change the representative who will serve as chairperson on written notice to other Party.

3.1.3 Responsibilities. The JSC shall perform the following functions, subject to the final decision-making authority of the respective Parties as set forth in Section 3.3.

(a) keep each Party reasonably informed as to the Development, Commercialization and other Exploitation of the Licensed Product(s) and Option Product(s) (after the Option Exercise Effective Date) in its respective territory;

(b) review (but not approve) the initial Global Development Plan for

each Licensed Product and Option Product (after the Option Exercise Effective Date), other than, for clarity, the Global Development Plan for zorevunersen attached hereto;

(c) review and approve any updates or amendments to each Global Development Plan;

(d) review (but not approve) each Party's territory-specific Development and Commercialization strategies and activities (including each Party's Territory Development Plan, and any updates thereto, pricing strategies and revenue forecasts, global branding strategies, and medical affairs) for the Licensed Product(s) and Option Product(s) (after the Option Exercise Effective Date) with respect to potential impact on the other Party's territory; *provided* that this JSC review right is intended to be high level and shall not extend to cover review of day-to-day activities;

(e) review (but not approve) the overall global strategy for Manufacturing of the Licensed Product(s) and Option Product(s) (after the Option Exercise Effective Date) for clinical and commercial uses, including plans for packaging, Labeling, supply chain and trade and distribution activities and risk mitigation strategies, subject to the provisions of the Clinical Supply Agreement;

(f) discuss (but not approve) Know-How Controlled by BIG for potential application to the Licensed Products or Option Products (after the Option Exercise Effective Date);

(g) establish subcommittees, as the JSC may deem necessary or appropriate for the advancement of the collaboration and delegate JSC's authorities set forth herein, as described more fully in Section 3.2 below;

(h) direct and oversee any subcommittee authorized by the JSC, including the JDC and JCC;

(i) resolve disputed matters that may arise at the subcommittees;

(j) perform any and all tasks and responsibilities that are otherwise expressly attributed to the JSC under the Agreement; and

(k) perform such other functions as the Parties may mutually agree in writing.

3.1.4 Meetings and Minutes. The JSC shall meet quarterly or as otherwise agreed to by the Parties. The JSC shall hold its meetings via teleconference or video conferences unless the Parties agree, on a meeting-by-meeting basis, to hold a meeting in person at mutually agreed location (which location will not be in the U.S. without BIG's prior written consent). Either Party may call meetings on no less than [***] notice. Each Party shall make all proposals for agenda items and shall provide all appropriate information to the other Party with respect to such proposed items at least [***] in advance of the applicable meeting; *provided that* under exigent circumstances requiring input by the JSC a party may provide its agenda items to the other Party within a shorter period of time in advance of the meeting, or may propose that there not be a

specific agenda for a particular meeting, so long as the other Party consents to such later addition of such agenda items or the absence of a specific agenda for such meeting, such consent not to be unreasonably withheld, conditioned or delayed. The chairperson of the JSC shall prepare and circulate for review and approval of the Parties minutes of each meeting within [***] after each meeting, which minutes will include, at a minimum, a list of participants, agenda topics, decisions made, misalignments, and action items, and such other matters as may be agreed upon by the JSC. The Parties shall agree on the minutes of each meeting promptly, but in no event later than the next meeting of the JSC.

3.1.5 Procedural Rules. The JSC shall have the right to adopt such standing rules as shall be necessary for its work, to the extent that such rules are not inconsistent with this Agreement. JSC members may attend a meeting either in person or by telephone, video conference, or similar means in which each participant can hear what is said by, and be heard by, the other participants. Alliance Managers and other employees or Third-Party designees of either Party that are not JSC members may attend meetings of the JSC to the extent reasonably necessary to address matters on the agenda; *provided that* such attendees (a) shall not vote or otherwise participate in the decision-making process of the JSC, and (b) are bound by obligations of confidentiality and non-disclosure equivalent to those set forth in Article 13. Any such Third Parties must be notified to the other Party's JSC members at least [***] in advance of the applicable meeting.

Section 3.2Subcommittees.

3.2.1 General. The JSC may establish subcommittees as it deems necessary or appropriate for the advancement of the collaboration, including a subcommittee for joint development ("**Joint Development Committee**" or the "**JDC**") as set forth in Section 3.2.2, and a subcommittee for joint commercialization ("**Joint Commercialization Committee**" or the "**JCC**") as set forth in Section 3.2.3. The JSC may disband any subcommittees if the function of such subcommittee is no longer relevant. Each subcommittee shall be composed of an equal number of representatives of each Party, which number shall be mutually agreed by the Parties. Each Party shall be free to change its representatives on written notice to the other Party or to send a substitute representative to any subcommittee meeting. Each Party's representatives and any substitute for a representative shall be bound by the obligations of confidentiality set forth in Article 13. Except as expressly provided in this Agreement, no subcommittee shall have the authority to bind the Parties hereunder (and, for clarity, no subcommittee authority shall exceed that of the JSC), and each subcommittee shall report to the JSC. Any matters arising within a subcommittee that are not resolved by members of such subcommittee shall be submitted to the JSC for resolution. All subcommittees shall hold meetings according to the meeting schedule of the JSC, unless the Parties mutually agree upon other schedules and procedures.

3.2.2 Joint Development Committee. The JDC shall oversee the Development of the Licensed Product(s) and Option Product(s) (after the Option Exercise Effective Date), and shall serve as a forum for the coordination of Development activities for the Licensed Products and Option Product(s) (after the Option Exercise Effective Date), including medical affairs, in each case subject to the oversight of the JSC. The JDC shall, consistent with the terms and conditions set forth in this Agreement:

(a) prepare and submit for approval by the JSC any amendments

proposed by either Party to a Global Development Plan (and Global Development Budget) for each Licensed Product and Option Product (after the Option Exercise Effective Date);

(b) review and oversee implementation of Development under the Global Development Plans, including monitoring progress of the Parties' activities under such Global Development Plans;

(c) serve as a forum to discuss any invention made, created or discovered as a result of activities performed under this Agreement;

(d) discuss strategies for obtaining Regulatory Approval for each Licensed Product and Option Product (after the Option Exercise Effective Date) in any economically significant country or region (which, in the case of the BIG Territory, will consist of the EU5, Japan and PRC and the top five (5) other countries by anticipated Net Sales in the BIG Territory) (including whether or not to seek Regulatory Approval in such country or region);

(e) review (but not approve) each Territory Development Plan and any updates thereto;

(f) discuss and recommend to the JSC as to whether a Proposed Global Study should be included in a Global Development Plan;

(g) develop a global publication strategy for the Licensed Products and Option Products, including a strategy for the publication of Data generated under this Agreement ("**Publication Strategy**");

(h) discuss and attempt to resolve any disputes between the Parties relating to the Development activities; and

(i) such other responsibilities allocated to the JDC by the JSC under this Agreement.

3.2.3 Joint Commercialization Committee. The JCC shall serve as a forum between the Parties for overseeing and coordinating the Commercialization of the Licensed Product(s) and Option Product(s) (after the Option Exercise Effective Date), and shall perform such other functions as are set forth herein or assigned by the JSC or as the Parties may mutually agree in writing, in each case subject to the oversight of the JSC. The JCC shall, consistent with the terms and conditions set forth in this Agreement:

(a) discuss the commercialization plan for each Licensed Product and Option Product (after the Option Exercise Effective Date), including any amendments thereto;

(b) discuss and oversee global trademark and branding strategy with respect to Licensed Product(s); and

(c) such other responsibilities allocated to the JCC by the JSC under this Agreement.

Section 3.3Decision Making.

3.3.1 Process. Except as set forth herein, in order to make any decision required of it hereunder, the JSC and each subcommittee must have present (in person, by videoconference or telephonically) at least two (2) representatives appointed by each Party. Decisions of the JSC and each subcommittee shall be by consensus, with each Party having one (1) vote irrespective of the number of representatives of such Party in attendance.

3.3.2 Dispute Resolution. If a dispute arises for a matter under the purview of a subcommittee that cannot be resolved by such subcommittee, the Alliance Manager of either Party may refer such dispute to the JSC for resolution. If a dispute arises for a matter under the purview of the JSC that cannot be resolved by the JSC within [***] after such matter was first referred to or arose at the JSC, the JSC representatives of either Party may cause such dispute to be referred to the Executive Officers for resolution. Such Executive Officers (or their designees) will in good faith seek to resolve the matter. Any final decision agreed to by the Executive Officers in writing shall be conclusive and binding on the Parties. If the Executive Officers are not able to agree on the resolution of any such issue within [***] after such issue was first referred to them, then the following shall apply:

(a) Stoke shall have final decision making authority with respect to [***];

(b) BIG shall have final decision making authority with respect to [***];

(c) [***].

(d) for any other disputed matters under the purview of the JSC or a subcommittee and not otherwise covered by clauses (a)-(c) above, either Party shall have the right to bring such dispute for resolution pursuant to the mechanisms set forth in Section 15.5.

3.3.3 Notwithstanding any to the contrary in this Section 3.3, [***]. This Section 3.3.3 shall not apply to territory-specific Development activities such as territory-specific clinical trials.

3.3.4 For clarity, disputes arising between the Parties in connection with or relating to this Agreement or any document or instrument delivered in connection herewith, and that are outside of the jurisdiction of the JSC, shall be resolved pursuant to Section 15.5.

3.3.5 Day-to-Day Conduct. Each Party will be responsible for day-to-day implementation and operations of the activities for which it has or is otherwise assigned responsibility under a Global Development Plan, *provided* that such implementation is not inconsistent with such Global Development Plan.

Section 3.4Limitation on Authority. The JSC and any subcommittee shall only have the powers assigned expressly to it in this Article 3 and elsewhere in this Agreement, and shall not have any power to amend, modify or waive compliance with this Agreement. In furtherance thereof, each Party shall retain the rights, powers and discretion granted to it under this Agreement and no such rights, powers or discretion shall be delegated or vested in the JSC or subcommittee

unless such delegation or vesting of rights is expressly provided for in this Agreement or the Parties expressly so agree in writing.

Section 3.5Discontinuation of JSC and Subcommittees. The JSC and each subcommittee shall continue to exist until [***].

Section 3.6Alliance Managers. Each Party shall promptly after the Effective Date appoint a senior representative of such Party having a general understanding of pharmaceutical Development and Commercialization issues who shall be the primary contacts between the Parties, and shall have such other responsibilities as the Parties may agree in writing after the Effective Date, which individual may be replaced at any time by notice in writing to the other Party (the "**Alliance Managers**"). The Alliance Managers shall (a) serve as the primary points of contact between the Parties under this Agreement, (b) take responsibility for providing that governance meetings and the production of meeting agendas and minutes occur as set forth in this Agreement (and the Alliance Managers shall facilitate such activities on behalf of the JSC or other relevant subcommittee), and that relevant action items resulting from such meetings are appropriately carried out or otherwise addressed, and (c) work together to manage and facilitate the communication between the Parties under this Agreement, including the resolution (in accordance with the terms of this Agreement) of issues between the Parties that arise in connection with this Agreement. The Alliance Managers shall not have final decision-making authority with respect to any matter under this Agreement. Each Alliance Manager shall attend meetings of the JSC and each subcommittee as an observer.

Section 3.7Cooperation. Each Party shall provide the JSC and each subcommittee, as applicable, such information as reasonably required under this Agreement or as otherwise reasonably requested by the other Party and reasonably available to such Party, to enable the JSC and each subcommittee to perform its designated role hereunder.

Section 3.8Expenses. Each Party shall be responsible for all travel and related Costs for its members and other representatives to attend meetings of, and otherwise participate on, the JSC and the subcommittees.

ARTICLE 4DEVELOPMENT

Section 4.1Global Development.

(a) **Development Plan**. On a Licensed Product-by-Licensed Product and Option Product-by-Option Product (after the Option Exercise Effective Date) basis, the Parties shall conduct Development activities for each such Licensed Product and Option Product, as applicable, that are reasonably expected to generate Data necessary for, or otherwise in support of, Regulatory Approval of such Licensed Product or Option Product in both the BIG Territory and the Stoke Territory as set forth in a development plan, which initial plan must be mutually agreed to by the Parties in writing (each such initial plan, as may be updated or amended as provided herein, a "**Global Development Plan**"). [***] Each Global Development Plan shall include (i) a reasonably detailed description of each Development activity (collectively, "**Global Development Activities**"), (ii) a budget for such activities set out on an activity-by-activity basis for each Calendar Quarter of the term of such Global Development Plan ("**Global Development Budget**"),

(iii) an allocation of responsibilities between Stoke and BIG for each such activity (the responsible Party the "**Lead Development Party**"), and (iv) the anticipated timing and timelines for conducting and completing each such activity. The initial Global Development Plan mutually agreed to by the Parties for the Initial Licensed Product (as it exists on the Effective Date) is set forth in Exhibit 4.1. The Parties intend that each Global Development Plan to be designed to include such Development activities as the Parties may reasonably determine are necessary or appropriate for the Parties to obtain Regulatory Approval for the Licensed Product or Option Product (after the Option Exercise Effective Date), as applicable, in both the Stoke Territory and BIG Territory (other than territory-specific activities that may be required by a specific country's Regulatory Authority, such as bridging studies).

(b) **Updates**. On an annual basis (no later than [***]), or more often as the Parties deem appropriate, the Parties shall update or amend (via the JSC), as appropriate, each then-current Global Development Plan or Global Development Budget for the upcoming Calendar Year. Such update or amendment shall reflect any agreed changes, re-prioritization of, or additions to the agreed upon Global Development Activities set forth therein. The Parties shall, in preparing updates or amendments to a Global Development Plan or Global Development Budget, endeavor to: (i) maintain, to the extent reasonably practical and appropriate, continuity in functions and commitments of personnel and physical resources of the Parties, and (ii) promote the efficient and coordinated continued Development of the relevant Licensed Product or Option Product globally. Once approved by the JSC, each updated or amended Global Development Plan and Global Development Budget shall become effective and supersede the previous version of such Global Development Plan and Global Development Budget as of the date of such approval or at such other time as decided by the JSC.

Section 4.2Territory Development Plans. On a Licensed Product-by-Licensed Product and Option Product-by-Option Product (after the Option Exercise Effective Date) basis, except for the activities allocated to a Party under a Global Development Plan, all Development of Licensed Product or Option Product by or on behalf of a Party or its Affiliates in its territory shall be conducted in accordance with a written development plan (each such plan, as updated and amended from time to time as permitted herein, a "**Territory Development Plan**" and, the Territory Development Plan for the BIG Territory, the "**BIG Territory Development Plan**" and the Territory Development Plan for the Stoke Territory, the "**Stoke Territory Development Plan**"). Each Party will prepare its initial Territory Development Plan and submit it to the JSC (or JDC, as appropriate) for review and discussion within [***] after the Effective Date for the Initial Licensed Product or, with respect to each other Licensed Product or Option Product (after the Option Exercise Effective Date), within [***] of the Parties' agreement on a Global Development Plan for such Licensed Product or Option Product. Each Territory Development Plan shall include with respect to the relevant Licensed Product or Option Product, at an executive summary level of detail, (a) all clinical and non-clinical studies planned or being conducted by such Party, its Affiliates or Sublicensees for such Party's territory, and anticipated timelines therefor, (b) an anticipated timeline for submitting MAAs in each country in such Party's territory, and (c) an anticipated timeline for obtaining Regulatory Approval in each country in such Party's territory, *provided* that notwithstanding any provision to the contrary, with respect to the foregoing subsections (b) and (c), the BIG Territory Development Plan is not required to contain information for countries in the BIG Territory other than EU5, Japan and PRC and the top five (5) other countries by anticipated Net Sales in the BIG Territory. On an annual basis (no later than [***]),

or more often as the Parties deem appropriate, for each Territory Development Plan, each Party shall submit an updated or amended version of such Territory Development Plan for the upcoming Calendar Year to the JSC (or JDC, as appropriate) for review and discussion. Each Party shall consider in good faith any reasonable comments provided by the other Party thereon.

Section 4.3Rights and Responsibilities of Development.

4.3.1 Performance. Each Party shall perform or cause to be performed, any and all of its Development activities for a Licensed Product or Option Product (after the Option Exercise Effective Date) in good scientific manner and in compliance with all applicable Laws and in accordance with the applicable Global Development Plan and Territory Development Plan.

4.3.2 Development Outside of Territory. Notwithstanding anything to the contrary in this Agreement, neither Party may conduct any Development activities for any Licensed Product or Option Product (after the Option Exercise Effective Date) in the other Party's territory unless such activity in the other Party's territory is either (a) an activity that is expressly set forth in the Global Development Plan and for which such Party is responsible, or (b) expressly authorized in writing by the other Party.

4.3.3 Subcontracting. Subject to Section 2.3, each Party shall have the right to subcontract its Development activities to a Third Party; *provided* that no such permitted subcontracting shall relieve the subcontracting Party of any obligation hereunder, and any act or omission of any such subcontractor shall constitute the act or omission of the subcontracting Party for all purposes hereunder.

4.3.4 Diligence.

(a) Each Party shall use Commercially Reasonable Efforts to perform the Development activities allocated to it under the Global Development Plan in accordance with the timelines set forth therein.

(b) BIG shall use Commercially Reasonable Efforts to [***].

Section 4.4Development Records; Report; and Data Sharing.

4.4.1 Development Records. Each Party shall maintain, in good scientific manner and in compliance with applicable Laws, complete and accurate books and records with respect to its Development of each Licensed Product and Option Product (after the Option Exercise Effective Date), which books and records shall (a) be appropriate for patent and regulatory purposes, (b) properly reflect all work done and results achieved in the performance of such Development activities, and (c) record only such activities relevant to this Agreement and shall not include or be commingled with records of activities outside the scope of this Agreement. Such books and records shall be retained by such Party for at least [***] after the expiration or termination of this Agreement or for such longer period as may be required by applicable Law. Each Party shall have the right to inspect and copy all books and records of the other Party pertaining to Development activities conducted under a Global Development Plan during normal business hours and upon reasonable notice. The inspecting Party shall maintain the books and records of the other Party disclosed thereby in confidence in accordance with Article 13.

4.4.2 Development Reports; Data Sharing. Without limiting Section 2.6, within [***], such Party shall provide to the JSC (or the JDC, if applicable) (a) an update of such Development activities it has performed, or caused to be performed, since the preceding report, its Development activities (including medical affairs activities) in-process and the future Development activities it expects to initiate during the following twelve (12)-month period and (b) subject to Section 4.6, a summary of all Data from Development activities described in such update that are generated from Development activities under the purview of a Global Development Plan or applicable Territory Development Plan, with such detailed Data to be provided upon request. All Data to be disclosed or made available by a Party pursuant to this Section 4.4.2 will be provided in the language in which it was created, *provided* that if such language is not English, then such Party shall, at its cost and expense, also provide a copy translated into English.

Section 4.5Costs of Development Activities.

4.5.1 Cost Sharing. Each Party shall bear all FTE Costs for all Development activities for each Licensed Product and Option Product (after Option Exercise Effective Date) that it or its Affiliates conducts, or is otherwise responsible for conducting under a Global Development Plan. With respect to Out-of-Pocket Expenses for Development activities in a Global Development Plan that do not exceed [***] of the applicable annual Global Development Budget (on an annual basis) as a result of cost overruns or Immaterial Changes (the "**Shared Global Development Costs**"), unless otherwise specified in a Global Development Plan, (a) BIG shall bear thirty percent (30%) of the Shared Global Development Costs; and (b) Stoke shall bear seventy percent (70%) of the amount set forth in the Global Development Budget; and, for any particular Development activity for which the cost of such activity exceeds [***] of the applicable annual Global Development Budget for such activity, unless otherwise agreed to by the Parties, the Lead Development Party for such activity shall be solely responsible for all Out-of-Pocket Expenses for such activity in excess of that [***] amount. By way of example, [***]. Shared Global Development Costs may include Out-of-Pocket Expenses for CMC activities; the conduct of clinical trials, including the cost of study sites, start-up fees and patient visit fees, laboratory services providers and contract research organizations, logistics costs (such as shipping, insurance and storage costs of clinical supply and placebo), advertising costs for recruitment of patients, and costs for branding, public relations, and communications plans, including publications and educational programs; the supply of the Licensed Product or Option Product, as applicable, for use in clinical trials (as well as the costs for placebo and any Third Party product); medical affairs activities; and pharmacovigilance, including establishing, updating, and maintaining a global safety database. Subject to Section 4.6, as between the Parties, each Party will be responsible for all costs and expenses (internal and external) incurred by or on behalf of it or its Affiliates in the course of performing Development activities for a Licensed Product or Option Product (after to Option Exercise Effective Date) that are outside the purview of a Global Development Plan.

4.5.2 Payments.

(a) All Shared Global Development Costs to be paid by one Party to the other Party will be made in arrears pursuant to invoices submitted by the owing Party to the paying Party.

(b) Within [***] after the end of each Calendar Quarter in which such

Shared Global Development Costs are incurred, each Party shall provide a report, in a mutually agreed upon format, to the finance officer of the other Party designated by such other Party. The reporting will include information regarding the total amount of the Shared Global Development Costs that it actually incurred in the preceding Calendar Quarter together with reasonable supporting documentation evidencing such Out-of-Pocket Expenses.

(c) With respect to Shared Global Development Costs incurred by Stoke prior to the effective date of a Global Development Plan for activities that will be performed after the effective date of such Global Development Plan (e.g., in the case of pre-payment by Stoke to a contract research organization), then Stoke shall have the right to include such costs in its reports for purposes of reconciliation in the first Calendar Quarter report.

(d) Based on such information, within [***] after the end of the applicable Calendar Quarter, Stoke will prepare and deliver to BIG a composite report that: (i) summarizes the Shared Global Development Costs incurred by each Party for such Calendar Quarter, (ii) calculates the portion of the total Shared Global Development Costs for which each Party is responsible for such Calendar Quarter in accordance with Section 4.5.1, and (iii) computes the amount in Dollars due to BIG or Stoke, as the case may be, for such Calendar Quarter based upon the Parties' respective cost allocations in accordance with Section 4.5.1.

(e) If a Party is owed any amount from the other Party for a particular Calendar Quarter, then (i) such Party shall send an invoice for such amount within [***] after delivery of the composite report to BIG, and (ii) the other Party shall make payment of such invoiced amount in Dollars to the other Party within [***] after its receipt of such invoice.

(f) Each Party shall have the right to audit the records of the other Party with respect to any purported Shared Global Development Costs included in such reports, in accordance with Section 8.11 of this Agreement.

(g) Subject to the foregoing, in no event will a Party be required to pay any amounts set forth in any invoice for Shared Global Development Costs for activities that were performed more than [***] prior to the issuance of such invoice.

Section 4.6Additional Global Studies.

4.6.1 Proposal for New Global Studies. If either Party (the "**Proposing Party**") intends to conduct any additional clinical or non-clinical studies (including expansions thereof) with respect to a Licensed Product or Option Product (after the Option Exercise Effective Date), the Proposing Party shall, prior to commencing such activity, first propose to the JSC (or the JDC, if applicable) to conduct such Development activity, study, experiment or clinical trial or expansion thereof as a global study (which proposal shall include a proposed budget of the reasonably anticipated Out-of-Pocket Costs for conducting the Proposed Global Study) under the Global Development Plan (each such activity, study, experiment or clinical trial or expansion thereof, a "**Proposed Global Study**"). If the other Party (the "**Non-Proposing Party**") does not consent to the inclusion of such Proposed Global Study in the applicable Global Development Plan, then the Proposing Party may conduct such Proposed Global Study on its own accord (and such Proposed Global Study shall be added to the Proposing Party's applicable Territory

Development Plan), *provided* that the Proposing Party may not conduct any part of such Proposed Global Study in the Non-Proposing Party's territory unless the Non-Proposing Party's provides advanced written consent; *provided further* that the Proposing Party shall not make material changes to the Proposed Global Study (*e.g.,* removing any arm of any clinical trial, adding a new arm to any clinical trial, or making other material changes), without re-presenting the applicable Development activity in accordance with this Section 4.6.1. If a Proposed Global Study is not included in a Global Development Plan, (a) the Proposing Party shall bear all the FTE Costs and Out-of-Pocket Expenses of such Proposed Global Study, (b) the Proposing Party will make all Data generated from the Proposed Global Study available for viewing by the Non-Proposing Party as part of its quarterly reporting requirements under Section 4.4.2, and (c) the Non-Proposing Party will not have the right to provide any input on Proposed Global Study (for clarity, subject to Section 3.3.2(a) and Section 3.3.2(b)) or use any Data generated from such Proposed Global Study except as set forth in Section 4.6.2 or to comply with safety reporting obligations or the Pharmacovigilance Agreement, or otherwise as necessary to support disclosure requirements under applicable Law.

4.6.2 If the Proposed Global Study is not included in the Global Development Plan, [***].

Section 4.7Backup Products. If the Parties mutually agree in writing that further Development or Commercialization of the Initial Licensed Product is not commercially feasible (whether due to scientific, safety, efficacy, commercial, or other concerns), the Parties may agree in writing to pursue Development and Commercialization of an Additional Licensed Product pursuant to a mutually agreed upon Global Development Plan for such Additional Licensed Product. In no event shall either Party conduct Development of any Licensed Product or any Option Product (after the Option Exercise Effective Date) unless the Parties have mutually agreed to conduct Development of such Licensed Product or Option Product under a Global Development Plan hereunder.

ARTICLE 5REGULATORY

Section 5.1Regulatory Responsibilities.

5.1.1 Responsibilities of Stoke. Unless otherwise agreed by the Parties, Stoke shall be the Marketing Authorization Application and Regulatory Approval holder for each Licensed Product and Option Product in the Stoke Territory and shall be responsible for and have final decision making authority with respect to all Regulatory Filings and interactions with Regulatory Authorities relating thereto, and the maintenance of Regulatory Approval thereof. Without limiting the foregoing and to the extent permitted under applicable Laws, Stoke shall (a) provide BIG with an opportunity to review and comment on all material Regulatory Filings for each Licensed Product and Option Product (after Option Exercise Effective Date) in the Stoke Territory and consider in good faith any comments from BIG with respect thereto; and (b) if permitted under applicable Law, provide BIG with access for at least one (1) BIG employee or agent to attend as an observer any meeting, conference or discussion (including any advisory committee meeting) with a Regulatory Authority in respect of each Licensed Product and Option Product (after Option Exercise Effective Date), in the Stoke Territory, to the extent such meeting, conference or discussion is reasonably expected to have a material effect on BIG's Exploitation of

such Licensed Product and Option Product (after Option Exercise Effective Date), in the BIG Territory.

5.1.2 Responsibilities of BIG. Unless otherwise agreed by the Parties, BIG or its designated Affiliate shall be the Marketing Authorization Application and Regulatory Approval holder for each Licensed Product and Option Product (after Option Exercise Effective Date) in the BIG Territory and shall be responsible for and have final decision making authority with respect to all Regulatory Filings and interactions with Regulatory Authorities relating thereto, and the maintenance of Regulatory Approval thereof. Without limiting the foregoing and to the extent permitted under applicable Laws, BIG shall (a) provide Stoke with an opportunity to review and comment on all material Regulatory Filings for each Licensed Product and Option Product (after Option Exercise Effective Date) in [***] and consider in good faith any comments from Stoke with respect thereto; and (b) if permitted under applicable Law, provide Stoke with access for at least one (1) Stoke employee or agent to attend as an observer any meeting, conference or discussion (including any advisory committee meeting) with a Regulatory Authority in respect of each Licensed Product and Option Product (after Option Exercise Effective Date) in [***], to the extent such meeting, conference or discussion is reasonably expected to have a material effect on Stoke's Exploitation of such Licensed Product and Option Product (after Option Exercise Effective Date) in the Stoke Territory.

Section 5.2Right of Reference. Subject to the terms and conditions of this Agreement, and to the extent permissible under applicable Law, each Party hereby grants the other Party a right of reference to use and access all Regulatory Filings Controlled by such Party or its Affiliates and specific to a Licensed Product or Option Product (after Option Exercise Effective Date) and Data (including CMC-related information) referenced therein solely for the purpose of and to the extent necessary or reasonably useful to support the other Party's Regulatory Filings for Licensed Products and Option Products (after Option Exercise Effective Date) in a country or other jurisdiction in the other Party's territory. Notwithstanding the foregoing, neither Party shall have a right of reference to use and access any portion of a Regulatory Filing and Data referenced therein Controlled by the other Party or its Affiliates to the extent specifically related to a study that is neither conducted under a Global Development Plan nor accessed through the opt-in mechanism of Section 4.6.2, unless such use and access is necessary for such Party (or its Affiliates) to comply with applicable Laws in a country or jurisdiction in its territory (e.g., for safety reporting purposes).

Section 5.3Regulatory Updates. Each Party shall keep the other Party reasonably informed through the JSC (and the JDC, if applicable) and of all material regulatory developments relating to each Licensed Product and Option Product (after Option Exercise Effective Date) in its respective territory.

Section 5.4Pharmacovigilance Agreement and Global Safety Database. Within [***] after the Effective Date with respect to the Initial Licensed Product and before commencement of dosing in a human for any new Licensed Product or Option Product (after Option Exercise Effective Date), the Parties shall negotiate and execute a reasonable and customary pharmacovigilance agreement setting forth the responsibilities of each Party with respect to pharmacovigilance matters relating to each Licensed Products and Option Product (after Option Exercise Effective Date) throughout the world, including which Party shall make determinations with respect to reporting any adverse drug experiences and monitoring of

prescription events in the Parties' respective territories. Each Party shall duly and punctually perform all of its obligations under such pharmacovigilance agreement. Stoke shall hold and maintain the global safety database for each Licensed Product and Option Product (after Option Exercise Effective Date), and the Out-of-Pocket Expenses for maintaining such global safety database shall be a Shared Global Development Cost under the relevant Global Development Plan. Stoke shall provide BIG with all information necessary or reasonably useful for BIG to comply with its pharmacovigilance responsibilities in the BIG Territory and BIG shall provide Stoke with all information necessary or reasonably useful for Stoke to comply with its pharmacovigilance responsibilities in the Stoke Territory, including, in each case and as applicable, any adverse drug experiences (including those events or experiences that are required to be reported to Regulatory Authorities under applicable Laws), from pre-clinical or clinical laboratory, animal toxicology and pharmacology studies, clinical studies and commercial experiences with a Licensed Product or Option Product (after Option Exercise Effective Date), in each case in the form specified in the pharmacovigilance agreement (or, until the pharmacovigilance agreement is executed, in the form reasonably requested by a Party).

ARTICLE 6MANUFACTURING AND SUPPLY

Section 6.1Technology Transfer. With respect to each Licensed Product and Option Product (after Option Exercise Effective Date), upon BIG's request, Stoke shall transfer and, subject to the terms and conditions of its agreements with its applicable CMOs, shall use Commercially Reasonable Efforts to cause its applicable CMOs to transfer to BIG or its designee all Licensed Know-How and samples of proprietary Materials Controlled by Stoke and necessary or reasonably useful for the Manufacture of such Licensed Product and Option Product (after Option Exercise Effective Date) (including Manufacture of all intermediates (if any) and non-commercially available components thereof, in each case to the extent they are subject to a separate Manufacturing process Controlled by Stoke) pursuant to the then-current process for the Manufacture of such Licensed Product and Option Product (after Option Exercise Effective Date) (such process, the "**Manufacturing Process**"). If Stoke's applicable CMO does not transfer to BIG the Licensed Know-How necessary or reasonably useful for the Manufacture of such Licensed Product and Option Product in accordance with the preceding sentence, Stoke shall independently make such transfer to BIG to the extent it is in possession and Control of the Licensed Know-How that the CMO should have transferred. Stoke shall provide reasonable technical support for a period of [***] after completion of such Manufacturing Process transfer to BIG or its designee as may be necessary or reasonably useful for BIG or its designee to use and practice the Manufacturing Process, such support not to exceed [***]. Upon BIG's request, Stoke shall also reasonably facilitate BIG or its Affiliate with entering into agreements directly with Stoke's or its Affiliates' Third Party contract manufacturers. Each Party shall be responsible for its own internal Costs incurred in connection with performing the Manufacturing technology transfer set forth in this Section 6.1, *provided* that the parties shall share equally (on a 50:50 basis) all amounts payable to Third Party CMOs in connection with transfer of Manufacturing Process.

Section 6.2Clinical Supply. Within [***] after the Effective Date, BIG and Stoke will enter into a separate, mutually-agreed upon clinical manufacturing and supply agreement on commercially reasonable terms (the "**Clinical Supply Agreement**"), pursuant to which Stoke or its designee shall Manufacture and supply to BIG all of BIG's requirements for each Licensed Product and Option Product (after Option Exercise Effective Date) for purposes of BIG's clinical

Development activities under this Agreement (including activities pursuant to the Global Development Plan and BIG's other Development activities permitted under this Agreement as set forth in the BIG Territory Development Plan for each Licensed Product and Option Product (after Option Exercise Effective Date)). The Clinical Supply Agreement will include the terms set forth on Exhibit 6.2. [***].

Section 6.3Commercial Supply.

6.3.1 BIG shall have the right to Manufacture or procure its own commercial supply of Licensed Product and Option Product (after Option Exercise Effective Date) for the BIG Territory. The Parties will cooperate to achieve a coordinated global supply of Licensed Product(s) and Option Product(s) (after Option Exercise Effective Date) by sharing, and requiring their respective CMOs to share (with respect to Stoke's CMOs as of the Effective Date, to the extent permitted under Stoke's agreement with such CMO), data from Manufacturing processes and trends, out-of-specification batches, and any proposed and actual process changes that may affect any attribute of the Licensed Product's or Option Product's (after Option Exercise Effective Date) quality or supply continuity.

6.3.2 [***].

6.3.3 [***].

Section 6.4Stoke Manufacture. Notwithstanding anything to the contrary herein, to the extent that Stoke Manufactures any supply of Licensed Product or Option Product for distribution in the BIG Territory, BIG may elect, at its cost and expense, to perform certain activities in connection with such Manufacturing sufficient (in BIG's reasonable judgment) to permit BIG's activities hereunder to constitute "manufacturing" in the relevant non-U.S. jurisdiction for purposes of Section 954(d) of the Internal Revenue Code of 1986, as amended, and the U.S. Treasury regulations promulgated thereunder.

ARTICLE 7COMMERCIALIZATION

Section 7.1Commercialization Responsibilities. Subject to oversight of the JSC (or JCC if applicable), as between the Parties:

7.1.1 BIG (itself or through its Affiliates or its or their Sublicensees) shall have the sole right and decision-making authority to Commercialize Licensed Product(s) and Option Product(s) (after Option Exercise Effective Date)in the Field in the BIG Territory at its sole Costs [***]; and

7.1.2 Stoke (itself or through its Affiliates or its or their Sublicensees) shall have the sole right and decision-making authority to Commercialize Licensed Product(s) and Option Product(s) (after Option Exercise Effective Date) in the Field in the Stoke Territory at its sole Costs [***].

Section 7.2Diligence. BIG shall use Commercially Reasonable Efforts to [***].

Section 7.3Commercialization Updates. Each party shall keep the other Party

reasonably informed through the JSC (or JCC if applicable) as to its Commercialization plans, strategies and activities in its territory, including [***]. During the Term, each Party shall update its Commercialization plan annually for the following Calendar Year and submit to the JSC (or JCC if applicable) for review no later than [***] prior to the beginning of the following Calendar Year. During the Term, to the extent permitted under applicable Laws, the Parties shall discuss through the JSC (or JCC if applicable) any global Commercialization activities (including associated Costs) that may benefit Licensed Product(s) and Option Product(s) (after Option Exercise Effective Date) globally. Notwithstanding anything to the contrary in this Agreement, the JSC and JCC shall be a forum for information exchange for Commercialization matters and shall not have any power to direct either Party with respect to its Commercialization activities under this Agreement.

Section 7.4Pricing. [***].

7.4.1 Restrictions on BIG. BIG shall not, shall ensure that its Affiliates do not, and shall contractually require that its and their Sublicensees and distributors do not, distribute, market, promote, offer for sale or sell any Licensed Product or Option Product (after Option Exercise Effective Date) directly or indirectly (a) to any Person for use in the Stoke Territory, or (b) to any Person in the BIG Territory that BIG or any of its Affiliates or any of its or their Sublicensees or distributors knows is likely to distribute, market, promote, offer for sale or sell such Licensed Product or Option Product for use in the Stoke Territory or assist another Person to do so.

7.4.2 Restrictions on Stoke. Stoke shall not, shall ensure that its Affiliates do not, and shall contractually require that its and their (sub)licensees and distributors do not, distribute, market, promote, offer for sale or sell any Licensed Product or Option Product (after Option Exercise Effective Date) directly or indirectly (a) to any Person for use in the BIG Territory, or (b) to any Person in the Stoke Territory that Stoke or any of its Affiliates or any of its or their (sub)licensees or distributors knows is likely to distribute, market, promote, offer for sale or sell such Licensed Product or Option Product for use in the BIG Territory or assist another Person to do so.

7.4.3 Additional Procedures. Without limiting the foregoing, to the extent permitted by applicable Law, each Party shall, in connection with marketing, selling or otherwise distributing Licensed Product or Option Product (after Option Exercise Effective Date) in its territory, require each Selling Party and any distributor or wholesaler to (a) provide a bona fide estimate of the demand for the Licensed Product or Option Product (after Option Exercise Effective Date), as applicable, in their respective country(ies) in the territory, and (b) use reasonable efforts to include in the agreement between such Selling Party, distributor or wholesaler on the one hand, and each of their customers on the other hand, provisions that would prevent such customers from exporting, marketing, selling or otherwise distributing the Licensed Product or Option Product (after Option Exercise Effective Date), as applicable, outside such Party's territory. If either Party or any of its Affiliates receives, or becomes aware of receipt by a licensee, sublicensee distributors and wholesalers of, any orders for a Licensed Product or Option Product (after Option Exercise Effective Date) from the other Party's territory, such Party shall refer such orders to the other Party. To the extent permitted by applicable Laws, the Parties shall coordinate and agree on other measures to prevent diversion of any Licensed Product and Option Product

(after Option Exercise Effective Date) in its territory.

ARTICLE 8PAYMENTS AND RECORDS

Section 8.1Upfront Payment. As partial consideration for the licenses and other rights granted by Stoke to BIG under this Agreement, BIG will pay Stoke a non-refundable, non-creditable upfront payment of One Hundred and Sixty Five Million Dollars ($165,000,000) within [***] after BIG's receipt of an invoice from Stoke for such amount on or after the Effective Date.

Section 8.2Option Exercise Fee. Promptly after BIG delivers to Stoke an Option Exercise Notice, Stoke shall issue an invoice for [***] ("**Option Exercise Fee**") to BIG and BIG shall pay to Stoke such invoiced amount within [***] after BIG's receipt thereof.

Section 8.3Milestone Payments.

8.3.1 Pre-Commercial Milestone.

(a) Subject to the terms and conditions of this Agreement, BIG shall pay to Stoke a one-time payment of [***]. For avoidance of doubt, the Pre-Commercial Milestone Event payment is payable only once with respect to the first Licensed Product to achieve such milestone event, and shall not be payable if such milestone event is achieved again by the same or a different Licensed Product. Following such first achievement, Stoke shall promptly issue an invoice to BIG for the milestone payment contemplated by this Section 8.3.1(a), and BIG shall pay such invoice within [***] of its receipt thereof.

(b) Subject to the terms and conditions of this Agreement, BIG shall pay to Stoke a one-time payment of [***]. For avoidance of doubt, the Pre-Commercial Milestone Event payment is payable only once with respect to the first Option Product to achieve such milestone event, and shall not be payable if such milestone event is achieved again by the same or a different Option Product. Following such first achievement, Stoke shall promptly issue an invoice to BIG for the milestone payment contemplated by this Section 8.3.1(b), and BIG shall pay such invoice within [***] of its receipt thereof.

(c) [***].

8.3.2 First Commercial Sale Milestones. Subject to the terms and conditions of this Agreement, BIG shall pay to Stoke the following milestone payments following the first achievement by BIG, its Affiliates or Sublicensees of the milestone events set forth in the following table (i) by a Licensed Product and (ii) after the Option Exercise Effective Date, by an Option Product:

First Commercial Sale Milestone Events	First Commercial Sale Milestone Payments
[***]	[***]
[***]	[***]

For clarity, the maximum aggregate milestone payment payable for all Licensed Products under this Section 8.3.2 is [***], and the maximum aggregate milestone payment payable for all Option Products under this Section 8.3.2 is [***].

8.3.3 Sales Milestones. Subject to the terms and conditions of this Agreement, BIG shall pay to Stoke the following one (1)-time milestone payments (each a "**Sales Milestone**") following the first achievement of the milestone events set forth in the following table:

Sales Milestone Events	Sales Milestone Payments
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

For clarity, the maximum aggregate sales milestone payments payable by BIG under this Section 8.3.3 for all Licensed Products is [***] and the maximum aggregate sales milestone payments payable by BIG under this Section 8.3.3 for all Option Products is [***]. If in a given Calendar Year, a milestone event for Licensed Products or Option Products, as applicable, in a lower row in the above table is achieved before the milestone event in the higher row(s) has been achieved by a Licensed Product or Option Product, as applicable, in a prior Calendar Year, then all the higher row(s) with lower dollar threshold shall be deemed achieved at the same time and the corresponding unpaid milestone payments shall become payable.

8.3.4 Milestone Payment Procedures. BIG shall send Stoke a written notice of the achievement of each of the milestone events set forth in Sections 8.3.1 and 8.3.2 within [***] following BIG becomes aware of such achievement. Following receipt of such notice, Stoke shall submit an invoice promptly to BIG for the amount of the corresponding milestone payment, and BIG shall pay to Stoke such invoiced amount within [***] after BIG's receipt thereof.

Section 8.4Royalties.

8.4.1 Royalty Rates. Subject to the terms and conditions of this Agreement, on a country-by-country basis, BIG shall pay to Stoke royalties on Net Sales of all Licensed Products and Option Products (after Option Exercise Effective Date) sold by BIG, its Affiliates or Sublicensees in the BIG Territory during each Calendar Quarter of the applicable Royalty Term at the following rates:

Aggregate Annual Net Sales of all Licensed Products	Royalty Rate
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

Aggregate Annual Net Sales of all Option Products	Royalty Rate
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

8.4.2 Royalty Reductions. Notwithstanding Section 8.4.1, with respect to each Licensed Product or Option Product, as applicable, in a country:

(a) upon (i) the expiration of the last-to-expire Valid Claim of Licensed Patent Rights or Option Patent Rights, as applicable, in such country that Covers [***], as applicable, in such country, and (ii) the expiration of the last-to-expire Valid Claim of the Licensed Patent Rights or Option Patent Rights, as applicable, in such country that Covers [***], as applicable, in such country, as applicable, the royalty rate set forth in Section 8.4.1 for such Licensed Product or Option Product in such country shall be reduced by [***];

(b) if a Generic Product that competes with such Licensed Product or Option Product, as applicable, is launched in such country, and the Net Sales of such Licensed Product or Option Product in a given Calendar Quarter after the launch of such Generic Product is (a) [***], then the royalty rate set forth in Section 8.4.1 for such Licensed Product or Option Product with respect to such country shall be reduced by [***] and (b) [***], then the royalty rate set forth in Section 8.4.1 for such Licensed Product or Option Product with respect to such country shall be reduced by [***];

(c) in the event that BIG obtains a license to any Third Party IP in a country in the BIG Territory pursuant to Section 8.5 that [***], BIG shall be entitled to deduct from any royalties payable under Section 8.4 for such Licensed Product or Option Product [***] actually paid to such Third Party in respect of such agreement for such Licensed Product or Option Product in such country ("**Third Party Payments**"), subject to Section 8.4.3; *provided* that credits

for deductions pursuant to this Section 8.4.2(c) that are not exhausted in any Calendar Quarter may be carried into future Calendar Quarters, subject to the preceding sentence; and

(d) if a court or a Governmental Authority requires BIG or any of its Affiliates or its or their respective Sublicensees to grant a compulsory license to a Third Party permitting such Third Party to make and sell such Licensed Product or Option Product, as applicable, in a country or other jurisdiction, then the Net Sales achieved by such Third Party in such country or other jurisdiction for such Licensed Product or Option Product for the purposes of calculating royalties under Section 8.4.1 shall be reduced by [***].

8.4.3 Mechanics of Adjustments; Floor. Any reductions set forth in Section 8.4.2 shall be applied to the royalty rate under Section 8.4.1 in the order in which the event triggering such reduction occurs; *provided* that the adjustments made pursuant to Section 8.4.2 shall not reduce by more than [***] the royalties that would otherwise be owed under Section 8.4.1. Any adjustments pursuant to Section 8.4.2 shall apply only to the relevant Licensed Product or Option Product, as applicable, in the relevant country and shall be allocated pro rata across each of the royalty tiers in the relevant Calendar Quarter.

8.4.4 Royalty Reports. Following the First Commercial Sale of any Licensed Product or Option Product (after the Option Exercise Effective Date), in any country in the BIG Territory, BIG shall furnish a written report to Stoke within [***] after the end of each Calendar Quarter showing the amounts payable to Stoke pursuant to this Section 8.4 for such Calendar Quarter, which amounts shall be converted to Dollars in accordance with Section 8.7. Each such report shall include, on a country-by-country basis for the BIG Territory, the total gross amount invoiced for each Licensed Product or Option Product sold, the Net Sales of each Licensed Product or Option Product, and the total royalties (in Dollars) payable for each Licensed Product or Option Product. Stoke shall submit an invoice to BIG promptly following receipt of such royalty report from BIG for the amount of the royalty payment, which invoiced amount shall be payable by BIG to Stoke within [***] after the receipt thereof.

Section 8.5Third Party Licenses.

(a) [***].

(b) [***].

Section 8.6Other Payment Procedures. For all amounts for which a Party (the "**Owing Party**") is obligated to reimburse or pay the other Party (the "**Owed Party**") pursuant to this Agreement for which no specific provision is made hereunder for the timing of such payment, the Owed Party shall send to the Owing Party an invoice for such amount within [***] after the Owed Party's determination that such amount is payable by the Owing Party, which invoice shall include a reference to the Section of this Agreement under which the Owed Party is requesting reimbursement or payment and be accompanied by reasonable documentation of the incurrence or accrual of the amounts to be reimbursed. Payment with respect to each such invoice shall be due within [***] after receipt by the Owing Party thereof and shall be made in accordance with Section 8.7. With respect to any invoice provided under this Agreement, if the Owing Party in good faith disputes any portion of any such invoice, it shall pay the undisputed portion and shall provide the

Owed Party with written notice of the disputed portion and its reasons therefor, and the Owing Party shall not be obligated to pay such disputed portion unless and until such dispute is resolved in favor of the Owed Party. The Parties shall use good faith efforts to resolve any such disputes promptly.

Section 8.7Mode of Payment; Foreign Exchange. All payments to a Party under this Agreement shall be made by deposit of Dollars in the requisite amount to such bank account as the receiving Party may from time to time designate by notice to the paying Party. For the purpose of calculating any sums due under, or otherwise reimbursable pursuant to, this Agreement (including the calculation of Net Sales expressed in currencies other than Dollars), a Party shall convert any amount expressed in a foreign currency into Dollar equivalent using its then-current standard exchange rate methodology employed for the translation of foreign currency sales into Dollars in accordance with Accounting Standards and consistently applied for the Calendar Quarter in which the Net Sales, or such other conversion standard as may be mutually agreed by the Parties.

Section 8.8Taxes.

8.8.1 General. Stoke hereby represents and warrants that it is (a) the legal and beneficial owner for all tax purposes of all Payments made or to be made to it hereunder, (b) established and taxable (for VAT or other indirect tax purposes) solely in the United States (excluding possessions and territories), and (c) does not maintain any permanent establishment (within the meaning of any applicable tax treaty) or other fixed place of business in any jurisdiction outside of the United States (excluding possessions and territories).

8.8.2 Withholding. Any and all payments to a Party (the "**Payee**") under this Agreement (each, a "**Payment**") shall be paid free and clear of any and all taxes, except for any withholding taxes required by applicable Law. The paying Party (the "**Payor**") will be entitled to deduct or withhold from the Payments otherwise payable under this Agreement any amounts that it is required by applicable Law to deduct or withhold. [***] If the Payee receives a refund or recognizes a credit or other cash tax savings as a result of such additional withholding taxes, the Payee shall pay to the Payor an amount equal to such refund, credit or cash tax savings (net of any taxes or reasonable out-of-pocket costs incurred as a result of such receipt or recognition). Without limiting the foregoing, if the Payee is entitled under any applicable tax treaty or other applicable Law to a reduction of the rate of, or the elimination of, applicable withholding tax, it may deliver to the Payor or the appropriate Governmental Authority (with the assistance of the Payor to the extent that this is reasonably required and requested by the Payee) the prescribed forms necessary to reduce the applicable rate of withholding or to relieve the Payor of its obligation to withhold such tax and the Payor shall apply the reduced rate of withholding or dispense with withholding, as the case may be; *provided* that the Payor has received evidence, in a form reasonably satisfactory to the Payor, of the Payee's delivery of all applicable forms (and, if necessary, its receipt of appropriate governmental authorization) at least [***] prior to the time that the Payments are due or otherwise reasonably sufficiently in advance of the due date as will permit the Payor to make such payment at such reduced rate or free of withholding; *provided*, further, that the Payor and Payee shall reasonably cooperate in regard to the identification, preparation, execution and delivery of the applicable tax forms or certificates to reduce or eliminate applicable withholding taxes to the extent permitted by applicable tax Law. If, in accordance with the foregoing, the Payor

withholds any amount, it shall pay to the Payee the balance when due, make timely payment to the proper taxing authority of the withheld amount, send to the receiving Party proof of such payment within [***] following such payment, and such withheld amounts will be treated hereunder as having been paid to the Person in respect of whom such deduction and withholding was made.

8.8.3 Value Added Tax.

(a) All Payments are exclusive of VAT. If VAT becomes payable under applicable Law in respect of any Payment under this Agreement, then the Payor will pay such VAT at the applicable rate in respect of such Payment. In such case, VAT (if any) will become due and payable upon presentation of a valid VAT invoice (or, where there is no provision in the legislation for the jurisdiction concerned that a VAT invoice is required to be issued, a written demand containing such information as is customary in that jurisdiction). All Parties agree that they will reasonably cooperate to ensure the use of any VAT exemptions, zero ratings, reduced-ratings, suspensions or other reliefs.

(b) In the event that Payments made to Stoke (or its assignee) as Payee become subject to VAT as a result of a determination by a tax authority or change in applicable tax Law after the date hereof or Stoke's (or its assignee's) becoming (or Stoke's or Stoke's assignee already being) established for VAT purposes outside of the United States after the Effective Date, then this Section 8.8.3(b) will apply notwithstanding anything to the contrary in Section 8.8.3(a). To the extent that any Payment is subject to a VAT reverse-charge mechanism under applicable Law, the Payor or its Affiliate, as applicable, will self-assess VAT and, subject to input VAT deduction of such self-assessed VAT, remit such amount to the applicable Governmental Authority. Where the prevailing legislation requires the recipient to self-account for VAT (for example, but not limited to, a reverse charge mechanism), then BIG covenants that it will correctly account for VAT in respect of the services received. Stoke (or its assignee) agrees that it will raise a tax invoice (or equivalent document) to support the charge to VAT. For the purposes of VAT, the Stoke (or its assignee) collaboration activities and rights and licenses provided by Stoke (or its assignee) under this Agreement will be considered to be taxed under Art 44 of Council Directive 2006/112/EC or any equivalent provision in the country of performance if performed outside the European Union and as such will be considered to be taxed for VAT purposes in the country of the recipient. Any supply of goods under this Agreement will be taxed in accordance with the prevailing VAT legislation. In the event that the local competent tax authority determines that additional VAT is chargeable, where Stoke (or its assignee) is the Payee, Stoke (or its assignee) in the first instance will undertake all reasonable steps to refute any such assertions by the local tax authority. Only once this process is completed may Stoke (or its assignee) raise valid tax invoices for the additional VAT liability. BIG, as the Payor, will take commercially reasonable steps to recover any such additional VAT liability from the same local tax authorities by submitting regular claims. Stoke (or its assignee) will provide all necessary assistance to facilitate the recovery of this tax. If the tax cannot be recovered, then BIG will be entitled to offset this tax against any and all future payments to Stoke (or its assignee) or, where necessary, invoice Stoke (or its assignee) directly for these amounts (and Stoke and/or its assignee shall pay to BIG such invoiced amounts). Each Party will be responsible for any penalties or interest accruing due to incorrect VAT treatment of the supplies of goods or services made by that Party or any failure to correctly account for VAT on any receipt of a supply of goods or services under this Agreement except where those penalties or interest arise as a result of the actions of the

other Party, in which case that acting Party will be liable to reimburse the other Party for such penalties and interest. Each Party will be responsible for reporting its own transactions to the local tax authorities if required under applicable Law for VAT purposes. There will be no shared, mutual or otherwise collective VAT filings that may suggest that the Parties are anything other than separately operational entities for VAT purposes.

Section 8.9Interest on Late Payments. Any undisputed amount owed by one Party to the other Party under this Agreement that is not paid on or before the date such payment is due shall bear interest at a rate per annum equal to [***] basis points above the prime rate as published by Bank of America, as adjusted from time to time, such interest to run from the date on which payment of such sum became due until payment thereof in full together with such interest.

Section 8.10Financial Records. BIG shall, and shall cause its Affiliates to, keep complete and accurate books and records pertaining to (a) Out-of-Pocket Expenses relating to its conduct of activities under each Global Development Plan, and (b) Net Sales of Licensed Products or Option Products (after the Option Exercise Effective Date) and any other amounts due hereunder in each case ((a) and (b)) in sufficient detail for Stoke to reasonably determine the accuracy of all amounts payable by BIG or reimbursable by Stoke hereunder. Stoke shall, and shall cause its Affiliates to, keep complete and accurate books and records pertaining to Out-of-Pocket Expenses relating to its conduct of activities under each Global Development Plan in sufficient detail for BIG to reasonably determine the accuracy of all amounts of Shared Global Development Costs reimbursable by BIG hereunder. Such books and records shall be retained by each Party and its Affiliates until [***] after the end of the period to which such books and records pertain, or for such longer period as may be required by applicable Law.

Section 8.11Audit.

8.11.1Procedures. Stoke may request that BIG permit and cause its Affiliates to permit an independent auditor of international recognition designated by Stoke and reasonably acceptable to BIG, at reasonable times and upon reasonable notice, to audit the books and records maintained pursuant to Section 8.10 to ensure the accuracy of all royalty reports and payments made hereunder. BIG may request that Stoke permit and cause its Affiliates to permit an independent auditor of international recognition designated by BIG and reasonably acceptable to Stoke, at reasonable times and upon reasonable notice, to audit the books and records maintained pursuant to Section 8.10 to ensure the accuracy of all Shared Global Development Costs payments made by BIG hereunder. Such examinations may not [***]. Except as provided below, the Costs of this audit shall be borne by the auditing Party, unless the audit reveals a variance of more than [***] from the reported amounts in the auditing Party's favor, in which case the audited Party shall bear the Costs of the audit. If such audit concludes that there is an undisputed underpayment or excess payment by one Party to the other Party, then within [***] after the date on which such audit is completed the Parties shall true up the payment discrepancy with interest from the date originally due as provided in 8.8.1. If the results of the audit are disputed, the audited Party will have [***] to invoke the dispute resolution provisions set forth in Section 15.5, and if the audited Party does not invoke the dispute resolution provisions set forth in Section 15.5 within such [***] period, the results of the audit will be deemed to be undisputed. The receiving Party of information subject to review under this Section 8.11 shall treat such information in accordance with the confidentiality provisions of Article 9, and the Parties shall cause the auditor to enter into a

reasonably acceptable confidentiality agreement with BIG obligating such firm to retain all such financial information in confidence pursuant to such confidentiality agreement.

Section 8.12Right to Offset. Unless prohibited by applicable Law, each Party shall have the right to offset any overdue, undisputed payment that is owed by the other Party but not paid against any payments owed by such Party under this Agreement.

Section 8.13Stoke Third Party Obligations. [***]

ARTICLE 9INTELLECTUAL PROPERTY

Section 9.1Intellectual Property Ownership.

9.1.1 Ownership of IP Arising Under This Agreement. Except as set forth in Section 9.1.3, the determination of ownership of Arising Know-How shall, for purposes of this Agreement, be made in accordance with United States patent law and other applicable Law in the United States without regard to conflicts of law, irrespective of where or when such conception, reduction to practice, generation, discovery, development, or making occurs. The Parties shall jointly own all rights, title and interest in and to all Joint IP. Each Party shall have a one-half undivided interest in all of the rights, title and interests in and to any and all Joint IP without a duty of accounting to the other Party, but subject, in all cases, to all terms and conditions of this Agreement. Each Party shall, and does hereby, assign a one-half undivided interest in all of its rights, title and interests in and to any and all Joint IP to the other Party.

9.1.2 Disclosure and Assistance Regarding Joint IP. Each Party shall promptly disclose to the other Party in writing and shall cause its Affiliates, and its and their Sublicensees to so disclose, the conception, discovery, development, or making of any Joint Know-How. Each Party will also promptly respond to reasonable requests from the other Party for additional information related thereto.

9.1.3 Exceptions. Notwithstanding Section 9.1.1, Stoke shall exclusively own all rights, title and interests in and to any and all Stoke Platform Improvement Know-How (and all intellectual property rights therein), regardless of inventorship. BIG shall (and shall cause its Affiliates and its and their Sublicensees to), and does hereby, assign all of its rights, title and interests in and to any and all Stoke Platform Improvement Know-How to Stoke. BIG shall (and shall cause its Affiliates and its and their Sublicensees to) promptly disclose to Stoke the conception, discovery, development, or making of any Stoke Platform Improvement Know-How. BIG shall (and shall cause its Affiliates and its and their Sublicensees to), promptly respond to reasonable requests from Stoke for additional information related thereto. All Stoke Platform Improvement Know-How shall be deemed the Confidential Information of Stoke.

9.1.4 Assignment Obligation. Each Party shall (and shall cause its Affiliates to) cause all Persons (including Third Parties) who perform activities for such Party or Affiliate under this Agreement to assign (or if such Party or Affiliate is unable to cause such Person to assign despite such Party's using reasonable efforts to negotiate such assignment, then provide a license under) their rights in any Arising Know-How (including all inventions resulting from such activities) to such Party. Each Party shall cause all of its Sublicensees to comply with the foregoing.

Section 9.2Maintenance and Prosecution of Patent Rights.

9.2.1 Stoke Platform Patent Rights. Stoke shall have the sole right, but not the obligation, at its sole Costs, to prepare, file, prosecute, and maintain the Stoke Platform Patent Rights anywhere in the world, and to conduct any proceeding regarding the validity and enforceability thereof, including any opposition, re-issuance, post-grant review, inter-partes review, reexamination request, nullity action, interference, or other similar post-grant proceedings and any appeals therefrom, before or in any patent office (each, a "**Defense Proceeding**") relating thereto, in each case using counsel of its choice. Stoke shall (a) reasonably consult with BIG prior to filing any Patent application that constitute Stoke Platform Patent Rights if such Patent application contains one or more claims that Cover (at the time of filing) a Licensed Product or Option Product (after the Option Exercise Effective Date), and (b) use reasonable efforts to file, at BIG's Cost and in consultation with BIG, one or more corresponding Patent applications in the BIG Territory (e.g., continuations or divisionals of such Patent application) that would be Licensed Product-Specific Patent Rights or Option Product-Specific Patent Rights, as applicable. Stoke shall keep BIG reasonably informed as to all material developments in connection with the preparation, filing, prosecution, maintenance of the Stoke Platform Patent Rights (including Defense Proceedings related thereto), and, with respect to any Stoke Platform Patent Right that Covers a Licensed Product or Option Product (after the Option Exercise Effective Date), Stoke will provide BIG with copies of all material filings or responses to be made to the Governmental Authorities with respect thereto and all other material submissions and correspondence with any Governmental Authorities regarding any such Stoke Platform Patent Rights, in each case, in sufficient time to allow for review and comment by BIG. BIG shall offer its comments or proposals, if any, promptly, and Stoke shall consider in good faith such comments and proposals.

9.2.2 Other Patent Rights.

(a) **BIG Territory**. From and after the Effective Date for the Licensed Products and the Option Exercise Effective Date for the Option Products, BIG shall have the first right, but not the obligation (subject to the next sentence), at its sole Costs, to prepare, file, prosecute and maintain all Licensed Product-Specific Patent Rights (in Stoke's name), Option Product-Specific Patent Rights (in Stoke's name), BIG Collaboration Patent Rights and Joint Patent Rights (in both Parties' names) in all countries and jurisdictions in the BIG Territory and to conduct any Defense Proceeding for such Patent Rights in the BIG Territory relating thereto, using counsel of its own choice. Reasonably promptly after the Effective Date, Stoke shall transfer to BIG (and BIG shall assume) the prosecution and maintenance of the Licensed Product-Specific Patent Rights in the BIG Territory, and promptly after the Option Exercise Effective Date, Stoke shall transfer to BIG (and BIG shall assume) the prosecution and maintenance of the Option Product-Specific Patent Rights in the BIG Territory. BIG shall notify Stoke of all material developments and all steps to be taken in connection with the preparation, filing, prosecution, maintenance of all such Patent Rights (including Defense Proceedings related thereto) and provide Stoke with copies of all material filings or responses to be made to the Governmental Authorities with respect thereto and all other material submissions and correspondence with any Governmental Authorities regarding any such Patent Rights, in each case, in sufficient time to allow for review and comment by Stoke. Stoke shall offer its comments or proposals, if any, promptly, and BIG shall consider in good faith such comments and proposals and shall implement any such comments or proposals in good faith, taking into consideration Stoke's global patent strategy for the

applicable Licensed Product or Option Product, *provided* that in the event any Patent Rights owned or controlled by Stoke are cited against any Licensed Product-Specific Patent Right or Option Product-Specific Patent Right (after the Option Exercise Effective Date) in any communication from any Governmental Authority in the BIG Territory, then BIG shall implement any comments from Stoke in any related filings or responses to be made to such Governmental Authorities. If BIG elects not to prepare, file, prosecute or maintain, or not to initiate or continue any Defense Proceeding related to, any Licensed Product-Specific Patent Rights, Option Product-Specific Patent Rights, BIG Collaboration Patent Rights or Joint Patent Rights in a particular country or other jurisdiction in the BIG Territory, BIG shall timely notify Stoke (in each case at least [***] in advance of any lapse of rights), and, at Stoke's request, the Parties shall promptly discuss such election by BIG. Subject to BIG's prior written consent with respect to the BIG Collaboration Patent Rights or Joint Patent Rights, such consent not to be unreasonably withheld, delayed or conditioned, (for clarity, BIG's prior written consent shall not be required for any Licensed Product-Specific Patent Rights or Option Product-Specific Patent Rights), Stoke may elect, upon written notice to BIG, to make such payment or take or continue such action, at Stoke's Costs using counsel of its own choice and BIG shall reasonably cooperate with Stoke in connection with such activities. If Stoke elects to prepare, file, prosecute or maintain any such Licensed Product-Specific Patent Rights, Option Product-Specific Patent Rights, BIG Collaboration Patent Rights or Joint Patent Rights in a particular country or other jurisdiction in the BIG Territory (including Defense Proceedings related thereto), then Stoke shall notify BIG of all material developments and all steps to be taken in connection with the prosecution of such Patent Rights (including Defense Proceedings related thereto) and provide BIG with copies of all material filings or responses to be made to Governmental Authorities with respect thereto and all other material submissions and correspondence with any patent authorities regarding any such Patent Rights, in each case, in sufficient time to allow for review and comment by BIG. BIG shall offer its comments or proposals, if any, promptly, and Stoke shall consider in good faith such comments and proposals and shall implement any such comments or proposals in good faith, taking into consideration BIG's patent strategy for the applicable Licensed Product or Option Product in the BIG Territory.

(b) **Stoke Territory**. Stoke shall have the sole right, but not the obligation, at its sole Costs, to prepare, file, prosecute, and maintain all Licensed Product-Specific Patent Rights and Option Product-Specific Patent Rights in all countries and jurisdictions in the Stoke Territory, and to conduct any Defense Proceeding relating thereto, in each case using counsel of its choice. With respect to BIG Collaboration Patent Rights and Joint Patent Rights, Stoke shall have the first right, but not the obligation, at its sole Costs, to prepare, file, prosecute, and maintain such Patent Rights in all countries and jurisdictions in the Stoke Territory, and to conduct any Defense Proceeding relating thereto, in either case, using counsel of its choice. Stoke shall reasonably notify BIG of all material developments and all steps to be taken in connection with the preparation, filing, prosecution and maintenance of all such Patent Rights (including Defense Proceedings related thereto) for which it has responsibility pursuant to the foregoing to the extent such development or steps is reasonably likely to materially impact the preparation, filing, prosecution, maintenance of any Patent Rights (including Defense Proceedings related thereto) Controlled by BIG (including licensed to BIG by Stoke hereunder) in the BIG Territory. If Stoke elects not to prepare, file, prosecute or maintain, or not to initiate or continue any Defense Proceeding related to, any BIG Collaboration Patent Rights or Joint Patent Rights in a particular country or other jurisdiction in the Stoke Territory, Stoke shall timely notify BIG, and BIG may elect, upon written notice to Stoke, to make such payment or continue such action with respect to

such BIG Collaboration Patent Rights or Joint Patent Rights, at BIG's Costs using counsel of its own choice and Stoke shall reasonably cooperate with BIG in connection with such activities. If BIG elects to prepare, file, prosecute or maintain any such BIG Collaboration Patent Rights or Joint Patent Rights in a particular country or other jurisdiction in the Stoke Territory (including Defense Proceedings related thereto), then BIG shall notify Stoke of all material developments and all steps to be taken in connection with the prosecution of such Patent Rights (including Defense Proceedings related thereto) and provide Stoke with copies of all material filings or responses to be made to Governmental Authorities with respect thereto and all other material submissions and correspondence with any patent authorities regarding any such Patent Rights, in each case, in sufficient time to allow for review and comment by Stoke. Stoke shall offer its comments or proposals, if any, promptly, and BIG shall consider in good faith such comments and proposals.

9.2.3 Settlement of Defense Proceedings. Neither Party may settle any Defense Proceeding without the other Party's written consent (such consent not to be unreasonably withheld, conditioned or delayed) if such settlement (a) is reasonably likely to diminish or have a material adverse effect on the rights or interest of the other Party, including any admission of fault, payment of damages or implementation of injunctive relief, or (b) admits the invalidity or unenforceability of, or otherwise impairs or materially adversely affects the scope of Patent Rights owned or controlled by the other Party in the other Party's territory.

9.2.4 Patent Term Extensions. To the extent applicable, the Parties will develop a strategy in order to avoid the loss of any rights that may otherwise be available to the Parties with respect to the Licensed Products or Option Products (after the Option Exercise Effective Date) under the Hatch-Waxman Act, the Supplementary Certificate of Protection of the member states of the European Union and other similar measures in any other country or jurisdiction with respect to patent term extensions, adjustments or restorations (any such right, a "**Patent Term Extension**"). Each Party shall cooperate in good faith with the other to implement such strategy. The Parties will discuss and decide which, if any, of the Licensed Product-Specific Patent Rights, Option Product-Specific Patent Rights, BIG Collaboration Patent Rights and Joint Patent Rights the Parties should seek Patent Term Extensions for with respect to any country or jurisdiction in each Party's territory (and a Party will be responsible for the costs of any such Patent Term Extension in its territory) in connection with a Licensed Product or Option Product (after the Option Exercise Effective Date), as applicable, *provided* that (a) Stoke shall have the final decision-making authority with respect to any Patent Term Extension for any such Patent Rights in the Stoke Territory, and (b) BIG shall have the final decision-making authority with respect to any such Patent Rights in the BIG Territory. The Parties shall cooperate with each other to the extent necessary to effectuate the intent of this Section 9.2.4.

9.2.5 Orange Book Listings. To the extent applicable, the Parties will develop a strategy as to which Licensed Product-Specific Patent Rights, Option Product-Specific Patent Rights, BIG Collaboration Patent Rights and Joint Patent Rights will be listed in the FDA's "Orange Book" and equivalents thereto in countries or jurisdictions outside the U.S. (and each Party will be responsible for the costs of any such listings in its territory) in connection with the Licensed Products and Option Products (after the Option Exercise Effective Date), *provided* that(a) Stoke shall have the final decision-making authority with respect to listing any such Patent Rights in the Stoke Territory, and (b) BIG shall have the final decision-making authority with respect to

listing any such Patent Rights in the BIG Territory. The Parties shall cooperate with each other to the extent necessary to effectuate the intent of this Section 9.2.5.

9.2.6 Common Interest Disclosures. With regard to any information or opinions disclosed pursuant to this Agreement by one Party to the other Party regarding intellectual property or technology owned by Third Parties, the Parties agree that they have a common legal interest in determining whether, and to what extent, Third Party intellectual property rights may affect the conduct of the Development, Manufacture or Commercialization of a Licensed Product or Option Product (after the Option Exercise Effective Date), and have a further common legal interest in defending against any actual or prospective Third Party claims based on allegations of misuse or infringement of intellectual property rights relating thereto. Accordingly, the Parties agree that all such information and materials obtained by a Party from the other Party will be used solely for purposes of the Parties' common legal interests with respect to the conduct of the Agreement. All information and materials will be treated as protected by the attorney-client privilege, the work product privilege, and any other privilege or immunity that may otherwise be applicable. By sharing any such information and materials, neither Party waives or limits any privilege or immunity that may apply to the shared information and materials. Neither Party shall have the authority to waive any privilege or immunity on behalf of the other Party without such other Party's prior written consent, nor shall the waiver of privilege or immunity resulting from the conduct of one Party be deemed to apply against the other Party.

9.2.7 Cooperation. The Parties recognize that they will prepare, file, prosecute, maintain, and conduct Defense Proceedings in countries and regions in their respective territories with respect to the same families of Patent Rights. Each Party acknowledges that the actions they take or fail to take, and the statements that they make to a Governmental Authority with respect to a Patent Right in its territory could adversely impact the patentability, scope, validity or enforceability of a Patent Right in the same patent family in the other Party's territory. As such, the Parties agree to cooperate in good faith to coordinate their strategies and tactics with respect to the Patent Rights for which they have prosecution responsibility. Further, Stoke agrees that it will not take or fail to take any actions in the Stoke Territory that, in BIG's reasonable judgment, would be expected to adversely impact the patentability, scope, validity or enforceability of the BIG Collaboration Patent Rights or Joint Patent Rights in the BIG territory; and, BIG agrees that it will not take or fail to take any actions in the BIG Territory that, in Stoke's reasonable judgment, would be expected to adversely impact the patentability, scope, validity or enforceability of the Licensed Patent Rights or Option Patent Rights in the Stoke Territory.

Section 9.3Enforcement.

9.3.1 Notice. Each Party shall promptly notify the other Party in writing if it becomes aware of any actual, alleged, threatened or suspected infringement of one or more granted claim(s) of the (a) Stoke Platform Patent Rights, Licensed Product-Specific Patent Rights, Option Product-Specific Patent Rights, BIG Collaboration Patent Rights or Joint Patent Rights, or any equivalent action (such as a declaratory judgment) alleging the invalidity, unenforceability or non-infringement of any such Patent Rights, or misappropriation or other violation of any Licensed Product-Specific Know-How, Option Product-Specific Know-How, BIG Collaboration Know-How or Joint Know-How, in each case in any country or jurisdiction in the BIG Territory, or (b) BIG Collaboration Patent Rights or Joint Patent Rights, or any equivalent action (such as a

declaratory judgment) alleging the invalidity, unenforceability or non-infringement of any such Patent Rights, or misappropriation or other violation of any BIG Collaboration Know-How or Joint Know-How, in each case in any country or jurisdiction in the Stoke Territory (each an "**Infringement**").

9.3.2 Stoke Platform Patent Rights. Stoke shall have the first right, but not the obligation, to pursue any Infringement claims against Third Parties with respect to Stoke Platform Patent Rights in the BIG Territory, at its sole expense. Stoke shall: (a) keep BIG fully informed of any material development in such claim, suit, or proceeding in the BIG Territory; (b) reasonably consider BIG's comments on such claim, suit, or proceeding in the BIG Territory; and (c) when applicable, allow BIG the opportunity to participate in the preparation of witnesses and other participants in such claim, suit, or proceeding in the BIG Territory. If Stoke decides not to take steps to abate or pursue an Infringement claim with respect to any Stoke Platform Patent Right in the BIG Territory within a commercially reasonable period of time (but not less than [***]) after the notice provided pursuant to Section 9.3.1, and such Infringement claim adversely affects or is expected to adversely affect the Exploitation of any Licensed Product or Option Product (after the Option Exercise Effective Date) in the Field in the BIG Territory (a "**Competitive Infringement**"), then Stoke shall timely inform BIG and BIG may pursue such Competitive Infringement claim against Third Parties with respect to Stoke Platform Patent Rights in the BIG Territory at its own expense (and in such case, Stoke may be represented in any such action by counsel of its own choice with respect to such action), *provided* that if Stoke provides a reasonable, objective rationale for not pursuing or continuing to pursue such Competitive Infringement claim (including a substantive concern regarding counter-claims by the infringing Third Party with respect to Patent Rights owned or Controlled by Stoke that relate to TANGO), then the Parties shall consider and discuss in good faith Stoke's reasonable comments and concerns and BIG shall not have the right to pursue such Competitive Infringement claim in the BIG Territory without Stoke's consent, such consent not to be unreasonably withheld, conditioned or delayed. Subject to the foregoing, if BIG elects to pursue such Competitive Infringement claim in the BIG Territory, BIG shall: (a) keep Stoke fully informed of any material developments in such claim, suit, or proceeding in the BIG Territory; (b) incorporate Stoke's reasonable comments on such claim, suit, or proceeding in the BIG Territory in good faith; and (c) when applicable, allow Stoke the opportunity to participate in the preparation of witnesses and other participants in such claim, suit, or proceeding; and (d) not settle any such claim, suit, or proceeding in the BIG Territory without Stoke's written consent (such consent not to be unreasonably withheld, conditioned or delayed) if such settlement is reasonably likely to materially diminish or have a material adverse effect on the rights or interest of Stoke.

9.3.3 Other Patent Rights. Subject to Section 9.3.4, BIG shall have the first right, but not the obligation, to pursue Infringement claims against Third Parties in the BIG Territory with respect to Licensed Product-Specific Patent Rights, Option Product-Specific Patent Rights, BIG Collaboration Patent Rights and Joint Patent Rights (for clarity, excluding any Stoke Platform Patent Rights), including subject to Section 9.4.2 as a defense or counterclaim or other related invalidity proceeding in connection with any Third Party Infringement Claim in the BIG Territory, at its sole expense. BIG shall retain control of the applicable claim, suit or proceeding with respect to such Infringement. If BIG pursues any such Infringement claim in accordance with this Section 9.3.3, then Stoke shall have the right to join as a party to such claim, suit, or proceeding in the BIG Territory and participate with its own counsel at its own expense; *provided* that BIG

shall retain control of such claim, suit, or proceeding. BIG shall: (a) keep Stoke reasonably informed of any material development in such claim, suit, or proceeding in the BIG Territory; (b) reasonably consider taking action to incorporate Stoke's comments on such claim, suit, or proceeding in the BIG Territory; (c) when applicable, allow Stoke the opportunity to participate in the preparation of witnesses and other participants in such claim, suit, or proceeding; and (d) not settle any such claim, suit, or proceeding except in a manner that it believes in good faith is in the best interests of the applicable Licensed Product or Option Product and that is consistent with Section 9.3.7. If BIG decides not to pursue an Infringement claim with respect to any Licensed Product-Specific Patent Right or Option Product-Specific Patent Right, as applicable, in the BIG Territory, BIG shall timely inform Stoke and Stoke may pursue such Infringement claim in the BIG Territory at its own expense, *provided* that if BIG provides a reasonable, objective rationale for not pursuing or continuing to pursue such Infringement claim (including a substantive concern regarding counter-claims by the infringing Third Party with respect to Licensed Product-Specific Patent Rights, Option Product-Specific Patent Rights, BIG Collaboration Patent Rights and Joint Patent Rights in the BIG Territory), then the Parties shall consider and discuss in good faith BIG's reasonable comments and concerns and Stoke shall not have the right to pursue such Infringement claim in the BIG Territory without BIG's consent, such consent not to be unreasonably withheld, conditioned or delayed. If Stoke elects to pursue such Infringement claim in the BIG Territory, Stoke shall: (a) keep BIG fully informed of any material development in such claim, suit, or proceeding in the BIG Territory; (b) reasonably consider taking action to incorporate BIG's comments on such claim, suit, or proceeding in the BIG Territory; and (c) when applicable, allow BIG the opportunity to participate in the preparation of witnesses and other participants in such claim, suit, or proceeding; and (d) not settle any such claim, suit, or proceeding in the BIG Territory without BIG's written consent (such consent not to be unreasonably withheld, conditioned or delayed) if such settlement is reasonably likely to materially diminish or have a material adverse effect on the rights or interest of BIG.

9.3.4 Generic Competition. BIG shall have the first right, but not the obligation, to pursue, manage and settle any litigation with respect to Generic Products (as used in this Section 9.3.4, Generic Product includes a generic product for which a generic or abbreviated Regulatory Approval application referencing a Licensed Product or Option Product (after the Option Exercise Effective Date) has been or is expected to be filed but has not been filed or approved) in the BIG Territory in connection with any Licensed Product-Specific Patent Rights or Option Product-Specific Patent Rights and any proceedings associated therewith in the BIG Territory, including any invalidity, unpatentability or unenforceability challenges, oppositions and post-grant proceedings in connection therewith in the BIG Territory. Stoke shall reasonably cooperate in any such action, at BIG's cost. If Stoke (a) reasonably believes that a Third Party may be filing or preparing or seeking to file a generic or abbreviated Regulatory Approval application in the BIG Territory that refers or relies on Regulatory Filings submitted by either Party to any Regulatory Authority, whether or not such filing may infringe a granted Valid Claim of Licensed Product-Specific Patent Rights or Option Product-Specific Patent Rights or (b) receives any notice of certification or similar in the BIG Territory regarding the Licensed Product-Specific Patent Rights or Option Product-Specific Patent Rights pursuant to applicable Laws claiming that any such Patent Rights are invalid or unenforceable or claiming that any such Patent Rights will not be infringed by the Manufacture or Commercialization of a Generic Product, it shall (i) immediately notify BIG in writing identifying the alleged applicant or potential applicant and furnishing the information upon which determination is based and (ii) provide BIG with a copy of any such notice

of certification or similar within [***] after the date of receipt thereof. Notwithstanding anything to the contrary in this Section 9.3.4, if, following good faith consultation between the Parties, the Parties reasonably believe a Stoke Platform Patent Right is the only type of Licensed Patent Right or Option Patent Right that a Generic Product in the BIG Territory is believed to infringe, then the procedures of Section 9.3.2 shall apply with respect to the enforcement of such Stoke Platform Patent Right against the Person(s) that own or control such Generic Product, *provided* that the time period referenced therein for Stoke to pursue an Infringement claim shall be shortened to [***] or such lesser period of time so as to avoid a loss of rights under applicable Law.

9.3.5 Stoke Territory. Stoke shall have the first right, but not the obligation, to pursue Infringement claims against Third Parties in the Stoke Territory with respect to BIG Collaboration Patent Rights and Joint Patent Rights, at its sole expense. Stoke shall retain control of the applicable claim, suit or proceeding with respect to such Infringement. If Stoke pursues any such Infringement claim in accordance with this Section 9.3.5, then BIG shall have the right to join as a party to such claim, suit, or proceeding in the Stoke Territory and participate with its own counsel at its own expense; *provided* that Stoke shall retain control of such claim, suit, or proceeding. Stoke shall: (a) keep BIG reasonably informed of any material development in such claim, suit, or proceeding in the Stoke Territory; (b) reasonably consider taking action to incorporate BIG's comments on such claim, suit, or proceeding in the Stoke Territory; (c) when applicable, allow BIG the opportunity to participate in the preparation of witnesses and other participants in such claim, suit, or proceeding; and (d) not settle any such claim, suit, or proceeding except in a manner that it believes in good faith is in the best interests of the applicable Licensed Product or Option Product and that is consistent with Section 9.3.7. If Stoke decides not to pursue an Infringement claim with respect to any BIG Collaboration Patent Right or Joint Patent Right in the Stoke Territory, Stoke shall timely inform BIG and BIG may pursue such Infringement claim in the Stoke Territory at its own expense, *provided* that if Stoke provides a reasonable, objective rationale for not pursuing or continuing to pursue such Infringement claim (including a substantive concern regarding counter-claims by the infringing Third Party with respect to Licensed Product-Specific Patent Rights, Option Product-Specific Patent Rights, BIG Collaboration Patent Rights and Joint Patent Rights in the Stoke Territory), then the Parties shall consider and discuss in good faith Stoke's reasonable comments and concerns and BIG shall not have the right to pursue such Infringement claim in the Stoke Territory without Stoke's consent, such consent not to be unreasonably withheld, conditioned or delayed. If BIG elects to pursue such Infringement claim in the Stoke Territory, BIG shall: (a) keep Stoke fully informed of any material development in such claim, suit, or proceeding in the Stoke Territory; (b) reasonably consider taking action to incorporate Stoke's comments on such claim, suit, or proceeding in the Stoke Territory; and (c) when applicable, allow Stoke the opportunity to participate in the preparation of witnesses and other participants in such claim, suit, or proceeding; and (d) not settle any such claim, suit, or proceeding in the Stoke Territory without Stoke's written consent (such consent not to be unreasonably withheld, conditioned or delayed) if such settlement is reasonably likely to materially diminish or have a material adverse effect on the rights or interest of Stoke.

9.3.6 Recovery. Except as otherwise agreed by the Parties in connection with a cost sharing arrangement, any recovery realized as a result of Infringement litigation described above in this Section 9.3 shall be first allocated to reimburse the Parties for their Costs incurred in connection with such Infringement litigation. Any remainder after such reimbursement is made shall be [***].

9.3.7 Cooperation. The Parties agree to cooperate reasonably in any Infringement action pursuant to this Section 9.3. If a Party brings such an action, then the other Party shall, if necessary, join in, or be named as a necessary party to, such action at the enforcing Party's cost. The enforcing Party shall have the right to settle such claim; *provided* that neither Party shall have the right to settle any action under this Section 9.3 in a manner that imposes any costs or liability on, or involves any admission by, the other Party, without the express written consent of such other Party. Additionally, the Parties recognize that in an action pursuant to this Section 9.3, they may enforce Patent Rights in countries and regions in their respective territories with respect to the same families of Patent Rights. Each Party acknowledges that the actions they take or fail to take, and the statements that they make in an action pursuant to this Section 9.3 with respect to a Patent Right in its territory could adversely impact the patentability, scope, validity or enforceability of a Patent Right in the other Party's territory. As such, the Parties agree to cooperate in good faith to coordinate their enforcement strategies and tactics with respect to the Patent Rights for which they have enforcement rights under this Agreement. Further, Stoke agrees that it will not take or fail to take any actions with respect to the Patent Rights for which it has enforcement rights under this Agreement that, in BIG's reasonable judgment, would be expected to, and to minimize any potential adverse impact of their actions or inactions and statements, on the patentability, scope, validity or enforceability of the Patent Rights in the same or related patent families in the BIG Territory; and, BIG agrees that it will not take or fail to take any actions with respect to the Patent Rights for which it has enforcement rights under this Agreement that, in Stoke's reasonable judgment, would be expected to, and to minimize any potential adverse impact of their actions or inactions and statements, on the patentability, scope, validity or enforceability of the Patent Rights in the same or related patent families in the Stoke Territory. For clarity, the foregoing cooperation obligations shall not apply to an Infringement action pursuant to Section 9.3.2 that is not a Competitive Infringement.

Section 9.4Infringement Claims by Third Parties.

9.4.1 Notice. If the Exploitation of a Licensed Product or Option Product (after the Option Exercise Effective Date) in the BIG Territory pursuant to this Agreement results in, or may result in, any claim, suit or proceeding by a Third Party alleging patent infringement by BIG (or its Affiliates or Sublicensees) (a "**Third Party Infringement Claim**"), the Party first becoming aware of such Third Party Infringement Claim shall promptly notify the other Party thereof in writing.

9.4.2 Control. Unless BIG seeks indemnification for such Third Party Infringement Claim pursuant to Article 11 (*provided*, *however*, that in all instances Stoke shall control a Third Party Claim of Section 11.1(c)), BIG shall have the sole right, but not the obligation, to defend and control the defense and settlement of any Third Party Infringement Claim at its own expense (but subject to deduction as provided below), using counsel of its own choice. Stoke may participate in any such Third Party Infringement Claim with counsel of its choice at its own expense whether or not it is a named defendant. BIG shall keep Stoke reasonably informed of all material developments in connection with any Third Party Infringement Claim. BIG shall have the right to settle any Third Party Infringement Claim in its reasonable discretion; *provided* that BIG shall not have the right to settle any Third Party Infringement Claim in a manner that imposes any costs or liability on, or involves any admission by, Stoke, without the express written consent of Stoke. Notwithstanding anything to the contrary herein, BIG shall not have the right to

enforce, defend, license, or otherwise utilize any Stoke Platform Patent Right, Licensed Patent Right or Option Patent Right in such defense or settlement of a Third Party Infringement Claim without Stoke's prior written consent. If Stoke is named as a defendant in a Third Party Infringement Claim, BIG agrees to allow Stoke reasonable opportunity to participate in the defense of the Third Party Infringement Claim.

9.4.3 Recovery. Any recoveries by a Party of any sanctions awarded to such Party and against a party asserting a claim being defended under this Section 9.4 shall be applied as follows: such recovery shall be applied first to (a) reimburse each Party for its Costs incurred in connection with defending such Third Party Infringement Claim and (b) the balance of any such recoveries shall be [***].

Section 9.5Product Trademarks. As between the Parties, each Party shall have the sole right to determine, develop, prosecute, use, grant licenses or other rights to, enforce and defend, and shall own all right, title and interest in and to and all goodwill associated with, the Trademarks that are used in connection with the Development or Commercialization of the Licensed Products and Option Products in such Party's territory. The Parties shall discuss through the JSC (or JCC, if applicable) their selection of the Trademark(s) to be used for the Licensed Products and Option Products in their respective territories in advance of such use and consider the other Party's comments in good faith. Further, the Parties may mutually agree to coordinate their use of a single Trademark for a Licensed Product or Option Product. Each Party shall not, and shall cause its Affiliates and Sublicensees not to, (a) subject to the foregoing sentence, use any Trademark that is confusingly similar to, misleading or deceptive with respect to or that dilutes any (or any part) of the Trademarks owned by the other Party pursuant to this Section 9.5 or (b) do any act that endangers, destroys or similarly affects, in any material respect, the value of the goodwill pertaining to the Trademarks owned by the other Party pursuant to this Section 9.5.

Section 9.6International Nonproprietary Name. The Parties shall cooperate in good faith to select an International Nonproprietary Name or other name or identifier for each Licensed Product and Option Product. Stoke shall have the sole responsibility to apply for submission to the World Health Organization for the International Nonproprietary Name, and submission to the United States Adopted Names Council for the United States Adopted Name.

ARTICLE 10REPRESENTATIONS, WARRANTIES AND COVENANTS

Section 10.1Mutual Representations and Warranties. Each Party represents and warrants to the other Party as of the Effective Date that:

10.1.1 It is duly organized, validly existing and in good standing under the laws of the jurisdiction of its organization and has all requisite power and authority, corporate or otherwise, to execute, deliver and perform this Agreement.

10.1.2 The execution and delivery of this Agreement and the performance by it of the transactions contemplated hereby have been duly authorized by all necessary corporate action and do not violate: (a) such Party's charter documents, bylaws or other organizational documents; (b) in any material respect, any agreement, instrument or contractual obligation to which such Party is bound; (c) any requirement of any applicable Law; or (d) any order, writ, judgment,

injunction, decree, determination or award of any court or governmental agency presently in effect applicable to such Party.

10.1.3 This Agreement is a legal, valid and binding obligation of such Party enforceable against it in accordance with its terms and conditions, subject to the effects of bankruptcy, insolvency or other laws of general application affecting the enforcement of creditor rights, judicial principles affecting the availability of specific performance and general principles of equity (whether enforceability is considered a proceeding at law or in equity).

10.1.4 It is not under any obligation, contractual or otherwise, to any Person that conflicts with or is inconsistent in any material respect with the terms of this Agreement or that would impede the diligent and complete fulfillment of its obligations hereunder.

Section 10.2Additional Representations, Warranties and Covenants of Stoke. Stoke additionally represents, warrants and covenants to BIG as of (a) the Effective Date, except as set forth in the disclosure schedules delivered by Stoke on the Effective Date (the "**Initial Disclosure Schedule**") and (b) the Bring-Down Date with respect to the Option Products only, except as set forth in the disclosure schedules delivered by Stoke on the Bring-Down Date (the "**Bring-Down Disclosure Schedule**"):

10.2.1 All Licensed Patent Rights and Option Patent Rights existing in the BIG Territory as of the Effective Date and Bring-Down Date, as applicable, are listed completely and accurately on Exhibit 10.2.1 (the "**Existing Patent Rights**") and, except for Existing Patent Rights in-licensed by Stoke under the Existing Agreement, Stoke or its Affiliates are the sole and exclusive owner of the entire right, title and interest in the Existing Patent Rights free of any encumbrance, lien or claim of ownership by any Third Party. All Existing Patent Rights (a) are properly assigned to Stoke or the applicable licensor under an Existing Agreement; (b) inventorship has been properly identified; (c) are subsisting and are not invalid or unenforceable, in whole or in part, (d) are being diligently prosecuted in the respective patent offices in the BIG Territory in accordance with applicable Law and (e) have been filed and maintained properly and correctly and all applicable fees have been paid on or before the due date for payment. To Stoke's knowledge, (i) all relevant references, documents, and information have been provided to the relevant patent examiner at the relevant patent offices in connection with the prosecution of the Existing Patent Rights, and (ii) there are no prior art references or prior use or other information that, in Stoke's reasonable opinion, would render any pending Patent application within the Existing Patent Rights not patentable, or invalid or unenforceable if issued. Each of the Existing Patent Rights properly identifies each and every inventor of the claims thereof as determined in accordance with the Laws of the jurisdiction in which such Existing Patent Right is issued, or such application is pending.

10.2.2 The Existing Patent Rights represent all Patent Rights within Stoke's or its Affiliates' ownership or Control relating to the Licensed Products and Option Products, or the Exploitation thereof, as of the Effective Date or Bring-Down Date, as applicable.

10.2.3 There are no claims, judgments, or settlements against, or amounts with respect thereto, owed by Stoke or any of its Affiliates relating to the Existing Patent Rights or the existing Licensed Know-How or Option Know-How. No claim or litigation has been brought or

threatened by any Person alleging, and Stoke has no knowledge of any claim, whether or not asserted, that (a) the Existing Patent Rights are invalid or unenforceable or (b) the Existing Patent Rights or the existing Licensed Know-How or Option Know-How, or the disclosing, copying, making, assigning, or licensing of the Existing Patent Rights or the existing Licensed Know-How or Option Know-How, or the Exploitation of the Licensed Products or Option Products as contemplated herein, does or will violate, infringe, misappropriate, or otherwise conflict or interfere with, any Patent Right or other Intellectual Property Right of any Person (including trade secrets). There are no pending, alleged or, to the knowledge of Stoke, threatened (i) inter partes reviews, post-grant reviews, interferences, re-examinations or oppositions involving the Existing Patent Rights that are in or before any patent authority (or other Governmental Authority performing similar functions) or (ii) inventorship challenges involving the Existing Patent Rights that are in or before any patent or other Governmental Authority.

10.2.4 To Stoke's knowledge, no Person (a) has infringed or is infringing or threatening to infringe any Existing Patent Right or (b) has misappropriated or is misappropriating or threatening to misappropriate the existing Licensed Know-How or Option Know-How.

10.2.5 To Stoke's knowledge, Stoke's Development and BIG's Exploitation of the Licensed Products and Option Products as contemplated herein will not violate, infringe, misappropriate or otherwise conflict or interfere with any Patent Right or other intellectual property or proprietary right of any Third Party.

10.2.6 The conception, development, and reduction to practice of the Existing Patent Rights and Licensed Know-How or Option Know-How existing as of the Effective Date or Bring-Down Date, as applicable, have not constituted or involved the misappropriation of trade secrets or other rights or property of any Person.

10.2.7 All agreements existing as of the Effective Date by and between Stoke or any of its Affiliates, on the one hand, and one (1) or more Third Parties, on the other hand, pursuant to which Stoke Controls any material (including all exclusively in-licensed) Licensed IP existing in the BIG Territory are listed on Exhibit 10.2.7 (such listed agreement the "**Existing Agreement**"). The rights and obligations of the Parties hereunder are consistent with the Existing Agreements. None of Stoke or its Affiliates (a) is in breach of any Existing Agreement or (b) has received any written notice of breach or termination under any Existing Agreement from the counterparty thereto. To the knowledge of Stoke, (x) no facts or circumstances exist that would reasonably be expected to give rise to any such breach or termination and (y) no counterparty is in breach of any Existing Agreement. Stoke has provided BIG true, complete, and correct copies of all such agreements; *provided* that such copies may have been redacted with respect to financial and other sensitive terms that are not applicable to Stoke's obligations or BIG's rights or obligations hereunder.

10.2.8 To Stoke's knowledge, Stoke has all rights in and to, and Controls, all Know-How and Patent Rights necessary to Exploit the Licensed Product and Option Products as contemplated herein and as licensed to BIG hereunder, and such Know-How and Patent Rights are not subject to any other license or agreement to which Stoke or any of its Affiliates is a party (other than the Existing Agreements) that would conflict with the rights granted to BIG hereunder.

10.2.9 Neither Stoke nor any of its Affiliates has, directly or indirectly, sold, assigned, transferred, encumbered, licensed, conveyed, or otherwise disposed of any of its rights, title or interest in or to, or granted any license, option or other right to a Third Party in, to or under, any Licensed Product or Option Product or any Know-How or Patent Rights with respect thereto in the BIG Territory. Neither Stoke nor any of its Affiliates will enter into any agreement, whether written or oral, with respect to the assignment, transfer, license, conveyance, or encumbrance of, or otherwise assign, transfer, license, convey, or encumber its right, title, or interest in or to the Licensed Patent Rights, Option Patent Rights, Licensed Know-How or Option Know-How (including by granting any covenant not to sue with respect thereto) or any Patent Rights, Know-How or other Intellectual Property Right that would be Licensed Patent Rights, Option Patent Rights, Licensed Know-How or Option Know-How but for such assignment, transfer, license, conveyance, or encumbrance that is inconsistent with the rights and licenses granted to BIG under this Agreement.

10.2.10 All material existing Licensed Know-How and Option Know-How have been kept confidential or have been disclosed to Third Parties only under terms of confidentiality or in a patent filing. To the knowledge of Stoke, no breach of such confidentiality has been committed by any Third Party.

10.2.11 Each Person who has or has had any rights in or to any Licensed Patent Rights, Option Patent Rights, Licensed Know-How or Option Know-How owned or purported to be owned by Stoke has assigned and has executed an agreement assigning its entire right, title, and interest in and to such Licensed Patent Rights, Option Patent Rights, Licensed Know-How or Option Know-How to Stoke. All current and former officers, employees, agents, and consultants of Stoke or any of its Affiliates who are inventors of or have otherwise contributed (or will so contribute during the Term) in a material manner to the creation or development of any Licensed Patent Rights, Option Patent Rights, Licensed Know-How or Option Know-How owned or purported to be owned by Stoke have executed and delivered to Stoke or such Affiliate an assignment or other agreement regarding the protection of proprietary information and the assignment to Stoke or such Affiliate of any Licensed Patent Rights, Option Patent Rights, Licensed Know-How or Option Know-How, the current form of which has been made available for review by BIG. To Stoke's knowledge, no current officer, employee, agent, or consultant of Stoke or any of its Affiliates is in violation of any term of any assignment or other agreement regarding the protection of Patent Rights or other intellectual property or proprietary information of Stoke or such Affiliate or of any employment contract or any other contractual obligation relating to the relationship of any such Person with Stoke.

10.2.12 No employee of Stoke who is an inventor of any Existing Patent Right is or was employed outside of the U.S. at the time such invention was conceived or reduced to practice. To the extent applicable, each of Stoke and its Affiliates will pay all required inventor reward and remuneration to its or their employees, contractors or other Persons who perform Development activities for the Licensed Products or Option Products or who conceive, discover, develop or otherwise make any invention under or in connection with this Agreement.

10.2.13 Neither Stoke nor any of its Affiliates, nor any of its or their respective officers, employees, or agents (including (sub)licensees) has made an untrue statement of material fact or fraudulent statement to the FDA or any other Regulatory Authority with respect

to the Stoke Platform or Exploitation of any Licensed Product or Option Product, failed to disclose a material fact required to be disclosed to the FDA or any other Regulatory Authority with respect to the Stoke Platform or Exploitation of any Licensed Product or Option Product, or committed an act, made a statement, or failed to make a statement with respect to (i) the Exploitation of the Stoke Platform or (ii) the Exploitation of any Licensed Product or Option Product, in each case, that could reasonably be expected to provide a basis for the FDA to invoke its policy respecting "Fraud, Untrue Statements of Material Facts, Bribery, and Illegal Gratuities", set forth in 56 Fed. Reg. 46191 (September 10, 1991) and any amendments thereto or any analogous laws or policies in the BIG Territory.

10.2.14 Stoke and its Affiliates and its and their (sub)licensees have conducted, and their respective contractors and consultants have conducted (i) all Development of the Stoke Platform and (ii) all Exploitation of the Licensed Products and Option Products (including the generation, preparation, maintenance and retention of all regulatory documentation), in each case, that they have conducted prior to the Effective Date or Bring-Down Date, as applicable, in accordance with good laboratory practices and GCP where applicable, and all other applicable Law.

10.2.15 The inventions claimed by the Existing Patent Rights or included in the existing Licensed Know-How or Option Know-How (a) were not conceived, discovered, developed or otherwise made in connection with any research activities funded, in whole or in part, by the federal government of the United States or any agency thereof, (b) are not a "subject invention" as that term is described in 35 U.S.C. Section § 201(e), (c) are not otherwise subject to the provisions of the Patent and Trademark Law Amendments Act of 1980, as amended, codified at 35 U.S.C. §§ 200-212, as amended, as well as any regulations promulgated pursuant thereto, including in 37 C.F.R. part 401 and (d) are not the subject of any licenses, options or other rights of any other Governmental Authority or any Third Party, within or outside the United States.

10.2.16 Stoke and its Affiliate will not, and will not affirmatively assist or enable any Third Party to, assert a claim against BIG or any of its Affiliates for patent infringement in connection with the Exploitation of any Licensed Product or Option Product in the BIG Territory.

10.2.17 Stoke and its Affiliates have not ever been, are not currently, nor are they the subject of a proceeding that could lead to it becoming a Debarred Entity, Excluded Entity, or Convicted Entity and it will not use in any capacity, in connection with the obligations to be performed under this Agreement, any person who is a Debarred Individual, Excluded Individual, or a Convicted Individual.

10.2.18 Stoke and its Affiliates and its and their (sub)licensees have complied with all Anti-Corruption Laws in all material respects. Stoke and its Affiliates have in place, and undertake that they shall continue to update and maintain during the Term, an internal compliance program under which Stoke (or its Affiliates') employees and agents are required to comply with all Anti-Corruption Laws. Stoke's and its Affiliates' respective employees and agents are regularly trained on the requirements of its compliance program and compliance with applicable Anti-Corruption Laws.

10.2.19 The Processing of Personal Data by Stoke (including any transfer of Personal Data across national borders) in connection with the Licensed Products and Option Products is and has been in material compliance with Data Security and Privacy Laws in all countries and jurisdictions in the world, all privacy related consents and notices that apply to the Licensed Products and Option Products, and the requirements of any contract or codes of conduct to which Stoke is a party ("**Stoke Privacy and Security Obligations**"). Stoke has provided all necessary privacy notices related to research participants and has an appropriate legal basis under Data Security and Privacy Laws to Process all Personal Data in connection with the Licensed Products and Option Products. Stoke has developed, implemented, and maintains a compliance program, policies and procedures, and training programs to ensure ongoing compliance with the Stoke Privacy and Security Obligations. Stoke has commercially reasonable physical, technical, organizational, and administrative security measures and policies in place to protect all Personal Data collected by it or on its behalf from and against unauthorized Processing. Stoke is and has complied in all material respects with the Stoke Privacy and Security Obligations relating data breach reporting and notification obligations.

10.2.20 In the last five (5) years, Stoke has not received written notice of any alleged material violation from a Regulatory Authority or other Third Party of any Stoke Privacy and Security Obligations and has no knowledge of facts that would give rise to such a violation. Stoke is not under investigation by any Regulatory Authority for a violation of Data Security and Privacy Laws.

10.2.21 The execution, delivery, and performance of this Agreement and the other agreements and instruments contemplated hereby, and the consummation of the transactions contemplated hereunder complies with the Stoke Privacy and Security Obligations. Stoke has and will have the full right and authority to provide to BIG the Personal Data Processed by Stoke in connection with the Licensed Products and Option Products for the purposes contemplated in this Agreement. In the event the consummation of this Agreement and the transactions contemplated herein require Stoke to transfer Personal Data across national borders, Stoke shall ensure the lawful export of Personal Data, the terms of which may be outlined in a separate agreement between BIG and Stoke.

10.2.22 Stoke has made available to BIG: (a) the file wrapper and other documents and materials relating to the prosecution, defense, maintenance, validity, and enforceability of the Existing Patent Rights, (b) the Existing Agreements, and (c) existing Know-How in its possession or Control regarding or related to the Stoke Platform or Licensed Products or Option Products, including information regarding the safety and efficacy of the Licensed Products and Option Products identified or generated using the Stoke Platform, and in each case ((a), (b) and (c)), all such materials, documents, Existing Agreements and Know-How are true, complete and correct.

10.2.23 From and after the Effective Date and until the expiration or earlier termination of this Agreement in its entirety or with respect to a Licensed Product or an Option Product, as applicable, Stoke shall not, and shall cause its Affiliates not to, (a) knowingly misappropriate, infringe or use without authorization any intellectual property rights of a Third Party in connection with the performance of its activities under this Agreement, (b) enter into any agreement, whether written or oral, with respect to, or otherwise assign, transfer, license, convey

or otherwise encumber (including by granting any covenant not to sue with respect to) any Licensed Product and Option Product in a manner that is inconsistent with or otherwise diminishes the rights and licenses granted to BIG and its Affiliates hereunder (including the licenses to be granted to BIG upon exercise of an Option).

Section 10.3Additional Representations, Warranties and Covenants of BIG. BIG represents, warrants and covenants to Stoke as of the Effective Date (except as specifically stated otherwise) that:

10.3.1BIG has all rights, authorizations and consents necessary to grant all rights and licenses it purports to grant to Stoke under Section 2.1.2.

10.3.2neither BIG nor any of its Affiliates has granted any right or license to any Third Party that would conflict with or limit the scope of any of the rights or licenses granted to Stoke under Section 2.1.2.

10.3.3there are no claims, judgments, settlements, litigations, suits, actions, disputes, arbitration, judicial or legal, administrative, or other proceedings or governmental investigations pending or, to BIG's knowledge, threatened against BIG (or any of its Affiliates) which would be reasonably expected to adversely affect or restrict the ability of BIG to consummate the transactions contemplated under this Agreement or to perform its obligations under this Agreement.

10.3.4BIG and its Affiliates will not, and will not affirmatively assist or enable any Third Party to, to assert a claim against Stoke or any of its Affiliates for patent infringement in connection with the Exploitation of any Licensed Product or Option Product in the Stoke Territory.

10.3.5BIG and its Affiliates have not ever been, are not currently, nor are they the subject of a proceeding that could lead to it becoming a Debarred Entity, Excluded Entity, or Convicted Entity and it will not use in any capacity, in connection with the obligations to be performed under this Agreement, any person who is a Debarred Individual, Excluded Individual, or a Convicted Individual.

10.3.6BIG and its Affiliates have in place, and undertake that they shall continue to update and maintain during the Term an internal compliance program under which BIG (or its Affiliates') employees and agents are required to comply with all Anti-Corruption Laws. BIG's and its Affiliates' respective employees and agents are regularly trained on the requirements of its compliance program and compliance with applicable Anti-Corruption Laws.

10.3.7 The Processing of Personal Data by BIG (including any transfer of Personal Data across national borders) in connection with the Licensed Products and Option Products will be in material compliance with Data Security and Privacy Laws in all countries and jurisdictions in the world, all privacy related consents and notices that apply to the Licensed Products and Option Products, and the requirements of any contract or codes of conduct to which BIG is a party ("**BIG Privacy and Security Obligations**"). BIG will provide all necessary privacy notices related to research participants and will have an appropriate legal basis under Data Security and Privacy Laws to Process all Personal Data in connection with the Licensed Products and Option

Products. BIG has developed, implemented, and maintains a compliance program, policies and procedures, and training programs to ensure ongoing compliance with the BIG Privacy and Security Obligations. BIG has commercially reasonable physical, technical, organizational, and administrative security measures and policies in place to protect all Personal Data collected by it or on its behalf from and against unauthorized Processing. BIG will comply in all material respects with the BIG Privacy and Security Obligations relating data breach reporting and notification obligations.

10.3.8 In the last five (5) years, BIG has not received written notice of any alleged material violation from a Regulatory Authority or other Third Party of any BIG Privacy and Security Obligations and has no knowledge of facts that would give rise to such a violation. BIG is not under investigation by any Regulatory Authority for a violation of Data Security and Privacy Laws.

10.3.9BIG will have the full right and authority to provide to Stoke the Personal Data Processed by BIG in connection with the Licensed Products and Option Products for the purposes contemplated in this Agreement. In the event the consummation of this Agreement and the transactions contemplated herein require BIG to transfer Personal Data across national borders, BIG shall ensure the lawful export of Personal Data, the terms of which may be outlined in a separate agreement between Stoke and BIG.

10.3.10 From and after the Effective Date and until the expiration or earlier termination of this Agreement in its entirety or with respect to a Licensed Product or an Option Product, as applicable, BIG shall not, and shall cause its Affiliates not to, knowingly misappropriate, infringe or use without authorization any intellectual property rights of a Third Party in connection with the performance of its activities under this Agreement.

10.3.11 BIG and its Affiliates and its and their Sublicensees will comply with all Anti-Corruption Laws in all material respects. BIG and its Affiliates have in place, and undertake that they shall continue to update and maintain during the Term, an internal compliance program under which BIG (or its Affiliates') employees and agents are required to comply with all Anti-Corruption Laws. BIG's and its Affiliates' respective employees and agents are regularly trained on the requirements of its compliance program and compliance with applicable Anti-Corruption Laws.

Section 10.4Mutual Covenants. BIG covenants to Stoke that as of the Effective Date (except as specifically stated otherwise), and Stoke covenants to BIG that (i) as of the Effective Date (except as specifically stated otherwise) and (ii) as of each applicable Bring-Down Date, as applicable (except as specifically stated otherwise) that:

10.4.1 Each Party shall not, and shall cause its Affiliates not to, use in any capacity, in connection with the obligations to be performed under this Agreement, any Person who is a Debarred Individual, Excluded Individual, or a Convicted Individual. Each Party covenants that if, during the Term, it or its Affiliates become a Debarred Entity, Excluded Entity, or Convicted Entity, or listed on the FDA's Disqualified/Restricted List, or if any employee or agent performing any of its obligations hereunder becomes a Debarred Individual, Excluded Individual, or a Convicted Individual, or added to the FDA's Disqualified/Restricted List, such Party shall

promptly notify the other Party in writing and such Party shall prohibit such Person from performing work under this Agreement. If Stoke or its Affiliates become a Debarred Entity, Excluded Entity, or Convicted Entity, or listed on the FDA's Disqualified/Restricted List, BIG shall have the right to terminate this Agreement effective immediately upon written notice to Stoke.

10.4.2 Each Party shall, and shall cause its Affiliates and its and their Sublicensees to, (a) comply with all Anti-Corruption Laws and (b) not, directly or indirectly, offer, give, pay, promise to pay, or authorize the payment of any bribes, kickbacks, influence payments, or other unlawful or improper inducements to any Person in whatever form (including gifts, travel, entertainment, contributions, or anything else of value).

Section 10.5Bring-Down Disclosure Schedule. The Parties agree that any disclosure made by Stoke pursuant to the Bring-Down Disclosure Schedule shall not be deemed to amend or supplement the Initial Disclosure Schedule for purposes of the representations and warranties under Section 10.2 and the indemnification provisions Section 11.2. For clarity, an exception made by Stoke in the Bring-Down Disclosure Schedule may not cure a deficiency in the Initial Disclosure Schedule, and cannot cure a breach of any covenant or obligation of Stoke hereunder.

Section 10.6DISCLAIMER. EXCEPT AS OTHERWISE EXPRESSLY SET FORTH IN THIS ARTICLE 10, NEITHER PARTY MAKES ANY REPRESENTATIONS OR EXTENDS ANY WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING WARRANTIES OF MERCHANTABILITY, QUALITY, OR FITNESS FOR A PARTICULAR PURPOSE. NOTHING IN THIS AGREEMENT SHALL BE CONSTRUED AS A REPRESENTATION MADE OR WARRANTY GIVEN BY EITHER PARTY THAT EITHER PARTY WILL BE SUCCESSFUL IN OBTAINING ANY PATENT RIGHTS, OR THAT ANY PATENT RIGHTS WILL ISSUE BASED ON A PENDING APPLICATION. WITHOUT LIMITING THE RESPECTIVE RIGHTS AND OBLIGATIONS OF THE PARTIES EXPRESSLY SET FORTH HEREIN, EACH PARTY SPECIFICALLY DISCLAIMS ANY GUARANTEE THAT THE LICENSED PRODUCTS OR OPTION PRODUCTS WILL BE SUCCESSFUL, IN WHOLE OR IN PART.

ARTICLE 11INDEMNIFICATION

Section 11.1By Stoke. Stoke shall defend BIG, its Affiliates, and each of their respective directors, officers, employees and agents (the "**BIG Indemnified Parties**"), at Stoke's Costs, and will indemnify and hold BIG and the other BIG Indemnified Parties harmless from and against any claims, losses, costs, damages, fees or expenses (including reasonable legal fees and expenses) (collectively, "**Losses**") to the extent resulting from any claims, actions, suits or proceedings brought by a Third Party (including product liability claims) (a "**Third Party Claim**") arising out of (a) the gross negligence or willful misconduct of Stoke, its Affiliates or their respective Sublicensees in connection with its activities under this Agreement; (b) the material breach of this Agreement or the representations, warranties and covenants made hereunder by Stoke; (c) [***]; or (d) the Development, Manufacture, Commercialization or other Exploitation of a Licensed Product or Option Product by or on behalf of Stoke or its Affiliates or their respective Sublicensees prior to the Effective Date or during the Term, except, in each case ((a), (b), (c) and (d)) for (i) Losses indemnifiable by BIG in whole or in part under Section 11.2, as to which Losses each Party

shall indemnify the other Party and the BIG Indemnified Parties or Stoke Indemnified Parties, as applicable, to the extent of its respective liability for such Losses; and (ii) Losses arising from the Clinical Supply Agreement, which shall be governed solely by the Clinical Supply Agreement.

Section 11.2By BIG. BIG shall defend Stoke, its Affiliates and their respective directors, officers, employees and agents (the "**Stoke Indemnified Parties**"), at BIG's Costs, and will indemnify and hold Stoke and the other Stoke Indemnified Parties harmless from and against any Losses to the extent resulting from any Third Party Claims arising out of (a) the gross negligence or willful misconduct of BIG, its Affiliates, or their respective Sublicensees in connection with its activities under this Agreement; (b) the material breach of this Agreement or the representations, warranties and covenants made hereunder by BIG; or (c) the Development, Commercialization or other Exploitation of a Licensed Product or Option Product by or on behalf of BIG, its Affiliates, or their respective Sublicensees during the Term; except, in each case of ((a), (b), and (c)) for (i) Losses indemnifiable by Stoke in whole or in part under Section 11.1, as to which Losses each Party shall indemnify the other Party and the BIG Indemnified Parties or Stoke Indemnified Parties, as applicable, to the extent of its respective liability for such Losses; and (ii) Losses arising from the Clinical Supply Agreement, which shall be governed solely by the Clinical Supply Agreement.

Section 11.3Procedure. The foregoing indemnity obligations shall be conditioned upon (a) the indemnified Party ("**Indemnitee**") promptly notifying the indemnifying Party ("**Indemnitor**") in writing of the assertion or the commencement of the relevant Third Party Claim (*provided, however*, that any failure or delay to notify shall not excuse any obligation of the Indemnitor, except to the extent the Indemnitor is actually prejudiced thereby), (b) the Indemnitee granting the Indemnitor sole management and control, at the Indemnitor's sole expense, of the defense of such Third Party Claim and its settlement, *provided, however*, that the Indemnitor shall not settle any such Third Party Claim without the prior written consent of the Indemnitee if such settlement does not include a complete release from liability or if such settlement would involve the Indemnitee undertaking an obligation (including the payment of money by the Indemnitee), would bind or impair the Indemnitee, or includes any admission of wrongdoing by the Indemnitee or that any Intellectual Property Rights of Indemnitee or this Agreement is invalid, narrowed in scope or unenforceable, *provided, further,* that the assumption of the defense of a Third Party Claim by the Indemnitor shall not be construed as an acknowledgment that the Indemnitor is liable to indemnify the Indemnitee in respect of the Third Party Claim, nor shall it constitute a waiver by the Indemnitor of any defenses it may assert against the Indemnitee's claim for indemnification, and (c) the Indemnitee reasonably cooperating with the Indemnitor (at the Indemnitee's expense). The Indemnitee shall have the right (at its own expense) to be present in person or through counsel at all legal proceedings giving rise to the right of indemnification. Notwithstanding the foregoing, Indemnitee shall be entitled to participate in, but not control, the defense of such Third Party Claim and to engage counsel of its choice for such purpose; *provided, however*, that such engagement shall be at the Indemnitee's sole Costs unless (i) the engagement thereof has been specifically authorized in writing by the indemnifying Party in writing, (ii) Indemnitor has failed to assume the defense and engage counsel in accordance with Section 11.3 (in which case the Indemnitee shall control the defense) or (iii) the interests of the Indemnitee and Indemnitor with respect to such Third Party Claim are sufficiently adverse to prohibit the representation by the same counsel of both Parties under applicable Law, ethical rules or equitable principles. In such event, the Indemnitee shall not settle or compromise such Third Party claim without the prior written consent

of the Indemnitor, such consent not to be unreasonably withheld, conditioned or delayed. The Indemnitee shall furnish promptly to the Indemnitor copies of all papers and official documents received in respect of any Losses and Third Party Claims.

ARTICLE 12LIMITATIONS OF LIABILITY; INSURANCE

Section 12.1LIMITATION OF DAMAGES. IN NO EVENT SHALL A PARTY BE LIABLE HEREUNDER TO THE OTHER PARTY FOR ANY PUNITIVE, INDIRECT, SPECIAL, INCIDENTAL OR CONSEQUENTIAL DAMAGES (INCLUDING LOST REVENUE, LOST PROFITS, OR LOST SAVINGS) HOWEVER CAUSED AND UNDER ANY THEORY, EVEN IF IT HAS NOTICE OF THE POSSIBILITY OF SUCH DAMAGES; *PROVIDED* THAT THE LIMITATIONS SET FORTH IN THIS SECTION 12.1 SHALL NOT APPLY WITH RESPECT TO ANY BREACH OF ARTICLE 9, BREACH OF ARTICLE 13 OR FRAUD, WILLFUL MISCONDUCT OR GROSS NEGLIGENCE OF A PARTY. ADDITIONALLY, NOTHING IN THIS SECTION 12.1 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF A PARTY UNDER ARTICLE 11 WITH RESPECT TO ANY DAMAGES PAID TO A THIRD PARTY IN CONNECTION WITH A THIRD PARTY CLAIM.

Section 12.2Insurance. Each Party will, at their own respective expense procure and maintain insurance policies adequate to cover their obligations hereunder as such obligations arise during the Term and consistent with the normal business practices of prudent biopharmaceutical companies of similar size and scope. Such insurance will not create a limit to either Party's liability hereunder. Without limiting the foregoing, each Party shall, from and after such time as it commences any clinical trials of Licensed Products or Option Products during the Term, obtain and keep in force and for a period of not less than (a) three (3) years after termination or expiration of this Agreement, or (b) the date that all statutes of limitation covering claims or suits that may be instituted for personal injury based on the sale or use of the Licensed Product or Option Products have expired, commercial general liability insurance from a minimum "A-" AM Best rated insurance company, including contractual liability and product liability or clinical trials, if applicable, with coverage limits of not less than Five Million Dollars ($5,000,000) per occurrence and Ten Million Dollars ($10,000,000) in the aggregate, taking into account any applicable umbrella coverage. Such policies shall be primary and non-contributing with respect to any other similar insurance policies available to each Party or its Affiliates. Upon the other Party's request thereafter, each Party shall furnish the other with a certificate of insurance signed by an authorized representative of such Party's insurance underwriter evidencing the insurance coverage required by this Agreement and providing for at least [***] prior written notice to the other Party of any cancellation, termination or reduction of such insurance coverage. Notwithstanding any provision to the contrary in this Agreement, BIG may self-insure, in whole or in part, the insurance requirements described in this Section 12.2.

ARTICLE 13CONFIDENTIALITY

Section 13.1Confidential Information.

13.1.1Confidential Information. Each Party (the "**Receiving Party**") may receive during the course and conduct of activities under this Agreement, proprietary or

confidential information of the other Party (the "**Disclosing Party**") as furnished to the Receiving Party by or on behalf of the Disclosing Party. The term "**Confidential Information**" means all non-public information or materials of any kind, whether in written, oral, graphical, machine-readable or other form, whether or not marked as confidential or proprietary, which are transferred, disclosed or made available by Disclosing Party or at the request of Receiving Party, including any of the foregoing of Affiliates or Third Parties.

13.1.2Restrictions. Starting from the Effective Date and lasting until [***], Receiving Party will keep all Disclosing Party's Confidential Information in confidence with the same degree of care with which Receiving Party holds its own confidential information (but in no event less than a commercially reasonable degree of care). Receiving Party will not use Disclosing Party's Confidential Information except in connection with the performance of its obligations and exercise of its rights under this Agreement. Receiving Party may disclose Disclosing Party's Confidential Information without Disclosing Party's prior written consent to Receiving Party's Affiliates and their employees, subcontractors, consultants or agents who have a need to know such Confidential Information in order to perform its obligations and exercise its rights under this Agreement and who are bound by written confidentiality obligations and restrictions on use and disclosure substantially similar to this Article 13. Receiving Party will use diligent efforts to cause those entities and persons to comply with the restrictions on use and disclosure in this Section 13.1.2. Receiving Party assumes responsibility for those entities and persons maintaining Disclosing Party's Confidential Information in confidence and using same only for the purposes described herein.

13.1.3Exceptions. Receiving Party's obligation of nondisclosure and the limitations upon the right to use the Disclosing Party's Confidential Information will not apply to the extent that Receiving Party can demonstrate that the Disclosing Party's Confidential Information: (a) was known to Receiving Party or any of its Affiliates, without any obligation to keep it confidential or any restriction on its use, prior to the time of disclosure; (b) is or becomes public knowledge through no fault or omission of Receiving Party or any of its Affiliates; (c) is obtained by Receiving Party or any of its Affiliates from a Third Party lawfully in possession thereof and without any obligation to keep it confidential or restriction on its use; (d) has been independently discovered or developed by employees, subcontractors, consultants or agents of Receiving Party or any of its Affiliates without the aid, application, use or reference of Disclosing Party's Confidential Information, as evidenced by contemporaneous written records; or (e) was released from the restrictions set forth in this Agreement by express prior written consent of the Disclosing Party. Confidential Information disclosed to the Receiving Party hereunder shall not be deemed to fall within the foregoing exceptions merely because it is embraced by more general information that falls within such exceptions.

13.1.4Permitted Disclosures. Receiving Party may disclose Disclosing Party's Confidential Information to the extent (and only to the extent) such disclosure is reasonably necessary in the following instances:

(a) in order to comply with applicable Law (including any securities law or regulation or the rules of a securities exchange) or with a legal or administrative proceeding, or in connection with prosecuting or defending litigation;

(b) in connection with preparing, filing or seeking Regulatory Approvals and other Regulatory Filings for any Licensed Product or Option Product;

(c) in connection with filing, prosecuting, maintaining and enforcing Patent Rights in connection with Receiving Party's rights and obligations pursuant to this Agreement; and

(d) in connection with exercising its rights hereunder, to its Affiliates, potential and future collaborators (including Sublicensees) or independent contractors; permitted acquirers or assignees; and investment bankers, investors and lenders;

provided, however, that (i) with respect to Sections 13.1.4(a) or 13.1.4(b), unless prohibited by applicable Laws, Receiving Party will notify Disclosing Party of Receiving Party's intent to make any disclosure pursuant thereto sufficiently prior to making such disclosure so as to allow Disclosing Party adequate time to take whatever action it may deem appropriate to protect the confidentiality of the information to be disclosed; and (ii) with respect to Section 13.1.4(d), each of those named people and entities are bound by written confidentiality obligations and restrictions on use substantially similar to this Article 13, *provided* that financial terms shall not be disclosed to any such potential acquirer or investor who, at the time of such disclosure, is involved or has invested in a program related to a Competing Product**.**

Section 13.2Terms of this Agreement; Publicity.

13.2.1Restrictions. The Parties agree that the terms of this Agreement will be treated as Confidential Information of both Parties, and thus may be disclosed only as permitted by Section 13.1.4. Except as required by Law or as permitted under Section 13.1.4, each Party agrees not to issue any press release or public statement disclosing information relating to this Agreement or the transactions contemplated hereby or the terms hereof without the prior written consent of the other Party, not to be unreasonably withheld, conditioned or delayed (or as such consent may need to be obtained in accordance with Section 13.3). Notwithstanding the foregoing, a press release substantially in the form attached hereto as Exhibit 13.2.1 shall be issued by the Parties on or as promptly as practicable after the Effective Date upon coordination of the Parties. Neither Party shall be required to seek the permission of the other Party to disclose any information regarding the terms of this Agreement or any amendment hereto that has already been publicly disclosed by such Party or by the other Party, in accordance with this Section 13.2.1, *provided* that such information remains accurate as of such time and *provided* the frequency and form of such disclosure are reasonable.

13.2.2Review. Subject to Section 13.2.1, to the extent required by Law or as permitted under Section 13.1.4, if either Party (the "**Issuing Party**") desires to issue a press release (other than as set forth on Exhibit 13.2.1) or other public statement disclosing information relating to this Agreement or the transactions contemplated hereby or the terms hereof, the Issuing Party will provide the other Party (the "**Reviewing Party**") with a copy of the proposed press release or public statement (the "**Release**"). The Issuing Party will specify with each such Release, taking into account the urgency of the matter being disclosed, a reasonable period of time within which the Reviewing Party may provide any comments on such Release (but in no event less than [***] unless earlier disclosure is required by Law). If the Reviewing Party notifies in writing that it does

not have any comments or fails to respond within the period notified by the Issuing Party, then it shall be deemed that the Reviewing Party has agreed that the Issuing Party may issue such the proposed press release or public statement in the form provided to the Reviewing Party. If the Reviewing Party provides any comments, the Parties will consult on such Release and the Issuing Party shall consider the Reviewing Party's comments in good faith.

Section 13.3Publication. Each Party (in such capacity the "**Publishing Party**") agrees that, except as required by applicable Laws, it shall not issue or make any academic, scientific and medical publication or public presentation disclosing any results generated under this Agreement at any time following the Effective Date, whether in the Stoke Territory or the BIG Territory, with respect to the Development, Manufacturing or Commercialization of any Licensed Product or Option Product, including studies relating to medical affairs activities conducted under this Agreement with respect to any Licensed Product or Option Product (any such publication or presentation, "**Publication**" and such results, "**Covered Results**")in each case without the JDC's prior approval, in accordance with the following:

13.3.1 The Publishing Party shall submit to the JDC any proposed Publication that contains Covered Results or otherwise contains any Confidential Information of the other Party (the "**Non-Publishing Party**").

13.3.2 The JDC shall review any such Publication for consistency with the Publication Strategy, and shall determine whether any portion of such Publication containing the Non-Publishing Party's Confidential Information (other than Covered Results) should be modified or deleted. Written copies of such proposed Publication required to be submitted hereunder shall be submitted to the JDC no later than [***] before submission for publication or presentation (the "**Review Period**"). The JDC shall provide its written comments with respect to such publications and presentations within [***] (or such longer period determined by the JDC) after its receipt of such written copy, and the Publishing Party shall delete any Confidential Information of the Non-Publishing Party identified by the JDC.

13.3.3 To the extent the non-Publishing Party has made a material contribution to the generation of Covered Results, it shall have the right to co-author or otherwise join the Publishing Party in such publication or presentation. The Parties will each comply with standard academic practice regarding authorship of scientific publications and recognition of contribution of other parties in any publication under this Agreement.

Section 13.4Relationship to the Confidentiality Agreement. This Agreement supersedes the Confidential Disclosure Agreement; *provided, however,* that all "Confidential Information" disclosed or received by the Parties thereunder will be deemed "Confidential Information" hereunder and will be subject to the terms and conditions of this Agreement.

Section 13.5Attorney-Client Privilege. Neither Party is waiving, nor will be deemed to have waived or diminished, any of its attorney work product protections, attorney-client privileges or similar protections and privileges recognized under the applicable Law of any jurisdiction as a result of disclosing information pursuant to this Agreement, or any of its Confidential Information (including Confidential Information related to pending or threatened litigation) to the Receiving Party, regardless of whether the Disclosing Party has asserted, or is or may be entitled to assert,

such privileges and protections. The Parties may become joint defendants in proceedings to which the information covered by such protections and privileges relates and may determine that they share a common legal interest in disclosure between them that is subject to such privileges and protections, and in such event, may enter into a joint defense agreement setting forth, among other things, the foregoing principles but are not obligated to do so.

ARTICLE 14TERM & TERMINATION

Section 14.1Term of this Agreement. The term of this Agreement shall commence on the Effective Date, and unless terminated earlier as provided in this Article 14, shall continue in full force and effect until expiration of the last-to-expire Royalty Term with respect to all Licensed Product(s) and Option Product(s) in the BIG Territory (the "**Term**"). Upon the expiration of the Royalty Term on a Licensed Product-by-Licensed Product or Option Product-by-Option Product and country-by-country basis, the licenses granted by Stoke to BIG and the licenses granted from BIG to Stoke with respect to such Licensed Product or Option Product in such country shall become fully paid-up, royalty-free, perpetual and irrevocable.

Section 14.2Termination by Mutual Agreement. The Parties shall have the right to terminate this Agreement in its entirety (or in part) upon mutual written agreement. In such case, the Parties shall agree in writing on the effects of such termination (including the costs of transition or wind-down of activities).

Section 14.3Termination by Either Party.

14.3.1Material Breach. Each Party will have the right to terminate this Agreement upon written notice to the other Party if such other Party materially breaches this Agreement and has not cured such breach or such breach is not curable within [***] after notice of such breach from the non-breaching Party, *provided* that, if such breach [***] is not reasonably capable of cure within such [***] period but is capable of cure if given additional time, the breaching Party may submit a reasonable cure plan prior to the end of such [***] period, in which case, if the other Party agrees to such plan (such agreement not to be unreasonably withheld, conditioned or delayed), the other Party will not have the right to terminate this Agreement during an additional [***] period, so long as the breaching Party is using best efforts to implement such cure plan throughout such additional [***] period. Notwithstanding the foregoing in this Section 14.3.1, in the event of a good faith dispute as to whether a material breach by the breaching Party has occurred, the foregoing cure period with respect thereto will be tolled pending final resolution of such dispute in accordance with the terms of this Agreement; *provided, however*, if such dispute relates to payment, such tolling of the cure period will only apply with respect to payment of the disputed amounts, and not with respect to any undisputed amount. It is understood that termination pursuant to this Section 14.3.1 shall be a remedy of last resort and may be invoked only in the case where the breach cannot be reasonably remedied by the payment of money damages.

14.3.2Termination for Anti-Corruption Non-Compliance. Notwithstanding the cure periods and the right to cure in Section 14.3.1, each Party may terminate this Agreement in its entirety with [***] notice to the other Party for breach of the representations, warranties and covenants of Section 10.2.18 and Section 10.4.2, subject to the other Party's right to dispute whether such breach has occurred, which right the other Party may exercise by providing written

notice of such dispute to the first Party within [***] after the other Party's receipt of the applicable notice of termination.

14.3.3Insolvency. Each Party will have the right to terminate this Agreement if, at any time, the other Party: (a) files in any court or agency pursuant to any statute or regulation of any state or country, a petition in bankruptcy or insolvency or for reorganization or for an arrangement or for the appointment of a receiver or trustee of the other Party or of its assets; (b) proposes a written agreement of composition or extension of its debts; (c) is served with an involuntary petition against it, filed in any insolvency proceeding, and such petition will not be dismissed within [***] after the filing thereof; (d) passes a resolution for its winding up or proposes to be or is a party to any dissolution or liquidation; or (e) makes an assignment for the benefit of its creditors.

Section 14.4Termination by BIG.

14.4.1Without Cause. Beginning on the date that is [***] after the Effective Date, BIG, in its sole discretion for any reason or no reason, may terminate this Agreement and the respective rights and obligations thereunder in whole, or in part on a country-by-country or Licensed Product-by-Licensed Product or Option Product-by-Option Product basis by providing to Stoke at least [***] prior written notice.

14.4.2Safety. BIG may terminate this Agreement and the respective rights and obligations thereunder in whole, or in part on a Licensed Product-by-Licensed Product or Option Product-by-Option Product basis immediately upon written notice to Stoke [***].

Section 14.5Termination by Stoke.

(a) If there has not been a First Commercial Sale of a Licensed Product in at least [***] of the [***] within [***] after the first Regulatory Approval (using Centralized Approval Procedure) for a Licensed Product in the European Union, then Stoke shall have the right to terminate the license to the Licensed Product in the European Union.

(b) If there has not been a First Commercial Sale of a Licensed Product in Japan within [***] after the first Regulatory Approval for a Licensed Product in Japan, then Stoke shall have the right to terminate the license to the Licensed Product in Japan.

(c) If there has not been a First Commercial Sale of a Licensed Product in the PRC within [***] after the first Regulatory Approval for a Licensed Product in the PRC, then Stoke shall have the right to terminate the license to the Licensed Product in the PRC.

(d) Any termination of a license pursuant to Section 14.5(a)-(c) must be in writing and must be made within not later than [***] after the date on which such termination right accrued (i.e., not later than [***] of the date of the first Regulatory Approval in the applicable country or region).

Section 14.6[***].

Section 14.7Effects of Termination. Upon termination of this Agreement in its entirety,

the following shall apply (and if this Agreement is terminated in part on a country-by-country or Licensed Product-by-Licensed Product or Option Product-by-Option Product basis, the following shall also apply to the terminated Licensed Product or Option Product or country, *mutatis mutandis*):

14.7.1Termination of Licenses. Except as set forth elsewhere in this Article 14, all licenses and other rights granted hereunder by one Party to the other Party shall terminate and all obligations imposed hereunder upon a Party shall terminate.

14.7.2Non-Cancellable Costs. If termination is by BIG pursuant to Section 14.4.1 or by Stoke pursuant to Section 14.3, Section 14.5 or Section 14.6, then with respect to each Global Development Plan in effect as of the effective date of such termination, BIG shall be liable for and shall pay its share (as set forth in the Global Development Plan and this Agreement) of all Shared Global Development Costs in connection with activities under such Global Development Plan ongoing as of the date of the applicable notice of termination and in accordance with the relevant Global Development Budget; *provided* that such Shared Global Development Costs are incurred by either Party no later than [***] after the date of the applicable notice of termination. For clarity, BIG shall not be liable for any cancelable Shared Global Development Costs in connection with new activities under such Global Development Plan that commence after the date of the applicable notice of termination.

14.7.3 Inventory. BIG shall have the right for a period of [***] to sell off its then-existing inventory of all Licensed Product(s) and Option Product(s) (with any such sales being subject to the milestone payments of Sections 8.3.2 and 8.3.3 and the royalty payment obligations under Section 8.4).

14.7.4Confidential Information. Except to the extent a Party requires the use of the other Party's Confidential Information to perform its obligations or exercise its rights under this Section 14.7, each Party shall at the other Party's written request, destroy all Confidential Information of the other Party that is in its possession as of the effective date of termination (with the exception of one copy of such Confidential Information, which may be retained by the legal department of the Party that received such Confidential Information to confirm compliance with the non-use and non-disclosure provisions of this Agreement), *provided* that each Party may retain and continue to use such Confidential Information of the other Party to the extent necessary to exercise any surviving rights, licenses or obligations under this Agreement. Notwithstanding the foregoing, a Party shall not be required to destroy any of the other Party's Confidential Information stored in computer files created during automatic system back up that are subsequently stored securely by it.

14.7.5Clinical Trials. BIG shall with respect to any ongoing clinical trials of the Licensed Product(s) or Option Product(s) that BIG or its Affiliates or Sublicensees is conducting as of the effective date of termination (a) subject to (b), wind-down and terminate any such ongoing clinical trials in accordance with accepted biopharmaceutical industry norms and appropriate professional and ethical standards and applicable Laws, at BIG's costs and expense; or (b) to the extent Stoke elects to invoke reversion of Licensed Product(s) or Option Product(s) pursuant to Section 14.7.7 and if permitted by applicable Law and not reasonably expected to have a material adverse effect on patient safety, and at Stoke's request, transfer such ongoing clinical trials of the

Licensed Product(s) or Option Product(s) to Stoke, in which case Stoke shall assume all responsibilities for such clinical trial upon completion of transfer thereof (including transfer of the relevant IND or CTA to Stoke). Upon Stoke's request to assume responsibilities for an ongoing clinical trial, BIG shall promptly, at BIG's own costs (unless this Agreement is terminated by BIG pursuant to Section 14.3.1 or Section 14.3.2, in which case Stoke shall bear the Costs of such transfer), (a) transfer to Stoke all Regulatory Filings and Data (including safety data) held by BIG with respect to all ongoing clinical trials, (b) assign, novate or transfer to Stoke all ongoing contracts with contract research organizations and clinical trial sites that relate to ongoing clinical trials including any master services agreements and clinical trial agreements, or to the extent such assignment, novation or transfer is not permissible under such contract or applicable Law, facilitate Stoke's engagement with such subcontractors, (c) continue to support such Clinical Trial until transfer thereof to Stoke is complete, and (d) provide Stoke with reasonable assistance required for the forgoing transfer to Stoke. For clarity, after such assumption of responsibilities by Stoke, Stoke shall have sole discretion on whether to wind-down, in accordance with accepted biopharmaceutical industry norms and appropriate professional and ethical standards and applicable Laws, any on-going clinical trials or whether to continue such clinical trials.

14.7.6Sublicense Survival. Notwithstanding anything to the contrary in Section 14.7, upon a termination of this Agreement by Stoke pursuant to Section 14.3, Section 14.5 or Section 14.6, (a) BIG may provide written notice to Stoke within [***] following the notice of termination that it desires to have any relevant Sublicensee become a direct licensee of Stoke under this Agreement, (b) during a period of [***] following the date of delivery of notice of termination of this Agreement pursuant to Section 14.3, Section 14.5 or Section 14.6, BIG shall respond, or shall cause such Sublicensee to respond to Stoke's reasonable requests for true and correct information and documentation to enable Stoke to confirm the compliance of such Sublicensee with all applicable Laws and the terms of the applicable sublicense agreement, and (c) if such Sublicensee was not in material breach of the terms of this Agreement in regard to such sublicense agreement, and was in compliance with all applicable Laws at the date of delivery of the applicable notice of termination of this Agreement, then Stoke shall be deemed to have entered into a direct license, effective from the effective date of termination of this Agreement, with such Sublicensee and in such license: (i) the scope shall be the same as the scope of such Sublicensee's sublicense agreement (which shall not exceed the scope of the rights granted under Section 2.1.1 or Section 2.2.4(a), as applicable, of this Agreement); and (ii) except with respect to scope, all terms of such direct license shall be identical to the terms of such sublicense agreement (*provided* that such sublicense agreement is consistent with and the applicable Sublicensee agrees to be subject to the terms of this Agreement and subject to any necessary adjustments to make the sublicense function as a direct license with Stoke).

14.7.7Reversion of Products. Upon termination of this Agreement by BIG pursuant to Section 14.4.1 or by Stoke pursuant to Section 14.3.1, Section 14.3.2, Section 14.5 or Section 14.6, each terminated Licensed Product and Option Product shall become a "Reversion Product" upon Stoke's request, which request shall be made within [***] after the effective date of termination. Upon such request, the following shall apply to the extent applicable:

(a) Subject to Section 14.7.5, to the extent that BIG, its Affiliates or Sublicensees (who have not become direct licensee pursuant to Section 14.7.6) holds any Regulatory Filings or Regulatory Approvals for a Reversion Product, at Stoke's request, all of

BIG's and its Affiliates' rights, title and interests therein shall be assigned and transferred to Stoke or its designee, and BIG shall cause each such Sublicensee to assign or transfer its rights, title and interests therein to Stoke or its designee. BIG shall (or shall cause it Affiliates and such Sublicensees to) notify the applicable Regulatory Authorities and take any other action reasonably necessary to effect the transfer. At Stoke's request, all material documents necessary to further Exploit a Reversion Product and all Data generated for a Reversion Product, to the extent Controlled by BIG, it Affiliates or such Sublicensees, and all its or their right, title and interest therein and thereto, shall be assigned and transferred to Stoke or its designee as promptly as reasonably practicable thereafter.

(b) At Stoke's request, any existing agreements between BIG or its Affiliates and any Third Party that are solely related to the Exploitation of a Reversion Product, and all of BIG's and its Affiliates' right, title and interest therein and thereto, shall at Stoke's option be terminated, or assigned and transferred to Stoke or its designee, to the extent permissible pursuant to the terms thereof (and for any such agreement that by its terms cannot be so assigned, BIG shall reasonably cooperate with Stoke to provide to Stoke the benefits of such agreement).

(c) At Stoke's request, should BIG, its Affiliates or Sublicensees (who have not become direct licensee pursuant to Section 14.7.6) own or control any inventory of the Reversion Product suitable for use or sale in the BIG Territory or Stoke Territory, and subject to BIG's right to sell off inventory under Section 14.7.3, Stoke shall have the right (but not the obligation) to purchase such Reversion Product from BIG at a price equal to the actual costs (excluding FTE Costs) of procuring such Reversion Product.

(d) At Stoke's request, BIG shall assign (or, if applicable, cause its Affiliates or Sublicensees (who have not become direct licensee pursuant to Section 14.7.6) to assign) to Stoke all of its or their right, title and interest in and to any trademark (including goodwill) or internet domain name or social media accounts that are specifically related to the Reversion Product and used by BIG or any of its Affiliates or such Sublicensees in the Exploitation of the Reversion Product prior to the effective date of termination.

(e) Upon the effectiveness of such termination, BIG shall and hereby does grant to Stoke a royalty-free, fully paid up, non-exclusive, perpetual, transferable, worldwide license, with the right to grant sublicenses through multiple tiers of sublicensees, under any BIG Collaboration IP existing as of the date of such termination that is necessary to Exploit such Reversion Product anywhere in the terminated territory.

(f) Without limiting the foregoing, at Stoke's request and to the extent permissible pursuant to applicable Laws, BIG shall transition all ongoing material Exploitation activities and contracts (other than as related to clinical trials), including with respect to Development and Commercialization, undertaken by BIG and its Affiliates hereunder to Stoke or Stoke's designee and shall use good faith, diligent efforts to cause to be transitioned any such activities undertaken by any of BIG's Sublicensees (who have not become direct licensee pursuant to Section 14.7.6).

(g) At Stoke's request and discretion, BIG shall return to Stoke or destroy all Reversion Product literature, samples and other sales or sales training materials in the

possession of BIG, its Affiliates and shall use good faith, diligent efforts to cause Sublicensees (who have not become direct licensee pursuant to Section 14.7.6) as promptly as practical after the date of such termination (to the extent then existing).

(h) The Parties will use Commercially Reasonable Efforts to complete all transfer and transition activities required in this Section 14.7.7 as promptly as practicable following the effective date of such termination, and in any event until such transfer and transition is complete. As between the Parties, BIG shall be responsible for all costs and expenses in connection with the activities under this Section 14.7.7 (unless this Agreement is terminated by BIG pursuant to Section 14.3.1 or Section 14.3.2, in which case Stoke shall bear the Costs).

Section 14.8BIG Rights in Lieu of Termination.

14.8.1 On a Licensed Product-by-Licensed Product and Option Product-by-Option Product basis, if, at any time during after the completion of Global Development Activities under the Global Development Plan applicable to such Licensed Product or Option Product, as applicable, BIG has the right to terminate this Agreement pursuant to Section 14.3.1 due to Stoke's material breach of (i) [***] or (ii) [***], *provided* that [***], then BIG may, by written notice to Stoke (which notice Stoke may dispute in accordance with Section 15.5), elect to continue this Agreement as modified by this Section 14.8, in which case, effective as of the date BIG delivers such notice of such election to Stoke (or if such notice is disputed, the date that such dispute is finally resolved in BIG's favor):

(a) All payments payable by BIG to Stoke pursuant to Section 8.3 and Section 8.4 with respect to any affected Licensed Product(s) and Option Product(s) thereafter shall be reduced by [***] relative to the amount calculated in accordance with Section 8.3 and Section 8.4 (including application of any royalty floor) before application of the reduction in this Section 14.8.1(a); and

(b) All other provisions of this Agreement shall remain in full force and effect without change.

On a Licensed Product-by-Licensed Product and Option Product-by-Option Product basis, if BIG exercises its rights under this Section 14.8.1, then BIG may not seek any other monetary remedy with respect to the same material breach of Stoke that gives rise to BIG's rights under this Section 14.8.1.

14.8.2 If, at any time during the Term, BIG has the right to terminate this Agreement pursuant to Section 14.3.1 due to Stoke's failure to fulfill its obligations under the Global Development Plan, then as an alternative to exercising its right of termination, BIG will have the right to elect, at BIG's sole discretion, to conduct the activities allocated to Stoke under the applicable Global Development Plan to which such failure relates by notifying Stoke of such election, in which event such activities under such Global Development Plan will be deemed to be allocated to BIG for the purposes of this Agreement. Stoke will promptly take all actions, including by transferring relevant Regulatory Filings, Data and assign or transfer CRO contracts, necessary for BIG to conduct such activities that were previously allocated to Stoke. BIG shall be entitled to deduct all Costs that it incurs in performing such activities that were originally allocated to Stoke

against milestone payments and royalties payable to Stoke.

Section 14.9Survival. The following provisions will survive termination or expiration of this Agreement: Article 1, Section 4.4.1 (as set forth therein), Section 4.5 (solely with respect to amounts accrued prior to the effective date of termination or expiration), Section 8.1 through 8.4.4 (solely with respect to amounts accrued prior to the effective date of termination or expiration), Section 8.7 through Section 8.11, Section 8.12 through Section 8.13 (solely with respect to amounts accrued prior to the effective date of termination or expiration), Section 9.1, Article 11, Section 12.1, Section 12.2 (as set forth therein), Article 13, Section 14.7, Section 14.9 and Article 15. Termination or expiration of this Agreement are neither Party's exclusive remedy and will not relieve the Parties of any liability or obligation which accrued hereunder prior to the effective date of such termination or expiration nor preclude either Party from pursuing all rights and remedies it may have hereunder or at law or in equity with respect to any breach of this Agreement nor prejudice either Party's right to obtain performance of any obligation. All other rights and obligations will terminate upon termination or expiration of this Agreement.

ARTICLE 15MISCELLANEOUS

Section 15.1Entire Agreement; Amendment. This Agreement, the letter by and between Biogen Inc., BIG and Stoke, dated February 14, 2025, and all exhibits and schedules attached hereto or thereto, constitute the entire agreement between the Parties as to the subject matter hereof. All prior negotiations, representations, warranties, agreements, statements, promises and understandings with respect to the subject matter of this Agreement, including the Confidential Disclosure Agreement, are hereby superseded by this Agreement. No amendment, supplement or other modification to any provision of this Agreement shall be binding unless in writing and signed by authorized representatives of BIG and Stoke.

Section 15.2Rights in Bankruptcy. All rights and licenses granted under or pursuant to any section of this Agreement are, and shall otherwise be deemed to be, for purposes of Section 365(n) of the U.S. Bankruptcy Code or any analogous Laws in any other country or jurisdiction, licenses of rights to "intellectual property" as defined under Section 101(35A) of the U.S. Bankruptcy Code, or the analogous provisions in any other country or jurisdiction. The Parties agree that the Parties, as licensees of such rights under this Agreement, shall retain and may fully exercise all of their rights and elections under the U.S. Bankruptcy Code or any analogous provisions in any other country or jurisdiction. The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against either Party under the U.S. Bankruptcy Code or any analogous provisions in any other country or jurisdiction, the Party hereto that is not a Party to such proceeding shall be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property and all embodiments of such intellectual property, which, if not already in the non-subject Party's possession, shall be promptly delivered to it (i) upon any such commencement of a bankruptcy proceeding upon the non-subject Party's written request therefor, unless the Party subject to such proceeding elects to continue to perform all of its obligations under this Agreement or (ii) if not delivered under clause (i) above, following the rejection of this Agreement by or on behalf of the Party subject to such proceeding upon written request therefor by the non-subject Party.

Section 15.3Independent Contractors. The relationship between BIG and Stoke

created by this Agreement is solely that of independent contractors. This Agreement does not create any agency, distributorship, employee-employer, partnership, joint venture or similar business relationship between the Parties, including for all tax purposes. No such Party is a legal representative of the other Party, and no such Party can assume or create any obligation, representation, warranty or guarantee, express or implied, on behalf of the other Party for any purpose whatsoever. Each such Party shall use its own discretion and shall have complete and authoritative control over its employees. The Parties agree and acknowledge that neither owes any fiduciary duties to the other.

Section 15.4Governing Law; Service. This Agreement shall be governed by and construed in accordance with the laws of the state of New York, excluding any conflicts or choice of law rule or principle that might otherwise refer construction or interpretation of this Agreement to the substantive law of another jurisdiction, except that any issue which depends upon the validity, scope or enforceability of any Patent Rights shall be determined in accordance with the laws of the country in which such Patent Right was issued or was pending. The Parties agree to exclude the application to this Agreement of the United Nations Convention on Contracts for the International Sale of Goods. Each Party further agrees that service of any process, summons, notice or document by registered mail to its address set forth in Section 15.6 shall be effective service of process for any action, suit or proceeding brought against it under this Agreement in the applicable court.

Section 15.5Dispute Resolution.

15.5.1Procedures.

(a) Except as provided in Section 3.3 and Section 15.7, any disputes arising out of or in connection with this Agreement, including any questions regarding its existence, validity, interpretation, breach or termination (a "**Dispute**") shall first be referred to the Executive Officers of the Parties, who shall confer in good faith on the resolution of such Dispute. Any final decision mutually agreed to by the Executive Officers, which shall be reflected in a written document executed by the Parties, shall be conclusive and binding on the Parties. If the Executive Officers are not able to agree on the resolution of any such Dispute within [***] (or such other period of time as mutually agreed by the Executive Officers) after such Dispute was first referred to them in writing, then, except as set forth in clause (b) of this Section 15.5.1, such Dispute shall be resolved through binding arbitration in accordance with Section 15.5.2.

(b) Nothing contained in this Agreement shall deny either Party the right to seek injunctive or other equitable relief from a court of competent jurisdiction in the context of a *bona fide* emergency or prospective irreparable harm, and such an action may be filed and maintained notwithstanding any ongoing arbitration proceeding or other procedures set forth herein.

15.5.2 Any Dispute not resolved by the Executive Officers shall be submitted to the International Chamber of Commerce (the "**ICC**") for resolution by arbitration before three (3) arbitrators, under the Arbitration Rules of the ICC as of the Effective Date, and the following shall apply:

(a) Each Party shall select one (1) arbitrator, and the two (2) Party-selected arbitrators shall then together select the third arbitrator.

(b) The place of arbitration shall be Toronto, Canada (unless another location is otherwise agreed to by the Parties). The proceedings and related communications shall be in English.

(c) The decision and award of the arbitration panel shall be made by majority decision and shall be final, non-appealable and binding on the Parties hereto and their successors and assigns. Judgment may be entered thereon in a court of competent jurisdiction. The arbitral award shall be accompanied by a reasoned opinion.

(d) Except as expressly permitted in this Agreement or required by applicable Law, no Party nor arbitrator may disclose the existence, content or results of any arbitration hereunder without the prior written consent of the Parties. Any documents submitted to or issued by the arbitration panel shall be kept confidential and shall not be disclosed, except that any such documents may be disclosed (i) as reasonably necessary in connection with any action to enforce or collect the award or (ii) to the extent discoverable or admissible in any action arising out of or in connection with this Agreement.

(e) The fees and expenses of the arbitrators and any administrative fees associated with the arbitration will be shared equally by the Parties. Unless otherwise set forth in the arbitration award, the Parties will otherwise bear their respective expenses (including their respective legal, expert and other fees and costs) of the arbitration.

(f) Nothing in this Agreement will preclude either Party from applying to any court of competent jurisdiction at any time during the pendency of an arbitration hereunder or otherwise to (i) enforce the arbitration provisions in this section of this Agreement (including with respect to maintaining the confidentiality of any arbitration proceedings and non-public information) or (ii) seek equitable relief, including interim, provisional or similar relief (including a temporary restraining order or a preliminary injunction).

Section 15.6Language for Communications; Notice.

(a) All communications, meetings (including meeting materials) and notices to be made or given by one Party to the other Party pursuant to this Agreement will be in the English language.

(b) Any notice required or permitted to be given by this Agreement shall be in writing, in English and refer specifically to this Agreement. Any and all notices or other communications (other than day-to-day business or operational communications between the Parties) or deliveries required or permitted to be provided hereunder shall be in writing and shall be deemed given and effective if (a) delivered by hand or by overnight courier with tracking capabilities, (b) mailed postage prepaid by registered, or certified mail with receipt confirmation or (c) delivered electronically via email with the notice in the body of the email or attached as a PDF or other electronic file and with receipt acknowledged by the recipient (excluding automatic replies) and followed up by delivery via clauses (a) or (b) within five (5) Business Days, and addressed or sent as set forth below unless changed by notice so given:

If to Stoke:

Stoke Therapeutics, Inc.
45 Wiggins Avenue
Bedford, MA 01730
Email: [***]
Attention: Chief Executive Officer

with a copy (which shall not constitute notice) to:

Stoke Therapeutics, Inc.
45 Wiggins Avenue
Bedford, MA 01730
Email: [***]
Attention: Legal

If to BIG: Biogen International GmbH
Neuhofstrasse 30
Address: 6340 Baar
Switzerland
Attention: President, European Region

with a copy (which shall not constitute notice) to:

Biogen Inc.
Address: 225 Binney Street
Cambridge, MA 02142
Email: [***]
Attention: Chief Legal Officer

Any such notice delivered via method (a) or (b) shall be deemed given on the date received. Any such notice delivered via method (c) shall be deemed given on the date that the recipient acknowledges receipt of the electronic notice by replying to the sender a message confirming receipt, such acknowledgement and confirmation shall not be unreasonably delayed, withheld, or conditions. A Party may add, delete, or change the Person or address or email address to which notices should be sent at any time upon written notice delivered to the Party's notices in accordance with this Section 15.6. Each Party providing notice under methods (a) or (b) above shall concurrently with the physical delivery of such notice send each recipient an email with a courtesy PDF copy of the notice, which shall not constitute notice.

Section 15.7Specific Performance. The Parties agree that irreparable damage may occur in the event any provision hereof was not performed in accordance with the terms hereof and that the Parties will be entitled to seek specific performance of the terms hereof, in addition to any other remedy at law or in equity.

Section 15.8Compliance with Law; Severability. Nothing in this Agreement shall be construed to require the commission of any act contrary to Law. If any one or more provisions of this Agreement is held to be invalid, illegal or unenforceable under any present or future Law and if the rights or obligations of either Party under this Agreement will not be materially and adversely affected thereby, (a) such provision shall be fully severable, (b) this Agreement shall be construed and enforced as if such illegal, invalid or unenforceable provision had never comprised a part hereof, (c) the remaining provisions of this Agreement shall remain in full force and effect and shall not be affected by the illegal, invalid or unenforceable provision or by its severance herefrom, and (d) in lieu of such illegal, invalid or unenforceable provision, there shall be added automatically as a part of this Agreement a legal, valid and enforceable provision as similar in terms to such illegal, invalid or unenforceable provision as may be possible and reasonably acceptable to the Parties.

Section 15.9Non-Use of Names. Neither Party shall not use the name, trademark, logo, or physical likeness of the other Party or any of its respective officers, directors or employees, or any adaptation of any of them, in any advertising, promotional or sales literature, without the other Party's prior written consent, not to be unreasonably withheld, delayed or conditioned. Each Party shall require its Affiliates and Sublicensees to comply with the foregoing.

Section 15.10Successors and Assigns. Neither this Agreement nor any of the rights or obligations created herein may be assigned by either Party, in whole or in part, without the prior written consent of the other Party, such consent not to be unreasonably withheld, conditioned or delayed, except that (a) either Party shall be free to assign this Agreement without the other Party's consent (i) in whole or in part to an Affiliate of such Party, *provided* that such Party shall remain liable and responsible to the other Party for the performance and observance of all duties and obligations by such Affiliate, or (ii) in whole in connection with any sale of all or substantially all of the assets of the Party to a Third Party, whether by merger, consolidation, divestiture, restructure, sale of stock, sale of assets or otherwise (a "**Sale Transaction**"), *provided* that the Person to which this Agreement is assigned expressly agrees in writing to assume and be bound by all obligations of the assigning Party under this Agreement; (b) with respect to all Licensed Products or with respect to all Option Products, on and after the First Commercial Sale of the first Licensed Product or the first Option Product, as applicable, BIG shall have the right to assign this Agreement without Stoke's consent in connection with any sale of all or substantially all of the assets of BIG that relate to the Licensed Products or the Option Products, as applicable, to a Third Party, whether by divestiture or asset sale, *provided* that the Person to which this Agreement is partially assigned expressly agrees in writing to assume and be bound by all obligations of BIG under this Agreement that has been so assigned, and (c) on a Licensed Product-by-Licensed Product and Option Product-by-Option Product basis after the First Commercial Sale of such Licensed Product or Option Product, Stoke shall have the right to assign its right to receive royalty payments for such Licensed Product or Option Product hereunder without BIG's consent to a *bona fide* Third Party engaged in the business of royalty monetization; *provided* that such Party shall remain liable and responsible to the other Party for the performance and observance of all duties and obligations by such Third Party, *provided further,* that for the avoidance of doubt, BIG will not be obligated to provide any information or assistance to such Third Party other than as expressly provided in this Agreement, *provided, further,* that such Third Party expressly agrees in writing to be bound by Section 8.8.3 hereof. A copy of a written agreement by an assignee described above shall be provided to the non-assigning Party within [***] (or as soon as

practicable) of execution of such written agreement. This Agreement shall bind and inure to the benefit of the successors and permitted assigns of the Parties hereto. Any attempted assignment of this Agreement in contravention of this Section 15.10 shall be null and void.

Section 15.11Waivers. A Party's consent to or waiver, express or implied, of the other Party's breach of any of its obligations hereunder shall not be deemed to be or construed as a consent to or waiver of any other breach of the same or any other obligations of such other Party. A Party's failure to complain of any act, or failure to act, by the other Party, to declare the other Party in default, to insist upon the strict performance of any obligation or condition of this Agreement or to exercise any right or remedy consequent upon a breach thereof, no matter how long such failure continues, shall not constitute a waiver by such Party of its rights hereunder, of any such breach, or of any other obligation or condition. A Party's consent in any one instance shall not limit or waive the necessity to obtain such Party's consent in any future instance and in any event no consent or waiver shall be effective for any purpose hereunder unless such consent or waiver is in writing and signed by the Party granting such consent or waiver.

Section 15.12No Third Party Beneficiaries. Nothing in this Agreement shall be construed as giving any Person, other than the Parties hereto and their successors and permitted assigns, any right, remedy or claim under or in respect of this Agreement or any provision hereof, except for the provisions of Article 11 (with respect to which the persons to which Article 11 applies shall be Third Party beneficiaries for Article 11 only in accordance with the terms and conditions of Article 11).

Section 15.13Force Majeure. Except with respect to delays or nonperformance by a Party caused by the negligent or intentional act or omission of such Party, any delay or nonperformance by such Party (other than a delay or failure to make payment) will not be considered a breach of this Agreement to the extent such delay or nonperformance is caused by acts of God, natural disasters, acts or failures to act of the government (including any Regulatory Authority or other Governmental Authority or civil or military authority), fire, floods, epidemics, pandemic, quarantine, energy crises, war or riots or other similar cause outside of the reasonable control of such Party arising after the Effective Date (each, a "**Force Majeure Event**"), *provided* that the Party affected by such Force Majeure Event will promptly begin or resume performance as soon as reasonably practicable after the event has abated.

Section 15.14Headings; Exhibits; Appendices. Article and Section headings used herein are for convenient reference only, and are not a part of this Agreement. All Exhibits are incorporated herein by this reference.

Section 15.15Interpretation. Except where the context otherwise requires, wherever used, the singular shall include the plural, the plural the singular, and the use of any gender shall be applicable to all genders. The term "including" as used herein means including, without limiting the generality of any description preceding such term. The word "will" shall be construed to have the same meaning and effect as the word "shall". The words "herein", "hereof" and "hereunder" and words of similar import will be construed to refer to this Agreement in its entirety and not to any particular provision hereof. The word "or" has the inclusive meaning represented by the phrase "and/or". This Agreement was prepared in the English language, which language shall govern the interpretation of, and any dispute regarding, the terms of this Agreement. The Parties and their

counsel have cooperated in the drafting and preparation of this Agreement, and this Agreement therefore shall not be construed against any Party by virtue of its role as the drafter thereof.

Section 15.16Counterparts Electronic or Facsimile Signatures. This Agreement may be executed in any number of counterparts, each of which shall be an original, but all of which together shall constitute one instrument. This Agreement may be executed and delivered electronically or by facsimile and upon such delivery such electronic or facsimile signature will be deemed to have the same effect as if the original signature had been delivered to the other Party.

[*signature page follows*]

IN WITNESS WHEREOF, the Parties have executed this Agreement as of the Effective Date.

STOKE THERAPEUTICS, INC.

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

By: /s/ Eric Olson
Name: Eric Olson
Title: Chief Business Officer

BIOGEN INTERNATIONAL GMBH

By: /s/ Wolfram Schmidt
Name: Wolfram Schmidt
Title: President, Europe

Exhibit 6.2

Principal Terms of Clinical Supply Agreement

Summary	This Term Sheet summarizes the key terms for the Clinical Supply Agreement described in Section 6.2 of the Agreement. Any capitalized terms used herein that are not defined shall have the meanings assigned to them in the Agreement.
Manufacture and Supply	Stoke shall Manufacture (or have Manufactured) and supply to BIG, BIG's requirements of the Licensed Product in the form of fully released bulk labeled vials ("**Finished Drug Product**") and placebo (the Finished Drug Product and placebo collectively the "**Product**") for use by BIG to conduct its Development activities, including clinical Development activities under the Agreement, in the BIG Territory. All products Manufactured and delivered under the Clinical Supply Agreement will conform to the applicable specifications, certificate of analysis, and certificate of conformance for such products, and will have been Manufactured in accordance with cGMP, the Quality Agreement, any applicable Regulatory Approval, and applicable Law.
Subcontracting	Stoke may not subcontract any of its obligations under the Clinical Supply Agreement to a Third Party without BIG's prior written consent, provided that Stoke may use the any subcontractors identified in the Global Development Plan in conducting its obligations under the Clinical Supply Agreement (each such approved Third Party a "**Stoke CMO**"). In the event Stoke intends to enter into any new agreement (or amendment any material provision of an existing agreement) with a Stoke CMO, it shall notify BIG of such proposed new agreement or amendment and provide BIG the reasonably opportunity to review and comment on such proposed agreement or amendment. Stoke, BIG, and each Stoke CMO (including existing Stoke CMOs) will enter into a three -way confidentiality agreement to permit the exchange of information relating to the manufacture of product under the Clinical Supply Agreement, provided that all communications with any Stoke CMO shall be managed by Stoke. If Stoke desires to change the facility used by Stoke to manufacture Product to be supplied to BIG or qualify a new source for Product to be supplied by Stoke to BIG, then Stoke will seek approval for

	such facility change or qualification of such new source from BIG, which consent will not be unreasonably withheld, conditioned or delayed. Stoke shall remain solely responsible for the acts, omissions and performance of any Stoke CMO as if such act, omissions and performance had been performed by Stoke itself under the Clinical Supply Agreement and applicable Quality Agreement.
Forecast; KPIs; Order Mechanics.	The Clinical Supply Agreement will include commercially reasonable terms for forecast, key performance indicators, and order mechanics.
Delivery Terms	Stoke will deliver the Product to BIG to a destination specified by BIG, FCA at the CMOs facility (Incoterms 2020) ("**Delivery**").
Supply Price	BIG shall pay Stoke for conforming Product at a price equal to [***] (as defined in more detail in the Clinical Supply Agreement, the "**Supply Price**"). At the time of Delivery, Stoke will invoice BIG at the estimated Supply Price for the Product. BIG shall pay such invoice within [***] after receipt thereof (subject to BIG's acceptance of such Product). The Clinical Supply Agreement will include a mechanism for quarterly true ups to reflect the actual Supply Price. BIG will have the right to audit such costs as set forth in the License Agreement.
Quality & Documentation	The Parties shall enter into one or more quality agreements for the Product ("**Quality Agreements**"). In the event of any inconsistency between the Quality Agreements and the Clinical Supply Agreement, the Quality Agreements shall govern with respect to quality assurance matters, and the Clinical Supply Agreement shall govern with respect to all other matters. Delivery of the Product will be accompanied by a Certificate of Analysis and other such documents as may be required pursuant to the Clinical Supply Agreement, Quality Agreements and applicable Law. BIG will have the right to perform quality audits with Stoke on Stoke's CMO under terms to be set forth in the Quality Agreement and the Clinical Supply Agreement.
Manufacturing Representations and Warranties	The Clinical Supply Agreement will contain a representation, warranty and covenant by Licensee that it has obtained (or will obtain) as of the effective date and will maintain during the term of the Clinical Supply Agreement (or, in the event Licensee delegates

	the importation activities to a Third Party or an Affiliate, will cause the applicable Third Party or Affiliate to obtain and maintain during the term of the Clinical Supply Agreement) the necessary customs clearance and/or import permits) of the applicable Governmental Authority in the BIG Territory for the import of the Finished Drug Product into the BIG Territory, as well as other customary manufacturing and supply related representations and warranties.
Inspection, Acceptance and Rejection	The Clinical Supply Agreement shall contain customary terms regarding BIG's acceptance and rejection of Product for defects. For patent defects, BIG shall have up to [***] following the Delivery of Product to inspect such product and notify Stoke if any such Product fails to meet the warranties or if the quantity of such Product Delivered is short ("**Non-Conforming Product**"). In the event of any Non-Conforming Product, Stoke shall, at BIG's option, (a) promptly replace (or supply in the event of a shortage) such Non-Conforming Product, or (b) refund any amounts paid by BIG for such Non-Conforming Product. Without limiting the foregoing, in all cases (except for a shortage), to the extent Stoke has Delivered any Non-Conforming Product, Stoke shall also reimburse BIG for any shipping costs and other out-of-pocket costs (such costs to be defined in the Clinical Supply Agreement) incurred by BIG for such Non-Conforming Product. The Clinical Supply Agreement shall also contain customary terms regarding BIG's acceptance and rejection of Product for latent defects.
Governing Law	State of New York.
Limitation of Liability and Other Customary Provisions	The Clinical Supply Agreement shall provide that Stoke's liability under the Clinical Supply Agreement to BIG, its Affiliates and Sublicensees, in the aggregate, shall be limited to the extent Stoke may recover from the Stoke CMO under the relevant Stoke CMO agreement; *provided*, however, that this limitation will not apply to Losses arising from Stoke's breach of contract, negligence, or willful misconduct. The Clinical Supply Agreement will additionally contain other customary provisions, including indemnification, audit and inspection provisions, confidentiality, record-keeping, tax and insurance provisions.

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, Ian F. Smith, 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: May 13, 2025

/s/ Ian F. Smith

Ian F. Smith
Interim 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: May 13, 2025

/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, Ian F. Smith, Interim 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 March 31, 2025 (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: May 13, 2025

/s/ Ian F. Smith
Ian F. Smith
Interim 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 March 31, 2025 (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: May 13, 2025

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