Filed pursuant to Rule 424(b)(3)

Registration No. 333-221791

PROSPECTUS SUPPLEMENT NO. 7

(To the Prospectus dated February 6, 2018)

39,714,143 Shares of Common Stock

This Prospectus Supplement No. 7 supplements the prospectus dated February 6, 2018 (the "Prospectus"), relating to the offering and resale of up to 39,714,143 shares of common stock of Exicure, Inc. by the selling stockholders identified in the Prospectus. This Prospectus Supplement should be read in conjunction with the Prospectus which is to be delivered with this Prospectus Supplement. Any statement contained in the Prospectus shall be deemed to be modified or superseded to the extent that information in this Supplement modifies or supersedes such statement. Any statement that is modified or superseded shall not be deemed to constitute a part of the Prospectus except as modified or superseded by this Supplement.

This Prospectus Supplement is being filed to update and supplement the information in the Prospectus with the information contained in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2018 filed with the Securities and Exchange Commission on November 6, 2018 (the "Form 10-Q"), all set forth below.

Investing in our common stock involves a high degree of risk. Before making an investment decision, please read "Risk Factors" on page 11 of the Prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this Prospectus Supplement (or the Prospectus including any supplements or amendments thereto) is truthful or complete. Any representation to the contrary is a criminal offense.

The date of this Prospectus Supplement is November 6, 2018.

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

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

For the quarterly period ended September 30, 2018

or

☐ **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: 000-55764

EXICURE, INC.
(Exact name of registrant as specified in its charter)

Delaware	**81-5333008**
(State or other jurisdiction of incorporation or organization)	**(IRS Employer Identification No.)**

8045 Lamon Avenue
Suite 410
Skokie, IL 60077
(Address of principal executive offices)

Registrant's telephone number, including area code (847) 673-1700

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, a 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 by Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of October 31, 2018, there were 44,358,000 shares of the registrant's common stock, par value $0.0001 per share, outstanding.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains express or implied forward-looking statements within the meaning of 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") that are based on our management's belief and assumptions and on information currently available to our management. All statements other than statements of historical fact contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as "may," "could," "will," "would," "should," "expect," "plan," "anticipate," "believe," "estimate," "intend," "predict," "seek," "contemplate," "project," "continue," "potential," "ongoing" or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the initiation, timing, progress and results of our research and development programs, preclinical studies, clinical trials and Investigational New Drug application, or IND, Investigational Medicinal Product Dossier, Clinical Trial Application, or CTA, New Drug Application, or NDA, or other regulatory submissions;
- our receipt and timing of any milestone payments or royalties under any current or future research collaboration and license agreements or arrangements;
- our ability to identify and develop therapeutic candidates for treatment of additional disease indications;
- our or a current or future collaborator's ability to obtain and maintain regulatory approval of any of our therapeutic candidates;
- the rate and degree of market acceptance of any approved therapeutic candidates;
- the commercialization of any approved therapeutic candidates;
- our ability to establish and maintain collaborations and retain commercial rights for our therapeutic candidates in the collaborations;
- the implementation of our business model and strategic plans for our business, technologies and therapeutic candidates;
- our estimates of our expenses, ongoing losses, future revenue and capital requirements, including our expectations relating to our need for additional financing;
- our ability to obtain additional funds for our operations;
- our ability to obtain and maintain intellectual property protection for our technologies and therapeutic candidates and our ability to operate our business without infringing the intellectual property rights of others;
- the ability of third party supply and manufacturing partners to supply the materials and components for, and manufacture, our research and development, preclinical and clinical trial supplies;
- our ability to attract and retain qualified key management and technical personnel;
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act of 2012, or the JOBS Act;
- statements regarding internal controls under the section titled "Controls and Procedures" Part I Item 4;
- our financial performance; and

- the impact of government regulation and developments relating to our competitors or our industry.

These statements relate to future events or our future operational or financial performance, and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under the section titled "Risk Factors" Part II - Item 1A and elsewhere in this Quarterly Report on Form 10-Q.

Any forward-looking statement in this Quarterly Report on Form 10-Q reflects our current view with respect to future events and is subject to these and other risks, uncertainties and assumptions relating to our business, results of operations, industry and future growth. Given these uncertainties, you should not place undue reliance on these forward-looking statements. No forward-looking statement is a guarantee of future performance. You should read this Quarterly Report on Form 10-Q and the documents we reference in this Quarterly Report on Form 10-Q and have filed with the Securities and Exchange Commission (the "SEC") as exhibits thereto completely and with the understanding that our actual future results may be materially different from any future results expressed or implied by these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Quarterly Report on Form 10-Q also contains estimates, projections and other information concerning our industry, our business and the markets for certain therapeutics, including data regarding the estimated size of those markets, their projected growth rates and the incidence of certain medical conditions. Information that is based on estimates, forecasts, projections or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained these industry, business, market and other data from reports, research surveys, studies and similar data prepared by third parties, industry, medical and general publications, government data and similar sources. In some cases, we do not expressly refer to the sources from which these data are derived.

PART I - FINANCIAL INFORMATION

Item 1. Financial Statements.

EXICURE, INC.

UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share data)

	September 30, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$ 32,446	$ 25,764
Accounts receivable	52	—
Unbilled revenue receivable	7	13
Receivable from related party	4	17
Prepaid expenses and other assets	1,855	1,844
Total current assets	34,364	27,638
Property and equipment, net	1,130	1,317
Other noncurrent assets	32	32
Total assets	$ 35,526	$ 28,987
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Current portion of long-term debt	$ 4,926	$ —
Accounts payable	1,341	1,049
Accrued expenses and other current liabilities	3,035	1,273
Current portion of deferred revenue	—	1,034
Total current liabilities	9,302	3,356
Long-term debt, net	—	4,855
Common stock warrant liability	984	523
Other noncurrent liabilities	42	278
Total liabilities	$ 10,328	$ 9,012
Stockholders' equity:		
Common stock, $0.0001 par value per share; 200,000,000 shares authorized, 44,358,000 issued and outstanding, September 30, 2018; 39,300,823 shares issued and outstanding, December 31, 2017	4	4
Additional paid-in capital	75,433	53,586
Accumulated deficit	(50,239)	(33,615)
Total stockholders' equity	25,198	19,975
Total liabilities and stockholders' equity	$ 35,526	$ 28,987

See Accompanying Notes to Unaudited Condensed Consolidated Financial Statements.

EXICURE, INC.

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(in thousands, except share and per share data)

	Three Months Ended, September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue:				
Collaboration revenue	$ 57	$ 2,497	$ 112	$ 7,624
Total revenue	57	2,497	112	7,624
Operating expenses:				
Research and development expense	4,001	3,121	11,111	9,910
General and administrative expense	1,919	1,270	5,952	4,806
Total operating expenses	5,920	4,391	17,063	14,716
Operating loss	(5,863)	(1,894)	(16,951)	(7,092)
Other income (expense), net:				
Interest expense	(170)	(201)	(497)	(616)
Other income (loss), net	709	163	(210)	140
Total other income (loss), net	539	(38)	(707)	(476)
Net loss	$ (5,324)	$ (1,932)	$ (17,658)	$ (7,568)
Basic and diluted loss per common share	$ (0.13)	$ (1.12)	$ (0.44)	$ (41.27)
Basic and diluted weighted-average common shares outstanding	41,527,922	1,725,906	40,121,295	183,395

See Accompanying Notes to Unaudited Condensed Consolidated Financial Statements.

EXICURE, INC.

UNAUDITED CONDENSED CONSOLIDATED STATEMENT OF CHANGES IN STOCKHOLDERS' EQUITY

(in thousands, except shares)

	Common Stock		Additional Paid-in-Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	$			
Balance at December 31, 2017	**39,300,823**	**$ 4**	**$53,586**	**$(33,615)**	**$ 19,975**
Adoption of new accounting standard - ASC 606	—	—	—	1,034	1,034
Balance at January 1, 2018	**39,300,823**	**$ 4**	**$53,586**	**$(32,581)**	**$ 21,009**
Exercise of options	22,494	—	41	—	41
Equity-based compensation	—	—	1,300	—	1,300
Issuance of common stock to consultants, net	145,466	—	436	—	436
Issuance of common stock in private placement, net	4,889,217	—	20,070		20,070
Net loss	—	—	—	(17,658)	(17,658)
Balance at September 30, 2018	**44,358,000**	**$ 4**	**$75,433**	**$(50,239)**	**$ 25,198**

See Accompanying Notes to Unaudited Condensed Consolidated Financial Statements.

EXICURE, INC.

UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

	Nine Months Ended September 30,	
	2018	**2017**
Cash flows from operating activities:		
Net loss	$ (17,658)	$ (7,568)
Adjustments to reconcile net loss to cash used in operating activities:		
Depreciation and amortization	266	159
Equity-based compensation	1,300	1,104
Amortization of long-term debt issuance costs and fees	71	150
Other	291	—
Change in fair value of warrant liabilities	461	(201)
Changes in operating assets and liabilities:		
Unbilled revenue receivable and accounts receivable	(46)	(201)
Receivable from related party	13	2
Prepaid expenses and other current assets	135	(1,835)
Accounts payable	278	1,361
Accrued expenses and other current liabilities	1,602	(1,209)
Deferred revenue	—	(6,207)
Other noncurrent liabilities	(237)	(2)
Net cash used in operating activities	(13,524)	(14,447)
Cash flows from investing activities:		
Capital expenditures	(65)	(726)
Net cash used in investing activities	(65)	(726)
Cash flows from financing activities:		
Proceeds from common stock offering	22,001	20,302
Proceeds from exercise of common stock options	41	43
Repayment of long-term debt	—	(595)
Payment of common stock financing costs	(1,771)	(1,264)
Net cash provided by financing activities	20,271	18,486
Net increase in cash and cash equivalents	6,682	3,313
Cash and cash equivalents - beginning of period	25,764	19,623
Cash and cash equivalents - end of period	$ 32,446	$ 22,936
Supplemental disclosure of cash flow information		
Non-cash financing activities:		
Issuance of common stock for professional services	$ 436	$ —
Issuance of common stock warrants	—	211
Common stock issuance costs (accounts payable and accrued expenses)	160	1,773
Non-cash investing activities:		
Capital expenditures (accounts payable and accrued expenses)	14	—

See Accompanying Notes to Unaudited Condensed Consolidated Financial Statements.

1. Description of Business and Basis of Presentation

Description of Business

Exicure is a clinical-stage biotechnology company developing gene regulatory and immuno-oncology therapeutics based on the Company's proprietary Spherical Nucleic Acid ("SNA") technology. We believe the design of the Company's SNAs gives rise to chemical and biological properties that may provide advantages over other nucleic acid therapeutics and enable therapeutic activity outside of the liver. The Company intends to build a leading nucleic acid therapeutics company focused on the discovery and development of therapeutics based on the Company's proprietary SNA technology, either on its own or in collaboration with pharmaceutical partners.

Throughout this Quarterly Report on Form 10-Q, the terms "the Company" and "Exicure" refer to Exicure, Inc. and its 100% owned subsidiary, Exicure Operating Company. Exicure Operating Company holds all material assets, and conducts all business activities and operations, of the Company.

References to the "Merger" refer to the September 26, 2017 transaction whereby Max-1 Acquisition Sub, Inc., a wholly-owned subsidiary of Max-1 Acquisition Corporation, or Max-1, merged with and into Exicure Operating Company (f/k/a Exicure, Inc.), a privately-held Delaware corporation, with Exicure Operating Company remaining as the surviving entity and a wholly-owned operating subsidiary of Max-1 (which then changed its name to Exicure, Inc.). References to the "2017 Private Placement" refer to the transactions following the Merger that occurred in several closings on September 26, 2017, October 27, 2017 and November 2, 2017 in which we sold to accredited investors approximately $31,513 worth of shares of common stock (before deducting placement agent fees and expenses which are approximately $3,966), or 10,504,196 shares, at a price of $3.00 per share.

On July 9, 2015, AuraSense Therapeutics, LLC was converted into AuraSense Therapeutics, Inc., a privately-held Delaware corporation, and on the same date changed its name to Exicure, Inc., which actions together are referred to in these notes to unaudited condensed consolidated financial statements as the corporate conversion. In connection with the corporate conversion, the accumulated deficit of AuraSense Therapeutics, LLC of $18,837 was reclassified to additional paid-in capital.

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements as of September 30, 2018 and December 31, 2017, and for the three and nine months ended September 30, 2018 and 2017, have been presented in conformity with generally accepted accounting principles in the United States ("GAAP").

Principles of Consolidation

The accompanying unaudited condensed consolidated financial statements include the accounts of Exicure, Inc. and its 100% owned subsidiary, Exicure Operating Company. All intercompany transactions and accounts are eliminated in consolidation.

Going Concern

As of September 30, 2018, the Company has generated an accumulated deficit of $69,076 since inception and expects to incur significant expenses and negative cash flows for the foreseeable future. Based on the Company's current operating plans, it believes that existing working capital at September 30, 2018 is sufficient to fund its current operating plans into the first quarter of 2020. Management believes that it will be able to obtain additional working capital through equity financings, partnerships and licensing, or other arrangements, to fund operations. However, there can be no assurance that such additional financing will be available and, if available, can be obtained on terms acceptable to the Company. If the Company is unable to obtain such additional financing, the Company will need to reevaluate future operating plans. Accordingly, there is substantial doubt regarding the Company's ability to continue as a going concern.

The accompanying unaudited condensed consolidated interim financial statements have been prepared as though the Company will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might be necessary should the Company be unable to continue as a going concern.

Unaudited Interim Financial Information

The accompanying interim condensed consolidated balance sheet as of September 30, 2018, the interim condensed consolidated statements of operations for the three and nine months ended September 30, 2018 and 2017, the interim condensed consolidated statement of changes in stockholders' equity for the nine months ended September 30, 2018, and the interim condensed consolidated statements of cash flows for the nine months ended September 30, 2018 and 2017, are unaudited. The interim unaudited condensed consolidated financial statements have been prepared on the same basis as the annual audited financial statements; and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the Company's financial position as of September 30, 2018, the results of its operations for the three and nine months ended September 30, 2018 and 2017, and the results of its cash flows for the nine months ended September 30, 2018 and 2017. The financial data and other information disclosed in these notes related to the three and nine months ended September 30, 2018 and 2017 are unaudited. The results for the three and nine months ended September 30, 2018 are not necessarily indicative of results to be expected for the year ending December 31, 2018, or any other interim periods, or any future year or period.

Use of Estimates

The preparation of the financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Management bases its estimates on certain assumptions which it believes are reasonable in the circumstance and while actual results could differ from those estimates, management does not believe that any change in those assumptions in the near term would have a significant effect on the Company's financial position, results of operations or cash flows. Actual results in future periods could differ from those estimates.

Revision of Prior Period Financial Statements

In connection with preparing our condensed consolidated interim financial information for the three months ended March 31, 2018, we identified errors that affected prior interim and annual periods related to the timing of recognition of research and development expense related to a contract for the clinical trial of one of our therapeutic candidates. We evaluated whether our previously issued consolidated financial statements were materially misstated and concluded that the errors individually and in the aggregate were not material to any of our previously issued financial statements. We revised the financial statements to correct the immaterial errors, and the accompanying comparative financial statements reflect these corrections. The correction of the errors increased prepaid expense and other current assets by $933, decreased accrued expenses by $96, and decreased accumulated deficit by $1,028 at December 31, 2017; decreased research and development expense, operating loss, and net loss by $381 and loss per share by $0.22 for the three months ended September 30, 2017; and decreased research and development expense, operating loss, and net loss by $1,369 and loss per share by $7.46 for the nine months ended September 30, 2017.

2. Significant Accounting Policies

There have been no changes to the significant accounting policies disclosed in the Company's most recent Annual Report on Form 10-K, except as required by recently adopted accounting pronouncements, as discussed below.

Revenue Recognition

Effective January 1, 2018, the Company adopted the provisions of Accounting Standards Codification ("ASC") 606, *Revenue from Contracts with Customers* using the modified retrospective method for all contracts not completed as of the date of adoption. The reported results for 2018 reflect the application of ASC 606 guidance, while the reported results for 2017 were prepared under the guidance of ASC 605, *Revenue Recognition* (ASC 605).

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 Company expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that are within the scope of ASC 606, the Company performs the following five steps:

1. *Identify the contract with the customer.* A contract with a customer exists when (i) the Company enters into an enforceable contract with a customer that defines each party's rights regarding the goods or services to be transferred and identifies the related payment terms, (ii) the contract has commercial substance, and (iii) the Company determines that collection of substantially all consideration for goods and services that are transferred is probable based on the customer's intent and ability to pay the promised consideration. The Company applies judgment in determining the customer's intent and ability to pay, which is based on a variety of factors including the customer's historical payment experience, or in the case of a new customer, published credit and financial information pertaining to the customer.
2. *Identify the performance obligations in the contract.* Performance obligations promised in a contract are identified based on the goods and services that will be transferred to the customer that are both capable of being distinct, whereby the customer can benefit from the good or service either on its own or together with other available resources, and are distinct in the context of the contract, whereby the transfer of the good or service is separately identifiable from other promises in the contract. To the extent a contract includes multiple promised goods and services, the Company must apply judgment to determine whether promised goods and services are both capable of being distinct and distinct in the context of the contract. If these criteria are not met, the promised goods and services are accounted for as a combined performance obligation.
3. *Determine the transaction price.* The transaction price is determined based on the consideration to which the Company will be entitled in exchange for transferring goods and services to the customer. To the extent the transaction price includes variable consideration, the Company estimates the amount of variable consideration that should be included in the transaction price utilizing either the expected value method or the most likely amount method, depending on the nature of the variable consideration. Variable consideration is included in the transaction price if, in the Company's judgment, it is probable that a significant future reversal of cumulative revenue under the contract will not occur. Any estimates, including the effect of the constraint on variable consideration, are evaluated at each reporting period for any changes. Determining the transaction price requires significant judgment.
4. *Allocate the transaction price to performance obligations in the contract.* If the contract contains a single performance obligation, the entire transaction price is allocated to the single performance obligation. However, if a series of distinct services that are substantially the same qualifies as a single performance obligation in a contract with variable consideration, the Company must determine if the variable consideration is attributable to the entire contract or to a specific part of the contract. Contracts that contain multiple performance obligations require an allocation of the transaction price to each performance obligation on a relative standalone selling price basis unless the transaction price is variable and meets the criteria to be allocated entirely to a performance obligation or to a distinct service that forms part of a single performance obligation. The consideration to be received is allocated among the separate performance obligations based on relative standalone selling prices.
5. *Recognize revenue when or as the Company satisfies a performance obligation.* The Company satisfies performance obligations either over time or at a point in time. Revenue is recognized over time if either (i) the customer simultaneously receives and consumes the benefits provided by the entity's performance, (ii) the entity's performance creates or enhances an asset that the customer controls as the asset is created or enhanced, or (iii) the entity's performance does not create an asset with an alternative use to the entity and the entity has an enforceable right to payment for performance completed to date. If the entity does not satisfy a performance obligation over time, the related performance obligation is satisfied at a point in time by transferring the control of a promised good or service to a customer. Examples of control are using the

asset to produce goods or services, enhance the value of other assets, or settle liabilities, and holding or selling the asset.

Licenses of intellectual property: 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 recognizes revenues from consideration allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the licenses. For licenses that are combined with other promises, the Company utilizes judgment to assess the nature of the combined 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.

Milestone payments: At the inception of each arrangement that includes development milestone payments, the Company evaluates the probability of reaching the milestones and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur in the future, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received and therefore revenue recognized is constrained as management is unable to assert that a reversal of revenue would not be possible. The transaction price is then allocated to each performance obligation on a relative standalone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, the Company re-evaluates the probability of achievement of such development 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 collaboration revenues and earnings in the period of adjustment.

Royalties: For arrangements that include sales-based royalties, including milestone payments based on levels 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 its collaboration agreements.

As of September 30, 2018, the Company's only revenue recognized is related to the Purdue Collaboration (see Note 3).

Recently Adopted Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2014-09 (ASC 606), *Revenue from Contracts with Customers*. This ASU, as amended by ASU 2015-14, affects any entity that either enters into contracts with customers to transfer goods and services or enters into contracts for the transfer of nonfinancial assets. ASU 2014-09 replaces most existing revenue recognition guidance in GAAP when it becomes effective. The standard's core principle is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. In doing so, companies will need to use more judgment and make more estimates than under the currently effective guidance. These may include identifying performance obligations in the contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. ASU 2014-09 is effective for Exicure in the first quarter of 2018 and early adoption is permitted beginning in the first quarter of 2017. The Company adopted ASC 606 on a modified retrospective basis. See above "Revenue Recognition" for a discussion of the Company's updated policies related to revenue recognition effective January 1, 2018.

Impact of adoption of ASC 606

The Company adopted ASC 606 using the modified retrospective method. The cumulative effect of applying the new guidance to all contracts with customers that were not completed as of January 1, 2018 was recorded as an adjustment to accumulated deficit as of the adoption date. As a result of applying the modified retrospective method to adopt the new guidance, the Company recorded reductions to both accumulated deficit and deferred revenue, current of $1,034 as of the date of adoption.

As a result of the adoption of ASC 606: (i) there were no impacts to the totals of our cash flows from operating activities, cash flows from investing activities, or cash flows from financing activities in the accompanying unaudited condensed consolidated statement of cash flows for the nine months ended September 30, 2018; (ii) there were no impacts to the balances of the accompanying unaudited condensed consolidated balance sheet as of September 30, 2018, (iii) there were no impacts to total revenue, operating loss, or net loss in the accompanying unaudited condensed consolidated statement of operations for the three months ended September 30, 2018, and (iv) total revenue, operating loss, and net loss were lower by $1,034 each in the accompanying unaudited condensed consolidated statement of operations for the nine months ended September 30, 2018.

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments*. ASU 2016-15 addresses the classification of certain specific cash flow issues including debt prepayment or extinguishment costs, settlement of certain debt instruments, contingent consideration payments made after a business combination, proceeds from the settlement of certain insurance claims and distributions received from equity method investees. ASU 2016-15 is effective for the Company in the first quarter of 2018 and early adoption is permitted. An entity that elects early adoption must adopt all of the amendments in the same period. The Company adopted this guidance on January 1, 2018. The adoption of ASU 2016-15 did not have a material impact to the Company's statement of cash flows.

In May 2017, the FASB issued ASU 2017-09, *Compensation - Stock Compensation (Topic 718): Scope of Modification Accounting*. ASU 2017-09 clarifies when changes to the terms or conditions of a share-based payment award must be accounted for as modifications. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award changes as a result of the change in terms or conditions. ASU 2017-09 will be applied prospectively to awards modified on or after the adoption date. ASU 2017-09 is effective for the Company for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Early adoption is permitted. The Company adopted this guidance on January 1, 2018. The adoption of ASU 2017-09 did not have a material impact to the Company's financial statements.

Recent Accounting Pronouncements Not Yet Adopted

In February 2016, FASB issued ASU 2016-02, *Leases (Topic 842)*, which requires lessees to recognize right-of-use assets and lease liabilities on the balance sheet. ASU 2016-02 is to be applied using a modified retrospective approach at the beginning of the earliest comparative period in the financial statements. ASU 2016-02 will be effective for the Company beginning in the first quarter of 2019. Early adoption is permitted. The Company is currently evaluating the impact of adopting this standard on its financial statements.

3. Purdue Collaboration

On December 2, 2016, the Company entered into a research collaboration, option and license agreement with Purdue Pharma, L.P. ("Purdue"), referred to herein as the "Purdue Collaboration." Purdue has the option to obtain from us the full worldwide development and commercial rights to AST-005 (the Company's therapeutic candidate that targets tumor necrosis factor), an option to obtain three additional collaboration targets and a further option to obtain from us the full worldwide development and commercial rights to any therapeutic candidates developed targeting the three additional collaboration targets. Additionally, Purdue has rights of first offer to some potential collaboration targets. These rights of first offer are subject to limitations in time and scope. In connection with the Purdue Collaboration, the Company received a non-refundable development fee of $10,000. In addition, the Company is eligible to receive up to $776,500 upon successful completion of certain research, regulatory and

commercial sales milestones. The research milestones are payable upon target identification and IND-enabling pre-clinical development, per program, with an aggregate total of up to $16,500. The regulatory milestones are payable upon the initiation or completion of clinical trials, and regulatory approval in the United States and outside the United States, per program, with an aggregate total of up to $410,000. The commercial sales milestones are payable upon achievement of specified aggregate product sales thresholds and total up to $350,000. In the event a therapeutic candidate subject to the collaboration results in commercial sales, the Company is eligible to receive royalties ranging from the low single digits to a maximum of 10% on future net sales of such commercialized therapeutic candidates. Additionally, Purdue had an obligation to invest in a qualified equity financing of the Company if such financing was completed before June 2, 2017. The Company did not complete such qualified equity financing before June 2, 2017.

In April 2018, Purdue notified the Company it had declined to exercise its option to develop AST-005 at that time, but that it also intended to retain rights relating to the TNF target, and Purdue reserved its right to continue joint development, with Exicure, of new anti-TNF drug candidates and to retain its exclusivity and other rights to AST-005. Purdue has not indicated that it has any plans to pursue AST-005 at this time.

Prior to the adoption of ASC 606, the upfront payment of $10,000 was accounted for pursuant to ASC 605 and was recorded as deferred revenue and recognized on a ratable basis over the estimated performance period of the relevant research and development activities. On January 1, 2018, in connection with the adoption of ASC 606, the Company recorded the unamortized deferred revenue of $1,034 as an adjustment to the beginning balance of retained deficit at January 1, 2018. See Note 2, *Significant Accounting Policies*, for more information related to the adoption of ASC 606.

The Company identified multiple performance obligations as part of the Purdue Collaboration agreement, including the upfront payment of $10,000, discussed above, and the research and development services. The Company determined that the performance obligations should not be combined, the license should be recognized at the time the license is granted, and the research and development services should be recognized at the time the service is performed. The Purdue Collaboration agreement includes contingent promises related to specified research, development and regulatory milestones and sale-based milestones. Each contingent promise related to contingent and milestone payment is evaluated to determine whether it represents a material right. The Company recognizes any payment that is contingent upon the achievement of a substantive milestone entirely in the period in which it is determined that the revenue is not subject to a significant reversal. To date, the Company has not recognized any contingent payments in connection with the Purdue Collaboration agreement as revenue.

During the three and nine months ended September 30, 2018, the Company recognized collaboration revenue of $57 and $112, respectively, which consisted entirely of research and development activities that will be reimbursed by Purdue and is presented on a gross basis in the accompanying statement of operations. During the three and nine months ended September 30, 2017, the Company recognized collaboration revenue of $2,497 and $7,624, respectively, which included $428 and $1,417, respectively, of research and development activities that was reimbursed by Purdue and is presented on a gross basis in the accompanying statement of operations.

(in thousands, except share and per share data)

4. Supplemental Balance Sheet Information

Property and equipment, net

	September 30, 2018	December 31, 2017
Scientific equipment	$ 1,954	$ 1,797
Leasehold improvements	192	192
Furniture and fixtures	41	31
Computers and software	26	26
Construction in process	14	120
Property and equipment, gross	2,227	2,166
Less: accumulated depreciation	(1,097)	(849)
Property and equipment, net	$ 1,130	$ 1,317

Depreciation and amortization expense was $90 and $70 for the three months ended September 30, 2018 and 2017, respectively, and $266 and $159, for the nine months ended September 30, 2018 and 2017, respectively.

Accrued expenses and other current liabilities

	September 30, 2018	December 31, 2017
Accrued legal expenses	$ 1,068	$ 251
Accrued payroll-related expenses	732	718
Accrued clinical, contract research and manufacturing costs	651	205
Other accrued expenses	584	99
Accrued expenses and other current liabilities	$ 3,035	$ 1,273

5. Debt

On January 15, 2018, the Company and Hercules Technology Growth Capital Limited ("Hercules") amended its loan agreement so that amortization payments due for the thirteen (13) consecutive months commencing on December 1, 2017 through and including December 1, 2018 were deferred. Commencing on January 1, 2019, and continuing on the first business day of each month thereafter, the loan, including the deferred payments, shall begin amortizing in equal monthly installments of principal and interest based upon an amortization schedule equal to eighteen (18) consecutive months. Any remaining obligations under the loan agreement and other loan documents are due and payable on the maturity date on September 1, 2019.

At September 30, 2018 and December 31, 2017, the carrying value of the current and noncurrent portion of long-term debt is $4,926 and $4,855, respectively.

(in thousands, except share and per share data)

At September 30, 2018, the principal maturities of the long-term debt were as follows:

	September 30, 2018
2018	$ —
2019	4,999
Principal balance outstanding	4,999
less: unamortized discount	(65)
less: unamortized debt issuance costs	(8)
Long-term debt	4,926
Current portion	4,926
Noncurrent portion	$ —

The Company paid interest on debt of $146 and $158 during the three months ended September 30, 2018 and 2017, respectively, and $425 and $470 during the nine months ended September 30, 2018 and 2017, respectively.

6. Stockholders' Equity

Preferred Stock

As of September 30, 2018 and December 31, 2017, the Company had 10,000,000 shares of preferred stock, par value $0.0001 authorized and no shares issued and outstanding.

Common Stock

As of September 30, 2018 and December 31, 2017, the Company had authorized 200,000,000 shares of common stock, par value $0.0001. As of September 30, 2018, the Company had 44,358,000 shares issued and outstanding. As of December 31, 2017, the Company had 39,300,823 shares issued and outstanding.

The holders of shares of the Company's common stock are entitled to one vote per share on all matters to be voted upon by Exicure stockholders and there are no cumulative rights. Subject to preferences that may be applicable to any outstanding preferred stock, the holders of shares of the Company's common stock are entitled to receive ratably any dividends that may be declared from time to time by Exicure's board of directors (the "Board") out of funds legally available for that purpose. In the event of the Company's liquidation, dissolution or winding up, the holders of shares of Exicure common stock are entitled to share ratably in all assets remaining after payment of liabilities, subject to prior distribution rights of preferred stock then outstanding. Exicure common stock has no preemptive or conversion rights or other subscription rights. There are no redemption or sinking fund provisions applicable to Exicure common stock. The outstanding shares of Exicure common stock are fully paid and non-assessable.

August 2018 Private Placement

On August 22, 2018, we entered into subscription agreements with several accredited investors, pursuant to which we agreed to issue and sell a total of 4,889,217 shares of the Company's common stock, at a purchase price of $4.50 per share, resulting in approximately $22,001 in gross proceeds to the Company (the "August 2018 Private Placement"). The aggregate net proceeds from the August 2018 Private Placement (after deducting placement agent fees and expenses of the offering of $1,931) were $20,070.

The Company also entered into a registration rights agreement with the investors in the August 2018 Private Placement, which required us to file a "resale" registration statement with the SEC covering the Shares issued in the August 2018 Private Placement within 30 calendar days from the final closing of the August 2018 Private Placement Offering. The Company filed and caused to become effective a registration statement with the SEC on October 5,

(in thousands, except share and per share data)

2018 registering the resale of 5,034,683 shares of our common stock, consisting of (i) 4,889,217 shares that were privately issued through the August 2018 Private Placement and (ii) 145,466 shares that were privately issued on February 1, 2018 in connection with consulting services.

In connection with the closing of the August 2018 Private Placement, the placement agents received an aggregate of $1,680 in cash placement fees, and the Company reimbursed up to $87 of expenses incurred by the placement agents in connection with this closing of the August 2018 Private Placement.

Common Stock Warrants

In connection with the 2017 Private Placement, placement agents received warrants to purchase an aggregate of 413,320 shares of Exicure common stock (the "Warrants") in connection with all closings of the 2017 Private Placement. The Warrants expire on March 27, 2021, have an exercise price of $3.00 per share, and have been issued on the same terms in all closings of the 2017 Private Placement. The Warrants are classified as a liability. The common stock warrant liability is remeasured each period at fair value. As of September 30, 2018, Warrants to purchase 413,320 shares of common stock remain outstanding. See Note 10, *Fair Value Measurements* for more information on the fair value of the common stock warrant liability.

7. Equity-Based Compensation

As of September 30, 2018, the aggregate number of common stock options available for grant under the 2017 Equity Incentive Plan was 928,443.

Equity-based compensation expense is classified in the statements of operations as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Research and development expense	$ 133	$ 43	$ 353	$ 129
General and administrative expense	383	319	947	975
	$ 516	$ 362	$ 1,300	$ 1,104

Unamortized equity-based compensation expense at September 30, 2018 was $3,635, which is expected to be amortized over a weighted-average period of 2.8 years.

The Company utilizes the Black-Scholes option-pricing model to determine the fair value of common stock option grants. The Black-Scholes option-pricing model was developed for use in estimating the fair value of traded options that have no vesting restrictions and are fully transferable. The model also requires the input of highly subjective assumptions. In addition to an assumption on the expected term of the option grants as discussed below, application of the Black-Scholes model requires additional inputs for which we have assumed the values described in the table below:

(in thousands, except share and per share data)

	Nine Months Ended September 30,	
	2018	2017
Expected term	5.3 to 6.0 years	5.3 to 6.5 years
Risk-free interest rate	2.72% to 2.87%; weighted avg. 2.78%	1.97% to 2.17%; weighted avg. 2.07%
Expected volatility	78.1% to 82.4%; weighted avg. 80.6%	80.8% to 83.1%; weighted avg. 81.0%
Forfeiture rate	5%	5%
Expected dividend yield	—%	—%

The expected term is based upon the "simplified method" as described in Staff Accounting Bulletin Topic 14.D.2. Currently, the Company does not have sufficient experience to provide a reasonable estimate of an expected term of its common stock options. The Company will continue to use the "simplified method" until there is sufficient experience to provide a more reasonable estimate in conformance with ASC 718-10-30-25 through 30-26. The risk-free interest rate assumptions were based on the U.S. Treasury bond rate appropriate for the expected term in effect at the time of grant. The expected volatility is based on calculated enterprise value volatilities for publicly traded companies in the same industry and general stage of development. The estimated forfeiture rates were based on historical experience for similar classes of employees. The dividend yield was based on expected dividends at the time of grant.

The fair value of the underlying common stock and the exercise price for the common stock options granted during the nine months ended September 30, 2018 and 2017 are summarized in the table below:

Common Stock Options Granted During Period Ended:	Fair Value of Underlying Common Stock	Exercise Price of Common Stock Option
Nine months ended September 30, 2018	$3.00 to $5.82; weighted avg. $3.45	$3.00 to $5.82; weighted avg. $3.45
Nine months ended September 30, 2017	$4.21	$4.21

The weighted-average grant date fair value of common stock options granted in the nine months ended September 30, 2018 and 2017 was $2.40 and $2.92 per common stock option, respectively.

(in thousands, except share and per share data)

A summary of common stock option activity as of the periods indicated is as follows:

	Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (thousands)
Outstanding - December 31, 2017	3,672,620	$ 1.79	7.5	$ 5,221
Granted	1,277,744	3.45		
Exercised	(22,494)	1.81		
Forfeited	(36,282)	2.29		
Outstanding - September 30, 2018	4,891,588	$ 2.22	7.6	$ 9,523
Exercisable - September 30, 2018	3,021,753	$ 1.62	6.8	$ 7,541
Vested and Expected to Vest - September 30, 2018	4,778,505	$ 2.20	7.5	$ 9,421

The aggregate intrinsic value of common stock options exercised during the nine months ended September 30, 2018 and 2017 was $51 and $202, respectively.

8. Income Taxes

The Company incurred a pretax loss in each of the three and nine months ended September 30, 2018 and 2017, which consists entirely of loss in the U.S. and resulted in no provision for income tax expense during the periods then ended. The effective tax rate is 0% in each of the three and nine months ended September 30, 2018 and 2017 because the Company has generated tax losses and has provided a full valuation allowance against its deferred tax assets.

9. Loss Per Common Share

Basic loss per common share is calculated by dividing net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted loss per common share is calculated using the treasury share method by giving effect to all potentially dilutive securities that were outstanding. Potentially dilutive options and warrants to purchase common stock that were outstanding during the periods presented were excluded from the diluted loss per share calculation because such shares had an anti-dilutive effect due to the net loss reported in those periods. Therefore, basic and diluted loss per common share is the same for each of the three and nine months ended September 30, 2018 and 2017.

The following is the computation of loss per common share for the three and nine months ended September 30, 2018 and 2017:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Net loss	$ (5,324)	$ (1,932)	$ (17,658)	$ (7,568)
Weighted-average basic and diluted common shares outstanding	41,527,922	1,725,906	40,121,295	183,395
Loss per share - basic and diluted	$ (0.13)	$ (1.12)	$ (0.44)	$ (41.27)

The outstanding securities presented below were excluded from the calculation of net loss per common share, because such securities would have been anti-dilutive due to the Company's net loss per share during the periods ending on the dates presented:

(in thousands, except share and per share data)

	September 30,	
	2018	2017
Options to purchase common stock	4,891,588	3,678,359
Warrants to purchase common stock	413,320	163,174

10. Fair Value Measurements

ASC Topic 820, Fair Value Measurement, establishes a three-tier fair value hierarchy, which prioritizes the inputs used in measuring fair value, as follows: Level 1 Inputs - unadjusted quoted prices in active markets for identical assets or liabilities accessible to the reporting entity at the measurement date; Level 2 Inputs - other than quoted prices included in Level 1 inputs that are observable for the asset or liability, either directly or indirectly, for substantially the full term of the asset or liability; and Level 3 Inputs - unobservable inputs for the asset or liability used to measure fair value to the extent that observable inputs are not available, thereby allowing for situations in which there is little, if any, market activity for the asset or liability at measurement date.

The Company uses the market approach and Level 1 inputs to value its cash equivalents.

The Company's long-term debt bore interest at the prevailing market rates for instruments with similar characteristics and, accordingly, the carrying value for this instrument also approximates its fair value and the financial measurement is also classified within Level 2 of the fair value hierarchy.

The Company's common stock warrant liability (refer to Note 6, *Stockholders' Equity* for more information) is classified within Level 3 of the fair value hierarchy. The fair value of the common stock warrant liability was determined using the Black-Scholes option-pricing model.

The following weighted-average assumptions were used to estimate the fair value of the common stock warrant liability at September 30, 2018:

	September 30, 2018
Expected term	2.5 years
Risk-free interest rate	2.83%
Expected volatility	81.78%
Expected dividend yield	—%

A 10% change in the estimate of expected volatility at September 30, 2018 would increase or decrease the fair value of the common stock warrant liability in the amount of $55. A 10% change in the estimate of fair value of the common stock at September 30, 2018 would increase or decrease the fair value of the common stock warrant liability in the amount of $141.

(in thousands, except share and per share data)

The following is a reconciliation of the Company's liabilities measured at fair value on a recurring basis using unobservable inputs (Level 3) for the nine months ended September 30, 2018:

	Fair Value Measurements Using Significant Unobservable Inputs (Level 3)
	Common Stock Warrant Liability
Balance at January 1, 2018	$ 523
Loss included in other income (expense), net	461
Balance at September 30, 2018	$ 984

11. Commitments and Contingencies

Leases

The Company conducts all operations in a facility under an operating lease which commenced in March 2012 and was originally scheduled to end in February 2015. The lease was extended for an additional six years through February 2021 during the first quarter of 2014 and includes a renewal option. During the second quarter of 2016, the Company amended the lease agreement to include additional space to be used primarily for administrative functions effective in May 2016. Lease payments include a fixed payment amount as well as contingent payments related to a proportionate share of operating and real estate expenses. At the inception of the lease, the lessor paid for leasehold improvements totaling $52 which has been capitalized and is being amortized over the lease term. The fixed payment amounts, including those in connection with the amended lease agreement in the second quarter of 2016, increase over the term of the lease but rent expense is recognized on a straight-line basis resulting in the recognition of deferred rent liability of $41 and $48 as of September 30, 2018 and December 31, 2017, respectively, calculated on the basis of the extended lease agreement.

Rent expense consisted of the following:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Straight-line rent expense	$ 83	$ 83	$ 249	$ 249
Contingent rent expense	67	77	231	232
Total rent expense	$ 150	$ 160	$ 480	$ 481

(in thousands, except share and per share data)

Future minimum lease payments as of September 30, 2018 are as follows:

Years Ending December 31,	Operating Leases
2018	85
2019	347
2020	353
2021	59
Thereafter	—
Total	$ 844

Northwestern University License Agreements

On December 12, 2011, (1) AuraSense, LLC assigned to the Company all of its worldwide rights and interests under AuraSense, LLC's 2009 license agreement with Northwestern University ("NU") in the field of the use of nanoparticles, nanotechnology, microtechnology or nanomaterial-based constructs as therapeutics or accompanying therapeutics as a means of delivery, but expressly excluding diagnostics (the "assigned field"); (2) in accordance with the terms and conditions of this assignment, the Company assumed all liabilities and obligations of AuraSense, LLC as set forth in its license agreement in the assigned field; and (3) in order to secure this assignment and the patent rights from NU, the Company agreed (i) to pay NU an annual license fee, which may be credited against any royalties due to NU in the same year, (ii) to reimburse NU for expenses associated with the prosecution and maintenance of the license patent rights, (iii) to pay NU royalties based on any net revenue generated by the Company's sale or transfer of any licensed product, and (iv) to pay NU, in the event the Company grants a sublicense under the licensed patent rights, the greater of a percentage of all sublicensee royalties or a percentage of any net revenue generated by a sublicensee's sale or transfer of any licensed product. In August 2015, we entered into a restated license agreement with NU (the "restated license agreement"). In February 2016, we obtained exclusive license as to NU's rights in certain SNA technology we jointly own with NU. Our license to NU's rights is limited to the assigned field, however we have no such limitation as to our own rights in this jointly owned technology. In June 2016, we entered into an exclusive license with NU to obtain worldwide rights to certain inhibitors of glucosylceramide synthase and their use in wound healing in diabetes. Our rights and obligations in these 2016 agreements are substantially the same as in the restated license agreement from August 2015 (collectively referred to as "the Northwestern University License Agreements"). As of September 30, 2018, the Company has paid to NU an aggregate of $3,844 in consideration of each of the obligations described above.

12. Related-Party Transactions

Since its inception in 2011, the Company has shared facilities, certain staff members and certain operating expenses with AuraSense, LLC, our former parent and largest stockholder. On an infrequent basis, the Company also pays certain expenses directly on behalf of AuraSense, LLC which are related to AuraSense, LLC's grants, and AuraSense, LLC sometimes pays expenses directly on behalf of the Company. These costs are summarized and directly billed between the Company and AuraSense, LLC on a quarterly basis. In addition, certain expense and administrative activities are shared between the Company and AuraSense, LLC. Effective January 1, 2016, the Company and AuraSense, LLC amended its shared services agreement to simplify the billing arrangement. Under the amended shared services agreement, the Company bills AuraSense, LLC $8 per quarter for indirect costs incurred by the Company plus a specified rate for hours worked by Company scientists on projects directly related to AuraSense, LLC. The amended shared services arrangement continues to require direct non-labor expenses incurred by the Company to be billed to AuraSense, LLC. Effective January 1, 2017, the Company and AuraSense, LLC further amended its shared services agreement so that the quarterly fee related to administrative activities billed by the Company to AuraSense, LLC be reduced to $3 per quarter. This decrease is to reflect the current and expected future reduction in administrative activities to be provided by the Company to AuraSense, LLC.

(in thousands, except share and per share data)

The amounts due from AuraSense, LLC in connection with the above mentioned activities were $4 and $17 at September 30, 2018 and December 31, 2017, respectively.

The following is a summary of amounts billed to AuraSense, LLC and recognized in the accompanying unaudited condensed consolidated statement of operations in connection with the above mentioned activities:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2018	2017	2018	2017
Quarterly fee for indirect costs	3	3	$ 9	$ 9
Direct costs of AuraSense LLC paid by the Company	2	1	24	4
	$ 5	$ 4	$ 33	$ 13

The Company received consulting services from, and paid fees to, one of its co-founders who is not an employee but serves as a member of the Board. The Company paid $75 in each of the nine months ended September 30, 2018 and 2017 in connection with these consulting services and these amounts are recognized as an expense in the accompanying unaudited condensed consolidated statement of operations.

13. Subsequent Events

The Company has evaluated subsequent events which may require adjustment to or disclosure in the accompanying unaudited condensed consolidated financial statements and has concluded that there are no subsequent events or transactions that occurred subsequent to the balance sheet date that would require recognition or disclosure in the accompanying unaudited condensed consolidated financial statements.

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 results of operations together with our unaudited condensed consolidated interim financial statements and the related notes and other financial information included in this Quarterly Report on Form 10-Q. Management's Discussion is designed to provide an understanding of our operations and financial performance and should be read in conjunction with our Annual Report on Form 10-K for the fiscal year ended December 31, 2017. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties as described under the heading "Special Note Regarding Forward-Looking Statements" elsewhere in this Quarterly Report on Form 10-Q. You should review the disclosure under the heading "Risk Factors" in this Quarterly Report on Form 10-Q for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Operating Overview

We are a clinical-stage biotechnology company developing gene regulatory and immuno-oncology therapeutics based on our proprietary Spherical Nucleic Acid, or SNA, technology. SNAs are nanoscale constructs consisting of densely packed synthetic nucleic acid sequences that are radially arranged in three dimensions. We believe the design of our SNAs gives rise to distinct chemical and biological properties that may provide advantages over other nucleic acid therapeutics and enable therapeutic activity outside of the liver. Since our SNAs have shown in a Phase 1 clinical trial and in preclinical studies that they can cross certain biological barriers when administered locally, we believe that they have the therapeutic potential to target diseases not typically addressed with other nucleic acid therapeutics. We have demonstrated the ability to cross certain biological barriers in a Phase 1 clinical trial of two therapeutic candidates, AST-008 and AST-005, and in preclinical studies of one other therapeutic candidate, XCUR17.

Clinical development programs

We currently have three clinical programs: AST-008, XCUR17, and AST-005.

AST-008

AST-008 is an SNA consisting of toll-like receptor 9, or TLR9, agonists designed for immuno-oncology applications. TLR9 agonists bind to and activate TLR9 receptors. We believe AST-008 may be used for immuno-oncology applications as a monotherapy or in combination with checkpoint inhibitors. We conducted a Phase 1 clinical trial of AST-008 in the United Kingdom. The Phase 1 clinical trial was a first-in-human clinical trial of AST-008 evaluating the safety, tolerability, pharmacokinetics, and pharmacodynamics of AST-008 in healthy volunteers. The trial was a randomized, single ascending dose, or SAD, trial. Sixteen healthy subjects were recruited and organized into four SAD cohorts. Based on our analyses of the Phase 1 clinical trial results, AST-008 was shown to be safe and tolerable in all subjects, with no serious adverse events and no dose limiting toxicity. All AST-008-related adverse events were of short duration, reversible and consistent with TLR9 activation. In addition, AST-008 was shown to elicit high levels of certain cytokines as well as to activate important effector cells of the immune system, including T cells and natural killer cells, which are the main drivers of an anti-tumor response.

The Company's IND for AST-008 has been opened by the FDA and we have been informed by the FDA that our proposed Phase 1b/2 clinical trial may proceed. We expect to dose the first patient late this year. The Phase 1b/2 trial is an open-label trial of intra-tumorally dosed AST-008 in combination with pembrolizumab. The trial will begin with an AST-008 dose finding Phase 1b stage, followed by a Phase 2 expansion stage. In the Phase 1b, we plan to enroll patients with superficial injectable tumors, but will prioritize those with Merkel cell carcinoma, cutaneous squamous cell carcinoma, melanoma, and squamous cell carcinoma of the head and neck. We expect to report the preliminary data from the Phase 1b stage in late 2019.

XCUR17

XCUR17 is an SNA that targets the mRNA which encodes interleukin 17 receptor alpha, or IL-17RA, a protein that is considered essential in the initiation and maintenance of psoriasis. Although the availability of inhibitors of TNF revolutionized the systemic treatment of severe psoriasis, studies of disease pathogenesis have shifted attention to the IL-17 pathway, in which IL-17RA is a key driver of psoriasis. Our strategy is to reduce the levels of IL-17RA in the skin by topically applying XCUR17. In preclinical studies, XCUR17 inhibited IL-17RA in the keratinocytes of the skin. In the third quarter of 2018, we completed the dosing of the 21 mild to moderate psoriasis patients enrolled in our Phase 1 clinical trial. No additional patients will be enrolled and we expect to report the topline results from this Phase 1 trial by the end of 2018. The endpoints of the clinical trial include safety and tolerability, the measurement of IL-17RA mRNA from skin biopsies, skin biopsy histology, and optionally other assessments of mRNA targets in the psoriasis network.

AST-005

AST-005 is an SNA targeting TNF for the treatment of mild to moderate psoriasis and is intended to be administered locally in a gel to psoriatic lesions. In a completed Phase 1 clinical trial, AST-005, when topically administered to the skin of patients with mild to moderate psoriasis, resulted in no drug associated adverse events, and demonstrated a reduction of TNF mRNA. The TNF mRNA reduction elicited by the highest strength of AST-005 gel was statistically significant when compared to the effects of the vehicle.

On December 2, 2016, we entered into a research collaboration, option and license agreement with Purdue, referred to as the Purdue Collaboration. As part of our collaboration with Purdue, a Phase 1b clinical trial was conducted in Germany to evaluate the effect of AST-005 gel in patients with chronic plaque psoriasis. The trial evaluated the safety, tolerability, and plaque thickness following topical application of different strengths of AST-005 formulated as a topical gel. The trial demonstrated that AST-005 is safe and tolerable in patients at higher doses than were previously studied, however, the study did not result in a statistically significant decrease in echo lucent band thickness, one of the key indicators of efficacy in patients with psoriasis. In April 2018, Purdue notified the Company that it had declined to exercise its option to develop AST-005 at that time, but that it also intended to retain rights relating to the TNF target, and Purdue reserved its right to continue joint development, with Exicure, of new anti-TNF drug candidates and to retain its exclusivity and other rights to AST-005. Purdue has not indicated that it has any plans to pursue AST-005 at this time.

Preclinical development activities

We believe that one of the key strengths of our proprietary SNAs is that they have the potential to enter a number of different cells and organs. As a consequence, we are also conducting early stage research activities in neurology, ophthalmology, pulmonology, and gastroenterology.

In June 2018, Exicure and The Ohio State University Wexner Medical Center presented a poster at the Cure SMA Annual Conference titled: “Nusinersen in spherical nucleic acid (SNA) format improves efficacy both in vitro in SMA patient fibroblasts and in Δ7 SMA mice and reduces toxicity in mice.” It was observed in the preclinical study that nusinersen in SNA format prolonged survival by four-fold (maximal survival of 115 days compared to 28 days for nusinersen-treated mice) as well as doubled the levels of healthy full-length SMN2 mRNA and protein in SMA patient fibroblasts when compared to nusinersen. Based on the results of this preclinical study, we intend to further pursue our early stage research activities in neurological applications.

We presented pre-clinical data in a poster session at the 14th Annual Meeting of the Oligonucleotide Therapeutics Society(OTS) in Seattle, Washington from September 30-October 3, 2018. The first poster, authored and presented by Exicure scientists, titled “Spherical Nucleic Acids: Oral delivery and efficacy in TNBS-induced IBD model,” highlighted the local delivery benefits of our proprietary platform technology. The imaging data presented in the poster showed bio-distribution of SNAs following oral delivery in the GI tract of mice, including tissue penetration and cellular internalization. In addition, in a mouse model of inflammatory bowel disease, oral administration of SNAs showed improved clinical symptoms, colon gross pathology, body weight, and survival. A second poster showcased data generated as part of a scientific collaboration between the Company and Regulus Therapeutics. The second poster, titled “Spherical Nucleic Acid (SNA) construct improves functional delivery of

anti-*micro*RNA oligonucleotide in pre-clinical studies *in vitro* and *in vivo,*" highlighted improved delivery to the liver following systemic administration in mice and indicated the potential to broaden the application of our proprietary SNA technology to miRNA targeting.

Other operating, financing, and cash flow considerations

Since our inception in 2011, we have devoted substantial resources to the research and development of SNAs and the protection and enhancement of our intellectual property. We have no products approved for sale and all of our $15.6 million in revenue since inception through September 30, 2018 has been earned through our research collaboration, license, and option agreement with Purdue or as a primary contractor or as a subcontractor on government grants. In addition to our revenue, from inception through September 30, 2018, we have funded our operations through private placements of preferred stock with gross proceeds totaling $42.8 million, sales of common stock in the 2017 Private Placement with gross proceeds totaling $31.5 million, sales of common stock in the August 2018 Private Placement with gross proceeds totaling $22.0 million and debt financing totaling $6.0 million. As of September 30, 2018, our cash and cash equivalents were $32.4 million.

Since our inception, we have incurred significant operating losses. Our net loss was $5.3 million and $1.9 million for the three months ended September 30, 2018 and 2017, respectively, and $17.7 million and $7.6 million for the nine months ended September 30, 2018 and 2017, respectively. As of September 30, 2018, our accumulated deficit was $69.1 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research programs and from general and administrative costs associated with our operations.

We expect to continue to incur significant and increasing losses in the foreseeable future. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- conduct further preclinical studies and clinical trials of AST-008 and XCUR17;
- increase research and development for the discovery and development of additional therapeutic candidates;
- advance other therapeutic candidates into preclinical and clinical development;
- increase our research and development to enhance our technology;
- procure clinical trial materials;
- seek regulatory approval for our therapeutic candidates that successfully complete clinical trials;
- maintain, expand and protect our intellectual property portfolio;
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- operate as a public company.

We have not generated any commercial product revenue nor do we expect to generate substantial revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our therapeutic candidates. Successful therapeutic development and regulatory approval are subject to significant uncertainty and we expect will take at least five years. If we obtain regulatory approval for any of our therapeutic candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Other sources of revenue could include a combination of research and development payments, license fees and other upfront payments, milestone payments, and royalties in connection with our current and any future collaborations and licenses. Until such time, if ever, that we generate revenue from whatever source, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings and research collaboration and license agreements. We may be unable to raise capital or enter into such other arrangements when needed or on favorable terms. Our failure to raise capital or enter into such other arrangements

as and when needed would have a negative impact on our financial condition and our ability to develop our therapeutic candidates.

Recent Developments

August 2018 Private Placement

On August 22, 2018, we entered into subscription agreements with several accredited investors, pursuant to which we issued and sold a total of 4,889,217 shares of the Company's common stock, at a purchase price of $4.50 per share, resulting in approximately $22.0 million in gross proceeds to the Company (the "August 2018 Private Placement"). The aggregate net proceeds from the August 2018 Private Placement (after deducting accrued or paid placement agent fees and expenses of the offering of $1.9 million) were $20.1 million.

Segment Reporting

We view our operations and manage our business as one segment, which is the discovery, research and development of treatments based on our SNA technology.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with GAAP. The preparation of these 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 financial statements, as well as the revenue and expenses incurred during the reported periods. We base our estimates on 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 apparent from other sources. Changes in estimates are reflected in reported results for the period in which they become known. Actual results may differ from these estimates under different assumptions or conditions.

Our critical accounting policies require the most significant judgments and estimates in the preparation of our consolidated financial statements. There have been no significant changes to our critical accounting policies from those which were discussed in our Annual Report on Form 10-K for the year ended December 31, 2017, with the exception of our policy relating to revenue recognition.

As discussed in Note 2 of the accompanying unaudited condensed consolidated financial statements, we adopted Accounting Standards Update No. 2014-09, *Revenue from Contracts with Customers (Topic 606)* ("ASU 2014-09") effective January 1, 2018. Refer to Note 2 of the accompanying unaudited condensed consolidated financial statements for a description of policy related to revenue recognition.

Recently adopted accounting pronouncements

Refer to Note 2 of the accompanying unaudited condensed consolidated financial statements for a description of recently adopted accounting pronouncements.

Recent accounting pronouncements not yet adopted

Refer to Note 2 of the accompanying unaudited condensed consolidated financial statements for a description of recent accounting pronouncements not yet adopted.

Results of Operations

Comparison of the Three Months Ended September 30, 2018 and 2017

The following table summarizes the results of our operations for the three months ended September 30, 2018 and 2017:

	Three Months Ended September 30,			
(dollars in thousands)	**2018**	**2017**	**Change**	
Revenue:				
Collaboration revenue	$ 57	$ 2,497	$ (2,440)	(98)%
Total revenue	57	2,497	(2,440)	(98)%
Operating expenses:				
Research and development expense	4,001	3,121	880	28 %
General and administrative expense	1,919	1,270	649	51 %
Total operating expenses	5,920	4,391	1,529	35 %
Operating loss	(5,863)	(1,894)	(3,969)	210 %
Other income (expense), net:				
Interest expense	(170)	(201)	31	(15)%
Other income (loss), net	709	163	546	335 %
Total other income (loss), net	539	(38)	577	n/m
Net loss	$ (5,324)	$ (1,932)	$ (3,392)	176 %

Revenue

	Three Months Ended September 30,			
(dollars in thousands)	**2018**	**2017**	**Change**	
Collaboration revenue	$ 57	$ 2,497	$ (2,440)	(98)%
Total revenue	$ 57	$ 2,497	$ (2,440)	(98)%

Revenue in both the three months September 30, 2018 and 2017 related to the Purdue Collaboration, and decreased from $2.5 million for the three months ended September 30, 2017 to $0.1 million for the three months ended September 30, 2018, or approximately 98%, mostly due to the absence of revenue recognized in the prior period related to the amortization of the upfront payment, and certain reimbursable research and development activities, under the Purdue Collaboration. In connection with the Purdue Collaboration, we received a non-refundable development fee of $10.0 million in December 2016. Prior to the adoption of ASC 606, the upfront payment of $10.0 million was accounted for pursuant to ASC 605 and was recorded as deferred revenue and recognized on a ratable basis over the estimated performance period of the relevant research and development activities. On January 1, 2018, in connection with the adoption of ASC 606, we recorded the unamortized deferred revenue of $1.0 million as an adjustment to the beginning balance of accumulated deficit at January 1, 2018.

The collaboration revenue of $0.1 million during the three months ended September 30, 2018 related to research and development activities that is reimbursable by Purdue and is presented on a gross basis in the accompanying consolidated statement of operations. We recognized $2.5 million of collaboration revenue in the three months ended September 30, 2017, which consisted of three months of amortization of deferred revenue mentioned above and $0.4 million related to research and development activities that was reimbursed by Purdue and is presented on a gross basis in the accompanying consolidated statement of operations.

We do not expect to generate any product revenue for the foreseeable future. However, future revenue may include amounts attributable to partnership activities including, a combination of research and development payments, license fees and other upfront payments, milestone payments, product sales and royalties, and reimbursement of certain research and development expenses, in connection with the Purdue Collaboration or any future collaboration and licenses.

Research and development expense

The following table summarizes our research and development expenses incurred during the periods indicated:

	Three Months Ended September 30,			
(dollars in thousands)	**2018**	**2017**	**Change**	
Clinical development programs expense	$ 1,799	$ 1,458	$ 341	23%
Platform and discovery-related expense	972	821	151	18%
Employee-related expense	982	618	364	59%
Facilities, depreciation, and other expenses	248	224	24	11%
Total research and development expense	$ 4,001	$ 3,121	$ 880	28%
Full time employees	20	17	3	

Research and development expense was $4.0 million for the three months ended September 30, 2018 and $3.1 million for the three months ended September 30, 2017, an increase of $0.9 million, or 28%. The increase in research and development expense of $0.9 million was primarily due to higher employee-related expenses of $0.4 million, higher clinical development programs expense of $0.3 million, and higher platform and discovery-related expense of $0.2 million.

Higher employee-related expense of $0.4 million was due to higher compensation and related costs, non-cash stock-based compensation, and relocation costs all mostly in connection with the hire of our Chief Operating Officer as well as in connection with salary increases for existing employees.

The net increase in clinical development programs expense of $0.3 million was mostly due to higher costs for the preparation of a Phase 2 trial of XCUR17 as well as higher costs for the Phase 1 trial of XCUR17 and higher costs for the preparation of a Phase 1b/2 trial of AST-008. These increases were partially offset by lower costs associated with AST-005 (for which the Phase 1b clinical trial, subject of the Purdue Collaboration, ended during the first quarter of 2018). Included in clinical development expense for the three months ended September 30, 2018 and 2017 was approximately $0.1 million and $0.4 million, respectively, of expense that is reimbursed by Purdue (included in revenue) related to the Phase 1b trial of AST-005.

We expect our research and development expenses to increase in the fourth quarter of 2018 and in 2019 as we continue spending on our clinical development programs, further develop our SNA technology platform and broaden our pipeline of SNA-based candidates.

General and administrative expense

	Three Months Ended September 30,			
(dollars in thousands)	**2018**	**2017**	**Change**	
General and administrative expense	$ 1,919	$ 1,270	$ 649	51%
Full time employees	7	7	—	

General and administrative expense was $1.9 million for the three months ended September 30, 2018 and $1.3 million for the three months ended September 30, 2017, an increase of $0.6 million, or 51%. This increase is due to

higher costs associated with being a public company of $0.3 million, including higher expense for investor and public relations, director and officer insurance, and transfer agent, stock exchange and other regulatory compliance related matters, as well as higher legal costs of $0.2 million mostly related to 2018 financing activities, and higher compensation and related expense of $0.1 million associated with salary increases.

Interest expense

Interest expense consists of interest expense pursuant to the loan and security agreement with Hercules that we closed on February 17, 2016 with an initial advance of $6.0 million.

Other income (loss), net

Other income (loss), net consists of interest income earned on our cash and cash equivalents, fair value adjustments of our common stock warrant liabilities, and gains and losses on foreign currency transactions. The increase in other income (loss), net of $0.5 million is mostly due to a non-cash gain of $0.6 million recognized in the three month ended September 30, 2018 in connection with the fair value adjustment of our common stock warrant liability.

Comparison of the Nine Months Ended September 30, 2018 and 2017

The following table summarizes the results of our operations for the nine months ended September 30, 2018 and 2017:

	Nine Months Ended September 30,			
(dollars in thousands)	**2018**	**2017**	**Change**	
Revenue:				
Collaboration revenue	$ 112	$ 7,624	$ (7,512)	(99)%
Total revenue	112	7,624	(7,512)	(99)%
Operating expenses:				
Research and development expense	11,111	9,910	1,201	12 %
General and administrative expense	5,952	4,806	1,146	24 %
Total operating expenses	17,063	14,716	2,347	16 %
Operating loss	(16,951)	(7,092)	(9,859)	139 %
Other income (expense), net:				
Interest expense	(497)	(616)	119	(19)%
Other income (loss), net	(210)	140	(350)	n/m
Total other income (loss), net	(707)	(476)	(231)	49 %
Net loss	$ (17,658)	$ (7,568)	$ (10,090)	133 %

Revenue

	Nine Months Ended September 30,			
(dollars in thousands)	**2018**	**2017**	**Change**	
Collaboration revenue	$ 112	$ 7,624	$ (7,512)	(99)%
Total revenue	$ 112	$ 7,624	$ (7,512)	(99)%

Revenue in both the nine months September 30, 2018 and 2017 related to the Purdue Collaboration, and decreased from $7.6 million for the nine months ended September 30, 2017 to $112 thousand for the nine months ended September 30, 2018, or approximately 99%, mostly due to the absence of revenue recognized in the prior period related to the amortization of the upfront payment, and certain reimbursable research and development activities, under the Purdue Collaboration. In connection with the Purdue Collaboration, we received a non-refundable development fee of $10.0 million in December 2016. Prior to the adoption of ASC 606, the upfront payment of $10.0 million was accounted for pursuant to ASC 605 and was recorded as deferred revenue and recognized on a ratable basis over the estimated performance period of the relevant research and development activities. On January 1, 2018, in connection with the adoption of ASC 606, we recorded the unamortized deferred revenue of $1.0 million as an adjustment to the beginning balance of accumulated deficit at January 1, 2018.

The collaboration revenue of $0.1 million during the nine months ended September 30, 2018 related to research and development activities that is reimbursable by Purdue and is presented on a gross basis in the accompanying consolidated statement of operations. We recognized $7.6 million of collaboration revenue in the nine months ended September 30, 2017, which consisted of nine months of amortization of deferred revenue mentioned above and $1.4 million related to research and development activities that was reimbursed by Purdue and is presented on a gross basis in the accompanying consolidated statement of operations.

We do not expect to generate any product revenue for the foreseeable future. However, future revenue may include amounts attributable to partnership activities including, a combination of research and development payments, license fees and other upfront payments, milestone payments, product sales and royalties, and

reimbursement of certain research and development expenses, in connection with the Purdue Collaboration or any future collaboration and licenses.

Research and development expense

The following table summarizes our research and development expenses incurred during the periods indicated:

	Nine Months Ended September 30,			
(dollars in thousands)	2018	2017	Change	
Clinical development programs expense	$ 4,559	$ 4,852	$ (293)	(6)%
Platform and discovery-related expense	2,980	2,494	486	19 %
Employee-related expense	2,822	1,916	906	47 %
Facilities, depreciation, and other expenses	750	648	102	16 %
Total research and development expense	$ 11,111	$ 9,910	$ 1,201	12 %
Full time employees	20	17	3	

Research and development expense was $11.1 million for the nine months ended September 30, 2018 and $9.9 million for the nine months ended September 30, 2017, an increase of $1.2 million, or 12%. The increase in research and development expense of $1.2 million was primarily due to higher employee-related expenses of $0.9 million and higher platform and discovery-related expense of $0.5 million, partially offset by a net decrease in costs related to our clinical development programs of $0.3 million.

Higher employee-related expense of $0.9 million was due to higher compensation and related costs, non-cash stock-based compensation, and relocation costs all mostly in connection with the hire of our Chief Operating Officer as well as in connection with salary increases for existing employees. The increase in platform and discovery-related expense of $0.5 million is mostly due to higher costs to maintain our intellectual property portfolio.

The net decrease in clinical development programs expense of $0.3 million was mostly due to lower costs associated with AST-005 (for which the Phase 1b clinical trial, subject of the Purdue Collaboration, ended during the first quarter of 2018), partially offset by costs for the preparation of a Phase 2 trial of XCUR17. Included in clinical development expense for the nine months ended September 30, 2018 and 2017 was approximately $0.1 million and $1.4 million, respectively, of expense that is reimbursed by Purdue (included in revenue) related to the Phase 1b trial of AST-005.

We expect our research and development expenses to increase in the fourth quarter of 2018 and in 2019 as we continue spending on our clinical development programs, further develop our SNA technology platform and broaden our pipeline of SNA-based therapeutic candidates.

General and administrative expense

	Nine Months Ended September 30,			
(dollars in thousands)	2018	2017	Change	
General and administrative expense	$ 5,952	$ 4,806	$ 1,146	24%
Full time employees	7	7	—	

General and administrative expense was $6.0 million for the nine months ended September 30, 2018 and $4.8 million for the nine months ended September 30, 2017, an increase of $1.1 million, or 24%. This increase is due to higher costs associated with being a public company of $0.8 million, including higher expense for investor and public relations, director and officer insurance, and transfer agent, stock exchange and other regulatory compliance related matters, as well as higher compensation and related expense of $0.4 million associated with salary increases.

Interest expense

Interest expense consists of interest expense pursuant to the loan and security agreement with Hercules that we closed on February 17, 2016 with an initial advance of $6.0 million.

Other income (loss), net

Other income (loss), net consists of interest income earned on our cash and cash equivalents, fair value adjustments of our common stock warrant liabilities, and gains and losses on foreign currency transactions. The increase in other income (loss), net of $0.4 million is mostly due to a non-cash loss of $0.5 million recognized in the nine month ended September 30, 2018 in connection with the fair value adjustment of our common stock warrant liability.

Liquidity and Capital Resources

Overview

To date we have primarily funded our operations through private placements of equity securities, the Purdue Collaboration, a debt financing, and grants from governmental agencies. Since inception and through September 30, 2018, we have received approximately $117.3 million in aggregate gross proceeds from these transactions, including: $42.8 million in aggregate gross proceeds from private placement offerings of preferred stock; $31.5 million in gross proceeds from sales of common stock in the 2017 Private Placement; $22.0 million in gross proceeds from the sales of common stock in the August 2018 Private Placement; an upfront payment of $10.0 million in connection with the Purdue Collaboration; $6.0 million in debt financing; and an aggregate of $5.0 million from grants awarded by governmental agencies.

Since our inception, we have not generated any product revenue and have incurred recurring net losses. Our Company is not profitable, and we cannot provide any assurance that we will ever be profitable. As of September 30, 2018, we have an accumulated deficit of $69.1 million. Based on our current operating plans, existing working capital at September 30, 2018 is sufficient to sustain operations into the first quarter of 2020. Management believes that it will be able to obtain additional working capital through equity financings, partnerships and licensing, or other arrangements, to fund operations. However, there can be no assurance that such additional financing will be available and, if available, can be obtained on terms acceptable to us. If we are unable to obtain such additional financing, we will need to reevaluate future operating plans. Accordingly, there is substantial doubt regarding the Company's ability to continue as a going concern.

See "—Funding Requirements" below for additional information on our future capital needs.

Cash Flows

The following table shows a summary of our cash flows for the nine months ended September 30, 2018 and 2017:

(in thousands)	Nine Months Ended September 30,	
	2018	**2017**
	(unaudited)	(unaudited)
Net cash used in operating activities	$ (13,524)	$ (14,447)
Net cash used in investing activities	(65)	(726)
Net cash provided by financing activities	20,271	18,486
Net increase in cash and cash equivalents	$ 6,682	$ 3,313

Operating activities

Net cash used in operating activities was $13.5 million and $14.4 million for the nine months ended September 30, 2018 and 2017, respectively. The decrease in cash used in operating activities of $0.9 million was primarily due to the absence of the first quarter 2017 payment of $1.5 million in connection with the Northwestern University License Agreements, partially offset by higher cash used for working capital.

Investing activities

Net cash used in investing activities was $65 thousand and $0.7 million for the nine months ended September 30, 2018 and 2017, respectively. Cash used in investing activities for each of the nine months ended September 30, 2018 and 2017 was primarily due to the purchase of scientific equipment.

Financing activities

Net cash provided by financing activities of $20.3 million for the nine months ended September 30, 2018 is primarily due to the sale of common stock in the August 2018 Private Placement. On August 22, 2018, we sold 4,889,217 shares of the Company's common stock at a purchase price of $4.50 per share, resulting in approximately $22.0 million in gross proceeds to the Company. The aggregate net proceeds from the August 2018 Private Placement (after deducting accrued or paid placement agent fees and expenses of the offering of $1.9 million) were $20.1 million.

Net cash provided by financing activities of $18.5 million for the nine months ended September 30, 2017 is primarily due to the sale of common stock in the initial closing of the 2017 Private Placement. On September 26, 2017, we sold 6,767,360 shares of the Company's common stock at a purchase price of $3.00 per share, resulting in approximately $20.3 million in gross proceeds to the Company. The aggregate net proceeds from the initial closing of the 2017 Private Placement on September 26, 2017 (after deducting accrued or paid placement agent fees and expenses of $3.0 million) were $17.2 million. The increase in cash provided by financing activities in the nine months ended September 30, 2017 due to the initial closing of the 2017 Private Placement was partially offset by the repayment of debt of $0.6 million

Hercules Loan and Security Agreement

On February 17, 2016, we entered into a loan and security agreement with Hercules. The loan agreement provided for funding in an aggregate principal amount of up to $10.0 million in two separate tranches. The first tranche was funded on February 17, 2016 in the amount of $6.0 million. A second tranche of $4.0 million was available provided that we met certain milestones on or before December 31, 2016. We did not meet these milestones and, therefore, we did not draw the second tranche, the availability of which expired on December 31, 2016. The principal balance of the term loan under the Hercules loan facility bears interest at a floating per annum interest rate (based on a year consisting of 360 days) equal to the greater of either (i) 9.95% or (ii) the sum of (a) 9.95% plus (b) the prime rate (as reported in The Wall Street Journal) minus 3.50%. We were required to make interest-only payments through June 2017. Commencing on July 1, 2017, the loan began amortizing in equal monthly installments of principal and interest in an amount sufficient to fully amortize the outstanding principal balance of the loan over the remaining scheduled monthly payments due prior to the maturity date on September 1, 2019. Pursuant to an amendment dated January 15, 2018, amortization payments due for the thirteen (13) consecutive months commencing on December 1, 2017 through and including December 1, 2018 were deferred. Commencing on January 1, 2019, and continuing on the first business day of each month thereafter, the loan, including the deferred payments, shall begin amortizing in equal monthly installments of principal and interest based upon an amortization schedule equal to eighteen (18) consecutive months. Any remaining obligations under the loan agreement and other loan documents are due and payable on the maturity date. On the earliest to occur of the maturity date, the date we prepay the term loan in full or the date the loan otherwise becomes due and payable, we must pay the lender under the agreement an additional charge equal to 3.85% of the total amounts funded under the loan agreement. In addition, if we prepaid the term loan on or prior to February 1, 2017, we would have been required to pay a prepayment charge equal to 3% of the amount being prepaid, if we prepaid the term loan after February 1, 2017 but on or prior to February 1, 2018, we would have been required to pay a prepayment charge equal to 2% of the amount being prepaid, and if we prepay the term loan after February 1, 2018, we must pay a

prepayment charge of 1% of the amount being prepaid. As of the date of this Quarterly Report on Form 10-Q, we have not prepaid the Hercules term loan. The loan agreement was amended on October 10, 2016 to revise the language granting Hercules a contingent security interest in certain of our assets.

The term loan under the Hercules loan facility is secured by substantially all of our assets, other than intellectual property, which is the subject of a negative pledge. Under the loan agreement, we are subject to certain customary covenants that limit or restrict our ability to, among other things, incur additional indebtedness, grant any security interests, pay cash dividends, repurchase our common stock, make loans, or enter into certain transactions without Hercules' prior consent.

Under the loan agreement, Hercules or its affiliates have a right to participate in a single subsequent unregistered financing by us in an amount of up $1.0 million on the same terms, conditions and pricing afforded to others participating in such financing. Hercules has not yet exercised this right to participate which expires on the earliest to occur of the maturity date, the date we prepay the term loan in full or the date the loan otherwise becomes due and payable.

Funding Requirements

We expect that our primary uses of capital will continue to be third-party clinical research and development services, compensation and related expenses, laboratory and related supplies, legal and other regulatory expenses and general overhead costs. Based on our current operating plans, existing working capital at September 30, 2018 is sufficient to sustain operations into the first quarter of 2020. However, we may require additional capital for the further development of our existing therapeutic candidates and may also need to raise additional funds sooner to pursue other development activities related to additional therapeutic candidates. We believe that we will be able to obtain additional working capital through equity financings, partnerships and licensing, or other arrangements to fund our current operating plans, which we believe will allow us to execute on the strategy and pipeline development as described in this Quarterly Report on Form 10-Q. To the extent that we raise additional capital through future equity financings, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing common stockholders. If we raise additional funds through the issuance of debt securities, these securities could contain covenants that would restrict our operations. We cannot assure you that such additional financing, if available, can be obtained on terms acceptable to us. If we are unable to obtain such additional financing, we would need to reevaluate our future operating plans.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially as a result of a number of factors. 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. Our future capital requirements are difficult to forecast and will depend on many factors, including:

- the terms and timing of any other collaboration, licensing and other arrangements that we may establish;
- the initiation, progress, timing and completion of preclinical studies and clinical trials for our potential therapeutic candidates;
- the number and characteristics of therapeutic candidates that we pursue;
- the progress, costs and results of our preclinical studies and clinical trials;
- the outcome, timing and cost of regulatory approvals;
- delays that may be caused by changing regulatory requirements;
- the cost and timing of hiring new employees to support our continued growth;
- unknown legal, administrative, regulatory, accounting, and information technology costs as well as additional costs associated with operating as a public company;

- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims;
- the costs of filing and prosecuting intellectual property rights and enforcing and defending any intellectual property-related claims;
- the costs and timing of procuring clinical and commercial supplies of our therapeutic candidates;
- the extent to which we acquire or in-license other therapeutic candidates and technologies; and
- the extent to which we acquire or invest in other businesses, therapeutic candidates or technologies.

Please see the section titled "Risk Factors" elsewhere in this Quarterly Report on Form 10-Q for additional risks associated with our substantial capital requirements.

Until such time, if ever, we generate product revenue, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings and research collaboration and license agreements. We may be unable to raise capital or enter into such other arrangements when needed or on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our therapeutic candidates.

Contractual Obligations and Commitments

There have been no material changes outside the ordinary course of business during the period covered by this Quarterly Report on Form 10-Q from the contractual obligations and commitments discussed in our Annual Report on Form 10-K for the year ended December 31, 2017.

Off-balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

JOBS Act

In April 2012, the Jumpstart Our Business Startups Act of 2012 (the "JOBS Act") was enacted by the federal government. Section 107 of the JOBS Act provides that an emerging growth company can take advantage of the extended transition period for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

In addition, as an emerging growth company, we will not be required to provide an auditor's attestation report on our internal control over financial reporting in future annual reports on Form 10-K as otherwise required by Section 404(b) of the Sarbanes-Oxley Act.

Item 3. Quantitative and Qualitative Disclosures about Market Risk.

The primary objectives of our investment activities are to ensure liquidity and to preserve principal while at the same time maximizing the income we receive from our marketable securities without significantly increasing risk. Some of the securities that we invest in may have market risk related to changes in interest rates. As of September 30, 2018 and December 31, 2017, we had cash equivalents of $32.4 million and $25.8 million, respectively, consisting of interest-bearing money market accounts. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term maturities of our cash equivalents and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our cash equivalents. To minimize the risk in the future, we intend to maintain our portfolio of cash equivalents and short-term investments in a variety of securities, including commercial paper, money market funds, government and non-government debt securities and corporate obligations.

We are subject to interest rate risk in connection with our borrowings under the $6.0 million term loan with Hercules. The principal balance of the term loan under the Hercules loan facility bears interest at a floating per annum interest rate (based on a year consisting of 360 days) equal to the greater of (i) 9.95% or (ii) the sum of (a) 9.95% plus (b) the prime rate (as reported in The Wall Street Journal) minus 3.50% which bears interest at a variable per annum rate calculated for any day as the greater of (i) the prime rate plus 6.80%, and (ii) 10.55%. We currently do not engage in any interest rate hedging activity and we have no intention to do so in the foreseeable future. Based on the current interest rate of the term loan with Hercules and the scheduled payments thereunder, we believe a 100 basis point increase in interest rates would not have a material impact on our financial condition or results of operations.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

As we are an emerging growth company and a newly public company, we have not prepared a formal management's report on internal control over financial reporting, as would otherwise be required by Section 404 of the Sarbanes-Oxley Act of 2002, nor have we engaged an independent registered public accounting firm to perform an audit of our internal control over financial reporting as of any balance sheet date in our condensed consolidated interim financial statements. Our compliance with Section 404 of the Sarbanes-Oxley Act will first be subject to management's assessment regarding internal control over financial reporting in connection with the filing of our Annual Report on Form 10-K for the fiscal year ending December 31, 2018, and we will not be required to have an independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting until the filing of our first Annual Report on Form 10-K after we lose emerging growth company status, which may not be until the 2022 Annual Report on Form 10-K.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2018. 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 its 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 the evaluation of our disclosure controls and procedures as of September 30, 2018, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were not effective to provide reasonable assurance that all information required to be disclosed in reports filed or submitted under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, due to a material weakness in internal control over financial reporting. The material weakness related to a deficiency in the Company's information and communication controls, which led to ineffectively designed controls over management's review of certain research and development contracts to ensure expenses were recognized as incurred by third-party contract research organizations. Specifically these ineffectively designed controls, which arose in a prior period and were identified in the quarter ended March 31, 2018, resulted in an immaterial error, which was corrected in this Quarterly Report on Form 10-Q in previously issued financial statements as disclosed in Note 1 to the unaudited condensed consolidated interim financial statements for the period ended March 31, 2018.

Changes in Internal Control over Financial Reporting

During the quarter ended September 30, 2018, we worked towards remediating the deficiencies that led to the material weakness by enhancing the information used for periodic assessment of contract progress and increasing

the frequency of communication in the process for accounting for certain research and development contracts with contract research organizations to ensure expenses are recognized as incurred. The material weakness will not be considered remediated until the applicable remedial controls operate for a sufficient period of time and management has concluded, through testing, that these controls are operating effectively. We are working to remediate the material weakness as quickly and efficiently as possible and expect that the material weakness will be remediated by the end of fiscal 2018.

Other than the remediation efforts related to the material weakness discussed above, no changes occurred in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act during the fiscal quarter ended September 30, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we may be subject to legal proceedings. We are not currently a party to or aware of any proceedings that we believe will have, individually or in the aggregate, a material adverse effect on our business, financial condition or results of operations.

Item 1A. Risk Factors.

We are providing the following cautionary discussion of risk factors, uncertainties and assumptions that we believe are relevant to our business. These are factors that, individually or in the aggregate, we think could cause our actual results to differ materially from expected and historical results and our forward-looking statements. We note these factors for investors as permitted by Section 21E of the Exchange Act and Section 27A of the Securities Act. You should understand that it is not possible to predict or identify all such factors. Consequently, you should not consider this section to be a complete discussion of all potential risks or uncertainties that may substantially impact our business. Moreover, we operate in a competitive and rapidly changing environment. New factors emerge from time to time and it is not possible to predict the impact of all of these factors on our business, financial condition or results of operations.

Risks Related to Our Business

We are a clinical-stage biotechnology company with a history of losses. We expect to continue to incur significant losses for the foreseeable future and may never achieve or maintain profitability, which could result in a decline in the market value of our common stock.

We are a biotechnology company developing gene regulatory and immuno-oncology therapeutics based on our proprietary SNA technology. We have a limited operating history. Since our inception in June 2011, we have devoted our resources to the development of SNA technology. We have had significant operating losses since our inception. As of September 30, 2018, we have generated an accumulated deficit of $69.1 million. For the nine months ended September 30, 2018 and 2017, our net loss was $17.7 million and $7.6 million, respectively. Substantially all of our losses have resulted from expenses incurred in connection with our research programs and from general and administrative costs associated with our operations. Our technology and therapeutic candidates are in early stages of development, and we are subject to the risks of failure inherent in the development of therapeutic candidates based on novel technologies.

We have not generated, and do not expect to generate, any product revenue for the foreseeable future, and we expect to continue to incur significant operating losses for the foreseeable future due to the cost of research and development, preclinical studies, clinical trials, and the regulatory approval process for therapeutic candidates. The amount of future losses is uncertain. Our ability to achieve profitability, if ever, will depend on, among other things, us, or any current or future collaborators, successfully developing therapeutic candidates, obtaining regulatory approvals to market and commercialize therapeutic candidates, manufacturing any approved products on commercially reasonable terms, establishing a sales and marketing organization or suitable third party alternatives for any approved product and raising sufficient funds to finance business activities. If we, or any current or future collaborators, are unable to develop and commercialize one or more of our therapeutic candidates or if sales revenue from any therapeutic candidate that receives approval is insufficient, we will not achieve profitability, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our approach to the discovery and development of innovative therapeutic treatments based on our technology is unproven and may not result in marketable products.

We plan to develop a pipeline of therapeutic candidates using our proprietary SNAs as therapeutic agents. We believe that therapeutic candidates identified with our therapeutic discovery technology may offer an improved therapeutic approach to small molecules and antibodies, as well as several advantages over linear oligonucleotide-based therapeutics. However, the scientific research that forms the basis of our efforts to develop therapeutic candidates based on our SNAs and the identification and optimization of SNAs is relatively new. Further, the scientific evidence to support the feasibility of developing therapeutic treatments based on SNAs is both preliminary and limited.

Therapeutic candidates based on SNA technology have not been extensively tested in humans, and a number of clinical trials conducted by other companies using oligonucleotide technologies have not been successful. We may discover that the SNAs do not possess certain properties required for therapeutic treatment to be effective, such as the ability to remain stable in the human body for the period of time required for the therapeutic candidate to reach the target tissue or the ability to cross the cell membrane and enter into cells within the target tissue for effective delivery. We currently have only limited data, and no conclusive evidence, to suggest that we can introduce these necessary drug-like properties into SNAs. We may spend substantial funds attempting to introduce these properties and may never succeed in doing so. In addition, therapeutic candidates based on SNAs may demonstrate different chemical and pharmacological properties in patients than they do in laboratory studies. Even if therapeutic candidates have successful results in animal studies, they may not demonstrate the same chemical and pharmacological properties in humans and may interact with human biological systems in unforeseen, ineffective or harmful ways. As a result, we may never succeed in developing a marketable therapeutic, we may not become profitable and the value of our common stock would decline.

Further, the U.S. Food and Drug Administration (the "FDA"), has limited experience with SNA-based therapeutics. No regulatory authority has granted approval to any person or entity, including us, to market and commercialize therapeutics using SNAs, which may increase the complexity, uncertainty and length of the regulatory approval process for our therapeutic candidates. We and any current or future collaborators may never receive approval to market and commercialize any therapeutic candidate. Even if we or a future collaborator obtain regulatory approval, the approval may be for disease indications or patient populations that are not as broad as we intended or desired or may require labeling that includes significant use or distribution restrictions or safety warnings. We or a future collaborator may be required to perform additional or unanticipated clinical trials to obtain approval or be subject to post-marketing testing requirements to maintain regulatory approval. If our SNA technology proves to be ineffective, unsafe or commercially unviable, our technology and pipeline would have little, if any, value, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our therapeutic candidates are in early stages of development and may fail in development or suffer delays that materially and adversely affect their commercial viability.

We have no therapeutics on the market and all of our therapeutic candidates are in early stages of development. Our ability to achieve and sustain profitability depends on obtaining regulatory approvals, including an institutional review board ("IRB"), approval to conduct clinical trials at particular sites for, and successfully commercializing, our therapeutic candidates, either alone or with third parties. Before obtaining regulatory approval for the commercial distribution of our therapeutic candidates, we or an existing or a future collaborator must conduct extensive preclinical studies and clinical trials to demonstrate the safety and efficacy in humans of our therapeutic candidates. Preclinical studies and clinical trials are expensive, difficult to design and implement, can take many years to complete and are uncertain as to outcome. The start or end of a clinical trial is often delayed or halted due to changing regulatory requirements, manufacturing challenges, required clinical trial administrative actions, slower than anticipated patient enrollment, changing standards of care, availability or prevalence of use of a comparative therapeutic or required prior therapy, clinical outcomes or financial constraints. For instance, delays or difficulties in patient enrollment or difficulties in retaining trial participants can result in increased costs, longer development times or termination of a clinical trial. Clinical trials of a new therapeutic candidate require the enrollment of a sufficient number of patients, including patients who are suffering from the disease the therapeutic candidate is intended to treat and who meet other eligibility criteria. Rates of patient enrollment are affected by many factors, including the size of the patient population, the eligibility criteria for the clinical trial, the age and condition of the patients, the stage and severity of disease, the nature of the protocol, the proximity of patients to clinical sites and the availability of effective treatments for the relevant disease.

A therapeutic candidate can unexpectedly fail at any stage of preclinical and clinical development. In our completed Phase 1 trial, AST-005 did not show an antipsoriatic effect. There is no guarantee that AST-005 will show an antipsoriatic effect in future clinical trials of longer duration. The historical failure rate for therapeutic candidates is high due to scientific feasibility, safety, efficacy, changing standards of medical care and other variables. The results from preclinical studies or early clinical trials of a therapeutic candidate may not predict the results that will be obtained in later phase clinical trials of the therapeutic candidate. We, the FDA, an IRB, an independent ethics committee, or other applicable regulatory authorities may suspend clinical trials of a therapeutic candidate at any time for various reasons, including a finding that subjects participating in such trials are being exposed to unreasonable and significant risk of illness or injury. Similarly, an IRB or ethics committee may suspend a clinical trial at a particular trial site. We may not have the financial resources to continue development of, or to enter into collaborations for, a therapeutic candidate if we experience any problems or other unforeseen events that delay or prevent regulatory approval of, or our ability to commercialize, therapeutic candidates, including:

- negative or inconclusive results from our clinical trials or the clinical trials of others for therapeutic candidates similar to ours, leading to a decision or requirement to conduct additional preclinical testing or clinical trials or abandon a program;
- therapeutic-related side effects experienced by participants in our clinical trials or by individuals using therapeutics similar to our therapeutic candidates;
- delays in submitting INDs or CTAs, or comparable foreign applications or delays or failure in obtaining the necessary approvals from regulators or IRBs to commence a clinical trial, or a suspension or termination of a clinical trial once commenced;
- conditions imposed by the FDA or comparable foreign authorities, such as the European Medicines Agency ("EMA"), or European Union national competent authorities, regarding the scope or design of our clinical trials;
- delays in enrolling research subjects in clinical trials;
- high drop-out rates of research subjects;
- inadequate supply or quality of therapeutic candidate components or materials or other supplies necessary for the conduct of our clinical trials;
- greater than anticipated clinical trial costs;
- poor effectiveness of our therapeutic candidates during clinical trials;
- unfavorable FDA or other regulatory agency inspection and review of a clinical trial site;
- failure of our third party contractors or investigators to comply with regulatory requirements or otherwise meet their contractual obligations in a timely manner, or at all;
- delays and changes in regulatory requirements, policy and guidelines, including the imposition of additional regulatory oversight around clinical testing generally or with respect to our technology in particular, especially in light of the novelty of our therapeutic candidates;
- varying interpretations of data by the FDA and similar foreign regulatory agencies; or
- refusal of the FDA to accept data from clinical trials conducted outside the United States, or acceptance of these data subject to certain conditions by the FDA.

Product development involves a lengthy and expensive process with an uncertain outcome, and results of earlier preclinical studies and clinical trials may not be predictive of future clinical trial results.

Clinical testing is expensive and generally takes many years to complete, and the outcome is inherently uncertain. Failure can occur at any time and at any stage during the clinical trial process. The results of preclinical studies and early clinical trials of our therapeutic candidates may not be predictive of the result of any subsequent clinical trials. Therapeutic candidates that have shown promising results in early stage clinical trials may still suffer significant setbacks in subsequent clinical trials. We will have to conduct trials in our proposed indications to verify the results obtained to date and to support any regulatory submissions for further clinical development. A number of companies in the biopharmaceutical industry have suffered significant setbacks in clinical trials due to lack of efficacy or adverse safety

profiles despite promising results in earlier clinical trials. Moreover, clinical data is often susceptible to varying interpretations and analyses. We do not know whether Phase 1, Phase 2, Phase 3, or other clinical trials we may conduct will demonstrate consistent or adequate efficacy and safety with respect to the proposed indication for use sufficient to receive regulatory approval or market our therapeutic candidates. If we experience delays in the completion of, or termination of, any clinical trial of our therapeutic candidates, the commercial prospects of our therapeutic candidates may be harmed, and our ability to generate product revenues from any of these therapeutic candidates will be delayed. In addition, any delays in completing clinical trials will increase our costs, slow down our therapeutic candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences could materially and adversely affect our business, financial condition, results of operations or prospects.

We will need substantial additional funds to advance the development of our therapeutic candidates, and we cannot guarantee that we will have sufficient funds available in the future to develop and commercialize our current or future therapeutic candidates.

If our existing therapeutic candidates or our future therapeutic candidates enter and advance through preclinical studies and clinical trials, we will need substantial additional funds to expand our development, regulatory, manufacturing, marketing, and sales capabilities or contract with other organizations to provide these capabilities for us. We have used substantial funds to develop our therapeutic candidates and will require significant funds to conduct further research and development and preclinical studies and clinical trials of our therapeutic candidates, to seek regulatory approvals for our therapeutic candidates and to manufacture and market products, if any, that are approved for commercial sale. As of September 30, 2018 and December 31, 2017, we had $32.4 million and $25.8 million in cash and cash equivalents, respectively. Based on our current operating plans, existing working capital at September 30, 2018 is sufficient to sustain operations into the first quarter of 2020. Our future capital requirements and the period for which we expect our existing resources to support our operations may vary significantly from what we expect. Our monthly spending levels vary based on new and ongoing development and corporate activities. Since the length of time and activities associated with successful development of our therapeutic candidates is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities. To execute our business plan, we will need, among other things:

- to obtain the human and financial resources necessary to develop, test, obtain regulatory approval for, manufacture and market our therapeutic candidates;
- to build and maintain a strong intellectual property portfolio and avoid infringing the intellectual property of third parties;
- to establish and maintain successful licenses, collaborations and alliances;
- to satisfy the requirements of clinical trial protocols, including patient enrollment;
- to establish and demonstrate the clinical efficacy and safety of our therapeutic candidates;
- to obtain regulatory approvals;
- to manage our spending as costs and expenses increase due to preclinical studies and clinical trials, regulatory approvals, and commercialization;
- to obtain additional capital to support and expand our operations; and
- to market our products to achieve acceptance and use by the medical community in general.

If we are unable to obtain funding on a timely basis or on acceptable terms, we may have to delay, reduce or terminate our research and development programs and preclinical studies or clinical trials, if any, limit strategic opportunities or undergo reductions in our workforce or other corporate restructuring activities. We also could be required to seek funds through arrangements with collaborators or others that may require us to relinquish rights to some of our technology or therapeutic candidates that we would otherwise pursue on our own. We do not expect to realize revenue from product sales, milestone payments or royalties in the foreseeable future, if at all. Our revenue sources are, and will remain, extremely limited unless and until our therapeutic candidates are clinically tested, approved for commercialization and successfully marketed. To date, we have primarily financed our operations through the sale of equity securities, payments received in connection with our research collaboration, license, and option agreement with Purdue or as a primary contractor or as a subcontractor on government grants, and proceeds from our loan agreement with Hercules. We

will be required to seek additional funding in the future and intend to do so through either collaborations, public or private equity offerings or debt financings, credit or loan facilities or a combination of one or more of these funding sources. Our ability to raise additional funds will depend on financial, economic and other factors, many of which are beyond our control. Additional funds may not be available to us on acceptable terms or at all. If we raise additional funds by issuing equity securities, our stockholders will suffer dilution and the terms of any financing may adversely affect the rights of our stockholders. In addition, as a condition to providing additional funds to us, future investors may demand, and may be granted, rights superior to those of existing stockholders. Debt financing, if available, may involve restrictive covenants limiting our flexibility in conducting future business activities, and, in the event of insolvency, debt holders would be repaid before holders of equity securities received any distribution of corporate assets.

Our quarterly operating results may fluctuate significantly or may fall below the expectations of investors or securities analysts, each of which may cause our stock price to fluctuate or decline.

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expense related to our therapeutic candidates or future development programs;
- results of clinical trials, or the addition or termination of clinical trials or funding support by us, or a future collaborator or licensing partner;
- our execution of any collaboration, licensing or similar arrangement, and the timing of payments we may make or receive under such existing or future arrangements or the termination or modification of any such existing or future arrangements;
- any intellectual property infringement lawsuit or opposition, interference or cancellation proceeding in which we may become involved;
- additions and departures of key personnel;
- strategic decisions by us or our competitors, such as acquisitions, divestitures, spin-offs, joint ventures, strategic investments or changes in business strategy;
- whether or not any of our therapeutic candidates receives regulatory approval, market acceptance and demand for such therapeutic candidates;
- regulatory developments affecting our therapeutic candidates or those of our competitors; and
- changes in general market and economic conditions.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially.

We are dependent on Purdue for the successful development of therapeutic candidates in our collaboration arrangement with Purdue.

On December 2, 2016, we entered into a research collaboration, option and license agreement with Purdue. As part of the agreement, Purdue has the option to obtain from us the full worldwide development and commercial rights to AST-005, an option to obtain three additional collaboration targets and a further option to obtain from us the full worldwide development and commercial rights to any therapeutic candidates developed targeting the three additional collaboration targets. In April 2018, Purdue notified the Company it had declined to exercise its option to develop AST-005 at that time, but that it also intended to retain rights relating to the TNF target, and Purdue reserved its right to continue joint development with Exicure of new anti-TNF drug candidates and to retain its exclusivity and other rights to AST-005. Additionally, Purdue has rights of first offer to some potential collaboration targets. These rights of first offer are subject to limitations in time and scope. Purdue has not indicated that it has any plans to pursue AST-005 at this time. In December 2016, in connection with the Purdue Collaboration, we received a non-refundable development fee of $10.0 million. In addition, we are eligible to receive up to $776.5 million upon successful completion of certain research, regulatory and commercial sales milestones. There can be no assurance these milestones will be achieved as they are subject to highly significant risks and uncertainties, many of which are outside of our control. In the event a therapeutic candidate subject to the collaboration results in commercial sales, we are eligible to receive royalties ranging from the low single digits to a maximum of 10% on future net sales of such commercialized therapeutic candidates.

The success of our collaboration programs with Purdue depends largely upon the efforts of Purdue. Purdue has sole discretion in determining and directing the efforts and resources, including the ability to discontinue all efforts and resources, it applies to the development and, if approval is obtained, commercialization and marketing of the therapeutic candidates covered by the collaboration. Purdue may not be effective in obtaining approvals for the therapeutic candidates developed under the collaboration arrangement or marketing or arranging for necessary supply, manufacturing or distribution relationships for any approved products. Purdue may change its strategic focus or pursue alternative technologies in a manner that results in reduced, delayed or no revenue to us. Purdue has a variety of marketed products, and its own corporate objectives may not be consistent with our best interests. If Purdue fails to develop, obtain regulatory approval for or ultimately commercialize any therapeutic candidate under our collaboration or if Purdue terminates our collaboration, our business, financial condition, results of operations and prospects could be materially and adversely affected. In addition, any dispute or litigation proceedings we may have with Purdue in the future could delay development programs, create uncertainty as to ownership of intellectual property rights, distract management from other business activities and generate substantial expense.

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

We rely on third party clinical investigators, contract research organizations ("CROs"), clinical data management organizations and consultants to design, conduct, supervise and monitor preclinical studies and clinical trials for our therapeutic candidates. Because we rely on third parties and do not have the ability to conduct preclinical studies or clinical trials independently, we have less control over the timing, quality and other aspects of preclinical studies and clinical trials than we would if we conducted them on our own. These investigators, CROs 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 away from our programs. The third parties with which we contract might not be diligent, careful or timely in conducting our preclinical studies or clinical trials, resulting in the preclinical studies or clinical trials being delayed or unsuccessful.

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. The FDA requires preclinical studies to be conducted in accordance with applicable Good Laboratory Practices ("GLPs"), and clinical trials to be conducted in accordance with applicable FDA regulations and Good Clinical Practices ("GCPs"), including requirements for conducting, recording and reporting the results of preclinical studies and clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Any adverse development or delay in our preclinical studies or clinical trials could have a material adverse effect on our business, financial condition, results of operations and prospects.

Because we rely on third party manufacturing and supply partners, our supply of research and development, preclinical studies and clinical trial materials may become limited or interrupted or may not be of satisfactory quantity or quality.

We rely on third party partners to manufacture and supply the materials and components for our research and development, preclinical study and clinical trial supplies. We do not own manufacturing facilities or supply sources for such components and materials. Our manufacturing requirements include oligonucleotides and lipids. We procure our nonclinical toxicology and clinical development materials from a single source supplier on a purchase order basis. There can be no assurance that our supply of research and development, preclinical study and clinical trial therapeutic candidates and other materials will not be limited, interrupted, restricted in certain geographic regions or of satisfactory quality or continue to be available at acceptable prices. In particular, any replacement of our drug product manufacturers could require significant effort and expertise because there may be a limited number of qualified replacements.

The manufacturing process for a therapeutic candidate is subject to oversight by the FDA and foreign regulatory authorities. Suppliers and manufacturers must meet applicable manufacturing requirements and undergo rigorous facility and process validation tests required by regulatory authorities in order to comply with regulatory requirements, such as current Good Manufacturing Practices ("cGMPs"). In the event that any of our suppliers or manufacturers fails to comply

with such requirements or to perform its obligations to us in relation to quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may be forced to manufacture the materials ourselves, for which we currently do not have the capabilities or resources, or enter into an agreement with another third party, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills or technology required to manufacture our therapeutic candidates may be unique or proprietary to the original manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills or technology to another third party and a feasible alternative may not exist. These factors would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another third party manufacture our therapeutic candidates. If we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop therapeutic candidates in a timely manner or within budget.

We expect to continue to rely on third party manufacturers if we receive regulatory approval for any therapeutic candidate. To the extent that we have existing, or enter into future, manufacturing arrangements with third parties, we will depend on these third parties to perform their obligations in a timely manner consistent with contractual and regulatory requirements, including those related to quality control and assurance. If we are unable to obtain or maintain third party manufacturing for therapeutic candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our therapeutic candidates successfully. Our or a third party's failure to execute on our manufacturing requirements and comply with cGMP could adversely affect our business in a number of ways, including:

- an inability to initiate or continue preclinical studies or clinical trials of our therapeutic candidates under development;
- delay in submitting regulatory applications, or receiving regulatory approvals, for therapeutic candidates;
- loss of the cooperation of a future collaborator;
- subjecting manufacturing facilities of our therapeutic candidates to additional inspections by regulatory authorities;
- requirements to cease distribution or to recall batches of our therapeutic candidates; and
- in the event of approval to market and commercialize a therapeutic candidate, an inability to meet commercial demands for our therapeutics.

We may not successfully engage in strategic transactions, including any collaborations we seek, which could adversely affect our ability to develop and commercialize therapeutic candidates, impact our cash position, increase our expense, and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as collaborations, acquisitions of companies, asset purchases and out- or in-licensing of therapeutic candidates or technologies. In particular, we will evaluate and, if strategically attractive, seek to enter into collaborations, including with major biotechnology or pharmaceutical companies. The competition for collaborators is intense, and the negotiation process is time-consuming and complex. Any new collaboration may be on terms that are not optimal for us, and we may be unable to maintain any new collaboration if, for example, development or approval of a therapeutic candidate is delayed, sales of an approved therapeutic candidate do not meet expectations or the collaborator terminates the collaboration. Any such collaboration, or other strategic transaction, may require us to incur non-recurring or other charges, increase our near- and long-term expenditures and pose significant integration or implementation challenges or disrupt our management or business. These transactions would entail numerous operational and financial risks, including exposure to unknown liabilities, disruption of our business and diversion of our management's time and attention in order to manage a collaboration or develop acquired therapeutics, therapeutic candidates or technologies, incurrence of substantial debt or dilutive issuances of equity securities to pay transaction consideration or costs, higher than expected collaboration, acquisition or integration costs, write-downs of assets or goodwill or impairment charges, increased amortization expenses, difficulty and cost in facilitating the collaboration or combining the operations and personnel of any acquired business, impairment of relationships with key suppliers, manufacturers or customers of any acquired business due to changes in management and ownership and the inability to retain key employees of any acquired business. Accordingly, although there can be no assurance that we will undertake or successfully complete any transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks and may have a material adverse effect on our business, results of operations, financial condition and prospects. Conversely, any failure to enter into any collaboration or other strategic transaction that would be beneficial to us could delay the development and potential commercialization of

our therapeutic candidates and have a negative impact on the competitiveness of any therapeutic candidate that reaches market.

We face competition from entities that have developed or may develop therapeutic candidates for our target disease indications, including companies developing novel treatments and technology platforms based on modalities and technology similar to ours. If these companies develop technologies, including delivery technologies, or therapeutic candidates more rapidly than we do or their technologies are more effective, our ability to develop and successfully commercialize therapeutic candidates may be adversely affected.

The development and commercialization of therapeutic candidates is highly competitive. We compete with a number of multinational pharmaceutical companies and specialized biotechnology companies, as well as technology being developed at universities and other research institutions. Our competitors have developed, are developing or will develop therapeutic candidates and processes competitive with our therapeutic candidates. Competitive therapeutic treatments include those that have already been approved and accepted by the medical community and any new treatments that enter the market. We believe that a significant number of therapeutics are currently under development, and may become commercially available in the future, for the treatment of conditions for which we may try to develop therapeutic candidates. There is intense and rapidly evolving competition in the biotechnology, pharmaceutical and oligonucleotide therapeutics fields. While we believe that our SNA technology, its associated intellectual property and our scientific and technical know-how give us a competitive advantage in this space, competition from many sources remains. Our competitors include larger and better funded pharmaceutical, biotechnology and oligonucleotide therapeutics companies. Moreover, we also compete with current and future therapeutics developed at universities and other research institutions.

We are aware of several companies that are developing oligonucleotide delivery platforms and oligonucleotide-based therapeutics. These competitors include Ionis Pharmaceuticals, Inc., Alnylam Pharmaceuticals, Inc., Dicerna Pharmaceuticals, Inc., Arbutus Biopharma Corp., Wave Life Sciences Ltd., Dynavax Technologies Corp., Idera Pharmaceuticals, Inc., Mologen AG, and Checkmate Pharmaceuticals, Inc. These and other competitors compete with us in recruiting scientific and managerial talent, and for funding from pharmaceutical companies.

Our success will partially depend on our ability to develop and protect therapeutics that are safer and more effective than competing therapeutics. Our commercial opportunity and success will be reduced or eliminated if competing therapeutics are safer, more effective, or less expensive than the therapeutics we develop.

If our therapeutic candidates are approved for the indications we are currently pursuing, they will compete with a range of therapeutic treatments that are either in development or currently marketed. A number of therapeutics for treating psoriasis and cancers are on the market or in clinical development. For the treatment of psoriasis, marketed therapies range from small molecules like topical steroids to biologics, such as AbbVie Inc.'s adalimumab. In addition, numerous compounds are in clinical development for psoriasis treatment. With respect to immunogenic cancers such as melanoma, the most common treatments are chemotherapeutic compounds, radiation therapy and now immunotherapeutic antibodies such as ipilimumab, atezolizumab and pembrolizumab.

Many of our competitors have significantly greater financial, technical, manufacturing, marketing, sales and supply resources or experience than we do. If we successfully obtain approval for any therapeutic candidate, we will face competition based on many different factors, including the safety and effectiveness of our therapeutics, the ease with which our therapeutics can be administered and the extent to which patients accept relatively new routes of administration, the timing and scope of regulatory approvals for these therapeutics, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Competing therapeutics could present superior treatment alternatives, including by being more effective, safer, less expensive or marketed and sold more effectively than any therapeutics we may develop. Competitive therapeutics may make any therapeutics we develop obsolete or noncompetitive before we recover the expense of developing and commercializing our therapeutic candidates. Such competitors could also recruit our employees, which could negatively impact our level of expertise and our ability to execute our business plan.

The market may not be receptive to our therapeutic candidates based on a novel therapeutic modality, and we may not generate any future revenue from the sale or licensing of therapeutic candidates.

Even if approval is obtained for a therapeutic candidate, we may not generate or sustain revenue from sales of the product due to factors such as whether the product can be sold at a competitive cost and otherwise accepted in the market. The therapeutic candidates that we are developing are based on our SNA technology. Market participants with significant influence over acceptance of new treatments, such as physicians and third party payors, may not adopt a treatment based on SNA technology, and we may not be able to convince the medical community and third party payors to accept and use,

or to provide favorable reimbursement for, any therapeutic candidates developed by us or any current or future collaborators. Market acceptance of our therapeutic candidates will depend on, among other factors:

- the timing of our receipt of any marketing and commercialization approvals;
- the terms of any approvals and the countries in which approvals are obtained;
- the safety and efficacy of our therapeutic candidates;
- the prevalence and severity of any adverse side effects associated with our therapeutic candidates;
- limitations or warnings contained in any labeling approved by the FDA or other regulatory authority;
- relative convenience and ease of administration of our therapeutic candidates;
- the willingness of patients to accept any new methods of administration;
- the success of our physician education programs;
- the availability of adequate government and third party payor reimbursement;
- the pricing of our products, particularly as compared to alternative treatments; and
- availability of alternative effective treatments for indications our therapeutic candidates are intended to treat and the relative risks, benefits and costs of those treatments.

With our focus on SNAs, these risks may increase to the extent the space becomes more competitive or less favored in the commercial marketplace. Additional risks apply in relation to any disease indications we may pursue which are classified as rare diseases and allow for orphan drug designation by regulatory agencies in major commercial markets, such as the U.S., Europe and Japan. Because of the small patient population for a rare disease, if pricing is not approved or accepted in the market at an appropriate level for an approved product with orphan drug designation, such therapeutic may not generate enough revenue to offset costs of development, manufacturing, marketing and commercialization despite any benefits received from the orphan drug designation, such as market exclusivity, assistance in clinical trial design or a reduction in user fees or tax credits related to development expense. Market size is also a variable in disease indications not classified as rare. Our estimates regarding potential market size for any indication may be materially different from what we discover to exist at the time we commence commercialization, if any, for a therapeutic, which could result in significant changes in our business plan and have a material adverse effect on our business, financial condition, results of operations and prospects.

If a therapeutic candidate that has orphan drug designation subsequently receives the first FDA approval for the indication for which it has such designation, the therapeutic candidate is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same therapeutic candidate for the same indication, except in very limited circumstances, for seven years. Orphan drug exclusivity, however, could also block the approval of one of our therapeutic candidates for seven years if a competitor obtains approval of the same therapeutic candidate as defined by the FDA or if our therapeutic candidate is determined to be contained within the competitor's therapeutic candidate for the same indication or disease.

As in the U.S., we may apply for designation of a therapeutic candidate as an orphan drug for the treatment of a specific indication in the European Union before the application for marketing authorization is made. Sponsors of orphan drugs in the European Union can enjoy economic and marketing benefits, including up to ten years of market exclusivity for the approved indication. During such period, marketing applications for similar medicinal products will not be accepted, unless certain exceptions apply. In the EU, a "similar medicinal product" is a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication.

Any inability to attract and retain qualified key management and technical personnel would impair our ability to implement our business plan.

Our success largely depends on the continued service of key management and other specialized personnel, including David A. Giljohann, Ph.D., our Chief Executive Officer, David S. Snyder, our Chief Financial Officer, Ekambar Kandimalla, Ph.D., our Chief Scientific Officer, and Matthias G. Schroff, our Chief Operating Officer. The loss of one or more members of our management team or other key employees or advisors could delay our research and development

programs and materially harm our business, financial condition, results of operations and prospects. The relationships that our key managers have cultivated within our industry make us particularly dependent upon their continued employment with us. We are dependent on the continued service of our technical personnel because of the highly technical nature of our therapeutic candidates and our technology and the specialized nature of the regulatory approval process. Because our management team and key employees are not obligated to provide us with continued service, they could terminate their employment with us at any time without penalty. We do not maintain key person life insurance policies on any of our management team members or key employees. Our future success will depend in large part on our continued ability to attract and retain highly qualified scientific, technical and management personnel, as well as personnel with expertise in clinical testing, manufacturing, governmental regulation and commercialization. We face competition for personnel from other companies, universities, public and private research institutions, government entities and other organizations.

If our therapeutic candidates advance into clinical trials, we may experience difficulties in managing our growth and expanding our operations.

We have limited experience in therapeutic development and limited experience with clinical trials of therapeutic candidates. As our therapeutic candidates enter and advance through preclinical studies and clinical trials, we will need to expand our development, regulatory and manufacturing capabilities or contract with other organizations to provide these capabilities for us. In the future, we expect to have to manage additional relationships with collaborators or partners, suppliers and other organizations. Our ability to manage our operations and future growth will require us to continue to improve our operational, financial and management controls, reporting systems and procedures. We may not be able to implement improvements to our management information and control systems in an efficient or timely manner and may discover deficiencies in existing systems and controls.

If any of our therapeutic candidates are approved for marketing and commercialization and we are unable to develop sales, marketing and distribution capabilities on our own or enter into agreements with third parties to perform these functions on acceptable terms, we will be unable to successfully commercialize any such future therapeutics.

We currently have no sales, marketing or distribution capabilities or experience. If any of our therapeutic candidates is approved, we will need to develop internal sales, marketing and distribution capabilities to appropriately commercialize such therapeutics, which would be expensive and time-consuming, or enter into collaborations with third parties to perform these services. If we decide to market our approved therapeutics directly, we will need to commit significant financial and managerial resources to develop a marketing and sales force with technical expertise and supporting distribution, administration and compliance capabilities. If we rely on third parties with such capabilities to market our approved therapeutics or decide to co-promote therapeutics with collaborators, we will need to establish and maintain compliant marketing and distribution arrangements with third parties, and there can be no assurance that we will be able to enter into such arrangements on acceptable terms or at all. In entering into third party marketing or distribution arrangements, any revenue we receive will depend upon the efforts of the third parties and there can be no assurance that such third parties will establish adequate sales and distribution capabilities or be successful in gaining market acceptance of any approved therapeutic. If we are not successful in commercializing any therapeutic approved in the future, either on our own or through third parties, our business, financial condition, results of operations and prospects could be materially and adversely affected.

If we fail to comply with U.S. or foreign regulatory requirements, regulatory authorities could withhold marketing or commercialization approvals, limit or withdraw any marketing or commercialization approvals we may receive and subject us to other penalties that could materially harm our business.

We and our therapeutic candidates, as well as our suppliers, contract manufacturers, distributors, and contract testing laboratories are subject to extensive regulation by governmental authorities in the European Union, the U.S., and other countries, with the regulations differing from country to country.

If we or current or future collaborators, manufacturers or service providers fail to comply with applicable requirements, these regulatory authorities could refuse to issue necessary approvals for marketing and commercialization. Even if we receive marketing and commercialization approval of a therapeutic candidate, we and our third party service providers will be subject to continuing regulatory requirements, including a broad array of regulations related to establishment, registration and product listing, manufacturing processes, risk management measures, quality and pharmacovigilance systems, pre- and post-approval clinical data, labeling, advertising and promotional activities for such therapeutic, record keeping, distribution, and import and export of therapeutics for any therapeutic for which we obtain marketing approval. We are required to submit safety and other post market information and reports and are subject to continuing regulatory review, including in relation to adverse patient experiences with the therapeutic and clinical results that are reported after a therapeutic is made commercially available, both in the U.S. and any foreign jurisdiction in which

we seek regulatory approval. The FDA and certain foreign regulatory authorities, such as the EMA, have significant post-market authority, including the authority to require labeling changes based on new safety information and to require post-market studies or clinical trials to evaluate safety risks related to the use of a therapeutic or to require withdrawal of the therapeutic from the market. The FDA also has the authority to require a Risk Evaluation and Mitigation Strategies, or REMS, plan either before or after approval, which may impose further requirements or restrictions on the distribution or use of an approved therapeutic. The EMA now routinely requires risk management plans, or RMPs, as part of the marketing authorization application process, and such plans must be continually modified and updated throughout the lifetime of the product as new information becomes available. In addition, for nationally authorized medicinal products, the relevant governmental authority of any European Union member state can request an RMP whenever there is a concern about the risk/benefit balance of the product.

The manufacturer and manufacturing facilities we use to make a future therapeutic, if any, will also be subject to periodic review and inspection by the FDA and other regulatory agencies, including for continued compliance with cGMP requirements. The discovery of any new or previously unknown problems with our third party manufacturers, manufacturing processes or facilities may result in restrictions on the therapeutic, manufacturer or facility, including withdrawal of the therapeutic from the market. If we rely on third party manufacturers, we will not have control over compliance with applicable rules and regulations by such manufacturers. Any product promotion and advertising will also be subject to regulatory requirements and continuing regulatory review. If we or our future collaborators, manufacturers or service providers fail to comply with applicable continuing regulatory requirements in the U.S. or foreign jurisdictions in which we seek to market our therapeutics, we or they may be subject to, among other things, fines, warning and untitled letters, clinical holds, delay or refusal by the FDA or foreign regulatory authorities to approve pending applications or supplements to approved applications, suspension, refusal to renew or withdrawal of regulatory approval, recalls, seizures or administrative detention of products, refusal to permit the import or export of therapeutics, operating restrictions, inability to participate in government programs including Medicare and Medicaid, and total or partial suspension of production or distribution, injunction, restitution, disgorgement, debarment, civil and criminal penalties and criminal prosecution.

Price controls imposed in foreign markets may adversely affect our future profitability.

In some countries, particularly member states of the European Union, the pricing of prescription drugs is subject to governmental control at the national level, and in some cases also at the regional level. In these countries, pricing negotiations with governmental authorities can take considerable time after receipt of marketing approval for a therapeutic. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing and reimbursement negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we or current or future collaborators may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our SNA therapeutic candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If reimbursement of any therapeutic candidate approved for marketing is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business, financial condition, results of operations or prospects could be adversely affected.

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

Our business exposes us to significant product liability risks inherent in the development, testing, manufacturing and marketing of therapeutic treatments. Product liability claims could delay or prevent completion of our development programs. If we succeed in marketing therapeutics, such claims could result in an investigation by certain regulatory authorities, such as the FDA or foreign regulatory authorities, of the safety and effectiveness of our therapeutics, our manufacturing processes and facilities or our marketing programs and potentially a recall of our therapeutics or more serious enforcement action, limitations on the approved indications for which they may be used or suspension or withdrawal of approvals. Regardless of the merits or eventual outcome, liability claims may also result in decreased demand for our therapeutics, injury to our reputation, costs to defend the related litigation, a diversion of management's time and our resources, substantial monetary awards to trial participants or patients and a decline in our stock price. We currently have product liability insurance that we believe is appropriate for our stage of development and may need to obtain higher levels of product liability insurance prior to marketing any of our therapeutic 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 adverse effect on our business.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements which could have an adverse effect on our business.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include, but is not limited to, intentional failures to comply with FDA, the Centers for Medicare and Medicaid Services ("CMS"), the Department of Health and Human Services ("HHS"), Office of Inspector General ("OIG") or other agency regulations, applicable laws, regulations, guidance or codes of conduct set by foreign governmental authorities or self-regulatory industry organizations, or provide accurate information to any governmental authorities, such as the FDA, comply with manufacturing standards we may establish, comply with federal and state healthcare fraud and abuse laws and regulations, 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, regulations, guidance and codes of conduct intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws, regulations, guidance and codes of conduct may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements.

Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions, including, fines, debarment, or disqualification of those employees from participation in certain government-regulated activities, and serious harm to our reputation. This could include violations of the U.S. federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive.

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 be in compliance with such laws, regulations, guidance or codes of conduct. 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 exclusion from participation in the U.S. federal healthcare programs, the imposition of significant fines or other sanctions.

Compliance with governmental regulations regarding the treatment of animals used in research could increase our operating costs, which would adversely affect the commercialization of our technology.

The Animal Welfare Act, or AWA, is the federal law that covers the treatment of certain animals used in research. Currently, the AWA imposes a wide variety of specific regulations that govern the humane handling, care, treatment and transportation of certain animals by producers and users of research animals, most notably relating to personnel, facilities, sanitation, cage size, and feeding, watering and shipping conditions. Third parties with whom we contract are subject to registration, inspections and reporting requirements under the AWA. Furthermore, some states have their own regulations, including general anti-cruelty legislation, which establish certain standards in handling animals. Comparable rules, regulations, and or obligations exist in many foreign jurisdictions. If we or our contractors fail to comply with regulations concerning the treatment of animals used in research, we may be subject to fines and penalties and adverse publicity, and our operations could be adversely affected.

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

Despite the implementation of security measures, our internal computer systems and those of our CROs and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Such events could cause interruptions of our operations. For instance, the loss of preclinical study or clinical trial data involving our therapeutic candidates could result in delays in our development and regulatory filing efforts and significantly increase our costs. In addition, theft or other exposure of data may interfere with our ability to protect our intellectual property, trade secrets, and other information critical to our operations. We can provide no assurances that certain sensitive and proprietary information relating to one or more of our therapeutic candidates has not been, or will not in the future be, compromised. Although we have invested resources to enhance the security of our computer systems, there can be no assurances we will not experience additional unauthorized intrusions into our computer systems, or those of our CROs and other contractors and consultants, that we will

successfully detect future unauthorized intrusions in a timely manner, or that future unauthorized intrusions will not result in material adverse effects on our financial condition, reputation, or business prospects. Payments related to the elimination of ransomware may materially affect our financial condition and results of operations.

Certain data breaches must also be reported to affected individuals and the government, and in some cases to the media, under provisions of HIPAA, as amended by HITECH, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive. Financial penalties may also apply in some data breaches where noncompliance with the applicable law is identified.

To the extent that any disruption or security breach were to result in a loss of, or damage to, our data, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the development of our therapeutic candidates could be delayed.

We are subject to European data protection laws, including the new EU General Data Protection Regulation 2016/679, or GDPR. If we fail to comply with existing or future data protection regulations, our business, financial condition, results of operations and prospects may be materially adversely affected.

By virtue of our clinical trial activities in the United Kingdom and Germany, we are subject to European data protection laws, including GDPR. The GDPR which came into effect on May 25, 2018, establishes new requirements applicable to the processing of personal data (*i.e*., data which identifies an individual or from which an individual is identifiable), affords new data protection rights to individuals (e.g., the right to erasure of personal data) and imposes penalties for serious breaches of up to 4% annual worldwide turnover or €20 million, whichever is greater. Individuals (*e.g*., study subjects) also have a right to compensation for financial or non-financial losses (*e.g*., distress). There may be circumstances under which a failure to comply with GDPR, or the exercise of individual rights under the GDPR, would limit our ability to utilize clinical trial data collected on certain subjects. The GDPR will likely impose additional responsibility and liability in relation to our processing of personal data. This may be onerous and materially adversely affect our business, financial condition, results of operations and prospects.

If we do not comply with laws regulating the protection of the environment and health and human safety, our business could be adversely affected.

Our research, development and manufacturing involve the use of hazardous materials and various chemicals. We maintain quantities of various flammable and toxic chemicals in our facilities in Skokie, Illinois that are required for our research, development and manufacturing activities. We are subject to federal, state and local laws and regulations governing the use, manufacture, storage, handling and disposal of these hazardous materials. We believe our procedures for storing, handling and disposing these materials in our Skokie facilities comply with the relevant guidelines of Skokie, the state of Illinois, and the Occupational Safety and Health Administration of the U.S. Department of Labor. Although we believe that our safety procedures for handling and disposing of these materials comply with the standards mandated by applicable regulations, the risk of accidental contamination or injury from these materials cannot be eliminated. If an accident occurs, we could be held liable for resulting damages, which could be substantial. We are also subject to numerous environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of animals and biohazardous materials. 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 these materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological or hazardous materials. Additional federal, state and local laws and regulations affecting our operations may be adopted in the future. We may incur substantial costs to comply with, and substantial fines or penalties if we violate, any of these laws or regulations.

Our information technology systems could face serious disruptions that could adversely affect our business.

Our information technology and other internal infrastructure systems, including corporate firewalls, servers, leased lines and connection to the Internet, face the risk of systemic failure that could disrupt our operations. A significant disruption in the availability of our information technology and other internal infrastructure systems could cause interruptions and delays in our research and development work.

Our current operations are concentrated in one location and any events affecting this location may have material adverse consequences.

Our current operations are located in our facilities situated in Skokie, Illinois. Any unplanned event, such as flood, fire, explosion, earthquake, extreme weather condition, medical epidemics, power shortage, telecommunication failure or other natural or man-made accidents or incidents that result in us being unable to fully utilize the facilities, may have a material adverse effect on our ability to operate our business, particularly on a daily basis, and have significant negative consequences on our financial and operating conditions. Loss of access to these facilities may result in increased costs, delays in the development of our therapeutic candidates or interruption of our business operations. As part of our risk management policy, we maintain insurance coverage at levels that we believe are appropriate for our business. However, in the event of an accident or incident at these facilities, we cannot assure you that the amounts of insurance will be sufficient to satisfy any damages and losses. If our facilities are unable to operate because of an accident or incident or for any other reason, even for a short period of time, any or all of our research and development programs may be harmed. Any business interruption may have a material adverse effect on our business, financial position, results of operations and prospects.

The investment of our cash, cash equivalents and fixed income marketable securities is subject to risks which may cause losses and affect the liquidity of these investments.

As of September 30, 2018 and as of December 31, 2017, we had $32.4 million and $25.8 million in cash and cash equivalents, respectively. We historically have invested excess cash in certificates of deposit or money market mutual funds that invest in U.S. government or U.S. government agency securities, floating rate and variable rate demand notes of U.S. and foreign corporations, and commercial paper. These investments are subject to general credit, liquidity, market and interest rate risks, including potential future impacts similar to the impact of U.S. sub-prime mortgage defaults that have affected various sectors of the financial markets and caused credit and liquidity issues. We may realize losses in the fair value of these investments, an inability to access cash in these investments for a potentially meaningful period, or a complete loss of these investments, which would have a negative effect on our financial statements.

In addition, should our investments cease paying or reduce the amount of interest paid to us, our interest income would suffer. The market risks associated with our investment portfolio may have an adverse effect on our results of operations, liquidity and financial condition.

Changes in accounting rules and regulations, or interpretations thereof, could result in unfavorable accounting charges or require us to change our compensation policies.

Accounting methods and policies for biotechnology companies, including policies governing revenue recognition, research and development and related expenses, and accounting for stock-based compensation, are subject to review, interpretation and guidance from our auditors and relevant accounting authorities, including the SEC. Changes to accounting methods or policies, or interpretations thereof, may require us to reclassify, restate or otherwise change or revise our historical financial statements, including those contained in this Quarterly Report on Form 10-Q.

We previously identified a material weakness in our internal control over financial reporting, and if we are unable to implement and maintain effective internal control over financial reporting in the future, investors may lose confidence in the accuracy and completeness of our financial reports, the market price of our common stock may be adversely effected, and we may become subject to litigation and regulatory investigation.

During the quarter ended March 31, 2018, we identified a material weakness in internal control over financial reporting related to a deficiency in the Company's information and communication controls, which led to ineffectively designed controls over management's review of certain research and development contracts to ensure expenses were recognized as incurred by third-party contract research organizations. Those ineffectively designed controls arose in a prior period and resulted in an immaterial error. The immaterial error has been corrected in previously issued financial statements as disclosed in Note 1 to the accompanying unaudited condensed consolidated financial statements for the period ended September 30, 2018.

As further described above and in Part I, Item 4 "Controls and Procedures," of this Form 10-Q, management is implementing a remediation plan to implement controls in the near term by enhancing the information used for periodic assessment of contract progress and increasing the frequency of communication in the process for accounting for certain research and development contracts with contract research organizations to ensure expenses are recognized as incurred. If the additional controls and procedures we are implementing in order to remediate the material weakness are not effective, or if we identify new material weaknesses in the future in our internal controls over financial reporting, we may not detect

errors in a timely manner and our condensed consolidated financial statements may be materially misstated. We or our independent registered public accounting firm may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting, which could harm our operating results, cause investors to lose confidence in our reported financial information and cause the trading price of our stock to fall. We may also fail to report our financial results on a timely and accurate basis, which could result in sanctions, lawsuits, or other adverse consequences that would materially harm our business. In addition, we could become subject to investigations by the national stock exchange on which our securities are eventually listed, the SEC, or other regulatory authorities, and become subject to litigation from investors and stockholders, which could harm our reputation and our financial condition, or divert financial and management resources from our core business.

We have incurred significant losses since our inception and expect to incur continued losses in the future. We must obtain additional funds to finance our operations and to remain a going concern.

Our recurring losses from operations raise substantial doubt about our ability to continue as a going concern. As a result, we included an explanatory paragraph in our unaudited condensed consolidated financial statements for the period ended September 30, 2018 with respect to this uncertainty. Our ability to continue as a going concern will require us to obtain additional funding. Based on our current operating plan, we believe that our available cash and cash equivalents as of September 30, 2018 is sufficient to fund our current operating plans into the first quarter of 2020. We have based these estimates, however, on assumptions that may prove to be wrong, and we could spend our available financial resources much faster than we currently expect and need to raise additional funds sooner than we anticipate. The perception of our ability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate our research and development programs and commercialization efforts.

Our business may be affected by litigation and government investigations.

We may from time to time receive inquiries and subpoenas and other types of information requests from government authorities and others and we may become subject to claims and other actions related to our business activities. While the ultimate outcome of investigations, inquiries, information requests and legal proceedings is difficult to predict, defense of litigation claims can be expensive, time-consuming and distracting, and adverse resolutions or settlements of those matters may result in, among other things, modification of our business practices, costs and significant payments, any of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Risks Related to Intellectual Property

If we are not able to obtain and enforce patent protection for our technology or therapeutic candidates, development and commercialization of our therapeutic candidates may be adversely affected.

Our success depends in part on our ability to obtain and maintain patents and other forms of intellectual property rights, including in-licenses of intellectual property rights of others, for our therapeutic candidates, methods used to manufacture our therapeutic candidates and methods for treating patients using our therapeutic candidates, as well as our ability to preserve our trade secrets, to prevent third parties from infringing upon our proprietary rights and to operate without infringing upon the proprietary rights of others. As of September 30, 2018, our patent portfolio consists of over 60 issued patents and allowed patent applications and over 120 pending patent applications. We may not be able to apply for patents on certain aspects of our therapeutic candidates in a timely fashion or at all. Our existing issued and granted patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing therapeutics and technology. There is no guarantee that any of our pending patent applications will result in issued or granted patents, that any of our issued or granted patents will not later be found to be invalid or unenforceable or that any issued or granted patents will include claims that are sufficiently broad to cover our therapeutic candidates or to provide meaningful protection from our competitors. Moreover, the patent position of pharmaceutical and biotechnology companies can be highly uncertain because it involves complex legal and factual questions. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent that our current and future proprietary technology and therapeutic candidates are covered by valid and enforceable patents or are effectively maintained as trade secrets. If third parties disclose or misappropriate our proprietary rights, it may materially and adversely impact our position in the market.

The U.S. Patent and Trademark Office, or USPTO, and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in

partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case. The standards applied by the USPTO and foreign patent offices in granting patents are not always applied uniformly or predictably. For example, there is no uniform worldwide policy regarding patentable subject matter or the scope of claims allowable in biotechnology and pharmaceutical patents. As such, we do not know the degree of future protection that we will have on our proprietary therapeutics and technology. While we will endeavor to try to protect our therapeutic candidates with intellectual property rights such as patents, as appropriate, the process of obtaining patents is time-consuming, expensive and sometimes unpredictable.

In addition, there are numerous recent changes to the patent laws and proposed changes to the rules of the USPTO, which may have a significant impact on our ability to protect our technology and enforce our intellectual property rights. For example, the Leahy-Smith America Invents Act enacted in 2011, involves significant changes in patent legislation. The U.S. Supreme Court has ruled on several patent cases in recent years, some of which cases either narrow the scope of patent protection available in certain circumstances or weaken the rights of patent owners in certain situations. The 2013 decision by the Supreme Court in *Association for Molecular Pathology v. Myriad Genetics, Inc.* precludes a claim to a nucleic acid having a stated nucleotide sequence that is identical to a sequence found in nature and unmodified. We currently are not aware of an immediate impact of this decision on our patents or patent applications because we are developing oligonucleotide therapeutics which contain modifications that we believe are not found in nature. However, this decision has yet to be clearly interpreted by courts and by the USPTO. We cannot assure you that the interpretations of this decision or subsequent rulings will not adversely impact our patents or patent applications. 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 may weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

Once granted, patents may remain open to opposition, interference, re-examination, post-grant review, inter partes review, nullification or derivation action in court or before patent offices or similar proceedings for a given period after allowance or grant, during which time third parties can raise objections against such initial grant. In the course of such proceedings, which may continue for a protracted period of time, the patent owner may be compelled to limit the scope of the allowed or granted claims thus attacked, or may lose the allowed or granted claims altogether. In addition, there can be no assurance that:

- Others will not or may not be able to make, use or sell compounds that are the same as or similar to our therapeutic candidates but that are not covered by the claims of the patents that we own or license.
- We or our licensors, or any current or future collaborators, are the first to make the inventions covered by each of our issued patents and pending patent applications that we own or license.
- We or our licensors, or any current or future collaborators, are the first to file patent applications covering certain aspects of our inventions.
- Others will not independently develop similar or alternative technologies or duplicate any of our technology without infringing our intellectual property rights.
- A third party will not challenge our patents and, if challenged, a court may not hold that our patents are valid, enforceable and infringed.
- Any issued patents that we own or have licensed will provide us with any competitive advantages, or will not be challenged by third parties.
- We will develop additional proprietary technologies that are patentable.
- The patents of others will not have an adverse effect on our business.
- Our competitors will not conduct research and development activities in countries where we lack enforceable patent rights and then use the information learned from such activities to develop competitive therapeutics for sale in our major commercial markets.

We currently license patent rights from Northwestern University and may in the future license patent rights from third party owners or licensees. If Northwestern University or such other owners or licensees do not properly or successfully obtain, maintain or enforce the patents underlying such licenses, or if they retain or license to others any competing rights, our competitive position and business prospects may be adversely affected.

We do, and will continue to, rely on intellectual property rights licensed from third parties to protect our technology. We are a party to a number of licenses that give us rights to third party intellectual property that is necessary or useful for our business. In particular, we have a license from Northwestern University, which provides us the exclusive worldwide right under certain patents and patent applications owned by Northwestern University to exploit therapeutics and processes using nanoparticles, nanotechnology, microtechnology and nanomaterial-based constructs as therapeutics or accompanying therapeutics as a means of administration. We may also license additional third party intellectual property in the future. Our success will depend in part on the ability of our licensors to obtain, maintain and enforce patent protection for our licensed intellectual property, and in particular, for those patents to which we have secured exclusive rights. Our licensors may not successfully prosecute the patent applications licensed to us. Even if patents issue or are granted, our licensors may fail to maintain these patents, may determine not to pursue litigation against other companies that are infringing these patents, or may pursue litigation less aggressively than we would. Further, we may not obtain exclusive rights, which would allow for third parties to develop competing therapeutics. Without protection for, or exclusive rights to, the intellectual property we license, other companies might be able to offer substantially identical therapeutics for sale, which could adversely affect our competitive business position and harm our business prospects. In addition, the U.S. government has certain rights to the inventions covered by the patent rights licensed to us by third parties and Northwestern University, as an academic research and medical center, has reserved the right to practice the patent rights it has licensed to us (i) for research, teaching and/or other educationally related purposes (including the right to distribute materials for such purposes) and (ii) for use in the field of diagnostics (including theradiagnostics) and in any field other than the field of use licensed to us.

Other companies or organizations may challenge our or our licensors' patent rights or may assert patent rights that prevent us from developing and commercializing our therapeutic candidates.

Oligonucleotide and SNA-based therapeutics are a relatively new scientific field. We have obtained grants and issuances of SNA therapeutic patents and have licensed many of these patents from a third party on an exclusive basis for therapeutics applications. The issued patents and pending patent applications in the U.S. and in key markets around the world that we own or license claim many different methods, compositions and processes relating to the discovery, development, manufacture and commercialization of SNA therapeutics. Specifically, we own and have licensed a portfolio of patents, patent applications and other intellectual property covering SNA compositions of matter as well as their methods of use.

As the field of SNA therapeutics matures, patent applications are being processed by national patent offices around the world. There is uncertainty about which patents will issue, and, if they do, as to when, to whom, and with what claims. In addition, third parties may attempt to invalidate our intellectual property rights. Even if our rights are not directly challenged, disputes could lead to the weakening of our intellectual property rights. Our defense against any attempt by third parties to circumvent or invalidate our intellectual property rights could be costly to us, could require significant time and attention of our management and could have a material adverse effect on our business and our ability to successfully compete.

There are many issued and pending patents that claim aspects of oligonucleotide chemistry and modifications that we may need to apply to our SNA therapeutic candidates. There are also many issued patents that claim targeting genes or portions of genes that may be relevant for SNA therapeutics we wish to develop. Thus, it is possible that one or more organizations will hold patent rights to which we will need a license. If those organizations refuse to grant us a license to such patent rights on reasonable terms, we may not be able to market therapeutics or perform research and development or other activities covered by these patents.

We may be unable to protect our intellectual property rights throughout the world.

Obtaining a valid and enforceable issued or granted patent covering our technology in the U.S. and worldwide can be extremely costly. In jurisdictions where we have not obtained patent protection, competitors may use our technology to develop their own therapeutics and, further, may export otherwise infringing therapeutics to territories where we have patent protection, but where it is more difficult to enforce a patent as compared to the U.S. Competitor therapeutics may compete with our future therapeutics in jurisdictions where we do not have issued or granted patents or where our issued or granted patent claims or other intellectual property rights are not sufficient to prevent competitor activities in these jurisdictions. The legal systems of certain countries, particularly certain developing countries, make it difficult to enforce

patents and such countries may not recognize other types of intellectual property protection, particularly that relating to biotechnology and pharmaceuticals. This could make it difficult for us to prevent the infringement of our patents or marketing of competing therapeutics in violation of our proprietary rights generally in certain jurisdictions. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

We generally file a provisional patent application first, also known as a priority filing, at the USPTO. An international application under the Patent Cooperation Treaty, or PCT, is usually filed within twelve months after the priority filing. Based on the PCT filing, national and regional patent applications may be filed in the U.S., European Union, Japan, Australia and Canada and, depending on the individual case, also in any or all of, *inter alia*, China, India, South Korea, and Mexico. We have so far not filed for patent protection in all national and regional jurisdictions where such protection may be available. In addition, we may decide to abandon national and regional patent applications before grant. Finally, the grant proceeding of each national or regional patent is an independent proceeding which may lead to situations in which applications might in some jurisdictions be refused by the relevant registration authorities, while granted by others. It is also quite common that depending on the country, various scopes of patent protection may be granted on the same therapeutic candidate or technology.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws in the U.S., and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. If we or our licensors encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished and we may face additional competition from others in those jurisdictions. Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors are forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position in the relevant jurisdiction may be impaired and our business and results of operations may be adversely affected.

We or our licensors, or any current or future strategic partners, may become subject to third party claims or litigation alleging infringement of patents or other proprietary rights or seeking to invalidate patents or other proprietary rights, and we may need to resort to litigation to protect or enforce our patents or other proprietary rights, all of which could be costly, time consuming, delay or prevent the development and commercialization of our therapeutic candidates, or put our patents and other proprietary rights at risk.

We or our licensors, or any current or future strategic partners, may be subject to third party claims for infringement or misappropriation of patent or other proprietary rights. We are generally obligated under our license agreements to indemnify and hold harmless our licensors for damages arising from intellectual property infringement by us. If we or our licensors, or any current or future strategic partners, are found to infringe a third party patent or other intellectual property rights, we could be required to pay damages, potentially including treble damages, if we are found to have willfully infringed. In addition, we or our licensors, or any current or future strategic partners, may choose to seek, or be required to seek, a license from a third party, which may not be available on acceptable terms, if at all. Even if a license can be obtained on acceptable terms, the rights may be non-exclusive, which could give our competitors access to the same technology or intellectual property rights licensed to us. If we fail to obtain a required license, we or any current or future collaborator may be unable to effectively market therapeutic candidates based on our technology, which could limit our ability to generate revenue or achieve profitability and possibly prevent us from generating revenue sufficient to sustain our operations. In addition, we may find it necessary to pursue claims or initiate lawsuits to protect or enforce our patent or other intellectual property rights. The cost to us in defending or initiating any litigation or other proceeding relating to patent or other proprietary rights, even if resolved in our favor, could be substantial, and litigation would divert our management's attention. 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. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could delay our research and development efforts and limit our ability to continue our operations.

If we were to initiate legal proceedings against a third party to enforce a patent covering one of our therapeutics or our technology, the defendant could counterclaim that our patent is invalid or unenforceable. In patent litigation in the U.S., defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability during patent litigation is

unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on one or more of our therapeutics or certain aspects of our technology. Such a loss of patent protection could have a material adverse impact on our business. Patents and other intellectual property rights also will not protect our technology if competitors design around our protected technology without legally infringing our patents or other intellectual property rights.

It is also possible that we have failed to identify relevant third party patents or applications. For example, U.S. applications filed before November 29, 2000 and certain U.S. applications filed after that date that will not be filed outside the U.S. remain confidential until patents issue. Patent applications in the U.S. and elsewhere are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Therefore, patent applications covering our therapeutics or technology could have been filed by others without our knowledge. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our SNA technology, our therapeutics or the use of our therapeutics. Third party intellectual property right holders may also actively bring infringement claims against us. We cannot guarantee that we will be able to successfully settle or otherwise resolve such infringement claims. If we are unable to successfully settle future claims on terms acceptable to us, we may be required to engage in or continue costly, unpredictable and time-consuming litigation and may be prevented from or experience substantial delays in marketing our therapeutics. If we fail in any such dispute, in addition to being forced to pay damages, we may be temporarily or permanently prohibited from commercializing any of our therapeutic candidates that are held to be infringing. We might, if possible, also be forced to redesign therapeutic candidates so that we no longer infringe the third party intellectual property rights. Any of these events, even if we were ultimately to prevail, could require us to divert substantial financial and management resources that we would otherwise be able to devote to our business.

If we fail to comply with our obligations under any license, collaboration or other agreements, we may be required to pay damages and could lose intellectual property rights that are necessary for developing and protecting our therapeutic candidates or we could lose certain rights to grant sublicenses.

Our current licenses impose, and any future licenses we enter into are likely to impose, various development, commercialization, funding, milestone, royalty, diligence, sublicensing, insurance, patent prosecution and enforcement, and other obligations on us. If we breach any of these obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and the licensor may have the right to terminate the license, which could result in us being unable to develop, manufacture and sell therapeutics that are covered by the licensed technology or could enable a competitor to gain access to the licensed technology. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights in such unlicensed intellectual property. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future therapeutics, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in therapeutics that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize therapeutics, we may be unable to achieve or maintain profitability.

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

In addition to seeking patent protection for certain aspects of our therapeutic candidates, we also consider trade secrets, including confidential and unpatented know-how important to the maintenance of our competitive position. We protect trade secrets and confidential and unpatented know-how, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to such knowledge, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants that obligate them to maintain confidentiality and assign their inventions to us. 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. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts in the U.S. and certain foreign jurisdictions are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Under the terms of the Northwestern University License Agreements, Northwestern University could publish research findings relating to the patent rights licensed to us by Northwestern University, which could have a material adverse effect on our business.

We are also subject both in the U.S. and outside the U.S. to various regulatory schemes regarding requests for the information we provide to regulatory authorities, which may include, in whole or in part, trade secrets or confidential commercial information. While we are likely to be notified in advance of any disclosure of such information and would likely object to such disclosure, there can be no assurance that our challenge to the request would be successful.

We may be subject to claims that we or our employees or consultants have wrongfully used or disclosed alleged trade secrets of our employees' or consultants' former employers or their clients. These claims may be costly to defend and if we do not successfully do so, we may be required to pay monetary damages and may lose valuable intellectual property rights or personnel.

Many of our employees were previously employed at universities or pharmaceutical or biotechnology companies, including our competitors or potential competitors. Although no claims against us are currently pending, we may be subject to claims that these employees or we have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of their former employers. Litigation may be necessary to defend against these claims. If we fail in defending such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. A loss of key research personnel or their work product could hamper our ability to commercialize, or prevent us from commercializing, our therapeutic candidates, which could severely harm our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names or may be forced to stop using these names, which we need for name recognition by potential partners or customers in our markets of interest. If we are unable to establish name recognition based on our trademarks and trade names, we may not be able to compete effectively and our business may be adversely affected.

Third parties may independently develop similar or superior technology.

There can be no assurance that others will not independently develop, or have not already developed, similar or more advanced technologies than our technology; or that others will not design around, or have not already designed around, aspects of our technology and/or our trade secrets developed therefrom. If third parties develop technology similar or superior to our technology, or they successfully design around our current or future technology, our competitive position, business prospects, and results of operations could be materially and adversely affected.

The intellectual property which we have licensed from Northwestern University was discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements, and a preference for U.S. industry. Compliance with such regulations may limit our exclusive rights, subject us to expenditure of resources with respect to reporting requirements, and limit our ability to contract with non-U.S. manufacturers.

We have licensed certain intellectual property from Northwestern University pursuant to the Northwestern University License Agreements. The Northwestern University License Agreements indicate that the rights licensed to us by Northwestern University are subject to the obligations to and the rights of the U.S. government, including those set forth in the Bayh-Dole Act of 1980, or the Bayh-Dole Act. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future therapeutics based on the licensed Northwestern University intellectual property. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or nonexclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations, also referred to as "march-in rights." While the U.S. government has sparingly used, and to the Company's knowledge never successfully exercised, such march-in rights, any exercise of the march-in rights by the U.S. government could harm our competitive position, business, financial condition, results of operations, and prospects. If the U.S. government exercises such march-in rights,

we may receive compensation that is deemed reasonable by the U.S. government in its sole discretion, which may be less than what we might be able to obtain in the open market. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources.

In addition, the U.S. government requires that any therapeutics embodying any invention generated through the use of U.S. government funding be manufactured substantially in the U.S. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. therapeutic manufacturers for therapeutics covered by such intellectual property.

Risks Related to Government Regulation

We may be unable to obtain U.S. or foreign regulatory approval and, as a result, unable to commercialize our therapeutic candidates.

Our therapeutic candidates are subject to extensive governmental regulations relating to, among other things, research, testing, development, manufacturing, safety, efficacy, approval, recordkeeping, reporting, labeling, storage, packaging, advertising and promotion, pricing, marketing, sampling, and distribution of therapeutics. Rigorous preclinical testing and clinical trials and an extensive regulatory approval process are required to be successfully completed in the U.S. and in many foreign jurisdictions before a new therapeutic can be marketed. Satisfaction of these and other regulatory requirements is costly, time consuming, uncertain and subject to unanticipated delays. It is possible that none of the therapeutic candidates we may develop will obtain the regulatory approvals necessary for us or any current or future collaborators to begin selling them.

We have very limited experience in conducting and managing the clinical trials necessary to obtain regulatory approvals, including approval by the FDA as well as foreign regulatory authorities, such as the EMA and European Union national competent authorities. The time required to obtain FDA and foreign regulatory approvals is unpredictable but typically takes many years following the commencement of clinical trials, depending upon the type, complexity and novelty of the therapeutic candidate. The standards that the FDA and its foreign counterparts use when regulating us are not always applied predictably or uniformly and can change. Any analysis we perform of data from preclinical and clinical activities is subject to confirmation and interpretation by regulatory authorities, which could delay, limit or prevent regulatory approval. We may also encounter unexpected delays or increased costs due to new government regulations, for example, from future legislation or administrative action, or from changes in the policy of the FDA or foreign regulatory authorities during the period of therapeutic development, clinical trials and regulatory review by the FDA or foreign regulatory authorities. It is impossible to predict whether legislative changes will be enacted, or whether FDA or foreign laws, regulations, guidance or interpretations will be changed, or what the impact of such changes, if any, may be.

Because the therapeutics we are developing may represent a new class of therapeutic, the FDA and its foreign counterparts have not yet established any definitive policies, practices or guidelines in relation to these therapeutics. While we believe the therapeutic candidates that we are currently developing are regulated as new drugs under the Federal Food, Drug, and Cosmetic Act, or the FDCA, the FDA could decide to regulate them or other therapeutics we may develop as biologics under the Public Health Service Act. The lack of policies, practices or guidelines may hinder or slow review by the FDA or foreign regulatory authorities of any regulatory filings that we may submit. Moreover, the FDA may respond to these submissions by defining requirements we may not have anticipated. Such responses could lead to significant delays in the clinical development of our therapeutic candidates. In addition, because there may be therapeutic candidates approved for some of the diseases for which we may seek approval, in order to receive regulatory approval, we may need to demonstrate through clinical trials that the therapeutic candidates we develop to treat these diseases, if any, are not only safe and effective, but safer or more effective than existing products.

Any delay or failure in obtaining required approvals could have a material adverse effect on our ability to generate revenues from the particular therapeutic candidate for which we are seeking approval. Furthermore, any regulatory approval to market a therapeutic may be subject to limitations on the approved uses for which we may market the therapeutic or the labeling or other restrictions. Regulatory authorities also may impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the therapeutic. In addition, the FDA has the authority to require a REMS plan as part of a NDA or a Biologics License Application, or BLA, or after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug or

biologic, such as limiting prescribing to certain physicians or medical centers that have undergone specialized training, limiting treatment to patients who meet certain safe-use criteria and requiring treated patients to enroll in a registry. These limitations and restrictions may limit the size of the market for the therapeutic and affect coverage and reimbursement by third party payors.

We are also subject to numerous foreign regulatory requirements governing, among other things, the conduct of clinical trials, manufacturing and marketing authorization, pricing and third party reimbursement. The foreign regulatory approval process varies among countries and may include all of the risks associated with FDA approval described above as well as risks attributable to the satisfaction of local regulations in foreign jurisdictions. Moreover, the time required to obtain approval may differ from that required to obtain FDA approval. Approval by the FDA does not ensure approval by regulatory authorities outside the U.S. and vice versa.

Certain of our therapeutic candidates may require companion diagnostics in certain indications. Failure to successfully develop, validate and obtain regulatory clearance or approval for such tests could harm our product development strategy or prevent us from realizing the full commercial potential of our therapeutic candidates.

Certain of our therapeutic candidates may require companion diagnostics to identify appropriate patients for those therapeutic candidates in certain indications. Companion diagnostics are subject to regulation by the FDA and comparable foreign regulatory authorities as a medical device and may require separate regulatory authorization prior to commercialization. We may rely on third parties for the design, development, testing and manufacturing of these companion diagnostics, the application for and receipt of any required regulatory authorization, and the commercial supply of these companion diagnostics. If these parties are unable to successfully develop companion diagnostics for these therapeutic candidates, or experience delays in doing so, the development of our therapeutic candidates may be adversely affected and we may not be able to obtain marketing authorization for these therapeutic candidates. Furthermore, our ability to market and sell, as well as the commercial success, of any of our therapeutic candidates that require a companion diagnostic will be tied to, and dependent upon, the receipt of required regulatory authorization and the continued ability of such third parties to make the companion diagnostic commercially available on reasonable terms in the relevant geographies. Any failure to develop, validate, obtain and maintain marketing authorization for a companion diagnostic and supply such companion diagnostic will harm our business, results of operations and financial condition

If we or current or future collaborators, manufacturers or service providers fail to comply with healthcare laws and regulations, we or they could be subject to enforcement actions, which could affect our ability to develop, market and sell our therapeutics and may harm our reputation.

Although we do not currently have any products on the market, once our therapeutic candidates or clinical trials are covered by federal health care programs, we will be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal, state and foreign governments of the jurisdictions in which we conduct our business. Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any therapeutic candidates for which we obtain marketing approval. Our arrangements with third-party payors and customers may expose 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 or distribute our therapeutic candidates for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include, but are not limited to, the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons from soliciting, receiving, offering or providing remuneration, directly or indirectly, to induce either the referral of an individual for a healthcare item or service, or the purchasing or ordering of an item or service, for which payment may be made, in whole or in part, under a federal healthcare program, such as Medicare or Medicaid;
- the U.S. federal False Claims Act, which 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 or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- HIPAA includes a fraud and abuse provision referred to as the HIPAA All-Payor Fraud Law, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program (i.e., not just federal healthcare programs), or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially

false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, 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;

- HIPAA, as amended by HITECH, and its implementing regulations, which impose obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouses as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information, and require notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information;

- the federal Physician Payment Sunshine Act and the implementing regulations, also referred to as "Open Payments," issued under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively, the ACA, which require that manufacturers of pharmaceutical and biological drugs reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program report to the Department of Health and Human Services all consulting fees, travel reimbursements, research grants, and other payments, transfers of value or gifts made to U.S.-licensed physicians and U.S. teaching hospitals with limited exceptions; and

- analogous state laws and regulations, such as, state anti-kickback and false claims laws potentially applicable to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts; and state transparency laws that require the reporting of certain pricing information; among other state laws.

Ensuring that our future business arrangements with third-parties comply with applicable healthcare laws and regulations could involve substantial costs. If our operations are found to be in violation of any such requirements, we may be subject to penalties, including civil or criminal penalties, monetary damages, the curtailment or restructuring of our operations, or exclusion from participation in government contracting, healthcare reimbursement or other government programs, including Medicare and Medicaid, any of which could adversely affect our financial results. Although an effective compliance program can mitigate the risk of investigation and prosecution for violations of these laws, these risks cannot be entirely eliminated. Any action against us for an alleged or suspected violation could cause us to incur significant legal expenses and could divert our management's attention from the operation of our business, even if our defense is successful. In addition, achieving and sustaining compliance with applicable laws and regulations may be costly to us in terms of money, time and resources.

If we or current or future collaborators, manufacturers or service providers fail to comply with applicable federal, state or foreign laws or regulations, we could be subject to enforcement actions, which could affect our ability to develop, market and sell our therapeutics successfully and could harm our reputation and lead to reduced acceptance of our therapeutics by the market. These enforcement actions include, among others:

- adverse regulatory inspection findings;

- warning or untitled letters;

- voluntary product recalls or public notification or medical product safety alerts to healthcare professionals;

- restrictions on, or prohibitions against, marketing our therapeutics;

- restrictions on, or prohibitions against, importation or exportation of our therapeutics;

- suspension of review or refusal to approve pending applications or supplements to approved applications;

- exclusion from participation in government-funded healthcare programs;

- exclusion from eligibility for the award of government contracts for our therapeutics;

- FDA debarment;

- suspension or withdrawal of therapeutic approvals;
- seizures or administrative detention of therapeutics;
- injunctions; and
- civil and criminal penalties and fines.

Any therapeutics we develop may become subject to unfavorable pricing regulations, third party coverage and reimbursement practices or healthcare reform initiatives, thereby harming our business.

The regulations that govern marketing approvals, pricing and reimbursement for new therapeutics vary widely from country to country. Some countries require approval of the sale price of a therapeutic before it can be marketed. In many countries, the pricing review period begins after marketing or therapeutic licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. Although we intend to monitor these regulations, our programs are currently in the early stages of development and we will not be able to assess the impact of price regulations for a number of years. As a result, we might obtain regulatory approval for a therapeutic in a particular country, but then be subject to price regulations that delay our commercial launch of the therapeutic and negatively impact the revenues we are able to generate from the sale of the therapeutic in that country.

Our ability to commercialize any therapeutics successfully also will depend in part on the extent to which coverage and reimbursement for these therapeutics and related treatments will be available from government health administration authorities, private health insurers and other organizations. However, there may be significant delays in obtaining coverage for newly-approved therapeutics. Moreover, eligibility for coverage does not necessarily signify that a therapeutic will be reimbursed in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution costs. Also, interim payments for new therapeutics, if applicable, may be insufficient to cover our costs and may not be made permanent. Thus, even if we succeed in bringing one or more therapeutics to the market, these therapeutics may not be considered cost-effective, and the amount reimbursed for any therapeutics may be insufficient to allow us to sell our therapeutics on a competitive basis. Because our programs are in the early stages of development, we are unable at this time to determine their cost effectiveness or the likely level or method of reimbursement. Increasingly, the third party payors who reimburse patients or healthcare providers, such as government and private insurance plans, are seeking greater upfront discounts, additional rebates and other concessions to reduce the prices for therapeutics. If the price we are able to charge for any therapeutics we develop, or the reimbursement provided for such therapeutics, is inadequate in light of our development and other costs, our return on investment could be adversely affected.

We currently expect that some therapeutics we develop may need to be administered under the supervision of a physician on an outpatient basis. Under currently applicable U.S. law, certain therapeutics that are not usually self-administered (including injectable therapeutics) may be eligible for coverage under Medicare through Medicare Part B. Medicare Part B is part of original Medicare, the federal health care program that provides health care benefits to the aged and disabled, and covers outpatient services and supplies, including certain pharmaceutical products that are medically necessary to treat a beneficiary's health condition. Specifically, Medicare Part B coverage may be available for eligible beneficiaries when the following, among other requirements, have been satisfied:

- the product is reasonable and necessary for the diagnosis or treatment of the illness or injury for which the product is administered according to accepted standards of medical practice;
- the product is typically furnished incident to a physician's services;
- the product has been approved by the FDA.

Under the Medicaid Drug Rebate Statute, a manufacturer must participate in the Medicaid Drug Rebate Program in order to receive payment for its covered outpatient drugs under Medicare Part B (the Medicare program that generally covers physician-administered, outpatient drugs). 42 U.S.C. § 1396r-8(a)(1). In addition, manufacturers who participate in the Medicaid Drug Rebate Program are also required to (1) sign the Pharmaceutical Pricing Agreement and participate in the 340B Drug Pricing Program, and (2) sign the VA Master Agreement for inclusion of the manufacturer's drugs on the Federal Supply Schedule ("FSS"). *Id*. The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services as a condition for states to receive federal matching funds for the manufacturer's outpatient drugs furnished to Medicaid patients. Under the 340B Drug Pricing Program, the manufacturer must extend discounts to entities eligible to

participate in the program. Average prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of therapeutics from countries where they may be sold at lower prices than in the U.S. Self-administered therapeutics are typically reimbursed under Medicare Part D, and therapeutics that are administered in an inpatient hospital setting are typically reimbursed under Medicare Part A under a bundled payment. It is difficult for us to predict how Medicare coverage and reimbursement policies will be applied to our therapeutics in the future and coverage and reimbursement under different federal healthcare programs are not always consistent. Medicare reimbursement rates may also reflect budgetary constraints placed on the Medicare program.

Third-party payors often rely upon Medicare coverage policies and payment limitations in setting their own reimbursement rates. Our inability to promptly obtain coverage, and adequate reimbursement from both government-funded and private payors for new therapeutics we develop and for which we obtain regulatory approval could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our financial condition.

We believe that the efforts of governments and third party payors to contain or reduce the cost of healthcare, and specifically, therapeutics, and legislative and regulatory proposals to broaden the availability of healthcare will continue to affect the business and financial condition of pharmaceutical and biotechnology companies. A number of legislative and regulatory changes in the healthcare system in the U.S. and other major healthcare markets have been proposed. These developments could, directly or indirectly, affect our ability to sell our therapeutics, if approved, at a favorable price.

For example, in the U.S., in 2010, the U.S. Congress passed the ACA, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of health spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional policy reforms.

Although the future of the ACA is uncertain, provisions of the ACA addressing coverage and reimbursement of pharmaceutical products that may be of importance to our potential therapeutic candidates include the following:

- Increases to pharmaceutical manufacturer rebate liability under the Medicaid Drug Rebate Program due to an increase in the minimum basic Medicaid rebate on most branded prescription drugs and the application of Medicaid rebate liability to drugs used in risk-based Medicaid managed care plans.
- The expansion of the 340B Drug Pricing Program to require discounts for "covered outpatient drugs" sold to certain children's hospitals, critical access hospitals, freestanding cancer hospitals, rural referral centers, and sole community hospitals.
- Requirements imposed on pharmaceutical companies to offer discounts on brand-name drugs to patients who fall within the Medicare Part D coverage gap, commonly referred to as the "Donut Hole." In February 2018, Congress passed the Bipartisan Budget Act of 2018, which, beginning in 2019, increased the discount to be paid by pharmaceutical companies from 50% to 70% of a brand-name drug's negotiated price and added biosimilars to the coverage gap discount program.
- Requirements imposed on pharmaceutical companies to pay an annual non-tax-deductible fee to the federal government based on each company's market share of prior year total sales of branded drugs to certain federal healthcare programs, such as Medicare, Medicaid, Department of Veterans Affairs, and Department of Defense. Since we currently expect our branded pharmaceutical sales to constitute a small portion of the total federal healthcare program pharmaceutical market, we do not currently expect this annual assessment to have a material impact on our financial condition.
- For therapeutic candidates classified as biologics, marketing approval for a follow-on biologic therapeutic may not become effective until 12 years after the date on which the reference innovator biologic therapeutic was first licensed by the FDA, with a possible six-month extension for pediatric therapeutics. After this exclusivity ends, it may be possible for biosimilar manufacturers to enter the market, which is likely to reduce the pricing for such therapeutics and could affect our profitability if our therapeutics are classified as biologics.

Separately, pursuant to the health reform legislation and related initiatives, the Centers for Medicare and Medicaid Services, or CMS, is working with various healthcare providers to develop, refine, and implement Accountable Care Organizations, or ACOs, and other innovative models of care for Medicare and Medicaid beneficiaries, including the Bundled Payments for Care Improvement Initiative, the Financial Alignment Initiative Demonstration, and other models.

The continued development and expansion of ACOs and other innovative models of care will have an uncertain impact on any future reimbursement we may receive for approved therapeutics administered by such organizations.

From time to time, legislation is drafted, introduced and passed in the U.S. Congress that could significantly change the statutory provisions governing coverage, reimbursement, sales, and marketing of products regulated by CMS or other government agencies. In addition to new legislation, CMS, OIG, and other agency rules and policies addressing fraud and abuse, privacy, and coverage and reimbursement, among other things, are often revised or interpreted in ways that may significantly affect our business and our products. In particular, we expect that the Administration and the U.S. Congress may continue to seek to modify, repeal, or otherwise invalidate all, or certain provisions of, the U.S. healthcare reform legislation. Since taking office, President Trump has continued to support the repeal of all or portions of the ACA. President Trump has also issued an executive order in which he stated that it is his administration's policy to seek the prompt repeal of the ACA and in which he directed executive departments and federal agencies to waive, defer, grant exemptions from, or delay the implementation of the provisions of the ACA to the maximum extent permitted by law. Congress has also enacted legislation that repeals certain portions of the ACA, including the Tax Cuts and Jobs Act, passed in December 2017, which included a provision that eliminates the penalty under the ACA's individual mandate, effective January 1, 2019, as well as the Bipartisan Budget Act of 2018, passed in February 2018, which, among other things, repealed the Independent Payment Advisory Board that was established by the ACA and was intended to reduce the rate of growth in Medicare spending. However, attempts to completely repeal the ACA have been unsuccessful to date. There is still uncertainty with respect to the impact President Trump's Administration and the U.S. Congress may have, if any, and any changes will likely take time to unfold. For example, in May 2018, the Trump Administration recently published in the Federal Register a request for information regarding its Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs (the "RFI"). In the RFI, the Administration sought feedback on actions it could take to restrict or reduce the use of rebates, including by prohibiting the use of rebates in contracts between Medicare Part D plan sponsors and drug manufactures and removing federal Anti-Kickback Statute safe harbor protection for such rebates. Subsequent to this, in July 2018, the Administration submitted to the White House Office of Management and Budget ("OMB") for regulatory review a proposed rule entitled "Removal of Safe Harbor Protection for Rebates to Plans or PBMs Involving Prescription Pharmaceuticals and Creation of New Safe Harbor Protection." The Administration has not specified the substantive changes it may make to the federal Anti-Kickback Statute safe harbors or subregulatory guidance and the rule is not yet publicly available. The Administration's Blueprint also suggests that the Administration is looking at options to require drug-pricing transparency, including requiring the inclusion of drug list prices in direct-to-consumer advertising. In August 2018, the Administration sent a proposed rule to OMB titled "Medicare and Medicaid Programs; Regulations to Require Drug Pricing Transparency." Like the federal Anti-Kickback Statute proposed rule, the substance of this rule is also not currently publicly available, but many speculate that the proposal seeks to implement direct-to-consumer advertising requirements regarding list price noted above. Separately, in a July 2018 speech outlining the Trump Administrations' healthcare regulatory reform efforts, the HHS Secretary announced that the Administration will soon begin considering changes to federal health privacy regulations. Such reforms, however, could have an adverse effect on anticipated revenues from therapeutic candidates that we may successfully develop and for which we may obtain regulatory approval and may affect our overall financial condition and ability to develop therapeutic candidates. The financial impact of U.S. healthcare reform legislation over the next few years will depend on a number of factors, including the policies reflected in implementing regulations and guidance and changes in sales volumes for therapeutics affected by the legislation.

However, we cannot predict the ultimate content, timing or effect of any healthcare reform legislation or the impact of potential legislation on us.

The healthcare industry is heavily regulated in the U.S. at the federal, state, and local levels, and our failure to comply with applicable requirements may subject us to penalties and negatively affect our financial condition.

As a healthcare company, our operations, clinical trial activities and interactions with healthcare providers may be subject to extensive regulation in the U.S., particularly if the company receives FDA approval for any of its therapeutics in the future. For example, if we receive FDA approval for a therapeutic for which reimbursement is available under a federal healthcare program (e.g., Medicare, Medicaid), it would be subject to a variety of federal laws and regulations, including those that prohibit the filing of false or improper claims for payment by federal healthcare programs (e.g., the False Claims Act), prohibit unlawful inducements for the referral of business reimbursable by federal healthcare programs (e.g., the federal Anti-Kickback Statute), and require disclosure of certain payments or other transfers of value made to U.S.-licensed physicians and teaching hospitals, or Open Payments. We are not able to predict how third parties will interpret these laws and apply applicable governmental guidance and may challenge our practices and activities under one or more of these laws. If our past or present operations are found to be in violation of any of these laws, we could be subject to civil and criminal penalties, which could hurt our business, our operations and financial condition.

Similarly, HIPAA prohibits, among other offenses, knowingly and willfully executing a scheme to defraud any health care benefit program, including private payors, or falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for items or services under a health care benefit program. To the extent that we act as a business associate to a healthcare provider engaging in electronic transactions, we may also be subject to the privacy and security provisions of HIPAA, as amended by HITECH, which restricts the use and disclosure of patient-identifiable health information, mandates the adoption of standards relating to the privacy and security of patient-identifiable health information, and requires the reporting of certain security breaches to healthcare provider customers with respect to such information. Additionally, many states have enacted similar laws that may impose more stringent requirements on entities like ours. Failure to comply with applicable laws and regulations could result in substantial penalties and adversely affect our financial condition and results of operations.

Our ability to obtain services, reimbursement or funding from the federal government may be impacted by possible reductions in federal spending.

U.S. federal government agencies currently face potentially significant spending reductions. The Budget Control Act of 2011, or the BCA, established a Joint Select Committee on Deficit Reduction, which was tasked with achieving a reduction in the federal debt level of at least $1.2 trillion. That committee did not draft a proposal by the BCA's deadline. As a result, automatic cuts, referred to as sequestration, in various federal programs were scheduled to take place, beginning in January 2013, although the American Taxpayer Relief Act of 2012 delayed the BCA's automatic cuts until March 1, 2013. While the Medicare program's eligibility and scope of benefits are generally exempt from these cuts, Medicare payments to providers and Part D health plans are not exempt. The BCA did, however, provide that the Medicare cuts to providers and Part D health plans would not exceed two percent. President Obama issued the sequestration order on March 1, 2013, and cuts went into effect on April 1, 2013. Additionally, the Bipartisan Budget Act of 2018 extended sequestration for Medicare through fiscal year 2027.

The U.S. federal budget remains in flux, which could, among other things, cut Medicare payments to providers. Although the BBA passed in February 2018 enacts a two-year federal spending agreement and raises the federal spending cap on non-defense spending for fiscal years 2018 and 2019, the Medicare program is frequently identified as a target for spending cuts. The full impact on our business of any future cuts in Medicare or other programs is uncertain. In addition, we cannot predict any impact President Trump's administration and the U.S. Congress may have on the federal budget. If federal spending is reduced, anticipated budgetary shortfalls may also impact the ability of relevant agencies, such as the FDA or the National Institutes of Health, to continue to function at current levels. Amounts allocated to federal grants and contracts may be reduced or eliminated. These reductions may also impact the ability of relevant agencies to timely review and approve therapeutic research and development, manufacturing, and marketing activities, which may delay our ability to develop, market, and sell any therapeutics we may develop.

If any of our therapeutic candidates receives marketing approval and we or others later identify undesirable side effects caused by the therapeutic candidate, our ability to market and derive revenue from the therapeutic candidates could be compromised.

In the event that any of our therapeutic candidates receive regulatory approval and we or others identify undesirable side effects, adverse events or other problems caused by one of our therapeutics, any of the following adverse events could occur, which could result in the loss of significant revenue to us and materially and adversely affect our results of operations and business:

- regulatory authorities may withdraw their approval of the therapeutic or seize the therapeutic;
- we may need to recall the therapeutic or change the way the therapeutic is administered to patients;
- additional restrictions may be imposed on the marketing of the particular therapeutic or the manufacturing processes for the therapeutic or any component thereof;
- we may be subject to fines, restitution or disgorgement of profits or revenues, injunctions, or the imposition of civil penalties or criminal prosecution;
- regulatory authorities may require the addition of labeling statements, such as a "black box" warning or a contraindication;
- regulatory authorities may require us to implement a REMS, or to conduct post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the therapeutic;

- we may be required to create a Medication Guide outlining the risks of such side effects for distribution to patients;
- we could be sued and held liable for harm caused to patients;
- the therapeutic may become less competitive; and
- our reputation may suffer.

Significant developments stemming from the United Kingdom's recent referendum on membership in the European Union could have a material adverse effect on our business.

On June 23, 2016, the United Kingdom held a referendum and voted in favor of leaving the European Union. This referendum has created political and economic uncertainty, particularly in the United Kingdom and the European Union, and this uncertainty may last for years. Any business we conduct, now and in the future, in the United Kingdom, the European Union, and worldwide could be affected during this period of uncertainty, and perhaps longer, by the impact of the United Kingdom's referendum. The referendum, and the likely withdrawal of the United Kingdom from the European Union it triggers, has caused and, along with events potentially occurring in the future as a consequence of the United Kingdom's withdrawal, including the possible breakup of the United Kingdom, may continue to cause significant volatility in global financial markets, including in global currency and debt markets. This volatility could cause a slowdown in economic activity in the United Kingdom, Europe, or globally, which could adversely affect our operating results and growth prospects. In addition, our business could be negatively affected by new trade agreements between the United Kingdom and other countries, including the U.S., and by the possible imposition of trade or other regulatory barriers in the United Kingdom.

It is currently unclear how regulations affecting clinical trials, the approval of our future therapeutic candidates, and the sale of these therapeutic candidates will be affected by this referendum either in the United Kingdom or elsewhere in Europe. These possible negative impacts, and others resulting from the United Kingdom's actual or threatened withdrawal from the European Union, may adversely affect our operating results and growth prospects.

Risks Related to Our Common Stock

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

We are an "emerging growth company" as defined in the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including (1) not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, (2) reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and (3) exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In addition, as an emerging growth company, we are only required to provide two years of audited financial statements and two years of selected financial data. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have total annual gross revenue of $1.07 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31, or if we issue more than $1.0 billion in non-convertible debt during any three-year period before that time, in which case we would no longer be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller reporting company" which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. 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.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Our stock price may be volatile and purchasers of our common stock could incur substantial losses.

If a market for our common stock develops, its market price could fluctuate substantially due to a variety of factors, including the other risks described in this section titled "Risk Factors" and the following:

- the success of competitive therapeutics or technologies;
- results of our preclinical studies and clinical trials of our therapeutic candidates, or those of our competitors, or any current or future collaborators;
- regulatory or legal developments in the U.S. and other countries, especially changes in laws or regulations applicable to our therapeutics;
- introductions and announcements of new therapeutics 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 therapeutics, 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, therapeutics or therapeutic candidates;
- developments concerning any current or future collaborations, including but not limited to those with our sources of manufacturing supply and our commercialization partners;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, 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 therapeutics;
- 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;
- market conditions in the pharmaceutical and biotechnology sectors;
- 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;
- natural disasters and other calamities; and
- general economic, industry and market conditions.

In addition, the stock markets in general, and the markets for pharmaceutical and biotechnology stocks in particular, have experienced extreme volatility that has been often unrelated to the operating performance of the issuer. These broad market and industry factors may seriously harm the market price of our common stock, regardless of our operating performance.

The future issuance of equity or of debt securities that are convertible into equity may dilute your investment and reduce your equity interest.

We may choose to raise additional capital in the future, depending on market conditions, strategic considerations and operational requirements. To the extent that we raise additional capital through the issuance of shares or other securities convertible into shares, our stockholders will be diluted. Future issuances of our common stock or other equity securities, or the perception that such sales may occur, could adversely affect the prevailing market price of our common stock and impair our ability to raise capital through future offerings of equity or equity-linked securities. For example, we have filed, and the SEC has declared effective, a registration statement to register the resale of up to 39,714,143 shares of our common stock issued in connection with the Merger and the 2017 Private Placement or held by pre-Merger stockholders of us. Additionally, we have filed, and the SEC has declared effective, a registration statement to register the resale of up to 5,034,683 shares of our common stock, consisting of (i) 4,889,217 shares that were privately issued through the August 2018 Private Placement and (ii) 145,466 shares that were privately issued in connection with consulting services on February 1, 2018. Such registration statements will permit the resale of these shares at any time while the registration statements remain effective. The resale of a substantial number of shares of our common stock in the public market could adversely affect the market price for our common stock and make it more difficult for you to sell shares of our common stock at times and prices that you feel are appropriate. Accordingly, the adverse market and price pressures resulting from an offering pursuant to this registration statement may continue for an extended period of time and continued negative pressure on the market price of our common stock could have a material adverse effect on our ability to raise additional equity capital.

Our debt obligations expose us to risks that could adversely affect our business, operating results and financial condition and may result in further dilution to our stockholders.

We have entered into a loan and security agreement with Hercules pursuant to which we may borrow in an aggregate principal amount of up to $10.0 million from Hercules at a floating per annum interest rate (based on a year consisting of 360 days) equal to the greater of either (i) 9.95% or (ii) the sum of (a) 9.95% plus (b) the prime rate (as reported in *The Wall Street Journal*) minus 3.50%. We were required to make interest only payments on the amounts borrowed until June 2017. Commencing on July 1, 2017, the loan began amortizing in equal monthly installments of principal and interest in an amount sufficient to fully amortize the outstanding principal balance of the loan over the remaining scheduled monthly payments due prior to the maturity date on September 1, 2019. Pursuant to an amendment dated January 15, 2018, amortization payments due for the thirteen (13) consecutive months commencing on December 1, 2017 through and including December 1, 2018 were deferred. Commencing on January 1, 2019, and continuing on the first business day of each month thereafter, the loan, including the deferred payments, shall begin amortizing in equal monthly installments of principal and interest based upon an amortization schedule equal to eighteen (18) consecutive months. Any remaining obligations under the loan agreement and other loan documents (other than the warrant) are due and payable on the maturity date. On the earliest to occur of the maturity date, the date we prepay the term loan in full or the date the loan otherwise becomes due and payable, we must pay the lender under the agreement an additional charge equal to 3.85% of the total amounts funded under the loan agreement. To the extent we desire to prepay the indebtedness prior to maturity, and after the date hereof, we would be obligated to pay a prepayment penalty to Hercules of 1% of the amounts being prepaid. The loan agreement was amended on October 10, 2016 to revise the language granting Hercules a contingent security interest in certain of our assets. Under the loan agreement, Hercules or its affiliates have a right to participate in a single subsequent unregistered financing by us in an amount of up $1.0 million on the same terms, conditions and pricing afforded to others participating in such financing. Hercules has not yet exercised this right to participate.

Our ability to make payments on this indebtedness depends on our ability to generate cash in the future. We expect to experience negative cash flow for the foreseeable future as we fund our operations and capital expenditures. There can be no assurance that we will be in a position to repay this indebtedness when due or obtain extensions of the maturity date. We anticipate that we will need to secure additional funding in order for us to be able to satisfy our obligations when due. We cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. If that additional funding involves the sale of equity securities or convertible securities, it would result in the issuance of additional shares of our capital stock, which would result in dilution to our stockholders. The indebtedness is secured by substantially all of our assets other than intellectual property, on which we have given Hercules a negative pledge. In addition, under the loan agreement, we are subject to certain customary covenants that limit or restrict our ability to,

among other things, incur additional indebtedness, grant any security interests, pay cash dividends, repurchase our common stock, make loans, or enter into certain transactions without the prior consent of Hercules.

This level of debt could have important consequences to you as an investor in our securities. For example, it could:

- limit our flexibility in planning for the development, clinical testing, approval and marketing of our products;
- place us at a competitive disadvantage compared to any of our competitors that are less leveraged than we are;
- increase our vulnerability to both general and industry-specific adverse economic conditions; and
- limit our ability to obtain additional funds.

The employment agreements with our executive officers may require us to pay severance benefits to officers in connection with termination of employment or upon a change of control of us, which could harm our financial condition.

Each of David A. Giljohann, our Chief Executive Officer, Ekambar Kandimalla, our Chief Scientific Officer, David S. Snyder, our Chief Financial Officer, and Matthias G. Schroff, our Chief Operating Officer, is entitled to receive cash severance equal to twelve months, six months, six months, and six months, respectively, of his base salary if his employment is terminated by us without cause (as such term is defined in his employment offer letter). In addition, our 2015 Plan, which was assumed by us in the Merger, generally provides for accelerated vesting of equity awards upon the involuntary termination of an employee within the twelve month period following a change in control (as defined under the plan) and accelerated vesting of equity awards upon a change of control (as defined under the plan) for each of our executive officers. This vesting acceleration is intended to provide each of our executive officers with the full benefit of their equity awards and reward them for a successful outcome for our stockholders. The accelerated vesting of equity awards could result in dilution to our existing stockholders and harm the market price of our common stock. The payment of these severance benefits could harm our financial condition. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

Our common stock recently commenced trading on the OTCQB instead of a national exchange or quotation system, so you may be unable to sell your shares to raise money or otherwise desire to liquidate your shares.

Our common stock is currently quoted on the OTC Market Group's OTCQB Market quotation system under the ticker symbol "XCUR." The OTCQB are regulated quotation services that display real-time quotes, last sale prices and volume limitations in over-the-counter securities. Trading in shares quoted on the OTCQB is often thin and characterized by volatility in trading prices. This volatility may be caused by a variety of factors, including the lack of readily available price quotations, the absence of consistent administrative supervision of bid and ask quotations, lower trading volume and market conditions. As a result, there may be wide fluctuations in the market price of the shares of our common stock for reasons unrelated to operating performance, and this volatility, when it occurs, may have a negative effect on the market price for our securities. Moreover, the OTCQB is not a stock exchange, and trading of securities on them is often more sporadic than the trading of securities listed on a national quotation system or stock exchange. Accordingly, our stockholders may not be able to realize a fair price from their shares when they determine to sell them or may have to hold them for a substantial period of time until the market for our common stock improves.

Our common stock may not be eligible for listing or quotation on any securities exchange.

We do not currently meet the initial quantitative listing standards of any national securities exchange. We cannot assure you that we will be able to meet the initial listing standards of any national securities exchange, or, if we do meet such initial listing standards, that we will be able to maintain any such listing. Until our common stock is listed on a national securities exchange, we expect that it will continue to be eligible and quoted on the OTCQB. In those venues, however, an investor may find it difficult to obtain accurate quotations as to the market value of our common stock. Further, the national securities exchanges are adopting so-called "seasoning" rules that will require that we meet certain requirements, including prescribed periods of time trading over-the-counter and minimum filings of periodic reports with the SEC, before we are eligible to apply for listing on such national securities exchanges. In addition, if we fail to meet the criteria set forth in SEC regulations, various requirements would be imposed by law on broker-dealers who sell our securities to persons other than established customers and accredited investors. Consequently, such regulations may deter broker-dealers from recommending or selling our common stock, which may further affect its liquidity. This would also make it more difficult for us to raise additional capital

The designation of our common stock as a "penny stock" would limit the liquidity of our common stock.

Our common stock may be deemed a "penny stock" (as that term is defined under Rule 3a51-1 of the Exchange Act) in any market that may develop in the future. Generally, a "penny stock" is a common stock that is not listed on a securities exchange and trades for less than $5.00 a share. Prices often are not available to buyers and sellers and the market may be very limited. Penny stocks in start-up companies are among the riskiest equity investments. Broker-dealers who sell penny stocks must provide purchasers of these stocks with a standardized risk-disclosure document prepared by the SEC. The document provides information about penny stocks and the nature and level of risks involved in investing in the penny stock market. A broker must also provide purchasers with bid and offer quotations and information regarding broker and salesperson compensation and make a written determination that the penny stock is a suitable investment for the purchaser and obtain the purchaser's written agreement to the purchase. Many brokers choose not to participate in penny stock transactions. Because of the penny stock rules, there may be less trading activity in penny stocks in any market that develops for our common stock in the future and stockholders are likely to have difficulty selling their shares.

FINRA sales practice requirements may limit a stockholder's ability to buy and sell our stock.

The Financial Industry Regulatory Authority, or FINRA, has adopted rules requiring that, in recommending an investment to a customer, a broker-dealer must have reasonable grounds for believing that the investment is suitable for that customer. Prior to recommending speculative or low-priced securities to their non-institutional customers, broker-dealers must make reasonable efforts to obtain information about the customer's financial status, tax status, investment objectives and other information. Under interpretations of these rules, FINRA has indicated its belief that there is a high probability that speculative or low-priced securities will not be suitable for at least some customers. If these FINRA requirements are applicable to us or our securities, they may make it more difficult for broker-dealers to recommend that at least some of their customers buy our common stock, which may limit the ability of our stockholders to buy and sell our common stock and could have an adverse effect on the market for and 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. Our research coverage by securities and industry analysts is currently limited. In addition, because we did not become a reporting company by conducting an underwritten initial public offering of our common stock, and because we are not listed on a national securities exchange, security analysts of brokerage firms may not provide wider coverage of our Company. In addition, investment banks may be less likely to agree to underwrite secondary offerings on our behalf than they might if we became a public reporting company by means of an underwritten initial public offering, because they may be less familiar with our Company as a result of more limited coverage by analysts and the media, and because we became public at an early stage in our development. The failure to receive wider research coverage or support in the market for our shares will have an adverse effect on our ability to develop a liquid market for our common stock and the trading price for our stock would be negatively impacted.

In the event we obtain wider securities or industry analyst coverage, 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 target studies and operating results fail to meet the expectations of analysts, our stock price would likely decline. If one or more of these 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 our stock price or trading volume to decline.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Based on the beneficial ownership of our common stock as of September 30, 2018, our executive officers and directors, together with holders of five percent or more of our outstanding common stock and their respective affiliates, will beneficially own approximately 42.4 percent of our outstanding common stock. As a result, these stockholders, if acting together, will continue to have significant influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets and any other significant corporate transaction. The interests of these stockholders may not be the same as or may even conflict with your interests. For example, these stockholders could delay or prevent a change of control of our Company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our company or our assets and might affect the prevailing market price of our common stock. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

Anti-takeover provisions in our charter documents and under the General Corporation Law of the State of Delaware could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our management.

Provisions in our amended and restated certificate of incorporation and our bylaws may delay or prevent an acquisition of us or a change in our management. These provisions include a classified board of directors, a prohibition on actions by written consent of our stockholders, and the ability of the board of directors to issue preferred stock without stockholder approval. In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which prohibits stockholders owning in excess of 15% of the outstanding combined organization voting stock from merging or combining with the combined organization. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then-current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

Anti-takeover provisions in our charter documents could discourage, delay or prevent a change in control of us and may affect the trading price of our common stock.

Our corporate documents and the DGCL contain provisions that may enable our board of directors to resist a change in control of us even if a change in control were to be considered favorable by our stockholders. These provisions:

- stagger the terms of our board of directors and require 66 and 2/3% stockholder voting to remove directors, who may only be removed for cause;
- authorize our board of directors to issue "blank check" preferred stock and to determine the rights and preferences of those shares, which may be senior to our common stock, without prior stockholder approval;
- establish advance notice requirements for nominating directors and proposing matters to be voted on by stockholders at stockholders' meetings;
- prohibit our stockholders from calling a special meeting and prohibit stockholders from acting by written consent;
- require 66 and 2/3% stockholder voting to effect certain amendments to our certificate of incorporation and bylaws; and
- prohibit cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates.

These provisions could discourage, delay or prevent a transaction involving a change in control of us. These provisions could also discourage proxy contests and make it more difficult for stockholders to elect directors of their choosing and cause us to take other corporate actions our stockholders desire.

The requirements of being a public company may strain our resources and divert management's attention.

As a public company, we are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, the Dodd-Frank Act, and other applicable securities rules and regulations. In the future, we may also be subject to the listing requirements of the NASDAQ Stock Market. Despite recent reforms made possible by the JOBS Act, compliance with these rules and regulations nonetheless increases our legal and financial compliance costs, makes some activities more difficult, time-consuming or costly, and increases demand on our systems and resources, particularly after we will no longer be an "emerging growth company." The Exchange Act requires, among other things, that we file annual, quarterly, and current reports with respect to our business and operating results.

As a result of disclosure of information in this report and in other filings required of a public company, our business and financial condition are more visible, which we believe may result in threatened or actual litigation, including by competitors and other third parties. If such claims are successful, our business and operating results could be harmed, and even if the claims do not result in litigation or are resolved in our favor, these claims, and the time and resources necessary to resolve them, could divert the resources of our management and adversely affect our business, brand and reputation and results of operations.

We also expect that being a public company and these new rules and regulations will make it more expensive for us to obtain director and officer liability insurance, and we may be required to accept reduced coverage or incur substantially higher costs to obtain coverage. These factors could also make it more difficult for us to attract and retain qualified members of our board of directors, particularly to serve on our audit committee and compensation committee, and qualified executive officers.

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 incur significant costs from class action litigation due to our expected stock volatility.

Our stock price may fluctuate for many reasons, including as a result of public announcements regarding the progress of our development efforts or the development efforts of current or future collaborators or competitors, the addition or departure of our key personnel, variations in our quarterly operating results and changes in market valuations of pharmaceutical and biotechnology companies. This risk is especially relevant to us because pharmaceutical and biotechnology companies have experienced significant stock price volatility in recent years. When the market price of a stock has been volatile as our stock price may be, holders of that stock have occasionally brought securities class action litigation against the company that issued the stock. If any of our stockholders were to bring a lawsuit of this type against us, even if the lawsuit is without merit, we could incur substantial costs defending the lawsuit. The lawsuit could also divert the time and attention of our management.

Our amended and restated certificate of incorporation designates the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.

Our amended and restated certificate of incorporation provides that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or agents to us or our stockholders, any action asserting a claim arising pursuant to any provision of the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws or any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein and the claim not being one which is vested in the exclusive jurisdiction of a court or forum other than the Court of Chancery or for which the Court of Chancery does not have subject matter jurisdiction. Any person purchasing or otherwise acquiring any interest in any shares of our common stock shall be deemed to have notice of and to have consented to this provision of our amended and restated certificate of incorporation. This choice of forum provision may limit our stockholders' ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, employees or agents, which may discourage such lawsuits against us and our directors, officers, employees and agents even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could have a material adverse effect on our business, financial condition or results of operations.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future and we may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, 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 net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research tax credits) to

offset its post-change income or taxes may be limited. The Merger, our prior equity offerings and other changes in our stock ownership may have resulted in ownership changes. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which are outside of our control. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. 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.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

On August 22, 2018, in connection with the closing of the August 2018 Private Placement, we issued a total of 4,889,217 shares of the Company's common stock at a purchase price of $4.50 per share, resulting in approximately $22.0 million in gross proceeds to the Company.

The offer and sale of the Shares in the August 2018 Private Placement were exempt from registration under Section 4(a)(2) of the Securities Act of 1933, as amended or Rule 506 of Regulation D promulgated by the SEC. The closing of the 2018 August Private Placement was conducted on a "reasonable best efforts" basis.

Item 3. Defaults Upon Senior Securities.

None

Item 4. Mine Safety Disclosures.

Not applicable.

Item 5. Other Information.

None

Item 6. Exhibits.

Except as so indicated in Exhibit 32.1, the following exhibits are filed as part of, or incorporated by reference into, this Quarterly Report on Form 10-Q.

Exhibit Number	Exhibit Description
3.1(1)	Certificate of Amendment to Certificate of Incorporation, filed with the Secretary of State of the State of Delaware on September 26, 2017.
3.2(1)	Form of Amended and Restated Certificate of Incorporation, as filed with the Secretary of State of the State of Delaware on November 15, 2017.
3.3(1)	Amended and Restated Bylaws, as currently in effect.
10.1(2)	Form of Subscription Agreement by and between the Company and each investor in connection with the closing of the August 2018 Private Placement.
31.1(3)	Rule 13a-14(a)/15d-14(a) Certification of Principal Executive Officer
31.2(3)	Rule 13a-14(a)/15d-14(a) Certification of Principal Financial Officer
32.1*	Section 1350 Certifications of Principal Executive Officer and Principal Financial Officer
101.INS(3)	XBRL Instance Document
101.SCH(3)	XBRL Taxonomy Extension Schema Document
101.CAL(3)	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF(3)	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB(3)	XBRL Taxonomy Extension Label Linkbase Document
101.PRE(3)	XBRL Taxonomy Extension Presentation Linkbase Document

+ Indicates a management contract or compensatory plan.

* The certifications attached as Exhibit 32.1 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the SEC and are not to be incorporated by reference into any filing of Exicure, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of such Form 10-Q), irrespective of any general incorporation language contained in such filing.

(1) Incorporated by reference to the indicated exhibit in our Current Report on Form 8-K filed on October 2, 2017 and as amended by our Current Report on Form 8-K/A filed on November 7, 2017.

(2) Incorporated by reference to the indicated exhibit in our Current Report on Form 8-K filed on August 28, 2018.

(3) Filed herewith.

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.

Date: November 6, 2018

EXICURE, INC.

By: /s/ David S. Snyder

David S. Snyder

Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

CERTIFICATIONS

I, David A. Giljohann, Ph.D., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Exicure, 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(s) 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(s) 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: November 6, 2018

/s/ David A. Giljohann, Ph.D.

David A. Giljohann, Ph.D.
President and Chief Executive Officer

Exhibit 31.2

CERTIFICATIONS

I, David S. Snyder, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Exicure, 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(s) 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 my 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(s) 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: November 6, 2018

/s/ David S. Snyder

David S. Snyder

Chief Financial Officer

Exhibit 32.1

SECTION 1350 CERTIFICATIONS*

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. § 1350), David A. Giljohann, Ph. D., President and Chief Executive Officer of Exicure, Inc. (the "Company"), and David S. Snyder, Chief Financial Officer of the Company, each hereby certifies that, to the best of his knowledge:

1. The Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2018, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report"), fully complies with the requirements of Section 13(a) or 15(d) of the Exchange Act; and

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

Dated: November 6, 2018

/s/ David A. Giljohann, Ph.D.	/s/ David S. Snyder
David A. Giljohann, Ph.D.	**David S. Snyder**
President and Chief Executive Officer	**Chief Financial Officer**

* This certification accompanies the Quarterly Report on Form 10-Q, to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Quarterly Report on Form 10-Q), irrespective of any general incorporation language contained in such filing.