Dicerna Pharmaceuticals Inc
Form 10-Q
August 08, 2018

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, DC 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 June 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: 001-36281

DICERNA PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	20-5993609
(State or other jurisdiction of incorporation or organization)	(IRS Employer Identification No.)

87 Cambridgepark Drive

Cambridge, MA 02140

(Address of principal executive offices and zip code)

(617) 621-8097

(Registrant's telephone number, including area code)

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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

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

Large accelerated filer		Accelerated filer
Non-accelerated filer	(Do not check if a smaller reporting company)	Smaller reporting company
		Emerging growth company

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

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

As of August 6, 2018, there were 52,949,660 shares of the registrant's common stock, par value $0.0001 per share, outstanding.

DICERNA PHARMACEUTICALS, INC.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q includes “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”). All statements other than statements of historical fact are “forward-looking statements” for purposes of this Quarterly Report on Form 10-Q. In some cases, you can identify forward-looking statements by terminology such as “may,” “could,” “will,” “would,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “intend,” “contemplate,” “project,” “continue,” “potential,” “ongoing,” “goal,” or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- how long we expect to maintain liquidity to fund our planned level of operations and our ability to obtain additional funds for our operations;
- the initiation, timing, progress and results of our research and development programs, preclinical studies, any clinical trials and Investigational New Drug application, Clinical Trial Application, New Drug Application, and other regulatory submissions;
- our ability to identify and develop product candidates for treatment of additional disease indications;
- our or a collaborator’s ability to obtain and maintain regulatory approval of any of our product candidates;
- the rate and degree of market acceptance of any approved product candidates;
- the commercialization of any approved product candidates;
- our ability to establish and maintain additional collaborations and retain commercial rights for our product candidates in the collaborations;
- the implementation of our business model and strategic plans for our business, technologies, and product candidates;
- our estimates of our expenses, ongoing losses, future revenue, and capital requirements;
- our ability to obtain and maintain intellectual property protection for our technologies and product candidates and our ability to operate our business without infringing the intellectual property rights of others;
- our reliance on third parties to conduct our preclinical studies or any clinical trials;
- our reliance on third-party suppliers and manufacturers to supply the materials and components for, and manufacture, our research and development, preclinical and clinical trial drug supplies;
- our ability to attract and retain qualified key management and technical personnel;
- our dependence on our existing collaborator, Boehringer Ingelheim International GmbH (“BI”) for developing, obtaining regulatory approval for, and commercializing product candidates in the collaboration;
- our receipt and timing of any milestone payments or royalties under our research collaboration and license agreement with BI or any future arrangements with any other collaborators;
- our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act of 2012;
- our financial performance; and
- developments relating to our competitors or our industry.

These statements relate to future events or to our future 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 set forth in Part II, Item 1A — “Risk Factors” below and for the reasons described 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 operations, results of operations, industry and future growth. Given these uncertainties, you should not place undue reliance on 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 drugs, 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.

Except where the context otherwise requires, in this Quarterly Report on Form 10-Q, “we,” “us,” “our,” “Dicerna,” and the “Company” refer to Dicerna Pharmaceuticals, Inc. and, where appropriate, its consolidated subsidiaries.

Trademarks

This Quarterly Report on Form 10-Q includes trademarks, service marks, and trade names owned by us or by other companies. All trademarks, service marks, and trade names included in this Quarterly Report on Form 10-Q are the property of their respective owners.

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements
DICERNA PHARMACEUTICALS, INC.

Condensed Consolidated Balance Sheets

(Unaudited)

(In thousands, except share data and par value)

	June 30, 2018	December 31, 2017
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$42,426	$68,789
Held-to-maturity investments	39,875	44,889
Withholding tax receivable	1,583	1,583
Prepaid expenses and other current assets	3,728	3,415
Total current assets	87,612	118,676
NONCURRENT ASSETS:		
Property and equipment—net	1,293	1,512
Restricted cash equivalents	744	744
Other noncurrent assets	66	70
Total noncurrent assets	2,103	2,326
TOTAL ASSETS	$89,715	$121,002
LIABILITIES AND STOCKHOLDERS' EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$3,757	$4,920
Accrued expenses and other current liabilities	6,299	5,726
Current portion of deferred revenue	6,180	6,180
Total current liabilities	16,236	16,826
NONCURRENT LIABILITIES:		
Long-term payable and accrued interest	8,904	—
Deferred revenue, net of current portion	—	3,090
Total noncurrent liabilities	8,904	3,090
TOTAL LIABILITIES	25,140	19,916
COMMITMENTS AND CONTINGENCIES		
STOCKHOLDERS' EQUITY:		
Preferred stock, $0.0001 par value—5,000,000 shares authorized; no shares issued or outstanding at June 30, 2018 or December 31, 2017	—	—
Common stock, $0.0001 par value—150,000,000 shares authorized; 52,867,771 and 51,644,841 shares issued and outstanding at	5	5

June 30, 2018 and December 31, 2017, respectively		
Additional paid-in capital	431,749	417,037
Accumulated deficit	(367,179)	(315,956)
Total stockholders' equity	64,575	101,086
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	$89,715	$121,002

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

Dicerna Pharmaceuticals, Inc.

Condensed Consolidated Statements of Operations

(Unaudited)

(In thousands, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenue from collaborative arrangements	$1,545	$—	$3,090	$—
Operating expenses:				
Research and development	10,339	9,068	20,232	17,811
General and administrative	4,760	4,066	9,095	8,188
Litigation expense	22,244	2,234	25,428	3,608
Total operating expenses	37,343	15,368	54,755	29,607
Loss from operations	(35,798)	(15,368)	(51,665)	(29,607)
Other income (expense):				
Interest income	330	143	619	181
Interest expense	(176)	—	(176)	—
Total other income, net	154	143	443	181
Net loss	(35,644)	(15,225)	(51,222)	(29,426)
Dividends on redeemable convertible preferred stock	—	(2,622)	—	(2,622)
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	—	(6,144)	—	(6,144)
Net loss attributable to common stockholders	$(35,644)	$(23,991)	$(51,222)	$(38,192)
Net loss per share attributable to common stockholders—basic and diluted	$(0.68)	$(1.15)	$(0.98)	$(1.84)
Weighted average common shares outstanding—basic and diluted	52,555,751	20,794,193	52,141,849	20,792,925

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

Dicerna Pharmaceuticals, Inc.

Condensed Consolidated Statements of Cash Flows

(Unaudited)

(In thousands)

	Six Months Ended June 30, 2018	Six Months Ended June 30, 2017
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$(51,222)	$(29,426)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash litigation expense	10,315	—
Stock-based compensation	3,525	4,033
Depreciation	393	363
Loss on disposal of property and equipment	—	51
Amortization of (discount)/premium on investments	(195)	(35)
Changes in operating assets and liabilities:		
Long-term payable	8,904	—
Deferred revenue	(3,090)	—
Prepaid expenses and other assets	(313)	(1,013)
Accounts payable	(1,163)	(599)
Accrued expenses and other liabilities	573	(272)
Net cash used in operating activities	(32,273)	(26,898)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property and equipment	(173)	(58)
Maturities of held-to-maturity investments	35,000	25,000
Purchases of held-to-maturity investments	(29,790)	(49,908)
Net cash provided by (used in) investing activities	5,037	(24,966)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	—	69,700
Proceeds from stock option exercises and issuances under Employee Stock Purchase Plan	908	87
Settlement of restricted stock for tax withholding	(35)	(11)
Net cash provided by financing activities	873	69,776
NET (DECREASE) INCREASE IN CASH, CASH EQUIVALENTS AND RESTRICTED CASH EQUIVALENTS	(26,363)	17,912

CASH, CASH EQUIVALENTS AND RESTRICTED CASH EQUIVALENTS —		
Beginning of period	69,533	21,981
CASH, CASH EQUIVALENTS AND RESTRICTED CASH EQUIVALENTS —		
End of period	$43,170	$39,893
SUPPLEMENTAL CASH FLOW INFORMATION:		
NONCASH FINANCING ACTIVITIES:		
Dividends on redeemable convertible preferred stock	$—	$2,622
Deemed dividend related to beneficial conversion feature of redeemable preferred stock	$—	$6,144
Redeemable convertible preferred stock issuance costs included in accounts payable	$—	$450
NONCASH INVESTING ACTIVITIES:		
Property and equipment purchases included in accounts payable	$6	$—

Reconciliation of cash, cash equivalents and restricted cash equivalents within the Company's consolidated balance sheets:

	As of June 30, 2018	As of June 30, 2017
Cash and cash equivalents	42,426	38,777
Restricted cash equivalents	744	1,116
Cash, cash equivalents and restricted cash equivalents presented above	$43,170	$39,893

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

DICERNA PHARMACEUTICALS, INC.

Notes to Condensed Consolidated Financial Statements

(Unaudited)

(tabular amounts in thousands, except share and per share data and where otherwise noted)

1. Description of Business and Basis of Presentation

Business

Dicerna Pharmaceuticals, Inc. ("Dicerna" or the "Company"), a Delaware corporation founded in 2006 and located in Cambridge, Massachusetts, is a biopharmaceutical company focused on the discovery and development of innovative subcutaneously delivered ribonucleic acid interference ("RNAi")-based pharmaceuticals using its GalXC™ RNAi platform for the treatment of diseases involving the liver, including rare diseases, viral infectious diseases, chronic liver diseases, and cardiovascular diseases.

Basis of presentation

These condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP") and in accordance with the rules and regulations of the Securities and Exchange Commission ("SEC") for interim financial information. Accordingly, these condensed consolidated financial statements do not include all of the information and notes required by GAAP to constitute a complete set of financial statements. These condensed consolidated financial statements have been prepared on the same basis as the Company's annual consolidated financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's financial position at June 30, 2018 and results of operations and cash flows for the interim periods ended June 30, 2018 and 2017. These unaudited condensed consolidated interim financial statements should be read in conjunction with the Company's audited consolidated financial statements and notes thereto included in the Company's Annual Report on Form 10-K for the year ended December 31, 2017. The results of the three and six months ended June 30, 2018 are not necessarily indicative of the results to be expected for the year ending December 31, 2018 or for any other interim period or for any other future year.

Significant judgments and estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, and the disclosure of contingent assets and liabilities at the date of the Company's consolidated financial statements, as well as the revenues and expenses incurred during the reporting periods. On an ongoing basis, the Company evaluates judgments and estimates, including those related to revenue recognition and accrued expenses. The Company bases its estimates on historical experience and on various other factors that the Company believes 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 could differ materially from those estimates.

Liquidity

Based on the Company's current operating plan and liquidity, management believes that available cash, cash equivalents, and held-to-maturity investments will be sufficient to fund the Company's planned level of operations for at least the 12-month period following, August 8, 2018, which is the date that these condensed consolidated financial statements have been issued. Notwithstanding the availability of current liquidity, the Company's ability to fund its preclinical and clinical operations, including completion of its clinical trials, will depend on the Company's ability to raise additional capital through a combination of public or private equity offerings, debt financings, and research collaborations and license agreements. If the Company is unable to generate funding from one or more of these sources within a reasonable timeframe, it may have to delay, reduce, or terminate its research and development programs, preclinical or clinical trials, limit strategic opportunities, or undergo reductions in its workforce or other corporate restructuring activities.

Summary of 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 a result of adopting the Financial Accounting Standards Board's ("FASB") Accounting Standards Update ("ASU") No. 2014-09, Revenue from Contracts with Customers (Topic 606) ("Topic 606"), as discussed below.

Revenue recognition

The Company generates revenue from research collaboration and license agreements with third-party customers. Goods and services in the agreements typically include (i) the grant of licenses for the use of the Company's technology and (ii) the provision of services associated with the research and development of customer product candidates. Such agreements may provide for consideration to the Company in the form of upfront payments, research and development services, option payments, milestone payments, and royalty payments on licensed products.

The Company accounts for a contract when the Company has approval and commitment from both parties, when the rights of the parties are identified, when payment terms are identified, when the contract has commercial substance, and when collectability of consideration is probable.

In determining the appropriate amount of revenue to be recognized as the Company fulfills its obligations under each of its agreements, management completes the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations; (iii) measurement of the transaction price, including whether there are any constraints on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. As part of the accounting for the relevant arrangements, the Company develops assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the underlying contract. The Company uses key assumptions to determine the stand-alone selling price which may include, as applicable, relevant market data, forecasted revenues, development timelines, reimbursement rates for personnel costs, discount rates, or probabilities of technical and regulatory success.

Licenses of intellectual property: If a license granted to a customer to use the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenue from consideration allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company applies 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, to conclude upon the appropriate method of measuring progress for purposes of recognizing revenue related to consideration allocated to the performance obligation. 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 contract with a customer that includes development or regulatory milestone payments, the Company evaluates whether the milestones are considered probable of being reached 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, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or of the licensee, such as regulatory approvals, are assessed as to the probability of achieving the related milestones. At the end of each subsequent reporting period, the Company re-evaluates the probability of achievement of all milestones and any related constraint, and, if necessary, adjusts the estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis and are recorded as revenue and through earnings in the period of adjustment.

Options: Customer options, such as options granted to allow a licensee to choose to research and develop product candidates against target genes to be identified in the future, generally do not provide a material right to the customer and therefore do not give rise to a separate performance obligation. As such, the additional consideration that would result from a customer exercising an option in the future is not included in the transaction price for the current contract. Instead, additional option fee payments are recognized or begin being recognized as revenue when the licensee exercises the options, and the exercise of the option would be treated as a separate contract for accounting

purposes.

Research and development services: Arrangements that include a promise to provide research or development services at the licensee's discretion are assessed to determine whether the services provide a material right to the licensee and are capable of being distinct, are not highly interdependent or do not significantly modify one another, and if so, the services are accounted for as separate performance obligations as the services are provided to the customer. Otherwise, where research or development services are determined not to be capable of being distinct or distinct within the context of the contract, those services are added to the performance obligation that includes the underlying license.

Royalties: For arrangements that include sales-based royalties, including commercial milestone payments based on the level of sales, and when 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 out-licensing arrangement.

The Company receives payments from its licensees as established in each contract. Upfront payments and fees are recorded as deferred revenue upon receipt or when due, and may require deferral of revenue recognition to a future period until the Company performs its obligations under these arrangements. Where applicable, amounts are recorded as contracts receivable when the Company's right to consideration is unconditional. The Company does not assess whether a contract has a significant financing component if the expectation at contract inception is such that the period between payment by the licensees and the transfer of the promised goods or services to the licensees will be one year or less.

Recent Accounting Pronouncements

Adopted in 2018

Revenue recognition

In May 2014, the FASB issued Topic 606, which amends the guidance for accounting for revenue from contracts with customers, superseding the revenue recognition requirements in Accounting Standards Codification Topic 605, Revenue Recognition. Topic 606 is effective for annual reporting periods beginning after December 15, 2017. Under Topic 606, two adoption methods were allowed: retrospectively to all prior reporting periods presented, with certain practical expedients permitted, or retrospectively with the cumulative effect of initially adopting Topic 606 recognized at the date of initial application. The Company elected to apply Topic 606 retrospectively to all prior periods presented. Adoption of Topic 606 did not have a significant quantitative impact on the Company's consolidated financial statements. Adoption of Topic 606 has resulted in additional revenue-related disclosures in the notes to the Company's condensed consolidated financial statements, as discussed above and in note 6.

Income taxes

In October 2016, the FASB issued ASU No. 2016-16, Accounting for Income Taxes: Intra-Entity Asset Transfers of Assets Other than Inventory ("ASU 2016-16"), which is part of the FASB's simplification initiative aimed at reducing complexity in accounting standards. ASU 2016-16 eliminates the current exception that the tax effects of intra-entity asset transfers (intercompany sales) be deferred until the transferred asset is sold to a third party or otherwise recovered through use. Instead, the new guidance will require a reporting entity to recognize any tax expense from the sale of the asset in the seller's tax jurisdiction when the transfer occurs, even though the pre-tax effects of that transaction are eliminated in consolidation. Any deferred tax asset that arises in the buyer's jurisdiction would also be recognized at the time of the transfer. ASU 2016-16 is effective for public business entities in fiscal years beginning on January 1, 2018. Adoption of ASU 2016-16 did not have any impact on the Company's condensed consolidated financial statements.

Statement of cash flows

In August 2016, the FASB issued ASU No. 2016-15, Statement of Cash Flows (Topic 230) ("ASU 2016-15"), a consensus of the FASB's Emerging Issues Task Force ("EITF"). ASU 2016-15 is intended to reduce diversity in practice in how certain transactions are classified in the statement of cash flows and requires companies, among other matters, to use reasonable judgment to separate cash flows. Specifically, in the absence of specific guidance, ASU 2016-15 prescribes that an entity should classify each separately identifiable cash source and use on the basis of the nature of the underlying cash flows. The Company adopted ASU 2016-15 on January 1, 2018, with no significant impact on the Company's condensed consolidated financial statements.

In November 2016, the FASB issued ASU No. 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash ("ASU 2016-18"), a consensus of the FASB's EITF. ASU 2016-18 requires that the statement of cash flows explain the change

during the period in the total of cash and cash equivalents, including amounts generally described as restricted cash or restricted cash equivalents. Entities are required to reconcile such total to amounts on the balance sheet and disclose the nature of the restrictions. By requiring that the statement of cash flows explain the change during the period in the total of cash, cash equivalents and restricted cash, the new guidance eliminates current diversity in practice. The Company adopted ASU 2016-18 on January 1, 2018 and has applied this new guidance retrospectively to all periods presented. Consequently, transfers between restricted and unrestricted cash equivalents accounts are no longer reported as a cash flow in the Company’s condensed consolidated statement of cash flows.

Stock-based compensation

In May 2017, the FASB issued ASU No. 2017-09, Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting ("ASU 2017-09"), which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under ASU 2017-09, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions, whereas under previous guidance, judgments about whether certain changes to an award are substantive may impact whether or not modification accounting is applied in certain situations. The Company adopted ASU 2017-19 on January 1, 2018, with no impact on the Company's condensed consolidated financial statements.

Not yet adopted

Leases

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842) ("ASU 2016-02"), which amends the existing accounting standards for lease accounting, including requiring lessees to recognize most leases on their balance sheets and making targeted changes to lessor accounting. ASU 2016-02 will be effective for the Company beginning in the first quarter of 2019. ASU 2016-02 requires a modified retrospective transition approach for all leases existing at, or entered into after, the date of initial application, with an option to use certain transition relief. Management expects that the adoption of ASU 2016-02 will result in the recognition of a right of use asset and related liability associated with the Company's non-cancelable operating lease arrangement for office and laboratory space that was executed in 2014 (see note 7).

Nonemployee stock-based compensation

In June 2018, the FASB issued ASU No. 2018-07, Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting ("ASU 2018-07"). The ASU is intended to simplify the accounting for nonemployee share-based payments to be consistent with Accounting Standards Codification 718, Compensation—Stock Compensation (Topic 718) ("ASC 718"). Per ASU 2018-07, entities will be required to apply the measurement and classification requirements of ASC 718 to nonemployee awards instead of Equity—Equity Based Payments to Nonemployees (Topic 505). ASU 2018-07 will be effective for the Company beginning in the first quarter of 2019 with early adoption permitted. Management is currently evaluating the impact of ASU 2018-07 and expects that the adoption will not have a significant impact on the Company's consolidated financial statements.

2. Net Loss Per Share

The outstanding securities presented below were excluded from the calculation of net loss per 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.

	June 30,	
	2018	2017

Options to purchase common stock	7,556,554	6,212,437
Warrants to purchase common stock	2,198	87,901
Unvested restricted common stock	—	10,000
Redeemable convertible preferred stock	—	718,404
Total	7,558,752	7,028,742

3. Held-to-maturity investments

The following tables provide information relating to the Company's held-to-maturity investments (amounts in thousands).

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
As of June 30, 2018:				
Held-to-maturity investments				
U.S. treasury securities maturing in one year or less	$ 39,875	$ —	$ (17)	$ 39,858

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
As of December 31, 2017:				
Held-to-maturity investments				
U.S. treasury securities maturing in one year or less	$ 44,889	$ —	$ (30)	$44,859

4. Stock Option Plan and Stock-Based Compensation

During the three and six months ended June 30, 2018, the Company granted stock options to purchase 442,000 and 1,761,350 shares, respectively, of common stock to employees with aggregate grant date fair values of $3.8 million and $13.2 million, respectively, compared to stock options to purchase 352,500 and 1,330,997 shares of common stock granted to employees with aggregate grant date fair values of $0.7 million and $2.7 million, for the three and six months ended June 30, 2017, respectively.

The assumptions used to estimate the grant date fair value using the Black-Scholes option pricing model were as follows:

	Three Months Ended June 30, 2018	Six Months Ended June 30, 2018
Common stock price	$9.14 – $14.41	$9.14 – $14.41
Expected option term (in years)	5.50-6.25	5.50-6.25
Expected volatility	76.8% – 78.33%	75.89% –90.9%
Risk-free interest rate	2.65% – 2.84%	2.32% – 2.84%
Expected dividend yield	0.00%	0.00%

	Three Months Ended June 30, 2017	Six Months Ended June 30, 2017
Common stock price	$2.91 – $3.47	$2.49 – $3.47
Expected option term (in years)	5.50-6.25	5.50-6.25
Expected volatility	79.7% – 80.8%	79.4% – 80.8%
Risk-free interest rate	1.86% – 1.89%	1.86% – 2.07%
Expected dividend yield	0.00%	0.00%

The Company has classified stock-based compensation in its condensed consolidated statements of operations as follows (amounts in thousands):

	Three Months Ended June 30, 2018	Six Months Ended June 30, 2018	Three Months Ended June 30, 2017	Six Months Ended June 30, 2017
Research and development expense	$751	$1,557	$972	$1,917
General and administrative expenses	1,028	1,968	1,053	2,116
Total	$1,779	$3,525	$2,025	$4,033

5. Fair Value Measurements

A summary of the Company's financial assets that are measured or disclosed at fair value on a recurring basis as of June 30, 2018 and December 31, 2017 are presented below (amounts in thousands).

Description	At June 30, 2018	Level 1	Level 2	Level 3
Cash equivalents				
Money market fund	$38,258	$38,258	$	$ —
Held-to-maturity investments				
U.S. treasury securities	39,858	—	$39,858	—
Restricted cash equivalents				
Money market fund	744		744	—
Total	$78,860	$38,258	$40,602	$ —

Description	At December 31, 2017	Level 1	Level 2	Level 3
Cash equivalents				
Money market fund	$ 51,441	$51,441	$—	$ —
Held-to-maturity investments				
U.S. treasury securities	44,859	—	44,859	—
Restricted cash equivalents				
Money market fund	744	—	744	—
Total	$ 97,044	$51,441	$45,603	$ —

The Company's cash equivalents, which are in money market funds, are classified within Level 1 of the fair value hierarchy because they are valued using quoted prices as of June 30, 2018 and December 31, 2017.

The Company's restricted cash equivalents bore interest at the prevailing market rates for instruments with similar characteristics and, accordingly, the carrying value of these instruments also approximated their fair value. These financial instruments were classified within Level 2 of the fair value hierarchy because the inputs to the fair value measurement are valued using observable inputs as of June 30, 2018 and December 31, 2017.

The Company's held-to-maturity investments bore interest at the prevailing market rates for instruments with similar characteristics. The financial instruments were classified within Level 2 of the fair value hierarchy because the inputs to the fair value measurement are valued using observable inputs as of June 30, 2018 and December 31, 2017.

As of June 30, 2018, and December 31, 2017, the carrying amounts of the withholding tax receivable, accounts payable, and accrued expenses approximated their estimated fair values because of the short-term nature of these

financial instruments.

As of June 30, 2018, we had an $8.7 million long-term payable to Alnylam Pharmaceuticals, Inc. (“Alnylam”). The long-term payable carries an interest rate set at current market rates, which is the primary driver in our conclusion that the carrying value approximates fair value. There was no long-term payable for the period ended June 30, 2017.

For the three and six months ended June 30, 2018 and 2017, there were no transfers between Level 1 and Level 2.

6. Collaborative Research and License Agreement

On October 27, 2017, the Company entered into a collaborative research and license agreement with Boehringer Ingelheim International GmbH ("BI") (the "BI Agreement"), pursuant to which the Company and BI jointly research and develop product candidates for the treatment of chronic liver disease using the GalXC platform, Dicerna's proprietary RNAi-based technology. The BI Agreement is for the development of product candidates against one target gene with an option for BI to add the development of product candidates that target a second gene. Also, pursuant to the BI Agreement, Dicerna granted BI a worldwide license in connection with the research and development of the product candidates and will transfer to BI intellectual property rights of the product candidates selected by BI for clinical development and commercialization. Dicerna also may provide assistance to BI in order to help BI further develop selected product candidates. Under the terms of the BI Agreement, BI agreed to pay Dicerna a non-refundable upfront payment of $10.0 million for the first target, less a refundable withholding tax in Germany of $1.6 million. The German withholding tax was withheld by BI and remitted to the German tax authorities in accordance with local tax law; the Company received reimbursement of this tax in July 2018.

During the term of the research program, BI will reimburse Dicerna the cost of certain materials and third-party expenses that have been included in the preclinical studies. The Company is eligible to receive up to $191.0 million in potential development and commercial milestones related to the initial target. Dicerna is also eligible to receive royalty payments on potential global net sales, subject to certain adjustments, tiered from high single digits up to low double digits. BI's option to add a second target would provide for an option fee payment and success-based development and commercialization milestones and royalty payments to Dicerna.

Milestone payments that are contingent upon the Company's performance under the BI Agreement include potential developmental milestones totaling $99.0 million, including milestones for the first commercial sale. The Company has excluded the amounts from allocable consideration at the outset of the arrangement, as described below. All potential net sales milestones, totaling $95.0 million, will be accounted for in the same manner as royalties and recorded as revenue at the later of the achievement of the milestone or the satisfaction of the performance obligation.

The Company assessed the BI Agreement in accordance with Topic 606 and concluded that BI is a customer. The Company identified the following performance obligations under the contract: the license of intellectual property and conducting agreed-upon research program services. The Company has concluded that the license and research and development services do not have standalone value and are not capable of being distinct, therefore, the Company considers these as one performance obligation. The Company concluded the option underlying the transfer of future licenses and potential associated research for any not-yet-known target gene is not a performance obligation of the contract at inception because the option is not considered to be a material right, as the option fee reflects the standalone selling price of the option. The Company considered the level of BI's therapeutic expertise specifically related to RNAi, as well as BI's know-how vis-à-vis the Company's GalXC conjugates, and concluded that BI cannot currently benefit from the granted license on its own or together with other resources that are readily available to BI, including relationships with oligonucleotide vendors who synthesize GalXC conjugates under contract with the Company. As a result, the combination of the license of intellectual property together with the provision of research and development support services together represent the highest level of goods and services that can be deemed distinct.

Based on management's evaluation, the non-refundable upfront fee and the agreed-upon reimbursable third-party expenses constituted the amount of the consideration to be included in the transaction price and has been allocated to the performance obligations identified based on the Company's best estimate of the relative standalone selling price via application of a market assessment approach. None of the development milestones have been included in the transaction price, since none of such milestone amounts are within the control of the Company and are not considered probable to occur until confirmed by BI, at BI's sole discretion. Any consideration related to commercial sales-based

milestones (including royalties) will be recognized when the related sales occur, since these amounts have been determined to relate predominantly to the license granted to BI and therefore are recognized at the later of when the performance obligation is satisfied or the related sales occur. The Company will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

The $10.3 million transaction price is being recognized over the current research term, which is estimated to extend through June 30, 2019, which represents the Company's best estimate of the period of the obligation to provide research support services to BI, and is the expected period over which we estimate the deferred revenue balance will be recognized in revenue. Related revenue is being recognized on a straight-line basis, which is in management's judgment an appropriate measure of progress toward satisfying the performance obligation, largely in absence of evidence that obligations are fulfilled in a specific pattern.

The following table presents changes in the Company's deferred revenue accounts during the six months ended June 30, 2018 (amounts in thousands).

Six months ended June 30, 2018	Balance at beginning of period	Additions	Deductions	Balance at end of period
Current portion of deferred revenue	$ 6,180	—		$ 6,180
Deferred revenue, net of current portion	$ 3,090	—	$ (3,090)	$ —

The Company recognized revenues of $1.5 million and $3.1 million for the three and six months ended June 30, 2018, as a result of changes in the deferred revenue balances. There was no activity related to the Company's deferred revenue accounts during the three and six months ended June 30, 2017.

7. Commitments and Contingencies

Facility lease

Future minimum lease payments on the Company's non-cancelable operating lease for office and laboratory space are as follows (amounts in thousands):

Years Ending December 31,	Operating Lease
Remaining 2018	$ 817
2019	$ 1,678
2020*	1,580
Total	$ 4,075

*The end of the lease term is November 30, 2020.

Litigation

On June 10, 2015, Alnylam filed a complaint against the Company in the Superior Court of Middlesex County, Massachusetts (the "Court"). The complaint alleged misappropriation of confidential, proprietary, and trade secret information, as well as other related claims, in connection with the Company's hiring of a number of former employees of Merck & Co., Inc. ("Merck") and its discussions with Merck regarding the acquisition of its subsidiary, Sirna Therapeutics, Inc., which was subsequently acquired by Alnylam.

On April 18, 2018, the Company and Alnylam entered into a Confidential Settlement Agreement and General Release (the "Settlement Agreement"), resolving all ongoing litigation between the Company and Alnylam. The terms of the Settlement Agreement include mutual releases and dismissals with prejudice of all claims and counterclaims in the following litigation between the parties: (i) Alnylam Pharmaceuticals, Inc. v. Dicerna Pharmaceuticals, Inc.,

No. 15-4126 pending in the Massachusetts Superior Court for Middlesex County and (ii) Dicerna Pharmaceuticals, Inc., v. Alnylam Pharmaceuticals, Inc. No.1:17-cv-11466 pending in the United States District Court for the District of Massachusetts. Pursuant to the terms of the Settlement Agreement, the Company has agreed to make the following payments to Alnylam: (i) a $2.0 million upfront payment in cash; (ii) an additional $13.0 million in cash to be paid as 10% of any upfront or first year cash consideration that the Company receives pursuant to future collaborations related to Ga1NAc-conjugated RNAi research and development (excluding any amounts received or to be received by the Company from its existing collaboration with BI), provided that the $13.0 million must be paid by no later than April 28, 2022; and (iii) issuance of shares of the Company's common stock (the "Shares") pursuant to a share issuance agreement between the parties (the "Share Issuance Agreement").

Under the Settlement Agreement, for periods ranging from 18 months up to four years, the Company will be restricted in its development and other activities relating to oligonucleotide-based therapeutics directed toward a defined set of eight Alnylam targets (the "Oligo Restrictions"). The Oligo Restrictions pertain to targets where Dicerna does not have, or does not currently intend to have, a therapeutic program, or are expected to be consistent with Dicerna's execution on programs in the normal course of business. The Settlement Agreement does not include any admission of liability or wrongdoing by either party or any licenses to any intellectual property from either party.

On April 20, 2018, the Company and Alnylam entered into the Share Issuance Agreement, pursuant to which the Company agreed to issue to Alnylam 983,208 Shares in satisfaction of the Company's obligation under the Settlement Agreement to deliver Shares to Alnylam. The Share Issuance Agreement contains customary representations and warranties of each party. Pursuant to the terms of the

Share Issuance Agreement, Alnylam may not, without the prior approval of the Company, dispose of any of the Shares for a six-month period commencing on the closing date of the Share issuance. Thereafter, through the fifth anniversary of the closing date of the Share issuance, Alnylam will only dispose of the maximum number of Shares that it would be permitted to dispose if the Shares were subject to the volume restrictions set forth in Rule 144(e) of the Securities Act of 1933, as amended.

The Company paid the upfront payment of $2.0 million dollars in May 2018 and recorded the future payment of $13.0 million as a long-term payable discounted to present value of $8.7 million at an effective interest rate of 10%. The 983,208 shares issued pursuant to the Share Issuance Agreement was recorded at fair market value of $10.3 million based on the Company's closing share price on April 18, 2018, the date the Settlement Agreement was executed. The Settlement Agreement resulted in the Company recording an additional $21.0 million of litigation expenses for the three and six months ended June 30, 2018. The Company did not assign any value to the Oligo Restrictions as the Company did not incur additional losses or give up any value as a result of the restrictions.

Total litigation expense was $22.2 million and $25.4 million for the three and six months ended June 30, 2018, respectively, all of which related to the litigation with Alnylam, of which $21.0 million related to the Settlement Agreement. The Company recorded litigation expenses, also related to the Alnylam litigation, of $2.2 million and $3.6 million for three and six months ended June 30, 2017, respectively, which was previously recorded as a component of general and administrative expenses and recast to litigation expense for comparative purposes.

From time to time, the Company may be subject to various claims and legal proceedings. If the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount is reasonably estimable, the Company will accrue a liability for the estimated loss. There were no contingent liabilities recorded as of June 30, 2018 and December 31, 2017.

8. Subsequent Events

Under the BI Agreement, BI paid to Dicerna a non-refundable upfront payment of $10.0 million for the first target, less a refundable withholding tax in Germany of $1.6 million. The German withholding tax was withheld by BI and remitted to the German tax authorities in accordance with local tax law, and the Company received reimbursement of this tax in July of 2018.

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

The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those discussed here. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in this section as well as factors described in Part II, Item 1A —"Risk Factors."

Overview

Dicerna Pharmaceuticals, Inc. ("we", "the Company" or "Dicerna") is a biopharmaceutical company focused on the discovery and development of innovative subcutaneously delivered ribonucleic acid ("RNA") interference ("RNAi")-based pharmaceuticals using our GalXC™ RNAi platform for the treatment of diseases involving the liver, including rare diseases, viral infectious diseases, chronic liver diseases and cardiovascular diseases. Within these therapeutic areas, we believe our GalXC RNAi platform will allow us to build a broad pipeline of therapeutics with commercially attractive pharmaceutical properties, including a subcutaneous route of administration, infrequent dosing (e.g., dosing that is monthly or quarterly, and potentially even less frequent), high therapeutic index, and specificity to a single target gene.

All of our GalXC drug discovery and development efforts are based on the therapeutic modality of RNAi, a highly potent and specific mechanism for silencing the activity of a targeted gene. In this naturally occurring biological process, double-stranded RNA molecules induce the enzymatic destruction of the messenger ribonucleic acid ("mRNA") of a target gene that contains sequences that are complementary to one strand of the therapeutic double-stranded RNA molecule. The Company's approach is to design proprietary double-stranded RNA molecules that have the potential to engage the enzyme Dicer and initiate an RNAi process to silence a specific target gene. Our GalXC RNAi platform utilizes a particular structure of double-stranded RNA molecules configured for subcutaneous delivery to the liver. Due to the enzymatic nature of RNAi, a single GalXC molecule incorporated into the RNAi machinery can destroy hundreds or thousands of mRNAs from the targeted gene.

The GalXC RNAi platform supports Dicerna's long-term strategy to retain, subject to the evaluation of potential licensing opportunities as they may arise, a full or substantial ownership stake and to invest internally in diseases with focused patient populations, such as certain rare diseases. We see such diseases as representing opportunities that carry a relatively higher probability of success, with genetically and molecularly defined disease markers, high unmet need, a limited number of Centers of Excellence to facilitate reaching these patients, and the potential for more rapid clinical development programs. For more complex diseases with multiple gene dysfunctions and larger patient populations, we plan to pursue collaborations that can provide the enhanced scale, resources, and commercial infrastructure required to maximize these prospects, such as the BI Agreement, as defined and discussed below.

Development Programs

In choosing which development programs to advance, we apply scientific, clinical, and commercial criteria that we believe allow us to best leverage our GalXC RNAi platform and maximize value. The Company is focusing its efforts on three priority therapeutic programs that currently have a Clinical Trial Application ("CTA") filed, Investigational New Drug ("IND") application filed, or are in enabling studies in preparation to file additional regulatory clearances to initiate clinical trials. The Company is also focusing its efforts on a series of programs in the clinical candidate selection stage that may be elevated into IND/CTA enabling studies in the future, either on our own or in collaboration with larger pharmaceutical companies. Our three priority programs are: DCR-PHXC for the treatment of primary hyperoxaluria ("PH"); a program for an undisclosed rare disease; and DCR-HBVS for the treatment of chronic hepatitis B virus ("HBV") infection. Our programs in clinical candidate selection include a program for the treatment of hypercholesterolemia, for which DCR-PCSK9 has been selected as a provisional clinical candidate, and multiple programs targeting undisclosed targets in chronic liver diseases, cardiovascular diseases and additional rare diseases. In October 2017, we filed a CTA for our lead GalXC product candidate, DCR-PHXC, with the Medicines and

Healthcare products Regulatory Agency (“MHRA”) in the United Kingdom (“UK”), and in December 2017, we dosed the first human in the Group A portion of the Phase 1 clinical trial of DCR-PHXC. On March 30, 2018, we received a notice from the United States (“U.S.”) Food and Drug Administration (“FDA”) indicating that our proposed clinical investigation for DCR-PHXC referenced in our IND may proceed. In May 2018, the Company dosed the first PH patient with DCR-PHXC in the Group B portion of the Phase 1 clinical trial and received notice from the FDA granting Orphan Drug Designation to DCR-PHXC for treatment of PH. In July 2018, the European Medicines Agency (“EMA”)’s Committee for Orphan Medicinal Products (“COMP”) recommended designating DCR-PHXC as an orphan medicinal product for the treatment of PH in the EU and the recommendation was adopted by the European Commission in August 2018. We have received regulatory and ethical approvals for the clinical trial in the UK, France, and Germany. A CTA has been submitted and is pending approval in the Netherlands. We expect to file for additional regulatory clearances to commence clinical trials for our programs in 2018 and 2019.

The table below sets forth the state of development of our various GalXC RNAi platform product candidates as of August 8, 2018.

Our current GalXC RNAi platform development programs are as follows:

- Primary Hyperoxaluria. We are developing DCR-PHXC for the treatment of all types of PH. PH is a family of rare inborn errors of metabolism in which the liver produces excessive levels of oxalate, which in turn causes damage to the kidneys and to other tissues in the body. DCR-PHXC is currently being investigated in a Phase 1 clinical trial called PHYOX. In preclinical models of PH, DCR-PHXC reduces oxalate production to near-normal levels, ameliorating the disease condition.

PHYOX is a Phase 1 single ascending-dose study of DCR-PHXC in normal healthy volunteers ("NHVs") and patients with PH. The study is divided into two groups: Group A is a placebo-controlled, single-blind, single center study, which has enrolled 25 NHVs; Group B is an open-label, multi-center study enrolling up to 16 patients with PH type 1 ("PH1") and PH type 2 ("PH2"). The primary objective of the study is to evaluate the safety and tolerability of single doses of DCR-PHXC in both groups. The secondary objectives are to evaluate the pharmacodynamic effect of single doses of DCR-PHXC on biochemical markers, and to characterize the pharmacokinetics of single doses of DCR-PHXC in NHVs and patients with PH. We have submitted CTAs for the PHYOX study in the UK, France, and Germany and have received the appropriate regulatory and ethical approvals. A CTA has been submitted and is pending approval in the Netherlands. The FDA has accepted the Company's IND for the PHYOX study. We have completed the Group A portion of the study in NHV. While the study is still blinded toward treatment assignment, there have been no serious adverse events. There have been two mild-to-moderate transient injection site reactions lasting up to a total of 36 hours at the highest doses of 6 and 12 mg/kg. With the completion of the Group A portion of the study in NHVs, we have started on the Group B portion of the study and dosed the first PH patient with DCR-PHXC. Group B consists of three cohorts of patients dosed with PH1 at 1.5, 3, and 6 mg/kg. An additional fourth cohort that consists of patients with PH2 dosed at a flexible dosing level. We have enrolled 10 patients out of 16 (four PH1 patients in Cohort 1, four PH1 patients in Cohort 2, one PH1 patient in Cohort 3, and one PH2 patient in Cohort 4). We expect to report interim results from the PHYOX trial later in the third quarter and publicly present trial results in the fourth quarter of 2018. Additionally, we intend to initiate a multi-dose Phase 2/3 study in the first quarter of 2019, pending positive proof-of-concept ("POC") data and regulatory feedback.

- An undisclosed rare disease involving the liver. We are developing a GalXC-based therapeutic, targeting a liver-expressed gene involved in a serious rare disease. For competitive reasons, we have not yet publicly disclosed the target gene or disease. We have selected this target gene and disease based on criteria that include having a strong therapeutic hypothesis, a readily-identifiable patient population, the availability of a potentially predictive biomarker, high unmet medical need, favorable competitive positioning and what we believe is a rapid projected path to approval. The disease is a genetic disorder where mutations in the disease gene lead to the

production of an abnormal protein. The protein causes progressive liver damage and fibrosis, in some cases leading to cirrhosis and liver failure, and we believe that silencing of the disease gene will prevent production of the abnormal protein and thereby slow or stop progression of the liver fibrosis. Greater than 100,000 people in the U.S. are believed to be homozygous (i.e. having identical pairs of genes for any given pair of hereditary characteristics) for the mutation that causes the liver disease, and at least 10% of those people, and potentially a significantly higher fraction, are believed to have liver-associated disease as a consequence. We are seeking a risk-sharing collaborator for this program before we file regulatory clearances to initiate a clinical trial, which we expect to be prepared to file towards the end of 2018.

- Chronic Hepatitis B Virus infection. We have declared a GalXC RNAi platform-based product candidate for the treatment of HBV, DCR-HBVS, and are conducting formal non-clinical development studies. We expect to file regulatory clearances in New Zealand and Australia during the fourth quarter of 2018. Current therapies for HBV rarely lead to a long-term immunological cure as measured by the clearance of HBV surface antigen ("HBsAg") and sustained HBV deoxyribonucleic acid ("DNA") suppression in patient plasma or blood. DCR-HBVS targets HBV messenger RNA and leads to greater than 99% reduction in circulated HBsAg in mouse models of HBV infection. Based on these preclinical studies, and only if we receive appropriate regulatory approval to begin human clinical trials, we hope to determine the potential of DCR-HBVS to reduce HBsAg and HBV DNA levels in the blood of HBV patients in a commercially attractive subcutaneous dosing paradigm.
- Hypercholesterolemia (PCSK9 targeted therapy). We are using our GalXC RNAi platform to develop a therapeutic that targets the PCSK9 gene for the treatment of hypercholesterolemia. The Company has selected a provisional clinical candidate for the program but is continuing to explore ways to further optimize the program. PCSK9 is a validated target for hypercholesterolemia, and there are FDA-approved therapies targeting PCSK9 that are based on monoclonal antibody technology. Based on preclinical studies, we believe that our GalXC RNAi platform has the potential to produce a PCSK9-targeted therapy with attractive commercial properties, such as small subcutaneous injection volumes and less frequent dosing.
- Additional pipeline programs. We have developed a robust portfolio of additional targets and diseases that we plan to pursue either on our own or in collaboration with partners. We have applied our GalXC technology to multiple gene targets across our disease focus areas of rare diseases, chronic liver diseases and cardiovascular diseases. Pursuant to our strategy, we are seeking collaborations with larger and/or more experienced pharmaceutical companies to advance our programs in the areas of chronic liver diseases and cardiovascular diseases. Both these disease areas represent large and diverse patient populations, requiring complex clinical development and commercialization paths that we believe can be more effectively pursued in collaboration with larger pharmaceutical companies. For our additional rare diseases, we are continuing to assess their potential for clinical success and market opportunity while optimizing our GalXC molecules. For our additional pipeline programs (including PCSK9), we may utilize more advanced versions of our GalXC technology that further improve pharmaceutical properties of the GalXC molecules, including enhancing the duration of action and potency. We have further optimized our GalXC technology platform, enabling the development of next generation GalXC molecules. Improvements to our GalXC compound include modification of the tetraloop end of the molecule, which can be applied to any target gene, resulting in a substantially longer duration of action and higher potency of target gene silencing in animal models across multiple targets. Modification of the tetraloop only impacts the passenger strand and does not impact the guide strand. These modifications are unique to our GalXC molecules and, we believe, provide a competitive advantage for the Company.

In addition to the GalXC development programs outlined above, we are party to a collaborative research and license agreement with Boehringer Ingelheim International GmbH ("BI") (the "BI Agreement"), pursuant to which the Company and BI jointly research and develop product candidates for the treatment of chronic liver diseases, with an initial focus on nonalcoholic steatohepatitis ("NASH") using our GalXC platform. NASH is caused by the buildup of fat in the liver, potentially leading to liver fibrosis and cirrhosis. NASH has an especially high prevalence among obese and diabetic patients and is an area of high unmet medical need. The BI Agreement is for the development of product candidates against one target gene with an option for BI to add the development of product candidates that target a second gene. We are working exclusively with BI to develop the product candidates against the undisclosed target gene. We are

responsible for the discovery and initial profiling of the product candidates, including primary pre-clinical studies, synthesis, and delivery. BI is responsible for evaluating and selecting the product candidates for further development. If BI selects one or more product candidates, it will be responsible for further pre-clinical development, clinical development, manufacturing, and commercialization of those products. Also pursuant to the BI Agreement, we granted BI a worldwide license in connection with the research and development of the product candidates and will transfer to BI intellectual property rights of the product candidates selected by BI for clinical development and commercialization. We also may provide assistance to BI in order to help BI further develop selected product candidates. Pursuant to the BI Agreement, BI agreed to pay us a non-refundable upfront payment of $10.0 million for the first target. During the term of the research program, BI will reimburse us the cost of materials and third-party expenses that have been included in the preclinical studies up to an agreed-upon limit. We are eligible to receive up to $191.0 million in potential development and commercial milestones related to the initial target. We are also eligible to receive royalty payments on potential global net sales, subject to certain adjustments, tiered from high single digits up to low double digits. BI’s option to add a second target would provide for an option fee payment and success-based development and commercialization milestones and royalty payments to us.

We also have developed a wholly-owned clinical candidate, DCR-BCAT, targeting the ß-catenin oncogene. DCR-BCAT is based on an extended version of our earlier generation non-GalXC Dicer Substrate RNAi technology and is delivered by our lipid nanoparticle tumor delivery system, EnCore™. We plan to out-license, spin out or seek external funding to advance the DCR-BCAT opportunity, given our focus on our GalXC platform-based programs.

Corporate Developments

On April 18, 2018, we entered into a Confidential Settlement Agreement and General Release (the "Settlement Agreement") with Alnylam Pharmaceuticals, Inc. ("Alnylam"), resolving all ongoing litigation between the Company and Alnylam. The terms of the Settlement Agreement include mutual releases and dismissals with prejudice of all claims and counterclaims in the following litigation between the parties: (i) Alnylam Pharmaceuticals, Inc. v. Dicerna Pharmaceuticals, Inc., No. 15-4126 pending in the Massachusetts Superior Court for Middlesex County and (ii) Dicerna Pharmaceuticals, Inc., v. Alnylam Pharmaceuticals, Inc. No.1:17-cv-11466 pending in the United States District Court for the District of Massachusetts. Pursuant to the terms of the Settlement Agreement, we have agreed to make the following payments to Alnylam: (i) a $2 million upfront payment in cash; (ii) an additional $13 million in cash, to be paid as 10% of any upfront or first year cash consideration that we receive pursuant to future collaborations related to Ga1NAc-conjugated RNAi research and development (excluding any amounts received or to be received by the Company from its existing collaboration with BI), provided that the $13 million must be paid by no later than April 28, 2022; and (iii) issuance of shares of our common stock (the "Shares") pursuant to a share issuance agreement between the parties (the "Share Issuance Agreement").

Under the Settlement Agreement, for periods ranging from 18 months up to four years, we will be restricted in our development and other activities relating to oligonucleotide-based therapeutics directed toward a defined set of eight Alnylam targets (the "Oligo Restrictions"). The Oligo Restrictions pertain to targets where Dicerna does not have, or does not currently intend to have, a therapeutic program, or are expected to be consistent with our execution on programs in the normal course of business. The Settlement Agreement does not include any admission of liability or wrongdoing by either party or any licenses to any other intellectual property from either party.

On April 20, 2018, we entered into the Share Issuance Agreement, pursuant to which we agreed to issue to Alnylam 983,208 Shares in satisfaction of our obligation under the Settlement Agreement to deliver Shares to Alnylam. The Share Issuance Agreement contains customary representations and warranties of each party. Pursuant to the terms of the Share Issuance Agreement, Alnylam may not, without our prior approval, dispose of any of the Shares for a six-month period commencing on the closing date of the Share issuance. Thereafter, through the fifth anniversary of the closing date of the Share issuance, Alnylam will only dispose of the maximum number of Shares that it would be permitted to dispose if the Shares were subject to the volume restrictions set forth in Rule 144(e) of the Securities Act of 1933, as amended.

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 condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of our condensed consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements, as well as the revenue and expenses incurred during the reported periods. On an ongoing basis, we evaluate our estimates and judgments, including those related to revenue recognition and accrued expenses. 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.

The critical accounting policies that we believe impact significant judgments and estimates used in the preparation of our financial statements presented in this report are described in our Management's Discussion and Analysis of Financial Condition and Results of Operations in our Annual Report on Form 10-K filed with the SEC on March 8, 2018. There have been no changes to our critical accounting policies during the three or six months ended June 30, 2018 from those discussed in "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Significant Judgments and Estimates" in our Annual Report on Form 10-K filed with the SEC on March 8, 2018, except as discussed below.

Recent Accounting Pronouncements

A summary of recent accounting pronouncements that have been adopted or are expected to be adopted by the Company is included in note 1 to our condensed consolidated financial statements (see Part I, Item 1 –"Financial Statements" of this Quarterly Report on Form 10-Q). Additional information regarding relevant accounting pronouncements is provided below.

Adopted in 2018

Revenue recognition

In May 2014, the accounting guidance related to revenue recognition was amended to replace current guidance with a single, comprehensive standard for accounting for revenue from contracts with customers. The new guidance became effective for us on January 1, 2018. The new revenue standard applies to all contracts with customers, and only contracts with customers are in the scope of the new revenue standard. Once a contractual arrangement is scoped into the new guidance, revenue is recognized based on a model that includes identifying performance obligations and determining and allocating the transaction price to the performance obligations identified in the contract. Revenue is recognized as those performance obligations are satisfied. We elected to apply this new guidance retrospectively to all prior periods presented, and adoption of this new guidance did not have a significant quantitative impact on our condensed consolidated financial statements. Adoption of this guidance has resulted in additional revenue-related disclosures in the notes to our condensed consolidated financial statements.

Income taxes

New guidance issued in October 2017 related to income taxes is aimed at reducing complexity in accounting standards by eliminating the current exception that the tax effects of intra-entity asset transfers (such as intercompany sales or transfers of intellectual property) be deferred until the transferred asset is sold to a third party or otherwise recovered through use. Instead, the new guidance will require that a reporting entity recognize any tax expense from the sale of the asset in the seller's tax jurisdiction when the transfer occurs, even though the pre-tax effects of that transaction are eliminated in consolidation. Any deferred tax asset that arises in the buyer's jurisdiction would also be recognized at the time of the transfer. We adopted this new guidance on January 1, 2018, and such adoption did not have an impact on our condensed consolidated financial statements, largely given that we have not recorded any deferred tax assets or liabilities on our condensed consolidated balance sheet.

Statement of cash flows

In August 2017, the accounting guidance related to the statement of cash flows was amended with the intent of reducing diversity in practice as to the classification of certain transactions in the statement of cash flows. This guidance became effective for us on January 1, 2018, with no significant impact on our condensed consolidated financial statements. Additionally, in November 2017, new accounting guidance was issued related to the statement of cash flows implications related to restricted cash and cash equivalents. The guidance requires that the statement of cash flows explain the change during the period in the total of cash and cash equivalents, including amounts generally described as restricted cash or restricted cash equivalents. Entities are also required to reconcile such total to amounts on the balance sheet and disclose the nature of the restrictions. We applied this new guidance on January 1, 2018 and have made current and retrospective presentation adjustments such that transfers between restricted and unrestricted cash accounts no longer are reported as a cash flow in our condensed consolidated statement of cash flows.

Stock-based compensation

In May 2017, the accounting guidance related to stock-based compensation was amended to clarify when to account for a change to the terms or conditions of a share-based payment award as a modification. Per the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions, whereas under previous guidance, judgments about whether certain changes to an award are substantive may impact whether or not modification accounting is applied in certain situations. This new guidance is effective prospectively for annual periods beginning on or after December 15, 2017. We adopted this guidance on January 1, 2018, with no impact to our condensed

consolidated financial statements.

Not yet adopted

Leases

In February 2016, accounting guidance related to leases was issued that will require an entity to recognize leased assets and the rights and obligations created by those leased assets on the balance sheet and to disclose key information about an entity's leasing arrangements. This guidance will become effective for us on January 1, 2019, with early adoption permitted. We expect that the adoption of this guidance will impact our condensed consolidated financial statements and notes thereto, resulting, among other factors, from the recognition of a right of use asset and related liability related to our 2014 non-cancelable operating lease arrangement for our office and laboratory space in Cambridge, Massachusetts. As of June 30, 2018, and as presented below, our total future minimum lease obligation associated with this lease was $4.1 million, and a substantial portion of this commitment will remain outstanding at the time we adopt the new guidance. Our evaluation of this guidance and its full impact on our condensed consolidated financial statements will continue throughout 2018.

Nonemployee stock-based compensation

In June 2018, accounting guidance related to nonemployee share-based compensation was issued that will require an entity to apply the measurement and classification criteria consistent with the accounting for employee share-based compensation. This guidance will become effective for us on January 1, 2019, with early adoption permitted. We expect that the adoption of this guidance will not have a significant impact on our condensed consolidated financial statements.

Financial Operations Overview

Comparison of the Three and Six Months Ended June 30, 2018 and 2017

The following table summarizes the results of our operations for the periods indicated (amounts in thousands, except percentages).

	Three Months Ended June 30, 2018	Three Months Ended June 30, 2017	Increase/ (Decrease)	Six Months Ended June 30, 2018	Six Months Ended June 30, 2017	Increase/ (Decrease)
Revenue from collaborative arrangements	$ 1,545	$ —	$ 1,545	$ 3,090	$ —	$ 3,090
Expenses:						
Research and development	10,339	9,068	1,271	20,232	17,811	2,421
General and administrative	4,760	4,066	694	9,095	8,188	907
Litigation expense	22,244	2,234	20,010	25,428	3,608	21,820
Total operating expenses	37,343	15,368	21,975	54,755	29,607	25,148
Loss from operations	(35,798)	(15,368)	(20,430)	(51,665)	(29,607)	(22,058)
Other income (expense):						
Interest income	330	143	187	619	181	438
Interest expense	(176)	—	(176)	(176)	—	(176)
Total other income, net	154	143	11	443	181	262
Net loss	(35,644)	(15,225)	(20,419)	(51,222)	(29,426)	(21,796)
Dividends on redeemable convertible preferred stock	—	(2,622)	2,622	—	(2,622)	2,622
Deemed dividend related to beneficial conversion feature of redeemable convertible preferred stock	—	(6,144)	6,144	—	(6,144)	6,144
Net loss attributable to common stockholders	$ (35,644)	$ (23,991)	$ (11,653)	$ (51,222)	$ (38,192)	$ (13,030)

Revenue from collaborative arrangements

Revenue recognized during the three and six months ended June 30, 2018 relates primarily to the BI Agreement, pursuant to which BI agreed to pay us a non-refundable upfront payment of $10.0 million for the first target and agreed to reimburse us for the cost of certain materials and third-party expenses that have been included in the preclinical studies. Revenue recognized to date is primarily comprised of the periodic amortization of the aforementioned upfront payment.

We recognized $1.5 million and $3.1 million of the $10.3 million transaction price during the three and six months ended June 30, 2018, respectively, which explains the increase as compared to the three and six months ended June 30, 2017, respectively, during which no collaboration revenues were recorded.

We do not expect to generate any product revenue for the foreseeable future.

Research and development expenses

The following table summarizes our research and development expenses incurred during the periods indicated (amounts in thousands).

	Three Months Ended June 30,		Increase
	2018	2017	(Decrease)
Direct research and development expenses	$5,066	$4,016	$ 1,050
Platform-related expenses	1,425	1,707	(282)
Employee-related expenses	3,053	2,553	500
Facilities, depreciation and other expenses	795	792	3
Total	$10,339	$9,068	$ 1,271

	Six Months Ended June 30,		Increase
	2018	2017	(Decrease)
Direct research and development expenses	$9,831	$7,314	$ 2,517
Platform-related expenses	2,538	3,541	(1,003)
Employee-related expenses	6,255	5,347	908
Facilities, depreciation and other expenses	1,608	1,609	(1)
Total	$20,232	$17,811	$ 2,421

Research and development expenses were $10.3 and $20.2 million for the three and six months ended June 30, 2018, respectively, as compared to $9.1 million and $17.8 million for the three and six months ended June 30, 2017, respectively, due to higher direct research and development and employee-related expenses, partially offset by a reduction in platform-related expenses. The increase in the three months ended June 30, 2018 was primarily due to an increase of $0.7 million in clinical study costs and a $0.5 million increase in research and development salaries due to an increase in research and development headcount compared to the three months ended June 30, 2017. The increase in the six months ended June 30, 2018 was primarily due to a $1.4 million increase in toxicology studies and an $0.8 million increase in clinical studies.

The decrease in platform-related expenses for the three and six months ended June 30, 2018 was primarily due to $0.2 million and $0.7 million of lab material and supply costs, and $0.1 million and $0.2 million of contract research costs associated with applying our GalXC platform against gene targets, respectively.

We expect our overall research and development expenses to increase during the third and fourth quarters of 2018 as compared to the three and six months ended June 30, 2018, primarily as we complete clinical manufacturing activities, advance pre-clinical toxicology studies, and continue clinical activities associated with our lead product candidates.

General and administrative expenses

General and administrative expenses were $4.8 million and $9.1 million for the three and six months ended June 30, 2018, respectively, as compared to $4.1 million and $8.2 million for the three and six months ended June 30, 2017, respectively. The increase in the three and six months ended June 30, 2018 is predominantly related to higher consulting of $0.4 million and $0.3 million, and $0.1 million and $0.2 million of corporate legal expenses, respectively.

Litigation expenses

Litigation expenses, all related to the litigation with Alnylam, were $22.2 million and $25.4 million for the three and six months ended June 30, 2018, respectively, as compared to $2.2 million and $3.6 million for the three and six months ended June 30, 2017, respectively. The increase is predominantly due to $21.0 million of settlement expenses related to the litigation settlement recorded in the three months ended June 30, 2018.

Interest income

Interest income primarily represents interest earned from our money market accounts and held-to-maturity investments. Interest income was $0.3 million and $0.4 million for the three and six months ended June 30, 2018, respectively, as compared to $0.1 million and $0.2 million for the three and six months ended June 30, 2017, respectively. The increases were primarily due to higher cash and cash equivalent balances during the three and six months ended June 30, 2018 resulting from financing activities completed in April and December of 2017.

Interest expense

Interest expense of $0.2 million during the three and six months ended June 30, 2018 represents interest expense incurred on our long-term payable with Alnylam.

Dividends

There were no dividends recorded related to redeemable convertible preferred stock for the three and six months ended June 30, 2018, as all shares of the redeemable convertible preferred stock were converted into shares of our common stock on December 18, 2017.

Net loss attributable to common stockholders

Net loss attributable to common stockholders was $35.6 million and $51.2 million for the three and six months ended June 30, 2018, as compared to $24.0 million and $38.2 million for the three and six months ended June 30, 2017, respectively. This increase is attributable to higher operating expenses, including the $21.0 million in litigation settlement expenses, partially offset by higher revenues, interest income, and elimination of the redeemable convertible preferred stock, as discussed above.

Liquidity and Capital Resources

As of June 30, 2018, we had cash and cash equivalents and held-to-maturity investments of $82.3 million and $0.7 million in cash equivalents held in restriction.

On October 31, 2016, a universal shelf registration statement on Form S-3 permitting the sale of up to $150.0 million of our common stock and other securities was declared effective by the U.S. Securities and Exchange Commission (“SEC”). In December 2017, we sold an aggregate of 24,206,663 shares of our common stock, for gross proceeds of $46.0 million, pursuant to this registration statement.

On May 31, 2018, a universal shelf registration statement on Form S-3 permitting the sale of up to $250.0 million of our common stock and other securities was declared effective by the SEC.

Cash flows

The following table shows a summary of our cash flows for the periods indicated (amounts in thousands).

Six Months Ended

	June 30, 2018	2017
Net cash used in operating activities	$(32,273)	$(26,898)
Net cash provided by (used in) investing activities	5,037	(24,966)
Net cash provided by financing activities	873	69,776
(Decrease) increase in cash, cash equivalents, and restricted cash equivalents	$(26,363)	$17,912

Operating activities

Net cash used in operating activities was $32.3 million for the six months ended June 30, 2018, as compared to $26.9 million for the six months ended June 30, 2017. The increase of $5.4 million was primarily due to litigation and research and development expenses of $2.6 million and $2.4 million, respectively.

Investing activities

Net cash provided by investing activities was $5.0 million for the six months ended June 30, 2018, as compared to cash used in investing activities of $25.0 million for the six months ended June 30, 2017. The increase of $30.0 million was due to a $20.1 million decline in purchases of, and a $10.0 million increase in maturities of, held-to-maturity investments, respectively.

Financing activities

Net cash provided by financing activities was $0.9 million for the six months ended June 30, 2018, as compared to $69.8 million for the six months ended June 30, 2017. The decrease of $69.0 million was primarily due to receipt of $70.0 million in gross proceeds in April 2017 from the Private Placement of redeemable convertible preferred stock.

Funding requirements

We expect that our primary uses of capital will continue to be third-party clinical research and development services and manufacturing costs, compensation and related expenses, laboratory and related supplies, legal and other regulatory expenses, and general overhead costs. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates and the extent to which we may enter into additional collaborations with third parties to participate in their development and commercialization, we are unable to estimate the amounts of capital outlays and operating expenditures associated with our anticipated development activities. However, based on our current operating plan, we believe that available cash, cash equivalents, and held-to-maturity investments will be sufficient to fund our planned level of operations for at least the 12-month period following August 8, 2018. 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 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. Our future capital requirements are difficult to forecast and will depend on many factors, including:

- the receipt of any milestone payments under the BI Agreement;
- 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 product candidates;
- the number and characteristics of product candidates that we pursue;
- 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;
- 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 of responding to and defending ourselves against complaints and potential litigation (see Part II, Item 1—"Legal Proceedings" in this Quarterly Report on Form 10-Q);
- the costs and timing of procuring clinical and commercial supplies for our product candidates;
- the extent to which we acquire or in-license other product candidates and technologies; and
- the extent to which we acquire or invest in other businesses, product candidates, or technologies.

Until such time, if ever, that 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 in a reasonable timeframe would have a negative impact on our financial condition, and we may have to delay, reduce, or terminate our research and development programs, preclinical or clinical trials, limit strategic opportunities, or undergo reductions in our workforce or other corporate restructuring activities.

Please see the risk factors set forth in Part II, Item 1A –"Risk Factors" in this Quarterly Report on Form 10-Q for additional risks associated with our substantial capital requirements.

Contractual Obligations and Commitments

The following is a summary of our significant contractual obligations as of June 30, 2018 (amounts in thousands):

	Payments Due By Period				
	Total	Less Than 1 Year	More Than 1 Year and Less Than 3 Years	More Than 3 Years and Less Than 5 Years	More Than 5 Years
Operating lease obligation*	$4,075	$1,654	$2,421	$ —	$ —

*Represents future minimum lease payments under our existing non-cancelable operating lease for our office and laboratory space in Cambridge, Massachusetts. The end of the lease term is November 30, 2020.

We also have obligations to make future payments to licensors that become due and payable on the achievement of certain development, regulatory, and commercial milestones. We have not included any such potential obligations on our condensed consolidated balance sheet or in the table above, since the achievement and timing of these milestones were not probable or estimable as of June 30, 2018.

See also Part II, Item 1 –"Legal Proceedings" in this Quarterly Report on Form 10-Q for additional information related to litigation. We recorded $8.9 million of litigation liabilities related to Alnylam on our condensed consolidated balance sheet as of June 30, 2018.

Off-balance Sheet Arrangements

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

Item 3. QUANTITATIVE AND QUALITATIVE DISCLOSURE 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 June 30, 2018, we had cash and cash equivalents and held-to-maturity investments of $82.3 million. 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 and cash equivalents and held-to-maturity investments 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 and cash equivalents or held-to-maturity investments. To minimize the risk in the future, we intend to maintain our portfolio of cash and cash equivalents and held-to-maturity investments in a variety of securities, including commercial paper, money market funds, and government securities.

Item 4. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our periodic and current reports that we file under the Securities Exchange Act of 1934, as amended, with the SEC is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our chief executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

As of the end of the period covered by this Quarterly Report on Form 10-Q, we carried out an evaluation, under the supervision and with the participation of our management, including the chief executive officer and the chief financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures pursuant to Exchange Act Rule 13a-15. Based upon, and as of the date of, this evaluation, the chief executive officer and the chief financial officer concluded that our disclosure controls and procedures were effective. Accordingly, management believes that the financial statements included in this report fairly present in all material respects our financial condition, results of operations, and cash flows for the periods presented.

Changes in Internal Control Over Financial Reporting

There was no change in our internal control over financial reporting during the quarter ended June 30, 2018, which was identified in connection with management's evaluation required by Exchange Act Rules 13a-15 and 15d-15 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Inherent Limitations on the Effectiveness of Controls

Our management, including the chief executive officer and chief financial officer, does not expect that our disclosure controls and procedures or our internal control over financial reporting will prevent all error and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within the company have been detected. These inherent limitations include the realities that judgments in decision making can be faulty and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. The design of any system of controls is also based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions. Over time, controls may become inadequate because of changes in conditions, or the degree of compliance with the policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system,

misstatements due to error or fraud may occur and not be detected.

PART II: OTHER INFORMATION

Item 1. 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.

On June 10, 2015, Alnylam Pharmaceuticals, Inc. ("Alnylam") filed a complaint against the Company in the Superior Court of Middlesex Country, Massachusetts (the "Court"). The complaint alleged misappropriation of confidential, proprietary, and trade secret information, as well as other related claims, in connection with the Company's hiring of a number of former employees of Merck & Co., Inc. ("Merck") and its discussions with Merck regarding the acquisition of its subsidiary, Sirna Therapeutics, Inc. ("Sirna"), which was subsequently acquired by Alnylam.

On April 18, 2018, the Company and Alnylam entered into a Confidential Settlement Agreement and General Release (the "Settlement Agreement") resolving all ongoing litigation between the Company and Alnylam. The terms of the Settlement Agreement include mutual releases and dismissals with prejudice of all claims and counterclaims in the following litigation between the parties: (i) Alnylam Pharmaceuticals, Inc. v. Dicerna Pharmaceuticals, Inc., No. 15-4126 pending in the Massachusetts Superior Court for Middlesex County and (ii) Dicerna Pharmaceuticals, Inc., v. Alnylam Pharmaceuticals, Inc. No.1:17-cv-11466 pending in the United States District Court for the District of Massachusetts. Pursuant to the terms of the Settlement Agreement, the Company has agreed to make the following payments to Alnylam: (i) a $2.0 million upfront payment in cash; (ii) an additional $13.0 million in cash, to be paid as 10% of any upfront or first year cash consideration that the Company receives pursuant to future collaborations related to Ga1NAc-conjugated RNAi research and development (excluding any amounts received or to be received by the Company from its existing collaboration with BI), provided that the $13.0 million must be paid by no later than April 28, 2022; and (iii) issuance of shares of the Company's common stock (the "Shares") pursuant to a share issuance agreement between the parties (the "Share Issuance Agreement").

Under the Settlement Agreement, the Company is restricted in its development and other activities relating to oligonucleotide-based therapeutics directed toward a defined set of eight Alnylam targets, for periods ranging from 18 months up to four years (the "Oligo Restrictions"). The Oligo Restrictions pertain to targets where Dicerna does not have, or does not currently intend to have, a therapeutic program, or are expected to be consistent with Dicerna's execution on programs in the normal course of business. The Settlement Agreement does not include any admission of liability or wrongdoing by either party or any licenses to any other intellectual property from either party.

On April 20, 2018, the Company and Alnylam entered into the Share Issuance Agreement, pursuant to which the Company agreed to issue to Alnylam 983,208 Shares in satisfaction of the Company's obligation under the Settlement Agreement to deliver Shares to Alnylam. The Share Issuance Agreement contains customary representations and warranties of each party. Pursuant to the terms of the Share Issuance Agreement, Alnylam may not, without the prior approval of the Company, dispose of any of the Shares for a six-month period commencing on the closing date of the Share issuance. Thereafter, through the fifth anniversary of the closing date of the Share issuance, Alnylam will only dispose of the maximum number of Shares that it would be permitted to dispose if the Shares were subject to the volume restrictions set forth in Rule 144(e) of the Securities Act of 1933, as amended.

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 Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended. 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 will need to raise substantial additional funds to advance development of our product candidates, and we cannot guarantee that we will have sufficient funds available in the future to develop and commercialize our current or future product candidates.

We will need to raise substantial additional funds to expand our development, regulatory, manufacturing, marketing, and sales capabilities, whether internally or through other organizations. We have used substantial funds to develop our product candidates and delivery technologies and will require significant funds to conduct further research and development and preclinical testing and clinical trials of our product candidates, to seek regulatory approvals for our product candidates, and to manufacture and market products, if any are approved for commercial sale. As of June 30, 2018, we had $82.3 million in cash and cash equivalents and held-to-maturity investments. Based on our current operating plan and liquidity, we believe that our available cash, cash equivalents, and held-to-maturity investments will be sufficient to fund our planned level of operations for at least the 12-month period following August 8, 2018. 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. Because the length of time and activities associated with successful development of our product 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 product candidates;
- to build and maintain a strong intellectual property portfolio and avoid infringing 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 product candidates;
- to manage our spending as costs and expenses increase due to preclinical studies and clinical trials, regulatory approvals, manufacturing scale-up, 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.

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 technologies or product 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 product candidates are clinically tested, approved for commercialization, and successfully marketed. To date, we have financed our operations primarily through the sale of securities, debt financings, and credit and loan facilities. We will be required to seek additional funding in the future and intend to do so through a combination of public or private equity offerings, debt financings, and research collaborations and license agreements. Our ability to raise additional funds will depend on financial, economic, and other factors, many of which are beyond our control. For example, a number of factors, including the timing and outcomes of our clinical activities, our status as a smaller reporting company under SEC regulations, as well as conditions in the global financial markets, may present significant challenges to accessing the capital markets at a time when we would like or require, and at an increased cost of capital. 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 receive any distribution of corporate assets.

We are a biopharmaceutical company with a history of losses, 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 biopharmaceutical company with a limited operating history focused on the discovery and development of treatments based on the emerging therapeutic modality RNAi, a biological process in which RNA molecules inhibit gene expression. Since our inception in October 2006, we have devoted our resources to the development of DsiRNA molecules and delivery technologies. We have had significant operating losses since our inception. As of June 30, 2018, we had an accumulated deficit of $367.2 million. For the six months ended June 30, 2018 and for the years ended December 31, 2017, 2016, and 2015, our net loss was $51.2 million, $80.1 million, $59.5 million, and $62.8 million, respectively. Substantially all of our operating losses have resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations. Our technologies and product candidates are in early stages of development, and we are subject to the risks of failure inherent in the development of product candidates based on novel technologies.

We have not generated, and do not expect to generate, any revenue from product sales 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 and clinical trials, and the regulatory approval process for product candidates. The amount of future losses is uncertain. Our ability to achieve profitability, if ever, will depend on, among other things, us or our existing collaborators, or any future collaborators, successfully developing product candidates, obtaining regulatory approvals to market and commercialize product 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 our existing collaborators, or any future collaborators, are unable to develop and commercialize one or more of our product candidates or if sales revenue from any product 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 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 product candidates or future development programs;
- results of clinical trials, or the addition or termination of clinical trials or funding support by us, our existing collaborators, or any future collaborator or licensor;
- the timing of the release of results from any clinical trials conducted by us or our collaborator BI;
- 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 or misappropriation lawsuit or opposition, interference, re-examination, post-grant review, inter partes review, nullification, derivation action, or cancellation proceeding in which we may become involved;
- additions and departures of key personnel;
- strategic decisions by us and our competitors, such as acquisitions, divestitures, spin-offs, joint ventures, strategic investments, or changes in business strategy;
- if any of our product candidates receive regulatory approval, market acceptance and demand for such product candidates;
- if any of our third-party manufacturers fail to execute on our manufacturing requirements;

- regulatory developments affecting our product candidates or those of our competitors;
- disputes concerning patents, proprietary rights, or license and collaboration agreements that negatively impact our receipt of milestone payments or royalties or require us to make significant payments arising from licenses, settlements, adverse judgments, or ongoing royalties; and
- changes in general market and economic conditions.

If our quarterly operating results fluctuate or fall below the expectations of investors or securities analysts, the price of our common stock could fluctuate or decline substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

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

We plan to develop subcutaneously delivered RNAi-based pharmaceuticals using our GalXC RNAi platform for the treatment of rare diseases involving the liver and for other therapeutic areas involving the liver such as chronic liver diseases, as well as cardiovascular diseases and viral infectious diseases. We believe that product candidates identified with our drug discovery and delivery platform may offer an improved therapeutic approach to small molecules and monoclonal antibodies, as well as several advantages over earlier generation RNAi molecules. However, the scientific research that forms the basis of our efforts to develop product candidates is relatively new. The scientific evidence to support the feasibility of developing therapeutic treatments based on RNAi and GalXC is both preliminary and limited.

Relatively few product candidates based on RNAi have been tested in animals or humans, and a number of clinical trials conducted by other companies using RNAi technologies have not been successful. We may discover that GalXC does not possess certain properties required for a drug to be safe and effective, such as the ability to remain stable in the human body for the period of time required for the drug to reach the target tissue or the ability to cross the cell wall 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 GalXC. We may spend substantial funds attempting to introduce these properties and may never succeed in doing so. In addition, product candidates based on GalXC may demonstrate different chemical and pharmacological properties in patients than they do in laboratory studies. Even if product candidates, such as DCR-PHXC, 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 product, we may not become profitable, and the value of our common stock will decline.

Further, the FDA has relatively limited experience with RNAi or GalXC based therapeutics. No regulatory authority has granted approval to any person or entity, including us, to market and commercialize therapeutics using RNAi or GalXC, which may increase the complexity, uncertainty, and length of the regulatory approval process for our product candidates. We and our current collaborators, or any future collaborators, may never receive approval to market and commercialize any product candidate. Even if we or a 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 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 technologies based on GalXC prove to be ineffective, unsafe, or commercially unviable, our entire platform and pipeline would have little, if any, value, which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

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

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

community and third-party payors, including health insurers, to accept and use, or to provide favorable coverage or reimbursement for, any product candidates developed by us or our existing collaborator or any future collaborators. Market acceptance of our product 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 product candidates;
- the prevalence and severity of any adverse side effects associated with our product candidates;
- limitations or warnings contained in any labeling approved by the FDA or other regulatory authority;
- relative convenience and ease of administration of our product candidates;
- the willingness of physicians and patients to accept any new methods of administration;

- the success of our physician education programs;
- the availability of adequate government and third-party payor coverage and reimbursement;
- the pricing of our products, particularly as compared to alternative treatments;
- our ability to compliantly market and sell our products; and
- availability of alternative effective treatments for the disease indications our product candidates are intended to treat and the relative risks, benefits and costs of those treatments.

With our focus on the emerging therapeutic modality RNAi, these risks may increase to the extent the market becomes more competitive or less favorable to this approach. Additional risks apply to any disease indications we pursue which are for rare diseases. 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 rare disease product, such drug may not generate enough revenue to offset costs of development, manufacturing, marketing, and commercialization, despite any benefits received from our efforts to obtain orphan drug designation by regulatory agencies in major commercial markets, such as the U.S., the European Union ("EU"), and Japan. These benefits may include 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 that are not classified as rare. Our estimates regarding potential market size for any indication may be materially different from what we discover to exist if we ever get to the point of product commercialization, 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 product candidate that has orphan drug designation subsequently receives the first FDA approval for the indication for that designation, the product candidate is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same product 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 product candidates for seven years if a competitor obtains approval of the same product candidate as defined by the FDA.

Even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective, or makes a major contribution to patient care.

On August 3, 2017, the Congress passed the FDA Reauthorization Act of 2017, or FDARA. FDARA, among other things, codified the FDA's preexisting regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

As in the U.S., we may apply for designation of a product candidate as an orphan drug for the treatment of a specific indication in the EU before the application for marketing authorization is made. Sponsors of orphan drugs in the EU can enjoy economic and marketing benefits, including up to 10 years of market exclusivity for the approved indication. During such period, marketing authorization applications for a "similar medicinal product" will not be accepted, unless another applicant can show that its product is safer, more effective, or otherwise clinically superior to the orphan-designated product. 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. The respective orphan designation and exclusivity frameworks in the U.S. and in the EU are subject to change, and any such changes may affect our ability to obtain EU or U.S. orphan designations in the future.

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

We have no products on the market and all of our product candidates are in early stages of development. Our ability to achieve and sustain profitability depends on obtaining regulatory approvals, including ethics committee approval to conduct clinical trials at particular sites, and successfully commercializing our product candidates, either alone or with third parties, such as our collaborator BI. Before obtaining regulatory approval for the commercial distribution of our product candidates, we or a collaborator must conduct extensive preclinical tests and clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Preclinical testing 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 study 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 drug or required prior therapy, clinical outcomes, and 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 product candidate require the enrollment of a sufficient number of patients, including patients who are suffering from the disease the product 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 product candidate can unexpectedly fail at any stage of preclinical and clinical development. The historical failure rate for product candidates is high due to many factors, including scientific feasibility, safety, efficacy, and changing standards of medical care. The results from preclinical testing or early clinical trials of a product candidate may not predict the results that will be obtained in later phase clinical trials of the product candidate. We, the FDA, the applicable Institutional Review Board ("IRB"), an independent ethics committee, or other applicable regulatory authorities may suspend clinical trials of a product candidate at any time for various reasons, including a belief that individuals participating in such trials are being exposed to unacceptable health risks or adverse side effects. 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 product candidate if we experience any problems or other unforeseen events that delay or prevent regulatory approval of, or our ability to commercialize, product candidates, including:

- negative or inconclusive results from our clinical trials or the clinical trials of others for product candidates similar to ours, leading to a decision or requirement to conduct additional preclinical testing or clinical trials or abandon a program;
- serious and unexpected drug-related side effects experienced by participants in our clinical trials or by individuals using drugs similar to our product candidates;
- delays in submitting INDs 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"), regarding the scope or design of our clinical trials;
- delays in enrolling individuals in clinical trials;
- high drop-out rates of study participants;
- inadequate supply or quality of drug product or product 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 product 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; and
- varying interpretations of data by the FDA and foreign regulatory agencies.

We are dependent on BI for the successful development of product candidates in the collaboration.

On October 27, 2017, we entered into the BI Agreement to jointly research and develop candidate products using the GalXC platform to target specific disease-linked genes in the hepatocytes for the treatment of NASH. Under the terms of the BI Agreement, BI agreed to pay us a non-refundable upfront payment of $10.0 million, and we will be eligible to receive up to $191.0 million in development and commercial milestones related to the initial target and royalty payments on global net sales. Once a product candidate is selected, the success of our collaboration with BI and the

realization of the milestone and royalty payments under the BI Agreement depends entirely upon the efforts of BI, which may not be successful in obtaining approvals for the product candidates developed under the collaboration or in marketing, or arranging for necessary supply, manufacturing or distribution relationships for, any approved products. BI may change its strategic focus or pursue alternative technologies in a manner that results in reduced, delayed, or no revenue to us. BI has a variety of marketed products and product candidates under collaboration with other companies, possibly including some of our competitors, and BI's own corporate objectives may not be consistent with our interests. If BI fails to develop, obtain regulatory approval for, or ultimately commercialize any product candidate under our collaboration, or if BI terminates our collaboration, our business, financial condition, results of operations, and prospects could be materially and adversely affected. In

addition, if we have a dispute or enter into litigation with BI in the future, it 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, or any future 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 of our product candidates and will do the same for any clinical trials. 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, compliance, 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 from our programs. The third parties with which we contract might not be diligent, careful, compliant, 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 as well as applicable laws and regulations. The FDA and certain foreign regulatory authorities, such as the EMA, require preclinical studies to be conducted in accordance with applicable good laboratory practices and clinical trials to be conducted in accordance with applicable FDA regulations and applicable good clinical practices, 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 such event 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 supply and manufacturing companies and organizations to supply the materials, components, and manufacturing services for our research and development, preclinical study, and clinical trial drug supplies.

We do not own or lease manufacturing facilities or supply sources for such components and materials. Our manufacturing requirements include oligonucleotides and custom amidites, some of which we procure from a single source supplier on a purchase order basis. In addition, for each product candidate we typically contract with only one manufacturer for the formulation and filling of drug product. There can be no assurance that our supply of research and development, preclinical study, and clinical trial drugs 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 substance manufacturer could require significant effort and expertise because there may be a limited number of qualified replacements.

If we are at any time unable to provide an uninterrupted supply of our product candidates or, following regulatory approval, any products to patients, we may lose patients, physicians may elect to utilize competing therapeutics instead of our products, and our clinical trials may be adversely affected, which could materially and adversely affect our clinical trial outcome.

The manufacturing process for a product candidate is subject to FDA and foreign regulatory authority review. 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 standards, such as current good manufacturing practices ("cGMP"). In the event that any of our suppliers or manufacturers fails to comply with such requirements or to perform its obligations regarding quality, timing or otherwise, or if our supply of components or other materials becomes limited or interrupted for other reasons, we may experience shortages resulting in delayed shipments, supply constraints and/or stock-outs of our products, 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 product 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 product 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 product 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 product 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 product candidates, or to do so on commercially reasonable terms, we may not be able to develop and commercialize our product candidates successfully. Our or a third party's failure to execute on our manufacturing requirements could adversely affect our business in a number of ways, including:

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

We may not successfully engage in strategic transactions, including any additional collaborations we seek, which could adversely affect our ability to develop and commercialize product 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 product candidates or technologies. In particular, in addition to our current collaboration with BI, we will evaluate and, if strategically attractive, seek to enter into additional 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 or existing collaboration if, for example, development or approval of a product candidate is delayed, sales of an approved product 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 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 products, product 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 have a material adverse effect on our business, results of operations, financial condition, and prospects. Conversely, any failure to enter any collaboration or other strategic transaction that would be beneficial to us could delay the development and potential commercialization of our product candidates and have a negative impact on the competitiveness of any product candidate that reaches market.

We face competition from entities that have developed or may develop product 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 or product candidates more rapidly than we do or their technologies, including delivery technologies, are more effective, our ability to develop and successfully commercialize product candidates may be adversely affected.

The development and commercialization of drugs is highly competitive. We compete with a variety 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 may develop product candidates and processes competitive with our product candidates, some of which may become commercially available before any of our product 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 are aware of many companies that are working in the field of RNAi therapeutics, including major pharmaceutical companies and a number of biopharmaceutical companies including Alnylam, Arrowhead Pharmaceuticals, Inc. ("Arrowhead"), and Arbutus Biopharma Corporation. We believe that a significant number of products are currently under development, and may become commercially available in the future, for the treatment of conditions for which we may try to develop product candidates.

We also compete with companies working to develop antisense and other RNA-based drugs. Like RNAi therapeutics, antisense drugs target mRNA with the objective of suppressing the activity of specific genes. The development of antisense drugs is more advanced than that of RNAi therapeutics, and antisense technology may become the preferred technology for products that target mRNAs. Significant competition also exists from companies such as Alnylam and Arrowhead to discover and develop safe and effective means to deliver therapeutic RNAi molecules, such as DsiRNAs, to the relevant cell and tissue types.

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

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: Douglas M. Fambrough, III, Ph.D., our chief executive officer; Bob D. Brown, Ph.D., our chief scientific officer; Ralf Rosskamp, M.D., our chief medical officer; John B. Green, our chief financial officer; and James B. Weissman, our chief business 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 complex nature of our product candidates and technologies 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 other 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 product candidates advance into clinical trials, we may experience difficulties in managing our growth and expanding our operations.

We have limited experience in drug development and very limited experience with clinical trials of product candidates. As our product candidates enter and advance through preclinical studies and any 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, 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 product 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 products.

We currently have no sales, marketing, or distribution capabilities or experience. If any of our product candidates are approved, we will need to develop internal sales, marketing, and distribution capabilities to commercialize such products, which would be expensive and time-consuming, or enter into collaborations with third parties to perform these services. If we decide to market our products directly, we will need to commit significant financial, legal, 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 products or decide to co-promote products with collaborators, we will need to establish and maintain 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, compliant 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 product. If we are not successful in commercializing any product 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. and foreign regulatory requirements, regulatory authorities could limit or withdraw any marketing or commercialization approvals we may receive and subject us to other penalties that could materially harm our business.

The Company, our product candidates, our suppliers, and our contract manufacturers, distributors, and contract testing laboratories are subject to extensive regulation by governmental authorities in the EU, the U.S., and other countries, with the regulations differing from country to country.

Even if we receive marketing and commercialization approval of a product candidate, we and our third-party services 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, post-approval clinical studies, labeling, advertising and promotional activities, record keeping, distribution, adverse event reporting, import and export of pharmaceutical products, pricing, sales and marketing, and fraud and abuse requirements. 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 product and clinical results th