Dicerna Pharmaceuticals Inc
Form 10-Q
August 10, 2017
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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, 2017

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

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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 9, 2017, there were 20,844,429 shares of the registrant s common stock, par value $0.0001 per share, outstanding.

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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, 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 (IND) application, New Drug Application (NDA) 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 future clinical trials;

our reliance on third party supply and manufacturing partners 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, Kyowa Hakko Kirin Co., Ltd. (KHK), 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 KHK or arrangement with any future collaborator;

our expectations regarding the time during which we will be an emerging growth company under the Jumpstart Our Business Startups Act;

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.

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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.

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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, 2017	December 31, 2016
ASSETS		
CURRENT ASSETS:		
Cash and cash equivalents	$ 38,777	$ 20,865
Held-to-maturity investments (Note 3)	49,953	25,009
Prepaid expenses and other current assets	2,966	1,952
Total current assets	91,696	47,826
NONCURRENT ASSETS:		
Property and equipment net	1,837	2,234
Restricted cash equivalents	1,116	1,116
Other noncurrent assets	74	76
Total noncurrent assets	3,027	3,426
TOTAL ASSETS	$ 94,723	$ 51,252
LIABILITIES AND STOCKHOLDERS EQUITY		
CURRENT LIABILITIES:		
Accounts payable	$ 4,129	$ 4,318
Accrued expenses and other current liabilities	5,454	5,726
Total current liabilities	9,583	10,044
TOTAL LIABILITIES	9,583	10,044
Commitments and contingencies (Note 6)		
Redeemable convertible preferred stock, $0.0001 par value 5,000,000 shares authorized; 718,404 and no shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively (aggregate liquidation preference of $71,841 and $0 at June 30, 2017 and December 31, 2016, respectively) (Note 4)	71,872	

STOCKHOLDERS EQUITY:		
Common stock, $0.0001 par value 150,000,000 shares authorized at June 30, 2017 and December 31, 2016; 20,794,427 shares and 20,753,001 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	2	2
Additional paid-in capital	298,448	296,962
Accumulated deficit	(285,182)	(255,756)
Total stockholders equity	13,268	41,208
TOTAL LIABILITIES AND STOCKHOLDERS EQUITY	$ 94,723	$ 51,252

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

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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,	
	2017	2016	2017	2016
Revenue	$ 252	$	$ 385	$
Operating expenses:				
Research and development	9,320	11,032	18,196	22,296
General and administrative	6,300	4,656	11,796	9,140
Total operating expenses	15,620	15,688	29,992	31,436
Loss from operations	(15,368)	(15,688)	(29,607)	(31,436)
Interest income	143	66	181	121
Net loss	(15,225)	(15,622)	(29,426)	(31,315)
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	$ (23,991)	$ (15,622)	$ (38,192)	$ (31,315)
Net loss per share attributable to common stockholders basic and diluted	$ (1.15)	$ (0.75)	$ (1.84)	$ (1.51)
Weighted average common shares outstanding basic and diluted	20,794,193	20,726,108	20,792,925	20,706,388

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

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DICERNA PHARMACEUTICALS, INC.

Condensed Consolidated Statements of Cash Flows

(Unaudited)

(In thousands)

	Six Months Ended June 30,	
	2017	**2016**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (29,426)	$ (31,315)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	4,033	4,795
Depreciation and amortization	363	414
Net amortization of premium/discount on investments	(35)	64
Loss on disposal of property and equipment	51	
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,013)	53
Accounts payable	(599)	580
Accrued expenses and other liabilities	(276)	(181)
Deferred rent	4	48
Net cash used in operating activities	(26,898)	(25,542)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property and equipment	(58)	(279)
Maturities of held-to-maturity investments	25,000	18,500
Purchases of held-to-maturity investments	(49,908)	(20,016)
Net cash used in investing activities	(24,966)	(1,795)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	70,000	
Redeemable convertible preferred stock issuance costs	(300)	
Proceeds from stock option exercises and issuances under employee stock purchase plan	87	493
Settlement of restricted stock for tax withholding	(11)	(27)
Net cash provided by financing activities	69,776	466
INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	17,912	(26,871)
CASH AND CASH EQUIVALENTS Beginning of period	20,865	56,058
CASH AND CASH EQUIVALENTS End of period	$ 38,777	$ 29,187

SUPPLEMENTAL CASH FLOW INFORMATION:				
NONCASH FINANCING ACTIVITIES:				
Dividends on redeemable convertible preferred stock	$	2,622	$	
Deemed dividend related to beneficial conversion feature of redeemable convertible 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	$		$	29

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

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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. (the Company) 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, chronic liver diseases, cardiovascular diseases and viral infectious 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, 2017 and results of operations and cash flows for the interim periods ended June 30, 2017 and 2016. 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, 2016, as amended. The results of the three and six months ended June 30, 2017 are not necessarily indicative of the results to be expected for the year ending December 31, 2017 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 consolidated financial statements, as well as the revenue and expenses incurred during the reporting periods. On an ongoing basis, the Company evaluates judgments and estimates, including those related to accrued expenses, stock-based compensation and in relation to the accounting for, including cumulative dividends on, the Redeemable Convertible Preferred, as defined below. 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 risk

Based on the Company s current operating plan and liquidity, including the receipt of gross proceeds of $70.0 million from the issuance of the Company s Redeemable Convertible Preferred, as defined below, on April 11, 2017 (see Note 4), 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 10, 2017, 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 planned preclinical and clinical operations, including completion of its planned clinical trials, will depend on its 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, as amended, except as required by recently adopted accounting pronouncements, as discussed below, and as related to the Redeemable Convertible Preferred, as defined below.

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Recent Accounting Pronouncements

Adopted in 2017

Stock-based compensation

In March 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2016-09, *Compensation Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting* (ASU 2016-09), which involves several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities and classification in the statement of cash flows. Also under the new guidance, excess tax benefits and deficiencies are to be recognized as income tax expense or benefit in the income statement as discrete items in the reporting period in which they occur instead of an increase or decrease to stockholders equity. With regard to forfeitures, an entity may make an accounting policy election either to estimate the number of awards that are expected to vest or account for forfeitures when they occur. The Company adopted ASU 2016-09 on January 1, 2017, and as a result, it will track stock option deductions in its net operating loss deferred tax asset on a modified retrospective basis. In addition, the Company s policy has been to estimate forfeitures as of the grant date. The Company will continue to maintain its policy to estimate forfeiture as of the grant date in the future. Since the Company historically has maintained a full valuation allowance on its net deferred tax asset, there is no net impact to the Company s accumulated deficit or on its net loss per share attributable to common stockholders from the adoption of ASU 2016-09. As such, adoption of this guidance did not have any impact on the Company s consolidated financial statements.

Not yet adopted

Revenue recognition

In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers (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*, and creates a new Topic 606, *Revenue from Contracts with Customers* (Topic 606). Topic 606 is effective for annual reporting periods beginning after December 15, 2017, with early adoption permitted. Per Topic 606, two adoption methods are 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 has not yet determined which adoption method will be utilized or the effect that adoption of Topic 606 may have on the Company s consolidated financial statements. However, management has determined that income associated with the Company s National Institutes of Health (NIH) grant does not meet the definition of revenue under Topic 606 and that, while there will be no cumulative effect on initial adoption of Topic 606 related to the Company s grants, grant income will no longer be presented as revenue in the Company s consolidated statement of operations.

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 will be effective for public business entities in fiscal years beginning after December 15, 2017, including interim periods within those years. Management is currently evaluating the potential impact that this guidance may have on the Company s consolidated financial statements.

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 beginning in the first quarter of 2019, with early adoption permitted. 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 is currently evaluating the impact of adopting ASU 2016-02 on the Company s consolidated financial statements.

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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. ASU 2016-15 is effective for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Management is currently evaluating the potential impact that this guidance may have on the Company s 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, cash equivalents and amounts generally described as restricted cash or restricted cash equivalents. Entities will also be required to reconcile such total to amounts on the balance sheet and disclose the nature of the restrictions. ASU 2016-18 is effective for financial statements issued for fiscal years beginning after December 15, 2017, and interim periods within those fiscal years. Management is currently evaluating the potential impact that this guidance may have on the Company s consolidated financial statements.

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. Per 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. ASU 2017-19 is effective prospectively for annual periods beginning on or after December 15, 2017, with early adoption permitted. Management is currently evaluating the potential impact that this guidance may have on the Company s consolidated financial statements.

2. Net Loss per Share Attributable to Common Stockholders

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

	June 30, 2017	June 30, 2016
Options to purchase common stock	6,212,437	4,888,522
Warrants to purchase common stock	87,901	87,901
Unvested restricted common stock	10,000	25,859
Redeemable convertible preferred stock	718,404	

3. Held-to-maturity investments

The following tables provide information relating to the Company s held-to-maturity investments:

As of June 30, 2017:	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Held-to-maturity investments				
U.S. treasury securities maturing in one year or less	$ 49,953	$	$ (22)	$ 49,931

As of December 31, 2016:	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Held-to-maturity investments				
U.S. treasury securities maturing in one year or less	$ 25,009	$	$ (5)	$ 25,004

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4. Redeemable Convertible Preferred Stock

On April 11, 2017, pursuant to a redeemable convertible preferred stock purchase agreement (SPA) with seven institutional investors (Investors), led by funds advised by Bain Capital Life Sciences L.P. (Lead Investor), the Company issued and sold in a private placement 700,000 shares of its newly designated Redeemable Convertible Preferred Stock, par value $0.0001 per share (Redeemable Convertible Preferred), at a purchase price of $100.00 per share, for total gross proceeds of $70.0 million (Private Placement), less issuance costs of $0.8 million. The shares of Redeemable Convertible Preferred and the shares of common stock issuable upon conversion of the Redeemable Convertible Preferred were offered and sold by the Company pursuant to an exemption from the registration requirements of the Securities Act provided by Section 4(a)(2) thereunder.

In addition to the Lead Investor, other participants in the Private Placement included Cormorant Asset Management, Domain Associates, EcoR1 Capital, RA Capital and Skyline Ventures, among others. Domain Associates, RA Capital and Skyline Ventures are entities that are affiliated or were formerly affiliated with certain members of the Company s board of directors.

The Redeemable Convertible Preferred has the rights and preferences set forth in a Certificate of Designation, which was filed with the Secretary of State of the State of Delaware. Those rights and preferences are summarized below.

Conversion

The Company has the right to require the Investors to convert the Redeemable Convertible Preferred into common stock at any time following the earlier of the second anniversary of the closing of the Private Placement or the occurrence of certain agreed-upon milestone events, provided, that, in each case, the trading price of the Company s common stock exceeds 200% of $3.19 (the Conversion Price) for 45 out of 60 consecutive trading days. The Company s ability to require conversion shall be subject to (i) a 19.99% blocker provision to comply with NASDAQ Listing Rules (19.99% Conversion Blocker), (ii) for certain Investors, a 9.99% blocker provision (9.99% Conversion Blocker) that will prohibit beneficial ownership of more than 9.99% of the outstanding shares of the Company s common stock or voting power at any time, or (iii) applicable regulatory restrictions. The 19.99% Conversion Blocker and the 9.99% Conversion Blocker are hereinafter referred to as the Conversion Blockers. The Conversion Price is subject to proportionate adjustment for any stock split, stock dividend, combination or other similar recapitalization event.

At any time and from time to time at their election, the holders of Redeemable Convertible Preferred will have the option to convert the Redeemable Convertible Preferred into shares of the Company s common stock by dividing (i) the sum of the Accrued Value plus an amount equal to all accrued or declared and unpaid dividends on the Redeemable Convertible Preferred that have not previously been added to the Accrued Value by (ii) the Conversion Price in effect at the time of such conversion. The conversion of shares of Redeemable Convertible Preferred into shares of common stock is subject to the Conversion Blockers. Accrued Value means, with respect to each share of Redeemable Convertible Preferred, the sum of (i) $100.00 plus (ii) on each quarterly dividend date, an additional amount equal to the dollar value of any dividends on a share of Redeemable Convertible Preferred which have accrued on any dividend payment date and have not previously been added to such Accrued Value.

Redemption

On or at any time following the seventh anniversary of the closing of the Private Placement, (i) the Company shall have the right to redeem the Redeemable Convertible Preferred for a cash consideration equal to the sum of the Accrued Value, as of the date of redemption, plus an amount equal to all accrued or declared and unpaid dividends on

the Redeemable Convertible Preferred that have not previously been added to the Accrued Value, and (ii) the holders of a majority of the Redeemable Convertible Preferred shall also have the right to cause the Company to redeem the Redeemable Convertible Preferred at the same price.

Upon consummation of a specified change of control transaction, each holder of Redeemable Convertible Preferred will be entitled to receive in preference to the holders of common stock and any junior preferred stock, an amount equal to the greater of (i) 101% of the sum of the Accrued Value plus an amount equal to all accrued or declared and unpaid dividends on the Redeemable Convertible Preferred that have not previously been added to the Accrued Value, or (ii) the amount that such shares would have been entitled to receive if they had converted into common stock immediately prior to such event.

Liquidation preferences

In the event of the Company s liquidation, dissolution or winding up, the holder of each share of Redeemable Convertible Preferred will be entitled to receive, in preference to the holders of the common stock and any junior preferred stock, an amount per share equal to the greater of (i) the sum of the Accrued Value plus an amount equal to all accrued or declared and unpaid dividends on the

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Redeemable Convertible Preferred that have not previously been added to the Accrued Value, or (ii) the amount that such shares would have been entitled to receive if they had converted into common stock immediately prior to such liquidation, dissolution or winding up.

Voting and other rights

Except as set forth above or as otherwise required by law, holders of shares of Redeemable Convertible Preferred are entitled to vote together with shares of common stock (based on one vote per share of common stock into which the shares of Redeemable Convertible Preferred are convertible on the applicable record date) on any matter on which the holders of common stock are entitled to vote.

Additionally, for so long as any shares of Redeemable Convertible Preferred remain outstanding, without the approval of holders of a majority of the Redeemable Convertible Preferred, the Company may not, among other things, (i) amend, modify or fail to give effect to any right of holders of the Redeemable Convertible Preferred, (ii) change the authorized number of Redeemable Convertible Preferred or issue additional Redeemable Convertible Preferred or create a new class or series of equity securities or securities convertible into equity securities with equal or superior rights, preferences or privileges to those of the Redeemable Convertible Preferred in terms of liquidation preference, dividend rights or certain governance rights, (iii) issue shares of common stock or securities convertible into common stock while the Company has insufficient shares to effect the conversion of the Redeemable Convertible Preferred into common stock, (iv) declare or pay dividends or redeem or repurchase any capital stock (other than certain repurchases from employees, directors, advisors or consultants upon termination of service) or (v) incur certain indebtedness in excess of $10 million.

On March 28, 2017, in accordance with the terms of the SPA, the Company increased the size of its board of directors from eight to nine directors and approved the appointment of Adam M. Koppel, M.D., Ph.D., a managing director of the Lead Investor, as a director of the Company, effective as of the closing of the Private Placement on April 11, 2017. To the extent that such director is not re-elected at any time and, so long as the Lead Investor owns at least 25% of the Redeemable Convertible Preferred (or underlying common stock) owned by it at the closing of the Private Placement, the Lead Investor shall have the right to designate a board observer.

The Company also entered into an amended and restated registration rights agreement, by and among the Company and the Investors (Registration Rights Agreement). Pursuant to the Registration Rights Agreement, the Investors will be entitled to certain demand, shelf and piggyback registration rights with respect to the shares of common stock issuable upon conversion of the Redeemable Convertible Preferred, subject to the limitations set forth in the Registration Rights Agreement.

Dividends

Each holder of Redeemable Convertible Preferred is entitled to receive cumulative dividends on the Accrued Value of each share of Redeemable Convertible Preferred at an initial rate of 12% per annum, compounded quarterly and subject to two rate reductions, of 4% each, upon the occurrence of certain agreed-upon milestone events. Dividends on the Redeemable Convertible Preferred are payable in kind and will accrue on the Accrued Value of each share of Redeemable Convertible Preferred until the earlier of conversion, redemption, consummation of a change of control, a liquidation event, or upon failure to mandatorily convert due to the Conversion Blockers or applicable regulatory restrictions.

For accounting purposes, in accordance with the FASB s Accounting Standard Codification (ASC) Topic 480-10-S99, *Distinguishing Liabilities from Equity SEC Materials* (ASC 480-10-S99), the Company records the additional shares

issued as dividends at fair value at each declaration date. The fair value of the dividends is determined using a binary lattice model that captures the intrinsic value of the underlying common stock on the declaration date and the option value of the shares and future dividends. On June 30, 2017, the Company issued an aggregate of 18,404 additional shares of Redeemable Convertible Preferred as payment in kind of cumulative dividends. These dividends were charged against additional paid-in capital and increased the carrying value of the Redeemable Convertible Preferred as of June 30, 2017.

The lattice model used to determine fair value used an adjusted risk rate of 18.0%, a 6.75-year volatility of 70.0%, the underlying common stock price on the dividend date and other assumptions, including probability simulations of various outcomes largely associated with the conversion-related milestone events referred to below and with the progression of the Company s per common share price. Use of the lattice model resulted in a fair value estimate of the dividends declared during the three months ended June 30, 2017 of approximately $1.87 million, or approximately $0.03 million above the increase in the liquidation preference amounts of the Redeemable Convertible Preferred.

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Classification and measurement

At the date of issuance, the Redeemable Convertible Preferred was classified as temporary equity in the mezzanine section of the Company s consolidated balance sheet, since the underlying preferred shares are subject to redemption upon the occurrence of uncertain events not solely within the Company s control, pursuant to ASC 480-10-S99. As of June 30, 2017, the Redeemable Convertible Preferred was not currently redeemable, and management concluded that it is not probable that the Redeemable Convertible Preferred will become redeemable, primarily due to the existence of the conversion right held by the Company, as discussed above.

In accordance with ASC Topic 470-20, *Debt with Conversion and Other Options*, the Company recorded a beneficial conversion feature (BCF) related to the issuance of the Redeemable Convertible Preferred. The BCF was recognized separately at issuance by allocating a portion of the proceeds equal to the intrinsic value of that feature to additional paid-in capital. The BCF was calculated at the commitment date, which management has determined to be the date of issuance. Intrinsic value is the difference between the conversion price and the fair value of the Company s common stock into which the Redeemable Convertible Preferred is convertible, multiplied by the number of shares into which the issued shares of Redeemable Convertible Preferred are convertible. The Company recorded a deemed dividend charge of $6.1 million to reflect full and immediate accretion of the discount resulting from the BCF embedded within the Redeemable Convertible Preferred as a result of the shares being immediately convertible into shares of the Company s common stock at the option of the Investors. Additionally, during the three months ended June 30, 2017, and as noted above, the Company recorded Redeemable Convertible Preferred dividends of $2.6 million. Accretion of the discount resulting from the BCF and cumulative dividends, including accretion of share issuance costs, were non-cash transactions and have been reflected below net loss to arrive at net loss attributable to common stockholders.

The following table reflects the changes in Redeemable Convertible Preferred.

Balance at January 1, 2017	$
Issuance of Redeemable Convertible Preferred	70,000
Share issuance costs	(750)
Net proceeds	69,250
Discount resulting from the BCF at issuance	(6,144)
Accretion of the discount resulting from the BCF (deemed dividend)	6,144
Dividends accrued at the stated rate	1,841
Accretion of share issuance costs (additional dividends)	750
Liquidation preference	71,841
Fair value in excess of dividends accrued at the stated rate	31
Balance at June 30, 2017	$ 71,872

No shares of Redeemable Convertible Preferred were converted since the original issuance thereof through June 30, 2017. As of June 30, 2017, 42,774,585 shares of common stock were issuable assuming full conversion of all outstanding shares of the Redeemable Convertible Preferred, representing approximately 71% ownership of the Company by the Investors on an as-converted basis and after application of the Conversion Blockers.

5. Stock Option Plan and Stock-Based Compensation

During the three and six month periods ended June 30, 2017, the Company granted stock options to purchase 352,500 and 1,330,997 shares of common stock to employees with aggregate grant date fair values of $0.7 million and $2.7 million, respectively, compared to stock options to purchase 517,500 and 1,445,275 shares of common stock granted to employees with aggregate grant date fair values of $1.6 million and $6.8 million, for the comparable three and six month periods in 2016.

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, 2017		Six Months Ended June 30, 2017	
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%	

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	Three Months Ended June 30, 2016		Six Months Ended June 30, 2016	
Stock price	$3.26	$5.55	$3.26	$9.09
Expected option term (in years)	5.50	6.25	5.50	6.25
Expected volatility	75.1%	75.3%	70.9%	75.3%
Risk-free interest rate	1.20%	1.46%	1.20%	1.71%
Expected dividend yield	0.00%		0.00%	

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

	Three Months Ended June 30, 2017	Six Months Ended June 30, 2017	Three Months Ended June 30, 2016	Six Months Ended June 30, 2016
Research and development expenses	$ 972	$ 1,917	$ 1,126	$ 2,301
General and administrative expenses	1,053	2,116	1,284	2,494
Total	$ 2,025	$ 4,033	$ 2,410	$ 4,795

5. Fair Value Measurements

A summary of the Company s assets that are measured or disclosed at fair value as of June 30, 2017 and December 31, 2016 are presented below:

Description	At June 30, 2017	Level 1	Level 2	Level 3
Cash equivalents				
Money market fund	$ 26,040	$ 26,040	$	$
Held-to-maturity investments				
U.S. treasury securities	49,931		49,931	
Restricted cash equivalents				
Money market fund	1,116		1,116	
Total	$ 77,087	$ 26,040	$ 51,047	$

Description	At December 31, 2016	Level 1	Level 2	Level 3
Cash equivalents				
Money market fund	$ 12,853	$ 12,853	$	$
Held-to-maturity investments				
U.S. treasury securities	25,004		25,004	
Restricted cash equivalents				

Money market fund	1,116		1,116	
Total	$ 38,973	$ 12,853	$ 26,120	$

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, 2017 and December 31, 2016.

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, 2017 and December 31, 2016.

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, 2017 and December 31, 2016.

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As of June 30, 2017 and December 31, 2016, the carrying amounts of accounts payable and accrued expenses approximated their estimated fair values because of the short-term nature of these financial instruments.

For the three and six month periods ended June 30, 2017 and 2016 there were no transfers between Level 1 and Level 2.

6. 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:

12-Month Periods Ending June 30,	Operating Lease
2018	$ 1,606
2019	1,654
2020	1,703
2021*	718
Total	$ 5,681

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

Litigation

On June 10, 2015, Alnylam Pharmaceuticals, Inc. (Alnylam) filed a complaint against the Company in the Superior Court of Middlesex County, Massachusetts (the Court). The complaint alleges 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. The complaint seeks among other things, unspecified damages, attorneys fees, and an order permanently enjoining the Company from disclosing or using any of Alnylam s confidential information or trade secrets. The Court has set a trial date of April 23, 2018.

The Company believes that these allegations lack merit, has filed an answer denying all liability and intends to continue to vigorously defend all claims asserted. At this time, the Company has not recorded a liability in connection with these matters because management believes that any potential loss is neither probable nor reasonably estimable.

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 litigation liabilities recorded as of June 30, 2017 or December 31, 2016.

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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

Founded in 2006 as a Delaware corporation, we are a biopharmaceutical company focused on the discovery and development of innovative subcutaneously delivered ribonucleic acid RNAi-based pharmaceuticals using our GalXC™ RNAi platform for the treatment of diseases involving the liver, including rare diseases, chronic liver diseases, cardiovascular diseases and viral infectious diseases. Within these therapeutic areas, we believe our GalXC RNAi platform will allow us to build a broad pipeline 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 RNA (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. These proprietary molecules are generally referred to as Dicer Substrate short-interfering RNAs (DsiRNAs). Our GalXC RNAi platform utilizes a particular Dicer Substrate structure 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 high probabilities of success, with easily identifiable patient populations and 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.

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 four therapeutic programs: DCR-PHXC for the treatment of primary hyperoxaluria (PH); a program against an undisclosed rare disease; DCR-HBVS for the treatment of chronic hepatitis B virus (HBV) infection; and DCR-PCSK9 for the treatment of hypercholesterolemia. The Company s goal is to advance five programs into the clinic by the end of 2019. We plan to file a Clinical Trial Application (CTA) for our lead GalXC product candidate, DCR-PHXC, at the end of 2017, followed by additional IND applications in 2018 and 2019.

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The table below sets forth the state of development of our various product candidates as of August 9, 2017.

Our current 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. In preclinical models of PH, DCR-PHXC reduces oxalate production to near-normal levels, ameliorating the disease condition. DCR-PHXC is in preclinical development and has advanced into IND application-enabling studies. We plan to file a CTA for DCR-PHXC in late 2017 and commence human clinical trials in the first quarter of 2018.

On July 15, 2017, in a series of presentations at the 12th International Workshop on Primary Hyperoxaluria for Professionals, Patients and Families in Tenerife, Spain (12th International Workshop), we presented new preclinical data suggesting the potential utility of DCR-PHXC for treating all forms of PH. In particular, we presented research from animal models demonstrating how DCR-PHXC inhibits the lactate dehydrogenase A (*LDHA*) gene, which we have identified as potentially being an optimal therapeutic target in patients with PH. *LDHA* inhibition was shown in animal models to reduce oxalate to normal or near-normal levels in PH types 1, 2 and ethylene glycol-induced hyperoxaluria (a model for idiopathic PH).

LDHA reduction has a near-linear correlation with oxalate reduction and offers a minimal metabolic intervention. These benefits of *LDHA* inhibition may translate into consistent therapeutic activity even in the event of a missed dose. There are numerous case reports of *LDHA* deficiency naturally occurring in humans, with no reported adverse effects due to deficiency in the liver.

To facilitate DCR-PHXC development, we continue to advance our Primary HYperoxaluria Observational Study (PHYOS), an international, multicenter, observational study in patients with a genetically confirmed diagnosis of PH, type 1 (PH1). PHYOS is collecting data on key biochemical parameters implicated in the pathogenesis of PH1. We hope to use the data to better understand the baseline PH1 disease state, which will help guide long-term drug development plans. At the 12th International Workshop, we also reported data from 20 enrolled patients with a median age at screening of 21 years (range 12-61 years). The patients had been diagnosed at a median age of 7 years (range 1-59 years), and 14 patients (74%) had a medical history of renal stones. Over the six-month observation period, the variability (coefficient of variation) between 24-hour urine measurements of oxalate at different time points was 28%. These data will help our clinical team design future clinical studies using 24-hour urinary oxalate excretion as a surrogate marker for clinical benefit.

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. We plan to file an

IND application and/or CTA for this program in the second quarter of 2018.

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Chronic Hepatitis B Virus infection: We have recently initiated formal IND application-enabling work on DCR-HBVS, which targets HBV directly. We are using our GalXC RNAi platform to investigate potential pharmaceutical treatments for HBV. 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. Based on preclinical studies, we are evaluating whether our GalXC RNAi platform can produce an experimental HBV-targeted therapy that profoundly reduces HBsAg expression in HBV patients and that has the potential to be delivered in a commercially attractive subcutaneous dosing paradigm. We expect to file an IND application or a CTA at approximately the end of 2018.

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. Based on the Company s candidate development work during the fourth quarter of 2016, Dicerna is positioned to advance DCR-PCSK9, which targets the PCSK9 gene and will be evaluated for the treatment of statin-refractory patients with hypercholesterolemia, into formal preclinical development. 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.

In addition to our GalXC development programs, we have partnered an early generation of Dicer Substrate RNAi technology, non-GalXC technology, against two targets, the KRAS oncogene and an additional undisclosed gene, with the global pharmaceutical company, KHK, to use for development in oncology and formulated using KHK s proprietary drug delivery system. KHK is responsible for global development of the KRAS program, including all development expenses. For the KRAS product candidate, we retain an option to co-promote in the U.S. for an equal share of the profits from U.S. net sales. We are also developing, with KHK, a therapeutic candidate targeting a second cancer-related gene, which we are not identifying at this time. For each product candidate in our collaboration with KHK, we have the potential to receive clinical, regulatory and commercialization milestone payments of up to $110.0 million and royalties on net sales of each such product candidate. KHK is responsible for all preclinical and clinical development activities, including the selection of patient population and disease indications for clinical trials. According to information received from KHK, both product candidates are in preclinical development.

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

Redeemable Convertible Preferred Stock

On April 11, 2017, we issued and sold 700,000 shares of our newly designated Redeemable Convertible Preferred to the Investors in a Private Placement for aggregate gross proceeds of $70.0 million, less issuance costs of $0.8 million. In addition to the Lead Investor, other participants in the Private Placement included Cormorant Asset Management, Domain Associates, EcoR1 Capital, RA Capital and Skyline Ventures, among others. Domain Associates, RA Capital and Skyline Ventures are entities that are affiliated or were formerly affiliated with certain members of our board of directors.

We have the right to require the Investors to convert the Redeemable Convertible Preferred into common stock (Mandatory Conversion) at any time following the earlier of (i) the second anniversary of the closing of the Private

Placement or (ii) the occurrence of both of the following: (a) (1) the date that we first administer, after the issue date, a dose of a pharmaceutical product candidate (which such product candidate shall be one of the following candidates, or a variation thereof: DCR-PHXC, DCR-PCSK9 or the undisclosed rare disease program currently in pre-clinical development (each, a Product Candidate)) to a human being pursuant to an IND application filed by us with the FDA; or (2) after we have first administered, after the issue date, a dose of a Product Candidate to a human being pursuant to a clinical trial authorization with the Medicine and Healthcare Products Regulatory Agency in the EU and an IND application relating to such Product Candidate has become effective; and (b) the date we enter into a partnership or license agreement with a major company in the pharmaceutical or biotechnology industry relating to a non-Product Candidate, pursuant to which such company provides us with an up-front cash payment of a minimum amount agreed upon by us and the Lead Investor and agrees to customary future milestone and royalty payments, provided, that, in each case ((i) and (ii)), the trading price of our common stock exceeds 200% of the Conversion Price, as defined below, for 45 out of 60 consecutive trading days. Our ability to require conversion shall be subject to the Conversion Blockers and applicable regulatory restrictions. Conversion Price shall mean an initial price of $3.19 per share, subject to proportionate adjustment for any stock split, stock dividend, combination or other similar recapitalization event.

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Following the date of a Mandatory Conversion, any shares of Redeemable Convertible Preferred that are not converted as a result of the Conversion Blockers or applicable regulatory restrictions shall continue to be entitled to all of the rights of the holders of Redeemable Convertible Preferred except that they will no longer be entitled to further accrual of dividends, priority distribution of assets upon consummation of a change of control or a liquidation event and certain special voting provisions.

On or at any time following the seventh anniversary of the closing of the Private Placement, (i) we shall also have the right to redeem the Redeemable Convertible Preferred for a cash consideration equal to the sum of the Accrued Value, as of the date of redemption, plus an amount equal to all accrued or declared and unpaid dividends on the Redeemable Convertible Preferred that have not previously been added to the Accrued Value, and (ii) the holders of a majority of the Redeemable Convertible Preferred shall also have the right to cause us to redeem the Redeemable Convertible Preferred at the same price. Accrued Value means, with respect to each share of Redeemable Convertible Preferred, the sum of (i) $100.00 plus (ii) on each quarterly dividend date, an additional amount equal to the dollar value of any dividends on a share of Redeemable Convertible Preferred which have accrued on any dividend payment date and have not previously been added to such Accrued Value.

At any time and from time to time at their election, the holders of Redeemable Convertible Preferred will have the option to convert the Redeemable Convertible Preferred into shares of our common stock by dividing (i) the sum of the Accrued Value plus an amount equal to all accrued or declared and unpaid dividends on the Redeemable Convertible Preferred that have not previously been added to the Accrued Value by (ii) the Conversion Price in effect at the time of such conversion. The conversion of shares of Redeemable Convertible Preferred into shares of common stock is subject to the Conversion Blockers.

In the event of our liquidation, dissolution or winding up, the holder of each share of Redeemable Convertible Preferred will be entitled to receive, in preference to the holders of the common stock and any junior preferred stock, an amount per share equal to the greater of (i) the sum of the Accrued Value plus an amount equal to all accrued or declared and unpaid dividends on the Redeemable Convertible Preferred that have not previously been added to the Accrued Value, or (ii) the amount that such shares would have been entitled to receive if they had converted into common stock immediately prior to such liquidation, dissolution or winding up.

Upon consummation of a specified change of control transaction, each holder of Redeemable Convertible Preferred will be entitled to receive in preference to the holders of common stock and any junior preferred stock, an amount equal to the greater of (i) 101% of the sum of the Accrued Value plus an amount equal to all accrued or declared and unpaid dividends on the Redeemable Convertible Preferred that have not previously been added to the Accrued Value, or (ii) the amount that such shares would have been entitled to receive if they had converted into common stock immediately prior to such event.

In addition, for so long as any shares of Redeemable Convertible Preferred remain outstanding, without the approval of holders of a majority of the Redeemable Convertible Preferred, we may not, among other things, (i) amend, modify or fail to give effect to any right of holders of the Redeemable Convertible Preferred, (ii) change the authorized number of Redeemable Convertible Preferred or issue additional Redeemable Convertible Preferred or create a new class or series of equity securities or securities convertible into equity securities with equal or superior rights, preferences or privileges to those of the Redeemable Convertible Preferred in terms of liquidation preference, dividend rights or certain governance rights, (iii) issue shares of common stock or securities convertible into common stock while we have insufficient shares to effect the conversion of the Redeemable Convertible Preferred into common stock, (iv) declare or pay dividends or redeem or repurchase any capital stock (other than certain repurchases from employees, directors, advisors or consultants upon termination of service) or (v) incur certain indebtedness in excess of $10 million. Except as set forth above or as otherwise required by law, holders of shares of Redeemable

Convertible Preferred are entitled to vote together with shares of common stock (based on one vote per share of common stock into which the shares of Redeemable Convertible Preferred are convertible on the applicable record date) on any matter on which the holders of common stock are entitled to vote.

Per the Certificate of Designation, each holder of Redeemable Convertible Preferred is entitled to receive cumulative dividends on the Accrued Value of each share of Redeemable Convertible Preferred at an initial rate of 12% per annum, compounded quarterly and subject to two rate reductions, of 4% each, upon the occurrence of certain agreed-upon milestone events. Dividends on the Redeemable Convertible Preferred are payable in kind and will accrue on the Accrued Value of each share of Redeemable Convertible Preferred until the earlier of conversion, redemption, consummation of a change of control, a liquidation event, or upon failure to mandatorily convert due to the Conversion Blockers or applicable regulatory restrictions.

In accordance with the terms of the SPA, on March 28, 2017, our board of directors voted to increase the size of the board from eight directors to nine directors and approved the appointment of Adam M. Koppel, M.D., Ph.D., a managing director of the Lead Investor, as a director of our Company, effective as of the closing of the Private Placement on April 11, 2017, to fill the resulting vacancy. To the extent such director is not re-elected at any time and, so long as the Lead Investor owns at least 25% of the Redeemable Convertible Preferred (or underlying common stock) owned by it at the closing of the Private Placement, it shall have the right to designate a board observer. On June 30, 2017, the Lead Investor, which appointed one of its managing directors to our board of directors, owned approximately 19% of the Company on an as-converted basis and after application of the Conversion Blockers.

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We also entered into a Registration Rights Agreement, by and among us and the Investors. Pursuant to the Registration Rights Agreement, the Investors will be entitled to certain demand, shelf and piggyback registration rights with respect to the shares of common stock issuable upon conversion of the Redeemable Convertible Preferred, subject to the limitations set forth in the Registration Rights Agreement.

The shares of Redeemable Convertible Preferred and the shares of common stock issuable upon conversion of the Redeemable Convertible Preferred were offered and sold by us pursuant to an exemption from the registration requirements of the Securities Act provided by Section 4(a)(2) thereunder.

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 consolidated financial statements, which have been prepared in accordance with GAAP. The preparation of our consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the revenue and expenses incurred during the reported periods. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and stock-based compensation and in relation to the accounting for the Redeemable Convertible Preferred, including cumulative dividends thereon. 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 30, 2017, as amended. There have been no changes to our critical accounting policies during the three or six month periods ended June 30, 2017 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 30, 2017, as amended, except as related to management s judgment associated with the accounting for, including the valuation of dividends on, the Redeemable Convertible Preferred and 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 2017

Stock-based compensation

In March 2016, the accounting guidance related to various aspects of share-based payment transactions was amended, including income tax consequences, classification of awards as either equity or liabilities and classification on the statement of cash flows. Under the new guidance, excess tax benefits and deficiencies are to be recognized as income tax expense or benefit in the income statement as discrete items in the reporting period in which they occur instead of

an increase or decrease to stockholders equity. With regard to forfeitures, an entity may make an accounting policy election either to estimate the number of awards that are expected to vest or account for forfeitures when they occur. We adopted this new guidance on January 1, 2017, and as a result, we will track stock option deductions in our net operating loss deferred tax asset on a modified retrospective basis. In addition, our policy has been to estimate forfeitures as of the grant date. We will continue to maintain our policy to estimate forfeiture as of the grant date in the future. Since we historically have maintained a full valuation allowance on our net deferred tax asset, there is no net impact to our accumulated deficit or on our net loss per share attributable to common stockholders from the adoption of this new guidance. As such, adoption of this guidance did not have any impact on our consolidated financial statements.

Not yet adopted

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 will become effective for us on January 1, 2018.

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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. Entities have the option of using either a full retrospective or a modified retrospective approach to adopt this new guidance.

Our evaluation of the impact that the adoption of this guidance will have on our consolidated financial statements will continue throughout 2017, and while we have not yet determined which adoption method will be utilized or the effect that adoption of this new guidance may have on our consolidated financial statements, we have ascertained that we will apply the new standard only to contracts that are not completed as of January 1, 2018, as allowed by the standard s transitional guidance. We will evaluate the impact that the new guidance may have on current arrangements with customers, including our collaboration with KHK. As for grant income that currently is recognized as revenue in our consolidated financial statements, we have determined that this item does not meet the definition of revenue under Topic 606 and that, while there will be no cumulative effect on initial adoption of Topic 606 related to our grants, grant income will no longer be presented as revenue in our consolidated statement of operations. Instead, we expect to classify grant-related income as a direct reduction to the research and development expenses to which the grants relate.

Income taxes

New guidance issued in October 2016 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. This new guidance will be effective for us beginning on January 1, 2018, and we are currently evaluating the potential impact that this guidance may have on our consolidated financial statements. We have not recorded any deferred tax assets or liabilities on our consolidated balance sheet.

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 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, 2017, and as presented below, our total future minimum lease obligation associated with this lease was $5.7 million, and a substantial portion of this commitment will remain outstanding at the time that we adopt the new guidance. Our evaluation of this guidance and its full impact on our consolidated financial statements will continue throughout 2017.

Statement of cash flows

In August 2016, 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 will become effective for us on January 1, 2018, with early adoption permitted. Additionally, in November 2016, new accounting guidance was issued related to the statement of cash flows implications related to restricted cash and cash equivalents. This new guidance is effective for us beginning on January 1, 2018, and we will continue to evaluate the impact that the guidance may have on our consolidated financial statements, particularly as pertaining to our restricted cash equivalents.

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, with early adoption permitted. We are currently evaluating the potential impact that this guidance may have on our consolidated financial statements.

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Financial Operations Overview

Comparison of the Three and Six Month Periods Ended June 30, 2017 and 2016

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

	Three Months Ended June 30, 2017	Three Months Ended June 30, 2016	Increase/ (Decrease)		Six Months Ended June 30, 2017	Six Months Ended June 30, 2016	Increase/ (Decrease)	
Revenue	$ 252	$	$ 252		$ 385	$	$ 385	
Expenses:								
Research and development	9,320	11,032	(1,712)	(15.5%)	18,196	22,296	(4,100)	(18.4%)
General and administrative	6,300	4,656	1,644	35.3%	11,796	9,140	2,656	29.1%
Total operating expenses	15,620	15,688	(68)	(0.4%)	29,992	31,436	(1,444)	(5.8%)
Loss from operations	(15,368)	(15,688)	(320)	(2.0%)	(29,607)	(31,436)	(1,829)	(9.6%)
Interest income	143	66	77	116.7%	181	121	60	49.6%
Net loss	(15,225)	(15,622)	(397)	(2.5%)	(29,426)	(31,315)	(1,889)	(6.0%)
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	$ (23,991)	$ (15,622)	$ 8,369	53.6%	$ (38,192)	$ (31,315)	$ 6,877	22.0%

Revenue

Revenue recognized during the three and six month periods ended June 30, 2017 relates to a $2.0 million NIH grant, awarded in August 2016, related to cancer treatment research. Of the total $2.0 million grant, which covers the period from September 1, 2016 to February 28, 2018, $1.0 million is committed funding, and notice of the additional

$1.0 million funding commitment is expected in the third quarter of 2017, subject to NIH approval and availability of funds. Grant revenue represents reimbursable subcontractor and internal costs incurred that are specifically covered by the grant, and where applicable, funding for qualifying facilities and administrative expenses.

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 (in thousands):

	Three Months Ended June 30,		
	2017	2016	Increase/ (Decrease)
Direct research and development expenses	$ 4,141	$ 3,708	$ 433
Platform-related expenses	1,707	3,195	(1,488)
Employee-related expenses	2,680	3,330	(650)
Facilities, depreciation and other expenses	792	799	(7)
Total	$ 9,320	$ 11,032	$ (1,712)

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	Six Months Ended June 30,		
	2017	2016	Increase/ (Decrease)
Direct research and development expenses	$ 7,480	$ 7,223	$ 257
Platform-related expenses	3,541	6,003	(2,462)
Employee-related expenses	5,566	7,164	(1,598)
Facilities, depreciation and other expenses	1,609	1,906	(297)
Total	$ 18,196	$ 22,296	$ (4,100)

The increase in direct research and development expenses in both the three and six month periods ended June 30, 2017 is attributable to an overall increase in manufacturing activities and in toxicology study costs related to our new candidates under our GalXC platform, partially offset by a reduction in clinical and manufacturing activities related to our now discontinued PH1 and MYC programs, both of which will be fully wound down before the end of the current year. Platform-related expenses decreased substantially in both the three and six month periods ended June 30, 2017 due to the timing of activities related to discovery and early development programs, including supply and external study costs. Employee-related expenses decreased in both the three and six month periods ended June 30, 2017 due to an overall decrease in headcount from the prior year, along with a decrease in non-cash stock-based compensation costs.

We expect our overall research and development expenses to increase during the second half of 2017, primarily as we complete clinical manufacturing activities, advance pre-clinical toxicology studies and initiate clinical activities associated with our lead product candidates.

General and administrative expenses

General and administrative expenses were $6.3 million and $11.8 million for the three and six months ended June 30, 2017, as compared to $4.7 million and $9.1 million for the three and six months ended June 30, 2016, respectively. The increases are predominantly related to higher costs associated with the litigation with Alnylam as well as to higher salaries, benefits and other employee-related expenses.

Interest income

Interest income is comprised primarily of interest earned from our money market accounts and held-to-maturity investments. Interest income was $0.14 million and $0.18 million for the three and six months ended June 30, 2017, respectively, as compared to $0.07 million and $0.12 million for the three and six months ended June 30, 2016, respectively. The increases were primarily due to higher invested amounts in the 2017 periods as a result of the net proceeds from the Private Placement, which closed in the second quarter of 2017.

Dividends

Dividends of $2.6 million recorded during the three and six months ended June 30, 2017 represent the non-cash fair value of additional shares of Redeemable Convertible Preferred issued to the Investors as dividends paid in kind, as well as full accretion of share issuance costs. The fair value of the issued shares was determined using a binary lattice model that captures the intrinsic value of the underlying common stock on the declaration date and the option value of the shares and future dividends. Inputs to the lattice model include an adjusted risk rate, our common stock volatility, the underlying common stock price on the dividend date and management s judgment associated with probability

simulations of various outcomes. Dividends are valued at each dividend declaration date, based on various inputs and assumptions at that time, and, as such, quarterly dividend amounts may vary significantly from quarter to quarter. No common stock dividends were recorded during the three or six month periods ended June 30, 2017.

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Deemed dividends of $6.1 million (non-cash) represent the value of the BCF, which was accreted in full at issuance due to the fact that the underlying shares of Redeemable Convertible Preferred are immediately convertible. Management is required to evaluate whether a BCF exists at each Redeemable Convertible Preferred issuance date. Consequently, the intrinsic value of any future shares of Redeemable Convertible Preferred issued as dividends will be recorded as additional deemed dividends in future quarters.

Net loss attributable to common stockholders

Net loss attributable to common stockholders was $24.0 million and $38.2 million for the three and six months ended June 30, 2017, as compared to $15.6 million and $31.3 million for the same periods in 2016, respectively. The overall increases are due to the aforementioned changes in R&D and G&A expenses, as well as to the recording of dividends on the Redeemable Convertible Preferred.

Liquidity and Capital Resources

As of June 30, 2017, we had cash and cash equivalents and held-to-maturity investments of $88.7 million and $1.1 million in cash equivalents held in restriction.

Aggregate gross proceeds received on April 11, 2017 in connection with the closing of the Private Placement totaled $70.0 million, less related transaction costs of approximately $0.8 million.

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 SEC.

Cash flows

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

	Six Months Ended June 30,	
	2017	**2016**
Net cash used in operating activities	$ (26,898)	$ (25,542)
Net cash used in investing activities	(24,966)	(1,795)
Net cash provided by financing activities	69,776	466
Increase (decrease) in cash and cash equivalents	$ 17,912	$ (26,871)

Operating activities

Net cash used in operating activities was $26.9 million for the six months ended June 30, 2017, as compared to $25.5 million for the six months ended June 30, 2016. The increase of $1.4 million was primarily due to higher general and administrative expenses and negative working capital fluctuations, offset by lower overall research and development expenses.

We expect our cash used in operating activities to increase during the second half of 2017, as compared to the six months ended June 30, 2017, predominantly due to the ramping up of pre-clinical and clinical initiatives associated

with our lead product candidates.

Investing activities

Net cash used in investing activities was $25.0 million for the six months ended June 30, 2017, as compared to $1.8 million for the six months ended June 30, 2016. This change was due primarily to $49.9 million of purchases of held-to-maturity investments, partially offset by $25.0 million in maturities of held-to-maturity investments during the six months ended June 30, 2017, as compared to $20.0 million in purchases of held-to-maturity investments, partially offset by $18.5 million of maturities of held-to-maturity investments, during the six months ended June 30, 2016.

Financing activities

Net cash provided by financing activities was $69.8 million for the six months ended June 30, 2017, as compared to $0.5 million for the six months ended June 30, 2016. The increase of $69.3 million was principally due to the receipt of $70.0 million in gross proceeds from the Private Placement, partially offset by lower proceeds received in the current quarter in connection with stock option exercises and with issuances under the employee stock purchase plan.

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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, including the costs to defend the Alnylam claim of misappropriation of confidential information and trade secrets, 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 and liquidity, including the receipt of net proceeds of $69.3 million from the issuance of the Company s Redeemable Convertible Preferred, 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 10, 2017. 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 milestone payments under our collaboration agreement with KHK;
- 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, including the Alnylam complaint of misappropriation of confidential information and trade secrets (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, 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.

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Contractual Obligations and Commitments

The following is a summary of our significant contractual obligations as of June 30, 2017 (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*	$ 5,681	$ 1,606	$ 3,357	$ 718	$

* 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 City of Hope, an independent academic research and medical center (COH), Plant Bioscience Limited (PBL) and the Carnegie Institution of Washington (Carnegie) that become due and payable on the achievement of certain development, regulatory and commercial milestones. We have not included these commitments on our condensed consolidated balance sheet or in the table above, since the achievement and timing of these milestones are not probable or estimable as of June 30, 2017.

See also Part II, Item 1 Legal Proceedings in this Quarterly Report on Form 10-Q for additional information related to litigation. We have not recorded any accrual for contingent liabilities associated with legal proceedings on our condensed consolidated balance sheet as of June 30, 2017.

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.

Segment Reporting

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

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, 2017, we had cash and cash equivalents and held-to-maturity investments of $88.7 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.

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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

We continuously seek to improve the efficiency and effectiveness of our internal controls. This results in refinements to processes throughout the Company. There was no change in our internal control over financial reporting during the quarter ended June 30, 2017, which was identified in connection with our 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 also is 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 filed a complaint against the Company in the Superior Court of Middlesex Country, Massachusetts (the Court). The complaint alleges 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. The complaint seeks among other things, damages, attorneys fees, and an order permanently enjoining the Company from disclosing or using any of Alnylam s confidential information or trade secrets. The Court has set a trial date of April 23, 2018. This matter has caused us to incur significant legal fees and other costs to defend against this action and will continue to do so through the trial and

potentially beyond. We believe, however, that Alnylam s allegations lack merit. In response to the complaint, we filed an answer denying all liability, and we will continue to vigorously defend all claims asserted. We expect that a finding of liability against us is not probable. Accordingly, we cannot reasonably estimate any range of potential future charges, and we have not recorded any accrual for a contingent liability associated with this legal proceeding. However, an unfavorable resolution could potentially have a material adverse effect on our business, financial condition, and results of operations or prospects, potentially delay or limit our ability to use some of our research and development programs, and potentially result in paying monetary damages. Additionally, as we believe Alnylam s suit is without merit and intended only to cause competitive harm, we have filed a countersuit in the case against Alnylam for damages, and on August 8, 2017, we filed a complaint in the Federal District court for the District of Massachusetts asserting a federal antitrust claim against Alnylam.

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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 with other organizations to provide these capabilities for us. 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, 2017, we had $88.7 million in cash and cash equivalents and held-to-maturity investments. Based on our current operating plan and liquidity, including the receipt of net proceeds of $69.3 million in connection with the issuance of the Company s Redeemable Convertible Preferred on April 11, 2017, 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 10, 2017. 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 in general.

If we are unable to obtain funding on a timely basis or on acceptable terms, we may have to delay, reduce or terminate our research and development programs and preclinical studies or clinical trials, if any, limit strategic opportunities or undergo reductions in our workforce or other corporate restructuring activities. We also could be required to seek funds through arrangements with collaborators or others that may require us to relinquish rights to some of our 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, credit and loan facilities and payments received under our collaborations and license agreement with KHK. For example, on April 11, 2017, we issued and sold 700,000 shares of our newly designated Redeemable Convertible Preferred to the Investors in a Private Placement for aggregate gross proceeds of $70.0 million. 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. Additional funds may not be available to us on acceptable terms or at all. If we raise additional funds by issuing equity

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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. Our failure to raise capital or enter into such other arrangements within 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 or undergo reductions in our workforce or other corporate restructuring activities.

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, 2017, we had an accumulated deficit of $285.2 million. For the six months ended June 30, 2017 and for the years ended December 31, 2016, 2015 and 2014, our net loss attributable to common stockholders was $38.2 million, $59.5 million, $62.8 million and $47.9 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.

To date, we have generated revenue primarily from the receipt of upfront research funding, license and option exercise fees and preclinical payments under our research collaboration and license agreement with KHK. 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 KHK;

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, including Alnylam s lawsuit alleging misappropriation of confidential information and trade secrets;

additions and departures of key personnel;

strategic decisions by us or our competitors, such as acquisitions, divestitures, spin-offs, joint ventures, strategic investments or changes in business strategy;

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- 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;
- 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 could 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 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

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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 classified as rare diseases and allow for orphan drug designation by regulatory agencies in major commercial markets, such as the U.S., the EU and Japan. Because of the small patient population for a rare disease, if pricing is not approved or accepted in the market at an appropriate level for an approved product with orphan drug designation, such drug may not generate enough revenue to offset costs of development, manufacturing, marketing and

commercialization despite any benefits received from the orphan drug designation, such as market exclusivity, assistance in clinical trial design or a reduction in user fees or tax credits related to development expense. Market size is also a variable in disease indications not classified as rare. Our estimates regarding potential market size for any indication may be materially different from what we discover to exist 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 or if our product candidate is determined to be contained within the competitor s product candidate for the same indication or disease.

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 ten years of market exclusivity for the approved indication unless another applicant can show that its product is safer, more effective or otherwise clinically superior to the orphan-designated product. 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 KHK. 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 in humans of our product candidates.

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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 or financial constraints. For instance, delays or difficulties in patient enrollment or difficulties in retaining trial participants can result in increased costs, longer development times or termination of a clinical trial. Clinical trials of a new 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 h