MIRAGEN THERAPEUTICS, INC.
Form 10-Q
November 07, 2018
UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2018

or

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

For the transition period from to

Commission File No. 001-36483

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

Delaware	47-1187261
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

6200 Lookout Road, Boulder, CO 80301
(Address, including zip code, of registrant's principal executive offices)

(720) 643-5200
(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 x No o

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

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

Large accelerated filer o	Accelerated filer x
Non-accelerated filer o	Smaller reporting company x
Emerging growth company x	

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. o

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

As of October 31, 2018, there were 30,839,463 shares of the registrant's Common Stock outstanding.

MIRAGEN THERAPEUTICS, INC.
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PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

MIRAGEN THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share data)
(unaudited)

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 31,700	$ 47,441
Short-term investments	38,827	—
Accounts receivable	1,382	1,456
Prepaid expenses and other current assets	3,843	2,971
Total current assets	75,752	51,868
Property and equipment, net	751	563
Other assets	50	50
Total assets	$ 76,553	$ 52,481
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,206	$ 906
Accrued liabilities	4,336	2,991
Current portion of note payable	1,291	—
Total current liabilities	6,833	3,897
Note payable, net of current portion	8,914	9,922
Other liabilities	88	152
Total liabilities	15,835	13,971
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.01 par value; 100,000,000 shares authorized; 30,833,367 and 22,568,006 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	308	226
Additional paid-in capital	176,395	131,877
Accumulated other comprehensive loss	(6)	—
Accumulated deficit	(115,979)	(93,593)
Total stockholders' equity	60,718	38,510
Total liabilities and stockholders' equity	$ 76,553	$ 52,481

See accompanying notes to these condensed consolidated financial statements.

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MIRAGEN THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except share and per share data)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue:				
Collaboration revenue	$814	$ 1,493	$6,938	$ 1,991
Grant revenue	130	138	972	820
Total revenue	944	1,631	7,910	2,811
Operating expenses:				
Research and development	7,399	5,018	22,187	14,625
General and administrative	2,696	2,502	8,354	8,364
Total operating expenses	10,095	7,520	30,541	22,989
Loss from operations	(9,151)	(5,889)	(22,631)	(20,178)
Other income (expense):				
Interest and other income	362	113	890	245
Interest and other expense	(222)	(58)	(645)	(193)
Net loss	(9,011)	(5,834)	(22,386)	(20,126)
Change in unrealized loss on investments	(10)	—	(6)	—
Comprehensive loss	$(9,021)	$ (5,834)	$(22,392)	$ (20,126)
Net loss	$(9,011)	$ (5,834)	$(22,386)	$ (20,126)
Accretion of redeemable convertible preferred stock to redemption value	—	—	—	(5)
Net loss available to common stockholders	$(9,011)	$ (5,834)	$(22,386)	$ (20,131)
Net loss per share, basic and diluted	$(0.29)	$ (0.27)	$(0.77)	$ (1.11)
Weighted-average shares used to compute basic and diluted net loss per share	30,723,704	21,572,498	29,182,872	18,215,857

See accompanying notes to these condensed consolidated financial statements.

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MIRAGEN THERAPEUTICS, INC
CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(in thousands, except share data)
(unaudited)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2017	22,568,006	$ 226	$ 131,877	$ —	$ (93,593)	$ 38,510
Issuance of common stock in public offering, net of issuance cost	7,414,996	74	37,771	—	—	37,845
Issuance of common stock, net of issuance cost; at the market	372,852	4	2,653		—	2,657
Issuance of common stock in private placement, net of issuance costs	150,987	2	933	—	—	935
Shares issued for cash upon the exercise of stock options	274,082	2	177	—	—	179
Issuance of common stock for cash under employee stock purchase plan	52,444	—	242	—	—	242
Share-based compensation expense	—	—	2,742	—	—	2,742
Change in unrealized loss on investments	—	—	—	(6)	—	(6)
Net loss	—	—	—	—	(22,386)	(22,386)
Balance at September 30, 2018	30,833,367	$ 308	$ 176,395	$ (6)	$ (115,979)	$ 60,718

See accompanying notes to these condensed consolidated financial statements.

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MIRAGEN THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)
(unaudited)

	Nine Months Ended September 30,	
	2018	2017
Cash flows from operating activities:		
Net loss	$(22,386)	$(20,126)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation expense	2,742	1,693
Depreciation and amortization	206	227
Non-cash interest expense	283	85
Amortization of premiums and discounts on available-for-sale securities	(229)	—
Changes in operating assets and liabilities:		
Accounts receivable	74	(615)
Prepaid expenses and other assets	(927)	(719)
Accounts payable	287	(598)
Accrued and other liabilities	1,281	(871)
Net cash used in operating activities	(18,669)	(20,924)
Cash flows from investing activities:		
Purchases of short-term investments	(38,604)	—
Purchases of property and equipment	(394)	(212)
Cash acquired in reverse merger	—	1,280
Net cash provided by (used in) investing activities	(38,998)	1,068
Cash flows from financing activities:		
Proceeds from the sale of common stock - public offering	40,782	—
Payment of issuance costs associated with the sale of common stock - public offering	(2,890)	—
Proceeds from the sale of common stock - at the market	2,747	2,711
Payment of issuance costs associated with the sale of common stock - at the market	(82)	(172)
Proceeds from the sale of common stock - private financing	1,000	40,703
Payment of issuance costs associated with the sale of common stock - private placement	(52)	(1,216)
Payment of issuance costs associated with the shelf registration	—	(299)
Proceeds from stock purchases under employee stock purchase plan	242	110
Proceeds from the exercise of stock options	179	220
Payments of principal on note payable	—	(1,500)
Net cash provided by financing activities	41,926	40,557
Net increase (decrease) in cash and cash equivalents	(15,741)	20,701
Cash and cash equivalents at beginning of period	47,441	22,104
Cash and cash equivalents at end of period	$31,700	$42,805
Supplemental disclosure of cash flow information		
Cash paid for interest	$358	$112
Supplemental disclosure of non-cash investing and financing activities		
Amortization of public offering costs	$55	$—
Unpaid common stock issuance costs included in current liabilities	$13	$3
Change in unrealized gain (loss) on investments	$(6)	$—
Conversion of preferred stock to common stock	$—	$76,981
Liabilities assumed, net of non-cash assets received in reverse merger	$—	$1,076

Transfer of common stock issuance costs from prepaid expenses and other current assets to equity (private financing and at the market sales)	$—	$339
Reclassification of preferred stock warrant (accrued liability) to common stock warrant (equity)	$—	$51
Accretion of redeemable convertible preferred stock to redemption value	$—	$5

See accompanying notes to these condensed consolidated financial statements.

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MIRAGEN THERAPEUTICS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(unaudited)

1. DESCRIPTION OF BUSINESS

Miragen Therapeutics, Inc., a Delaware corporation (the "Company" or "Miragen"), is a clinical-stage biopharmaceutical company discovering and developing proprietary RNA-targeted therapies with a specific focus on microRNAs and their role in certain diseases where there is a high unmet medical need. microRNAs are short RNA molecules, or oligonucleotides, that regulate gene expression and play vital roles in influencing the pathways responsible for many disease processes. The Company has three clinical-stage product candidates, cobomarsen, remlarsen, and MRG-110. The Company is developing MRG-110 under a license and collaboration agreement (the "Servier Collaboration Agreement") with Les Laboratoires Servier and Institut de Recherches Servier (collectively, "Servier").

Liquidity

The Company has incurred annual net operating losses since its inception. As of September 30, 2018, the Company had an accumulated deficit of $116.0 million and a net loss of $9.0 million and $22.4 million for the three and nine months ended September 30, 2018, respectively. The Company's management believes that the $31.7 million of cash and cash equivalents and $38.8 million of short-term investments on hand at September 30, 2018 will be sufficient to fund its operations in the normal course of business and allow the Company to meet its liquidity needs into early 2020.

The Company has funded its operations to date principally through proceeds from equity financings, including notes payable that previously converted to equity explained further in Note 9. Common Stock. The Company will continue to require additional capital beyond early 2020 to continue its clinical development and potential commercialization activities. The amount and timing of future funding requirements will depend on many factors, including the pace and results of the Company's clinical development efforts, continued performance under the Servier Collaboration Agreement, securing additional partnerships and collaborations, and issuing debt or other financing vehicles. The Company's ability to secure additional capital is dependent upon a number of factors, some of which are outside of the Company's control, including success in developing its technology and drug product candidates, operational performance, and market conditions.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiary, Miragen Therapeutics Europe Limited ("Miragen Europe"), which was formed in January 2011 for the sole purpose of submitting regulatory filings in Europe. Miragen Europe has no employees or operations.

The financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("U.S. GAAP") and follow the requirements of the Securities and Exchange Commission (the "SEC") for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by U.S. GAAP can be condensed or omitted. These financial statements have been prepared on the same basis as the Company's annual financial statements and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments, which are necessary for a fair statement of the Company's financial information. These interim results are not necessarily indicative of the results to be expected for the year ending December 31, 2018, or for any other interim period, or for any other future year. The balance sheet as of December 31, 2017 has been derived from audited consolidated financial statements at that date but does not include all the information required by U.S. GAAP

for complete financial statements. All intercompany balances and transactions have been eliminated in consolidation.

The accompanying unaudited condensed consolidated financial statements and related financial information should be read in conjunction with the audited consolidated financial statements of the Company and the notes thereto contained in the Company's Form 10-K for the year ended December 31, 2017, filed with the SEC on March 15, 2018. The Company's management performed an evaluation of its activities through the date of filing of these financial statements and concluded that there are no subsequent events requiring disclosure, other than as disclosed.

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Use of Estimates

The Company's condensed consolidated financial statements are prepared in accordance with U.S. GAAP, which requires it to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and contingent liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Although these estimates are based on the Company's knowledge of current events and actions it may take in the future, actual results may ultimately differ from these estimates and assumptions.

Revenue Recognition

The Company recognizes revenue principally from its strategic alliance and collaboration agreement. Revenue is recognized from upfront payments for licenses and milestone payments that are generated from defined research or development events, as well as from the reimbursement of amounts for research and development services under its strategic alliance and collaboration agreement. The Company recognizes revenue when all four of the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) products have been delivered or services rendered; (3) the selling price is fixed or determinable; and (4) collectability is reasonably assured.

Multiple-element arrangements are examined to determine whether the deliverables can be separated or must be accounted for as a single unit of accounting. The Servier Collaboration Agreement, for example, includes a combination of upfront license fees, payments for research and development activities, and milestone payments that are evaluated to determine whether each deliverable under the agreement has value to the customer on a stand-alone basis and whether reliable evidence of fair value for the deliverable exists. Deliverables in an arrangement that do not meet this separation criteria are treated as a single unit of accounting, generally applying applicable revenue recognition guidance for the final deliverable to the combined unit of accounting.

The Company recognizes revenue from non-refundable upfront license fees over the term of performance when combined with other deliverables. When the performance period is not specified, the Company estimates the performance period based upon provisions contained within the agreement, such as the duration of the research or development term, the existence, or likelihood, of achievement of development commitments, and any other significant commitments. These advance payments are deferred and recorded as deferred revenue upon receipt, pending recognition, and are classified as a short-term or long-term liability in the accompanying condensed consolidated balance sheets. Expected performance periods are reviewed periodically and, if applicable, the amortization period is adjusted, which may accelerate or decelerate revenue recognition. The timing of revenue recognition, specifically as it relates to the amortization of upfront license fees, is significantly influenced by the Company's estimates.

The Company applies the milestone method of accounting to recognize revenue from milestone payments when earned, as evidenced by persuasive evidence that the milestone has been achieved and the payment is non-refundable, provided that the milestone event is substantive. A milestone event is defined as an event (1) that can only be achieved based in whole or in part on either the Company's performance or on the occurrence of a specific outcome resulting from the Company's performance; (2) for which there is substantive uncertainty at the inception of the arrangement that the event will be achieved; and (3) that would result in additional payments being due to the Company. Events for which the occurrence is either contingent solely upon the passage of time or the result of a counterparty's performance are not considered to be milestone events. A milestone event is substantive if all of the following conditions are met: (i) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value to the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone; (ii) the consideration relates solely to past performance; and (iii) the

consideration is reasonable relative to all the deliverables and payment terms (including other potential milestone consideration) within the arrangement. The Company assesses whether a milestone is substantive at the inception of each arrangement. If a milestone is deemed non-substantive, the Company accounts for the milestone payment using a method consistent with the related units of accounting for the arrangement over the estimated performance period.

Share-Based Compensation

The Company accounts for share-based compensation expense related to stock options granted to employees and members of its board of directors under its 2008 Equity Incentive Plan (the “2008 Plan”) and under its 2016 Equity Incentive Plan (the “2016 Plan”) by estimating the fair value of each stock option or award on the date of grant using the Black-Scholes option pricing model. The Company recognizes share-based compensation expense on a straight-line basis over the vesting term.

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The Company accounts for stock options issued to non-employees (other than board members) by valuing the award using an option pricing model and remeasuring such awards to the current fair value until the awards are vested or a performance commitment has otherwise been reached.

Research and Development

Research and development costs are expensed as incurred in performing research and development activities. The costs include employee-related expense including salaries, benefits, share-based compensation, fees for acquiring and maintaining licenses under third-party license agreements, consulting fees, costs of research and development activities conducted by third parties on the Company's behalf, laboratory supplies, depreciation, and facilities and overhead costs. The Company records research and development expense in the period in which the Company receives or takes ownership of the goods or when the services are performed. In circumstances where amounts have been paid in excess of costs incurred, the Company records a prepaid expense.

The Company records upfront and milestone payments to acquire contractual rights to licensed technology as research and development expenses when incurred if there is uncertainty in the Company receiving future economic benefit from the acquired contractual rights. The Company considers future economic benefits from acquired contractual rights to licensed technology to be uncertain until such a drug candidate is approved by the U.S. Food and Drug Administration or when other significant risk factors are abated.

Clinical Trial and Preclinical Study Accruals

The Company makes estimates of accrued expenses as of each balance sheet date in its condensed consolidated financial statements based on certain facts and circumstances at that time. The Company's accrued expenses for clinical trials and preclinical studies are based on estimates of costs incurred for services provided by clinical research organizations, manufacturing organizations, and other providers. Payments under the Company's agreements with external service providers depend on a number of factors, such as site initiation, patient screening, enrollment, delivery of reports, and other events. In accruing for these activities, the Company obtains information from various sources and estimates the level of effort or expense allocated to each period. Adjustments to the Company's research and development expenses may be necessary in future periods as its estimates change.

Cash and Cash Equivalents

All highly-liquid investments that have maturities of 90 days or less at the date of purchase are classified as cash equivalents. Cash equivalents are reported at cost, which approximates fair value due to the short maturities of these instruments.

Investments

The Company has designated its investments as available-for-sale securities and accounts for them at their respective fair values. The securities are classified as short-term or long-term based on the nature of the securities and their availability to meet current operating requirements. Securities that are readily available for use in current operations are classified as short-term available-for-sale securities and are reported as a component of current assets in the accompanying condensed consolidated balance sheets.

Securities that are classified as available-for-sale are measured at fair value, including accrued interest, with temporary unrealized gains and losses reported as a component of stockholders' equity until their disposition. The Company reviews available-for-sale securities at each period end to determine whether they remain available-for-sale based on its then current intent. The cost of securities sold is based on the specific identification method.

The securities are subject to a periodic impairment review. An impairment charge would occur when a decline in the fair value of the investments below the cost basis is judged to be other-than-temporary.

As of September 30, 2018, the Company's short-term available-for-sale securities had an amortized cost of $38.8 million, fair value of $38.8 million, and a gross unrealized loss of $6 thousand. The Company had no long-term investments as of September 30, 2018. The Company had no investments as of December 31, 2017.

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Fair Value of Financial Instruments

The following tables present information about the Company's financial assets and liabilities that have been measured at fair value and indicate the fair value of the hierarchy of the valuation inputs utilized to determine such fair value. In general, fair values determined by Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities. Fair value determined by Level 2 inputs utilize observable inputs other than Level 1 prices, such as quoted prices, for similar assets or liabilities, quoted market prices in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities. Fair values determined by Level 3 inputs are unobservable data points for the asset or liability, and include situations where there is little, if any, market activity for the asset or liability.

	September 30, 2018		December 31, 2017	
	Level 1	Level 3	Level 1	Level 3
	(in thousands)			
Assets:				
Money market funds (included in cash and cash equivalents) (1)	$32,539	$ —	$47,653	$ —
U.S. treasury securities (included in short-term investments)	38,827	—	—	—
Total assets	$71,366	$ —	$47,653	$ —
Liabilities:				
Common stock warrants (included in accrued and other liabilities)	$—	$ 82	$—	$ 82

(1) The sum of amounts presented for each period above differ from cash and cash equivalents reported in the condensed consolidated balance sheets due to uninvested cash balances and outstanding disbursements and deposits.

Certain of the Company's financial instruments are not measured at fair value on a recurring basis but are recorded at amounts that approximate their fair value due to the short-term nature of their maturities, such as cash and cash equivalents, accounts receivable, accounts payable, and accrued expenses. The carrying amount of the Company's note payable approximates its fair value (a Level 2 fair value measurement), reflecting interest rates currently available to the Company.

The Company accounts for warrants to purchase its stock pursuant to ASC Topic 470, Debt, and ASC Topic 480, Distinguishing Liabilities from Equity, and classifies warrants for common stock as liabilities or equity. The warrants classified as liabilities are reported at their estimated fair value and any changes in fair value are reflected in interest expense and other related expenses. The warrants classified as equity are reported at their estimated fair value with no subsequent remeasurement.

Concentrations of Credit Risk

Financial instruments that potentially subject the Company to concentrations of credit risk consist primarily of cash equivalents, which include short-term investments that have maturities of less than three months. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits. The Company has not experienced any losses in such accounts. The Company invests its excess cash primarily in deposits and money market funds held with one financial institution.

Property and Equipment

The Company carries its property and equipment at cost, less accumulated depreciation and amortization. Depreciation is computed using the straight-line method over the estimated useful lives of the assets, generally three to five years. Leasehold improvements are amortized over the shorter of the life of the lease (including any renewal periods that are deemed to be reasonably assured) or the estimated useful life of the assets. Construction in progress is not depreciated until placed in service. Repairs and maintenance costs are expensed as incurred and expenditures for major improvements are capitalized.

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Impairment of Long-Lived Assets

The Company assesses the carrying amount of its property and equipment whenever events or changes in circumstances indicate the carrying amount of such assets may not be recoverable. No impairment charges were recorded during the three and nine months ended September 30, 2018 and 2017.

Net Loss per Share

Basic net loss per share is calculated by dividing the net loss applicable to common stockholders by the weighted average number of shares of Common Stock outstanding during the period without consideration of Common Stock equivalents. Since the Company was in a loss position for all periods presented, diluted net loss per share is the same as basic net loss per share for all periods, as the inclusion of all potential common shares outstanding is anti-dilutive.

Comprehensive Loss

Comprehensive loss is comprised of net loss and adjustments for the change in unrealized gains and losses on investments. Unrealized accumulated comprehensive gains are reflected as a separate component in the statement of stockholders' equity. As of September 30, 2018, the Company had accumulated other comprehensive loss of $6 thousand. The Company had no accumulated other comprehensive income (loss) at December 31, 2017. The Company had no realized gain or loss during the three and nine months ended September 30, 2018 and 2017.

Income Taxes

The Company accounts for income taxes by using an asset and liability method of accounting for deferred income taxes. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. A valuation allowance is recorded to the extent it is more likely than not that a deferred tax asset will not be realized. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in operations in the period that includes the enactment date.

The Company's significant deferred tax assets are for net operating loss carryforwards, tax credits, accruals and reserves, and capitalized start-up costs. The Company has provided a valuation allowance for its entire net deferred tax assets since inception as, due to its history of operating losses, the Company has concluded that it is more likely than not that its deferred tax assets will not be realized.

The Company has no unrecognized tax benefits. The Company classifies interest and penalties arising from the underpayment of income taxes in the condensed consolidated statements of operations and comprehensive loss as general and administrative expenses. No such expenses have been recognized during the three and nine months ended September 30, 2018 and 2017.

The Tax Cuts and Jobs Act ("Tax Act") was signed into law on December 22, 2017. The Tax Act includes significant changes to the U.S. corporate income tax system, including: (i) a federal corporate rate reduction from 35% to 21%; (ii) limitations on the deductibility of interest expense and executive compensation; (iii) elimination of the corporate alternative minimum tax ("AMT") and a change in how existing AMT credits can be realized; (iv) change in the rules related to uses and limitations of net operating loss carryforwards created in tax years beginning after December 31, 2017; (v) reduction of the orphan drug credit from 50% to 25%; and (vi) transition of U.S. international taxation from a worldwide tax system to a territorial tax system. The Company does not anticipate the Tax Act to have a material impact on the condensed consolidated financial statements primarily due to the valuation allowance recorded against

its net deferred tax assets.

Segment Information

The Company operates in one operating segment and, accordingly, no segment disclosures have been presented herein. All equipment, leasehold improvements, and other fixed assets are physically located within the United States and all agreements with the Company's partners are denominated in U.S. dollars, except where noted.

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Recent Accounting Pronouncements – Not Yet Adopted

Revenue Recognition

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-09, Revenue from Contracts with Customers (Accounting Standards Codification Topic 606) ("ASC 606"), and has issued a number of clarifying ASUs subsequently, all of which outline a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The core principle of the revenue model is that "an entity recognizes revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services." The standard provides enhancements to the quality and consistency of how revenue is reported by companies, while also improving comparability in the financial statements of companies reporting using International Financial Reporting Standards or U.S. GAAP. The new standard also will require enhanced revenue disclosures, provide guidance for transactions that were not previously addressed comprehensively, and improve guidance for multiple-element arrangements. This accounting standard becomes effective for the Company for reporting periods beginning after December 15, 2018, and interim reporting periods thereafter. Early adoption is permitted for annual reporting periods (including interim periods) beginning after December 15, 2016. This new standard permits the use of either the retrospective or cumulative effect transition method.

The Company has elected to not early adopt ASC 606. The Company is assessing the impact of ASC 606 on its accounting policies and procedures and evaluating the new requirements as applied to existing revenue contracts. While this assessment is still in process, the Company does not believe the adoption of ASC 606 will have a material impact on its condensed consolidated financial statements because the Company has limited active contracts in effect. The Company also continues to evaluate the method of adoption and believes the selected implementation method will be dependent upon the contract or contracts that are in place at the transition date. In addition to the assessment of the impact of ASC 606, the Company is analyzing and updating its internal controls over financial reporting to ensure that information required to fulfill the new standard is properly secured and recorded. The Company will implement any changes as required by the adoption of ASC 606 beginning in the first quarter of 2019.

Leases

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842) and subsequent amendments to the initial guidance: ASU No. 2017-13, ASU No. 2018-10, and ASU No. 2018-11 (collectively, "Topic 842"). Topic 842 requires companies to generally recognize on the balance sheet operating and financing lease liabilities and corresponding right-of-use assets. The standard is effective for the Company for interim and annual reports beginning after December 15, 2019, with early adoption permitted. At adoption, this update will be applied using a modified retrospective approach, with an option to use certain transition relief. The Company currently expects that its building operating lease commitments will be subject to the new standard and recognized as operating lease liabilities and right-of-use assets upon its adoption of Topic 842, which will increase the Company's total assets and total liabilities that are reported relative to such amounts prior to adoption. The Company is continuing to evaluate the full impact of this standard on its condensed consolidated financial statements.

Share-based Compensation

In June 2018, the FASB issued ASU No. 2018-07, Compensation—Stock Compensation (Topic 718): Improvements to Non-employee Share-Based Payment Accounting, which simplifies the accounting for share-based payments to non-employees by aligning it with the accounting for share-based payments to employees, with specified exceptions. This standard is effective for the Company in the first quarter of 2020, and early adoption is permitted. The Company

expects the impact of the pending adoption of this standard will not have a significant impact on its condensed consolidated financial statements upon adoption.

Fair Value Measurement

In August 2018, the FASB issued ASU No. 2018-13, Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement, which modifies the disclosure requirements of fair value measurements. This standard is effective for the Company in the first quarter of 2020, and early adoption is permitted. The Company is currently evaluating the impact of the pending adoption of this standard on its condensed consolidated financial statements.

Other new pronouncements issued but not effective as of September 30, 2018 are not expected to have a material impact on the Company’s condensed consolidated financial statements.

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3. STRATEGIC ALLIANCE AND COLLABORATION WITH SERVIER

Collaboration revenue under the Servier Collaboration Agreement consisted of the following:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
	(in thousands)			
Collaboration revenue:				
Milestone payments	$—	$—	$3,690	$—
Reimbursed payments	814	1,493	3,248	1,991
Total collaboration revenue	$814	$1,493	$6,938	$1,991

In October 2011, the Company entered into the Servier Collaboration Agreement with Servier for the research, development, and commercialization of RNA-targeting therapeutics in cardiovascular disease. Under the Servier Collaboration Agreement, the Company granted Servier an exclusive license to research, develop, manufacture, and commercialize RNA-targeting therapeutics for certain microRNA targets in the cardiovascular field. In 2017, the Company and Servier agreed to amend the Servier Collaboration Agreement to remove all existing targets, add one new target (microRNA-92), and grant Servier the right to add one additional target through September 2019.

In April 2018, the Company and Servier entered into a seventh amendment to the Servier Collaboration Agreement (the “Servier Amendment”). The Servier Amendment, among other things, (i) updated the development plan for MRG-110 and cost-sharing provisions; (ii) provided for specified development cost reimbursement by Servier to the Company following a determination by a joint committee established by the parties under the Servier Collaboration Agreement that the outcome of a specified portion of a Phase 1 clinical trial has met its primary end point; and (iii) provided for additional development plan cost reimbursement by Servier to the Company following a determination by a joint committee established by the parties under the Servier Collaboration Agreement that a product candidate targeting microRNA-92 will proceed into a Phase 2 clinical trial.

Servier’s rights to each named target are limited to therapeutics in the field of cardiovascular disease, as defined, and in their territory, which is worldwide except for the United States and Japan. The Company retains all other rights including commercialization of therapeutics developed under the Servier Collaboration Agreement in the field of cardiovascular disease in the United States and Japan.

The Company is eligible to receive non-refundable development milestone payments of €5.8 million to €13.8 million ($6.7 million to $16.0 million as of September 30, 2018) and regulatory milestone payments of €10.0 million to €40.0 million ($11.6 million to $46.4 million as of September 30, 2018) for each target. Additionally, the Company may receive up to €175.0 million ($203.0 million as of September 30, 2018) in commercialization milestones, as well as quarterly royalty payments expressed in percentages ranging from the low-double digits to the mid-teens (subject to reductions for patent expiration, generic competition, third-party royalty, and costs of goods) on the net sales of any licensed product commercialized by Servier. Servier is obligated to make royalty payments for a period specified under the Servier Collaboration Agreement.

The Company applies the milestone method of accounting to recognize revenue from milestone payments when earned, as evidenced by persuasive evidence that the milestone has been achieved and the payment is non-refundable, provided that the milestone event is substantive. In March 2018, the Company and Servier initiated a Phase 1 clinical

trial of MRG-110. As a result, under the terms of the Servier Collaboration Agreement, the Company earned and received its first development milestone payment of €3.0 million (or $3.7 million). This amount is included as revenue in the accompanying condensed consolidated statements of operations and comprehensive loss during the nine months ended September 30, 2018.

As part of the Servier Collaboration Agreement, the Company established a multiple-year research collaboration, under which it jointly performs agreed upon research activities directed to the identification and characterization of named targets and oligonucleotides in the cardiovascular field, which is referred to as the Research Collaboration. The current amended term of the Research Collaboration extends through September 2019. Servier is responsible for funding certain costs of the Research Collaboration, as defined under the Servier Collaboration Agreement.

The development of each product candidate (commencing with registration enabling toxicology studies) under the Servier Collaboration Agreement is performed pursuant to a mutually agreed upon development plan to be conducted by the parties as necessary to generate data useful for both parties to obtain regulatory approval of such product candidates. Servier is

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responsible for a specified percentage of the cost of research and development activities under the development plan through the completion of one or more Phase 2 clinical trials and will reimburse the Company for a specified portion of such costs it incurs. The costs of Phase 3 clinical trials for each product candidate will be allocated between the parties at a specified percentage of costs. The applicable percentage for each product candidate will be based upon whether certain events under the Servier Collaboration Agreement occur, including if the Company enters into a third-party agreement for the development and/or commercialization of a product in the United States at least 180 days before the initiation of the first Phase 3 clinical trial, or if the Company subsequently enters into a U.S. partner agreement, or if it does not enter into a U.S. partner agreement but files for approval in the United States using data from the Phase 3 clinical trial.

Under the Servier Collaboration Agreement, the Company also granted Servier a royalty-free, non-exclusive license to develop a companion diagnostic in its territory for any therapeutic product that may be developed by Servier under the Servier Collaboration Agreement. The Company also granted Servier an exclusive, royalty-free license to commercialize such a companion diagnostic in its territory for use in connection with the development and commercialization of such therapeutic product in its territory.

The Servier Collaboration Agreement will expire as to each underlying product candidate when Servier's royalty obligations as to such product candidate have expired. Servier may also terminate the Servier Collaboration Agreement for: (i) convenience upon a specified number of days' prior notice to the Company or (ii) upon determination of a safety issue relating to development under the agreement upon a specified number of days' prior notice to the Company. Either party may terminate the Servier Collaboration Agreement upon a material breach by the other party that is not cured within a specified number of days. The Company may also terminate the agreement if Servier challenges any of the patents licensed by the Company to Servier.

The Company determined that the elements within the Servier Collaboration Agreement should be treated as a single unit of accounting because the delivered elements, the licenses, did not have stand-alone value to Servier at the time the license was granted. As such, the Company recognized license fees earned under the Servier Collaboration Agreement as revenue on a proportional performance basis over the estimated period to complete the activities under the Research Collaboration. The total period of performance is equal to the estimated term of the Research Collaboration. The Company measured its progress under the proportional performance method based on actual and estimated full-time equivalents. The Company received a total of $12.4 million (€9.0 million) in non-refundable license fees under the Servier Collaboration Agreement. Based on earlier estimates of the term of the Research Collaboration, these license fees had been fully recognized as revenue during the period from October 2011 through December 2016. Accordingly, no amounts were recognized as license revenue during the three and nine months ended September 30, 2018 and 2017, respectively.

Amounts incurred but not billed to Servier for research and related intellectual property activities totaled $0.8 million and $1.1 million as of September 30, 2018 and December 31, 2017, respectively. These amounts are included in prepaid expenses and other current assets in the Company's condensed consolidated balance sheets. As of September 30, 2018 and December 31, 2017, accounts receivable for Servier research and related intellectual property activities totaled $1.4 million.

4. REVERSE MERGER

In February 2017, the Company, then known as Signal Genetics, Inc. ("Signal"), completed its merger with Miragen Therapeutics, Inc., a then privately-held Delaware corporation ("Private Miragen"). Pursuant to the Agreement and Plan of Merger and Reorganization (the "Merger Agreement") by and among the Company, Private Miragen, and Signal Merger Sub, Inc., a wholly-owned subsidiary of the Company ("Merger Sub"), Merger Sub merged with and into Private Miragen, with Private Miragen surviving as a wholly-owned subsidiary of the Company (the "Merger").

Immediately, following the Merger, Private Miragen merged with and into the Company, with the Company as the surviving corporation (the “Short-Form Merger” and, together with the Merger, the “Mergers”). In connection with the Short-Form Merger, the Company changed its corporate name to “Miragen Therapeutics, Inc.”

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For accounting purposes, Private Miragen is considered to have acquired Signal in the Merger. Private Miragen was determined to be the accounting acquirer based upon the terms of the Merger and other factors including: (i) the Private Miragen security holders owned approximately 95.2% of the combined company's outstanding common stock immediately following the closing of the Mergers; (ii) former Private Miragen directors held all of the board seats in the combined company immediately following the closing of the Mergers; and (iii) Private Miragen management held key management positions of the combined company. The Merger has been accounted for as an asset acquisition rather than business combination because the assets acquired and liabilities assumed by Private Miragen do not meet the definition of a business as defined by U.S. GAAP. The net assets acquired in connection with this transaction were recorded at their estimated acquisition date fair values in February 2017 as of the date the Mergers were completed.

Immediately prior to the effective date of the Merger, all shares of preferred stock of Private Miragen converted into shares of common stock of Private Miragen on a one-for-one basis.

At the effective date of the Merger, the Company issued shares of its Common Stock to Private Miragen stockholders, at an exchange rate of approximately 0.7031 shares of Common Stock in exchange for each share of Private Miragen common stock outstanding immediately prior to the Merger. The exchange rate was calculated by a formula that was determined through arms-length negotiations between the Company and Private Miragen. The combined company assumed all the outstanding options, whether or not vested, under the 2008 Plan with such options representing the right to purchase a number of shares of Common Stock equal to approximately 0.7031 multiplied by the number of shares of Private Miragen common stock previously represented by such options.

Immediately after the Merger, there were 21,309,440 shares of Common Stock outstanding. In addition, immediately after the Merger, Private Miragen stockholders, warrant holders, and option holders owned approximately 95.9% of the aggregate number of shares of Common Stock, and the stockholders of the Company immediately prior to the Merger owned approximately 4.1% of the aggregate number of shares of Common Stock (each on a fully diluted basis). The accompanying unaudited condensed consolidated financial statements and notes to the unaudited condensed consolidated financial statements give retroactive effect to the exchange ratio and change in par value for all periods presented.

On February 13, 2017, prior to the effectiveness of the Merger, Signal had 1,024,960 shares of Common Stock outstanding and a market capitalization of $12.6 million. The estimated fair value of the net assets of Signal on February 13, 2017, prior to the Merger, was $0.2 million. The fair value of Common Stock on the Merger closing date, prior to the Merger, was above the fair value of the Company's net assets. As the Company's net assets were predominantly comprised of cash offset by current liabilities, the fair value of the Company's net assets as of February 13, 2017, prior to the Merger, is considered to be the best indicator of the fair value and, therefore, the purchase consideration.

The following table summarizes the net assets acquired based on their estimated fair values immediately prior to the Merger (in thousands):

Cash and cash equivalents	$1,280
Prepaid and other assets	248
Accrued liabilities	(1,324)
Net acquired tangible assets	$204

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5. PROPERTY AND EQUIPMENT

Property and equipment, net, consisted of the following:

	September 30, 2018	December 31, 2017
	(in thousands)	
Lab equipment	$2,490	$ 2,229
Leasehold improvements	741	737
Computer hardware and software	409	355
Furniture and fixtures	126	77
Property and equipment, gross	3,766	3,398
Less: accumulated depreciation and amortization	(3,015)	(2,835)
Property and equipment, net	$751	$ 563

During the three months ended September 30, 2018 and 2017, depreciation and amortization expense was $0.1 million. During the nine months ended September 30, 2018 and 2017, depreciation and amortization expense was $0.2 million. Depreciation and amortization expense is recorded primarily in research and development expense on the condensed consolidated statements of operations and comprehensive loss.

6. ACCRUED LIABILITIES

Accrued liabilities consisted of the following:

	September 30, 2018	December 31, 2017
	(in thousands)	
Accrued outsourced clinical trial and preclinical studies	$2,005	$ 581
Accrued employee compensation and related taxes	1,259	1,538
Accrued legal fees and expenses	358	185
Accrued equipment and lab materials	284	197
Accrued other professional service fees	101	232
Deferred and accrued facility lease obligations	83	74
Value of liability-classified stock purchase warrants	82	82
Other accrued liabilities	164	102
Total accrued liabilities	$4,336	$ 2,991

7. NOTES PAYABLE

2017 Silicon Valley Bank Loan Agreement

In November 2017, the Company entered into a loan and security agreement with Silicon Valley Bank (the “2017 SVB Loan Agreement”), which amended and restated the loan and security agreement Private Miragen entered into with Silicon Valley Bank in April 2015 (the “2015 SVB Loan Agreement”). Upon entry into the 2017 SVB Loan Agreement, the Company borrowed $10.0 million with a 30-month payment period following an 18-month interest-only payment period ending in November 2021. Under certain circumstances, the interest-only period can be extended by an additional six months. Amounts outstanding bear interest at the prime rate (5.25% at September 30, 2018), with a final payment fee equal to $0.9 million due upon maturity. As of September 30, 2018, no additional amounts are available under the 2017 SVB Loan Agreement.

The Company may elect to prepay prior to maturity all or any portion of the outstanding principal amounts under the 2017 SVB Loan Agreement, subject to a prepayment charge, depending on the date of prepayment or upon the occurrence of an event of default in which the Company's obligations to repay the outstanding principal is accelerated. The Company's obligations under the 2017 SVB Loan Agreement are secured by a first-priority security interest, right, and title in all business assets, excluding the Company's intellectual property, which is subject to a negative pledge.

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The 2017 SVB Loan Agreement includes customary representations, warranties, and covenants (affirmative and negative), including restrictive covenants that limit the Company's ability to: encumber or dispose of the collateral securing the loan; change the business of the Company; transfer a material portion of the Company's assets; acquire other businesses; and merge or consolidate with or into any other business organization; incur additional indebtedness; declare or pay any cash dividend or make a cash distribution on any class of stock or other equity interest; enter into specified material transactions with Company affiliates; make non-ordinary course payments or enter into any amendment regarding subordinated debt of the Company; or become an "investment company" under the Investment Company Act of 1940, as amended; in each case subject to specified exceptions.

The 2017 SVB Loan Agreement also includes standard events of default, including payment defaults; breaches of covenants following any applicable cure period; material breaches of representations or warranties; the occurrence of a material adverse change (as defined in the 2017 SVB Loan Agreement); events relating to bankruptcy or insolvency; breaches of material third-party agreements; the occurrence of an unsatisfied material judgment against the Company; and specified governmental actions against the Company, including specified actions by the U.S. Food and Drug Administration. Upon the occurrence of an event of default, Silicon Valley Bank may declare all outstanding obligations immediately due and payable, including a prepayment charge, and take such other actions as are set forth in the 2017 SVB Loan Agreement. Upon the occurrence of an event of default, at the Silicon Valley Bank's discretion, interest on the 2017 SVB Loan Agreement will accrue at 5.0% above the rate that is otherwise applicable thereto until the earlier of the repayment of the Company's obligations under the 2017 SVB Loan Agreement or the cure of such event of default.

2015 Silicon Valley Bank Loan Agreement

In April 2015, Private Miragen entered into the 2015 SVB Loan Agreement and $5.0 million was funded in May 2015, which had a 30-month payment period following an 18-month interest-only payment period that ended in November 2016. Interest accrued on amounts outstanding at the prime rate minus 0.25%, with a final payment fee equal to 5.50% of amounts borrowed. Upon the execution of the 2017 SVB Loan Agreement, the 2015 SVB Loan agreement was terminated in its entirety. As a result, the Company paid the remaining principal and final interest payment with proceeds from the 2017 SVB Loan Agreement. The Company accounted for the termination of the 2015 SVB Loan Agreement as an extinguishment and incurred a loss on debt extinguishment of $0.1 million, which was recorded within interest expense.

Amounts outstanding under the SVB loan agreements were as follows:

	September 30, 2018	December 31, 2017
	(in thousands)	
Principal amount outstanding	$10,000	$ 10,000
Unamortized debt discount	(80)	(119)
Accreted final payment fee	285	41
Total note payable	10,205	9,922
Less: current maturities	(1,291)	—
Long-term note payable, net of current portion	$8,914	$ 9,922

Future annual minimum principal payments under the 2017 SVB Loan Agreement as of September 30, 2018 for the respective calendar years are as follows (in thousands):

2018	$—
2019	2,333
2020	4,000
2021	3,667

Total	$10,000

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8. COMMITMENTS AND CONTINGENCIES

Indemnification Agreements

The Company has entered into indemnification agreements with each of its directors and officers whereby it has agreed to indemnify such persons for certain events or occurrences while the individual is, or was, serving as a director, officer, employee, or other agent of the Company. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited.

Employment Agreements

The Company has entered into agreements with its executives that provide for base salary, severance, eligibility for bonuses, and other generally available benefits. The agreements provide that the Company may terminate the employment of its executives at any time, with or without cause.

If an executive is terminated without cause, as defined in the employment agreements, or an executive resigns for good reason, as defined in the employment agreements, then the executive is entitled to receive, upon the execution of a release agreement, a severance package consisting of: (i) the equivalent of 12 months of the executive's base salary in effect immediately prior to date of termination; (ii) acceleration of vesting of the equivalent of 12 months of vesting of the executive's outstanding unvested stock options or other equity awards that were outstanding as of the effective date of the executive's employment agreement; and (iii) 12 months of continued health coverage.

If an executive is terminated without cause or resigns for good reason within one month prior to or 12 months following a change of control, as defined in the employment agreements, the executive is entitled to receive, upon the execution of a release agreement, a severance package consisting of: (i) the equivalent of 12 months of the executive's base salary in effect immediately prior to date of termination; (ii) the vesting in full of the executive's then-outstanding stock options or other equity awards subject to time-based vesting; and (iii) 12 months of continued health coverage. Solely in the case of the Company's Chief Executive Officer, if such termination occurs one month before or 12 months following a change of control, then, upon the execution of a release agreement, the executive is entitled to: (i) the equivalent of 24 months of the executive's base salary in effect immediately prior to the date of termination; (ii) the vesting in full of the executive's outstanding stock options or other equity awards subject to time-based vesting; and (iii) 12 months of continued health coverage.

License Agreements with the University of Texas

As of September 30, 2018, the Company had two exclusive patent license agreements (the "UT License Agreements") with the Board of Regents of The University of Texas System (the "University of Texas"). Under each of the UT License Agreements, the University of Texas granted the Company exclusive and nonexclusive licenses to certain patent and technology rights. The University of Texas is a minority stockholder of the Company.

In consideration of rights granted by the University of Texas, the Company is required to: (i) pay a nonrefundable upfront license documentation fee in the amount of $10 thousand per license; (ii) pay an annual license maintenance fee in the amount of $10 thousand per license starting one year from the date of each agreement; (iii) reimburse the University of Texas for actual costs incurred in conjunction with the filing, prosecution, enforcement, and maintenance of patent rights prior to the effective date; and (iv) bear all future costs of and manage the filing, prosecution, enforcement, and maintenance of patent rights. During the nine months ended September 30, 2018 and 2017, the Company incurred immaterial upfront and maintenance fees, which were recorded as research and development expense. All costs related to the filing, prosecution, and maintenance of patent and technology rights are recorded as general and administrative expense when incurred.

Under the terms of the UT License Agreements, the Company may be obligated to make the following future milestone payments for each licensed product candidate: (i) up to approximately $0.6 million upon the initiation of defined clinical trials; (ii) $2.0 million upon regulatory approval in the United States; and (iii) $0.5 million per region upon regulatory approval in other specified regions. Additionally, if the Company or any of its sublicensees successfully commercializes any product candidate subject to the UT License Agreements, it is responsible for royalty payments in the low-single digits based upon net sales of such licensed products and payments at a percentage in the mid-teens of any sublicense income, subject to specified exceptions. The University of Texas's right to these royalty payments will expire as to each license agreement upon the expiration of the last patent claim subject to the applicable UT License Agreement. During the nine months ended September 30, 2018, the Company made an immaterial milestone payment, which was recorded as research and development expense. Prior to September 30, 2018, the Company did not incur any milestone payments.

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The license term extends on a product-by-product and country-by-country basis until the expiration of the last to expire of the licensed patents that covers such product in such country. Upon expiration of the royalty payment obligation, the Company will have a fully-paid license in such country. The Company may also terminate each UT License Agreement for convenience upon a specified number of days' prior notice to the University of Texas. The University of Texas also has the right to earlier terminate the UT License Agreements after a defined date under specified circumstances where the Company has effectively abandoned its research and development efforts or has no sales. The UT License Agreements will terminate under customary termination provisions including automatic termination upon the Company's bankruptcy or insolvency, upon notice of an uncured material breach, and upon mutual written consent. All charges incurred under the UT License Agreements have been expensed to date due to the uncertainty as to future economic benefit from the acquired rights.

License Agreement with Roche Innovation Center Copenhagen A/S (formerly Santaris Pharma A/S)

In June 2010, Private Miragen entered into a license agreement with the Santaris Pharma A/S, which was subsequently acquired by F. Hoffmann-La Roche Ltd ("Roche") in 2014, and subsequently changed its name to Roche Innovation Center Copenhagen A/S ("RICC"). The agreement was amended in October 2011 and amended and restated in December 2012 (the "RICC License Agreement").

Under the RICC License Agreement, the Company has received exclusive and nonexclusive licenses from RICC to use specified technology of RICC (the "RICC Technology") for specified uses, including research, development, and commercialization of pharmaceutical products using this technology worldwide. Under the RICC License Agreement, the Company has the right to develop and commercialize the RICC Technology directed to four specified targets and the option to obtain exclusive product licenses for up to six additional targets. The acquisition of Santaris Pharma A/S by Roche was considered a change of control under the RICC License Agreement, and as such, certain terms and conditions of the RICC License Agreement changed, as contemplated and in accordance with the RICC License Agreement. These changes primarily relate to milestone payments reflected in the disclosures below. If the Company exercises its option to obtain additional product licenses or to replace the target families, it will be required to make additional payments to RICC.

Under the terms of the RICC License Agreement, milestone payments were previously decreased by a specified percentage as a result of the change of control by RICC referenced above. The Company is obligated to make milestone payments for each licensed product for up to $5.2 million, which is inclusive of a potential product license option fee. Certain of these milestones will be increased by a specified percentage if the Company undergoes a change of control during the term of the RICC License Agreement. If the Company grants a third party a sublicense to the RICC Technology, it is required to remit to Roche up to a specified percentage of the upfront and milestone and other specified payments it receives under its sublicense, and if such sublicense covers use of the RICC Technology in the United States or the entire European Union, the Company will not have any further obligation to pay the fixed milestone payments noted above. During the nine months ended September 30, 2018, the Company incurred $0.7 million in expense related to a milestone reached, which is included in research and development expense in the Company's condensed consolidated statements of operations and comprehensive loss.

If the Company or its sublicensee successfully commercializes any product candidate subject to the RICC License Agreements, then RICC is entitled to royalty payments in the mid-single digits on the net sales of such product, provided that if such net sales are made by a sublicensee under the RICC License Agreement, RICC is entitled to royalty payments equal to the lesser of a percentage in the mid-single digits on the net sales of such product or a specified percentage of the royalties paid to the Company by such sublicensee, subject to specified restrictions. The Company is obligated to make any such royalty payments until the later of: (i) a specified anniversary of the first commercial sale of the applicable product or (ii) the expiration of the last valid patent claim licensed by RICC under the RICC License Agreement underlying such product. Upon the occurrence of specified events, the royalty owed to

RICC will be decreased by a specified percentage.

The RICC License Agreement will terminate upon the latest of the expiration of all of RICC's royalty rights, the termination of the last Miragen target, or the expiration of its right to obtain a product license for a new target under the RICC License Agreement. The Company may also terminate the RICC License Agreement for convenience upon a specified number of days' prior notice to RICC, subject to specified terms and conditions. Either party may terminate the RICC License Agreement upon an uncured material breach by the other party and RICC may terminate the RICC License Agreement upon the occurrence of other specified events immediately or after such event is not cured within a specified number of days, as applicable.

All charges incurred under the RICC License Agreement have been expensed to date due to the uncertainty as to future economic benefit from the acquired rights.

During the nine months ended September 30, 2018 and 2017, the Company paid $0.3 million and $0.5 million, respectively, to RICC for raw materials to be used in its drug manufacturing process.

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Subcontract Agreement with Yale University

In October 2014, Private Miragen and Yale University ("Yale") entered into a subcontract agreement and then into a subaward agreement in March 2015 (the "Yale Agreements"), which were subsequently amended. Under the Yale Agreements, the Company is providing specified services regarding the development of a proprietary compound that targets miR-29 in the indication of idiopathic pulmonary fibrosis. Yale entered into the Yale Agreements in connection with a grant that Yale received from the National Institutes of Health ("NIH") for the development of a miR-29 mimic as a potential therapy for pulmonary fibrosis.

In consideration of the Company's services under the Yale Agreements, Yale has agreed to reimburse the Company up to a certain amount over five years, subject to the availably of funds under the grant and continued eligibility. Under the terms of the Yale Agreements, the Company retains all rights to any and all intellectual property developed solely by the Company in connection with the Yale Agreements. Yale has also agreed to provide the Company with an exclusive option to negotiate in good faith for an exclusive, royalty-bearing license from Yale for any intellectual property developed by Yale or jointly by the parties under the Yale Agreements. Yale is responsible for filing, prosecuting, and maintaining foreign and domestic patent applications and patents on all inventions jointly developed by the parties under the Yale Agreements. Through September 30, 2018, the Company received $0.8 million under the Yale Agreements.

The Yale Agreements terminate automatically on the date that Yale delivers its final research report to the NIH under the terms of the grant underlying the Yale Agreements. Each party may also terminate the Yale Agreements upon a specified number of days' notice in the event that the NIH's grant funding is reduced or terminated or upon material breach by the other party.

License Agreements with the t2cure GmbH

In October 2010, Private Miragen entered into a license and collaboration agreement (the "t2cure Agreement") with t2cure GmbH ("t2cure"), which was subsequently amended. Under the t2cure Agreement, the Company received a worldwide, royalty-bearing, and exclusive license to specified patent and technology rights relating to miR-92.

In consideration of rights granted by t2cure, Private Miragen paid an upfront fee of $46 thousand and agreed to: (i) pay an annual license maintenance fee in the amount of €3 thousand ($3 thousand as of September 30, 2018); and (ii) reimburse t2cure for costs incurred in conjunction with the filing, prosecution, enforcement, and maintenance of patent rights.

Under the terms of the t2cure Agreement, the Company is obligated to make the following future milestone payments for each licensed product, as defined in the t2cure Agreement: (i) up to approximately $0.7 million upon the initiation of certain defined clinical trials; (ii) $2.5 million upon regulatory approval in the United States; and (iii) up to $1.5 million per region upon regulatory approval in the European Union or Japan. Additionally, if the Company or any of its sublicensees successfully commercialize any product candidate subject to the t2cure Agreement, it is responsible for royalty payments equal to percentages in the low-single digits upon net sales of licensed products, and under certain circumstances, sublicense fees equal to a percentage of sublicense income received by it. The Company is obligated to make any such royalty payment until the later of: (i) the tenth anniversary of the first commercial sale of the applicable product or (ii) the expiration of the last valid claim to a patent licensed by t2cure under the t2cure Agreement covering such product. If such patent claims expire prior to the end of the ten-year term, then the royalty owed to t2cure will be decreased by a specified percentage. The Company also has the right to decrease its royalty payments by a specified percentage for royalties paid to third parties for licenses to certain third-party intellectual property.

The license term extends on a country-by-country basis until the later of: (i) the tenth anniversary of the first commercial sale of a licensed product in a country and (ii) the expiration of the last to expire valid claim that claims such licensed product in such country. Upon expiration of the royalty payment obligation, the Company will have a fully-paid license in such country. The Company has the right to terminate the t2cure Agreement at will, on a country-by-country basis, after 60 days' written notice. The t2cure Agreement will also automatically terminate upon the Company's bankruptcy or insolvency or upon notice of an uncured material breach.

The Company has expensed all charges incurred under the t2cure Agreement to date, due to the uncertainty as to future economic benefit from the acquired rights.

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License Agreement with The Brigham and Women's Hospital

In May 2016, Private Miragen and The Brigham and Women's Hospital ("BWH") entered into an exclusive patent license agreement (the "BWH License Agreement"). Under the BWH License Agreement, the Company has an exclusive, worldwide license, including a right to sublicense, to specified patent rights and a nonexclusive, worldwide license, including a right to sublicense, to specified technology rights of BWH, each related to certain microRNAs believed to be involved in various neurodegenerative disorders. As consideration for these rights, the Company is obligated to pay a specified annual license fee. BWH is also entitled to milestone payments of up to approximately $2.6 million for each of the Company's product candidates developed based on the patent rights subject to the BWH License Agreement plus a one-time sales milestone payment of $0.3 million for all product candidates developed based on the patent rights subject to the BWH License Agreement. If the Company were to successfully commercialize any product candidate subject to the BWH License Agreement, then BWH is entitled to royalty payments in the low-single digits on the net sales of such product. BWH's right to these royalty payments will expire on a product-by-product and country-by-country basis upon the expiration of the last patent claim in such country that is subject to the BWH License Agreement and covers the product, and the Company's license to such product in such country will become fully paid at such time. BWH is also entitled to a percentage in the low-double digits of any sublicense income from such product, subject to specified exceptions. The Company is also responsible for all costs associated with the preparation, filing, prosecution, and maintenance of the patent rights subject to the BWH License Agreement. Additionally, the Company is obligated to use commercially-reasonable efforts to develop a product under the BWH License Agreement and to meet specified diligence milestones thereunder.

The BWH License Agreement will terminate upon the expiration of all issued patents and patent applications subject to the patent rights under the agreement. The Company may also terminate the BWH License Agreement for convenience upon a specified number of days' prior notice to BWH. BWH may terminate the BWH License Agreement upon a breach by the Company of its payment obligations and upon the occurrence of other specified events that are not cured within a specified number of days, provided that such termination is automatic upon the Company's bankruptcy or insolvency.

Facility Lease

In December 2010, Private Miragen entered into a multi-year lease agreement for its current office and lab space. The agreement was subsequently amended to extend the term through August 2020. This lease is noncancelable. Minimum base lease payments, including the impact of tenant improvement allowances, under the operating lease are recognized on a straight-line basis over the full term of the lease.

During the three months ended September 30, 2018 and 2017, rent expense was $0.1 million. The Company is also required to pay for operating expenses related to the leased space, which were $0.1 million for the three months ended September 30, 2018 and 2017. During the nine months ended September 30, 2018 and 2017, rent expense was $0.3 million and $0.2 million, respectively. Operating expenses were $0.2 million for the nine months ended September 30, 2018 and 2017.

Future annual minimum payments under the lease as of September 30, 2018 for the respective calendar year were as follows (in thousands):

2018	$99
2019	404
2020	277
Total	$780

9. CAPITAL STOCK

Common Stock

The Company is authorized to issue 105,000,000 shares of its stock, of which 100,000,000 shares have been designated as Common Stock and 5,000,000 shares have been designated as preferred stock with a par value of $0.01 per share. The number of authorized shares of Common Stock may be increased or decreased by the affirmative vote of the holders of a majority of the Company's stock who are entitled to vote. Each share of Common Stock is entitled to one vote. The holders of Common Stock are entitled to receive dividends when and as declared or paid by its board of directors.

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Common Stock Purchase Agreement

In August 2018, the Company and The Leukemia & Lymphoma Society, Inc. ("LLS") entered into a Common Stock Purchase Agreement (the "LLS Stock Purchase Agreement") for the sale of up to $5.0 million of shares of Common Stock to LLS in a private placement (the "Offering"). Under the terms of the LLS Stock Purchase Agreement, the Company expects to raise up to approximately $5.0 million in gross proceeds by selling shares of Common Stock to LLS in up to five separate closings. The initial closing of the Offering was held on August 6, 2018. At the initial closing, the Company issued 150,987 shares of Common Stock at a price per share equal to $6.62, which resulted in net proceeds of approximately $0.9 million after expenses incurred in connection with the Offering. The price per share of Common Stock to be sold in any subsequent closing will be equal to the average of the volume weighted-average prices of a share of Common Stock on the Nasdaq Capital Market for the three trading days beginning with the first trading day after the date of achievement of the relevant milestone for each such closing. Each closing is subject to the Company's achievement of specified operational milestones under the LLS Stock Purchase Agreement and other customary closing conditions, provided, however, that each such closing must be completed prior to December 31, 2021.

Common Stock Sales Agreement

In March 2017, the Company entered into an at the market issuance Common Stock Sales Agreement (the "ATM Agreement") with Cowen and Company, LLC ("Cowen") under which the Company may offer and sell, from time to time at its sole discretion, shares of its Common Stock having an aggregate offering price of up to $50.0 million through Cowen as its sales agent.

Cowen may sell the Common Stock by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415 of the Securities Act of 1933, as amended, including without limitation sales made by means of ordinary brokers' transactions on The Nasdaq Capital Market or otherwise at market prices prevailing at the time of sale, in block transactions, or as otherwise directed by the Company. Cowen will use commercially-reasonable efforts to sell the Common Stock from time to time, based upon instructions from the Company (including any price, time, or size limits or other customary parameters or conditions the Company may impose). The Company will pay Cowen a commission equal to 3.0% of the gross sales proceeds of any Common Stock sold through Cowen under the ATM Agreement. The Company also has provided Cowen with customary indemnification rights.

The Company is not obligated to make any sales of Common Stock under the ATM Agreement. The offering of shares of Common Stock pursuant to the ATM Agreement will terminate upon the earlier of: (i) the sale of all Common Stock subject to the ATM Agreement or (ii) termination of the ATM Agreement in accordance with its terms.

During the nine months ended September 30, 2018, the Company sold, pursuant to the terms of the ATM Agreement, 372,852 shares of Common Stock, at a weighted average price of $7.37 per share, for aggregate net proceeds of approximately $2.7 million, including commissions to Cowen as sales agent. Through September 30, 2018, the Company sold, pursuant to the terms of the ATM Agreement, an aggregate of 1,213,386 shares of Common Stock, at a weighted average price of $8.74 per share, for aggregate net proceeds of approximately $10.2 million, including initial expenses for executing the "at the market offering" and commissions to Cowen as sales agent.

Common Stock Public Offering

In February 2018, the Company entered into an underwriting agreement (the "Underwriting Agreement") with underwriters relating to a public offering of its Common Stock. Under the Underwriting Agreement, in February 2018, the Company sold 7,414,996 shares of Common Stock at a price of $5.50 per share, which resulted in net proceeds of

approximately $37.9 million after deducting underwriting commissions and discounts and other offering expenses payable by the Company.

Private Miragen Common Stock Offering

In February 2017, immediately prior to the Merger and in accordance with subscription agreements entered into with certain investors in October 2016, Private Miragen issued and sold an aggregate of 9,045,126 shares of Private Miragen's common stock at a price per share of $4.50, or 6,359,628 shares of Common Stock at a price per share of $6.40 as adjusted for the exchange ratio in the Merger, which resulted in net proceeds of approximately $39.2 million, after giving effect to associated financing fees of $1.5 million.

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Series Preferred

As of September 30, 2018, the Company had no shares of preferred stock outstanding and had not designated the rights, preferences, or privileges of any class or series of preferred stock. The Company's board of directors has the authority to issue preferred stock at its discretion in one or more classes or series and to fix the designations, powers, preferences and rights, and the qualifications, limitations, or restrictions thereof, including dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences, and the number of shares constituting any class or series of preferred stock, without further vote or action by the stockholders.

10. WARRANTS

As of September 30, 2018, the Company had 49,349 Common Stock Warrants outstanding at a weighted average exercise price of $27.65. A summary of outstanding Common Stock purchase warrants as of September 30, 2018 is as follows:

Number of Underlying Shares	Exercise Price	Expiration Date
13,534	$80.70	2019 & 2020
11,718	$8.53	2025
24,097	$7.15	2024
49,349		

The Company had no stock purchase warrant activity during the nine months ended September 30, 2018.

11. SHARE-BASED COMPENSATION

Equity Incentive Plans

As of September 30, 2018, there were 1,645,892 options outstanding and no remaining equity awards available for future issuances under the 2008 Plan. All awards granted under the 2008 Plan that, after February 13, 2017, expire or terminate for any reason prior to exercise or settlement, are forfeited, or are reacquired, withheld, or not issued to satisfy a tax withholding obligation or to satisfy the exercise price of a stock award, will become available for grant under the 2016 Plan in accordance with its terms.

The 2016 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, other stock awards, and performance awards that may be settled in cash, stock, or other property. All employees and non-employee directors are eligible to participate in the 2016 Plan and may receive all types of awards other than incentive stock options. Incentive stock options may be granted under the 2016 Plan only to employees (including officers) and employees of the Company's affiliates.

The aggregate number of shares of Common Stock that may be issued under the 2016 Plan will not exceed 4,182,404 shares, which number is the sum of: (i) 1,681,294 shares, plus (ii) the number of shares subject to outstanding stock awards that were granted under the 2008 Plan, that, from and after the closing date of the Merger, expire or terminate for any reason prior to exercise or settlement, are forfeited because of the failure to meet a contingency or condition required to vest such shares, or are reacquired, withheld, or not issued to satisfy a tax withholding obligation in connection with an award or to satisfy the purchase price or exercise price of a stock award, if any, as such shares become available from time to time, plus (iii) 902,720 shares from previous automatic increases to the share reserve (as described in more detail below), including the automatic increase of 902,720 shares effected on January 1, 2018. In addition, the share reserve will automatically increase on January 1 of each year, for a period of not more than ten

years, commencing on January 1 of the year following the year in which the effective date of the 2016 Plan occurs, and ending on (and including) January 1, 2026, in an amount equal to 4% of the shares of Common Stock outstanding on December 31 of the preceding calendar year; however, the board of directors or compensation committee may act prior to January 1 of a given year to provide that there will be no January 1 increase in the share reserve for such year or that the increase in the share reserve for such year will be a lesser number of shares of Common Stock than would otherwise occur pursuant to the automatic increase. As of September 30, 2018, there were equity awards exercisable for 1,840,317 shares of Common Stock outstanding and 722,269 shares of Common Stock available for issuance pursuant to the terms under the 2016 Plan.

Options granted under the 2008 Plan and 2016 Plan have an exercise price equal to the market value of the Common Stock at the date of grant and expire ten years from the date of grant. Generally, options vest 25% on the first anniversary of the vesting

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commencement date and 75% ratably in equal monthly installments over the remaining 36 months. The Company has also granted options that vest in equal monthly or quarterly amounts over periods up to 48 months.

A summary of Common Stock option activity is as follows:

	Number of Options (in thousands)	Weighted Average Exercise Price
Outstanding at December 31, 2017	2,863	$ 4.85
Granted	1,069	$ 7.48
Exercised	(274)	$ 0.66
Forfeited or canceled	(141)	$ 9.80
Outstanding at September 30, 2018	3,517	$ 5.78

Fair Value Assumptions

The Company uses the Black-Scholes option pricing model to estimate the fair value of stock options granted under its equity compensation plans. The Black-Scholes model requires inputs for risk-free interest rate, dividend yield, volatility, and expected lives of the options. Because the Company has a limited history of stock purchase and sale activity, expected volatility is based on historical data from public companies that are similar to the Company in size and nature of operations. The Company will continue to use similar entity volatility information until its historical volatility is relevant to measure expected volatility for option grants. The Company accounts for forfeitures as they occur. The risk-free rate for periods within the contractual life of each option is based on the U.S. Treasury yield curve in effect at the time of the grant for a period commensurate with the expected term of the grant. The expected term (without regard to forfeitures) for options granted represents the period of time that options granted are expected to be outstanding and is derived from the contractual terms of the options granted and expected option-exercise behaviors. Prior to the Merger, Private Miragen estimated the fair value of underlying shares of its common stock using a third-party valuation report that derived the fair value using the probability-weighted expected return method. After the Merger, the fair value of the underlying Common Stock is based on the closing price of the Common Stock on The Nasdaq Capital Market at the date of grant.

Stock Options Granted to Employees

The weighted-average grant-date fair value of options granted during the nine months ended September 30, 2018 and 2017 was $5.51 and $8.32, respectively. The fair value was determined by the Black-Scholes option pricing model using the following weighted-average assumptions:

	Nine Months Ended September 30,	
	2018	2017
Expected term, in years	6.33	6.44
Expected volatility	84.9 %	83.8 %
Risk-free interest rate	2.6 %	2.1 %
Expected dividend yield	— %	— %
Weighted-average exercise price	$7.48	$11.48

Stock Options Granted to Non-Employees

The Company determines the value of Common Stock options issued to non-employees using the Black-Scholes option pricing model and adjusting the value of such awards to current fair value each reporting period until the awards are vested or a performance commitment has otherwise been reached. No Common Stock options were issued to non-employees during the nine months ended September 30, 2018 and 2017.

Employee Stock Purchase Plan

The 2016 Employee Stock Purchase Plan (“ESPP”) allows qualified employees to purchase shares of the Company's Common Stock at a price equal to 85% of the lower of: (i) the closing price at the beginning of the offering period or (ii) the closing price at the end of the offering period. The Company expects that a new 6-month offering period will begin each August 22 and

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February 22. As of September 30, 2018, the Company had 0.4 million shares available for issuance and 66 thousand shares had been issued under the ESPP.

Share-Based Compensation Expense

Share-based compensation related to all equity awards issued pursuant to the 2008 Plan and 2016 Plan and for estimated shares to be issued under the ESPP for the purchase periods active during each respective period is included in the condensed consolidated statements of operations and comprehensive loss as follows:

	Nine Months Ended September 30,	
	2018	2017
	(in thousands)	
Research and development	$933	$643
General and administrative	1,809	1,050
Total share-based compensation expense	$2,742	$1,693

As of September 30, 2018, the Company had $9.3 million of total unrecognized employee and non-employee share-based compensation costs, which the Company expects to recognize over a weighted-average remaining period of 2.8 years.

12. NET LOSS PER SHARE

Basic net loss per share is computed by dividing the net loss available to common stockholders by the weighted-average number of Common Stock outstanding. Diluted net loss per share is computed similarly to basic net loss per share except that the denominator is increased to include the number of additional shares of Common Stock that would have been outstanding if the potential shares of Common Stock had been issued and if the additional shares of Common Stock were dilutive. Diluted net loss per share is the same as basic net loss per share of Common Stock, as the effects of potentially dilutive securities are antidilutive.

Potentially dilutive securities include the following:

	September 30,	
	2018	2017
	(in thousands)	
Options to purchase Common Stock	3,517	2,969
Warrants to purchase Common Stock	49	25
Total	3,566	2,994

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FORWARD-LOOKING STATEMENTS

This quarterly report on Form 10-Q, or this Quarterly Report, contains forward-looking statements that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. All statements contained in this Quarterly Report other than statements of historical fact, including statements regarding our strategy, future operations, future financial position, future revenue, projected expenses, prospects, plans and objectives of management are forward-looking statements. The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “intend,” “plan,” “expect,” “predict,” “potential,” “opportunity,” “goals,” or “should,” and expressions are intended to identify forward-looking statements. Unless otherwise mentioned or unless the context requires otherwise, all references in this Quarterly Report to “Miragen,” “company,” “we,” “us” and “our” or similar references refer to Miragen Therapeutics, Inc., and our consolidated subsidiaries.

Such statements are based on management’s current expectations and involve risks and uncertainties. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors, including, without limitation:

- We have incurred losses since our inception, have a limited operating history on which to assess our business, and anticipate that we will continue to incur significant losses for the foreseeable future.

- Raising additional capital may cause dilution to our stockholders, restrict our operations, or require us to relinquish rights.

- We have never generated any revenue from product sales and may never be profitable.

- We are heavily dependent on the success of our product candidates, which are in the early stages of clinical development. Some of our product candidates have produced results only in early stage or pre-clinical settings, or for other indications than those for which we contemplate conducting development and seeking U.S. Food and Drug Administration, or FDA, approval for, and we cannot give any assurance that we will generate sufficient data for any of our product candidates to receive regulatory approval in our planned indications, which will be required before they can be commercialized.

- Regardless of clinical trial results, the FDA and other regulatory agencies may fail to approve our product candidates for marketing.

- If we obtain FDA approval for one of our product candidates that is granted orphan drug designation and receive the associated seven years’ marketing exclusivity, we may lose that exclusivity to the sponsor of a subsequent marketing application for the same drug and indication for several reasons, including a showing that the subsequent drug is clinically superior to the first.

- Clinical trials are costly, time consuming, and inherently risky, and we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

- The approach we are taking to discover and develop novel therapeutics that target microRNAs is unproven and may never lead to marketable products.

- Our microRNA-targeted therapeutic product candidates are based on a relatively novel technology, which makes it unusually difficult to predict the time and cost of development, and the time and cost, or likelihood, of obtaining regulatory approval. To date, no microRNA-targeted therapeutics have been approved for marketing in the United States.

- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent regulatory approval, limit the commercial viability of an approved label, or result in significant negative consequences following marketing approval, if any.

- We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.

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- We face substantial competition, and our competitors may discover, develop, or commercialize products faster or more successfully than us.

- We may be unable to realize the potential benefits of any collaboration.

- We may attempt to form collaborations in the future with respect to our product candidates, but we may not be able to do so, which may cause us to alter our development and commercialization plans.

- We may not be able to develop or identify technology that can effectively deliver any of our product candidates to the intended diseased cells or tissues, and any failure in such delivery technology could adversely affect and delay the development of any or all of our product candidates.

- If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business, or our market, our stock price and trading volume could decline.

We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. These forward-looking statements are subject to a number of risks, uncertainties, and assumptions, including those described in Part II, Item 1A, "Risk Factors" in this Quarterly Report and under a similar heading in any other periodic or current report we may file with the Securities and Exchange Commission, or SEC, in the future. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this Quarterly Report may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

We undertake no obligation to revise or publicly release the results of any revision to these forward-looking statements, except as required by law. Given these risks and uncertainties, readers are cautioned not to place undue reliance on such forward-looking statements. All forward-looking statements are qualified in their entirety by this cautionary statement.

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ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis should be read together with our condensed consolidated financial statements and the related notes thereto included in Part I, Item 1 of this Quarterly Report, our consolidated financial statements and related notes thereto for the year ended December 31, 2017, included in our Annual Report on Form 10-K filed with the SEC on March 15, 2018. This discussion and other parts of this report contain forward-looking statements reflecting our current expectations that involve risks and uncertainties, such as our plans, objectives, expectations, intentions, and beliefs. See "Forward-Looking Statements" for a discussion of the uncertainties, risks and assumptions associated with these statements. Actual results and the timing of events could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section entitled "Risk Factors" included elsewhere in this Quarterly Report.

Overview

We are a clinical-stage biopharmaceutical company discovering and developing proprietary RNA-targeted therapies with a specific focus on microRNAs and their role in certain diseases where there is a high unmet medical need. microRNAs are short RNA molecules, or oligonucleotides, that regulate gene expression and play vital roles in influencing the pathways responsible for many disease processes. Our lead programs include three clinical-stage product candidates, each of which we believe has the potential to treat multiple indications. We are independently developing cobomarsen and remlarsen and developing a third clinical-stage product candidate, MRG-110, in collaboration Les Laboratories Servier and Institute de Recherches Servier, or, collectively, Servier.

Cobomarsen is an inhibitor of miR-155, a microRNA that is found at abnormally high levels in malignant cells of several blood cancers, as well as certain cells involved in inflammation. In a Phase 1 clinical trial of cobomarsen in cutaneous T-cell lymphoma, or CTCL, a high percentage of patients treated systemically demonstrated improvement in mSWAT score, which is a measurement of the severity of skin disease over a patient's entire body and commonly used by clinicians treating CTCL. During the third quarter, we advanced our preparations to launch our global Phase 2 SOLAR clinical trial evaluating cobomarsen in patients diagnosed with CTCL, and we expect to begin dosing patients in the fourth quarter of 2018. In the SOLAR trial, we plan to evaluate the safety and efficacy of cobomarsen given by intravenous infusion in an active control comparison versus ZOLINZA (vorinostat) and to enroll approximately 65 patients per treatment group. The primary endpoint of the SOLAR trial is the rate of objective response, defined as 50% or greater improvement in the severity of a patient's skin disease over the entire body with no evidence of disease progression in the blood, lymph nodes, or viscera, and maintained for at least four consecutive months. Progression-free survival will be a secondary endpoint, and we plan to use patient-reported outcomes as additional endpoints to monitor quality of life improvements. Based on discussions with the FDA, we believe the results from this clinical trial could potentially allow us to apply for accelerated approval in the United States. The Leukemia & Lymphoma Society®, or LLS, is collaborating with us and providing funding in support of the SOLAR trial for the treatment of patients with CTCL. We expect to report data from this clinical trial in the second half of 2020.

Cobomarsen is also being evaluated in a Phase 1 clinical trial across three additional oncology indications, including adult T cell leukemia/lymphoma, or ATLL, diffuse large B-cell lymphoma, and chronic lymphocytic leukemia, for which the we have reported clinical response in ATLL, a highly morbid T-cell malignancy seen in patients previously infected with the human T lymphotropic virus type 1. We anticipate announcing additional ATLL clinical trial data in the first half of 2019.

Remlarsen is a replacement for miR-29, a microRNA that is found at abnormally low levels in a number of pathological fibrotic conditions, including cutaneous, cardiac, renal, hepatic, pulmonary, and ocular fibrosis, as well as

in systemic sclerosis. In a Phase 1 clinical trial of remlarsen, we observed a statistically-significant reduction in fibroplasia with no serious adverse events on incisional wound healing in trial participants who received remlarsen. In July 2018, we announced the initiation of a Phase 2 double-blinded, randomized clinical trial of remlarsen, which is designed to treat fibrotic diseases, in subjects with a predisposition for keloid formation. We anticipate that this clinical trial will enroll an initial 12-subject cohort at multiple clinical sites in the United States who are historically predisposed to keloid formation after trauma to the skin. Subjects will receive small, matching excisional wounds that will be sutured and then injected with either remlarsen or placebo. In this design, patients are serving as their own control, which increases the statistical power of the clinical trial. The lesions will be observed for up to 12 months to determine the presence or absence of keloid formation. We expect to report data from this clinical trial in the second half of 2019. We believe the results of the trial may help determine the dose, dose frequency, and number of patients necessary for a potential Phase 3 clinical trial of remlarsen in keloid revision.

MRG-110 is an inhibitor of miR-92, a microRNA expressed in endothelial cells. Inhibition of miR-92 has been shown to accelerate the formation of new blood vessels in preclinical models of heart failure, peripheral ischemia, and dermal wounding.

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MRG-110 is currently being evaluated in two Phase 1 clinical trials. Both clinical trials are designed to evaluate the safety, tolerability, and pharmacokinetics of MRG-110. The first clinical trial is a systemic dosing protocol that is intended to support the development of MRG-110 for the treatment of heart failure. In the second clinical trial, MRG-110 is being administered by intradermal injection in healthy volunteers receiving induced wounds through biopsy. In addition to assessing the safety and tolerability of MRG-110 after local administration, the second clinical trial is designed to provide data that can help validate MRG-110's intended mechanism of action in humans. The data generated in these clinical trials is expected to provide several clinically translatable biomarkers that may support future clinical trials for the treatment of heart failure as well as surgical incisions in high risk populations, severe lacerations, and chronic wounds. We expect to report data from these clinical trials in 2019. We are developing MRG-110 with Servier under our license and collaboration agreement, or the Servier Collaboration Agreement. We retain all commercial rights to MRG-110 in the United States and Japan, and Servier has commercial rights in the rest of the world.

In addition to our clinical-stage programs, we remain committed to research and continue to develop a pipeline of wholly-owned preclinical product candidates. We believe that our preclinical product candidates offer the potential to treat a number of indications including oncology, visual pathologies, neurodegeneration, and hearing loss. The goal of our translational medicine strategy is to progress rapidly to first-in-human trials once we have adequately established the pharmacokinetics (the movement of a drug into, through, and out of the body), pharmacodynamics (the effect and mechanism of action of a drug), safety, and manufacturability of the product candidate in preclinical studies.

In February 2017, we, then named Signal Genetics, Inc., completed a merger with a private corporation, then called Miragen Therapeutics, Inc., or Private Miragen, in which our wholly owned subsidiary was merged with and into Private Miragen. Immediately following this transaction, we completed a short-form merger with Private Miragen in which we were the surviving corporation and changed our name to Miragen Therapeutics, Inc. These transactions are referred to herein as the Merger.

Financial Operations Overview

Revenue

Our revenue consists primarily of upfront payments for licenses, milestone payments, and payments for other research services earned under our strategic alliance and collaboration agreement. We also recognize revenue for amounts received or receivable under certain grants we have been awarded.

In the future, we may generate revenue from a combination of license fees and other upfront payments, payments for research and development services, milestone payments, product sales, and royalties in connection with strategic alliances. We expect that any revenue we generate will fluctuate from quarter to quarter as a result of the timing of our achievement of preclinical, clinical, regulatory, and commercialization milestones, the timing and amount of payments relating to such milestones, and the extent to which any of our products are approved and successfully commercialized by us or our strategic alliance partners. If our strategic alliance partners do not elect or otherwise agree to fund our development costs pursuant to our strategic alliance agreements, or we or our strategic alliance partners fail to develop product candidates in a timely manner or to obtain regulatory approval for them, then our ability to generate future revenues, and our results of operations and financial position would be adversely affected.

Research and development expenses

Research and development costs are expensed as incurred and include costs associated with our research activities, drug discovery efforts, and development of our therapeutic programs, which includes:

- employee-related expenses, including salaries, benefits, travel, and share-based compensation expense;
- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, contract manufacturing organizations, or CMOs, other clinical trial-related vendors, consultants, and our scientific advisors;
- license fees related to the acquisition and retention of certain licensed technology and intellectual property rights; and
- facilities, depreciation, and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

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We occasionally make non-refundable advance payments for goods and services that will be used in future research and development activities. These payments are recorded as expense in the period in which we receive or take ownership of the goods or when the services are performed.

We record upfront and milestone payments to acquire contractual rights to licensed technology as research and development expenses when incurred if there is uncertainty in our receiving future economic benefit from the acquired contractual rights. We consider future economic benefits from acquired contractual rights to licensed technology to be uncertain until such a drug candidate is approved by the FDA or when other significant risk factors are abated.

We expect our research and development expenses to increase for the foreseeable future as we continue to conduct our ongoing clinical trials, initiate additional clinical trials, and advance our preclinical research programs. The process of conducting clinical trials and preclinical studies necessary to obtain regulatory approval is costly and time consuming. We, or our strategic alliance partners, may never succeed in achieving marketing approval for any of our product candidates. The probability of success for each product candidate may be affected by numerous factors, including clinical data, preclinical data, competition, manufacturing capability, and commercial viability.

Successful development of future product candidates is highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each future product candidate and are difficult to predict. We anticipate we will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to our ability to maintain or enter into new strategic alliances with respect to each program or potential product candidate, the scientific and clinical success of each future product candidate, and ongoing assessments as to each future product candidate’s commercial potential. We will need to raise additional capital and may seek additional strategic alliances in the future in order to advance our various programs.

General and administrative expenses

General and administrative expenses consist primarily of salaries and related benefits, including share-based compensation, related to our finance, accounting, human resources, legal, business development, and other support functions, professional fees for auditing, tax, and legal services, as well as insurance, board of director compensation, and other administrative expenses.

Other income, net

Other income, net consists primarily of interest income and expense, and various income or expense items of a non-recurring nature. We earn interest income from interest-bearing accounts, money market funds, and short-term investments. Interest expense is comprised of interest incurred under our note payable.

Critical Accounting Policies and Estimates

This discussion and analysis of financial condition and results of operations is based on our condensed consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or U.S. GAAP. The preparation of financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses. On an ongoing basis, we evaluate these estimates and judgments. We base our estimates on historical experience and on various assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. We believe that the accounting policy discussed below is critical to understanding our historical and future performance, as this policy relates to the more significant areas involving our judgments and estimates.

Clinical Trial and Preclinical Study Accruals

We make estimates of our accrued expenses as of each balance sheet date in our condensed consolidated financial statements based on certain facts and circumstances at that time. Our accrued expenses for preclinical studies and clinical trials are based on estimates of costs incurred for services provided by external service providers and for other trial-related activities. The timing and amount of expenses we incur though our external service providers depend on a number of factors, such as site initiation, patient screening, enrollment, delivery of reports, and other events. In accruing for these activities, we obtain information from various sources and estimate the level of effort or expense allocated to each period. Adjustments to our research and development expenses may be necessary in future periods as our estimates change.

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Results of Operations

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

	Three Months Ended September 30,	
	2018	2017
	(in thousands)	
Revenue	$944	$1,631
Research and development expenses	(7,399)	(5,018)
General and administrative expenses	(2,696)	(2,502)
Other income, net	140	55
Net loss	$(9,011)	$(5,834)

Revenue

Revenue decreased to $0.9 million during the three months ended September 30, 2018, from $1.6 million during the three months ended September 30, 2017. The $0.7 million decrease was primarily due to a $0.7 million decrease in research and development and intellectual property activities reimbursable to us by Servier under the Servier Collaboration Agreement.

Research and Development Expenses

Research and development expenses were $7.4 million during the three months ended September 30, 2018, compared to $5.0 million during the three months ended September 30, 2017. The $2.4 million increase in research and development expense was driven primarily by:

- increased clinical development and related outsourced manufacturing expenses of $2.1 million, primarily related to expenses incurred in connection with start up activities for the Phase 2 SOLAR clinical trial of cobomarsen, including costs to manufacturing cobomarsen, during the third quarter of 2018; and

- increased personnel-related costs of $0.3 million, including share-based compensation, due primarily to the growth of our research and development team.

General and Administrative Expenses

General and administrative expenses were $2.7 million during the three months ended September 30, 2018, compared to $2.5 million during the three months ended September 30, 2017. The increase in general and administrative expenses of $0.2 million was driven primarily by:

- an increase in personnel-related costs, including share-based compensation, of $0.4 million; partially offset by

- a decrease of $0.2 million in legal expenses related to intellectual property during the third quarter of 2018.

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

	Nine Months Ended September 30,	
	2018	2017
	(in thousands)	

Revenue	$7,910	$2,811
Research and development expenses	(22,187)	(14,625)
General and administrative expenses	(8,354)	(8,364)
Other income, net	245	52
Net loss	$(22,386)	$(20,126)

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Revenue

Revenue increased to $7.9 million during the nine months ended September 30, 2018, from $2.8 million during the nine months ended September 30, 2017. The increase was due primarily to a €3.0 million (or $3.7 million) milestone payment earned and received under the Servier Collaboration Agreement during the nine months ended September 30, 2018, as well as a $1.3 million increase in research and development and intellectual property activities reimbursable to us by Servier under the Servier Collaboration Agreement.

Research and Development Expenses

Research and development expenses were $22.2 million during the nine months ended September 30, 2018, compared to $14.6 million during the nine months ended September 30, 2017. The increase in research and development expense of $7.6 million was driven primarily by:

- increased clinical development and related manufacturing expenses of $4.9 million, primarily related to expenses incurred in connection with start up activities, including manufacturing costs, for the Phase 2 SOLAR clinical trial of cobomarsen and the initiation of a Phase 1 clinical trial of MRG-110 during of 2018;

- increased personnel-related costs of $1.7 million, including share-based compensation, due primarily to the growth of our research and development team; and

- increased technology license fees of $0.9 million primarily related to a milestone payment due to one of our licensors for the initiation of our first clinical trial for MRG-110 during 2018.

General and Administrative Expenses

General and administrative expenses were $8.4 million during each of the nine months ended September 30, 2018 and 2017. During 2018, legal expenses decreased by $0.9 million primarily related to the Merger that occurred in 2017. This decrease was offset by an increase in personnel-related and recruiting costs of $0.9 million, due primarily to the growth of our general and administrative team and increased share-based compensation charges.

Liquidity and Capital Resources

We have no products approved for commercial sale and have not generated any revenue from product sales. We have funded our operations to date principally through proceeds from equity financings of $165.2 million (including notes payable that previously converted to equity).

In February 2018, we entered into an underwriting agreement, or the Underwriting Agreement, with Jefferies LLC, Evercore Group L.L.C., and Deutsche Bank Securities Inc., as representatives of several underwriters, or the Underwriters, relating to a public offering of our common stock. In this offering, we sold 7,414,996 shares of common stock at a price of $5.50 per share, which resulted in net proceeds of approximately $37.9 million after deducting underwriting commissions and discounts and other offering expenses.

In March 2017, we entered into an at the market issuance Common Stock Sales Agreement, or the ATM Agreement, with Cowen and Company, LLC, or Cowen, under which we may offer and sell, from time to time, at our sole discretion, shares of our common stock having an aggregate offering price of up to $50.0 million through Cowen as our sales agent. Through September 30, 2018, we sold an aggregate of 1,213,386 shares of our common stock for aggregate gross proceeds of approximately $10.6 million. Cumulative net proceeds received through September 30, 2018 were approximately $10.2 million, including commissions to Cowen as sales agent and initial expenses for

executing the “at the market offering”.

In August 2018, we and LLS entered into a Common Stock Purchase Agreement, or the LLS Stock Purchase Agreement, for the sale of up to $5.0 million of shares of our common stock to LLS in a private placement, or the Offering. Under the terms of the LLS Stock Purchase Agreement, we expect to raise up to approximately $5.0 million in gross proceeds by selling shares of our common stock to LLS in up to five separate closings. At the initial closing in August 2018, we issued 150,987 shares of our common stock at a price per share equal to $6.62, which resulted in net proceeds of approximately $0.9 million after expenses incurred in connection with the Offering. The price per share of our common stock to be sold in any subsequent closing will be equal to the average of the volume weighted-average prices of a share of our common stock on the Nasdaq Capital Market for the three trading days beginning with the first trading day after the date of achievement of the relevant milestone for each such closing. Each closing is subject to our achievement of specified operational milestones under the LLS Stock Purchase

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Agreement and other customary closing conditions, provided, however, that each such closing must be completed prior to December 31, 2021.

Since our inception and through September 30, 2018, we have generated cumulative losses of $116.0 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We will continue to require substantial additional capital to continue our clinical development and potential commercialization activities. Accordingly, we will need substantial additional capital to continue to fund our operations. The amount and timing of future funding requirements will depend on many factors, including the pace and results of our clinical development efforts, continued performance under our Servier Collaboration Agreement, securing additional partnerships and collaborations, and issuing debt or other financing vehicles. Our ability to secure capital is dependent upon a number of factors, including success in developing our technology and drug product candidates. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop our product candidates. Changing circumstances may cause us to consume capital significantly faster or slower than we currently anticipate. If we are unable to acquire additional capital or resources, we will be required to modify our operational plans to complete future milestones. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available financial resources sooner than we currently anticipate. We may be forced to reduce our operating expenses and raise additional funds to meet our working capital needs, principally through the additional sales of our securities or debt financings or entering into strategic collaborations.

We expect to incur significant expenses and increased operating losses for at least the next several years as we continue the clinical development of, and seek regulatory approval for, our product candidates and add personnel necessary to operate as a public company with an advanced clinical candidate pipeline of product candidates. In addition, operating as a publicly-traded company involves the hiring of additional financial and other personnel, upgrading financial information systems, and incurring costs associated with operating as a public company. We expect that our operating losses will fluctuate significantly from quarter to quarter and year to year due to timing of clinical development programs and efforts to achieve regulatory approval.

If we raise additional funds through the issuance of debt, the obligations related to such debt could be senior to rights of holders of our capital stock and could contain covenants that may restrict our operations. Should additional capital not be available to us in the near term, or not be available on acceptable terms, we may be unable to realize value from our assets and discharge our liabilities in the normal course of business, which may, among other alternatives, cause us to further delay, substantially reduce, or discontinue operational activities to conserve our cash resources.

As of September 30, 2018, we had cash and cash equivalents of $31.7 million and short-term investments of $38.8 million. We believe our current resources will be sufficient to fund our operations in the normal course of business and allow us to meet our liquidity needs into early 2020.

Summarized cash flows for the nine months ended September 30, 2018 and 2017 are as follows:

	Nine Months Ended September 30,		
	2018	2017	Change
	(in thousands)		
Net cash used in operating activities	$(18,669)	$(20,924)	$2,255
Net cash provided by (used in) investing activities	(38,998)	1,068	(40,066)
Net cash provided by financing activities	41,926	40,557	1,369
Net increase (decrease) in cash and cash equivalents	$(15,741)	$20,701	$(36,442)

Operating Activities

Net cash used in operating activities was $18.7 million for the nine months ended September 30, 2018, compared to $20.9 million for the nine months ended September 30, 2017. The decrease was primarily the result of a $3.7 million decrease in payments of associated current liabilities and receipts associated with accounts receivable during the nine months ended September 30, 2018, as well as a $2.3 million increase in net loss offset by a $1.0 million increase in share-based compensation.

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Investing Activities

Net cash used in investing activities was $39.0 million during the nine months ended September 30, 2018 compared to net cash provided by investing activities of $1.1 million during the nine months ended September 30, 2017. The change in cash flow from investing activities was driven primarily by $38.6 million of cash and cash equivalents invested in short-term investments in 2018, and by $1.3 million of cash acquired in 2017 as a result of the Merger in 2017.

Financing Activities

Net cash provided by financing activities was $41.9 million for the nine months ended September 30, 2018, compared to $40.6 million during the nine months ended September 30, 2017. The increase in cash provided by financing activities was primarily driven by net proceeds associated with the sale of our common stock pursuant to the ATM Agreement of $2.7 million and the issuance of our common stock to LLS of $0.9 million during the nine months ended September 30, 2018, offset by higher net proceeds from the sale of common stock in the 2017 private financing as compared to our public offering in 2018.

Contractual Obligations and Commitments

As of September 30, 2018, we had no material commitments other than the liabilities reflected and commitments disclosed in our condensed consolidated financial statements.

Off-Balance Sheet Arrangements

We have not entered into any off-balance sheet arrangements and do not have any holdings in variable interest entities.

Implications of Being an Emerging Growth Company

In April 2012, the Jumpstart Our Business Startups Act, or the JOBS Act, was enacted. Section 107 of the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards.

We are an “emerging growth company,” as defined in the JOBS Act. We will remain an emerging growth company until the earlier of (1) the end of the first fiscal year following the fifth anniversary of our initial public offering, or December 31, 2019, (2) the beginning of the first fiscal year after our annual gross revenue is $1.07 billion or more, (3) the date on which we have, during the previous three-year period, issued more than $1.07 billion in non-convertible debt securities and (4) as of the end of any fiscal year in which the market value of our common stock held by non-affiliates exceeded $700 million as of the end of the second quarter of that fiscal year.

For as long as we remain an “emerging growth company,” we may take advantage of certain exemptions from various reporting requirements that are applicable to public companies that are not “emerging growth companies” including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation and financial statements in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote to approve executive compensation and shareholder approval of any golden parachute payments not previously approved. We have elected to avail ourselves of the extended transition period for adopting new or revised accounting standards. As a result of this election, our financial statements may not be comparable to companies that comply with public company effective dates.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Not applicable.

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ITEM 4. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file under the Securities Exchange Act of 1934, as amended, or the Exchange Act, is recorded, processed, summarized, and reported within the time periods specified in the rules and forms of the SEC, and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosures. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

As required by Rule 13a-15(b) and Rule 15d-15(b) of the Exchange Act, an evaluation was carried out under the supervision and with the participation of management, including our principal executive officer and principal financial officer, of the effectiveness of our disclosure controls and procedures (as defined in Exchange Act Rule 13a-15(e) and 15d-15(e)) as of the end of the quarter covered by this Quarterly Report. Based on the foregoing, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were effective at a reasonable level of assurance.

Changes in Internal Control Over Financial Reporting

There have been no changes in our internal control over financial reporting that occurred during our most recent fiscal quarter that have materially affected, or are reasonably likely to materially effect, our internal control over financial reporting.

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PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

Not applicable.

ITEM 1A. RISK FACTORS

Our business, financial condition, and operating results may be affected by a number of factors, whether currently known or unknown, including but not limited to those described below. Any one or more of such factors could directly or indirectly cause our actual results of operations and financial condition to vary materially from past or anticipated future results of operations and financial condition. Any of these factors, in whole or in part, could materially and adversely affect our business, financial condition, results of operations, and stock price. The following information should be read in conjunction with Part I, Item 2, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and the condensed consolidated financial statements and related notes in Part I, Item 1, “Financial Information” of this Quarterly Report.

Risks Related to Our Financial Condition and Capital Requirements

We have incurred losses since our inception, have a limited operating history on which to assess our business, and anticipate that we will continue to incur significant losses for the foreseeable future.

We are a clinical-stage biopharmaceutical company with a limited operating history. We have incurred net losses in each year since Private Miragen’s inception in 2006. During the nine months ended September 30, 2018 and 2017, net loss was $22.4 million and $20.1 million, respectively. As of September 30, 2018, we had an accumulated deficit of $116.0 million.

As of September 30, 2018, we had cash and cash equivalents of $31.7 million and short-term investments of $38.8 million. In February 2017, we received $40.7 million in financing through a common stock private placement. In March 2017, we entered into the ATM Agreement with Cowen, under which we may offer and sell, from time to time, at our sole discretion, shares of our common stock having an aggregate offering price of up to $50.0 million through Cowen as our sales agent. Through September 30, 2018, we had sold, pursuant to the terms of the ATM Agreement, 1,213,386 shares of our common stock, at a weighted average price of $8.74 per share, for aggregate gross proceeds of approximately $10.6 million. Net proceeds through September 30, 2018 were approximately $10.2 million, including initial expenses for executing the “at the market offering” and commissions to Cowen as sales agent. In February 2018, we entered into the Underwriting Agreement with the Underwriters relating to our public offering. Pursuant to the Underwriting Agreement, in February 2018 we sold 7,414,996 shares of our common stock at a price of $5.50 per share, which resulted in net proceeds of approximately $37.9 million after deducting underwriting commissions and discounts and other offering expenses payable by us. In August 2018, we entered into the LLS Stock Purchase Agreement with LLS, for the sale of up to $5.0 million of shares of our common stock to LLS in the Offering. Under the terms of the LLS Stock Purchase Agreement, we expect to raise approximately $5.0 million in gross proceeds by selling shares of our common stock in five separate closings to LLS. The initial closing of the Offering was held on August 6, 2018. At the initial closing, we issued 150,987 shares of our common stock at a price per share equal to $6.62, which resulted in net proceeds of approximately $0.9 million after expenses incurred in connection with the Offering. We believe that we have sufficient capital to fund our operations in the normal course of business and to meet our liquidity needs into early 2020.

We will continue to require substantial additional capital to continue our clinical development and potential commercialization activities. Accordingly, we will need to raise substantial additional capital to continue to fund our

operations. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our clinical development efforts. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop our product candidates. Changing circumstances may cause us to consume capital significantly faster or slower than we currently anticipate. If we are unable to acquire additional capital or resources, we will be required to modify our operational plans to complete future milestones. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available financial resources sooner than we currently anticipate. We may be forced to reduce our operating expenses and raise additional funds to meet our working capital needs, principally through the additional sales of our securities or debt financings or entering into strategic collaborations.

We have devoted substantially all of our financial resources to identify, acquire, and develop our product candidates, including conducting clinical trials and providing general and administrative support for our operations. To date, we have

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financed our operations primarily through the sale of equity securities and convertible promissory notes. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations, or grants. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We expect our losses to increase as our product candidates enter more advanced clinical trials. We have not yet commenced pivotal clinical trials for any product candidate and it may be several years, if ever, before we complete pivotal clinical trials or have a product candidate approved for commercialization. We expect to invest significant funds into the research and development of our current product candidates to determine the potential to advance these product candidates to regulatory approval.

If we obtain regulatory approval to market a product candidate, our future revenue will depend upon the size of any markets in which our product candidates may receive approval, and our ability to achieve sufficient market acceptance, pricing, reimbursement from third-party payors, and adequate market share for our product candidates in those markets. Even if we obtain adequate market share for our product candidates, because the potential markets in which our product candidates may ultimately receive regulatory approval could be very small, we may never become profitable despite obtaining such market share and acceptance of our products.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future and our expenses will increase substantially if and as we:

- continue the clinical development of our product candidates;
- continue efforts to discover and develop new product candidates;
- undertake the manufacturing of our product candidates or increase volumes manufactured by third parties;
- advance our programs into larger, more expensive clinical trials;
- initiate additional preclinical, clinical, or other trials or studies for our product candidates;
- seek regulatory and marketing approvals and reimbursement for our product candidates;
- establish a sales, marketing, and distribution infrastructure to commercialize any products for which we may obtain marketing approval and market for ourselves;
- seek to identify, assess, acquire, and/or develop other product candidates;
- make milestone, royalty, or other payments under third-party license agreements;
- seek to maintain, protect, and expand our intellectual property portfolio;
- seek to attract and retain skilled personnel; and
- experience any delays or encounter issues with the development and potential for regulatory approval of our clinical candidates such as safety issues, manufacturing delays, clinical trial accrual delays, longer follow-up for planned studies, additional major studies, or supportive studies necessary to support marketing approval.

Further, the net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

We have never generated any revenue from product sales and may never be profitable.

We have no products approved for commercialization and have never generated any revenue from product sales. Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaborators, to successfully complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize one or more of our product candidates. We do not anticipate generating revenue from product sales for the foreseeable future. Our ability to generate future revenue from product sales depends heavily on our success in many areas, including but not limited to:

- completing research and development of our product candidates;

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- obtaining regulatory and marketing approvals for our product candidates;
- manufacturing product candidates and establishing and maintaining supply and manufacturing relationships with third parties that are commercially feasible, meet regulatory requirements and our supply needs in sufficient quantities to meet market demand for our product candidates, if approved;
- marketing, launching, and commercializing product candidates for which we obtain regulatory and marketing approval, either directly or with a collaborator or distributor;
- gaining market acceptance of our product candidates as treatment options;
- addressing any competing products;
- protecting and enforcing our intellectual property rights, including patents, trade secrets, and know-how;
- negotiating favorable terms in any collaboration, licensing, or other arrangements into which we may enter;
- obtaining reimbursement or pricing for our product candidates that supports profitability; and
- attracting, hiring, and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Portions of our current pipeline of product candidates have been in-licensed from third parties, which make the commercial sale of such in-licensed products potentially subject to additional royalty and milestone payments to such third parties. We will also have to develop or acquire manufacturing capabilities or continue to contract with contract manufacturers in order to continue development and potential commercialization of our product candidates. For instance, our current costs of manufacturing our drug product are not commercially feasible and we will need to develop or procure our drug product in a commercially feasible manner in order to successfully commercialize any future approved product, if any. Additionally, if we are not able to generate revenue from the sale of any approved products, we may never become profitable.

Raising additional capital may cause dilution to our stockholders, restrict our operations, or require us to relinquish rights.

To the extent that we raise additional capital through the sale of equity, including pursuant to any sales under the ATM Agreement, the LLS Stock Purchase Agreement, convertible debt or other securities convertible into equity, the ownership interest of our stockholders will be diluted, and the terms of these new securities may include liquidation or other preferences that adversely affect rights of our stockholders. For instance, through September 30, 2018, we had sold, pursuant to the terms of the ATM Agreement, 1,213,386 shares of our common stock for aggregate gross proceeds of approximately $10.6 million, and, in August 2018, we sold an additional 150,987 shares of common stock to LLS under the LLS Stock Purchase Agreement for gross proceeds of $1.0 million. We anticipate that we will continue to make sales of our common stock under the ATM Agreement and the LLS Stock Purchase Agreement from time to time into the foreseeable future, and we may sell shares of our common stock of up to $50.0 million and $5.0 million in aggregate value under the ATM Agreement and the LLS Stock Purchase Agreement, respectively. Sales under the ATM Agreement or the LLS Stock Purchase Agreement dilute the ownership interest of our stockholders and may cause the price per share of our common stock to decrease. Debt financing, if available, would likely involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, making additional product acquisitions, or declaring dividends. For

instance, our loan and security agreement with Silicon Valley Bank limits our ability to enter into an asset sale, enter into any change of control, incur additional indebtedness, pay any dividends, or enter into specified transactions with our affiliates. If we raise additional funds through strategic collaborations or licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates or future revenue streams or grant licenses on terms that are not favorable to us. We cannot be assured that we will be able to obtain additional funding if and when necessary to fund our entire portfolio of product candidates to meet our projected plans. If we are unable to obtain funding on a timely basis, we may be required to delay or discontinue one or more of our development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on potential business opportunities, which could materially harm our business, financial condition, and results of operations.

We have also historically received funds from state and federal government grants for research and development. The grants have been, and any future government grants and contracts we may receive may be, subject to the risks and contingencies set forth below under the risk factor titled “Reliance on government funding for our programs may add uncertainty to our

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research and commercialization efforts with respect to those programs that are tied to such funding and may impose requirements that limit our ability to take specified actions, increase the costs of commercialization and production of product candidates developed under those programs and subject us to potential financial penalties, which could materially and adversely affect our business, financial condition, and results of operations." Although we might apply for government contracts and grants in the future, we cannot be certain that we will be successful in obtaining additional grants for any product candidates or programs.

Risks Related to the Development of Our Product Candidates

Clinical trials are costly, time consuming, and inherently risky, and we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Clinical development is expensive, time consuming, and involves significant risk. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of development. Events that may prevent successful or timely completion of clinical development include but are not limited to:

- inability to generate satisfactory preclinical, toxicology, or other in vivo or in vitro data or diagnostics to support the initiation or continuation of clinical trials;
- delays in reaching agreement on acceptable terms with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- delays in obtaining required approvals from institutional review boards or independent ethics committees at each clinical trial site;
- failure to permit the conduct of a clinical trial by regulatory authorities;
- delays in recruiting eligible patients in our clinical trials;
- failure by clinical sites or CROs or other third parties to adhere to clinical trial requirements;
- failure by our clinical sites, CROs or other third parties to perform in accordance with the good clinical practices requirements of the FDA or applicable foreign regulatory guidelines;
- patients dropping out of our clinical trials;
- adverse events or tolerability or animal toxicology issues significant enough for the FDA or other regulatory agencies to put any or all clinical trials on hold;
- occurrence of adverse events associated with our product candidates;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- the cost of clinical trials of our product candidates;
- negative or inconclusive results from our clinical trials, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon development programs in other ongoing or planned indications for a product candidate; and

- delays in reaching agreement on acceptable terms with third-party manufacturers and the time for manufacture of sufficient quantities of our product candidates for use in clinical trials.

Any inability to successfully complete clinical development and obtain regulatory approval for our product candidates could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional preclinical trials and the results obtained from such new formulation may not be consistent with previous results obtained. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow competitors to develop and bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and

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results of operations.

The approach we are taking to discover and develop novel therapeutics that target microRNAs is unproven and may never lead to marketable products.

The scientific discoveries that form the basis for our efforts to discover and develop our product candidates are relatively recent. To date, neither we nor any other company has received regulatory approval to market therapeutics utilizing microRNA-targeted molecules. The scientific evidence to support the feasibility of developing drugs based on these discoveries is both preliminary and limited. Successful development of microRNA-targeted therapeutic products by us will require solving a number of issues, including providing suitable methods of stabilizing the therapeutic product and delivering it into target cells in the human body. In addition, any product candidates that we develop may not demonstrate in patients the chemical and pharmacological properties ascribed to them in laboratory and preclinical trials, and they may interact with human biological systems in unforeseen, ineffective, or even harmful ways. For instance, our clinical and preclinical data to date has not been fully validated and we have no way of knowing if, after validation, our clinical trial data will be complete and consistent. If we do not successfully develop and commercialize product candidates based upon this technological approach, we may not become profitable and the value of our capital stock may decline.

Further, our focus on microRNA technology for developing product candidates as opposed to multiple, more proven technologies for drug development, increases the risk associated with our business. If we are not successful in developing an approved product using microRNA technology, we may not be able to identify and successfully implement an alternative product development strategy. In addition, work by other companies pursuing similar technologies may encounter setbacks and difficulties that regulators and investors may attribute to our product candidates, whether appropriately or not.

Our microRNA-targeted therapeutic product candidates are based on a relatively novel technology, which makes it unusually difficult to predict the time and cost of development and the time and cost, or likelihood, of subsequently obtaining regulatory approval. To date, no microRNA-targeted therapeutics have been approved for marketing in the United States.

We have concentrated our research and development efforts to date on a limited number of product candidates based on our microRNA-targeted therapeutic platform and identifying our initial targeted disease indications. Our future success depends on our successful development of viable product candidates. Only three of our product candidates, cobomarsen, remlarsen, and MRG-110, are in clinical development, and the remainder of our product candidates are in preclinical development. There can be no assurance that we will not experience problems or delays in developing our product candidates and that such problems or delays will not cause unanticipated costs, or that any such development problems can be solved.

Additionally, the FDA, the European Medicines Agency, and other regulatory authorities, have relatively limited experience with microRNA-targeted therapeutics. No regulatory authority has granted approval to anyone, including us, to market or commercialize microRNA-targeted therapeutics, which may increase the complexity, uncertainty, and length of the regulatory review and approval process for our product candidates. If our product candidates fail to prove to be safe and effective, and commercially viable, our product candidate pipeline would have little, if any, value, which would have a material adverse effect on our business, financial condition, or results of operations.

The clinical trial, product approval, and manufacturing requirements of the FDA, the European Medicines Agency, and other regulatory authorities, and the criteria these regulators use to evaluate the safety and efficacy of a product candidate, vary substantially according to the type, complexity, novelty, and intended use of the product candidate. The regulatory review and approval process for novel product candidates such as microRNA-targeted therapeutics can

be more expensive and take longer than for other, better known or more extensively studied product candidates. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or the European Union or from other countries or regions of the world, or how long it will take to commercialize our product candidates, even if approved for marketing. Approvals by one regulatory agency may not be indicative of the likelihood of approval by other regulatory bodies. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product candidate to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations, and prospects may be harmed.

We may not be able to develop or identify a technology that can effectively deliver our product candidates to the intended diseased cells or tissues, and any failure in such delivery technology could adversely affect and delay the development of any or all of our other product candidates.

In connection with our clinical trials of cobomarsen, remlarsen, and MRG-110, we have used various routes of administration,

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including intravenous, intralesional, subcutaneous, and intradermal injections. We cannot be certain that these routes of administration will be capable of delivering adequate levels of cobomarsen, remlarsen, MRG-110, or our other product candidates to produce a therapeutic response for any indication. While we are continuing to evaluate the use of subcutaneous, intravenous, and intradermal injections in different indications, and additional delivery technologies and routes of administration that might enable us to target specific cells with our product candidates, we cannot be certain whether we will be successful in developing effective routes of delivery. Our failure to effectively deliver any of our product candidates to the target diseased cells or tissues could adversely affect and delay the development of our product candidates.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial viability of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay, or terminate clinical trials. They additionally may result in a delay of regulatory approval by the FDA or comparable foreign authorities, or, even in the instance that an affected product candidate is approved, may result in a restrictive drug label.

Our cobomarsen, remlarsen, and MRG-110 product candidates have been studied in only a limited number of patients with a confirmed diagnosis or healthy volunteers. The most common adverse events of any grade were injection site reactions, including pain, itchiness, redness, and swelling when compounds were delivered intradermally or subcutaneously. We may experience a higher rate or severity of adverse events and comparable or higher rates of discontinuation of trial participants in our future clinical trials. There is no guarantee that additional or more severe side effects will not be identified during ongoing or future clinical trials of our product candidates for current and other indications. Undesirable side effects and negative results for other indications may negatively impact the development and potential for approval of our product candidates for their proposed indications.

Additionally, even if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may withdraw approvals of such products;
- regulatory authorities may require additional warnings on the drug label;
- we may be required to create a Risk Evaluation and Mitigation Strategy, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of a product candidate, even if approved, and could significantly harm our business, results of operations, and prospects.

Our product development program may not uncover all possible adverse events that patients who take our product candidates may experience. The number of subjects exposed to our product candidates and the average exposure time in the clinical development program may be inadequate to detect rare adverse events that may only be detected once

the product is administered to more patients and for greater periods of time.

Clinical trials by their nature utilize a sample of the potential patient population. However, with a limited number of subjects and limited duration of exposure, we cannot be fully assured that rare and severe side effects of our product candidates will be uncovered. Such rare and severe side effects may only be uncovered with a significantly larger number of patients exposed to the drug. If such safety problems occur or are identified after our product candidates reach the market, the FDA may require that we amend the labeling of the product or recall the product or may even withdraw approval for the product.

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Our microRNA-targeted therapeutic approach is novel. Negative public opinion and increased regulatory scrutiny of microRNA or other nucleic acid-based therapies may damage public perception of the safety of our product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.

MicroRNA therapy remains a novel technology, with no microRNA-targeted therapeutic product approved to date in the United States. Public perception may be influenced by claims that microRNA therapy is unsafe, and microRNA therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of the diseases targeted by our product candidates, prescribing therapies that involve the use of our product candidates in lieu of, or in addition to, existing therapies with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion regarding microRNA or other nucleic acid-based therapeutics could have an adverse effect on our business, financial condition, or results of operations and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. Serious adverse events in microRNA clinical trials for our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved, and a decrease in demand for any such product candidates. For instance, in June 2016, the FDA placed a regulatory hold on the clinical trial of a microRNA- or nucleic acid-focused biopharmaceutical company with a microRNA-targeted product candidate for the treatment of hepatitis C virus due to serious adverse events in that trial. Another microRNA-focused biopharmaceutical company also voluntarily halted an ongoing Phase 1 clinical trial for a microRNA-targeted therapy for multiple cancers in September 2016 due to multiple immune-related severe adverse events. We cannot predict what effect, if any, these clinical holds will have on the government and public perception of our product candidates.

We are heavily dependent on the success of our product candidates, which are in the early stages of clinical development. Some of our product candidates have produced results only in nonclinical settings, or for other indications than those for which we contemplate conducting development and seeking FDA approval, and we cannot give any assurance that we will generate data for any of our product candidates sufficiently supportive to receive regulatory approval in our planned indications, which will be required before they can be commercialized.

We have invested substantially all of our effort and financial resources to identify, acquire, and develop our portfolio of product candidates. Our future success is dependent on our ability to successfully further develop, obtain regulatory approval for, and commercialize one or more product candidates. We currently generate no revenue from sales of any products, and we may never be able to develop or commercialize a product candidate.

We currently have three product candidates in clinical trials. Of these product candidates, cobomarsen has been predominantly administered in patients with the mycosis fungoides form of CTCL, or MF. This is only one of the multiple indications for which we plan to develop this product candidate. Additionally, our clinical and preclinical data to date is not validated, and we have no way of knowing if after validation our clinical trial data will be complete and consistent. There can be no assurance that the data that we develop for our product candidates in our planned indications will be sufficiently supportive to obtain regulatory approval.

Based on discussions with the FDA, we believe the results from the SOLAR clinical trial could potentially allow us to apply for accelerated approval in the United States. We cannot guarantee that the outcome of this Phase 2 clinical trial will be sufficient to support, or if the FDA will grant us, accelerated approval of cobomarsen. If our data is not supportive of accelerated approval of cobomarsen, we cannot predict when, if ever, we will be able to seek approval of cobomarsen.

In addition, none of our product candidates have advanced into a pivotal clinical trial for our proposed indications, and it may be years before any such clinical trial is initiated and completed, if at all. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. We cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

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

Clinical testing is expensive and generally takes many years to complete, and the outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. Additionally, microRNAs are a new class of drug target and as such may

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have some potentially unknown risks from both an efficacy and safety perspective. The results of preclinical trials and early clinical trials of our product candidates may not be predictive of the results of larger, later-stage controlled clinical trials. Product candidates that have shown promising results in early-stage clinical trials may still suffer significant setbacks in subsequent clinical trials. Our clinical trials to date have been conducted on a small number of patients or healthy volunteers in limited numbers of clinical sites for a limited number of indications. We will have to conduct larger, well-controlled trials in our proposed indications to verify the results obtained to date and to support any regulatory submissions for further clinical development. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles despite promising results in earlier, smaller clinical trials. For instance, in June 2016, the FDA placed a regulatory hold on the clinical trial of a microRNA-focused biopharmaceutical company with a microRNA product candidate for the treatment of hepatitis C virus due to serious adverse events in that trial. Another microRNA-focused biopharmaceutical company also voluntarily halted an ongoing Phase 1 clinical trial for a microRNA therapy for multiple cancers in September 2016 due to multiple immune-related severe adverse events. Moreover, clinical data are often susceptible to varying interpretations and analyses. We do not know whether any Phase 2, Phase 3, or other clinical trials we are conducting or may conduct will demonstrate consistent or adequate efficacy and safety with respect to the proposed indication for use sufficient to receive regulatory approval or market our drug candidates.

We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and human resources, we may forego or delay pursuit of opportunities with some programs or product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or more profitable market opportunities. Our spending on current and future research and development programs and future product candidates for specific indications may not yield any commercially viable products. We may also enter into additional strategic collaboration agreements to develop and commercialize some of our programs and potential product candidates in indications with potentially large commercial markets. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaborations, licensing, or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

We may find it difficult to enroll patients in our clinical trials given the limited number of patients who have the diseases for which our product candidates are being studied. Difficulty in enrolling patients could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is essential to our success. The timing of our clinical trials depends in part on the rate at which we can recruit patients to participate in clinical trials of our product candidates, and we may experience delays in our clinical trials if we encounter difficulties in enrollment.

The eligibility criteria of our planned clinical trials may further limit the available eligible trial participants as we expect to require that patients have specific characteristics that we can measure or meet the criteria to assure their conditions are appropriate for inclusion in our clinical trials. For instance, our Phase 1 clinical trial of cobomarsen included patients with MF. The estimated prevalence of MF is 16,000 to 20,000 cases in the United States and only a subset of this group satisfied the enrollment criteria for our cobomarsen clinical trial. We may not be able to identify, recruit, and enroll a sufficient number of patients to complete our clinical trials in a timely manner because of the

perceived risks and benefits of the product candidate under study, the availability and efficacy of competing therapies and clinical trials, and the willingness of physicians to participate in our planned clinical trials. If patients are unwilling to participate in our clinical trials for any reason, the timeline for conducting trials and obtaining regulatory approval of our product candidates may be delayed.

If we experience delays in the completion of, or termination of, any clinical trials of our product candidates, the commercial prospects of our product candidates could be harmed, and our ability to generate product revenue from any of these product candidates could be delayed or prevented. In addition, any delays in completing our clinical trials would likely increase our overall costs, impair product candidate development, and jeopardize our ability to obtain regulatory approval relative to our current plans. Any of these occurrences may harm our business, financial condition, and prospects significantly.

We may face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use or misuse of our approved products, if any, or product candidates harm patients, or is perceived to harm patients even when such harm is unrelated to our approved products, if any, or product candidates, our regulatory

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approvals, if any, could be revoked or otherwise negatively impacted, and we could be subject to costly and damaging product liability claims. If we are unable to obtain adequate insurance or are required to pay for liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage, a material liability claim could adversely affect our financial condition.

The use or misuse of our product candidates in clinical trials and the sale of any products for which we may obtain marketing approval exposes us to the risk of potential product liability claims. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. Some of our microRNA-targeted therapeutics have shown adverse events in clinical trials, including injection site reactions and pain at the injection site, erythema, nausea, diarrhea, decreased white blood cell and platelet counts, neutropenia, elevated aspartate aminotransferase, alanine aminotransferase, uric acid, and creatine kinase levels, prolonged partial thromboplastin time, blurred vision, itchiness, fatigue, headache, and microscopic hematuria, among others. In almost all cases, these events were mild to moderate and self-limited. There is a risk that our future product candidates may induce similar or more severe adverse events. Patients with the diseases targeted by our product candidates may already be in severe and advanced stages of disease and have both known and unknown significant preexisting and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may or may not be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact, or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which an adverse event is unrelated to our product candidates, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may delay our regulatory approval process or impact and limit the type of regulatory approvals our product candidates receive or maintain.

As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition, or results of operations.

Although we have product liability insurance, which covers our clinical trials in the United States, for up to $5.0 million per occurrence, up to an aggregate limit of $5.0 million, our insurance may be insufficient to reimburse us for any expenses or losses we may suffer. We will also likely be required to increase our product liability insurance coverage for the advanced clinical trials that we plan to initiate. If we obtain marketing approval for any of our product candidates, we will need to expand our insurance coverage to include the sale of commercial products. There is no way to know if we will be able to continue to obtain product liability coverage and obtain expanded coverage, if we require it, in sufficient amounts to protect us against losses due to liability, on acceptable terms, or at all. We may not have sufficient resources to pay for any liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage. Where we have provided indemnities in favor of third parties under our agreements with them, there is also a risk that these third parties could incur liability and bring a claim under such indemnities. An individual may bring a product liability claim against us alleging that one of our product candidates causes, or is claimed to have caused, an injury or is found to be unsuitable for consumer use. Any such product liability claims may include allegations of defects in manufacturing, defects in design, failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. Any product liability claim brought against us, with or without merit, could result in:

- withdrawal of clinical trial volunteers, investigators, patients or trial sites, or limitations on approved indications;
- the inability to commercialize, or if commercialized, decreased demand for, our product candidates;
- if commercialized, product recalls, labeling, marketing or promotional restrictions, or the need for product modification;

- initiation of investigations by regulators;
- loss of revenues;
- substantial costs of litigation, including monetary awards to patients or other claimants;
- liabilities that substantially exceed our product liability insurance, which we would then be required to pay ourselves;
- an increase in our product liability insurance rates or the inability to maintain insurance coverage in the future on acceptable terms, if at all;

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- the diversion of management's attention from our business; and

- damage to our reputation and the reputation of our products and our technology.

Product liability claims may subject us to the foregoing and other risks, which could have a material adverse effect on our business, financial condition, or results of operations.

Risks Related to Regulatory Approval of Our Product Candidates and Other Legal Compliance Matters

A potential breakthrough therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek a breakthrough therapy designation from the FDA for some of our product candidates. A breakthrough therapy is defined as a drug or biological product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and for which preliminary clinical evidence indicates that the drug or biological product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For drugs or biological products that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA could also be eligible for priority review.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review, or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one of our product candidates is designated as a breakthrough therapy, the FDA may later decide that the product candidate no longer meets the conditions for designation and the designation may be rescinded.

We may seek Fast Track designation for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not actually lead to a faster development or regulatory review or approval process.

If a product candidate is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a product sponsor may apply for FDA Fast Track designation. If we seek Fast Track designation for a product candidate, we may not receive it from the FDA. However, even if we receive Fast Track designation, Fast Track designation does not ensure that we will receive marketing approval in any particular timeframe or at all. We may not experience a faster development or regulatory review or approval process with Fast Track designation compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track designation alone does not guarantee qualification for the FDA's priority review procedures.

We may attempt to obtain accelerated approval of our product candidates. If we are unable to obtain accelerated approval, we may be required to conduct clinical trials beyond those that we contemplate, or the size and duration of our pivotal clinical trials could be greater than currently planned, which could increase the expense of obtaining, reduce the likelihood of obtaining, and/or delay the timing of obtaining necessary marketing approvals. Even if we receive accelerated approval from the FDA, the FDA may require that we conduct confirmatory trials to verify clinical

benefit. If our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-approval requirements, the FDA may seek to withdraw accelerated approval.

We may seek accelerated approval for our product candidates, including cobomarsen. The FDA may grant accelerated approval to a product designed to treat a serious or life-threatening condition that provides meaningful therapeutic advantage over available therapies and demonstrates an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease. If granted, accelerated approval may be contingent on the sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's predicted effect on irreversible morbidity or mortality or other clinical benefit. The FDA may require that any such confirmatory study be initiated or substantially underway prior to the submission of an application for accelerated approval. If

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such post-approval studies fail to confirm the drug's clinical benefits relative to its risks, the FDA may withdraw its approval of the drug. If we choose to pursue accelerated approval, there can be no assurance that the FDA will agree that our proposed primary endpoint is an appropriate surrogate endpoint. Similarly, there can be no assurance that after subsequent FDA feedback that we will continue to pursue accelerated approval or any other form of expedited development, review, or approval, even if we initially decide to do so. Furthermore, if we submit an application for accelerated approval, there can be no assurance that such application will be accepted or that approval will be granted on a timely basis, or at all. The FDA also could require us to conduct further studies or trials prior to considering our application or granting approval of any type. We might not be able to fulfill the FDA's requirements in a timely manner, which would cause delays, or approval might not be granted because our submission is deemed incomplete by the FDA. A failure to obtain accelerated approval or any other form of expedited development, review or approval for a product candidate would result in a longer time period to commercialize such product candidate, could increase the cost of development of such product candidate, and could harm our competitive position in the marketplace.

Even if we receive accelerated approval from the FDA, we will be subject to rigorous post-approval requirements, including the completion of confirmatory post-approval clinical trial(s) to verify the clinical benefit of the product, and submission to the FDA of all promotional materials prior to their dissemination. The FDA could withdraw accelerated approval for multiple reasons, including our failure to conduct any required post-approval study with due diligence, or the inability of such study to confirm the predicted clinical benefit.

A failure to obtain accelerated approval or any other form of expedited review or approval for a product candidate could result in a longer time period prior to commercializing such product candidate, increase the cost of development of such product candidate, and harm our competitive position in the marketplace.

Even if we obtain regulatory approval for a product candidate, we will remain subject to ongoing regulatory requirements.

If any of our product candidates are approved, we will be subject to ongoing regulatory requirements with respect to manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing clinical trials, and submission of safety, efficacy, and other post-approval information, including both federal and state requirements in the United States, and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to continuously comply with FDA and comparable foreign requirements, including ensuring that quality control and manufacturing procedures conform to current Good Manufacturing Practices, or cGMP, regulations and corresponding foreign regulatory manufacturing requirements. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any new drug application or marketing authorization application.

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the marketed product. We will be required to report adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities. Any new legislation could result in delays in product development or commercialization, or increased costs to assure compliance. If our original marketing approval for a product candidate was granted accelerated approval by the FDA, we could be required to conduct a successful post-marketing clinical trial in order to confirm the clinical benefit of our products. An unsuccessful post-marketing clinical trial or failure to complete such a trial could result in the withdrawal of marketing approval.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the

promotion, marketing or labeling of a product, the regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any of our ongoing clinical trials;

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- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our contract manufacturers' facilities; or
- require a product recall.

Any government investigation of alleged violations of law would be expected to require us to expend significant time and resources in response and could generate adverse publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to develop and commercialize our products, and the value of the company and our operating results would be adversely affected.

In addition, if we were able to obtain accelerated approval of any of our drug candidates, the FDA may require us to conduct a confirmatory study to verify the predicted clinical benefit. Other regulatory authorities outside of the United States, may have similar requirements. The results from the confirmatory study may not support the clinical benefit, which could result in the approval being withdrawn. While operating under accelerated approval, we will be subject to certain restrictions that we would not be subject to upon receiving regular approval.

Healthcare legislative reform measures may have a material adverse effect on our business, financial condition, or results of operations.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Affordable Care Act, or ACA, was passed, which was intended to substantially change the way healthcare is financed by both governmental and private insurers, and significantly impact the U.S. pharmaceutical industry. The ACA, among other things, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted, or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed care organizations, establishes annual fees and taxes on manufacturers of specified branded prescription drugs, and promotes a new Medicare Part D coverage gap discount program.

Since its enactment, certain aspects of the ACA have faced and continue to face Congressional, judicial, and regulatory challenges, and both Congress and President Trump have delayed implementation or effectively repealed some of the ACA's requirements through legislation, Executive Orders, failures to fund, and other actions. As a result, we cannot predict how the ACA, its possible repeal, or any legislation Congress passes to replace the ACA or any possible replacement passed by Congress will affect our business.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted, and we expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand or lower pricing for our product candidates or additional pricing pressures.

We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations will be subject to various federal and state fraud and abuse laws, including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act, and Physician Payments Sunshine Act, and

regulations. These laws may impact, among other things, our relationships with healthcare professionals and our proposed sales, marketing, and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering, or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;

- federal civil and criminal false claims laws and civil monetary penalties law, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare,

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Medicaid, or other third-party payors that are false or fraudulent;

- the Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit, among other things, executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their implementing regulations, which imposes specified obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information without the appropriate authorization, on entities subject to the law, such as healthcare providers, health plans, and healthcare clearinghouses and their respective business associates that perform services for them that involve the creation, use, maintenance, or disclosure of individually identifiable health information;

- the federal Physician Payment Sunshine Act under the ACA requires manufacturers of drugs, devices, biologics, and medical supplies to report annually to the U.S. Department of Health and Human Services information related to payments and other transfers of value to physicians, other healthcare providers, and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers, as well as their immediate family members and applicable group purchasing organizations; and

- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws that may apply to items or services reimbursed by any third-party payor, including governmental and private payors, to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, and state laws governing the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent healthcare reform legislation has strengthened these laws. For example, the ACA, among other things, amends the intent requirement of the federal Anti-Kickback Statute and criminal healthcare fraud statutes, such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate the law. Moreover, the ACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, disgorgement, damages, fines, contractual damages, reputational harm, diminished profits and future earnings, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, including imprisonment, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Reliance on government funding for our programs may add uncertainty to our research and commercialization efforts with respect to those programs that are tied to such funding and may impose requirements that limit our ability to take specified actions, increase the costs of commercialization and production of product candidates developed under those programs and subject us to potential financial penalties, which could materially and adversely affect our business, financial condition, and results of operations.

During the course of our development of our product candidates, we have been funded in part through federal and state grants, including but not limited to the funding we received from Yale University, or Yale, pursuant to a subcontract agreement with Yale. In addition to the funding we have received to date, we have applied and intend to continue to apply for federal and state grants to receive additional funding in the future. Contracts and grants funded by the U.S. government, state governments and their related agencies include provisions that reflect the government's substantial rights and remedies, many of which are not typically found in commercial contracts, including powers of the government to:

- require repayment of all or a portion of the grant proceeds, in specified cases with interest, in the event we violate specified covenants pertaining to various matters that include a failure to achieve;

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- specify milestones or terms relating to use of grant proceeds, or to comply with specified laws;
- terminate agreements, in whole or in part, for any reason or no reason;
- reduce or modify the government's obligations under such agreements without the consent of the other party;
- claim rights, including intellectual property rights, in products and data developed under such agreements;
- audit contract related costs and fees, including allocated indirect costs;
- suspend the contractor or grantee from receiving new contracts pending resolution of alleged violations of procurement laws or regulations;
- impose U.S. manufacturing requirements for products that embody inventions conceived or first reduced to practice under such agreements;
- impose qualifications for the engagement of manufacturers, suppliers, and other contractors as well as other criteria for reimbursements;
- suspend or debar the contractor or grantee from doing future business with the government;
- control and potentially prohibit the export of products;
- pursue criminal or civil remedies under the False Claims Act, False Statements Act, and similar remedy provisions specific to government agreements; and
- limit the government's financial liability to amounts appropriated by the U.S. Congress on a fiscal year basis, thereby leaving some uncertainty about the future availability of funding for a program even after we have been funded for an initial period.

In addition to those powers set forth above, the government funding we may receive could also impose requirements to make payments based upon sales of our products, if any, in the future.

We may not have the right to prohibit the U.S. government from using specified technologies developed by it, and we may not be able to prohibit third-party companies, including our competitors, from using those technologies in providing products and services to the U.S. government. The U.S. government generally takes the position that we have the right to royalty-free use of technologies that are developed under U.S. government contracts. These and other provisions of government grants may also apply to intellectual property we license now or in the future.

In addition, government contracts and grants normally contain additional requirements that may increase our costs of doing business, reduce our profits, and expose us to liability for failure to comply with these terms and conditions. These requirements include, for example:

- specialized accounting systems unique to government contracts and grants;
- mandatory financial audits and potential liability for price adjustments or recoupment of government funds after such funds have been spent;

- public disclosures of some contract and grant information, which may enable competitors to gain insights into our research program; and

- mandatory socioeconomic compliance requirements, including labor standards, non-discrimination and affirmative action programs, and environmental compliance requirements.

If we fail to maintain compliance with any such requirements that may apply to us now or in the future, we may be subject to potential liability and to termination of our contracts.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or

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penalties or incur costs that could have a material adverse effect on our business, financial condition, or results of operations.

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the controlled storage, use, and disposal of hazardous materials, including the components of our product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling, and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts, and business operations, and cause environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by us and our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources, and state or federal or other applicable authorities may curtail our use of specified materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently, and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. We do not currently carry biological or hazardous waste insurance coverage.

Failure to comply with existing or future laws and regulations related to privacy or data security could lead to government enforcement actions (which could include civil or criminal fines or penalties), private litigation, other liabilities, and/or adverse publicity. Compliance or the failure to comply with such laws could increase the costs of our products and services, could limit their use or adoption, and could otherwise negatively affect our operating results and business.

Regulation of data processing is evolving, as federal, state, and foreign governments continue to adopt new, or modify existing, laws and regulations addressing data privacy and security, and the collection, processing, storage, transfer, and use of data. We and our partners may be subject to current, new, or modified federal, state, and foreign data protection laws and regulations (i.e., laws and regulations that address privacy and data security). These new or proposed laws and regulations are subject to differing interpretations and may be inconsistent among jurisdictions, and guidance on implementation and compliance practices are often updated or otherwise revised, which adds to the complexity of processing personal data. These and other requirements could require us or our partners to incur additional costs to achieve compliance, limit our competitiveness, necessitate the acceptance of more onerous obligations in our contracts, restrict our ability to use, store, transfer, and process data, impact our or our partners' ability to process or use data in order to support the provision of our products or services, or affect our or our partners' ability to offer our products and services in certain locations.

In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), that govern the collection, use, disclosure, and protection of health-related and other personal information could apply to our operations or the operations of our partners. In addition, we may obtain health information from third parties (including research institutions from which we may obtain clinical trial data) that are subject to privacy and security requirements under HIPAA. Depending on the facts and circumstances, we could be subject to criminal penalties, including if we knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA.

International data protection laws, including, without limitation, the European Union's General Data Protection Regulation, or the GDPR, that took effect in May 2018, and member state data protection legislation, may also apply to health-related and other personal information obtained outside of the United States. These laws impose strict obligations on the ability to process health-related and other personal information of data subjects in the European Union, including in relation to use, collection, analysis, and transfer of such personal information. These laws include several requirements relating to the consent of the individuals to whom the personal data relates, limitations on data processing, establishing a legal basis for processing, notification of data processing obligations or security incidents to appropriate data protection authorities or data subjects, the security and confidentiality of the personal data and various rights that data subjects may exercise.

The GDPR prohibits the transfer of personal data, without an appropriate legal basis, to countries outside of the European Economic Area, or EEA, such as the United States, which are not considered by the European Commission to provide an adequate level of data protection. Switzerland has adopted similar restrictions. Although there are legal mechanisms to allow for the transfer of personal data from the EEA and Switzerland to the United States, uncertainty about compliance with European Union data protection laws remains. For example, ongoing legal challenges in Europe to the mechanisms allowing

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companies to transfer personal data from the EEA to the United States could result in further limitations on the ability to transfer personal data across borders, particularly if governments are unable or unwilling to reach new or maintain existing agreements that support cross-border data transfers, such as the European Union-U.S. and Swiss-U.S. Privacy Shield framework. Additionally, other countries have passed or are considering passing laws requiring local data residency.

Under the GDPR, regulators may impose substantial fines and penalties for non-compliance. Companies that violate the GDPR can face fines of up to the greater of 20 million Euros or 4% of their worldwide annual turnover (revenue). The GDPR has increased our responsibility and liability in relation to personal data that we process, requiring us to put in place additional mechanisms to ensure compliance with the GDPR and other EU and international data protection rules.

Failure to comply with U.S. and international data protection laws and regulations could result in government enforcement actions (which could include civil or criminal penalties), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, patients about whom we or our partners obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights, failed to comply with data protection laws, or breached our contractual obligations related to security or privacy, even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business. Compliance with data protection laws may be time-consuming, require additional resources and could result in increased expenses, reduce overall demand for our products and services and make it more difficult to meet expectations of or commitments to customers or partners.

Any of these matters could materially adversely affect our business, financial condition, or operational results.

Risks Related to Our Intellectual Property

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

Presently, we have rights to the intellectual property, through licenses from third parties and under patents and patent applications that we own, to modulate only a subset of the known microRNA targets. Because our programs may involve a range of microRNA targets, including targets that require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license, or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties that we identify. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources, and greater clinical development and commercialization capabilities.

For example, we have previously collaborated and may continue to collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such right of first negotiation for intellectual property, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to it. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to third-party intellectual property rights, our business, financial condition, and prospects for growth could suffer.

We intend to rely on patent rights for our product candidates and any future product candidates. If we are unable to obtain or maintain exclusivity from the combination of these approaches, we may not be able to compete effectively in our markets.

We rely or will rely upon a combination of patents, trade secret protection, and confidentiality agreements to protect the intellectual property related to our technologies and product candidates. Our success depends in large part on our and our licensors' ability to obtain regulatory exclusivity and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technologies and product candidates.

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We have sought to protect our proprietary position by filing patent applications in the United States and abroad related to our technologies and product candidates that are important to our business. This process is expensive and time consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain and involves complex legal and factual questions for which legal principles remain unsolved. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue, and even if such patents cover our product candidates, third parties may challenge their validity, enforceability, or scope, which may result in such patents being narrowed, found unenforceable, or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates, or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

We, independently or together with our licensors, have filed several patent applications covering various aspects of our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent, or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

If we cannot obtain and maintain effective protection of exclusivity from our regulatory efforts and intellectual property rights, including patent protection or data exclusivity, for our product candidates, we may not be able to compete effectively, and our business and results of operations would be harmed.

We may not have sufficient patent term protections for our product candidates to effectively protect our business.

Patents have a limited term. In the United States, the statutory expiration of a patent is generally 20 years after it is filed. Additional patent terms may be available through a patent term adjustment process, resulting from the United States Patent and Trademark Office, or USPTO, delays during prosecution. Although various extensions may be available, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from generic medications.

Patent term extensions under the Hatch-Waxman Act in the United States and under supplementary protection certificates in Europe may be available to extend the patent or data exclusivity terms of our product candidates. We will likely rely on patent term extensions, and we cannot provide any assurances that any such patent term extensions will be obtained and, if so, for how long. As a result, we may not be able to maintain exclusivity for our product candidates for an extended period after regulatory approval, if any, which would negatively impact our business, financial condition, results of operations, and prospects. If we do not have sufficient patent terms or regulatory exclusivity to protect our product candidates, our business and results of operations will be adversely affected.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products, and recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of

our patent applications and the enforcement or defense of our issued patents.

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming, and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in specified circumstances and weakened the rights of patent owners in specified situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

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The USPTO has issued subject matter eligibility guidance to patent examiners instructing USPTO examiners on the ramifications of the Supreme Court rulings in Mayo Collaborative Services v. Prometheus Laboratories, Inc. and Association for Molecular Pathology v. Myriad Genetics, Inc., and applied the Myriad ruling to natural products and principles including all naturally occurring nucleic acids. In addition, the USPTO continues to provide updates to its guidance and this is a developing area. The USPTO guidance may make it impossible for us to pursue similar patent claims in patent applications we may prosecute in the future.

Our patent portfolio contains claims of various types and scope, including chemically modified mimics, inhibitors, as well as methods of medical treatment. The presence of varying claims in our patent portfolio significantly reduces, but may not eliminate, our exposure to potential validity challenges.

For our U.S. patent applications containing a claim not entitled to priority before March 16, 2013, there is a greater level of uncertainty in the patent law. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The USPTO has promulgated regulations and developed procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, did not come into effect until March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, or results of operations.

An important change introduced by the Leahy-Smith Act is that, as of March 16, 2013, the United States transitioned to a “first-to-file” system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to either: (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our patents or patent applications.

Among some of the other changes introduced by the Leahy-Smith Act are changes that limit where a patentee may file a patent infringement suit and new procedures providing opportunities for third parties to challenge any issued patent in the USPTO. Included in these new procedures is a process known as Inter Partes Review, or IPR, which has been generally used by many third parties over the past four years to invalidate patents. The IPR process is not limited to patents filed after the Leahy-Smith Act was enacted and would therefore be available to a third party seeking to invalidate any of our U.S. patents, even those filed before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal court necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Additionally, the rights of review and appeal for IPR decisions is an area of law that is still developing.

If we are unable to maintain effective proprietary rights for our product candidates or any future product candidates, we may not be able to compete effectively in our proposed markets.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information, or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations, and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees,

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consultants, advisors, and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, or that our trade secrets and other confidential proprietary information will not be disclosed, or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business, financial condition, or results of operations. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our ability to develop, manufacture, market, and sell our product candidates and use our proprietary technology without infringing the patent rights of third parties. Numerous third-party U.S. and non-U.S. issued patents and pending applications exist in the area of microRNA. We are aware of U.S. and foreign patents and pending patent applications owned by third parties that cover therapeutic uses of microRNA replacements and inhibitors. From time to time, we may also monitor these patents and patent applications. We may in the future pursue available proceedings in the U.S. and foreign patent offices to challenge the validity of these patents and patent applications. In addition, or alternatively, we may consider whether to seek to negotiate a license of rights to technology covered by one or more of such patents and patent applications. If any patents or patent applications cover our product candidates or technologies, we may not be free to manufacture or market our product candidates, including cobomarsen, remlarsen, or MRG-110, as planned, absent such a license, which may not be available to us on commercially reasonable terms, or at all.

It is also possible that we have failed to identify relevant third-party patents or applications. For example, applications filed before November 29, 2000 remain confidential until patents issue and applications filed after that date that will not be filed outside the United States can elect to remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates and technologies because patent searching is imperfect due to differences in terminology among patents, incomplete databases, and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale, or use of a current or future product candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable, or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to specified limitations, be later amended in a manner that could cover our technologies, our product candidates, or the use of our product candidates.

There have been many lawsuits and other proceedings involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits in federal courts, and interferences, oppositions, inter partes reviews, post-grant reviews, and reexamination proceedings before the USPTO and corresponding foreign patent offices. Numerous U.S. and foreign-issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of

employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products, or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

We may not be successful in meeting our obligations under our existing license agreements necessary to maintain our product candidate licenses in effect. In addition, if required in order to commercialize our product candidates, we may be unsuccessful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to the intellectual property, through licenses from third parties and under patents that we do not own, to develop and commercialize our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to maintain in effect these proprietary rights.

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Any termination of license agreements with third parties with respect to our product candidates would be expected to negatively impact our business prospects.

We may be unable to acquire or in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources, and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. Even if we are able to license or acquire third-party intellectual property rights that are necessary for our product candidates, there can be no assurance that they will be available on favorable terms.

We collaborate with U.S. and foreign academic institutions to identify product candidates, accelerate our research, and conduct development. Typically, these institutions have provided us with an option to negotiate an exclusive license to any of the institution's rights in the patents or other intellectual property resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue a program of interest to us.

If we are unable to successfully obtain and maintain rights to required third-party intellectual property, we may have to abandon development of that product candidate or pay additional amounts to the third party, and our business and financial condition could suffer.

The patent protection and patent prosecution for some of our product candidates is dependent on third parties.

While we normally seek and gain the right to fully prosecute the patents relating to our product candidates, there may be times when patents relating to our product candidates are controlled by our licensors. For instance, this is the case with our agreement with Santaris Pharma A/S, which subsequently changed its name to Roche Innovation Center Copenhagen A/S, or RICC, which was acquired by F. Hoffmann-La Roche Ltd, or Roche, in 2014 and subsequently changed its name to RICC, who is primarily responsible for the prosecution of patents and patent applications licensed to us under the applicable agreement. If RICC or any of our future licensors fail to appropriately and broadly prosecute and maintain patent protection for patents covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected, and we may not be able to prevent competitors from making, using, importing, and selling competing products. In addition, even where we now have the right to control patent prosecution of patents and patent applications we have licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensors in effect from actions prior to us assuming control over patent prosecution.

If we fail to comply with obligations in the agreements under which we license intellectual property and other rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license and supply agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing agreements impose, and we expect that future license agreements will impose, various diligence, milestone payments, royalties, purchasing, and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, our agreements may be subject to termination by the licensor, in which event we would not be able to develop, manufacture, or market products covered by the license or subject to supply commitments.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. If we or one of our licensing partners were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description, clarity, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable.

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Interference proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to us from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market.

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

We may be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we have written agreements and make every effort to ensure that our employees, consultants, and independent contractors do not use the proprietary information or intellectual property rights of others in their work for us, we may in the future be subject to any claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop our own products and may also export infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States.

These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries, particularly some developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally.

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

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Risks Related to Our Reliance on Third Parties

We rely on third parties to conduct our clinical trials, manufacture our product candidates, and perform other services. If these third parties do not successfully perform and comply with regulatory requirements, we may not be able to successfully complete clinical development, obtain regulatory approval, or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon third-party CROs to conduct, monitor, and manage our ongoing clinical programs. We rely on these parties for execution of clinical trials, and we manage and control only some aspects of their activities. We remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs and other vendors are required to comply with all applicable laws, regulations, and guidelines, including those required by the FDA and comparable foreign regulatory authorities for all of our product candidates in clinical development. If we or any of our CROs or vendors fail to comply with applicable laws, regulations, and guidelines, the results generated in our clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot be assured that our CROs and other vendors will meet these requirements, or that upon inspection by any regulatory authority, such regulatory authority will determine that efforts, including any of our clinical trials, comply with applicable requirements. Our failure to comply with these laws, regulations, and guidelines may require us to repeat clinical trials, which would be costly and delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs in a timely manner or do so on commercially reasonable terms. In addition, our CROs may not prioritize our clinical trials relative to those of other customers, and any turnover in personnel or delays in the allocation of CRO employees by the CRO may negatively affect our clinical trials. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, our clinical trials may be delayed or terminated, and we may not be able to meet our current plans with respect to our product candidates. CROs may also involve higher costs than anticipated, which could negatively affect our financial condition and operations.

In addition, we do not currently have, nor do we currently plan to establish, the capability to manufacture product candidates for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale without the use of third-party manufacturers. We plan to rely on third-party manufacturers and their responsibilities will include purchasing from third-party suppliers the materials necessary to produce our product candidates for our clinical trials and regulatory approval. There are expected to be a limited number of suppliers for the active ingredients and other materials that we expect to use to manufacture our product candidates, and we may not be able to identify alternative suppliers to prevent a possible disruption of the manufacture of our product candidates for our clinical trials, and, if approved, ultimately for commercial sale. Although we generally do not expect to begin a clinical trial unless we believe we have a sufficient supply of a product candidate to complete the trial, any significant delay or discontinuity in the supply of a product candidate, or the active ingredient or other material components in the manufacture of the product candidate, could delay completion of our clinical trials and potential timing for regulatory approval of our product candidates, which would harm our business and results of operations.

We rely and expect to continue to rely on third parties to manufacture our clinical product supplies, and we intend to rely on third parties to produce and process our product candidates, if approved, and our commercialization of any of our product candidates could be stopped, delayed, or made less profitable if those third parties fail to obtain approval of government regulators, fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices.

We do not currently have, nor do we currently plan to develop, the infrastructure or capability internally to manufacture our clinical supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We currently rely on outside vendors to manufacture our clinical supplies of our product candidates and plan to continue relying on third parties to manufacture our product candidates on a commercial scale, if approved.

We do not yet have sufficient information to reliably estimate the cost of the commercial manufacturing of our product candidates and our current cost to manufacture our drug products is not commercially feasible. Additionally, the actual cost to manufacture our product candidates could materially and adversely affect the commercial viability of our product candidates. As a result, we may never be able to develop a commercially viable product.

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In addition, our reliance on third-party manufacturers exposes us to the following additional risks:

- We may be unable to identify manufacturers on acceptable terms or at all.
- Our third-party manufacturers might be unable to timely formulate and manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any.
- Contract manufacturers may not be able to execute our manufacturing procedures appropriately.
- Our future third-party manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store, and distribute our products.
- Manufacturers are subject to ongoing periodic unannounced inspection by the FDA and some state agencies to ensure strict compliance with cGMPs and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.
- We may not own, or may have to share, the intellectual property rights to any improvements made by our third-party manufacturers in the manufacturing process for our product candidates.
- Our third-party manufacturers could breach or terminate their agreement with us.

Each of these risks could delay our clinical trials, as well as the approval, if any, of our product candidates by the FDA, or the commercialization of our product candidates, or could result in higher costs, or could deprive us of potential product revenue. In addition, we rely on third parties to perform release testing on our product candidates prior to delivery to patients. If these tests are not appropriately conducted and test data are not reliable, patients could be put at risk of serious harm and could result in product liability suits.

The manufacture of medical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of medical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state, and foreign regulations. Furthermore, if contaminants are discovered in our supply of our product candidates or in the manufacturing facilities, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot be assured that any stability or other issues relating to the manufacture of our product candidates will not occur in the future. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide our product candidates to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

We may be unable to realize the potential benefits of any collaboration.

Even if we are successful in entering into a collaboration with respect to the development and/or commercialization of one or more product candidates, there is no guarantee that the collaboration will be successful. Collaborations may

pose a number of risks, including:

- collaborators often have significant discretion in determining the efforts and resources that they will apply to the collaboration and may not commit sufficient resources to the development, marketing, or commercialization of the product or products that are subject to the collaboration;

- collaborators may not perform their obligations as expected;

- any such collaboration may significantly limit our share of potential future profits from the associated program and may require us to relinquish potentially valuable rights to our current product candidates, potential products, proprietary technologies, or grant licenses on terms that are not favorable to us;

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- collaborators may cease to devote resources to the development or commercialization of our product candidates if the collaborators view our product candidates as competitive with their own products or product candidates;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation, or the course of development, might cause delays or termination of the development or commercialization of product candidates, and might result in legal proceedings, which would be time consuming, distracting, and expensive;
- collaborators may be impacted by changes in their strategic focus or available funding, or business combinations involving them, which could cause them to divert resources away from the collaboration;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- the collaborations may not result in us achieving revenues to justify such transactions; and
- collaborations may be terminated and, if terminated, may result in a need for us to raise additional capital to pursue further development or commercialization of the applicable product candidate.

As a result, a collaboration may not result in the successful development or commercialization of our product candidates.

For instance, in October 2011, we entered into the Servier Collaboration Agreement with Servier for the research, development, and commercialization of RNA-targeting therapeutics in cardiovascular disease, which was subsequently amended. Under the Servier Collaboration Agreement, we have granted Servier an exclusive license to research, develop, and commercialize RNA-targeting therapeutics for one target in the cardiovascular field and the right to obtain such an exclusive license for one additional target through September 2019. Servier's rights to this target are limited to therapeutics in the cardiovascular field in their territory, which is worldwide except for the United States and Japan. We retain all rights for the named target in the United States and Japan and for any products or product candidates outside of the cardiovascular field. We cannot guarantee that any product candidate will ever be successfully commercialized under the Servier Collaboration Agreement. If no product candidate subject to the Servier Collaboration Agreement is successfully commercialized, we may never receive additional milestone or any royalty payments under the Servier Collaboration Agreement. Also, due to restrictions contained in the Servier Collaboration Agreement, we may not be able to effectively develop, market, or commercialize any such product candidate in the United States and Japan.

We enter into various contracts in the normal course of our business in which we indemnify the other party to the contract. In the event we have to perform under these indemnification provisions, we could have a material adverse effect on our business, financial condition, and results of operations.

In the normal course of business, we periodically enter into academic, commercial, service, collaboration, licensing, consulting, and other agreements that contain indemnification provisions. With respect to our academic and other research agreements, we typically indemnify the institution and related parties from losses arising from claims relating to the products, processes, or services made, used, sold, or performed pursuant to the agreements for which we have secured licenses, and from claims arising from our or our sublicensees' exercise of rights under the agreement. With respect to our collaboration agreements, we indemnify our collaborators from any third-party product liability claims that could result from the production, use, or consumption of the product, as well as for alleged infringements of any patent or other intellectual property right by a third party. With respect to consultants, we indemnify them from claims arising from the good faith performance of their services.

Should our obligation under an indemnification provision exceed applicable insurance coverage or if we were denied insurance coverage, our business, financial condition, and results of operations could be adversely affected. Similarly, if we are relying on a collaborator to indemnify us and the collaborator is denied insurance coverage or the indemnification obligation exceeds the applicable insurance coverage, and if the collaborator does not have other assets available to indemnify us, our business, financial condition, and results of operations could be adversely affected.

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Risks Related to Commercialization of Our Product Candidates

We currently have limited marketing and sales experience. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

Although some of our employees may have launched other pharmaceutical products in the past while employed at other companies, we have no experience selling and marketing our product candidates and we currently have no marketing or sales organization. To successfully commercialize any products that may result from our development programs, we will need to find one or more collaborators to commercialize our products or invest in and develop these capabilities, either on our own or with others, which would be expensive, difficult, and time consuming. Any failure or delay in the timely development of our internal commercialization capabilities could adversely impact the potential for success of our products.

If commercialization collaborators do not commit sufficient resources to commercialize our future products and we are unable to develop the necessary marketing and sales capabilities on our own, we will be unable to generate sufficient product revenue to sustain or grow our business. We may be competing with companies that currently have extensive and well-funded marketing and sales operations, particularly in the markets our product candidates are intended to address. Without appropriate capabilities, whether directly or through third-party collaborators, we may be unable to compete successfully against these more established companies.

We may attempt to form collaborations in the future with respect to our product candidates, but we may not be able to do so, which may cause us to alter our development and commercialization plans.

We may attempt to form strategic collaborations, create joint ventures, or enter into licensing arrangements with third parties with respect to our programs that we believe will complement or augment our existing business. We may face significant competition in seeking appropriate strategic collaborators, and the negotiation process to secure appropriate terms is time consuming and complex. We may not be successful in our efforts to establish such a strategic collaboration for any product candidates and programs on terms that are acceptable to us, or at all. This may be because our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort, our research and development pipeline may be viewed as insufficient, the competitive or intellectual property landscape may be viewed as too intense or risky, and/or third parties may not view our product candidates and programs as having sufficient potential for commercialization, including the likelihood of an adequate safety and efficacy profile.

Any delays in identifying suitable collaborators and entering into agreements to develop and/or commercialize our product candidates could delay the development or commercialization of our product candidates, which may reduce their competitiveness even if they reach the market. Absent a strategic collaborator, we would need to undertake development and/or commercialization activities at our own expense. If we elect to fund and undertake development and/or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we are unable to do so, we may not be able to develop our product candidates or bring them to market and our business may be materially and adversely affected.

If the market opportunities for our product candidates are smaller than we believe they are, we may not meet our revenue expectations and, assuming approval of a product candidate, our business may suffer. Because the patient populations in the market for our product candidates may be small, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth.

Given the small number of patients who have the diseases that we are targeting, our eligible patient population and pricing estimates may differ significantly from the actual market addressable by our product candidates. For instance, one of our Phase 1 clinical trials in cobomarsen was focused on MF. The estimated prevalence of MF is 16,000 to 20,000 cases in the United States, only a subset of which may benefit from treatment with cobomarsen. Our projections of both the number of people who have this disease, as well as the subset of people with this disease who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. Additionally, while we believe that the data in our Phase 1 clinical trials for cobomarsen and remlarsen are supportive of application to other indications, there can be no assurance that our clinical trials in those indications will support efficacy of our product candidates in such expanded indications. Likewise, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with

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our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our business, financial condition, results of operations and prospects.

We face substantial competition and our competitors may discover, develop, or commercialize products faster or more successfully than us.

The development and commercialization of new drug products is highly competitive. We face competition from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, universities, and other research institutions worldwide with respect to cobomarsen, remlarsen, MRG-110, and the other product candidates that we may seek to develop or commercialize in the future. We are aware that the following companies have therapeutics marketed or in development for CTCL: Argenx, Bristol-Myers Squibb Company, Celgene Corporation, Helsinn Group, innate Pharma, Kyowa Hakko Kirin, Merck & Co., Inc., Mylan Pharmaceuticals Inc., Novartis International AG, Spectrum Pharmaceuticals, Inc., Seattle Genetics, Inc., Takeda Pharmaceutical Company Ltd, and Valeant Pharmaceuticals International, Inc. We are also aware that the several companies have marketed therapeutics for pulmonary fibrosis, including Boehringer Ingelheim GmbH and F. Hoffmann-La Roche Ltd. Our competitors may succeed in developing, acquiring, or licensing technologies and drug products that are more effective or less costly than cobomarsen, remlarsen, MRG-110, or any other product candidates that we are currently developing or that we may develop, which could render our product candidates obsolete and noncompetitive.

In addition to the competition we face from alternative therapies for the diseases we intend to target with our product candidates, we are also aware of several companies that are also working specifically to develop microRNA-targeted therapeutics, including Regulus Therapeutics, Inc., and InteRNA Technologies, B.V. Further, there are several companies working to develop other types of oligonucleotide therapeutic products, including Ionis Pharmaceuticals, Inc., Alnylam Pharmaceuticals, Inc., Arrowhead Pharmaceuticals, Inc., Dicerna Pharmaceuticals, Inc., RXi Pharmaceuticals Corporation, Silence Therapeutics AG, and Translate Bio. Many of our competitors have substantially greater financial, technical, and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Third-party payors, including governmental and private insurers, may also encourage the use of generic products. For example, if cobomarsen, remlarsen, or MRG-110 is approved, it may be priced at a significant premium over other competitive products. This may make it difficult for cobomarsen, remlarsen, MRG-110, or any other future products to compete with these products.

If our competitors obtain marketing approval from the FDA or comparable foreign regulatory authorities for their product candidates more rapidly than us, it could result in our competitors establishing a strong market position before we are able to enter the market.

Many of our competitors have materially greater name recognition and financial, manufacturing, marketing, research, and drug development resources than we do. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Large pharmaceutical companies in particular have extensive expertise in preclinical and clinical testing and in obtaining regulatory approvals for drugs. In addition, academic institutions, government agencies, and other public and private organizations conducting research may seek patent protection with respect to potentially competitive products or technologies. These organizations may also establish exclusive collaborative or licensing relationships with our competitors. Failure of cobomarsen, remlarsen, MRG-110, or other product candidates to effectively compete against established treatment options or in the future with new products currently in development would harm our business, financial condition, results of operations, and prospects.

The commercial success of any of our current or future product candidates will depend upon the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.

Even with the approvals from the FDA and comparable foreign regulatory authorities, the commercial success of our products will depend in part on the healthcare providers, patients, and third-party payors accepting our product candidates as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, and third-party payors. The degree of market acceptance of any of our products will depend on a number of factors, including but not limited to:

- the efficacy of the product as demonstrated in clinical trials and potential advantages over competing treatments;

- the prevalence and severity of the disease and any side effects;

- the clinical indications for which approval is granted, including any limitations or warnings contained in a product's approved labeling;

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- the convenience and ease of administration;
- the cost of treatment;
- the willingness of the patients and physicians to accept these therapies;
- the perceived ratio of risk and benefit of these therapies by physicians and the willingness of physicians to recommend these therapies to patients based on such risks and benefits;
- the marketing, sales, and distribution support for the product;
- the publicity concerning our products or competing products and treatments; and
- the pricing and availability of third-party insurance coverage and reimbursement.

Even if a product displays a favorable efficacy and safety profile upon approval, market acceptance of the product remains uncertain. Efforts to educate the medical community and third-party payors on the benefits of the products may require significant investment and resources and may never be successful. If our products fail to achieve an adequate level of acceptance by physicians, patients, third-party payors, and other healthcare providers, we will not be able to generate sufficient revenue to become or remain profitable.

We may not be successful in any efforts to identify, license, discover, develop, or commercialize additional product candidates.

Although a substantial amount of our effort will focus on the continued clinical testing, potential approval, and commercialization of our existing product candidates, the success of our business is also expected to depend in part upon our ability to identify, license, discover, develop, or commercialize additional product candidates. Research programs to identify new product candidates require substantial technical, financial, and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. Our research programs or licensing efforts may fail to yield additional product candidates for clinical development and commercialization for a number of reasons, including but not limited to the following:

- our research or business development methodology or search criteria and process may be unsuccessful in identifying potential product candidates;
- we may not be able or willing to assemble sufficient resources to acquire or discover additional product candidates;
- our product candidates may not succeed in preclinical or clinical testing;
- our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval;
- competitors may develop alternatives that render our product candidates obsolete or less attractive;
- product candidates we develop may be covered by third parties' patents or other exclusive rights;
- the market for a product candidate may change during our program so that such a product may become unreasonable to continue to develop;

- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community, or third-party payors.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, or we may not be able to identify, license, discover, develop, or commercialize additional product candidates, which would have a material adverse effect on our business, financial condition, or results of operations and could potentially cause us to cease operations.

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Failure to obtain or maintain adequate reimbursement or insurance coverage for our products, if any, could limit our ability to market those products and decrease our ability to generate revenue.

The pricing, coverage, and reimbursement of our approved products, if any, must be sufficient to support our commercial efforts and other development programs, and the availability and adequacy of coverage and reimbursement by third-party payors, including governmental and private insurers, are essential for most patients to be able to afford expensive treatments. Sales of our approved products, if any, will depend substantially, both domestically and abroad, on the extent to which the costs of our approved products, if any, will be paid for or reimbursed by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or government payors and private payors. If coverage and reimbursement are not available, or are available only in limited amounts, we may have to subsidize or provide products for free, or we may not be able to successfully commercialize our products.

In addition, there is significant uncertainty related to the insurance coverage and reimbursement for newly-approved products. In the United States, the principal decisions about coverage and reimbursement for new drugs are typically made by Centers for Medicare & Medicaid Services, or CMS, which is an agency within the U.S. Department of Health and Human Services that decides whether and to what extent a new drug will be covered and reimbursed under Medicare. Private payors tend to follow the coverage reimbursement policies established by CMS to a substantial degree. It is difficult to predict what CMS will decide with respect to reimbursement for novel product candidates such as our and what reimbursement codes our product candidates may receive if approved.

Outside the United States, international operations are generally subject to extensive governmental price controls and other price-restrictive regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of products. In many countries, the prices of products are subject to varying price control mechanisms as part of national health systems. Price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our products, if any. Accordingly, in markets outside the United States, the potential revenue may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and private payors in the United States and abroad to limit or reduce healthcare costs may result in restrictions on coverage and the level of reimbursement for new products and, as a result, they may not cover or provide adequate payment for our products. Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs. We expect to experience pricing pressures in connection with products due to the increasing trend toward managed healthcare, including the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs, has increased and is expected to continue to increase in the future. As a result, profitability of our products, if any, may be more difficult to achieve even if they receive regulatory approval.

Risks Related to Our Business Operations

Our future success depends in part on our ability to retain our president and chief executive officer and to attract, retain, and motivate other qualified personnel.

We are highly dependent on William S. Marshall, Ph.D., our president and chief executive officer, the loss of whose services may adversely impact the achievement of our objectives. Dr. Marshall could leave our employment at any

time, as he is an “at will” employee. Recruiting and retaining other qualified employees, consultants, and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of highly qualified personnel in our industry, which is likely to continue. Additionally, this shortage of highly qualified personnel is particularly acute in the area where we are located. As a result, competition for personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in development and commercialization of our product candidates may make it more challenging to recruit and retain qualified personnel. The inability to recruit and retain qualified personnel, or the loss of the services of Dr. Marshall, may impede the progress of our research, development, and commercialization objectives and would negatively impact our ability to succeed in our product development strategy.

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We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.

As of September 30, 2018, we had 73 full-time employees. As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, sales, marketing, financial, legal, and other resources. Our management may need to divert a disproportionate amount of our attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees, and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Failure in our information technology and storage systems could significantly disrupt the operation of our business.

Our ability to execute our business plan and maintain operations depends on the continued and uninterrupted performance of our information technology, or IT, systems. IT systems are vulnerable to risks and damages from a variety of sources, including telecommunications or network failures, malicious human acts, and natural disasters. Moreover, despite network security and back-up measures, some of our and our vendors' servers are potentially vulnerable to physical or electronic break-ins, including cyber-attacks, computer viruses, and similar disruptive problems. These events could lead to the unauthorized access, disclosure, and use of non-public information. The techniques used by criminal elements to attack computer systems are sophisticated, change frequently, and may originate from less regulated and remote areas of the world. As a result, we may not be able to address these techniques proactively or implement adequate preventative measures. If our computer systems are compromised, we could be subject to fines, damages, litigation, and enforcement actions, and we could lose trade secrets, the occurrence of which could harm our business. Despite precautionary measures to prevent unanticipated problems that could affect our IT systems, sustained or repeated system failures that interrupt our ability to generate and maintain data could adversely affect our ability to operate our business.

Our ability to use net operating losses to offset future taxable income may be subject to limitation.

Our net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the newly enacted federal income tax law, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. It is uncertain if and to what extent various states will conform to the newly enacted federal tax law. In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. Our most recent analysis of possible ownership changes was completed for certain tax periods ending through the date of the Merger. The Merger resulted in an ownership change for us and, accordingly, our net operating loss carryforwards and certain other tax attributes are subject to limitation. Additional ownership changes in the future could result in additional limitations on our net operating loss carryforwards and certain other tax attributes. Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our net operating loss carryforwards and certain other tax attributes, which could have a material adverse effect on cash flow and results of operations.

The recently passed comprehensive tax reform bill could adversely affect our business and financial condition.

On December 22, 2017, new legislation was signed into law that significantly revises the Code. The newly enacted federal income tax law, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the new federal tax law is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the newly enacted federal tax law. The impact of this tax

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reform or any future tax laws on holders of our common stock is also uncertain and could be adverse. We urge our stockholders to consult with their legal and tax advisors with respect to this legislation and the potential tax consequences of investing in or holding our common stock.

Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.

We are subject to taxation in numerous U.S. states and territories and non-U.S. jurisdictions. As a result, our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors including passage of the newly enacted federal income tax law, the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes, and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

Risks Related to Ownership of our Common Stock

The market price of our common stock is expected to be volatile, and the market price of our common stock may drop in the future.

The market price of our common stock following the Merger has been, and may continue to be, subject to significant fluctuations. Market prices for securities of early-stage pharmaceutical, biotechnology, and other life sciences companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- our ability to obtain regulatory approvals for cobomarsen, remlarsen, MRG-110, or other product candidates, and delays or failures to obtain such approvals;
- failure of any of our product candidates, if approved, to achieve commercial success;
- failure to maintain our existing third-party license and supply agreements;
- changes in laws or regulations applicable to our product candidates;
- any inability to obtain adequate supply of our product candidates or the inability to do so at acceptable prices;
- adverse regulatory authority decisions;
- introduction of new products, services, or technologies by our competitors;
- failure to meet or exceed financial and development projections we may provide to the public;
- failure to meet or exceed the financial and development projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators, and the investment community;
- announcements of significant acquisitions, strategic collaborations, joint ventures, or capital commitments by us or our competitors;

- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;

- additions or departures of key personnel;

- significant lawsuits, including patent or stockholder litigation;

- if securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our business and stock;

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- changes in the market valuations of similar companies;
- general market or macroeconomic conditions;
- sales of our common stock by us or our stockholders in the future;
- trading volume of our common stock;
- announcements by commercial partners or competitors of new commercial products, clinical progress or the lack thereof, significant contracts, commercial relationships, or capital commitments;
- adverse publicity relating to microRNA-targeted therapeutics generally, including with respect to other products and potential products in such markets;
- the introduction of technological innovations or new therapies that compete with our potential products;
- changes in the structure of health care payment systems; and
- period-to-period fluctuations in our financial results.

Moreover, the capital markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

Additionally, a decrease in our stock price may cause our common stock to no longer satisfy the continued listing standards of The Nasdaq Capital Market. If we are not able to maintain the requirements for listing on The Nasdaq Capital Market, we could be delisted, which could have a materially adverse effect on our ability to raise additional funds as well as the price and liquidity of our common stock.

We incur costs and demands upon management as a result of complying with the laws and regulations affecting public companies.

As a result of the Merger, we incur significant legal, accounting, and other expenses that Private Miragen did not incur as a private company, including costs associated with public company reporting requirements. We also incur costs associated with corporate governance requirements, including requirements under the Sarbanes-Oxley Act, as well as new rules implemented by the SEC and The Nasdaq Stock Market LLC, or Nasdaq. These rules and regulations are expected to increase our legal and financial compliance costs and make some activities more time-consuming and costly. For example, our management team consists of the executive officers of Private Miragen prior to the Merger, some of whom have not previously managed and operated a public company. These executive officers and other personnel will need to devote substantial time to gaining expertise regarding operations as a public company and compliance with applicable laws and regulations. These rules and regulations may also make it difficult and expensive for us to obtain directors' and officers' liability insurance. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as our executive officers, which may adversely affect investor confidence and could cause our business or stock price to suffer.

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

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

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making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

Our bylaws provide that the Court of Chancery of the State of Delaware is the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or other employees.

Our bylaws provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a breach of fiduciary duty owed by any of our directors, officers, or other employees to us or our stockholders, any action asserting a claim against us arising pursuant to any provisions of the Delaware General Corporation Law, our certificate of incorporation or our bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage such lawsuits against our and our directors, officers, and other employees. If a court were to find the choice of forum provision contained in the bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

We do not anticipate that we will pay any cash dividends in the foreseeable future.

The current expectation is that we will retain our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain, if any, for the foreseeable future.

Historically, there has not been an active trading market for our common stock and we cannot guarantee an active market for our common stock will be sustained in the future. As a result, our stockholders may not be able to resell their shares of common stock for a profit, if at all.

Prior to the Merger, there had been no public market for Private Miragen's common stock. An active trading market for our shares of common stock has yet to develop, and even if an active market for our common stock were to develop, it may not be sustained. If an active market for our common stock is not sustained, it may be difficult for our stockholders to sell their shares at an attractive price or at all.

Future sales of shares by existing stockholders could cause our stock price to decline.

If our stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after legal restrictions on resale lapse, the trading price of our common stock could decline. In addition, shares of common stock that are subject to our outstanding options will become eligible for sale in the public market to the extent permitted by the provisions of various vesting agreements and Rules 144 and 701 under the Securities Act of 1933, as amended.

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

Our directors, officers, 5% stockholders, and their affiliates currently beneficially own a substantial portion of our outstanding voting stock. Therefore, these stockholders have the ability and may continue to have the ability to influence us through this ownership position. These stockholders may be able to determine some or all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of organizational documents, or approval of any merger, sale of assets, or other major

corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business, or our market, our stock price and trading volume could decline.

The trading market for our common stock is influenced by the research and reports that equity research analysts publish about us and our business. Equity research analysts may elect not to provide research coverage of our common stock and such lack of research coverage may adversely affect the market price of our common stock. In the event we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our common stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of us or fails to publish

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reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, and the rules and regulations of Nasdaq. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. We must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our annual report filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. As a private company, Private Miragen had never been required to test its internal controls within a specified period. This requires that we incur substantial professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. We may experience difficulty in meeting these reporting requirements in a timely manner for each period.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system’s objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our common stock could decline, and it could be subject to sanctions or investigations by Nasdaq, the SEC, or other regulatory authorities.

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

Not applicable.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

Not applicable.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

Not applicable.

ITEM 6. EXHIBITS

The exhibits listed in the Exhibit Index are required by Item 601 of Regulation S-K. The SEC file number for all items incorporated by reference herein from reports on Forms 10-K, 10-Q, and 8-K is 001-36483.

Exhibit Number	Description of Exhibit	Incorporated by Reference			Filed Herewith
		Form	Filing Date	Number	
3.1	Certificate of Incorporation of the Registrant.	10-Q	08/14/2014	3.1	
3.2	Certificate of Amendment of Certificate of Incorporation of the Registrant.	S-4	12/02/2016	3.3	
3.3	Certificate of Amendment of Certificate of Incorporation of the Registrant.	8-K	02/13/2017	3.1	

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3.4	Certificate of Amendment of Certificate of Incorporation of the Registrant.	8-K	02/13/2017	3.2
3.5	Amended and Restated Bylaws of the Registrant.	10-Q	08/15/2016	3.1
3.6	Amendment to the Amended and Restated Bylaws of the Registrant.	8-K	02/13/2017	3.3
3.7	Certificate of Ownership and Merger of the Registrant.	8-K	02/13/2017	3.4
10.1^	Amendment to Research Subaward Agreement, entered into as of June 29, 2018 and effective as of July 1, 2018, by and between Registrant and Yale University, as amended.	10-Q	08/08/2018	10.3
10.2†	Common Stock Purchase Agreement, dated August 6, 2018, by and between the Registrant and The Leukemia & Lymphoma Society, Inc.			
31.1	Certification of Principal Executive Officer pursuant to Rule13a-14(a) and Rule 15d-14(a) of the Securities and Exchange Act, as amended.			
31.2	Certification of Principal Financial Officer pursuant to Rule13a-14(a) and Rule 15d-14(a) of the Securities and Exchange Act, as amended.			
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.			
101.INS**	XBRL Instance Document			
101.SCH**	XBRL Taxonomy Extension Schema Document			
101.CAL**	XBRL Taxonomy Extension Calculation Linkbase Document			
101.DEF**	XBRL Taxonomy Extension Definition Linkbase Document			
101.LAB**	XBRL Taxonomy Extension Label Linkbase Document			
101.PRE**	XBRL Taxonomy Extension Presentation Linkbase Document			

† Confidential treatment has been requested as to portions of the exhibit. Confidential materials omitted and filed separately with the SEC.

^ Confidential treatment has been granted as to portions of the exhibit. Confidential materials omitted and filed separately with the SEC.

* This certification is being furnished pursuant to 18 U.S.C. Section 1350, and is not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is not to be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof.

** In accordance with Rule 406T of Regulation S-T, the Interactive Data Files in Exhibit 101 are deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, are deemed not filed for purposes of Section 18 of the Exchange Act of 1934, as amended, and otherwise are not subject to liability under these sections.

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

MIRAGEN THERAPEUTICS, INC.

Date: November 7, 2018 By: /s/ William S. Marshall
William S. Marshall, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

Date: November 7, 2018 By: /s/ Jason A. Leverone
Jason A. Leverone
Chief Financial Officer
(Principal Financial Officer; Principal Accounting Officer)