TOBIRA THERAPEUTICS, INC.

FORM 10-Q
(Quarterly Report)

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

FORM 10-Q

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

For the quarterly period ended September 30, 2013

OR

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

Commission File Number: 001-35953

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

Delaware	**No. 03-0422069**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

120 Mountain View Boulevard
Basking Ridge, New Jersey 07920
(Address of principal executive offices) (Zip Code)

(908) 580-2100
(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☐ No ☒

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ☒ No ☐

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

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

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

As of November 7, 2013, 21,310,614 shares of common stock, $0.001 par value per share, were outstanding.

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REGADO BIOSCIENCES, INC.

FORM 10-Q

Quarter Ended September 30, 2013

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PART I—FINANCIAL INFORMATION

Cautionary Statement Regarding Forward-Looking Statements

This quarterly report on Form 10-Q includes forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. For this purpose, any statements contained herein, other than statements of historical fact, including statements regarding the progress and timing of our product development programs and related trials; our future opportunities; our strategy, future operations, anticipated financial position, future revenues and projected costs; our management's prospects, plans and objectives; and any other statements about management's future expectations, beliefs, goals, plans or prospects constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. We may, in some cases, use words such as "anticipate," "believe," "could," "estimate," "expect," "intend," "may," "plan," "project," "should," "target," "will," "would" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including our "critical accounting estimates"; our plans to initiate and complete our single, open-label 13,200 subject Phase 3 trial of REG1; our ability to satisfy domestic and international regulatory requirements with respect to REG1 and our other product candidates, many of which are new and still evolving, and the labeling under any approval we may obtain; the performance of contract research organizations who conduct our clinical trials for us; the performance of third-party manufacturers who supply or manufacture our products; our ability to develop commercialization and marketing capabilities or to enter into strategic partnerships to develop and commercialize REG1 or any of our other product candidates; the timing and success of the commercialization of REG1 or any of our other product candidates; the rate and degree of market acceptance of REG1; the size and growth of the potential markets for REG1 and our ability to serve those markets; our plans to expand the indications of REG1; our ability to discover, develop and commercialize novel and innovative therapies using our proprietary technology platform; regulatory developments in the United States and foreign countries; competition from existing antithrombotic drugs or new antithrombotic drugs that may emerge; potential product liability claims; our ability to attract and retain a sufficient number of scientists, clinicians, sales personnel and other key personnel; our ability to obtain, maintain, defend and enforce intellectual property rights protecting our product candidates; the accuracy of our estimates regarding expenses and capital requirements and our ability to adequately support future growth. These and other risks are described in greater detail in this report under "Part II—Item 1A Risk Factors." If one or more of these factors materialize, or if any underlying assumptions prove incorrect, our actual results, performance or achievements may vary materially from any future results, performance or achievements expressed or implied by these forward-looking statements. In addition, any forward-looking statements in this quarterly report on Form 10-Q represent our views only as of the date of this quarterly report on Form 10-Q and should not be relied upon as representing our views as of any subsequent date. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements publicly at some point in the future, we specifically disclaim any obligation to do so, whether as a result of new information, future events or otherwise, except as may be required by law. Our forward-looking statements do not reflect the potential impact of any acquisitions, mergers, dispositions, business development transactions, joint ventures or investments we may enter into or make. We qualify all of our forward-looking statements by these cautionary statements.

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ITEM 1. FINANCIAL STATEMENTS

Regado Biosciences, Inc.
(*a development stage enterprise* **)**
CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)

	September 30, 2013 (Unaudited)	December 31, 2012
Assets		
Current assets:		
Cash and cash equivalents	$ 43,456	$ 14,764
Restricted cash	82	82
Prepaid expenses	5,089	257
Other assets	4,747	4,580
Total current assets	53,374	19,683
Debt issuance costs, net	44	87
Property and equipment, net	102	66
Intangible assets, net	2,002	1,771
Other non-current assets	2,051	196
Total assets	$ 57,573	$ 21,803
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 424	$ 203
Accrued expenses	5,028	786
Warrant liability	23	—
Current portion of long-term debt	500	2,571
Total current liabilities	5,975	3,560
Long-term debt	3,906	1,929
Total liabilities	9,881	5,489
Commitments (Note 4)		
Stockholders' equity:		
Series A convertible preferred stock; $0.001 par value; 0 shares designated, issued and outstanding at September 30, 2013 and 5,798,178 shares designated, issued and outstanding (liquidation preference of $5,798) at December 31, 2012	—	5,778
Series B convertible preferred stock; $0.001 par value; 0 shares designated, issued and outstanding at September 30, 2013 and 16,666,665 shares designated, issued and outstanding (liquidation preference of $20,000) at December 31, 2012	—	19,888
Series C convertible preferred stock; $0.001 par value; 0 shares designated, issued and outstanding at September 30, 2013 and 17,037,037 shares designated, issued and outstanding (liquidation preference of $23,000) at December 31, 2012	—	22,978
Series D convertible preferred stock; $0.001 par value; 0 shares designated, issued and outstanding at September 30, 2013 and 71,666,667 shares designated, issued and outstanding (liquidation preference of $51,600) at December 31, 2012	—	51,237
Series E convertible preferred stock; $0.001 par value; 0 shares designated, issued and outstanding at September 30, 2013 and 31,437,442 shares designated, issued and outstanding (liquidation preference of $22,635) at December 31, 2012	—	22,169
Common stock, $0.001 par value; 500,000,000 shares authorized; 21,303,012 and 221,272 shares issued and outstanding at September 30, 2013 and December 31, 2012, respectively	21	—
Additional paid-in-capital	178,519	4,804
Deficit accumulated during the development stage	(130,848)	(110,540)
Total stockholders' equity	47,692	16,314
Total liabilities and stockholders' equity	$ 57,573	$ 21,803

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

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Regado Biosciences, Inc.
(*a development stage enterprise*)
CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(UNAUDITED)
(In thousands, except share and per share data)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,		Period from Inception (December 19, 2001) to September 30,
	2013	2012	2013	2012	2013
Grant revenue	$ —	$ —	$ —	$ —	$ 832
Total revenue	—	—	—	—	832
Operating expenses:					
Research and development	(9,597)	(1,607)	(15,594)	(6,519)	(97,555)
General and administrative	(1,614)	(974)	(4,240)	(3,368)	(35,138)
Total operating expenses	(11,211)	(2,581)	(19,834)	(9,887)	(132,693)
Loss from operations	(11,211)	(2,581)	(19,834)	(9,887)	(131,861)
Other (expense) income:					
Realized gain on investments	—	—	—	—	176
Interest income	2	1	71	4	2,869
Interest expense	(146)	(137)	(545)	(438)	(2,032)
Other expense	(60)	—	—	—	—
Total other (expense) income	(204)	(136)	(474)	(434)	1,013
Net loss	$ (11,415)	$ (2,717)	$ (20,308)	$ (10,321)	$ (130,848)
Comprehensive loss	$ (11,415)	$ (2,717)	$ (20,308)	$ (10,321)	$ (130,848)
Loss per share - basic and diluted	$ (1.43)	$ (12.28)	$ (7.18)	$ (46.64)	$ (356.09)
Weighted-average common shares - basic and diluted	8,005,142	221,272	2,828,757	221,272	367,455

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

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Regado Biosciences, Inc.
(*a development stage enterprise*)
CONSOLIDATED STATEMENTS OF CASH FLOWS
(UNAUDITED)
(In thousands)

	For the Nine Months Ended September 30,		Period from Inception (December 19, 2001) to September 30,
	2013	2012	2013
Cash flows from operating activities:			
Net loss	$ (20,308)	$(10,321)	$ (130,848)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	41	32	2,344
Amortization of patents and licenses	75	302	1,863
Impairment of patents	10	8	174
Accrued final bank fee	33	—	33
Amortization of debt discount	37	—	37
Amortization of debt issuance costs	100	27	148
Change in fair value of warrant liability	(56)	—	(56)
Stock-based compensation	346	578	4,957
Loss on disposal of property and equipment	—	—	59
Gain on sale of investments	—	—	(176)
Other	64	—	64
Changes in operating assets and liabilities:			
Prepaid expenses	(4,832)	(164)	(5,175)
Other assets	(244)	(1,700)	(4,738)
Other non-current assets	(1,855)	—	(2,051)
Accounts payable	221	(116)	424
Accrued expenses	4,242	786	4,964
Net cash used in operating activities	(22,126)	(10,568)	(127,977)
Cash flows from investing activities:			
Change in restricted cash	—	—	(82)
Purchase of property and equipment	(77)	(14)	(2,508)
Proceeds received on disposal of property and equipment	—	—	5
Purchase of investments	—	—	(163,683)
Proceeds from sales of investments	—	—	163,859
Patent and license acquisition costs	(415)	(257)	(4,138)
Proceeds received from sale of patents	100	—	100
Net cash used in investing activities	(392)	(271)	(6,447)
Cash flows from financing activities:			
Proceeds from borrowing on convertible notes payable	—	6,781	14,902
Proceeds from borrowings on bank loan and other notes payable	4,500	—	9,000
Repayment of other notes payable	(4,500)	(1,072)	(4,500)
Payment of bank origination fee	(85)	—	(85)
Payment of debt issuance costs	(57)	(28)	(193)
Proceeds from issuance of common stock in IPO, net of underwriting discounts	43,418	—	43,418
Proceeds from issuance of common stock	—	—	191
Payment of IPO costs	(2,231)	—	(2,231)
Proceeds from sale of preferred stock, net of issuance costs	10,163	(53)	117,351
Proceeds from exercise of warrants	2	—	27
Net cash provided by financing activities	51,210	5,628	177,880
Net increase (decrease) in cash and cash equivalents	$ 28,692	$ (5,211)	$ 43,456
Cash and cash equivalents, beginning of period	14,764	9,235	—
Cash and cash equivalents, end of period	$ 43,456	$ 4,024	$ 43,456
Supplemental disclosures of cash flow information:			
Cash paid for interest	$ 465	$ 447	$ $1,286
Supplemental disclosure of non-cash investing and financing activities :			
Conversion of preferred stock into common stock	$132,213	—	$ 132,213
Conversion of note payable and interest into preferred stock	—	—	$ 15,151

Stock issued for cash held in escrow	—	—	$ 5,155

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

Regado Biosciences, Inc.
(a development stage enterprise)
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

1 Organization and Basis of Presentation

Organization; Description of Business

Regado Biosciences, Inc. (the "Company" or "we" or "our" or "us") is a development stage enterprise incorporated in the State of Delaware on December 19, 2011 operating primarily in Basking Ridge, New Jersey and Durham, North Carolina. We are focused on the discovery and development of novel, first-in-class, actively controllable antithrombotic drug systems for acute and sub-acute cardiovascular indications. Each of our product candidates consists of a two-component system: an antithrombotic aptamer and its specific active control agent. Our lead product candidate, REG1, is a two-component system consisting of pegnivacogin, an anticoagulant aptamer specifically targeting coagulation Factor IXa, and its complementary oligonucleotide active control agent, anivamersen. REG1 is being developed for use in patients with a wide variety of acute coronary syndromes, or ACS, undergoing a percutaneous coronary intervention, or PCI, a hospital-based procedure used to mechanically open or widen obstructed coronary arteries. Our actively controllable product candidates have the potential to improve patient outcomes, enhance the patient experience and reduce overall treatment costs. In September 2013, we commenced our single, open-label, 13,200 subjects Phase 3 trial of REG1 in patients undergoing PCI (excluding ST elevated myocardial infarction, or STEMI), or the REGULATE-PCI trial.

Reverse Stock Split

In May 2013, our board of directors and our stockholders approved an amendment to the amended and restated certificate of incorporation to effect a one-for-16.7 reverse split of shares of common stock and to increase the number of authorized shares of common stock to 500,000,000, each of which was effected on May 21, 2013.

Initial Public Offering

We completed our initial public offering ("IPO") in August 2013. Inclusive of the underwriters' exercise of the over-allotment option in connection with the IPO in September 2013, we issued 11,671,500 shares of common stock at a price of $4.00 per share, resulting in net proceeds of approximately $41.1 million, after deducting underwriting discounts of $3.3 million and offering costs of $2.3 million. Upon the closing of the IPO, all shares of convertible preferred stock then outstanding automatically converted into an aggregate of 9,396,767 shares of common stock.

Principles of Consolidation

In March 2013, we incorporated Regado Biosciences Europe Limited, a wholly owned subsidiary registered in England and Wales, in order to establish a legal presence in the European Union (EU) for the purpose of conducting clinical trials in the EU. Regado Biosciences Europe Limited had no operations for the three and nine months ended September 30, 2013.

The accompanying consolidated financial statements include the accounts of Regado Biosciences, Inc. and its wholly owned subsidiary, Regado Biosciences Europe Limited. There were no significant intercompany accounts or transactions that needed to be eliminated in consolidation.

Basis of Accounting and Going Concern Uncertainty

Our financial statements have been prepared on a going-concern basis, which contemplates the realization of assets and settlement of liabilities and commitments in the normal course of business. Operations since inception have consisted primarily of developing and acquiring product technologies and securing financing.

The accompanying financial statements have been prepared assuming that we will operate as a going concern. We have suffered negative cash flows from operating activities of $22.1 million during the nine months ended September 30, 2013 and a net accumulated deficit of $130.8 million since inception as of September 30, 2013. Prior to our IPO, we were funded primarily through the issuance of preferred stock and debt. We will require additional capital until such time that we can generate operating revenue in excess of operating expenditures. Our plans include continued product development and a move toward completion of clinical trials. We will continue to closely monitor and analyze expenses and make adjustments as necessary to prioritize business operations. We believe that the net proceeds from the IPO will be sufficient for us to fund the REGULATE-PCI trial through the first interim analysis, which we expect will occur by the beginning of the second quarter of 2014, and to fund our operations through the second quarter of 2014. We will need to raise additional financing in the first half of 2014 to fund projected operations through 2014 and we can provide no assurances that such additional financing will be available on favorable terms, or at all. Actual results may differ from estimates and the financial statements do not include any adjustments that might be necessary if we are unable to fund operations.

Unaudited Interim Financial Data

The accompanying interim consolidated financial statements are unaudited. These unaudited consolidated financial statements have been prepared in accordance with the rules and regulations of the United States Securities and Exchange Commission ("SEC") for interim financial information under Article 210.8-03 of Regulation S-X. Accordingly, they do not include all of the information and footnotes required by U.S. GAAP for complete financial statements. These unaudited interim consolidated financial statements should be read in conjunction with the audited financial statements and the accompanying notes for the year ended December 31, 2012 included in our final prospectus dated August 22, 2013 filed pursuant to Rule 424(b) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission on August 22, 2013. The unaudited interim consolidated financial statements have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments (consisting of normal recurring adjustments) necessary to state fairly our financial position as of September 30, 2013; the results of our operations for the three and nine months ended September 30, 2013 and 2012 and for the period from inception through September 30, 2013; and our cash flows for the nine months ended September 30, 2013 and 2012 and for the period from inception through September 30, 2013. The results of operations for the three and nine months ended September 30, 2013 are not necessarily indicative of the operating results for the full year or any other interim period.

2 Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

Reclassifications

We have reclassified supplies inventory, net to other current assets in the accompanying consolidated balance sheet at December 31, 2012. This reclassification did not have any impact on our loss from operations or net loss for the three or nine months ended September 30, 2012, or on total assets as of December 31, 2012.

We have reclassified all laboratory and clinical indirect costs and laboratory and clinical personnel stock compensations costs for the three and nine months ended September 30, 2012, and for the period from inception through December 31, 2012, from general and administrative expense to research and development expense in the accompanying consolidated statements of comprehensive loss. These reclassifications did not have any impact on our loss from operations or net loss for the three or nine months ended September 30, 2012, or for the period from inception through December 31, 2012.

We have reclassified patent and product license amortization and patent impairment costs for the three and nine months ended September 30, 2012, and for the period from inception through December 31, 2012, from general and administrative expense to research and development expense in the accompanying consolidated statements of comprehensive loss. These reclassifications did not have any impact on our loss from operations or net loss for the three or nine months ended September 30, 2012, or for the period from inception through December 31, 2012.

Fair Value of Financial Instruments

The carrying amount of certain of our financial instruments, including cash and cash equivalents, prepaid expenses, and accounts payable approximate fair value due to the short maturities of those financial instruments. The carrying amount of our debt at September 30, 2013 and December 31, 2012 approximated fair value. The estimated fair value of our debt at September 30, 2013 and December 31, 2012, respectively, was determined based on available market information for alternative financing under similar terms. In conjunction with the refinancing of our long term debt in May 2013, we held a warrant liability at September 30, 2013 that is required to be measured at fair value on a recurring basis (see Note 3).

Our valuation of financial instruments is based on a three-tiered approach, which requires that fair value measurements be classified and disclosed in one of three tiers. These tiers are: Level 1, defined as quoted prices in active markets for identical assets or liabilities; Level 2, defined as valuations based on observable inputs other than those included in Level 1, such as quoted prices for similar assets and liabilities in active markets, or other inputs that are observable or can be corroborated by observable input data; and Level 3, defined as valuations based on unobservable inputs reflecting our own assumptions, consistent with reasonably available assumptions made by other market participants.

Cash and Cash Equivalents

We consider all interest-bearing investments due on demand and all highly liquid debt instruments purchased with a maturity of three months or less to be cash equivalents. Cash and cash equivalents included cash of $253,000 and $235,000 at September 30, 2013 and December 31, 2012, respectively. Cash and cash equivalents at September 30, 2013 and December 31, 2012 also included investments of $43.2 million and $14.5 million, respectively, in money market funds invested in U.S. Treasury securities with original maturities of less than three months. Cash deposits are held in federally insured financial institutions in the United States of America. We maintain cash in accounts which are in excess of federally insured limits.

Significant Concentrations

The financial instruments that potentially subject us to concentrations of credit risk are cash and cash equivalents. Our cash and cash equivalents are maintained primarily with two financial institutions.

We do not have a manufacturing infrastructure and do not intend to develop one for the foreseeable future . We have agreements with third-party contract manufacturing organizations, or CMOs, to supply bulk drug substances for our product candidates and with third parties to formulate, package and distribute our drug product candidates. Our employees include professionals with expertise in pharmaceutical manufacturing development who oversee the manufacture and distribution of our drug product candidates by third-party companies. We believe that we have sufficient amounts of REG1 on hand to complete a minimum of 50% enrollment in the REGULATE-PCI trial. All of the drug substances used in our product candidates are manufactured by single suppliers. While we have not experienced any supply disruptions, the number of oligonucleotide manufacturers is limited. In the event it is necessary or advisable to acquire supplies from an alternative supplier, we might not be able to obtain them on commercially reasonable terms, if at all. It could also require significant time and expense to redesign our manufacturing processes to work with another company. Formulation and distribution of our finished drug product candidates are also conducted by a single supplier but we believe that alternative sources for these services are readily available on commercially reasonable terms (see Note 4).

Clinical Trial Supplies

We capitalize materials that will be used in our REG I clinical trials that also have an alternative future use in either ongoing or future clinical research and development projects. Clinical trial supplies may comprise material used to manufacture active pharmaceutical ingredients (“API”) used to develop our product candidates, in-process or completed API, in-process or completed unlabeled finished drug product and labeled finished drug product. Clinical trial supplies are stated at the lower of cost or market, using the first-in, first-out method (“FIFO”), and are reported in the accompanying consolidated balance sheets in other current assets. Clinical trial supplies that are determined to be unsuitable for future use are immediately expensed; otherwise clinical trial supplies are expensed when shipped to clinical sites for use in clinical studies or when used in other research and development projects.

We utilize CMOs to produce API and finished drug product for use in clinical trials. As we do not have facilities that meet the requisite regulatory requirements for storage of API or finished drug product produced, we use a third-party facility for storage. Upon release from the manufacturer, API is shipped to a third-party storage facility. For production of finished drug product, API is shipped from the storage facility to the finished drug product manufacturing site. Unlabelled finished drug product is either shipped from the manufacturer back to the third-party storage facility or directly to the third-party labeling site. Labeled finished drug product is held by the third-party labeling site until it is shipped to the clinical sites for trial use.

We do not have multiple sources of supply for the components of our finished drug product. If we are unable to obtain the supplies needed at a reasonable price or on a timely basis, it could have a material adverse effect on our ability to complete the development of our finished drug product.

As of September 30, 2013 clinical trial supplies included in other current assets were $4.7 million, of which $1.5 million and $3.2 million represented API held at the third-party storage facility and drug product located at depots, respectively. As of December 31, 2012, clinical trial supplies included in other current assets were $4.5 million which represented API held at the third-party storage facility.

Clinical Agreements

We enter into various clinical trial agreements with academic research organizations ("AROs") and clinical research organizations ("CROs") for the planning, management and execution of clinical trials. The financial terms of these agreements are subject to negotiation, vary from contract to contract, and may result in uneven payment flows. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. Costs for ARO and CRO contracts are recognized based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations, or information provided by vendors on their actual costs incurred; such costs are charged to research and development expense in the accompanying consolidated statement of comprehensive loss. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the research and development expense. Upfront refundable contract signing fees are amortized over the life of the respective contract; unamortized contract signing fees are included in other non-current assets.

Included in the accompanying consolidated balance sheet as of September 30, 2013 are $2.7 million of prepaid costs that will be applied to final invoices as required under the respective contract, and $1.9 million of other non-current assets which represent upfront contract signing costs that will be amortized over the life of the respective contract, or approximately three years.

In general, our ARO and CRO service agreements permit either party to terminate at will, although we would continue to be responsible for payment of all services completed (or pro-rata completed) at the time of notice of termination, plus any non-cancellable expenses that have been entered into by the ARO and CRO on the Company's behalf. Accordingly, such expenses would be accrued at time of contract termination and any prepaid expenses and unamortized advance payments would be expensed, accordingly.

Intangibles

The following information details the carrying amounts and accumulated amortization of our intangible assets subject to amortization (in thousands):

		As of September 30, 2013			As of December 31, 2012		
	Weighted Average Useful Life Remaining	Gross Carrying Amount	Accumulated Amortization	Net Carrying Amount	Gross Carrying Amount	Accumulated Amortization	Net Carrying Amount
Identifiable intangible assets:							
Patents	8.1 years	$ 1,706	$ (446)	$ 1,260	$ 1607	$ (349)	$ 1,258
Product licenses	2.1 years	1,554	(1,417)	137	1,547	(1,439)	108
Total	7.9 years	$ 3,260	$ (1,863)	$ 1,397	$ 3,154	$ (1,788)	$ 1,366

Expected future amortization expenses for intangible assets as of September 30, 2013 are as follows:

For the year ending December 31:	Amount
2013	$ 40
2014	157
2015	149
2016	149
2017	149
Thereafter	753
	$1,397

We record amortization of patent and product license costs in research and development expense in the accompanying consolidated statements of comprehensive loss.

Patents

Patent costs consist of expenditures incurred for various patent applications. Upon receiving a patent grant, such respective costs are amortized over the remaining life of the patent. As of September 30, 2013 and December 31, 2012, we had $605,000 and $405,000 of costs related to patents that have not yet been granted.

The impairment of patents occurs when a patent that has been applied for becomes expired or abandoned. Once the status of the patent changes to expired or abandoned, the associated costs for the patent application are expensed to research and development in the accompanying consolidated statements of comprehensive loss. Patent impairment expense recorded was $0 and $3,000 for the three months ended September 30, 2013 and 2012, respectively, and $10,000 and $8,000 for the nine months ended September 30, 2013 and 2012, respectively, and $174,000 for the period from inception to September 30, 2013.

Product Licenses

We have primary license agreements with Duke University, Archemix Corp. and Nektar Therapeutics AL and all of the licenses are being amortized over the stated contractual life.

Grant Revenue

We were awarded several grants under the Therapeutic Discovery Project Grant program during 2010, related to research amounts previously expensed and have historically received modest other grant funding. The total amount recognized within Grant Revenue on the consolidated statement of comprehensive loss was $0 for the three and nine months ended September 30, 2013 and 2012, respectively, and $832 for the period from inception to September 30, 2013.

Stock-based Compensation

We grant stock options to employees and non-employees with an exercise price equal to fair market value, as defined in the Equity Compensation Plan.

In accordance with FASB ASC Topic 718, Stock Compensation, as modified or supplemented, we measure compensation cost for share-based payment awards granted to employees and non-employee directors at fair value using the Black-Scholes option-pricing model. We recognize compensation expense on a straight-line basis over the service period for awards expected to vest. Share-based compensation cost related to share-based payment awards granted to non-employees is adjusted each reporting period for changes in the fair value of our common stock until the measurement date. The measurement date is generally considered to be the date when all services have been rendered or the date that options are fully vested. Expense is recognized over the related service period.

Research and Development

Research and development expenses consist of the costs associated with our research and discovery activities, conducting preclinical studies and clinical trials and activities related to regulatory filings. Research and development expenses consist of:

- employee salaries and related expenses, which include compensation benefits for the personnel involved in drug discovery and development activities, including stock based compensation;
- external research and development expenses incurred under agreements with third party AROs and CROs and investigative sites (see Note 2—Clinical Agreements);
- clinical trial supplies when used or upon determination that they have no alternative future use, and clinical trial supplies shipped to clinical sites for use in clinical studies (see Note 2—Clinical Trial Supplies);
- patent and product license amortization, and license fees for and milestone payments related to in-licensed products and technologies; and
- overhead costs related to facilities, depreciation, research and development management, and research and development support services and supplies.

Conducting a significant amount of research and development is central to our business model. Product candidates in late stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of late stage clinical trials. We plan to increase research and development expenses for the foreseeable future as we seek to complete the development of our lead product candidate, REG1.

Recent Accounting Pronouncements

Section 107 of the Jumpstart our Business Startups Act of 2012, or the JOBS Act, provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, for complying with new or revised accounting standards. In other words, an "emerging growth company" can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. However, we are choosing to "opt out" of such extended transition period, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision to opt out of the extended transition period for complying with new or revised accounting standards is irrevocable.

3. Fair Value of Financial Instruments

The following table (in thousands) sets forth our assets and liabilities that were measured at fair value on a recurring basis at September 30, 2013 and December 31, 2012 by level within the fair value hierarchy. As required by ASC 820-10, assets and liabilities measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement. Our assessment of the significance of a particular input to the fair value measurement in its entirety requires judgment and considers factors specific to the asset or liability.

	As of September 30, 2013				As of December 31, 2012			
Assets and Liabilities	Quoted Prices In Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance as of September 30, 2013	Quoted Prices In Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at December 31, 2012
Assets:								
Money market funds	$ 43,202	$ —	$ —	$ 43,202	$ 14,529	$ —	$ —	$ 14,529
Total assets at fair value	$ 43,202	$ —	$ —	$ 43,202	$ 14,529	$ —	$ —	$ 14,529
Liabilities:								
Warrant liability	$ —	$ —	$ 23	$ 23	$ —	$ —	$ —	$ —
Total liabilities at fair value	$ —	$ —	$ 23	$ 23	$ —	$ —	$ —	$ —

The change in the fair value measurement using significant unobservable inputs (Level 3) is summarized below (in thousands):

Balance at December 31, 2012	$ —
Allocation of long-term debt proceeds to warrant (Unaudited)	79
Change in fair value recorded as interest income (Unaudited)	(65)
Change in fair value recorded as interest expense (Unaudited)	9
Balance at September 30, 2013 (Unaudited)	$ 23

The warrant liability represents our allocation of a portion of the proceeds from the May 2013 Comerica Loan (as defined in Note 6). The allocation of the proceeds from the Comerica Loan was based on the fair value of the warrant liability on the date of grant. We accounted for the warrant liability in accordance with ASC Topic 815, Derivatives and Hedging, which requires that equity contracts not indexed to the issuer's own stock and not meeting the definition of a derivative should be reported as an asset or liability. We utilized the Binomial pricing model to determine the fair value of the warrant liability. We record changes in the fair value of the warrant liability as interest expense or interest income, as applicable.

We used significant assumptions in estimating the fair value of the warrant liability including the estimated volatility, risk free interest rate, estimated fair value of the preferred shares, and the estimated life of the warrant. These assumptions were used to establish an expected set of cash flows which were probability-weighted and discounted to present value to determine a fair value.

4 Commitments

Supply and Manufacturing Agreements

We have agreements with CMOs, to supply bulk drug substances for our product candidates and with third parties to formulate, package and distribute our drug product candidates(see Note 2—Significant Concentrations).

In July 2006, we entered into a supply and service agreement with Agilent Technologies, Inc., which was amended in July 2011, for the manufacture of pegnivacogin and anivamersen bulk drug substance for use in the clinical development of REG1. Drug substances are manufactured pursuant to a Good Manufacturing Practices and a Quality Agreement entered into in May 2010. Manufacture of bulk drug substance lots is on a purchase order basis, with no minimum purchase obligation.

In December 2006, we entered into a license, manufacturing and supply agreement with Nektar Therapeutics, or Nektar, for the supply of polyethylene glycol, or PEG, used in the manufacture of pegnivacogin. We must provide Nektar with a rolling forecast of our anticipated requirements for the succeeding six quarters, with respect to precommercial supply, or eight quarters, with respect to commercial supply, with a required lead time to commit to and guarantee availability of the reagent at an agreed upon pricing structure, which is subject to revision on an annual basis. In addition, we have agreed to pay Nektar specific development and commercial milestones and a specific royalty on product sales (see Milestone and Other Obligations below).

In March 2012, we entered into a clinical supply agreement with Althea Technologies, Inc., for the formulation and primary packaging of pegnivacogin and anivamersen for use in our clinical trials. Drug products are manufactured and the packaging is conducted pursuant to a Good Manufacturing Practices and a Quality Agreement entered into in January 2012. Formulation and packaging services are provided on a purchase order basis, with no minimum purchase obligation.

Clinical Agreements

We have various clinical trial agreements with AROs and CROs for the planning, management and execution of clinical trials. The financial terms of these agreements are subject to negotiation, vary from contract to contract, and may result in uneven payment flows. These contracts generally provide for termination on notice (see Note 2—Clinical Agreements).

Milestone and Other Obligations

Upon the commencement of our REGULATE-PCI trial which occurred in September 2013, we were obligated to make milestone payments of $500,000 to Duke University, or Duke, and $1.0 million to Archemix Corporation, or Archemix. Such amounts are included in accrued expenses in the accompanying balance sheet as of September 30, 2013 and in research and development expense in the accompanying statement of comprehensive loss for the three and nine month periods ended September 30, 2013. In addition, upon the filing of a new drug application, we are obligated to make additional milestone payments to Duke, Archemix and Nektar Therapeutics AL, Corporation. Given the uncertainty of the drug development process, we cannot be certain when, if ever, we will be required to make these milestone payments.

As a condition of closing the Series E Preferred Stock financing in December 2012, we assigned all intellectual property (“IP”) rights and titles in Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kyrgyzstan, Moldova, Russia, Tajikistan, Turkmenistan, Ukraine and Uzbekistan (the “Covered Territory”) to Domain Russia Investments Limited (“DRI”). Additionally, we agreed to assist an affiliate of DRI with certain development support related to the development of the IP. Concurrently with the signing of this agreement, we agreed to make a payment to DRI up to a maximum amount of $1.2 million based on an independent appraiser’s valuation of the IP rights transferred. Such independent appraiser’s valuation was received during 2013, the result of which confirmed that we do not have any further obligation pursuant to this agreement.

License Agreements

In December 2012, in connection with its Series E Preferred Stock financing, we entered into a Technology Transfer Agreement, or the Tech Transfer Agreement, with DRI. In accordance with the terms of the Tech Transfer Agreement, in May 2013 we entered into a Clinical Development and Collaboration Agreement with NovaMedica pursuant to which we agreed to assist NovaMedica in the development and commercialization of our product candidates in the Covered Territory, as defined. NovaMedica is required to reimburse us for any out-of-pocket expenses incurred by us in providing this assistance, including travel-related expenses.

Operating Leases

We maintain three separate office locations. On May 1, 2013, we entered into a lease agreement for 1,657 square feet of administrative office space located in Durham, North Carolina (the "Durham Office Lease"). The lease is for 36 months with annual lease obligations of $39,000, $40,000 and $41,000 for years one through three, respectively. We had rented an administrative office space in Cary, North Carolina under an operating lease agreement effective December 2012, with base monthly payments at $2,000 per month. The six month lease expired in May 2013 and was replaced with the Durham Office Lease. In April 2007, we entered into an operating lease for laboratory space in Durham, North Carolina. The lease expired July 31, 2012, and we are currently on a month-to-month basis with base rent of $11,000 per month. An initial letter of credit of $115,000 was given to the lessor for use of the facility, and the letter of credit was reduced to $46,000 on the fourth anniversary date of the lease. In November 2008, an operating lease was entered into for corporate headquarters in Basking Ridge, New Jersey. The lease expired on November 1, 2011 and is currently on a month-to-month basis. Monthly payments are fixed at $16,000 per month, with an additional $1,000 per month due for utilities.

Rent expense related to operating leases was $114,000 and $130,000 for the three months ended September 30, 2013 and 2012, respectively, $347,000 and $367,000 for the nine months ended September 30, 2013 and 2012, respectively, and $2.8 million for the period from inception to September 30, 2013.

Severance

Selected executive employees of ours have employment or severance agreements which provide for severance payments of up to two times base salary, bonuses and benefits upon termination, depending on the reasons for the termination. Certain other employees of ours have employment or severance agreements which generally provide for six months severance, including base salary, bonuses and benefits upon termination, depending on the reasons for the termination. All employees would be required to execute a release and settlement agreement.

Effective September 26, 2013, we reduced our workforce by eliminating 5 of our 32 full-time employees. The majority of the affected employees worked in drug discovery roles at our laboratory facility in North Carolina. The goal of our reduction in workforce was to enable us to focus management and financial resources on advancing our lead product candidate, REG1, in our REGULATE-PCI trial. In connection with the reduction in workforce, in the third quarter of 2013 we recorded an accrual of approximately $250,000 for incurred severance costs which were charged to research and development expense in the accompanying consolidated statement of comprehensive loss for the three and nine months ended September 30, 2013.

5 Accrued Expenses

The components of accrued expenses are as follows (in thousands):

	September 30, 2013	December 31, 2012
Accrued license milestones	$ 1,500	$ —
Accrued obligations under clinical contracts	1,640	—
Accrued legal and professional services	110	14
Accrued interest	24	40
Accrued compensation and benefits	1,646	478
Deferred revenue	—	100
Accrued expenses, other	108	154
Total accrued expenses	$ 5,028	$ 786

6 Long-term Debt

In May 2011, we entered into a loan and security agreement with MidCap Financial SBIC, LP pursuant to which we borrowed a total of $6.0 million, at the stated rate of LIBOR, at a 2% rate floor, plus 8% spread per annum. The loan was payable in monthly installments beginning September 2012 through August 2014. Our assets (including intellectual property) were collateral for the borrowings, and we were required to pay a 3% final payment of $180,000 regardless of when the loan was paid in full.

On May 13, 2013, we secured a venture debt loan with Comerica Bank (the "Comerica Loan"). We borrowed $4.5 million ("Tranche One"), and the proceeds of the loan were utilized to repay all amounts due to MidCap Financial SBIC, LP. The terms allow for an interest only period of 15 months at a rate of 7.25%, and the remaining principal and interest will be repaid starting September 2014 over a nine-month period (24 months in total). Maturities for 2014 and 2015 are $2.0 million and $2.5 million, respectively. Upon (i) Comerica's receipt of evidence satisfactory to Comerica that the 1,000 patient interim analysis in the REGULATE-PCI study is successful and performed by April 30, 2014 and (ii) our completion of the IPO and receipt of net proceeds of at least $50.0 million prior to June 30, 2013, we had the option to borrow an additional $4.0 million in the second tranche, or ("Tranche Two"). Since the latter of the Tranche Two conditions was not satisfied, Tranche Two is solely at the discretion of Comerica.

Interest expense recorded related to the Comerica Loan was $138,000 and $211,000 for the three and nine months ended September 30, 2013, respectively, and $211,000 for the period from inception to September 30, 2013. Interest expense recorded related to the MidCap Loan was $0 and $138,000 for the three months ended September 30, 2013 and 2012, respectively, $325,000 and $439,000 for the nine months ended September 30, 2013 and 2012, respectively, and $1.2 million for the period from inception to September 30, 2013.

In connection with the funding of Tranche One, we issued to Comerica a warrant to purchase 156,250 shares of the Series E Preferred Stock at a price of $0.72 per share, or the Warrant Price, subject to adjustment for stock splits, combinations, reclassifications or exchanges and certain dilutive issuances. After giving effect to our IPO and reverse stock split, the warrant was adjusted to a warrant to purchase 9,356 shares of our common stock at a price of $12.02 per share (see Note 3).

If we had borrowed the additional $4.0 million in Tranche Two, the warrant would have become exercisable for an additional number of shares of the Series E Preferred Stock equal to 100,000 divided by the Warrant Price, as defined, subject to adjustment for stock splits, combinations, reclassifications or exchanges and certain dilutive issuances. As previously noted, as we did not complete an IPO by the required milestone date, Tranche Two is solely at the discretion of Comerica.

Debt consists of (in thousands):

	September 30, 2013	December 31, 2012
Long-term debt	$ 4,500	$ 4,500
Less - unamortized discount	(127)	(—)
Less - current portion	(500)	(2,571)
Plus - fees due at closing(1)	33	—
Long-term debt, net	$ 3,906	$ 1,929

(1) On the date that all of the principal and interest of the Comerica Loan become due and payable, we must pay an end of term fee of $173,000 (the "Final Fee"). The Final Fee is being accreted to interest expense over the term of the Comerica Loan.

Included in the consolidated balance sheets within long-term debt are $85,000 in loan origination costs that are being amortized to interest expense over the remaining life of the Comerica Loan using the effective interest rate method.

In accounting for the Comerica Loan, the loan was separated into debt and warrant liability components. We utilized the Binomial pricing model to determine the fair value of the warrant liability component (see Note 3). The carrying amount of the debt component was determined by deducting the fair value of the warrant liability component from the par value of the Comerica Loan as a whole. The excess of the principal amount of the Comerica Loan component over its carrying amount, referred to as the debt discount, is amortized to interest expense over the term of the loan. The warrant liability component is re-measured at each reporting date and changes in the fair value of the warrant liability are recorded as interest expense or interest income, as applicable.

In accounting for the transaction costs related to the issuance of the Comerica Loan, we allocated the total costs incurred to the debt and warrant liability components of the Comerica Loan based on their relative values. Transaction costs attributable to the debt component are amortized to interest expense over the term of the Comerica Loan, and transaction costs attributable to the warrant liability component were immediately expensed.

7 Stockholders' Equity

IPO

We completed our initial public offering ("IPO") in August 2013. Inclusive of the underwriters' exercise of the over-allotment option in connection with the IPO in September 2013, we issued 11,671,500 shares of common stock, at a price of $4.00 per share, resulting in net proceeds to us of approximately $41.1 million after deducting underwriting discounts of $3.3 million and offering costs of $2.3 million. Pursuant to the IPO all shares of convertible preferred stock then outstanding automatically converted into an aggregate of 9,396,767 shares of common stock.

Tranching

On December 18, 2012, the first tranche of the Series E financing was completed with 31,437,442 preferred shares issued for $22.6 million. This included $15.5 million in cash plus $6.8 million in convertible loan principal and $339,000 in converted loan interest originally secured on May 3, 2012.

Per the Series E financing agreement executed on December 18, 2012, a second financing tranche of $10.3 million for 14,320,168 shares of Series E preferred stock took place on March 22, 2013. On March 22, 2013 a portion of the second tranche of the Series E financing was completed with 7,160,084 preferred shares issued for $5.2 million. The RMI Investments, S.a.r.l ("RMI") portion of the second tranche totaling $5.2 million was delivered into an escrow account at the time of the second tranche, and the RMI funds and Series E shares relating to the RMI investment were released on April 26, 2013.

The agreement also provided that a third financing tranche of $17.8 million for 24,770,476 shares of Series E preferred stock would take place on or before January 17, 2014. However, pursuant to the terms of a Termination Agreement entered into by the parties to the Series E Purchase Agreement, our obligation to sell additional shares of Series E Preferred Stock to the investors and the obligations of the investors to purchase additional shares of Series E Preferred Stock terminated immediately prior to the consummation of the IPO and no additional shares of Series E Preferred Stock will be sold pursuant thereto.

Reverse Stock Split

In May 2013, we executed an amendment to our Fifth Amended and Restated Certificate of Incorporation instituting a one–for-16.7 reverse split of common stock and an increase in the number of shares of common stock we are authorized to issue to 500,000,000.

Warrants

See Notes 3 and 6 regarding our issuance of a warrant for Series E Preferred Stock in connection with obtaining the Comerica Loan. As of September 30, 2013 and 2012, we had 16,332 and 20,449 of warrants outstanding, respectively, that were exercisable into common shares at a weighted average price of $6.96 and $0.17 per share, respectively, at the option of the warrant holder. During the nine months ended September 30, 2013, a warrant for 13,473 shares of common stock was exercised at an exercise price of $0.17.

8 Stock Based Compensation

Equity Compensation Plans

The 2013 Equity Compensation Plan (the "2013 Plan") adopted by our Board of Directors in May 2013, became effective upon consummation of the IPO in August 2013. There are 3,342,839 common shares authorized for future issuance under the 2013 Plan of which 558,449 were available as of September 30, 2013. Upon effectiveness of the 2013 Plan, stock options outstanding under the 2004 Equity Compensation Plan (the "2004 Plan") to acquire 1,365,478 shares of our common stock were assumed under the 2013 Plan, leaving stock options to acquire 34,342 shares of our common stock outstanding under the 2004 Plan. There will be no further awards made under the 2004 Plan.

Stock Options

The following table summarizes our aggregate Equity Compensation Plan activity:

	Number of Options	Weighted Average Exercise Price	Weighted Average Contractual Term (in years)	Aggregate Intrinsic Value (1)
Outstanding - December 31, 2012	1,409,659			
Granted	1,424,688			
Exercised	—			
Forfeited	(6,747)			
Expired	(8,868)			
Outstanding - September 30, 2013	2,818,732	$ 5.50	7.8	$ 6,259,849
Exercisable - September 30, 2013	1,265,907	$ 7.32	5.3	$ 2,020,523
Vested and expected to vest at September 30, 2013(2)	2,798,327	$ 5.51	7.8	$ 6,204,320

(1) Intrinsic value is the excess of the fair value of the underlying common shares as of September 30, 2013 over the weighted-average exercise price.
(2) The number of stock options expected to vest takes into account an estimate of expected forfeitures.

We use the Black-Scholes-Merton option pricing model to determine the fair value of our stock options. The determination of the fair value of stock-based payment awards on the date of grant using an option pricing model is affected by our stock price, as well as assumptions regarding a number of complex and subjective variables. These variables include our expected stock price volatility over the term of the awards, risk-free interest rate, actual employee exercise behaviors and expected dividends.

The following table shows the assumptions used to value stock options on the date of grant, as follows:

	Nine Months Ended September 30, 2013
Expected stock price volatility	43.29-43.34%
Risk-free interest rate	0.38-1.21%
Expected life of option (in years)	2-4
Weighted-average grant date fair value per share of options granted	$4.03
Estimated dividend yield	0.0%

Expected stock price volatility was calculated based on the weighted-average of historical information of similar public entities. We will continue to use a weighted-average approach using other similar public entities' volatility information until our historical volatility is relevant to measure expected volatility for future option grants. The risk-free rate was based on the U.S. Treasury yield curve in effect at the time of grant commensurate with the expected life assumption. The average expected life was determined using two experience groups based on anticipated exercise strategy and cancellation behavior for each group. A forfeiture rate of 1% and 0% was used for each experience group respectively. We have not paid and do not anticipate paying cash dividends; therefore, the expected dividend rate was assumed to be 0%.

Stock-Based Compensation Expense

Total stock-based compensation expense recognized based on the total grant date fair value of options vested was approximately $141,000 and $158,000 for the three months ended September 30, 2013 and 2012, respectively, and $346,000 and $578,000 for the nine months ended September 30, 2013 and 2012, respectively, and $5.0 million for the period from inception to September 30, 2013.

As of September 30, 2013, approximately $1.9 million of total unrecognized compensation cost related to unvested share options is expected to be recognized over a weighted-average period of 2.99 years.

9 Income Taxes

We estimate an annual effective tax rate of 0% for the year ending December 31, 2013 as the Company incurred losses for the three and nine-month periods ended September 30, 2013 and is forecasting additional losses through the fourth quarter, resulting in an estimated net loss for both financial statement and tax purposes for the year ending December 31, 2013. Therefore, no federal or state income taxes are expected and none have been recorded as of September 30, 2013. Income taxes have been accounted for using the liability method in accordance with FASB ASC 740.

Due to our history of losses since inception, there is not enough evidence at this time to support that we will generate future income of a sufficient amount and nature to utilize the benefits of its net deferred tax assets. Accordingly, the deferred tax assets have been reduced by a valuation allowance, since it has been determined that it is more likely than not that all of the deferred tax assets will not be realized.

10 Net Loss Per Share

Basic net loss per share is calculated by dividing the net loss by the weighted average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by dividing the net loss by the weighted-average number of common stock equivalents outstanding for the period determined using the treasury-stock method. Dilutive common stock equivalents are comprised of convertible preferred stock, options outstanding under our stock option plan and warrants. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted shares outstanding due to our net loss position.

The following table presents the computation of basic and diluted net loss per common share (in thousands, except share and per share data):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Net loss attributable to common shareholders	$ (11,415)	$ (2,717)	$ (20,308)	$ (10,321)
Weighted average common shares outstanding, basic and diluted	8,005,142	221,272	2,828,757	221,272
Net loss per share attributable to common shareholders, basic and diluted	$ (1.43)	$ (12.28)	$ (7.18)	$ (46.64)

Securities that may potentially dilute earnings per share in the future that have not been included in the calculation of diluted net loss per share because to do so would be anti-dilutive are as follows (in common equivalent shares):

	Three months ended September 30,		Nine months ended September 30,	
	2013	2012	2013	2012
Convertible preferred stock	—	6,656,799	—	6,656,799
Common stock options	2,818,732	1,410,415	2,818,732	1,410,415
Warrants	16,332	20,449	16,332	20,449
Total	2,835,064	8,087,663	2,835,064	8,087,663

In August 2013, all convertible preferred stock converted into common stock in conjunction with the consummation of our IPO. As of September 30, 2013 and 2012, we had 16,332 and 20,449 of warrants outstanding, respectively, that were exercisable into common shares at a weighted average price of $6.96 and $0.17 per share, respectively, at the option of the warrant holder.

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

Management's Discussion and Analysis of Financial Condition and Results of Operations is intended to help the reader understand the results of operations and financial condition of the Company. The interim financial statements included in this report and this Management's Discussion and Analysis of Financial Condition and Results of Operations should be read in conjunction with our audited consolidated financial statements and notes thereto for the year ended December 31, 2012, and the related Management's Discussion and Analysis of Financial Condition and Results of Operations, both of which are contained in our prospectus dated August 22, 2013 filed pursuant to Rule 424(b) under the Securities Act of 1933, as amended, or the Securities Act, with the Securities and Exchange Commission on August 22, 2013 (the "IPO Prospectus"). In addition to historical information, this Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, which are intended to be covered by the safe harbors created thereby. See "Cautionary Note Regarding Forward-Looking Statements" in this report. Our actual results and the timing of events could differ materially from those discussed in our forward-looking statements as a result of many factors, including those set forth under the "Part II – Item 1A Risk Factors" section and elsewhere in this report, as well as, in other reports and documents we file with the Securities and Exchange Commission from time to time. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances occurring after the date of this Quarterly Report on Form 10-Q.

Overview

We are a biopharmaceutical company focused on the discovery and development of novel, first-in-class, actively controllable antithrombotic drug systems for acute and sub-acute cardiovascular indications. We are pioneering the discovery and development of two-component drug systems consisting of a therapeutic aptamer and its specific active control agent. Our actively controllable product candidates have the potential to improve outcomes, enhance the patient experience and reduce overall treatment costs.

Our lead product candidate, REG1, consists of pegnivacogin, a highly potent and selective FIXa inhibiting anticoagulant, and anivamersen, its specific active control agent. We are developing REG1 as an anticoagulant for use in patients with a wide variety of cardiovascular conditions undergoing percutaneous coronary intervention, or PCI, a hospital-based procedure used to mechanically open or widen obstructed coronary arteries. Interventional cardiologists performing PCIs must consider the risk of major bleeding events in determining the level of anticoagulation administered to patients to prevent ischemic events, including death, stroke, myocardial infarction, or MI, or the need for revascularization of the artery. Because the anticoagulant effect of existing drugs persists long after administration, intervention cardiologists are forced to make a compromising medical decision because they lack the means to simultaneously reduce the risks of ischemic and major bleeding events. In 2005, we filed an investigational new drug application, or IND, for the use of REG1 in this initial indication. REG1 has been studied in three Phase 1 trials involving a total of 174 subjects and one Phase 2a proof-of-concept PCI trial involving 26 subjects. In November 2010, we completed a randomized, partially blinded, dose-ranging Phase 2b trial of REG1 involving 640 subjects, or the RADAR trial. In September 2013, we commenced our single, open-label, 13,200 subjects Phase 3 trial of REG1 in patients undergoing PCI (excluding ST elevated myocardial infarction, or STEMI), or the REGULATE-PCI trial.

We completed our initial public offering ("IPO") in August 2013. Inclusive of the underwriters' exercise of the over-allotment option in connection with the IPO in September 2013, we issued 11,671,500 shares of common stock at a price of $4.00 per share, resulting in net proceeds of approximately $41.1 million, after deducting underwriting discounts of $3.3 million and offering costs of $2.3 million. Pursuant to the IPO all shares of convertible preferred stock then outstanding automatically converted into an aggregate of 9,396,767 shares of common stock.

We are not profitable and do not expect to be profitable in the foreseeable future. We have suffered negative cash flows from operating activities of $22.1 million during the nine months ended September 30, 2013 and a net accumulated deficit of $130.8 million since inception as of September 30, 2013. We have devoted most of our financial resources to research and development, including our preclinical development activities and clinical trials. We have not completed development of any product candidate and we have therefore not generated any revenues from product sales. As a result, we expect to continue to incur net losses and negative cash flows for the foreseeable future. These net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. Our recurring losses from operations raise substantial doubt about our ability to continue as a going concern, and as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our financial statements as of and for the year ended December 31, 2012 with respect to this uncertainty. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve or maintain profitability.

Financial Operations Overview

Revenue

To date, we have not generated any product revenue. Our ability to generate product revenue, which we do not expect will occur for several years, if ever, will depend heavily on the successful development and eventual commercialization of our lead product candidate, REG1.

Research and Development Expenses

Research and development expenses consist of the costs associated with our research and discovery activities, conducting preclinical studies and clinical trials and activities related to regulatory filings. Our research and development expenses consist of:

- employee salaries and related expenses, which include all compensation benefits for the personnel involved in our drug discovery and development activities, including stock based compensation;
- external research and development expenses incurred under agreements with third party AROs and CROs and investigative sites;
- clinical trial supplies when used or upon determination that they have no alternative future use and clinical trial supplies shipped to clinical sites for use in clinical studies;
- license fees for and milestone payments related to in-licensed products and technologies; and
- overhead costs related to facilities, depreciation, research and development management, and research and development support services and supplies.

We expense research and development costs as incurred, with the exception of materials purchased and/or manufactured for use in clinical trials. We capitalize clinical trial supplies, which are comprised of materials that will be used in our clinical trials that also have an alternative future use in either ongoing or future clinical research or development projects. Capitalized clinical trial supplies that are determined to be unsuitable for future use are immediately expensed to research and development; otherwise, clinical trial supplies are expensed to research and development when shipped to clinical sites for use in clinical studies or when used in other research and development projects. Costs for clinical agreements, including ARO and CRO contracts, are recognized based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations, or information provided by vendors on their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in our financial statements as prepaid or accrued expenses.

Conducting a significant amount of research and development is central to our business model. Product candidates in late stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of late stage clinical trials. We expect our research and development expenses to increase in future periods for the foreseeable future as we seek to complete development of our lead product candidate, REG1, and to further develop our other product candidates.

We incurred aggregate research and development expenses of approximately $9.6 million and $1.6 million for the three months ended September 30, 2013 and 2012, respectively, $15.6 million and $6.5 million for the nine months ended September 30, 2013 and 2012, respectively, and $97.6 million since inception as of September 30, 2013. We expect to incur increased expenses primarily related to our REGULATE-PCI trial.

We track direct external development expenses and direct personnel expenses on each indication for our product candidates. Substantially all of our research and development expenses for REG1 have related to its initial indication, although we expect certain of the data obtained will support development of additional REG1 indications as well as the development of REG2. Indirect expenses, such as, overhead costs related to facilities, depreciation, research and development management, and research and development support services and supplies are not allocated to specific product candidates or indications, because the development projects tend to vary from period to period and internal resources are utilized across and benefit multiple programs over any given period of time. The following table is a summary of our research and development expenses for the three and nine months ended September 30, 2013 and 2012 and for the period from inception to September 30, 2013 (in thousands):

	Three months ended September 30		Nine months ended September 30		Period from Inception (December 19, 2001) to September 30, 2013
	2013	2012	2013	2012	
REG1	$ 8,777	$ 1,065	$13,466	$4,863	$ 78,695
REG3	244	96	487	294	6,180
REG2	177	81	383	250	4,561
Total direct expenses	9,198	1,242	14,366	5,407	89,436
Indirect expenses	399	365	1,258	1,112	8,119
Total research and development expense	$ 9,597	$ 1,607	$15,594	$6,519	$ 97,555

The successful development of our clinical and preclinical product candidates is highly uncertain. At this time, we can only reasonably estimate the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of any of our product candidates or the period, if any, in which material net cash inflows from those product candidates may commence. Our estimates are based on reasonable assumptions, past performance, experience and existing contracts. However, unforeseen changes may occur at any time due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the number of sites included in the trials;
- the number of countries included in the trials;
- the ability to recruit subjects to participate in the trial;
- the per subject trial costs;
- the length of time required to enroll suitable subjects, achieve interim milestones and complete clinical trials; and
- the cost and timeliness of obtaining clinical trial supplies.

Development timelines, probability of success and development costs vary widely. As a result of the uncertainties discussed above, we anticipate that we will make determinations as to which product candidates and indications to pursue and how much funding to direct to each product candidate and indication on an ongoing basis. Accordingly, we cannot currently estimate with any degree of certainty the amount of time or money that we will be required to expend in the future on the research and development of our product candidates.

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related benefit costs, including stock-based compensation for administrative personnel. Other general and administrative expenses include facility costs, and professional fees for legal, consulting, auditing and tax services. We anticipate that our general and administrative expenses will increase in future periods to support increases in our research and development activities and as a result of increased headcount, expanded infrastructure, and increased legal, compliance, accounting and investor and public relations expenses associated with being a public company.

Interest Income (Expense)

Interest income consists of interest earned on our cash and cash equivalents, as well as, fair value adjustments related to our warrant liability. We expect our interest income to increase as we invest the net proceeds from our IPO pending their use in our operations. Interest expense in 2013 primarily relates to the Comerica Loan (as described more fully under “Liquidity and Capital Resources”) and to our loan with MidCap Financial SBIC, LP, or MidCap, which was paid in full with proceeds from the Comerica Loan. Interest expense in 2012 related to interest incurred on our MidCap loan and on our convertible notes.

Critical Accounting Policies and Significant Judgments and Estimates

A “critical accounting policy” is one that is both important to the portrayal of our financial condition and results of operations and that requires management’s most difficult, subjective or complex judgments. Such judgments are often the result of a need to make estimates about the effect of matters that are inherently uncertain. The preparation of our financial statements in conformity with accounting principles generally accepted in the United States applicable to interim financial reporting requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results could differ materially from those estimates. There were no significant changes in critical accounting policies from those at December 31, 2012 except for the addition of the warrant liability (see Note 3 to the financial statements for further information). During the nine months ended September 30, 2013, we consistently applied the critical accounting policies discussed in the IPO Prospectus, which contained our financial statements for the years ended December 31, 2012 and 2011. For a complete discussion regarding these critical accounting policies, refer to the IPO Prospectus.

Results of Operations

Effective September 26, 2013, we reduced our workforce by eliminating 5 of our 32 full-time employees. The majority of the affected employees worked in drug discovery roles at our laboratory facility in North Carolina. The goal of our reduction in workforce was to enable us to focus management and financial resources on advancing our lead product candidate, REG1, in our REGULATE-PCI trial. In connection with the reduction in workforce, we incurred cash charges of approximately $250,000 in severance costs which were recorded in the third quarter of 2013. We believe that the reduction in workforce will result in annualized savings of approximately $625,000 beginning in the second quarter of 2014.

Three Months Ended September 30, 2013 and 2012

The following table sets forth certain information concerning our results of operations for the periods shown (in thousands):

	Three Months Ended September 30,		
	2013	2012	Increase (Decrease)
Operating expenses:			
Research and development	$ (9,597)	$(1,607)	$ 7,990
General and administrative	(1,614)	(974)	640
Total operating expenses	(11,211)	(2,581)	8,630
Other (expense) income:			
Interest income	2	1	1
Interest expense	(146)	(137)	9
Other expense	(60)	—	60
Total other (expense) income	(204)	(136)	68
Net loss	$(11,415)	$(2,717)	$ 8,698

Research and Development Expenses

Research and development expenses increased by $8.0 million for the three months ended September 30, 2013 compared to the three months ended September 30, 2012 due to the start of the REGULATE-PCI trial, including commencement of services provided by AROs and CROs, and to $1.5 million in accrued license agreement obligations which obligations were triggered upon the commencement of our REGULATE-PCI trial in September 2013.

General and Administrative Expenses

General and administrative expenses increased by $640,000 for the three months ended September 30, 2013 compared to the three months ended September 30, 2012. The increase was primarily due to market analysis costs for trademark research and increased insurance and other administrative costs associated with becoming a public company.

Other Income (Expense)

Interest income increased by $1,000 for the three months ended September 30, 2013, compared to the three months ended September 30, 2012 as a result of the IPO proceeds.

Interest expense increased by $9,000 for the three months ended September 30, 2013 compared to the three months ended September 30, 2012. The increase was primarily due to the fair value adjustment for our warrant liability.

Other expense increased by $60,000 for the three months ended September 30, 2013, compared to the three months ended September 30, 2012 from costs associated with the sale of patents.

Nine Months Ended September 30, 2013 and 2012

The following table sets forth certain information concerning our results of operations for the periods shown (in thousands):

	Nine Months Ended September 30,		
	2013	2012	Increase (Decrease)
Operating expenses:			
Research and development	$(15,594)	$ (6,519)	$ 9,075
General and administrative	(4,240)	(3,368)	872
Total operating expenses	(19,834)	(9,887)	9,947
Other (expense) income:			
Interest income	71	4	67
Interest expense	(545)	(438)	107
Total other (expense) income	(474)	(434)	40
Net loss	$(20,308)	$(10,321)	$ 9,987

Research and Development Expenses

Research and development expenses increased by $9.1 million for the nine months ended September 30, 2013 compared to the nine months ended September 30, 2012 due to the start of the REGULATE-PCI trial, including commencement of services provided by AROs and CROs, and to $1.5 million in accrued license agreement obligations which obligations were triggered upon the commencement of our REGULATE-PCI trial in September 2013.

General and Administrative Expenses

General and administrative expenses increased by $872,000 for the nine months ended September 30, 2013 compared to the nine months ended September 30, 2012 primarily due to market analysis costs for trademark research and increased insurance and other administrative costs associated with becoming a public company.

Other Income (Expense)

Interest income increased by $67,000 for the nine months ended September 30, 2013 compared to the nine months ended September 30, 2012 primarily due to a fair value adjustment related to our warrant liability.

Interest expense increased by $107,000 for the nine months ended September 30, 2013 compared to the nine months ended September 30, 2012. The increase was primarily due to the final fee payment of $192,000 related to the MidCap Financial loan, partially offset by lower interest charges in the 2013 period compared to the comparable period in 2012.

Liquidity and Capital Resources

Sources of Liquidity

To date, we have not generated any product revenue. We have funded our operations to date through sales of our equity and debt securities, bank borrowings and government grants. As of September 30, 2013, we had $43.5 million in cash and cash equivalents.

We completed our IPO in August 2013. Inclusive of the underwriters' exercise of the over-allotment option in connection with the IPO in September 2013, we issued 11,671,500 shares of common stock at a price of $4.00 per share, resulting in net proceeds of approximately $41.1 million, after deducting underwriting discounts of $3.3 million and offering costs of $2.3 million. Upon closing of the IPO, all shares of convertible preferred stock then outstanding automatically converted into an aggregate of 9,396,767 shares of common stock.

From inception through September 30, 2013, we received net cash proceeds of $5.8 million from the sale of our Series A Preferred Stock, $19.9 million from sales of our Series B Preferred Stock, $23.0 million from sales of our Series C Preferred Stock, $51.2 million from sales of our Series D Preferred Stock and $32.3 million from sales of our Series E Preferred Stock. Convertible note proceeds of $14.9 million are included in these totals, as well as convertible notes that have been converted to convertible preferred stock as of September 30, 2013.

Comerica Loan

In May 2013, we entered into a Loan and Security Agreement, or the Loan Agreement, with Comerica Bank, or Comerica. Pursuant to the terms of the Loan Agreement, we were initially eligible to borrow $4.5 million in an initial tranche, or Tranche One. Upon Comerica's receipt of evidence satisfactory to Comerica that (i) the 1,000 patient interim analysis in the REGULATE-PCI study is successful and performed by

April 30, 2014 and (ii) upon our completion of the IPO and receipt of net proceeds of at least $50 million prior to September 30, 2013, we had the option to borrow an additional $4 million in the second tranche, or Tranche Two. Since the Tranche Two conditions were not satisfied, Tranche Two is solely at the discretion of Comerica.

The Comerica loan bears interest at Comerica's Prime Reference Rate (as defined in the Loan Agreement) subject to a floor of 30 day LIBOR plus 250 basis points plus 4.0%. The Comerica loan is interest-only until September 1, 2014. We must repay the principal amount in nine approximately equal consecutive monthly installments commencing on September 1, 2014. The loan matures on May 10, 2015.

In connection with the funding of Tranche One, we issued a warrant to Comerica, or the Comerica Warrant, to purchase 156,250 shares of our Series E Preferred Stock at a price of $0.72 per share, or the Warrant Price, subject to adjustment for stock splits, combinations, reclassifications or exchanges and certain dilutive issuances. After giving effect to our IPO and reverse stock-split, the Comerica Warrant was adjusted to a warrant to purchase 9,356 shares of our common stock at a price of $12.02 per share (the "Adjusted Warrant Price"), respectively. If Comerica, in its sole discretion, permits us to borrow the additional $4 million in Tranche Two, the Comerica Warrant will become exercisable for an additional number of shares of our common stock equal to 5,988 divided by the Adjusted Warrant Price.

The proceeds of Tranche One were used to repay in full amounts outstanding under our loan and security agreement with MidCap Financial SBIC, LP which has been terminated, and for general operating purposes.

Under the terms of the Loan Agreement, we granted Comerica a first priority security interest in substantially all of our assets other than our intellectual property. The Loan Agreement does not contain any ongoing financial covenants.

The Loan Agreement provides that upon the occurrence of and during a period of default as defined therein, interest on the loan will accrue at a penalty rate. Upon the occurrence and during the continuance of a default, Comerica may, at its election, make all obligations under the Loan Agreement immediately due and payable, cease advancing money or extending credit, exercise its right of setoff, foreclose on our assets, dispose of collateral at a public or private sale, and exercise any other remedies available to a secured creditor at law or in equity.

Cash Flows

Our net cash flow from operating, investing and financing activities for the periods below were as follows (in thousands):

	Nine Months Ended September 30,	
	2013	**2012**
Net cash provided by (used in):		
Operating activities	$(22,126)	$(10,568)
Investing activities	(392)	(271)
Financing activities	51,210	5,628
Net increase (decrease) in cash and cash equivalents	$ 28,692	$ (5,211)

Operating Activities

Net cash used in operating activities was $22.1 million for the nine months ended September 30, 2013 and $10.6 million for the nine months ended September 30, 2012. Net cash used in operating activities for the nine months ended September 30, 2013 principally resulted from our net loss of $20.3 million and a higher level of prepaid expenses and other assets related to the REGULATE-PCI trial, partially offset by increases in accounts payable and accrued expenses. Net cash used in operating activities for the nine months ended September 30, 2012 principally resulted from our net loss of $10.3 million and a higher level of other assets (primarily comprised of clinical supplies), partially offset by a decrease in prepaid expenses.

Investing Activities

Net cash used in investing activities was $392,000 for the nine months ended September 30, 2013 and $271,000 for the nine months ended September 30, 2012. Net cash used in investing activities for the nine months ended September 30, 2013 and 2012 principally resulted from the acquisition of intellectual property rights.

Financing Activities

Net cash provided by financing activities was $51.2 million for the nine months ended September 30, 2013 and $5.6 million for the nine months ended September 30, 2012. Net cash provided by financing activities for the nine months ended September 30, 2013 resulted primarily from $41.1 million in net proceeds from the IPO, and to $10.2 million in net proceeds from the sale of Series E Preferred Stock. Net cash provided by financing activities for the nine months ended September 30, 2012 principally resulted from the receipt of $6.8 million in proceeds from issuing convertible notes payable, partially offset by note repayments on the MidCap loan.

Funding Requirements

We have not completed development of any of our product candidates. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We anticipate that our expenses will increase substantially as we:

- conduct our REGULATE-PCI trial;
- continue the research and development activities for our other product candidates;
- seek to discover additional product candidates;
- seek regulatory approvals for our product candidates that successfully complete clinical trials;
- establish a sales, marketing and distribution infrastructure if we do not secure a strategic partner to commercialize products for which we may obtain regulatory approval;
- increase manufacturing capabilities in preparation for commercial launch of any such products; and
- add operational, financial and management information systems and personnel, including personnel to support our product development, planned commercialization efforts and our operation as a public company.

We believe that our existing cash and cash equivalents will enable us to fund our REGULATE-PCI trial through the first interim analysis, which we expect to occur by the beginning of the second quarter of 2014, and to fund our operations through the second quarter of 2014. We need to raise additional financing in the near term to operate our business and fund our REGULATE-PCI trial to completion, which financing may not be available on favorable terms, or at all. Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed in the section entitled “Risk Factors” and elsewhere in this report. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect.

Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of preclinical studies and clinical trials for our product candidates and potential product candidates, including our REGULATE-PCI trial, and the continued development of our other product candidates;
- the number and characteristics of product candidates that we pursue;
- the terms and timing of any future collaboration, licensing or other arrangements that we may establish;
- the outcome, timing and cost of regulatory approvals;
- the cost of obtaining, maintaining, defending and enforcing intellectual property rights, including patent rights;
- the effect of competing technological and market developments;
- the cost and timing of completing commercial-scale outsourced manufacturing activities;
- market acceptance of any product candidates for which we may receive regulatory approval;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval; and
- the extent to which we acquire, license or invest in businesses, products or technologies.

Until we can generate a sufficient amount of revenue from our product candidates, if ever, we expect to finance future cash needs through public or private equity offerings, debt financings or corporate collaborations and licensing arrangements. Additional funds may not be available when we need them on terms that are acceptable to us, or at all. If adequate funds are not available, we may be required to delay our REGULATE-PCI trial, reduce the scope of or eliminate one or more of our research or development programs or our commercialization efforts. To the extent that we raise additional funds by issuing equity securities, our stockholders may experience additional dilution, and debt financing, if available, may involve restrictive covenants. To the extent that we raise additional funds through collaborations and licensing arrangements, it may be necessary to relinquish some rights to our technologies or our product candidates or grant licenses on terms that may not be favorable to us. We may seek to access the public or private capital markets whenever conditions are favorable, even if we do not have an immediate need for additional capital at that time.

We do not expect REG1 to be commercially available before 2017, if at all. The net proceeds of our IPO will not be sufficient for us to complete the REGULATE-PCI trial and we will need to raise substantial additional capital to complete the development and commercialization of REG1. We also will need to raise substantial additional capital to complete the development and commercialization of REG1 for additional indications and for our other product candidates. Since successful development of our product candidates is uncertain, we are unable to estimate the actual funds required to complete research and development and commercialize our products under development.

Future Contractual Obligations and Commitments

The following table (in thousands) reflects a summary of our estimates of future contractual obligations as of September 30, 2013. The information in the table reflects future unconditional payments and is based on the terms of the relevant agreements, appropriate classification of items under U.S. GAAP as currently in effect and certain assumptions, such as the interest rate on our variable debt that was in effect as of September 30, 2013. Future events could cause actual payments to differ from these amounts.

	1-3 Years
Principal(1)	$ 4,500
Interest(2)	515
Fees	173
Total	$ 5,188

(1) In May 2013, we obtained a $4.5 million loan from Comerica for working capital and general business purposes. See “Liquidity and Capital Resources—Comerica Loan.”

(2) The loan bears interest at Comerica’s prime reference rate subject to a floor of 30-day LIBOR plus 250 basis points plus 4.0% and matures on May 10, 2015.

Supply and Manufacturing Agreements

In July 2006, we entered into a supply and service agreement with Agilent Technologies, Inc., which was amended in July 2011, for the manufacture of pegnivacogin and anivamersen bulk drug substance for use in the clinical development of REG1. Drug substances are manufactured pursuant to a Good Manufacturing Practices and a Quality Agreement entered into in May 2010. Manufacture of bulk drug substance lots is on a purchase order basis, with no minimum purchase obligation.

In December 2006, we entered into a license, manufacturing and supply agreement with Nektar Therapeutics, or Nektar, for the supply of polyethylene glycol, or PEG, used in the manufacture of pegnivacogin. We must provide Nektar with a rolling forecast of our anticipated requirements for the succeeding six quarters, with respect to precommercial supply, or eight quarters, with respect to commercial supply, with a required lead time to commit to and guarantee availability of the reagent at an agreed upon pricing structure, which is subject to revision on an annual basis.

In March 2012, we entered into a clinical supply agreement with Althea Technologies, Inc., for the formulation and packaging of pegnivacogin and anivamersen drug product for use in our clinical trials. Drug products are manufactured and the packaging is conducted pursuant to a Good Manufacturing Practices and a Quality Agreement entered into in January 2012. Formulation and packaging services are provided on a purchase order basis, with no minimum purchase obligation.

Clinical Agreements

We have various clinical trial agreements with AROs and CROs for planning, management and execution of clinical trials. The financial terms of these agreements are subject to negotiation, vary from contract to contract, and may result in uneven payment flows. These contracts generally provide for termination on notice.

Milestone and Other Obligations

Upon the commencement of our REGULATE-PCI trial which occurred in September 2013, we were obligated to make milestone payments of $500,000 to Duke University, or Duke, and $1.0 million to Archemix Corporation, or Archemix. Such amounts are included in accrued expenses in the accompanying balance sheet as of September 30, 2013 and in research and development expense in the accompanying statement of comprehensive loss for the three and nine month periods ended September 30, 2013. In addition, upon the filing of a new drug application, we are obligated to make additional milestone payments to Duke, Archemix and Nektar Therapeutics AL, Corporation. Given the uncertainty of the drug development process, we cannot be certain when, if ever, we will be required to make these milestone payments.

As a condition of closing the Series E Preferred Stock financing in December 2012, we assigned all intellectual property ("IP") rights and titles in Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kyrgyzstan, Moldova, Russia, Tajikistan, Turkmenistan, Ukraine and Uzbekistan (the "Covered Territory") to Domain Russia Investments Limited ("DRI"). Additionally, we agreed to assist an affiliate of DRI with certain development support related to the development of the IP. Concurrently with the signing of this agreement, we agreed to make a payment to DRI up to a maximum amount of $1.2 million based on an independent appraiser's valuation of the IP rights transferred. Such independent appraiser's valuation was received during 2013, the result of which confirmed that we do not have any further obligation pursuant to this agreement.

License Agreements

In December 2012, in connection with its Series E Preferred Stock financing, we entered into a Technology Transfer Agreement, or the Tech Transfer Agreement, with DRI. In accordance with the terms of the Tech Transfer Agreement, in May 2013 we entered into a Clinical Development and Collaboration Agreement with NovaMedica pursuant to which we agreed to assist NovaMedica in the development and commercialization of our product candidates in the Covered Territory, as defined. NovaMedica is required to reimburse us for any out-of-pocket expenses incurred by us in providing this assistance, including travel-related expenses.

Lease Obligations

As of September 30, 2013, we did not have long-term lease agreements in effect for our laboratory facility in North Carolina and our office in New Jersey; we are currently leasing these facilities on a month-to-month basis. On May 1, 2013, we entered into a three-year lease for administrative office space in North Carolina. Annual rent under this lease is $39,000 for the first year, $40,000 for the second year and $41,000 for the third year.

Tax Loss Carryforwards

At December 31, 2012, we evaluated and assessed the expected near-term utilization of net operating loss carryforwards, book and taxable income trends, available tax strategies, and the overall deferred tax position. We believe that it is more likely than not that the benefit related to the deferred tax assets will not be realized, therefore we established the valuation allowance required as of December 31, 2012. If actual results differ from the assumptions made in our evaluation, we may record a change in the valuation allowance through income tax expense in the period such determination is made. We believe that our judgments and estimates are reasonable; however, actual results could differ. Our effective tax rate is zero due to continued taxable losses, which generate deferred tax assets which are offset in their entirety by related valuation allowances, due to the uncertainty in realizing these tax benefits. As such, we do not record quarterly accruals for corporate taxes. Deferred income tax assets and liabilities are calculated and reported at year end.

Off-Balance Sheet Arrangements

Since inception, we have not engaged in any off-balance sheet arrangements as defined in Item 303(a)(4) of Regulation S-K.

Recent Accounting Pronouncements

Section 107 of the Jumpstart our Business Startups Act of 2012, or the JOBS Act, provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, for complying with new or revised accounting standards. In other words, an "emerging growth company" can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. However, we are choosing to "opt out" of such extended transition period, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision to opt out of the extended transition period for complying with new or revised accounting standards is irrevocable.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our exposure to market risk is limited to our cash, cash equivalents and marketable securities, all of which have maturities of one year or less. The goals of our investment strategy are preservation of capital, fulfillment of liquidity needs and fiduciary control of cash and investments. We also seek to maximize income from our investments without assuming significant risk. To achieve our goals, we maintain a portfolio of cash equivalents and investments in a variety of securities that management believes to be of high credit quality. The securities in our investment portfolio are not leveraged, are classified as available for sale and are, due to their short-term nature, subject to minimal interest rate risk. We currently do not hedge interest rate exposure. Because of the short-term maturities of our investments, we do not believe that an increase in market rates would have a material negative impact on the value of our investment portfolio.

We do not have any material foreign currency exposure.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

As of September 30, 2013, our management, with the participation of our Chief Executive Officer and Principal Financial Officer, evaluated the effectiveness of our disclosure controls and procedures. The term "disclosure controls and procedures," as defined in Rules 13a-15 (e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the Company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost benefit relationship of possible controls and procedures. Based on this evaluation, management concluded that our disclosure controls and procedures were not effective due to a material weakness in our internal control over financial reporting that was identified in connection with the preparation of our consolidated financial statements for the years ended December 31, 2011 and 2012 and is described in the following paragraph.

In connection with the preparation of our consolidated financial statements for the years ended December 31, 2012 and 2011, we identified past accounting errors, which resulted in the restatement of our previously issued financial statements. We and our independent registered public accounting firm identified a material weakness in internal control over financial reporting related to these items which required adjustment, specifically: (i) accounting for the purchase of supplies used in the production of our drug product, and (ii) accounting for purchase orders related to manufacturing services where work was contracted but not yet performed. A material weakness is defined as a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of a Company's annual or interim financial statements will not be prevented or detected on a timely basis by the Company's internal controls.

We have implemented and are continuing to implement procedures and controls designed to remediate the material weakness and underlying significant deficiencies. Amongst other actions, we have recently expanded our in-house accounting and finance resources by two people; commenced implementation of enhanced review procedures; begun a comprehensive documentation of our internal controls and procedures; and implemented more formal procedures as to the evaluation of non-routine judgments and estimates. Although we believe these controls will be effective, we have not yet determined if we have successfully remediated the material weakness and those significant deficiencies.

Notwithstanding the identified material weakness in internal controls over financial reporting, management believes the consolidated financial statements included in this Quarterly Report on Form 10-Q fairly represent in all material respects our financial condition, results of operations and cash flows at and for the periods presented in accordance with U.S. GAAP.

Changes in Internal Control over Financial Reporting

Other than remediation efforts described above, there have been no changes in our internal control over financial reporting during the three months ended September 30, 2013, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings

From time to time and in the ordinary course of business, we are subject to various claims, charges and litigation. We are not currently party to any material legal proceedings.

Item 1A. Risk Factors

An investment in shares of our common stock involves a high degree of risk. You should carefully consider all the risk factors and uncertainties described below, in addition to other information contained in this Quarterly Report on Form 10-Q, including our financial statements and the related notes, before deciding whether to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

Risks Relating to Our Financial Position and Need for Additional Capital

We have never been profitable. Currently, we have no products approved for commercial sale, and to date we have not generated any revenue from product sales. As a result, our ability to curtail our losses and reach profitability is unproven, and we may never achieve or sustain profitability.

We have never been profitable and do not expect to be profitable in the foreseeable future. We have incurred net losses in each year since our inception, including net losses of approximately $20.3 million for the nine months ended September 30, 2013 and an accumulated deficit of approximately $130.8 million since inception as of September 30, 2013. We have devoted most of our financial resources to research and development, including our preclinical development activities and clinical trials. We have not completed development of any product candidate and we have therefore not generated any revenues from product sales. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve or maintain profitability. We expect to incur significant increased expenses as we continue our single, open-label 13,200 subject Phase 3 trial of REG1 in patients undergoing PCI (excluding ST elevated myocardial infarction, or STEMI), or the REGULATE-PCI trial, which commenced in September 2013. We also expect an increase in our expenses associated with creating additional infrastructure to support operations as a public company. As a result, we expect to continue to incur net losses and negative cash flows for the foreseeable future. These net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital.

Prior to our initial public offering (IPO) which was consummated in August 2013, we have financed our operations through the sale of our equity and debt securities, bank borrowings and government grants. The amount of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenues. In addition, we may not be able to enter into any collaboration that will generate significant cash. If we are unable to develop and commercialize one or more of our product candidates either alone or with collaborators, or if revenues from any product candidate that receives marketing approval are insufficient, we will not achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability.

We will need to raise additional capital to complete the REGULATE-PCI trial and commercialize REG1. If we are unable to raise sufficient capital, we would be forced to delay, reduce or eliminate our product development programs.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is expensive. We expect our research and development expenses to increase in connection with our ongoing activities, particularly as we commence our REGULATE-PCI trial, undertake additional clinical trials of our other product candidates and continue to work on our other research programs. We believe that our existing cash and cash equivalents will be sufficient for us to fund the REGULATE-PCI trial through the first interim analysis, which we expect will occur by the beginning of the second quarter of 2014, and to fund our operations through the second quarter of 2014. We will need to raise substantial additional capital to fund our operations and complete the development and commercialization of REG1, and to repay our debt with Comerica bank. If the U.S. Food and Drug Administration, or the FDA, or other regulators require that we perform additional studies beyond those we currently expect, or if there are any delays in completing our clinical trials or the development of any of our product candidates, our expenses could increase beyond what we currently anticipate and the timing of any potential product approval may be delayed. We have no commitments or arrangements for any additional financing to fund our research and development programs. We also will need to raise substantial additional capital in the future to complete the development and commercialization of REG1 for additional indications and for our other product candidates. Because successful development of our product candidates is uncertain, we are unable to estimate the actual funds required to complete research and development and commercialize our products under development.

Until we can generate a sufficient amount of revenue from our product candidates, if ever, we expect to finance future cash needs through public or private equity offerings, debt financings or corporate collaborations and licensing arrangements. Additional funds may not be available when we need them on terms that are acceptable to us, or at all. If adequate funds are not available, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs. To the extent that we raise additional funds by issuing equity securities, our stockholders will experience additional dilution, and debt financing, if available, may involve restrictive covenants. To the extent that we raise additional funds through collaborations and licensing arrangements, it may be necessary to relinquish some rights to our technologies or our product candidates or grant licenses on terms that may not be favorable to us. We may seek to access the public or private capital markets whenever conditions are favorable, even if we do not have an immediate need for additional capital at that time.

Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of preclinical studies and clinical trials for our product candidates and potential product candidates, including our REGULATE-PCI trial and the continued development of our other product candidates;
- the number and characteristics of product candidates that we pursue;
- the terms and timing of any future collaboration, licensing or other arrangements that we may establish;
- the outcome, timing and cost of regulatory approvals;
- the cost of obtaining, maintaining, defending and enforcing intellectual property rights, including patent rights;
- the effect of competing technological and market developments;
- the cost and timing of completing commercial-scale outsourced manufacturing activities;
- market acceptance of any product candidates for which we may receive regulatory approval;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval; and
- the extent to which we acquire, license or invest in businesses, products or technologies.

We have a limited operating history and we expect a number of factors to cause our operating results to fluctuate on a quarterly and annual basis, which may make it difficult to predict our future performance.

We are a development stage biopharmaceutical company with a limited operating history. Our operations to date have been primarily limited to developing our technology and undertaking preclinical studies and clinical trials of REG1 and any of our other product candidates. We have not yet obtained regulatory approvals for REG1 or any of our other product candidates. Consequently, any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history or commercialized products. Our financial condition and operating results have varied significantly in the past and will continue to fluctuate from quarter-to-quarter or year-to-year due to a variety of factors, many of which are beyond our control. Factors relating to our business that may contribute to these fluctuations include other factors described elsewhere in this report and also include:

- our ability to obtain additional funding to complete development of REG1 and to develop our other product candidates;
- delays in the commencement, enrollment and timing of clinical trials;
- the success of our clinical trials through all phases of clinical development, including our REGULATE-PCI trial;
- any delays in regulatory review and approval of product candidates in clinical development;
- our ability to obtain and maintain regulatory approval for REG1 or any of our other product candidates in the United States and foreign jurisdictions;
- potential side effects of our product candidates that could delay or prevent commercialization, limit the indications for any approved drug, require the establishment of risk evaluation and mitigation strategies, or REMS, or cause an approved drug to be taken off the market;
- our dependence on third-party manufacturers, or CMOs, to supply or manufacture our products;
- our dependence on clinical research organizations, or CROs, to conduct our clinical trials;
- our ability to establish or maintain collaborations, licensing or other arrangements;
- market acceptance of our product candidates;
- our ability to establish and maintain an effective sales and marketing infrastructure, either through the creation of a commercial infrastructure or through strategic collaborations;
- competition from existing products or new products that may emerge;
- the ability of patients or healthcare providers to obtain coverage of or sufficient reimbursement for our products;

- our ability to leverage our proprietary technology platform to discover and develop additional product candidates;
- our ability and our licensors' abilities to successfully obtain, maintain, defend and enforce intellectual property rights important to our business;

- our ability to attract and retain key personnel to manage our business effectively;
- our ability to build our finance infrastructure and improve our accounting systems and controls;
- potential product liability claims;
- potential liabilities associated with hazardous materials; and
- our ability to obtain and maintain adequate insurance policies.

Accordingly, the results of any quarterly or annual periods should not be relied upon as indications of future operating performance.

The audit opinion on our financial statements contains a going concern modification.

Based on our cash balances, recurring losses, net capital deficiency and debt outstanding as of December 31, 2012 and our projected spending in 2013, which raise substantial doubt about our ability to continue as a going concern, the audit opinion on our audited financial statements as of and for the year ended December 31, 2012 contains a going concern modification. We believe that our existing cash and cash equivalents will be sufficient to fund our projected operating requirements through the end of the second quarter of 2014. However, if we are unable to continue as a going concern, we might have to liquidate our assets and the values we receive for our assets in liquidation or dissolution could be significantly lower than the values reflected in our financial statements. Amounts due under our loan with Comerica Bank, or Comerica, may become immediately due and payable upon the occurrence of a material adverse change, as defined under the loan agreement. Under the terms of the Comerica loan agreement, we are subject to operational covenants, including limitations on our ability to incur liens or additional debt, pay dividends, redeem stock, make specified investments and engage in merger, consolidation or asset sale transactions, among other restrictions. In addition, the inclusion of a going concern statement by our auditors, our lack of cash resources and our potential inability to continue as a going concern may materially adversely affect our share price and our ability to raise new capital or to enter into critical contractual relations with third parties.

Risks Relating to the Development and Regulatory Approval of Our Product Candidates

Clinical failure can occur at any stage of clinical development. Because the results of earlier clinical trials are not necessarily predictive of future results, any product candidate we advance through clinical trials may not have favorable results in later clinical trials or receive regulatory approval.

Clinical failure can occur at any stage of clinical development. Clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical or preclinical trials. In addition, data obtained from trials are susceptible to varying interpretations, and regulators may not interpret our data as favorably as we do, which may delay, limit or prevent regulatory approval. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. Frequently, product candidates that have shown promising results in early clinical trials have subsequently suffered significant setbacks in later clinical trials. In addition, the design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. While members of our management team have experience in designing clinical trials, our company has limited experience in designing clinical trials and we may be unable to design and execute a clinical trial to support regulatory approval. Further, clinical trials of potential products often reveal that it is not practical or feasible to continue development efforts. For example, if the results of our REGULATE-PCI trial do not achieve the primary efficacy endpoints or demonstrate expected safety, the prospects for approval of REG1 would be materially and adversely affected. If REG1 or our other product candidates are found to be unsafe or lack efficacy, we will not be able to obtain regulatory approval for them and our business would be harmed.

REGULATE-PCI includes three interim analyses of REG1 by the Data Safety Monitoring Board, or the DSMB. The first interim analysis will be a general safety analysis after enrollment of 1,000 subjects, which is expected to occur by the beginning of the second quarter of 2014. The second interim analysis will be another general safety analysis after 25% of the subjects are enrolled, which is expected to occur by the end of the third quarter of 2014. The final interim analysis will be an analysis of the general safety and efficacies of REG1 after 50% of the subjects are enrolled, which is expected to occur by the end of 2014. If, as a result of any of those interim analyses, we or the DSMB determine that REG1 is not safe or that it is futile to continue the trial because of a lack of efficacy, the trial will be terminated. If the results of any one of these analyses is unfavorable, our business would be harmed.

We cannot be certain that REG1 or any of our other product candidates will receive regulatory approval, and without regulatory approval we will not be able to market our product candidates. Any delay in the regulatory review or approval of REG1 or any of our other product candidates will materially or adversely harm our business.

We have invested a significant portion of our efforts and financial resources in the development of REG1, our most advanced product candidate. Our ability to generate revenue related to product sales, which we do not expect will occur for at least the next several years, if ever, will depend on the successful development and regulatory approval of our product candidates. We commenced our REGULATE-PCI trial in September 2013. We may conduct our REGULATE-PCI trial only to learn that REG1 is not a safe or effective treatment, in which case the REGULATE-PCI trial may not lead to regulatory approval for REG1. Similarly, our clinical development programs for our other product candidates may not lead to regulatory approval from the FDA and similar foreign regulatory agencies. This failure to obtain regulatory approvals would prevent our product candidates from being marketed and would have a material and adverse effect on our business.

All of our product candidates require regulatory review and approval prior to commercialization. Any delays in the regulatory review or approval of our product candidates would delay market launch, increase our cash requirements and result in additional operating losses.

The process of obtaining FDA and other required regulatory approvals, including foreign approvals, often takes many years and can vary substantially based upon the type, complexity and novelty of the products involved. Furthermore, this approval process is extremely complex, expensive and uncertain. We may be unable to submit any new drug application, or an NDA, in the United States or any marketing approval application in foreign jurisdictions for any of our products. If we submit an NDA including any amended NDA or supplemental NDA, to the FDA seeking marketing approval for any of our product candidates, the FDA must decide whether to accept or reject the submission for filing. We cannot be certain that any of these submissions will be accepted for filing and reviewed by the FDA, or that the marketing approval application submissions to any other regulatory authorities will be accepted for filing and review by those authorities. We cannot be certain that we will be able to respond to any regulatory requests during the review period in a timely manner, or at all, without delaying potential regulatory action. We also cannot be certain that any of our product candidates will receive favorable recommendations from any FDA advisory committee or foreign regulatory bodies or be approved for marketing by the FDA or foreign regulatory authorities. In addition, delays in approvals or rejections of marketing applications may be based upon many factors, including regulatory requests for additional analyses, reports, data and studies, regulatory questions regarding data and results, changes in regulatory policy during the period of product development and the emergence of new information regarding REG1 or our other product candidates.

Data obtained from preclinical studies and clinical trials are subject to different interpretations, which could delay, limit or prevent regulatory review or approval of any of our product candidates. Furthermore, regulatory attitudes towards the data and results required to demonstrate safety and efficacy can change over time and can be affected by many factors, such as the emergence of new information, including on other products, policy changes and agency funding, staffing and leadership. We do not know whether future changes to the regulatory environment will be favorable or unfavorable to our business prospects.

In addition, the environment in which our regulatory submissions may be reviewed changes over time. For example, average review times at the FDA for NDAs have fluctuated over the last ten years, and we cannot predict the review time for any of our submissions with any regulatory authorities. Review times can be affected by a variety of factors, including budget and funding levels and statutory, regulatory and policy changes. Moreover, in light of widely publicized events concerning the safety risk of certain drug products, regulatory authorities, members of the U.S. Government Accountability Office, medical professionals and the general public have raised concerns about potential drug safety issues. These events have resulted in the withdrawal of drug products, revisions to drug labeling that further limit use of the drug products and establishment of REMS measures that may, for instance, restrict distribution of drug products. The increased attention to drug safety issues may result in a more cautious approach by the FDA to clinical trials. Data from clinical trials may receive greater scrutiny with respect to safety, which may make the FDA or other regulatory authorities more likely to terminate clinical trials before completion, or require longer or additional clinical trials that may result in substantial additional expense and a delay or failure in obtaining approval or may result in approval for a more limited indication than originally sought.

Delays in the commencement, enrollment and completion of our clinical trials could result in increased costs to us and delay or limit our ability to obtain regulatory approval for REG1 and our other product candidates.

Delays in the commencement, enrollment and completion of clinical trials could increase our product development costs or limit the regulatory approval of our product candidates. We commenced our REGULATE-PCI trial in September 2013; however, this clinical trial may not be completed on schedule, if at all. In addition, we do not know whether planned clinical trials of REG1 in additional indications and of our other product candidates will begin on time or will be completed on schedule or at all. The commencement, enrollment and completion of our REGULATE-PCI trial or other clinical trials can be delayed for a variety of reasons, including:

- inability to reach agreements on acceptable terms with prospective CROs and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- regulatory objections to commencing a clinical trial;
- inability to identify and maintain a sufficient number of trial sites, many of which may already be engaged in other clinical trial programs, including some that may be for the same indication as our product candidates;

- withdrawal of clinical trial sites from our clinical trials as a result of changing standards of care or the ineligibility of a site to participate in our clinical trials;
- inability to obtain institutional review board approval to conduct a clinical trial;
- difficulty recruiting and enrolling subjects to participate in clinical trials for a variety of reasons, including meeting the enrollment criteria for our study and competition from other clinical trial programs for the same indication as our product candidates;
- inability to retain subjects in clinical trials due to the treatment protocol, personal issues, side effects from the therapy or lack of efficacy; and
- difficulty in importing and exporting clinical trial materials and study samples.

In addition, our REGULATE-PCI trial or any of our other clinical trials may be suspended or terminated by us, the FDA or other regulatory authorities due to a number of factors, including:

- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- failure to pass inspection of the clinical trial operations or trial sites by the FDA or other regulatory authorities;
- failure of any CMOs that we use to comply with current Good Manufacturing Practices, or cGMP;
- unforeseen safety issues or any determination that a clinical trial presents unacceptable health risks;
- changes in the regulatory requirement and guidance; or
- lack of adequate funding to continue the clinical trial due to unforeseen costs resulting from enrollment delays, requirements to conduct additional trials and studies, increased expenses associated with the services of our CROs and other third parties or other reasons.

If we are required to conduct additional clinical trials or other testing of REG1 or our other product candidates beyond those currently contemplated, we may be delayed in obtaining, or may not be able to obtain, marketing approval for these product candidates.

We have never conducted a Phase 3 clinical trial or submitted an NDA before, and may be unable to do so for REG1 and other product candidates we are developing.

We commenced our REGULATE-PCI trial in September 2013. The conduct of Phase 3 clinical trials and the submission of a successful NDA is a complicated process. Although members of our management team have extensive industry experience, including in the development, clinical testing and commercialization of drug candidates, our company has never conducted a Phase 3 clinical trial before, has limited experience in preparing, submitting and prosecuting regulatory filings, and has not submitted an NDA before. Consequently, we may be unable to successfully and efficiently execute and complete these planned clinical trials in a way that leads to NDA submission and approval of REG1 and other product candidates we are developing. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we develop. Failure to commence or complete, or delays in, our planned clinical trials would prevent or delay commercialization of REG1 and other product candidates we are developing.

We have never performed a clinical trial comparing the safety or efficacy of REG1 to bivalirudin. Because our clinical trials used heparin as a comparator, the risk that our REGULATE-PCI trial does not achieve one or more of its primary endpoints may be increased.

We have never performed a clinical trial directly comparing the safety or efficacy of REG1 to bivalirudin. Our randomized, partially blinded, dose-ranging Phase 2b trial involving 640 subjects, or the RADAR trial, used standard of care heparin as the comparator and, as a result, we have no clinical trial data directly comparing REG1 and bivalirudin. The primary efficacy endpoint of our REGULATE-PCI trial is a 20.0% reduction in the occurrence of ischemic events using REG1 compared to bivalirudin and the primary safety endpoint of the trial is non-inferiority of REG1 compared to bivalirudin with respect to major bleeding events. Because we have no clinical trial data directly comparing REG1 to bivalirudin, the prediction of Phase 3 success based on Phase 2 results is complicated and the risk that REGULATE-PCI does not achieve one or more of these endpoints may be increased.

Our product candidates may cause serious adverse events or undesirable side effects which may delay or prevent marketing approval, or, if approval is received, require them to be taken off the market, require them to include safety warnings or otherwise limit their sales.

Serious adverse events or undesirable side effects from REG1 or any of our other product candidates could arise either during clinical development or, if approved, after the approved product has been marketed. For example, three severe allergic events occurred in our RADAR trial. In addition, in 2008 we terminated an exploratory Phase 2a trial of REG1 in subjects undergoing off-pump coronary arterial bypass grafting when the first enrolled subject experienced clotting in one of three bypass grafts. The results of future clinical trials, including REGULATE-PCI, may show that our product candidates cause serious adverse events or undesirable side effects, which could interrupt, delay or halt clinical trials, resulting in delay of, or failure to obtain, marketing approval from the FDA and other regulatory authorities.

If REG1 or any of our other product candidates cause serious adverse events or undesirable side effects:

- regulatory authorities may impose a clinical hold which could result in substantial delays and adversely impact our ability to continue development of the product;
- regulatory authorities may require the addition of labeling statements, specific warnings, a contraindication or field alerts to physicians and pharmacies;
- we may be required to change the way the product is administered, conduct additional clinical trials or change the labeling of the product;
- we may be required to implement a risk minimization action plan, which could result in substantial cost increases and have a negative impact on our ability to commercialize the product;
- we may be required to limit the patients who can receive the product;
- we may be subject to limitations on how we promote the product;
- sales of the product may decrease significantly;
- regulatory authorities may require us to take our approved product off the market;
- we may be subject to litigation or product liability claims; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase commercialization costs and expenses, which in turn could delay or prevent us from generating significant revenues from the sale of our products.

We have limited experience manufacturing the oligonucleotides comprising our product candidates at commercial scale and there are no established standards for their manufacture. As a result, delays in regulatory approval of our product candidates may occur. Also, manufacturing issues may arise that could cause delays or increase costs.

We have limited experience manufacturing the oligonucleotides comprising our product candidates at commercial scale. We, together with our CMOs, have developed manufacturing processes that have never been tested in commercial production. Our manufacturing process will be subject to approval by regulators before we can commence the manufacture and sale of an approved product. The standards of the International Conference on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use, which establishes basic guidelines and standards for drug development in the United States, the European Union, Japan and other countries, do not apply to oligonucleotides, including our product candidates. As a result, there is no established generally accepted manufacturing or quality standard for the production of our product candidates. Even though the FDA has agreed to the quality standards for the REG1 to be used in our REGULATE-PCI trial, the FDA has the ability to modify those standards at any time and foreign regulatory agencies may impose differing quality standards and quality control on the manufacture of our drug candidates. The lack of uniform manufacturing and quality standards among regulatory agencies may delay regulatory approval of our product candidates. Also, as we scale-up manufacturing of any approved product, our CMOs may encounter unexpected issues relating to the manufacturing process or the quality, purity and stability of the product and we may be required to refine or alter our manufacturing processes to address these issues. Resolving these issues could result in significant delays and may result in significantly increased costs. If we experience significant delays or other obstacles in producing any approved product for commercial scale, our ability to market and sell any approved products may be adversely affected and our business could suffer.

REG1 and our other product candidates employ novel mechanisms of action and may never be approved or accepted by their intended markets.

Our activities have focused on the discovery and development of therapeutic aptamers and their specific active control agents. Our future success depends on our ability to complete the REGULATE-PCI trial of REG1 successfully, obtain market approval for and successfully commercialize REG1, as well as our ability to develop and market other product candidates that use our proprietary technology platform. We believe only one therapeutic aptamer has been approved for commercial sale and no product candidate consisting of a therapeutic aptamer and its specific active control agent has ever received regulatory approval. The scientific discoveries that form the basis of our proprietary technology platform and our product candidates are relatively new. We are not aware of any other antithrombotic drugs that have the same mechanism of action as our product candidates and there can be no assurance that, even if approved, physicians will be willing to use them. If we do not successfully develop and commercialize product candidates based upon our technological approach, we may not become profitable and the value of our common stock may decline.

In addition, regulatory approval of novel product candidates such as REG1 and our other product candidates manufactured using novel manufacturing processes such as ours can be more expensive and take longer than for other, more well-known or extensively studied pharmaceutical or biopharmaceutical products, due to our and regulatory agencies' lack of experience with them. We believe that the FDA has only approved one aptamer product to date. This lack of experience may lengthen the regulatory review process, require us to conduct additional studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these product candidates or lead to significant post-approval limitations or restrictions.

The novel nature of REG1 and our other product candidates also means that fewer people are trained in or experienced with product candidates of this type, which may make it difficult to find, hire and retain capable personnel for research, development and manufacturing positions.

Further, our focus solely on controllable aptamer technology for developing drugs as opposed to multiple, more proven technologies for drug development increases the risks associated with the ownership of our common stock. If we do not obtain regulatory approval for REG1 and our other product candidates and achieve market acceptance for our approved products, we may be required to change the scope and direction of our product development activities. In that case, we may not be able to identify and implement successfully an alternative product development strategy.

Risks Relating to the Commercialization of Our Product Candidates

If any of our product candidates for which we receive regulatory approval do not achieve broad market acceptance, the revenues that are generated from their sales will be limited.

The commercial success of REG1 and our other product candidates, if approved, will depend upon the acceptance of these products among physicians, healthcare payors and patients. The degree of market acceptance of our product candidates will depend on a number of factors, including:

- limitations or warnings contained in a product's FDA-approved labeling;
- changes in the standard of care or the availability of alternative therapies for the targeted indications for any of our product candidates;
- limitations in the approved indications for our product candidates;
- demonstrated clinical safety and efficacy compared to other products;
- lack of significant adverse side effects;
- education, sales, marketing and distribution support;
- availability and degree of reimbursement from third-party payors;
- timing of market introduction and perceived effectiveness of competitive products;
- cost-effectiveness;
- availability of alternative therapies at similar or lower cost, including generics, biosimilar and over-the-counter products;
- adverse publicity about our product candidates or favorable publicity about competitive products;
- convenience and ease of administration of our products;
- potential product liability claims; and
- government-imposed pricing restrictions.

If our product candidates are approved, but do not achieve an adequate level of acceptance by physicians, healthcare payors and patients, sufficient revenue may not be generated from these products, and we may not become or remain profitable. In addition, efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful.

REG1 and each of our other product candidates consist of a therapeutic aptamer and its specific active control agent. These two components are administered at different times and in different strengths and the failure to administer the components correctly may expose a patient to significant risk. Physicians will need to be educated as to our products' novel mechanisms of action and trained as to the proper use of our products. Physicians may be unwilling to devote the time necessary to learn how to use our product candidates properly and may continue using other competing products even if our products are safer and more effective. As a result, the commercialization of any approved product may be slower than we expect and any approved product may not achieve the level of acceptance we anticipate. If physicians are unwilling to use our products as a result of their novel mechanisms of action or the need to be trained on their use, our business may suffer.

We do not have the capability to sell, distribute and market our product candidates. If we are unable to establish an effective sales force and marketing infrastructure, or enter into acceptable third-party sales and marketing or licensing arrangements, we may not be able to commercialize our product candidates successfully.

We do not have the capability to sell, distribute and market our product candidates. We will need to build a commercial organization or secure a strategic partner to commercialize REG1 or any other product candidates. If we are unable to build a commercial infrastructure or secure a strategic collaboration, our business and results of operations will be materially and adversely affected. Development of an internal commercial organization will require substantial resources and will be time consuming. These costs may be incurred in advance of any approval of our product candidates. In addition, we may not be able to hire a sales force in the United States that is sufficient in size or has adequate expertise in the medical markets that we intend to target. If we are unable to establish a sales and marketing capability, our operating results may be adversely affected. If we seek to enter into sales and marketing or licensing arrangements with third parties for the marketing and sale of any approved products, we may be unable to enter into any such arrangements on acceptable terms, or at all.

Even if our product candidates receive regulatory approval, we may still face future development and regulatory difficulties.

Even if regulatory approval is obtained for any of our product candidates, regulatory authorities may still impose significant restrictions on a product's indicated uses or marketing or impose ongoing requirements for potentially costly post-approval studies. Given the number of high profile adverse safety events with certain drug products, regulatory authorities may require, as a condition of approval, costly REMS, which may include safety surveillance, restricted distribution and use, patient education, enhanced labeling, expedited reporting of certain adverse events, pre-approval of promotional materials and restrictions on direct-to-consumer advertising. For example, any labeling approved for any of our product candidates may include a restriction on the term of its use, or it may not include one or more of our intended indications. Furthermore, any new legislation addressing drug safety issues could result in delays or increased costs during the period of product development, clinical trials and regulatory review and approval, as well as increased costs to assure compliance with any new post-approval regulatory requirements.

Our product candidates will also be subject to ongoing regulatory requirements for the labeling, packaging, storage, advertising, promotion, record-keeping and submission of safety and other post-market information. In addition, sellers of approved products, manufacturers and manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMP. As such, we and our CMOs are subject to continual review and periodic inspections to assess compliance with cGMP. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, and quality control. We will also be required to report certain adverse reactions and production problems, if any, to the FDA, and to comply with certain requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products for indications or uses for which they do not have 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, it may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If our product candidates fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning or other letters;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us to enter into a consent decree or permanent injunction, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- impose other civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications filed by us;
- impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products or require a product recall.

We expect that our existing and future product candidates will face competition and most of our competitors have significantly greater resources than we do.

The biopharmaceutical industry is characterized by intense competition and rapid innovation. Our potential competitors include large pharmaceutical and biotechnology companies, specialty pharmaceutical companies and generic or biosimilar drug companies. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety and tolerability profile, mechanism of action, control and predictability, convenience of dosing and pricing and reimbursement. Our most advanced product candidate, REG1, is being developed for use in patients undergoing percutaneous coronary intervention for a wide variety of cardiovascular conditions. If approved for this indication, REG1 would compete with a number of currently-marketed anticoagulants, including bivalirudin, currently marketed and sold by The Medicines Company under the brand name Angiomax ® in the United States, and heparin, or UFH, and low molecular weight heparin, or LMWH, both of which are available as biosimilars and currently manufactured and sold by multiple manufacturers. If REG1 is approved for this initial indication, we intend to seek approval for the use of REG1 in other cardiovascular indications. If approved for these additional indications, REG1 would potentially compete with the same treatments described above.

Many of our potential competitors have substantially greater:

- resources, including capital, personnel and technology;
- research and development capability;
- clinical trial expertise;
- regulatory expertise;
- intellectual property rights, including patent rights;
- expertise in obtaining, maintaining, defending and enforcing intellectual property rights, including patent rights;
- manufacturing and distribution expertise; and
- sales and marketing expertise.

Accordingly, our competitors may be more successful than us in obtaining regulatory approval for drugs and achieving widespread market acceptance. Our competitors' drugs may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render our product candidates obsolete or non-competitive before we can recover the expenses of their development and commercialization. We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. Finally, the development of new treatment methods for the diseases we are targeting could render our product candidates non-competitive or obsolete.

Reimbursement decisions by third-party payors may have an adverse effect on pricing and market acceptance of REG1 or any of our other product candidates. If there is not sufficient reimbursement for our products, it is less likely that our products will be widely used.

Market acceptance and sales of REG1 or any other product candidates that we develop will depend on reimbursement policies and may be affected by future healthcare reform measures. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs they will cover and establish payment levels. We cannot be certain that reimbursement will be available for REG1 or any other product candidates that we develop. Also, we cannot be certain that reimbursement policies will not reduce the demand for, or the price paid for, our products. If reimbursement is not available or is available on a limited basis, we may not be able to successfully commercialize REG1 or any other product candidates that we develop.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, also called the Medicare Modernization Act, or MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation established Medicare Part D, which expanded Medicare coverage for outpatient prescription drug purchases by the elderly but provided authority for limiting the number of drugs that will be covered in any therapeutic class. The MMA also introduced a new reimbursement methodology based on average sales prices for physician-administered drugs.

The United States and several foreign jurisdictions are considering, or have already enacted, a number of legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our products profitably. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding access to healthcare. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. We expect to experience pricing pressures in connection with the sale of REG1 and any other products that we develop, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative proposals.

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively, the Affordable Care Act, became law in the United States. The goal of the Affordable Care Act is to reduce the cost of healthcare and substantially change the way healthcare is financed by both governmental and private insurers. While we cannot predict what impact on federal reimbursement policies this legislation will have in general or on our business specifically, the Affordable Care Act may result in downward pressure on pharmaceutical reimbursement, which could negatively affect market acceptance of REG1 or any future product candidates. Members of the U.S. Congress and some state legislatures are seeking to overturn at least portions of the legislation and we expect they will continue to review and assess this legislation and possibly alternative healthcare reform proposals. We cannot predict whether new proposals will be made or adopted, when they may be adopted or what impact they may have on us if they are adopted.

If we obtain approval to commercialize any approved products outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.

If REG1 or any of our other product candidates are approved for commercialization outside of the United States, we intend to enter into agreements with third parties to market them on a worldwide basis or in more limited geographical regions, excluding the Covered Territory. We expect that we will be subject to additional risks related to entering into international business relationships, including:

- different regulatory requirements for drug approvals;
- reduced protection for intellectual property rights, including trade secret and patent rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, hurricanes, floods and fires; and
- difficulty in importing and exporting clinical trial materials and study samples.

Risks Relating to Our Dependence on Third Parties

We may not succeed in establishing and maintaining collaborative relationships, which may significantly limit our ability to develop and commercialize our product candidates successfully, if at all.

We intend to seek collaborative relationships for the development and commercialization of our product candidates, including REG1. Failure to obtain a collaborative relationship for REG1, particularly in the European Union and for other markets requiring extensive sales efforts, may significantly impair the potential for this product candidate. We will also need to enter into collaborative relationships to provide funding to support our other research and development programs. The process of establishing and maintaining collaborative relationships is difficult, resource intensive and involves significant uncertainty, including:

- a collaboration partner may shift its priorities and resources away from our product candidates due to a change in business strategies, or a merger, acquisition, sale or downsizing;
- a collaboration partner may seek to renegotiate or terminate their relationships with us due to unsatisfactory clinical results, manufacturing issues, a change in business strategy, a change of control or other reasons;
- a collaboration partner may cease development in therapeutic areas which are the subject of our strategic collaboration;
- a collaboration partner may not devote sufficient capital or resources towards our product candidates;
- a collaboration partner may change the success criteria for a product candidate thereby delaying or ceasing development of such candidate;

- a significant delay in initiation of certain development activities by a collaboration partner will also delay payment of milestones tied to such activities, thereby impacting our ability to fund our own activities;
- a collaboration partner could develop a product that competes, either directly or indirectly, with our product candidate;
- a collaboration partner with commercialization obligations may not commit sufficient financial or human resources to the marketing, distribution or sale of a product;
- a collaboration partner with manufacturing responsibilities may encounter regulatory, resource or quality issues and be unable to meet demand requirements;

- a partner may exercise a contractual right to terminate a strategic alliance;
- a dispute may arise between us and a partner concerning the research, development or commercialization of a product candidate resulting in a delay in milestones, royalty payments or termination of an alliance and possibly resulting in costly litigation or arbitration which may divert management attention and resources; and
- a partner may use our products or technology in such a way as to invite litigation from a third party.

If any collaborator fails to fulfill its responsibilities in a timely manner, or at all, our research, clinical development, manufacturing, or commercialization efforts related to that collaboration could be delayed or terminated, or it may be necessary for us to assume responsibility for expenses or activities that would otherwise have been the responsibility of our collaborator. If we are unable to establish and maintain collaborative relationships on acceptable terms or to successfully transition terminated collaborative agreements, we may have to delay or discontinue further development of one or more of our product candidates, undertake development and commercialization activities at our own expense or find alternative sources of capital.

We rely on third parties to conduct, supervise and monitor our clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business .

We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials. While we have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that our clinical trials are 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 are required to comply with the FDA's current good clinical practices requirements, or cGCP, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. The FDA enforces these cGCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving any marketing applications. Upon inspection, the FDA may determine that our clinical trials did not comply with cGCPs. In addition, our clinical trials, including our REGULATE-PCI trial, will require a sufficiently large number of test subjects to evaluate the safety and effectiveness of a product candidate. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of patients, our clinical trials may be delayed or we may be required to repeat such clinical trials, which would delay the regulatory approval process.

Our CROs are not our employees, and we are not able to control whether or not they devote sufficient time and resources to our clinical trials. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials, or other drug development activities which could harm our competitive position. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for such product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

We also rely on other third parties to store and distribute drug products for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, if approved, producing additional losses and depriving us of potential product revenue.

We do not have multiple sources of supply for the components used in REG1 and our other product candidates. If we were to lose a supplier, it could have a material adverse effect on our ability to complete the development of REG1 or our other product candidates or, if we obtain regulatory approval for REG1 or our other product candidates, to commercialize them.

We do not have multiple sources of supply for the components used in REG1 and our other product candidates. We also do not have long-term supply agreements with any of our suppliers. If for any reason we are unable to obtain drug compounds from a supplier, we would have to seek to obtain it from another oligonucleotide manufacturer. We may not be able to establish additional sources of supply for our product candidates, or may be unable to do so on acceptable terms. Such suppliers are subject to regulatory requirements, covering manufacturing, testing, quality control and record keeping relating to our product candidates and subject to ongoing inspections by the regulatory agencies. Failure by any of our suppliers to comply with applicable regulations may result in long delays and interruptions.

The number of oligonucleotide suppliers is limited. In the event it is necessary or desirable to acquire supplies from an alternative supplier, we might not be able to obtain them on commercially reasonable terms, if at all. It could also require significant time and expense to redesign our manufacturing processes to work with another company.

As part of any marketing approval, a manufacturer and its processes are required to be qualified by the FDA prior to commercialization. If supply from the approved supplier is interrupted, there could be a significant disruption in commercial supply. An alternative vendor would need to be qualified through an NDA supplement which could result in further delay. The FDA or other regulatory agencies outside of the United States may also require additional studies if a new supplier is relied upon for commercial production. Switching vendors may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

If we are unable to obtain the supplies we need at a reasonable price or on a timely basis, it could have a material adverse effect on our ability to complete the development of REG1 and our other product candidates or, if we obtain regulatory approval for REG1 or our other product candidates, to commercialize them.

We rely on third-party manufacturers to produce our product candidates. If we experience problems with any of these suppliers, the manufacturing of our product candidates or products could be delayed.

We do not have the capability to manufacture our product candidates and do not intend to develop that capability. As a result, we rely on CMOs to produce our product candidates. If REG1 or our other product candidates are approved for sale, we expect to enter into contracts with CMOs for the commercial scale production of the approved product. Reliance on CMOs entails risks, including:

- the inability to meet our product specifications and quality requirements consistently;
- inability to access production facilities on a timely basis;
- inability or delay in increasing manufacturing capacity;
- manufacturing and product quality issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for commercial level activity;
- a failure to satisfy the FDA's cGMP requirements and similar foreign standards on a consistent basis;
- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- the reliance on a single sources of supply which, if unavailable, would delay our ability to complete our clinical trials or to sell any product for which we have received marketing approval;
- the lack of qualified backup suppliers for supplies that are currently purchased from a single source supplier;
- operations of our CMOs or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the CMO or supplier;
- carrier disruptions or increased costs that are beyond our control; and
- the failure to deliver products under specified storage conditions and in a timely manner.

Any of these risks could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our products, cause us to incur higher costs and prevent us from commercializing our product candidates successfully. Manufacturing of our product candidates and any approved products could be disrupted or halted if our CMOs do not comply with cGMP or foreign manufacturing standards, even if the compliance failure does not relate to our product candidates or approved products. Furthermore, if any of our product candidates are approved and our CMOs fail to deliver the required commercial quantities of finished product on a timely basis and at commercially reasonable prices, and we are unable to find one or more replacement manufacturers capable of production at a substantially equivalent cost, in substantially equivalent volumes and quality and on a timely basis, we would likely be unable to meet demand for our products and could lose potential revenue. It may take several years to establish an alternative source of supply for our product candidates and to have any such new source approved by the FDA or a foreign regulator.

Risks Relating to Our Intellectual Property

It is difficult and costly to protect our proprietary rights, and we may not be able to ensure their protection.

Our commercial success will depend in part on obtaining and maintaining proprietary rights important to our business, as well as successfully defending and enforcing those proprietary rights if challenged. The procurement, defense and enforcement of intellectual property rights involve complex legal and factual questions. Changes in either the patent laws or in interpretations of patent laws in the United States and foreign jurisdictions may diminish the value of our intellectual property. Laws relating to patent rights continue to evolve in the United States and foreign jurisdictions, as does their interpretation by national patent offices and judicial systems, creating some uncertainty for patent applicants, patent owners and licensees.

Our ability to stop third parties from using our technology or making, using, selling, offering to sell or importing our products is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. If any patent we currently or in the future may own or license is deemed invalid or unenforceable, it could impact our commercial success. We cannot predict the breadth of claims that may be issued from any patent applications we currently or may in the future own or license from third parties.

The degree of future protection our proprietary rights may afford is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make, use, sell, offer to sell or import products that are similar to our product candidates but that are not

covered by the claims of our patents;

- we might not have been the first to make the inventions covered by our patent portfolio;
- we might not have been the first to file patent applications for these inventions;

- others may independently develop similar or alternative technologies or duplicate any of our technologies in a manner that does not violate our trade secrets;
- our proprietary rights may not provide us with any competitive advantages;
- we may not develop additional technologies or products that are patentable or suitable to maintain as trade secrets; or
- the proprietary rights of others may have an adverse effect on our business.

As of September 30, 2013, we are the owner of record of five issued or allowed U.S. patents and six issued or allowed non-U.S. patents, as well as the licensee of at least ten issued or allowed U.S. patents and at least eleven issued or allowed non-U.S. patents. We are actively pursuing an additional 12 U.S. patent applications, of which four are provisional and eight are non-provisional, one international patent application and 40 non-U.S. patent applications in twelve jurisdictions as the owner of record, in addition to at least two U.S. patent applications and 14 non-U.S. patent applications under license.

We also may rely on trade secrets to protect our technology, especially where we do not believe patent protection is appropriate or obtainable. Our ability to stop third parties from making, using, selling, offering to sell or importing our products or practicing our technology is dependent in part upon the extent to which we have rights in enforceable trade secrets that cover these activities. Trade secret rights can be lost through disclosure to third parties. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, outside scientific collaborators and other advisors may unintentionally or willfully disclose our trade secrets to third parties, resulting in loss of trade secret protection. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how, which would not constitute a violation of our trade secret rights. Enforcing a claim that a third party is engaged in the unlawful use of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, recognition of rights in trade secrets and a willingness to enforce trade secrets may differ in certain jurisdictions.

Intellectual property disputes are expensive and would consume time and resources and divert the attention of managerial and scientific personnel. We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights and we may be unable to enforce or protect our rights to, or use, our technology.

If we choose to go to court to attempt to stop another party from using our intellectual property without authorization, or any licensor of our intellectual property chooses to do the same, rights in our intellectual property may be lost. More specifically, rights in trade secrets we have or obtain may be lost as the result of disclosure associated with of our efforts to stop their unauthorized use. Rights in any patents we have or obtain may be lost as a result of our efforts to stop their unauthorized use, as the party charged with patent infringement has the right to ask the court to rule that such patents are invalid or should not be enforced against that third party. There is also the risk that, even if the validity of such patents is upheld, the court will refuse to stop the other party on the ground that such other party's activities do not infringe our rights to such patents. Apart from litigation, adversarial procedures are available in the patent offices of many countries, including the United States, that permit interested third parties to dispute the validity of issued patents or to otherwise impact the course of prosecution of pending patent applications. Intellectual property disputes are expensive and would consume time and resources and divert the attention of managerial and scientific personnel even if we were successful in stopping the unauthorized use of our intellectual property rights. Moreover, the patent laws in the United States and internationally continue to evolve, creating uncertainty as to the likelihood that we will be able to obtain patents and increase the likelihood of challenge to any patents we obtain or license.

Furthermore, a third party may claim that we or our manufacturing partners are engaged in unauthorized use of intellectual property owned by the third party, including patent rights, and may go to court to stop us from engaging in our normal operations and activities, including making or selling our product candidates. These lawsuits are costly and could affect our results of operations and divert the attention of managerial and scientific personnel. There is a risk that a court would decide that we or our CMOs are engaged in unauthorized use of the third party's valid and enforceable intellectual property, including patent rights, and would order us or our CMOs to stop the activities protected by these rights. In that event, we may not have a viable alternative to the unauthorized use and may need to halt commercialization of the relevant product. In addition, there is a risk that a court will order us or our CMOs to pay the other party damages for having used the other party's intellectual property in an unauthorized manner. In the future, we may agree to indemnify our CMOs against certain intellectual property claims brought by third parties. Patent rights involve complex factual and legal issues; as a result, it is not always clear to industry participants, including us, whether activities or products are covered by patent rights, or by which patent rights. The breadth of patent claims is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our products or methods either do not infringe the claims of the relevant patents or that the patent claims are invalid, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires the alleged infringer to overcome the presumption of validity enjoyed by issued patents.

Because some patent applications may be maintained in secrecy until the patents are issued, publication of patent applications is delayed, and publications in the scientific literature often lag behind actual discoveries, we cannot be certain that others have not filed patent applications for technology covered by our pending applications, or that we were the first to invent the technology. Our competitors may have filed, and may in the future file, patent applications covering technology important to our business. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies and serve as a bar to patentability of our own patent filings. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference proceeding declared by the U.S. Patent and Trademark Office to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar invention prior to our own invention, resulting in a loss of our U.S. patent position with respect to such invention.

Some of our competitors may be able to sustain the costs of patent-related disputes, including patent litigation, more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

Risks Related to Employee Matters and Managing Growth

We will need to expand our operations and increase the size of our company, and we may experience difficulties in managing growth.

As we advance our product candidates through preclinical studies and clinical trials and develop new product candidates, we will need to increase our product development, scientific and administrative headcount to manage these programs. In addition, to meet our obligations as a public company, we will need to increase our general and administrative capabilities. Our management, personnel and systems currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and various projects requires that we:

- successfully attract and recruit new employees with the expertise and experience we will require;
- manage our clinical programs effectively, which we anticipate being conducted at numerous clinical sites;
- develop a marketing, distribution and sales infrastructure if we seek to market our products directly; and
- continue to improve our operational, manufacturing, financial and management controls, reporting systems and procedures.

If we are unable to successfully manage this growth and increased complexity of operations, our business may be adversely affected.

We may not be able to manage our business effectively if we are unable to attract and retain key personnel.

We may not be able to attract or retain qualified management, finance, scientific and clinical personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. If we are not able to attract and retain necessary personnel to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

Our industry has experienced a high rate of turnover of management personnel in recent years. We are highly dependent on the development, regulatory, commercialization and business development expertise of our executive officers and key employees. If we lose one or more of our executive officers or key personnel, our ability to implement our business strategy successfully could be seriously harmed. Any of our executive officers or key employees may terminate their employment at any time. We have entered into change of control and severance agreements with certain of our officers as part of our retention efforts. Replacing executive officers and key employees may be difficult, will be costly and may take an extended period of time because of the limited number of individuals in our industry with the mix of skills and experience required to develop, gain regulatory approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel. Our failure to attract and retain key personnel could materially harm our business.

Failure to build our finance infrastructure and improve our accounting systems and controls could impair our ability to comply with the financial reporting and internal controls requirements for publicly traded companies.

As a public company, we will operate in an increasingly demanding regulatory environment, which requires us to comply with applicable provisions of the Sarbanes-Oxley Act of 2002, or Sarbanes-Oxley Act, and the related rules and regulations of the Securities and Exchange Commission, expanded disclosure requirements, accelerated reporting requirements and more complex accounting rules. Company responsibilities required by the Sarbanes-Oxley Act include establishing corporate oversight and adequate internal control over financial reporting and disclosure controls and procedures. Effective internal controls are necessary for us to produce reliable financial reports and are important to help prevent financial fraud.

We rely on consultants to perform certain of our accounting and financial reporting functions. We will need to hire additional finance personnel and build our financial infrastructure as we transition to operating as a public company, including complying with the applicable requirements of Section 404 of the Sarbanes-Oxley Act. We may be unable to do so on a timely basis.

Until we are able to expand our finance and administrative capabilities and establish necessary financial reporting infrastructure, we may not be able to prepare and disclose, in a timely manner, our financial statements and other required disclosures or comply with the applicable provisions of the Sarbanes-Oxley Act or existing or new reporting requirements. If we cannot provide reliable financial reports or prevent fraud, our business and results of operations could be harmed and investors could lose confidence in our reported financial information.

We have had a material weakness in our internal control over financial reporting.

Prior to our IPO in August 2013, we had not been a public reporting company and have had limited accounting personnel and systems to adequately execute accounting processes and limited other supervisory resources with which to address internal control over financial reporting. We and our independent registered public accounting firm identified a material weakness in internal control over financial reporting for the years ended December 31, 2012 and 2011. Under standards established by the Public Company Accounting Oversight Board, a deficiency in internal control over financial reporting exists when the design or operation of a control does not allow management or personnel, in the normal course of performing their assigned functions, to prevent or detect misstatements on a timely basis. A material weakness is a deficiency or combination of deficiencies in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected and corrected on a timely basis. We are in the process of remediating the material weakness identified by us and our independent registered public accounting firm; however, we cannot assure that there will not be additional material weaknesses and significant deficiencies that our independent registered public accounting firm or we will identify. If we identify such issues or if we are unable to produce accurate and timely financial statements, our stock price may be adversely affected and we may be unable to maintain compliance with applicable securities laws and listing requirements.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Other Risks Relating to Our Business

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 intend to focus on the regulatory approval of REG1, including the completion of the REGULATE-PCI trial. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on existing and future product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic alliance, 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 face potential product liability exposure, and if successful claims are brought against us, we may incur substantial liability and may have to limit development of a product candidate or commercialization of an approved product.

The use of our product candidates in clinical trials and the sale of any products for which we may obtain marketing approval expose us to the risk of product liability claims. Product liability claims may be brought against us or our CMOs by participants enrolled in our clinical trials, patients, healthcare providers or others using, administering or selling our products. If we cannot successfully defend ourselves against any such claims, we would incur substantial liabilities. Regardless of merit or eventual outcome, product liability claims may result in:

- withdrawal of clinical trial participants;
- termination of clinical trial sites or entire trial programs;
- costs of related litigation;
- substantial monetary awards to patients or other claimants;
- decreased demand for an approved product and loss of revenue;
- impairment of our business reputation;

- diversion of management and scientific resources from our business operations; and
- the inability to commercialize an approved product.

We have obtained limited product liability insurance coverage for our clinical trials domestically and in selected foreign countries where we are conducting clinical trials. Our products liability insurance coverage is currently limited to $10.0 million per occurrence and $10.0 million in the aggregate per year. As such, our insurance coverage may not reimburse us or may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to product liability. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain marketing approval for our product candidates, but we may be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could cause our stock price to decline and could adversely affect our results of operations and business.

Our operations involve hazardous materials, which could subject us to significant liabilities.

Our research and development processes involve the controlled use of hazardous materials. Our operations produce hazardous waste products. We cannot eliminate the risk of accidental contamination or discharge or injury from these materials. Federal, state and local laws and regulations govern the use, manufacture, storage, handling and disposal of these materials. We could be subject to civil damages in the event of exposure of individuals to hazardous materials. In addition, claimants may sue us for injury or contamination that results from our use of these materials and our liability may exceed our total assets. We have general liability insurance of up to $1.0 million per occurrence, with an annual aggregate limit of $2.0 million, which excludes pollution liability. This coverage may not be adequate to cover all claims related to our hazardous materials. Furthermore, if we were to be held liable for a claim involving hazardous materials, this liability could exceed our insurance coverage, if any, and our other financial resources. Compliance with environmental and other laws and regulations may be expensive and current or future regulations may impair our research, development or production efforts.

Our insurance policies are expensive and protect us only from some business risks, which will leave us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, employment practices liability, property, auto, workers' compensation, products liability and directors' and officers' insurance. We also expect that operating as a public company will make it more difficult and more expensive for us to obtain director and officer liability insurance, and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, it may be more difficult for us to attract and retain qualified people to serve on our board of directors, our board committees or as executive officers. We do not know, however, if we will be able to maintain existing insurance with adequate levels of coverage. Any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our cash position and results of operations.

Risks Relating to Ownership of Our Common Stock

Our executive officers, directors and principal stockholders will have the ability to control all matters submitted to our stockholders for approval.

Our executive officers, directors and stockholders, in the aggregate, beneficially own shares representing 78.6% of our common stock. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, will control the election of directors and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. This concentration of voting power could delay or prevent an acquisition of us on terms that other stockholders may desire. The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

We do not anticipate paying cash dividends on our common stock, and accordingly, stockholders must rely on stock appreciation for any return on their investment.

We have never declared or paid any cash dividend on our common stock and do not anticipate paying cash dividends on our common stock in the future. Our Loan Agreement with Comerica Bank prohibits us from paying cash dividends. As a result, the only return to stockholders will be appreciation in the price of our common stock, which may never occur. Investors seeking cash dividends should not invest in our common stock.

Our stock price may be volatile, and investors in our common stock could incur substantial losses.

Our stock price has fluctuated in the past and may be volatile in the future. The stock market in general and the market for biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price of shares of our common stock could be subject to wide fluctuations in response to many risk factors listed in this section, and others beyond our control, including:

- results and timing of our clinical trials;
- results of clinical trials of our competitors' products;
- regulatory actions with respect to our products or our competitors' products;
- actual or anticipated fluctuations in our financial condition and operating results;
- actual or anticipated changes in our growth rate relative to our competitors;
- actual or anticipated fluctuations in our competitors' operating results or changes in their growth rate;
- competition from existing products or new products that may emerge;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- issuance of new or updated research or reports by securities analysts;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- additions or departures of key management or scientific personnel;
- disputes or other developments related to proprietary rights, including patents, litigation matters and our ability to obtain, maintain, defend or enforce proprietary rights relating to our products and technologies;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;
- market conditions for biopharmaceutical stocks in general; and
- general economic and market conditions.

Furthermore, the stock markets have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many companies. These fluctuations often have been unrelated or disproportionate to the operating performance of those companies. These broad market and industry fluctuations, as well as general economic, political and market conditions such as recessions, interest rate changes or international currency fluctuations, may negatively impact the market price of shares of our common stock. In addition, such fluctuations could subject us to securities class action litigation, which could result in substantial costs and divert our management's attention from other business concerns, which could potentially harm our business.

We may be subject to securities litigation, which is expensive and could divert management attention.

Our stock price has fluctuated in the past and may be volatile in the future, and in the past, companies that have experienced volatility in the market price of their stock have been subject to an increased incidence of securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

An active trading market for our common stock may not be maintained

Our stock is currently traded on NASDAQ, but we can provide no assurance that we will be able to maintain an active trading market on NASDAQ or any other exchange in the future. If an active market for our common stock is not maintained, it may be difficult for our stockholders to sell shares without depressing the market price for the shares or at all. An inactive market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

If securities or industry analysts do not publish research or reports about our business, or publish negative reports about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will continue to cover us or provide

favorable coverage. If one or more of the analysts who cover us downgrade our stock or change their opinion of our stock, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our stock price or trading volume to decline.

A significant portion of our total outstanding shares of common stock is restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur in the future. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. As of September 30, 2013, we have 21,303,012 outstanding shares of common stock. Of these shares, 3,750,863 may be resold in the public market immediately and the remaining 17,552,149 shares are currently restricted under securities laws or as a result of lock-up agreements, which restrict their transfer for a period of 180 days after August 22, 2013, the date of the IPO prospectus. Moreover, after this offering, holders of shares of our common stock will have rights, subject to some conditions, to require us to file registration statements covering the resale or other disposition of up to 9,396,767 shares of our common stock or to require us to include those shares in registration statements that we may file for ourselves or other stockholders. We registered up to 3,342,839 shares of common stock that we may issue under our equity compensation plans. Such shares can be freely sold in the public market upon issuance and once vested, subject to the 180 day lock-up period under the lock-up agreements.

You may be diluted by exercises of outstanding options and warrants.

As of September 30, 2013, we had outstanding options to purchase an aggregate of 2,818,732 shares of our common stock at a weighted average exercise price of $5.50 per share and warrants to purchase an aggregate of 16,332 shares of our common stock at a weighted average exercise price of $6.96 per share. The exercise of such outstanding options and warrants will result in dilution of your investment. In addition, as described below, you may experience additional dilution if we issue common stock in the future. As a result of this dilution, you may receive significantly less than the full purchase price you paid for the shares in the event of liquidation.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities; our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities. If we sell common stock, convertible securities or other equity securities, your investment in our common stock will be diluted. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

We are an "emerging growth company," and will be able take advantage of reduced disclosure requirements applicable to "emerging growth companies," which could make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or JOBS Act, and, for as long as we continue to be an "emerging growth company," we intend to take advantage of certain exemptions from various reporting requirements applicable to other public companies but not to "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 in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an "emerging growth company" for up to five years, or until the earliest of (i) the last day of the first fiscal year in which our annual gross revenues exceed $1 billion, (ii) the date that we become a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act, which would occur if the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the last business day of our most recently completed second fiscal quarter, or (iii) the date on which we have issued more than $1 billion in non-convertible debt during the preceding three year period. We cannot predict if investors will find our common stock less attractive if we choose to rely on these exemptions. If some investors find our common stock less attractive as a result of any choices to reduce future disclosure, there may be a less active trading market for our common stock and our stock price may be more volatile.

We will incur significantly increased costs and devote substantial management time as a result of operating as a public company particularly after we are no longer an "emerging growth company."

As a newly public company, we are incurring significant legal, accounting and other expenses that we did not incur as a private company. For example, we are required to comply with certain of the requirements of the Sarbanes-Oxley Act and the Dodd-Frank Wall Street Reform and Consumer Protection Act, as well as rules and regulations subsequently implemented by the Securities and Exchange Commission, and The NASDAQ Capital Market, our stock exchange, including the establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. We expect that compliance with these requirements will increase our legal and financial compliance costs and will make some activities more time consuming and costly. In addition, we expect that our management and other personnel will need to divert attention from operational and other business matters to devote substantial time to these public company requirements. In particular, we expect to incur significant expenses and devote substantial management effort toward ensuring compliance with the requirements of Section 404 of the Sarbanes-Oxley Act. In that regard, we currently do not have an internal audit function, and we will need to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge.

However, for as long as we remain an "emerging growth company" as defined in the JOBS Act, we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other 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 in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We intend to take advantage of these reporting exemptions until we are no longer an "emerging growth company."

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

After we are no longer an "emerging growth company," we expect to incur additional management time and cost to comply with the more stringent reporting requirements applicable to companies that are deemed accelerated filers or large accelerated filers, including complying with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act.

We cannot predict or estimate the amount of additional costs we may incur as a result of becoming a public company or the timing of such costs.

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

Provisions in our amended and restated certificate of incorporation and our amended and restated bylaws may delay or prevent a merger, acquisition or other change of control that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions include:

- classifying our board of directors into three classes;
- authorizing the issuance of "blank check" convertible preferred stock, the terms of which may be established and shares of which may be issued without stockholder approval;
- limiting the removal of directors by the stockholders;
- requiring a supermajority vote of stockholders to amend our certificate of incorporation or bylaws;
- prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- eliminating the ability of stockholders to call a special meeting of stockholders;
- establishing advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings; and
- establishing Delaware as the exclusive jurisdiction for certain stockholder litigation against us.

In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, who are responsible for appointing the members of our management team. In addition, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits, with some exceptions, stockholders owning in excess of 15% of our outstanding voting stock from merging or combining with us. Although we believe these provisions together 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.

If securities or industry analysts do not publish research or reports about our business, or publish negative reports about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will cover us or provide favorable coverage. The price of our common stock could decline if one or more equity analysts downgrade our common stock or if analysts issue other unfavorable commentary or cease publishing reports about us or our business.

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

(a) Sales of Unregistered Securities

Per the Series E financing agreement executed on December 18, 2012, a second financing tranche of $10.3 million for 14,320,168 shares of Series E preferred stock took place on March 22, 2013. On March 22, 2013 a portion of the second tranche of the Series E financing was

completed with 7,160,084 preferred shares issued for $5.2 million. The RMI Investments, S.a.r.l portion of the second tranche totaling $5.2 million was delivered into an escrow account at the time of the second tranche, and the RMI funds and Series E shares relating to the RMI investment were released on April 26, 2013.

On May 13, 2013, in connection with a loan agreement, we issued to the Comerica Bank, or the Bank a warrant to purchase 156,250 shares of the Series E Preferred Stock at a price of $0.72 per share, or the Warrant Price, subject to adjustment for stock splits, combinations, reclassifications or exchanges and certain dilutive issuances. After giving effect to the IPO and reverse stock split, the warrant was adjusted to a warrant to purchase 9,356 shares of our common stock at a price of $12.02 per share. See "Liquidity and Capital Resources—Comerica Loan" for additional details regarding this transaction.

Upon the closing of the IPO, all outstanding shares of our Series A, B, C, D and E preferred stock were converted into an aggregate of 9,396,767 shares of common stock.

No underwriters were used in the foregoing transactions. All sales of securities described above were made in reliance upon the exemption from registration provided by Section 4(2) of the Securities Act (and/or Regulation D and Rule 506 promulgated there under) for transactions by an issuer not involving a public offering. All of the foregoing securities are deemed restricted securities for the purposes of the Securities Act.

(b) Use of Proceeds from Initial Public Offering of Common Stock

During the third quarter of 2013, we completed our IPO issuing 11,671,500 shares of common stock, inclusive of the exercise of the underwriters' overallotment option, at a price of $4.00 per share, resulting in net proceeds to us of approximately $41.1 million, after deducting approximately $3.3 million of underwriting discounts and commissions and offering-related expenses reasonably estimated to be $2.3 million. The offer and sale of all of the shares in the offering were registered under the Securities Act pursuant to a registration statement on Form S-1, which was declared effective on August 21, 2013 (File No. 333-188209). Cowen and Company, LLC and BMO Capital Markets Corp. acted as book-running managers for the offering and as representatives of the underwriters.

No offering costs were paid directly or indirectly to any of our director or officers or persons owning ten percent or more of any class of our equity securities or to any other affiliates, other than payments in the ordinary course of business to officers for salaries and to non-employee directors as compensation for board or board committee service. There has been no material change in the planned use of proceeds from our IPO as described in the final prospectus dated August 22, 2013 filed with the Securities and Exchange Commission pursuant to Rule 424(b) under the Securities Act on August 22, 2013. As of September 30, 2013, we have not used any of our IPO proceeds.

Item 6. Exhibits

The exhibits filed or furnished as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which Exhibit Index is incorporated herein by reference.

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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

REGADO BIOSCIENCES, INC.

DATED: November 8, 2013

By: /s/ David J. Mazzo
David J. Mazzo
President, Chief Executive Officer and Director
(Principal Executive Officer)

DATED: November 8, 2013

By: /s/ Christopher E. Courts
Christopher E. Courts
Vice President, Finance
(Principal Accounting and Financial Officer)

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EXHIBIT INDEX

Exhibit No.	Description
3.1	Sixth Amended and Restated Certificate of Incorporation of Regado Biosciences, Inc. (Incorporated herein by reference to Exhibit 3.1 of the Current Report on Form 8-K filed by the registrant on September 3, 2013).
3.2	Form of Amended and Restated Bylaws of Regado Biosciences, Inc. (Incorporated herein by reference to Exhibit 3.2 of the Current Report on Form 8-K filed by the registrant on September 3, 2013).
4.1	Specimen Common Stock certificate of Regado Biosciences, Inc (Incorporated by reference to Exhibit 4.1 to the registrant's Registration Statement on Form S-1, as amended (File No. 333-188209)).
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) and 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) and 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101**	The following materials from the Registrant's quarterly report on Form 10-Q for the quarter ended September 30, 2013, formatted in XBRL (Extensible Business Reporting Language): (i) the Unaudited Consolidated Balance Sheets, (ii) the Unaudited Consolidated Statements of Comprehensive Income (Loss), (iii) the Unaudited Consolidated Statements of Cash Flows, and (iv) Notes to Unaudited Consolidated Financial Statements.

* Filed herewith.

** XBRL (Extensible Business Reporting Language) information is furnished and not deemed filed or a part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, or Section 18 of the Securities Exchange Act of 1934, as amended, and otherwise is not subject to liability under these sections.

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER

I, David J. Mazzo, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Regado Biosciences, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 8, 2013

/s/ David J. Mazzo
David J. Mazzo
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER

I, Christopher E. Courts, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Regado Biosciences, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 8, 2013

/s/ Christopher E. Courts
Christopher E. Courts
Vice President Finance
(Principal Accounting and Financial Officer)

Exhibit 32.1

CERTIFICATION OF THE PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO RULE 13a-14(b)
OF THE SECURITIES EXCHANGE ACT OF 1934 AND 18 U.S.C. SECTION 1350

In connection with the Quarterly Report on Form 10-Q of Regado Biosciences, Inc. (the "Company") for the period ended September 30, 2013 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, David J. Mazzo, Chief Executive Officer of the Company, hereby certifies, to the knowledge of the undersigned, pursuant to 18 U.S.C. Section 1350, that:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

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

Date: November 8, 2013

/s/ David J. Mazzo
David J. Mazzo
Chief Executive Officer
(Principal Executive Officer)

This Certification is being furnished solely to accompany the Report pursuant to 18 U.S.C. § 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed "filed" by the Company for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and shall not be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Report, irrespective of any general incorporation language contained in such filing.

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Exhibit 32.2

CERTIFICATION OF THE PRINCIPAL FINANCIAL OFFICER
PURSUANT TO RULE 13a-14(b)
OF THE SECURITIES EXCHANGE ACT OF 1934 AND 18 U.S.C. SECTION 1350

In connection with the Quarterly Report on Form 10-Q of Regado Biosciences, Inc. (the "Company") for the period ended September 30, 2013 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Christopher E. Courts, Vice President Finance of the Company, hereby certifies, to the knowledge of the undersigned, pursuant to 18 U.S.C. Section 1350, that:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

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

Date: November 8, 2013

/s/ Christopher E. Courts
Christopher E. Courts
Vice President Finance
(Principal Accounting and Financial Officer)

This Certification is being furnished solely to accompany the Report pursuant to 18 U.S.C. § 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed "filed" by the Company for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and shall not be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Report, irrespective of any general incorporation language contained in such filing.

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.