Raptor Pharmaceutical Corp
Form 10-Q
November 07, 2013

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

FORM 10-Q

þ 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

For the transition period from_____to_____

Commission File Number: 000-25571

Raptor Pharmaceutical Corp.
(Exact name of registrant as specified in its charter)

Delaware	86-0883978
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

5 Hamilton Landing, Suite 160, Novato, CA 94949
(Address of principal executive offices) (Zip Code)

(415) 408-6200
(Registrant's telephone number, including area code)

_____
(Former name, former address and former fiscal year, if changed since last report)

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 þ No ¨

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes þ 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.

Large accelerated filer¨ Accelerated filer þ
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 þ

There were 61,291,784 shares of the registrant's common stock, par value $0.001, outstanding as of October 31, 2013.

RAPTOR PHARMACEUTICAL CORP.

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PART I - FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

Raptor Pharmaceutical Corp.
Condensed Consolidated Balance Sheets
(In thousands, except shares and per share data, or unless otherwise specified)

	September 30, 2013 (unaudited)	December 31, 2012 (1)
ASSETS		
Current assets:		
Cash and cash equivalents	$88,334	$36,313
Restricted cash	500	163
Short-term investments	0	22,096
Accounts receivable, net	5,519	0
Inventories, net	2,218	0
Prepaid expenses and other	2,764	1,610
Total current assets	99,335	60,182
Intangible assets, net	3,272	2,156
Goodwill	3,275	3,275
Fixed assets, net	1,309	416
Deposits	153	26
Deferred offering costs	172	109
Debt issuance costs	2,929	1,959
Total assets	$110,445	$68,123
LIABILITIES AND STOCKHOLDERS' EQUITY		
Liabilities		
Current liabilities:		
Accounts payable	$1,847	$4,599
Accrued liabilities	9,260	2,150
Deferred revenue	3,010	0
Common stock warrant liability	10,339	16,405
Deferred rent	157	6
Capital lease liability – current	18	8
Total current liabilities	24,631	23,168
Note payable	50,000	25,000
Capital lease liability - long-term	45	11
Total liabilities	74,676	48,179
Commitments and contingencies – see Note 8		
Stockholders' equity:		
Preferred stock, $0.001 par value per share, 15,000,000 shares authorized, zero shares issued and outstanding	0	0
Common stock, $0.001 par value per share, 150,000,000 shares authorized 61,142,756 and 52,424,649 shares issued and outstanding at September 30, 2013 and December 31, 2012, respectively	61	52

Additional paid-in capital	229,164	155,945
Accumulated other comprehensive loss	(193)	(115)
Accumulated deficit	(193,263)	(135,938)
Total stockholders' equity	35,769	19,944
Total liabilities and stockholders' equity	$110,445	$68,123

(1) Derived from the Company's audited consolidated financial statements as of December 31, 2012.

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

Raptor Pharmaceutical Corp.
Condensed Consolidated Statements of Comprehensive Loss
(Unaudited)
(In thousands, except per share data, or unless otherwise specified)

	Three Months Ended		Nine Months Ended	
	September 30, 2013	August 31, 2012	September 30, 2013	August 31, 2012
Net product sales	$6,603	$0	$6,624	$0
Operating expenses:				
Cost of sales	442	0	867	0
Research and development	6,791	6,436	21,418	16,427
Selling, general and administrative	8,334	5,830	25,576	12,387
Total operating expenses	15,567	12,266	47,861	28,814
Loss from operations	(8,964)	(12,266)	(41,237)	(28,814)
Interest income	3	78	174	276
Interest expense	(2,287)	(1)	(4,088)	(2)
Foreign currency transaction gain/ (loss)	15	23	(20)	86
Realized gain/ (loss) on short-term investments	0	215	(129)	215
Unrealized gain/ (loss) on short-term investments	0	(143)	0	33
Adjustment to fair value of common stock warrants	(6,044)	1,872	(12,025)	995
Net loss	(17,277)	(10,222)	(57,325)	(27,211)
Other comprehensive gain (loss):				
Foreign currency translation adjustment	11	(34)	(78)	(43)
Comprehensive loss	$(17,266)	$(10,256)	$(57,403)	$(27,254)
Net loss per share:				
Basic and diluted	$(0.29)	$(0.21)	$(1.01)	$(0.56)
Weighted-average shares outstanding used to compute:				
Basic and diluted	59,964	49,766	56,658	48,899

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

Raptor Pharmaceutical Corp.
Condensed Consolidated Statement of Stockholders' Equity
For the Nine Months Ended September 30, 2013
(Unaudited)
(In thousands, except per share data, or unless otherwise specified)

	Common stock Shares	Amount	Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total
Balance at December 31, 2012	52,425	$ 52	$ 155,945	$ (115)	$ (135,938)	$ 19,944
Exercise of common stock warrants	3,401	3	9,806	0	0	9,809
Exercise of common stock options	378	1	1,515	0	0	1,516
Employee stock-based compensation expense	0	0	5,417	0	0	5,417
Consultant stock-based compensation expense	0	0	3	0	0	3
Reclassification of the fair value of warrant liabilities upon exercise	0	0	18,091	0	0	18,091
Issuance of common stock under an at-the-market sales agreement, net of commissions and fundraising costs totaling $1,572	4,939	5	38,387	0	0	38,392
Foreign currency translation loss	0	0	0	(78)	0	(78)
Net loss	0	0	0	0	(57,325)	(57,325)
Balance at September 30, 2013	61,143	$ 61	$ 229,164	$ (193)	$ (193,263)	$ 35,769

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

Raptor Pharmaceutical Corp.
Condensed Consolidated Statements of Cash Flows
(unaudited)
(In thousands, except per share data, or unless otherwise specified)

	For the nine months ended	
	September 30, 2013	August 31, 2012
Cash flows from operating activities:		
Net loss	$(57,325)	$(27,211)
Adjustments to reconcile net loss to net cash used in operating activities:		
Employee stock-based compensation expense	5,417	3,570
Consultant stock-based compensation expense	3	72
Fair value adjustment of common stock warrants	12,025	(995)
Amortization of intangible assets	134	109
Depreciation of fixed assets	152	32
Realized (gain) on sale of fixed assets	(12)	0
Realized (gain) loss on short-term investments	129	(215)
Unrealized (gain) on short-term investments	0	(33)
Amortization of debt issuance costs	289	0
Amortization of deferred offering costs	373	32
Write-off of intangible assets and other intellectual property	0	900
Changes in assets and liabilities:		
Accounts receivable, net	(5,519)	0
Inventories, net	(2,218)	0
Prepaid expenses and other	(1,154)	(2,781)
Intangible assets	(1,250)	0
Deposits	(127)	0
Accounts payable	(2,752)	(664)
Accrued liabilities	7,110	994
Deferred revenue	3,010	0
Deferred rent	151	(7)
Net cash used in operating activities	(41,564)	(26,197)
Cash flows from investing activities:		
Purchase of fixed assets	(1,060)	(364)
Sale of fixed assets	27	0
Change in restricted cash	(337)	(56)
Purchase of short-term investments	(147)	(15,040)
Sale of short-term investments	22,114	30,000
Net cash provided by investing activities	20,597	14,540
Cash flows from financing activities:		
Proceeds from the sale of common stock under an ATM sales agreement	39,964	7,684
Proceeds from the exercise of common stock warrants	9,809	3,057
Proceeds from the exercise of common stock options	1,516	325
Note payable	25,000	0
Fundraising costs	(1,572)	(360)

Debt issuance costs	(1,259)	0
Deferred offering costs	(436)	(166)
Capital lease, net	44	8
Net cash provided by financing activities	73,066	10,548
Effect of exchange rates on cash and cash equivalents	(78)	(43)
Net increase (decrease) in cash and cash equivalents	52,021	(1,152)
Cash and cash equivalents, beginning of period	36,313	24,732
Cash and cash equivalents, end of period	$88,334	$23,580

Supplemental cash flow information:		
Interest paid	$2,314	$3
Supplemental disclosure of non-cash financing activities:		
Fair value of warrant liability reclassified to equity upon exercise	$18,091	$7,106

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

RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
SEPTEMBER 30, 2013
(UNAUDITED)

1. DESCRIPTION OF BUSINESS AND SIGNIFICANT ACCOUNTING POLICIES

Raptor Pharmaceutical Corp. (the "Company" or "Raptor") is a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases.

The Company's first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules ("PROCYSBI"), received approval from the U.S. Food and Drug Administration ("FDA") on April 30, 2013 for the management of nephropathic cystinosis in adults and children six years and older. The European equivalent, PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received marketing authorization on September 6, 2013 from the European Commission ("EC") as an orphan medicinal product for the treatment of proven nephropathic cystinosis for marketing in the European Union ("EU"). PROCYSBI received 7 years and 10 years of market exclusivity as an orphan drug in the U.S. and the EU, respectively. The Company commenced commercial sales of PROCYSBI in the U.S. in mid-June 2013 and plans to launch PROCYSBI in the EU in the first half of 2014. Prior to the second quarter of 2013, the Company had been in its development stage as defined by Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") 915, Development Stage Entities. With FDA approval of PROCYSBI and the commencement of commercial sales in the second quarter of 2013, the Company was no longer considered to be in the development stage. In the near-term, the Company's ability to generate revenues is entirely dependent upon sales of PROCYSBI in the U.S. and the EU.

Raptor's pipeline also includes its proprietary delayed-release form of cysteamine (DR Cysteamine or "RP103") in Phase 2/3 development for Huntington's disease and RP103 in Phase 2b development for nonalcoholic fatty liver disease ("NAFLD") in children. Raptor's other preclinical programs are based upon novel drug candidates that are designed to treat primary liver cancer and various other diseases.

The Company is subject to a number of risks, including: the level of commercial sales of PROCYSBI in the U.S. and the ability to launch PROCYSBI in the EU; the uncertainty of whether the Company's research and development efforts will result in expanded indications for PROCYSBI or additional commercial products; competition from larger organizations; reliance on licensing the proprietary technology of others; dependence on key personnel; uncertain patent protection; and the need to raise capital through equity and/or debt financings. See the section titled "Risk Factors" included elsewhere in this Quarterly Report on Form 10-Q.

Change in Fiscal Year End

In December 2012, Raptor's board of directors approved a change in the Company's fiscal year end from August 31 to December 31. The accompanying condensed consolidated financial statements cover the period from January 1, 2013 through September 30, 2013, representing the first nine months of Raptor's recently adopted fiscal year. The prior year's comparable nine-month period covers December 1, 2011 through August 31, 2012, which is reported on the basis of Raptor's previous fiscal year end. As a result of the change in Raptor's fiscal year end, the quarterly periods of Raptor's newly adopted fiscal year do not coincide with the historical quarterly periods that Raptor had previously reported. The Company did not recast the results for the 2012 fiscal periods because the financial reporting processes in place at the time included certain procedures that were only performed on a quarterly basis. Consequently, to recast these periods would have been impractical and would not have been cost-justified. The Company believes the

comparative information provided for the three and nine-month periods ended August 31, 2012 provides a meaningful comparison to the three- and nine-month periods ended September 30, 2013, and that there are no factors, seasonal or otherwise, that have a material impact on the comparability of information or trends.

Basis of Presentation

The accompanying condensed consolidated financial statements reflect the results of operations of Raptor and have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP") and in conjunction with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all information and footnotes required by GAAP for complete financial statements. In the opinion of management, the accompanying condensed consolidated financial statements include all adjustments (including normal recurring accruals) considered necessary for the fair presentation of the Company's consolidated financial position, results of operations and cash flows for the periods presented. This Form 10-Q should be read in conjunction with the audited financial statements and accompanying notes in the Company's Transition Report on Form 10-KT for the four-month period ended December 31, 2012, as amended.

The Company's condensed consolidated financial statements include the accounts of the Company's direct and indirect wholly owned subsidiaries, Raptor Pharmaceuticals Inc., formerly known as Raptor Therapeutics Inc. which merged with Raptor Discoveries Inc. in December 2012 prior to changing its name and Raptor European Products, LLC, such subsidiaries incorporated in Delaware on August 1, 2007, and February 14, 2012, respectively, and Raptor Pharmaceuticals Europe B.V. (domiciled in the Netherlands and formed on December 15, 2009), Raptor Pharmaceuticals France SAS (incorporated in France on October 30, 2012), Raptor Pharmaceuticals Germany GmbH (formed on September 28, 2013 and incorporated in Germany as a German company with limited liability on October 15, 2013) and RPTP European Holdings C.V. (located in the Grand Caymans and formed on February 16, 2012). All intercompany accounts have been eliminated. The Company's condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. Through September 30, 2013, the Company had accumulated losses of approximately $193.3 million. Management expects to incur further losses for the foreseeable future.

The Company believes that based upon its projected PROCYSBI net sales and planned operations, its cash and cash equivalents as of September 30, 2013 of approximately $88.3 million will be sufficient to meet its projected operational requirements and obligations through at least the end of 2014.

Use of Estimates

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

Revenue Recognition and Deferred Revenue

The Company recognizes revenue in accordance with the FASB ASC 605, Revenue Recognition, when the following criteria have been met: persuasive evidence of an arrangement exists; delivery has occurred and risk of loss has passed; the seller's price to the buyer is fixed or determinable and collectability is reasonably assured. The Company determines that persuasive evidence of an arrangement exists based on written contracts that define the terms of the arrangements. Pursuant to the contract terms, the Company determines when title to products and associated risk of loss has passed onto the customer. The Company assesses whether the fee is fixed or determinable based on the payment terms associated with the transaction and whether the sales price is subject to refund or adjustment. The Company assesses collectability based primarily on the customer's payment history and creditworthiness. Without sufficient credit history, the Company determines a customary collectability percentage.

PROCYSBI is currently only available for distribution from the Company's U.S. specialty pharmacy partner, which ships directly to patients. PROCYSBI is not available in retail pharmacies. Prior authorization of coverage level by

patients' private insurance plans, our patient assistance program ("PAP") or government payors is a prerequisite to the shipment of PROCYSBI to patients. Revenue is recognized once the product has been shipped by the specialty pharmacy and receipt confirmed by patients. Billings to the Company's distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenues by the Company until such deliveries to patients occur.

The Company records revenue net of expected discounts, distributor fees, returns and rebates, including those paid to Medicare and Medicaid in the U.S. Allowances are recorded as a reduction of revenue at the time product sales are recognized. Allowances for government rebates and discounts are established based on the actual payor information, which is known at the time of shipment, and the government-mandated discounts applicable to government-funded programs. The allowances are adjusted to reflect known changes in the factors that may impact such allowances in the quarter the changes are known.

Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price, with cost determined on a first-in, first-out basis. Inventories are reviewed periodically to identify slow-moving or obsolete inventory based on sales activity, both projected and historical, as well as product shelf-life. In evaluating the recoverability of inventories produced in preparation for product launches, the Company considers the probability that revenue will be obtained from the future sale of the related inventory. Prior to the approval of PROCYSBI by the FDA on April 30 2013, the Company recorded manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to approval, the Company began capitalizing these costs as commercial inventory. On September 30, 2013, net inventories were approximately $2.2 million, which consisted of $1.8 million of raw materials, $0.3 million of work-in-process and $0.5 million of finished goods, offset by a reserve allowance of $0.4 million. Upon launching PROCYSBI in mid-June 2013, the Company began recognizing cost of sales. During the second quarter ended June 30, 2013, the Company recorded a $0.4 million reserve as cost of sales expense representing commercial inventory that was capitalized subsequent to FDA approval but written off due to an unanticipated minor change in the finished product presentation. No write-off occurred in the third quarter and is not expected to be repeated in the future. Cost of sales includes the cost of inventory sold and reserved, manufacturing and supply chain costs, product shipping and handling costs, amortization of licensing approval milestone payments and licensing royalties payable to the University of California, San Diego ("UCSD").

Comprehensive Loss

Components of comprehensive loss are reported in the Company's Condensed Consolidated Statements of Comprehensive Loss in the period in which they are recognized. The components of comprehensive loss include net loss and foreign currency translation adjustments.

Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less, when purchased, to be cash equivalents. The Company maintains cash and cash equivalents, which consist principally of money market funds with high credit quality financial institutions. Such amounts exceed Federal Deposit Insurance Corporation insurance limits. Restricted cash represents compensating balances required by the Company's U.S. bank as collateral for credit cards.

Short-term Investments

The Company typically invests in short-term investments in high credit-quality funds in order to obtain higher yields on its cash available for investment. The Company had no short-term investments at September 30, 2013 compared to approximately $22.1 million at December 31, 2012. The Company regularly evaluates its short-term investment fund options and in the future may invest a portion of its cash and cash equivalents balance ($88.3 million as of September 30, 2013) in higher credit-quality and higher-yielding short-term investment funds.

Such investments are not insured by the Federal Deposit Insurance Corporation. The Company completed an evaluation of its investments and determined that it did not have any other-than-temporary impairments at December 31, 2012. The investments were placed in financial institutions with strong credit ratings.

Accounts Receivable

Trade accounts receivable are recorded net of product sales allowances for prompt-payment discounts, chargebacks and doubtful accounts. Estimates for chargebacks and prompt-payment discounts are based on contractual terms and the Company's expectations regarding the utilization rates. As of September 30, 2013, the Accredo Health Group, Inc.

("Accredo"), Raptor's exclusive distributor in the U.S., is the Company's only significant customer for PROCYSBI in the U.S. Raptor's distributor in the EU will be the Almac Group, Ltd. for the commercial launch in the EU anticipated to occur in the first half of 2014.

Functional Currency

The Company's consolidated functional currency is the U.S. dollar. Raptor Pharmaceuticals Europe B.V. ("BV"), Raptor Pharmaceuticals France SAS ("SAS") and RPTP European Holdings C.V. ("CV"), the Company's European subsidiaries and Cayman-based subsidiary, respectively, use the European Euro as their functional currency. At each quarter end, BV's, SAS's and CV's balance sheets are translated into U.S. dollars based upon the quarter-end exchange rate, while their statements of comprehensive loss are translated into U.S. dollars based upon an average of the Euro's value between the beginning and end date of the reporting period. BV's, SAS's and CV's equity are adjusted for any translation gain or loss.

Fair Value of Financial Instruments

The carrying amounts of certain of the Company's financial instruments including cash and cash equivalents, restricted cash, prepaid expenses, accounts payable, accrued liabilities and capital lease liability approximate fair value due either to length of maturity or interest rates that approximate prevailing market rates unless otherwise disclosed in these condensed consolidated financial statements. The warrant liability is carried at fair value which is determined using the Black-Scholes option valuation model at the end of each reporting period.

The Company uses a fair value approach to value certain assets and liabilities. Fair value is the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. The Company uses a fair value hierarchy, which distinguishes between assumptions based on market data (observable inputs) and an entity's own assumptions (unobservable inputs). The hierarchy consists of three levels:

- Level one - Quoted market prices in active markets for identical assets or liabilities;
- Level two - Inputs other than level one inputs that are either directly or indirectly observable; and
- Level three - Unobservable inputs developed using estimates and assumptions, which are developed by the reporting entity and reflect those assumptions that a market participant would use.

Determining which category an asset or liability falls within the hierarchy requires significant judgment. The Company evaluates its hierarchy disclosures each quarter. Assets and liabilities measured at fair value on a recurring basis at September 30, 2013 and December 31, 2012 are summarized as follows:

(In thousands)

Assets	Level 1	Level 2	Level 3	September 30, 2013
Fair value of cash equivalents	$71,434	$0	$0	$71,434
Restricted cash	0	500	0	500
Total	$71,434	$500	$0	$71,934
Liabilities				
Fair value of common stock warrants	$0	$0	$10,339	$10,339
Total	$0	$0	$10,339	$10,339

Assets	Level 1	Level 2	Level 3	December 31, 2012

Fair value of cash equivalents	$35,069	$0	$0	$35,069
Restricted cash	0	163	0	163
Short-term investments	22,096	0	0	22,096
Total	$57,165	$163	$0	$57,328
Liabilities				
Fair value of common stock warrants	$0	$0	$16,405	$16,405
Total	$0	$0	$16,405	$16,405

Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine (developed as PROCYSBI and RP103), capitalized licensing milestone payments to UCSD based upon drug approval and an out-license acquired in the merger of the Company's subsidiary with and into Raptor Pharmaceuticals Corp. in September 2009 ("2009 Merger"). The other intangible assets related to intellectual property of PROCYSBI/RP103 are amortized using the straight-line method over the estimated useful life of 20 years, which is the life of the intellectual property patents. The 20-year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. Licensing milestone payments are amortized upon relevant regulatory approval through 2027, the remaining life of the patent. Intangible assets related to the out-license are amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents.

Goodwill

Goodwill represents the excess of the value of the purchase consideration over the identifiable assets acquired in the 2009 Merger. Goodwill is reviewed annually, or when an indication of impairment exists. When an impairment analysis is performed, if deemed necessary, a write-down in valuation is recorded.

Fixed Assets

Fixed assets, which mainly consist of leasehold improvements, furniture and fixtures, lab equipment, computer hardware and software and capital lease equipment, are stated at cost. Depreciation is computed using the straight-line method over the related estimated useful lives, except for leasehold improvements and capital lease equipment, which are depreciated over the shorter of the useful life of the asset or the lease term. Significant additions and improvements that have useful lives estimated at greater than one year are capitalized, while repairs and maintenance are charged to expense as incurred.

Impairment of Long-Lived Assets

The Company evaluates its long-lived assets for indicators of possible impairment by comparison of the carrying amounts to future net undiscounted cash flows expected to be generated by such assets when events or changes in circumstances indicate the carrying amount of an asset may not be recoverable. Should an impairment exist, the impairment loss would be measured based on the excess carrying value of the asset over the asset's fair value or discounted estimates of future cash flows.

Accrued Liabilities

Accrued liabilities include estimates of certain expenses for which the Company has not yet been invoiced and which requires management's judgment in determining appropriate expenses to accrue. For example, because of the nature of how clinical trials are invoiced by clinical sites, especially outside of the U.S. where there is a significant time lag between the services provided by the clinical site and the time the clinical site bills the Company for their services, the Company must estimate such clinical site expenses on a monthly basis as clinical trial expenses. Although the Company believes its accrued liabilities reflect the best information available to it, the Company's actual expenses could differ from its estimates.

Common Stock Warrant Liabilities

The warrants issued by the Company in the 2010 private placement contain a cash-out provision which may be triggered upon request by the warrant holders if the Company is acquired or upon the occurrence of certain other

fundamental transactions involving the Company. This provision requires these warrants to be classified as liabilities and to be marked to market at each period-end commencing on August 31, 2010. The warrants issued by the Company in its December 2009 equity financing contain a conditional obligation that may require the Company to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under FASB ASC Topic 480, Distinguishing Liabilities from Equity ("ASC 480"), a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, the Company has classified the warrants as liabilities and will mark them to fair value at each period end. The common stock warrants are re-measured at the end of every reporting period with the change in value reported in the Company's Condensed Consolidated Statements of Comprehensive Loss. Warrants which are recorded as liabilities that are exercised are re-measured and marked to market the day prior to exercise. Upon exercise of such warrants, the fair value of such warrants is reclassified to equity.

Deferred Offering Costs

Deferred offering costs represent expenses incurred to raise equity capital related to financing transactions which have not yet been completed as of the balance sheet dates.

Note Payable and Debt Issuance Costs

Note payable consists of the Company's loan agreement with HealthCare Royalty Partners II, L.P. ("HC Royalty"), as lender, under which Raptor agreed to borrow $50.0 million in two $25.0 million tranches. The first tranche was received in December 2012 and the second tranche was received in May 2013. The loan bears interest at an annual fixed rate of 10.75% of outstanding principal and quarterly interest payments are included in interest expense in the Condensed Consolidated Statements of Comprehensive Loss. Principal payments, when made, reduce the note payable balance. There is a synthetic royalty component based on net product sales, including PROCYSBI, in a calendar year, and such royalty is payable quarterly. The royalty fees payable to HC Royalty are included in interest expense in the Condensed Consolidated Statements of Comprehensive Loss. Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan using the effective interest method. The amortization of debt issuance costs is included in interest expense in the Condensed Consolidated Statements of Comprehensive Loss.

Research and Development Costs

Research and development costs are charged to expense as incurred. Research and development expenses include medical, clinical, regulatory and scientists' salaries and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, commercial drug manufacturing prior to obtaining marketing approval, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated human resources and facilities expenses. Research and development expenses are offset by contra-expenses, which are reimbursements of research and development expenses received either from research collaborators or from government grants or tax rebates.

Income Taxes

Income taxes are recorded under the liability method, under which deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to affect taxable income. Valuation allowances are established when necessary to reduce deferred tax assets to the amount expected to be realized.

The Company's effective income tax rate is 0% for the three and nine months ended September 30, 2013. The Company has determined that its effective tax rate for fiscal year ended August 31, 2012 and the short tax year from September 1, 2012 to December 31, 2012 is 0%. Based on the weight of available evidence, including cumulative losses since inception and expected future losses, the Company has determined that it is more likely than not that the deferred tax asset amount will not be realized and therefore a full valuation allowance has been provided on the Company's net deferred tax assets.

Utilization of the Company's net operating loss ("NOL") carryovers may be subject to substantial annual limitation due to the ownership change rules under the Internal Revenue Code and similar state income tax law provisions including those related to the suspension and limitation of NOL carryovers for certain tax years. Such an annual limitation could result in the expiration of the NOL carryovers before utilization.

The Company accounts for income taxes under FASB ASC No. 740-10, Accounting for Uncertainty in Income Taxes. Under this approach, deferred tax assets and liabilities are recognized based on anticipated future tax consequences, using currently enacted tax laws attributed to temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts calculated for income tax purposes.

The Company recognizes interest and/or penalties related to income tax matters as a component of income tax expense. As of September 30, 2013, there were no accrued interest or penalties related to uncertain tax positions. As of September 30, 2013, there were no unrecognized tax benefits for which the liability for such taxes were recognized as deferred liabilities.

The Company files U.S. Federal, California and other state income tax returns. In addition, the Company files income tax returns in France and the Netherlands. The Company is currently not subject to any income tax examinations. Due to the Company's NOLs, generally all tax years remain open.

Net Loss per Share

Net loss per share is calculated by dividing net loss by the weighted-average shares of common stock outstanding during the period. Diluted net income per share is calculated by dividing net income by the weighted-average shares of common stock outstanding and potential shares of common stock during the period. For all periods presented, potentially dilutive securities are excluded from the computation of fully diluted net loss per share as their effect is anti-dilutive. Potentially dilutive securities include:

(In thousands)	Nine months ended September 30, 2013	August 31, 2012
Warrants to purchase common stock	1,145	5,188
Options to purchase common stock	8,230	6,125
Total potentially dilutive securities	9,375	11,313

Net loss per share, basic and diluted, was $(0.29) and $(0.21) for the three months ended September 30, 2013 and August 31, 2012, respectively, and $(1.01) and $(0.56) for the nine months ended September 30, 2013 and August 31, 2012, respectively.

Stock Option Plan

Effective September 1, 2006, the Company adopted the provisions of FASB ASC Topic 718, Accounting for Compensation Arrangements, ("ASC 718") (previously listed as Statement of Financial Accounting Standards ("SFAS") No. 123 (revised 2004), Share-Based Payment) in accounting for its stock option plans. Under ASC 718, compensation cost is measured at the grant date based on the fair value of the equity instruments awarded and is recognized over the period during which an employee is required to provide service in exchange for the award, or the requisite service period, which is usually the vesting period. The fair value of the equity award granted is estimated on the date of the grant. The Company accounts for stock options issued to third parties, including consultants, in accordance with the provisions of the FASB ASC Topic 505-50, Equity-Based Payments to Non-Employees ("ASC 505-50") (previously listed as Emerging Issues Task Force Consensus No. 96-18, Accounting for Equity Instruments That Are Issued to Other Than Employees for Acquiring, or in Conjunction with Selling Goods or Services). See Note 7, Stock Option Plans, for further discussion of employee stock-based compensation.

For the quarter ended September 30, 2013, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 1.51%; 5 year expected life; 67.18% volatility; 2.5% turnover rate; and 0% dividend rate.

The Black-Scholes inputs were based on the following factors:

- the risk-free interest rate was based upon the Company's review of current constant maturity treasury bill rates for five years;

- the expected life of five years was based upon the Company's assessment of the ten-year term of the stock options issued, the fact that the Company has commercial sales and option holders have started to and will likely continue to exercise their stock options that are fully vested;
- the volatility was based on the actual annualized volatility of the Company's common stock price as quoted on NASDAQ since the closing of the 2009 Merger on September 30, 2009;
- the turnover rate was based on an assessment of the Company's historical employee turnover; and
- the dividend rate was based on the Company's current decision to not pay dividends on its stock during its current stage.

Reclassifications

Certain amounts previously reported under specific financial statement captions have been reclassified to be consistent with the current period presentation.

2. INTANGIBLE ASSETS AND GOODWILL

On December 14, 2007, the Company acquired the intellectual property and other rights to develop PROCYSBI/RP103 to treat various clinical indications from UCSD by way of a merger with Encode Pharmaceuticals, Inc., a privately held development stage company ("Encode"), which held the intellectual property license with UCSD. The intangible assets acquired in the merger with Encode were recorded at approximately $2.6 million, primarily based on the value of the Company's common stock and warrants issued to the Encode stockholders. Based upon FDA and EC approval of PROCYSBI, the Company paid and capitalized $750,000 and $500,000, respectively, earned by UCSD as a licensing milestone payment which is being amortized through 2027, the life of the licensed patents.

Intangible assets originally recorded as a result of the 2009 Merger were approximately $1.1 million of which $0.9 million was written off as of August 31, 2012 as discussed below.

Summary of intangible assets acquired as discussed above:

(In thousands)	September 30, 2013	December 31, 2012
Intangible asset (IP license for PROCYSBI/RP103) related to the Encode merger	$ 2,620	$ 2,620
Intangible assets (out-license) related to the 2009 Merger	240	240
Intangible assets (UCSD license FDA and EC approval milestones)	1,250	0
Total intangible assets	4,110	2,860
Less accumulated amortization	(838)	(704)
Intangible assets, net	$ 3,272	$ 2,156

The intangible assets related to PROCYSBI/RP103 are being amortized monthly over 20 years, which are the lives of the intellectual property patents and the estimated useful life. The 20-year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. The intangible assets related to the out-license are amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents. At August 31, 2012, the Company determined that the capitalized acquired in-process research and development cost of $0.9 million, representing the tezampanel and NGX 426 program acquired in the 2009 Merger, was impaired due to the Company's decision to discontinue development of this product candidate for thrombosis due to regulatory hurdles that would require significant expenditures which the Company chose not to prioritize for funding. The Company performed an impairment analysis and determined that the fair value of this intangible asset was zero. As such, the Company expensed $0.9 million as in-process research and development as part of research and development expense on the Company's consolidated statements of comprehensive loss for the fiscal year ended August 31, 2012. During the three and nine months ended September 30, 2013, the Company did not identify any impairment losses.

The Company amortized approximately $47,000 and $37,000 of intangible assets during the three-month periods ended September 30, 2013 and August 31, 2012, respectively, and $134,000 and $109,000 during the nine-month periods ended September 30, 2013 and August 31, 2012, respectively. These amounts relate to the amortization of the intangible assets listed above which includes milestone fees earned by UCSD in the second and third quarters of 2013 of $750,000 and $500,000, respectively, upon the FDA and EC approval of PROCYSBI.

The following table summarizes the actual and estimated amortization expense for intangible assets for the periods indicated:

Amortization period (In thousands)	Amortization expense
Year ending December 31, 2013 – estimate	$ 194
Year ending December 31, 2014 – estimate	238
Year ending December 31, 2015 – estimate	238
Year ending December 31, 2016 – estimate	238
Year ending December 31, 2017 – estimate	238

Goodwill of approximately $3.3 million represents the excess of total consideration recorded for the 2009 Merger over the value of the assets assumed. The Company tested the carrying value of goodwill for impairment as of the end of its transition period for the four month period ended December 31, 2012 and determined that there was no impairment. Intangible assets are tested for impairment whenever events indicate that their carrying values may not be recoverable. During the fiscal year ended August 31, 2012, the tezampanel/NGX426 asset was written off with a carrying value of approximately $0.9 million.

3. FIXED ASSETS

Fixed assets consisted of:

Category (In thousands)	September 30, 2013	December 31, 2012	Estimated useful lives
Leasehold improvements	$ 0	$ 146	Shorter of life of asset or lease term
Office furniture	497	35	7 years
Laboratory equipment	902	593	5 years
Computer hardware and software	426	204	3 years
Capital lease equipment	68	27	Shorter of life of asset or lease term
Total at cost	1,893	1,005	
Less: accumulated depreciation	(584)	(589)	
Total fixed assets, net	$ 1,309	$ 416	

Depreciation expense for the three months ended September 30, 2013 and August 31, 2012 was approximately $74,000, and $29,000, respectively. Depreciation expense for the nine months ended September 30, 2013 and August 31, 2012 was approximately $152,000 and $32,000, respectively. Accumulated depreciation on capital lease equipment was approximately $6,000 and $8,000 as of September 30, 2013 and December 31, 2012, respectively.

4. NOTE PAYABLE AND DEBT ISSUANCE COSTS

Note payable consists of the Company's loan agreement with HC Royalty, as lender, under which the Company agreed to borrow $50.0 million in two $25.0 million tranches. The Company drew down the first tranche in the amount of $25.0 million in December 2012 and the second tranche in May 2013. The loan bears interest at an annual fixed rate of 10.75% of outstanding principal and quarterly interest payments are included in interest expense in the Company's Condensed Consolidated Statements of Comprehensive Loss for the quarter ended September 30, 2013. Principal payments, when made, reduce the Company's note payable balance. There is a synthetic royalty component based on net product revenues, including PROCYSBI, in a calendar year, and such royalty is payable quarterly. With respect to the first $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.25% of the first $25.0 million of PROCYSBI and future approved product net revenues for such calendar year, 3.0% of the PROCYSBI and future approved product net revenues for such calendar year in excess of $25.0 million and not in excess of $50.0 million, and 1.0% of the PROCYSBI and future approved product net revenues for such calendar year in excess of $50.0 million, payable quarterly. With respect to the second $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.0% of the first $25.0 million of PROCYSBI and future approved product net revenues for such calendar year, 3.0% of the PROCYSBI and future approved product net revenues for such calendar year in excess of $25.0 million and not in excess of $50.0 million, and 1.0% of the PROCYSBI and future approved product net revenues for such calendar year in excess of $50.0 million, payable quarterly.

The Company received marketing approval of PROCYSBI from the FDA on April 30, 2013 and commenced shipment of PROCYSBI during June 2013, and as a result, royalties became payable to HC Royalty based upon net

revenues of PROCYSBI. Interest expense on the loan and royalty costs payable to HC Royalty are classified as interest expense in the Company's Condensed Consolidated Statements of Comprehensive Loss and accrued liabilities on the Company's Condensed Consolidated Balance Sheets. Interest expense on the loan for the three and nine months ended September 30, 2013 was approximately $2.3 million and $4.1 million, respectively. As of September 30, 2013, the Company's note payable balance was $50.0 million and accrued royalty interest was $0.8 million.

During the quarter ended September 30, 2013, using the effective interest rate method, the Company performed an analysis of its estimated future revenues and determined that it did not need to accrue additional interest expense on its loan at September 30, 2013. The loan and the Company's obligation to make any payments shall terminate immediately when all payments received by HC Royalty equal $97.5 million. If, by December 20, 2014, net revenues for the immediately preceding four fiscal quarters exceed $100.0 million, then the loan and the Company's obligation to make any payments shall terminate immediately when all payments received by HC Royalty from the Company equal $90.0 million. Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan using the effective interest method. The amortization of debt issuance costs is included in interest expense in the Company's Condensed Consolidated Statements of Comprehensive Loss.

5. CAPITAL STRUCTURE

As of September 30, 2013 and December 31, 2012, there were 61,142,756 and 52,424,649 shares, respectively, of the Company's common stock issued and outstanding.

Common Stock Issuances under Stock Option Plans

The Company received approximately $1.5 million from the exercise of stock options for the three- and nine-month periods ended September 30, 2013, resulting in the issuance of 370,352 shares and 378,366 shares, respectively, of common stock.

Common Stock Issuance under At-The-Market Sales Agreement

On April 30, 2012, the Company entered into a Sales Agreement with Cowen and Company, LLC ("Cowen"), under which the Company could, at its discretion, sell its common stock with a sales value of up to a maximum of $40.0 million through offerings deemed to be "at-the-market" ("ATM") on the NASDAQ Stock Market. The Company pays Cowen as the sole sales agent a commission of 3.0% of the gross sales price for any sales made under the ATM. The common stock is sold at prevailing market prices at the time of the sale of common stock, and, as a result, prices will vary.

On July 3, 2013, the Company and Cowen amended and restated the Sales Agreement (the "Amended and Restated Sales Agreement") to increase the aggregate gross sales proceeds that may be raised to $100,000,000, of which approximately $37.1 million was previously sold pursuant to the original Sales Agreement dated April 30, 2012.

Sales in the ATM offerings are being made pursuant to the prospectus supplement dated April 30, 2012, as amended by Amendment No. 2 dated July 3, 2013, which supplements the Company's prospectus dated February 3, 2012, filed as part of the shelf registration statement that was declared effective by the Securities and Exchange Commission ("SEC") on February 3, 2012. Cumulatively through September 30, 2013, the Company sold 7,599,474 shares under the ATM offerings at a weighted-average selling price of $7.08 per share for net proceeds of approximately $52.1 million.

Common Stock Warrants

For the three- and nine-month periods ended September 30, 2013, the Company received approximately $3.1 million and $9.8 million, respectively, from the exercise of warrants in exchange for the issuance of 1,067,445 shares and 3,400,725 shares, respectively, of the Company's common stock.

The table reflects the number of common stock warrants outstanding as of September 30, 2013:

(In thousands, except per share amounts)	Number of shares exercisable	Exercise price	Expiration date
Issued in connection with Encode merger	233	$2.87	12/13/2015
Issued to placement agents in August 2009	65	$1.50	7/31/2014
TorreyPines warrants assumed in 2009 Merger	4	$157.08	9/26/2015
Issued to registered direct investors in Dec. 2009	75	$2.45	12/22/2014
Issued to private placement investors in Aug. 2010	670	$3.075	8/12/2015
Issued to placement agent in Aug. 2010	98	$3.075	8/12/2015
Total warrants outstanding	1,145	$3.37	*

* Weighted-average exercise price

The warrants issued by the Company in the August 2010 private placement and the December 2009 equity financing contain a conditional obligation that may require the Company to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under ASC 480, a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, the Company has classified the warrants from both financings as liabilities and will mark them to fair value at each period end.

A Black-Scholes option-pricing model was used to obtain the fair value of the warrant liabilities. These warrants were issued in the December 2009 and August 2010 equity financings using the following assumptions at September 30, 2013 and December 31, 2012:

	December 2009 equity financing Series A		August 2010 private placement investors and placement agent	
	September 30, 2013	December 31, 2012	September 30, 2013	December 31, 2012
Fair value ($ millions)	$0.9	$ 2.6	$9.4	$ 13.8
Black-Scholes inputs:				
Stock price	$14.92	$ 5.85	$14.92	$ 5.85
Exercise price	$2.45	$ 2.45	$3.075	$ 3.075
Risk free interest rate	0.21 %	0.25 %	0.29 %	0.31 %
Volatility	95 %	100 %	95 %	112 %
Expected term (years)	1.25	2.0	1.75	2.5
Dividend	0	0	0	0

Marked-to-Market

As a result of the marking-to-market of the warrant liability at quarter-end and the day prior to the exercise of warrants subject to warrant liability accounting, the Company recorded a loss of approximately $6.0 million and a gain of approximately $1.9 million for the three months ended September 30, 2013 and August 31, 2012, respectively, and a loss of approximately $12.0 million and a gain of approximately $1.0 million for the nine months ended September 30, 2013 and August 31, 2012, respectively, in the line item adjustment to fair value of common stock warrants in its Condensed Consolidated Statements of Comprehensive Loss.

Below is the activity of the warrant liabilities for the nine months ended September 30, 2013 and August 31, 2012:

	For the nine months ended	
(In millions)	September 30, 2013	August 31, 2012
Fair value of December 2009 direct offering warrants (including placement agent warrants) at beginning of the nine-month periods ended September 30, 2013 and August 31, 2012	$2.6	$6.7
December 2009 direct offering warrants exercised	(4.2)	(4.5)
Adjustment to mark to market common stock warrants	2.5	0.7
December 2009 direct offering common stock warrant liability at fair value at September 30, 2013 and August 31, 2012	0.9	2.9
Fair value of August 2010 private placement warrants (including broker warrants) at beginning of the nine-month periods ended September 30, 2013 and August 31, 2012	13.8	18.7
August 2010 private placement warrants exercised	(13.9)	(2.7)
Adjustment to mark to market common stock warrants	9.5	(1.6)
August 2010 private placement common stock warrant liability at fair value at September 30, 2013 and August 31, 2012	9.4	14.4
Total warrant liability at September 30, 2013 and August 31, 2012, respectively	$10.3	$17.3

Effect of Raptor's Stock Price and Volatility Assumptions on the Calculation of Fair Value of Warrant Liabilities

As discussed above, the Company uses the Black-Scholes option pricing model as its method of valuation for warrants that are subject to warrant liability accounting. The determination of the fair value as of the reporting date is affected by Raptor's stock price as well as assumptions regarding a number of highly complex and subjective variables which could provide differing variables. These variables include, but are not limited to, expected stock price volatility over the term of the security and risk-free interest rate. In addition, the Black-Scholes option pricing model requires the input of an expected life for the securities which the Company has estimated based upon the stage of its development. The fair value of the warrant liability is revalued each balance sheet date utilizing Black-Scholes valuation model computations with the decrease or increase in fair value being reported in the Condensed Consolidated Statement of Comprehensive Loss as other income or expense, respectively. The Company's reported net loss was approximately $57.3 million for the nine months ended September 30, 2013. If the Company's September 30, 2013 closing stock price had been 10% lower, its net loss would have been approximately $1.2 million lower. If the Company's September 30, 2013 closing stock price had been 10% higher, its net loss would have been approximately $1.2 million higher. If the Company's September 30, 2013 volatility assumption had been 10% lower, its net loss would have been approximately $0.1 million lower. If the Company's September 30, 2013 volatility assumption had been 10% higher,

its net loss would have been approximately $0.1 million higher.

6. ACCRUED LIABILITIES

Accrued liabilities consisted of:

(In thousands)	September 30, 2013	December 31, 2012
Clinical trials and related costs	$ 2,022	$ 641
Employee bonuses and commissions	1,756	502
Commercial and administrative consulting	1,007	167
Salaries and employee benefits	1,106	742
Professional fees	815	67
Interest expense on loan	809	0
Revenue deductions	783	0
Manufacturing costs	533	0
Royalties	363	0
Other	66	31
Total accrued liabilities	$ 9,260	$ 2,150

7. STOCK OPTION PLANS

Stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following:

Period*	Risk-free interest rate	Expected life of stock option	Annual volatility
Quarter ended August 31, 2012	0.68 %	5 years	124.9 %
Quarter ended September 30, 2013	1.51 %	5 years	67.18 %

* Dividend rate is 0% for all periods presented.

If factors change and different assumptions are employed in the application of ASC 718, the compensation expense recorded in future periods may differ significantly from what was recorded in the current period.

Employee and consultant stock-based compensation expense has been included in the Condensed Consolidated Statements of Comprehensive Loss as follows:

	Three Months Ended		Nine Months Ended	
(In thousands)	September 30, 2013	August 31, 2012	September 30, 2013	August 31, 2012
Research and development	$381	$309	$1,123	$819
Selling, general and administrative	1,442	984	4,297	2,823
Total	$1,823	$1,293	$5,420	$3,642

A summary of the activity in the 2010 Equity Incentive Plan, as amended (the "Plan"), the 2006 Equity Compensation Plan, as amended, and the Company's other stock option plans, is as follows:

(In thousands, except per share amounts)	Option shares	Weighted-average exercise price	Exercisable	Weighted-average fair value of options granted
Outstanding at December 31, 2012	7,791	$ 5.79	3,494	$ 3.48
Granted	130	5.39	0	3.04
Exercised	(8)	0.85	0	0.62
Canceled	(28)	13.94	0	4.25
Outstanding at March 31, 2013	7,885	5.76	4,031	3.47
Granted	478	6.58	0	4.01
Exercised	0	0	0	0
Canceled	(27)	20.70	0	4.73
Outstanding at June 30, 2013	8,336	5.76	4,525	3.55
Granted	317	11.06	0	5.32
Exercised	(370)	4.07	0	3.27
Canceled	(53)	38.14	0	0
Outstanding at September 30, 2013	8,230	5.83	4,653	3.71

The weighted-average intrinsic values of stock options were as follows:

	Options outstanding and expected to vest for the quarter ended		Options exercisable for the quarter ended	
(In thousands)	September 30, 2013	August 31, 2012	September 30, 2013	August 31, 2012
Intrinsic value	$80,455	$4,841	$48,773	$4,131
Number of options	8,230	6,125	4,653	2,995

There were approximately 4.1 million shares available for grant as of September 30, 2013 under the Plan which includes a 3.0 million share increase to the Plan that was approved at the Company's Annual Shareholders' Meeting held on July 23, 2013. The amendments to the Plan approved at the Company's Annual Shareholders' Meeting held on March 22, 2011 allow for 50% accelerated vesting of unvested stock options upon a change of control as defined in the Plan. The amended and restated award agreement, subject to the terms of any applicable employment agreement, extends the termination date of the awards granted under the Plan that are vested as of such termination date due to (a) an employee's or a non-employee director's retirement at age 62 or older which employee or non-employee director has at least five (5) years of continuous service with the Company prior to such retirement, (b) the termination of a non-employee director's board membership for reasons other than for cause or retirement and (c) an employee's or a non-employee director's death (during his or her continuous service with the Company or within 90 days' of such continuous service with the Company) or permanent disability, to eighteen (18) months from the date of termination of continuous service with the Company. No further grants will be made under any previous or assumed stock option plans.

As of September 30, 2013, the options outstanding under all of the Company's stock option plans consisted of the following:

	Options outstanding			Options vested and exercisable	
Range of exercise prices	Number of options outstanding and expected to vest (#, in thousands)	Weighted-average remaining contractual life (yrs.)	Weighted-average exercise price ($)	Number of options exercisable (#, in thousands)	Weighted-average exercise price ($)
$0 to $2.00	71	5.69	$ 1.78	69	$ 1.77
$2.01 to $3.00	1,229	5.23	2.67	1,090	2.63
$3.01 to $4.00	1,685	7.14	3.51	1,466	3.52
$4.01 to $5.00	299	8.13	4.79	127	4.73
$5.01 to $6.00	3,979	8.48	5.29	1,762	5.26
$6.01 to $8.00	662	9.06	6.77	101	6.64
$8.01 to $12.00	225	9.82	10.56	0	0
$12.01 to $15.00	42	10.00	14.54	0	0
$15.01 to $964.24	38	2.31	230.05	38	230.05
	8,230	7.75	5.83	4,653	5.89

At September 30, 2013, the total unrecognized compensation cost was approximately $14.0 million. The weighted-average period over which it is expected to be recognized is approximately 3 years.

8. COMMITMENTS AND CONTINGENCIES

The Company maintains several contracts with suppliers, contract manufacturers, research organizations, clinical organizations, drug labelers and distributors and clinical sites, primarily to assist with clinical research and clinical and commercial manufacturing and distribution of PROCYSBI and clinical manufacturing of drug product for the Company's HD and NAFLD clinical collaborations. With the exception of the items listed below and updates as noted under Note 4 – Notes Payable and Debt Issuance Costs, the Company's contractual obligations did not change significantly during the quarter ended September 30, 2013 compared to those discussed in the Company's Transition Report on Form 10-KT for the four month period ended December 31, 2012, filed with the SEC on March 14, 2013, as amended by Form 10-KT/A filed with the SEC on September 19, 2013.

On April 25, 2013, the Company executed a seven-year lease for a facility in Novato, California which it moved into at the end of June 2013 to accommodate personnel growth. For the period June 2013 through May 2014, the Company is obligated to make lease payments of $19,460 per month. On June 10, 2013, the Company amended the lease to add space to accommodate its research laboratory. The Company is obligated to make additional lease payments of $1,870 per month for the period June 2013 through May 2014 under this amendment. The Company

plans to move to an adjacent facility when it becomes available in 2014. Rental expense for the larger adjacent facility will be higher than the rental expense for the current facility.

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

You should read the following discussion in conjunction with our condensed consolidated financial statements as of September 30, 2013, and the notes to such condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q. All references to "the Company", "we", "our" and "us" include the activities of Raptor Pharmaceutical Corp., Raptor Pharmaceuticals Inc., or Raptor Pharmaceuticals, Raptor European Products, LLC, RPTP European Holdings C.V., Raptor Pharmaceuticals Europe B.V., Raptor Pharmaceuticals France SAS and Raptor Pharmaceuticals Germany GmbH.

This Quarterly Report on Form 10-Q, including this "Management's Discussion and Analysis of Financial Condition and Results of Operations" section, contains "forward-looking statements," within the meaning of the Private Securities Litigation Reform Act of 1995. In some cases, these statements can be identified by the use of terminology such as "believes," "expects," "anticipates," "plans," "may," "might," "will," "could," "should," "would," "projects," "predicts," "intends," "continues," "estimates," "potential," "opportunity" or the negative of these terms or other comparable terminology. All such statements, other than statements of historical fact, including but not limited to statements regarding our future results of operations, projected revenues and expenses, business strategies and transactions, partnering relationships and research collaborations, operating efficiencies or synergies, competitive positions, growth opportunities for existing intellectual properties, technologies or products, plans and objectives of management, clinical studies and regulatory filings and markets for our securities involve substantial risks and uncertainties and constitute forward-looking statements for the purpose of the safe harbor provided by Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements are necessary estimates reflecting the best judgment of our management on the date on which they were made. You should not place undue reliance on these statements, which only reflect information available as of the date that they were made. We cannot give you any assurance that such forward-looking statements will prove to be accurate and such forward-looking events may not occur. Our business' actual operations, performance, development and results might differ materially from any forward-looking statement due to various known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q. Unless required by law, we do not undertake any obligation and disclaim any intention to update or release publicly any revisions to these forward-looking statements after the filing of this Quarterly Report on Form 10-Q.

Change in Fiscal Year End

In December 2012, our board of directors approved a change in our fiscal year end from August 31 to December 31. The following discussions cover the period from January 1, 2013 through September 30, 2013, representing the first nine months of our recently adopted fiscal year. The prior year's comparable nine-month period covers December 1, 2011 through August 31, 2012, which is reported on the basis of our previous fiscal year end. As a result of the change in our fiscal year end, the quarterly periods of our newly adopted fiscal year do not coincide with the historical quarterly periods that we had previously reported. We did not recast the results for the 2012 fiscal periods because the financial reporting processes in place at the time included certain procedures that were only performed on a quarterly basis. Consequently, to recast those periods would have been impractical and would not have been cost-justified. We believe the comparative information provided for the three- and nine-month periods ended August 31, 2012 provide a meaningful comparison to the three- and nine-month periods ended September 30, 2013 and that there are no factors, seasonal or otherwise, that have a material impact on the comparability of information or trends.

Plan of Operation and Overview

We are a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases.

Our first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules, or PROCYSBI, received marketing approval from the U.S. Food and Drug Administration, or FDA, on April 30, 2013 for the management of nephropathic cystinosis in adults and children six years and older. The European equivalent, PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received marketing authorization on September 6, 2013 from the European Commission, or EC, as an orphan medicinal product for the treatment of proven nephropathic cystinosis for marketing in the European Union, or EU. PROCYSBI received 7 years and 10 years of market exclusivity as an orphan drug in the U.S. and the EU, respectively. We commenced commercial sales of PROCYSBI in the U.S. in mid-June 2013 and plan to launch PROCYSBI in the EU in the first half of 2014.

We expect to have at least 160 U.S. commercial patients on PROCYSBI by the end of 2013 and have priced PROCYSBI at an average price of $250,000 per patient per year. In September 2013, we executed an agreement to participate in the U.S. State Medicare/Medicaid rebate program, which will be reflected in our net revenues from patients receiving State assistance.

PROCYSBI

PROCYSBI is a new therapy for the management of nephropathic cystinosis. PROCYSBI (formerly known as RP103 for cystinosis) capsules contain cysteamine bitartrate formulated into innovative microspheronized beads that are individually coated to create a delayed-release formulation with extended-release properties, allowing patients to maintain therapeutic systemic drug levels for a full 12-hour dosing period. The enteric coated beads are pH sensitive and bypass the stomach for dissolution and absorption in the more alkaline environment of the proximal small intestine. Randomized controlled clinical trials and extended treatment with PROCYSBI therapy demonstrated consistent cystine depletion as monitored by levels of the biomarker (and surrogate marker), white blood cell cystine.

About Nephropathic Cystinosis

Nephropathic cystinosis comprises 95% of cases of cystinosis, a rare, life-threatening metabolic lysosomal storage disorder that causes toxic accumulation of cystine in all cells, tissues and organs in the body. Elevated cystine leads to progressive, irreversible tissue damage and multi-organ failure, including kidney failure, blindness, muscle wasting and premature death. Nephropathic cystinosis is usually diagnosed in infancy after children present with symptoms including markedly increased urination, thirst, dehydration, gastrointestinal distress, failure to thrive, rickets, photophobia and specific kidney symptoms specific to Fanconi syndrome. Management of cystinosis requires lifelong therapy. If left untreated, the disease is usually fatal by the end of the first decade of life. There are an estimated 500 patients reported with cystinosis living in the U.S. and 2,000 worldwide. In October 2013, we executed a collaboration agreement with DaVita Clinical Research to screen blood samples from patients with end-stage renal disease in an effort to identify patients with unrecognized late-onset nephropathic cystinosis.

Cystine depletion is the only known treatment strategy for nephropathic cystinosis. Strict adherence to cystine depletion therapy is critical to achieve optimal clinical outcomes. Poor adherence results in poor sustained control of cystine levels, and patients consequently experience poor clinical outcomes, including kidney insufficiency leading to dialysis and kidney transplantation, muscle wasting and in some cases, premature death. Even brief interruptions in daily therapy can permit toxic accumulation of cystine, exposing tissues to renewed, progressive deterioration.

RP103 for Huntington's Disease

RP103 is currently being evaluated in a phase 2/3 clinical study as a potentially disease modifying agent in the treatment of Huntington's disease, or HD. HD, formerly called Huntington's chorea, is a rare, inherited neurodegenerative disorder. HD causes neuronal degeneration in the cerebral cortex and basal ganglia, which play a key role in movement and behavior control. The cumulative damage to these areas results in the hallmark symptoms of HD: chorea (jerky movements), neuropsychiatric symptoms, loss of executive functioning and dementia. HD is caused by an autosomal dominant mutation in a gene called Huntingtin, which means any child of an affected person typically has a 50% chance of inheriting the disease. The Huntingtin gene encodes a protein that is also called "huntingtin." Expansion of a CAG triplet repeat within the Huntingtin gene results in a mutant form of the protein, which gradually damages cells in the brain. HD manifests as a triad of movement, cognitive and psychiatric symptoms which progress gradually in severity over many years, eventually causing severe physical and mental disability and potentially early death. The symptoms of HD usually become evident between the ages 35-44 years, but the onset can also begin from childhood to late life (>75 years).

Brain Derived Neurotropic Factor, or BDNF, is a secreted protein that helps support the survival, growth and differentiation of new neurons and synapses. BDNF is a member of the nerve growth factor family. It is induced by cortical neurons, and is necessary for survival of striatal neurons in the brain. Two master genes, Huntingtin, or Htt, and huntingtin-associated protein, or Hap1, govern BDNF axonal transport and secretion. Additionally, expression of the Bdnf gene is reduced in both Alzheimer's and Huntington's disease patients and HD patients are believed to be deficient in BDNF. The Bdnf gene may play a role in the regulation of stress response and in the biology of mood disorders. Cysteamine and its dimer cystamine have been shown in preclinical studies to increase levels of BDNF, and reduce associated tremor and abnormal movements in various animal models of Huntington's disease. Cysteamine's reported ability to inhibit caspase-3, inhibit transglutaminase 2 and reduce cellular and mitochondrial oxidative stress have also been proposed to be potentially beneficial in the treatment of Huntington's disease.

Centre Hospitalier Universitaire, or CHU, d'Angers, France, is currently conducting a Phase 2/3 clinical trial of RP103, our proprietary formulation of delayed-release cysteamine bitartrate, in which 96 patients were enrolled. This 36-month randomized trial is comprised of an 18-month blinded, placebo-controlled phase followed by an 18-month open-label phase in which all patients transition to RP103. The trial commenced in October 2010, with full enrollment achieved in June 2012. The primary endpoint of the trial is change from the baseline of the Total Motor Score of the Unified Huntington's Disease Rating Scale, or UHDRS. Blood levels of BDNF are being measured as a secondary endpoint and potential biomarker. Under the collaboration agreement with CHU d'Angers, we supply RP103 and placebo capsules for the clinical trial and open-label extension study in exchange for regulatory and commercial rights to the clinical trial results. Clinical expenses of the study are covered by a grant from the Programme Hospitalier de Recherche Clinique, which is funded by the French government. Interim results of this study following the first 18 months of treatment are anticipated in the first quarter of calendar 2014.

The treatment options for HD patients are very limited, with no drugs that address the underlying pathophysiology. Drugs that are available provide symptomatic relief of chorea and mood swings associated with HD. In preclinical studies, cysteamine has shown the potential to slow the progression of HD by increasing the levels and intracellular transport of BDNF in mice and non-human primates.

In 2008, we received FDA orphan drug designation for cysteamine formulations, including RP103, for the potential treatment of HD. We plan to apply for orphan drug designation in the EU pending availability of interim clinical data which we anticipate receiving in the first quarter of 2014.

RP103 for Non-alcoholic Fatty Liver Disease in Children

Non-alcoholic fatty liver disease, or NAFLD, is the hepatic component of metabolic syndrome and is associated with deposition of triglycerides in the hepatocytes in individuals who do not consume alcohol in amounts generally considered to be harmful to the liver. NAFLD is commonly associated with elements of metabolic syndrome, such as obesity, diabetes mellitus and hypertriglyceridemia. Additional factors include family history of diabetes and high blood lipids in people who are not obese. NAFLD refers to a spectrum of conditions ranging from simple fat accumulation in the liver to steatohepatitis, cirrhosis and hepatocellular carcinoma.

- Non-alcoholic fatty liver, or NAFL – A benign condition with simple fat accumulation within liver cells (hepatic steatosis).
- Non-alcoholic steatohepatitis, or NASH – An aggressive form of NAFLD characterized by hepatic steatosis and inflammation with hepatocyte injury (ballooning) with or without fibrosis.
- Cirrhosis – 15% to 25% of patients with NASH progress to cirrhosis with consequential complications over 10 to 20 years. Cirrhosis is characterized by the replacement of healthy liver tissue with fibrosis and scar tissue, leading to loss of liver function. NASH cirrhosis is a risk factor for development of hepatocellular carcinoma, or HCC.

NAFLD prevalence is increasing along with the rise of obesity. Advanced NAFLD is now among the most common reasons why patients are referred for liver transplantation.

According to the World Gastroenterology Organization Global Guidelines, the prevalence of NAFLD in children is about 15% in the U.S. and western countries. NAFLD is underdiagnosed in children due to lack of recognition, screening or appreciation of associated complications by healthcare providers. Children may not be recognized as obese during office visits and age-appropriate norms for body mass index may go unacknowledged. Liver disease is screened by measuring serum alanine aminotransferase, or ALT, and aspartate aminotransferase, or AST, starting at 10 years old in obese children and those with a body mass index of 85th to 94th percentile with other risk factors.

Currently there are no drug treatment options for NAFLD. Disease management is through counseling with lifestyle changes in diet, exercise and weight reduction.

We believe that cysteamine may be useful for the treatment of NAFLD. Cysteamine is itself a potent antioxidant which may reduce oxidative damage resulting from excessive accumulation of fats in liver cells. Cysteamine is also known to increase levels of a key cellular antioxidant, glutathione, or GSH, with the potential to further reduce oxidative cellular damage. Glutathione is composed of the amino acids cysteine, glutamate and glycine. The availability of cysteine, which exists primarily as cystine, is the major rate-limiting factor in GSH production. Cysteamine may bind to extracellular cystine and enhance its cellular uptake, thereby increasing the cellular thiol pool and making more cysteine available for glutathione synthesis. Finally, cysteamine is known to inhibit transglutaminase, an enzyme responsible for generation of fibrotic tissue, an important aspect of late-stage NAFLD.

Phase 2a clinical trial results with a prototype of RP103 for the potential treatment of NASH and NAFLD showed that patients receiving enteric-coated cysteamine exhibited a marked decline in serum transaminase levels during the treatment period of 26 weeks. Seven of 11 juvenile childhood NAFLD patients entering the study with elevated ALT and AST achieved more than 50% reduction in ALT and 6 of 11 reduced levels to normal range. AST levels were also improved, with patients averaging 41% reduction by the end of the 6-month treatment phase. This reduction in serum liver enzymes was largely sustained during the 6-month post-treatment monitoring phase and other important liver function markers showed positive trends, suggesting improvements in hepatic histopathology. Levels of cytokeratin 18, or CK-18, a potential serum marker of disease activity in NASH and NAFLD, decreased by an average

of 45%. Adiponectin levels showed a positive increase by an average of 35% during the treatment period. Reduced adiponectin levels are thought to be a marker of the pathogenesis and progression of NASH and NAFLD.

The Phase 2a trial results were consistent with ALT and AST reductions seen in patients who achieve a 10% weight loss, although body mass index did not change significantly during both the treatment and post-treatment phases in the Phase 2a clinical trial. In this Phase 2a clinical trial, the prototype of RP103 demonstrated a favorable safety profile, with mean gastrointestinal symptom scores of 1.1 (the maximum score of 14 indicates the most severe gastrointestinal symptoms) at baseline and 0.7 after 6 months of treatment.

In June 2012, we announced the dosing of the first patient in a Phase 2b clinical trial – Cysteamine Bitartrate Delayed-Release for the Treatment of Non-alcoholic Fatty Liver Disease in Children, or CyNCh, which is evaluating the safety and efficacy of RP103 as a potential treatment of NAFLD in children. The clinical trial is being conducted under a Cooperative Research and Development Agreement, or CRADA, with the National Institute of Diabetes and Digestive and Kidney Diseases, or NIDDK, part of the National Institutes of Health, or NIH. The trial is expected to enroll a total of 160 pediatric participants at ten U.S. centers in the NIDDK-sponsored NAFLD Clinical Research Network.

Raptor and NIDDK share the costs of conducting the CyNCh clinical trial. The primary objective of this randomized, multicenter, double-blind, placebo-controlled Phase 2b clinical trial is to evaluate whether 52 weeks of RP103 treatment reverses liver tissue damage caused by NAFLD as measured by changes in NAFLD Activity Score, or NAS, a histological rating scale of disease activity (based on scoring lobular inflammation, hepatocyte ballooning and steatosis from a liver biopsy), in conjunction with no worsening of liver tissue fibrosis. Secondary endpoints include blood markers for liver health including ALT and AST as well as safety and tolerability. We anticipate reaching full enrollment in the first half of 2014 and interim data for this study is anticipated in the first half of 2015.

Other Clinical-Stage Product Candidate

Convivia™ for ALDH2 Deficiency

We are developing Convivia, our proprietary oral formulation of 4-methylpyrazole, or 4-MP, for the potential treatment of acetaldehyde toxicity resulting from ALDH2 deficiency.

We own the intellectual property portfolio pertaining to Convivia, including method of use and formulation patents. In June 2010, we granted an exclusive license to commercialize Convivia in Taiwan to Uni Pharma Co., Ltd. Under this agreement, Uni Pharma is responsible for clinical development, registration and commercialization of Convivia in Taiwan. We continue to seek partners in other Asian countries to license Convivia.

Preclinical Product Candidates

Our preclinical programs include our cysteamine dioxygenase, or ADO, program, to improve treatment of diseases for which cysteamine is therapeutic and our HepTide™ program to treat hepatocellular carcinoma and other cancers susceptible to induced lysosomal storage.

Other Development Areas

Securing Additional and Complementary Technology Licenses from Others

We plan to establish additional research collaborations with prominent universities and research labs and to secure licenses from these universities and labs for technology resulting from the collaborations. No assurances can be made regarding our ability to establish such collaborations over the next 12 months, or at all. We may obtain complementary products through joint ventures or through merger and/or acquisitions with other biotechnology companies.

Future Activities

We expect that our near-term efforts will be focused on:

- Increasing sales of PROCYSBI and providing comprehensive reimbursement, education and persistency support to commercial patients in the U.S.;
- Negotiating reimbursement country by country and launching PROCYSBI within the European Union in 2014;
- Filing a New Drug Submission, or NDS, for PROCYSBI with Health Canada in the first half of 2014;
- Conducting clinical trials that evaluate PROCYSBI in cystinosis patients that are cysteamine-naïve, as well as other supporting trials in underdeveloped markets;
- Developing select global markets with significant numbers of known cystinosis patients;
- Screening for undiagnosed and unidentified late-onset nephropathic cystinosis;
- Supporting our clinical trials of RP103 for the potential treatment of HD in adults and NAFLD in children;
- Supporting our novel preclinical programs and identifying promising in-licensing candidates; and
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Continuing the development of our RP103 clinical pipeline in other indications including tissue fibrotic disease, Rett Syndrome and mitochondrial disorders.

Results of Operations

Quarter ended September 30, 2013 and fiscal quarter ended August 31, 2012

Revenue

On April 30, 2013, the FDA granted marketing approval in the U.S. for the sale of our first product, PROCYSBI (cysteamine bitartrate) delayed-release capsules, for the management of nephropathic cystinosis in adults and children ages six and older. On September 6, 2013, the European equivalent, PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received marketing authorization from the European Commission as an orphan medicinal product for the treatment of proven nephropathic cystinosis for marketing in the EU. For the quarter ended September 30, 2013, we recognized $6.6 million in PROCYSBI net product sales. The first U.S. sales of PROCYSBI commenced in June 2013 and the launch of PROCYSBI in the EU is anticipated to commence in the first half of 2014; there were no product sales for the fiscal quarter ended August 31, 2012.

Cost of Sales

Prior to FDA approval on April 30, 2013, our commercial manufacturing costs have been recorded as research and development expenses. As a result, our cost of sales for the next several quarters will reflect a lower average per unit cost of goods than will be recorded in the future. Cost of sales for the quarter ended September 30, 2013 were $0.4 million and primarily included capitalized commercial product sold, amortization of licensing milestone payments, royalty fees payable to UCSD on our net product sales and other indirect costs such as distribution, labeling, shipping and supplies. We began capitalizing commercial inventory costs upon FDA approval of PROCYSBI on April 30, 2013.

Research and Development

Research and development expenses include medical, clinical, regulatory and scientists' salaries and benefits; expenses associated with the manufacturing and testing of PROCYSBI inventory for our commercial launch in the U.S. which were expensed prior to drug approval; preclinical studies; clinical trials; regulatory and clinical consultants; research supplies and materials; amortization of intangible assets and allocated human resources and facilities expenses.

Research and development expenses increased approximately 6% to $6.8 million for the quarter ended September 30, 2013 from $6.4 million in the comparable fiscal quarter ended August 31, 2012. This increase was primarily due to higher external costs for clinical studies and lab services of $0.7 million and higher staffing costs of $0.5 million, offset by our prior year's write-off of an intangible asset of $0.9 million.

Major program expenses recorded as research and development expenses:

(In millions)	Three Months Ended September 30, 2013	August 31, 2012
PROCYSBI: cystinosis (commercial)	$3.9	$ 2.8
RP103: HD (clinical)	(0.1)	0.2
RP103: NAFLD in children (clinical)	0.4	0.8
Preclinical programs	0.3	0.2

Minor or inactive programs	0.2	1.0
Research and development personnel and other costs not allocated to programs	2.1	1.4
Total research and development expenses	$6.8	$ 6.4

Selling, General and Administrative Expenses

Selling, general and administrative expenses primarily includes finance, executive and commercial operations salaries and benefits; commercial expenses, such as reimbursement and marketing studies; commercial launch expenses for PROCYSBI, including the set-up of RaptorCares.com to provide patient support and establish a customer relationship management system for our U.S. PROCYSBI sales team; intellectual property, legal and audit fees and other administrative and facilities costs.

Selling, general and administrative expenses increased approximately 43% to $8.3 million for the quarter ended September 30, 2013 from $5.8 million in the comparable fiscal quarter ended August 31, 2012. This increase was primarily due to staffing increases of $1.8 million, non-cash stock compensation expense of $0.5 million and in ramping up sales and marketing costs for the commercialization and U.S. launch of PROCYSBI of $0.3 million.

Interest Income

Interest income for the quarter ended September 30, 2013 and fiscal quarter ended August 31, 2012 was $3,000 and $78,000, respectively.

Interest Expense

Interest expense for the quarter ended September 30, 2013 and the fiscal quarter ended August 31, 2012 was $2.3 million and $1,000, respectively. The increase in interest expense was due primarily to the $50.0 million loan agreement that we entered into with HealthCare Royalty Partners II, L.P., or HC Royalty, in December 2012, of which net proceeds of $23.4 million and $23.7 million were received in December 2012 and May 2013, respectively. During the quarter ended September 30, 2013, we recognized interest expense of $2.3 million incurred under the HC Royalty loan agreement.

Foreign Currency Transaction Gains

Foreign currency transaction gains for the quarter ended September 30, 2013 and the fiscal quarter ended August 31, 2012 were $15,000 and $23,000, respectively.

Unrealized Gain/(Loss) on Short-Term Investments

Unrealized gain/(loss) on short-term investments represents the change in net asset value of our previously held short-term bond fund. There were no unrealized gains or losses on short-term investments for the quarter ended September 30, 2013 when compared to an unrealized loss of $143,000 for the fiscal quarter ended August 31, 2012.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of $6.0 million for the quarter ended September 30, 2013 compared to a gain of $1.9 million for the fiscal quarter ended August 31, 2012. The loss for the quarter ended September 30, 2013 was due primarily to an increase in our stock price of $5.57 per share offset by a decrease in the number of remaining warrants outstanding due to warrant exercises. The gain in the comparable fiscal quarter ended August 31, 2012 was due primarily to a decrease in our stock price of $0.40 per share. The gains/(losses) on the revaluation of stock warrants are non-cash.

Nine months ended September 30, 2013 and August 31, 2012

Revenue

For the nine months ended September 30, 2013, we recognized $6.6 million in PROCYSBI net product revenues. The first U.S. sales of PROCYSBI commenced in June 2013 and launch of PROCYSBI in the EU is anticipated to commence in the first half of 2014; there were no product sales for the nine-month period ended August 31, 2012.

Cost of Sales

Cost of sales for the nine months ended September 30, 2013 was $0.9 million and primarily included capitalized commercial product sold, reserves for commercial product scrapped, amortization of licensing milestone payments, royalty fees payable to UCSD on our net product sales and other indirect costs such as distribution, labeling, shipping and supplies. We began capitalizing commercial inventory costs upon FDA approval of PROCYSBI on April 30, 2013. Included in cost of sales is $0.4 million of commercial inventory that was written off during the second quarter ended June 30, 2013 as a result of an unanticipated minor change in the finished product presentation. This write-off is not expected to be repeated in the future.

Research and Development

Research and development expenses increased approximately 30% to $21.4 million for the nine-month period ended September 30, 2013 from $16.4 million for the comparable nine-month period ended August 31, 2012. This increase was primarily due to higher external costs for clinical studies and lab services of $4.3 million and higher staffing expenses of $1.6 million, offset by a prior year write-off of an intangible asset of $0.9 million.

Research and development expenses for the remainder of 2013 are expected to decrease relative to the comparable prior 12-month period due to the prior expensing of commercial manufacturing costs of PROCYSBI as research and development expenses in anticipation of commercial launch. Subsequent to FDA approval on April 30, 2013, we started capitalizing these costs as commercial inventory. However, research and development expenses associated with our development efforts of RP103 for our HD and NASH programs will continue to increase.

Major program expenses recorded as research and development expenses:

(In millions)	Nine Months Ended September 30, 2013	Nine Months Ended August 31, 2012
PROCYSBI: cystinosis (commercial)	$11.3	$ 8.0
RP103: HD (clinical)	0.3	0.8
RP103: NAFLD in children (clinical)	1.4	1.5
Preclinical programs	0.6	0.4
Minor or inactive programs	0.6	1.2
Research and development personnel and other costs not allocated to programs	7.2	4.5
Total research and development expenses	$21.4	$ 16.4

Any of our programs could be partnered for further development and/or could be accelerated, slowed or terminated due to scientific results, challenges in obtaining funding or changes in operational plans. In addition, the timing and costs of development of our programs beyond the next 12 months are highly uncertain and difficult to estimate. See risks and other factors described under the section captioned "Risk Factors" in Part II, Item 1A of this Quarterly Report on Form 10-Q.

Selling, General and Administrative Expenses

Selling, general and administrative expenses increased approximately 106% to $25.6 million for the nine-month period ended September 30, 2013 from $12.4 million for the comparable nine-month period ended August 31, 2012. This increase was primarily due to the ramping up of sales and marketing expenses for the commercialization and U.S. launch of PROCYSBI of $6.6 million, staffing increases of $5.1 million and non-cash stock compensation expense of $1.5 million.

We anticipate selling, general and administrative expenses for the remainder of 2013 to increase relative to the comparable prior 12-month period due to increased commercialization activities of PROCYSBI in both the U.S. and EU and increases in headcount across various functions to support these activities.

Interest Income

Interest income for the nine-month periods ended September 30, 2013 and August 31, 2012 was $0.2 million and $0.3 million, respectively.

Interest Expense

Interest expense for the nine-month periods ended September 30, 2013 and August 31, 2012 was $4.1 million and $2,000, respectively. The increase in interest expense was primarily due to the $50.0 million loan agreement that we entered into with HC Royalty in December 2012, of which net proceeds of $23.4 million and $23.7 million were received in December 2012 and May 2013, respectively. During the nine-month period ended September 30, 2013, we recognized interest expense of $4.1 million incurred under the HC Royalty loan agreement.

Foreign Currency Transaction Gain/(Loss)

Foreign currency transaction gain/(loss) for the nine-month periods ended September 30, 2013 and August 31, 2012 were a loss of $20,000 and a gain of $86,000, respectively.

Unrealized Gain/(Loss) on Short-Term Investments

Unrealized gain/(loss) on short-term investments represents the change in net asset value of our previously held short-term bond fund. We did not have any short term-investments during the nine-month period ended September 30, 2013 and therefore we had no unrealized gain/(loss) on short-term investments, compared to an unrealized gain of $33,000 for the nine-month period ended August 31, 2012.

Adjustment to the Fair Value of Common Stock Warrants

The loss for the nine-month period ended September 30, 2013 of $12.0 million was primarily attributable to an increase in our stock price of $9.07 per share since December 31, 2012 offset by a decrease in the number of remaining warrants outstanding due to warrant exercises. The comparable gain for the nine-month period ended

August 31, 2012 of $1.0 million was primarily attributable to a decrease in the number of remaining warrants outstanding due to warrant exercises and a decrease of $0.55 per share in our common stock price from November 30, 2011 to August 31, 2012. The losses on the revaluation of stock warrants are non-cash.

Critical Accounting Policies and Estimates

Our condensed consolidated financial statements and accompanying notes are prepared in accordance with U.S. generally accepted accounting principles, or GAAP. Preparing financial statements requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenue and expenses. These estimates and assumptions are affected by management's application of accounting policies. We believe that understanding the basis and nature of the estimates and assumptions involved with the following aspects of our condensed consolidated financial statements is critical to an understanding of our consolidated financial position.

For a complete discussion of our critical accounting policies, refer to "Application of Critical Accounting Policies" in "Item 7 – Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Transition Report on Form 10-KT for the four-month period ended December 31, 2012 filed on March 14, 2013, as amended by Form 10-KT/A filed with the SEC on June 19, 2013. Additional critical accounting policies described below are related to our receipt of FDA approval on April 30, 2013 to market our drug PROCYSBI.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires our management to make certain estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the dates of our condensed consolidated financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

Revenue Recognition

We recognize revenue in accordance with FASB ASC 605, Revenue Recognition, when the following criteria have been met: persuasive evidence of an arrangement exists; delivery has occurred and risk of loss has passed; the seller's price to the buyer is fixed or determinable and collectability is reasonably assured. We determine that persuasive evidence of an arrangement exists based on written contracts that defined the terms of the arrangements. Under the contract terms, we determine when title to products and associated risk of loss has passed onto the customer. We assess whether the fee is fixed or determinable based on the payment terms associated with the transaction and whether the sales price is subject to refund or adjustment. We assess collectability based primarily on the customer's payment history and creditworthiness. Without sufficient credit history, we determine a customary collectability percentage.

PROCYSBI is currently only available for distribution from our U.S. specialty pharmacy partner which ships directly to patients. PROCYSBI is not available in retail pharmacies. Prior authorization of coverage level by patients' private insurance plans, our patient assistance program or government payors is a prerequisite to the shipment of PROCYSBI to patients. Revenue is recognized once the product has been shipped by the specialty pharmacy and receipt confirmed by patients. Billings to our distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenues by us until such deliveries to patients occur.

We record revenue net of expected discounts, distributor fees, returns and rebates, including those paid to Medicare and Medicaid in the U.S. Allowances are recorded as a reduction of revenue at the time product sales are recognized. Allowances for government rebates and discounts are established based on the actual payor information, which is known at the time of shipment, and the government-mandated discounts applicable to government-funded programs. The allowances are adjusted to reflect known changes in the factors that may impact such allowances in the quarter the changes are known.

Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price with cost determined on a first-in, first-out basis. Inventories are reviewed periodically to identify slow-moving or obsolete inventory based on sales activity, both projected and historical, as well as product shelf-life. In evaluating the recoverability of inventories produced in preparation for product launches, we consider the probability that revenue will be obtained from the future sale of the related inventory. Prior to the approval of PROCYSBI by the FDA on April 30, 2013, we recorded manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to approval, we began capitalizing these costs as inventory is manufactured. We did not capitalize inventory at December 31, 2012. We began recognizing costs of sales in the second quarter of 2013. Cost of sales includes the cost of inventory sold and reserved, amortization of licensing approval milestone payments, royalty fees due to UCSD on our net product revenues and other indirect costs.

Accounts Receivable

Trade accounts receivable are recorded net of product sales allowances for prompt-payment discounts, chargebacks and doubtful accounts. Estimates for chargebacks and prompt-payment discounts are based on contractual terms and our expectations regarding the utilization rates.

Fair Value of Financial Instruments

The carrying amounts of certain of our financial instruments including cash and cash equivalents, restricted cash, prepaid expenses, accounts payable, accrued liabilities and capital lease liability approximate fair value due either to length of maturity or interest rates that approximate prevailing market rates unless otherwise disclosed in our condensed consolidated financial statements. The warrant liability is carried at fair value which is determined using the Black-Scholes option valuation model at the end of each reporting period.

Accrued Liabilities

Accrued liabilities include estimates for certain expenses which we have not yet been invoiced and that requires management's judgment in determining appropriate expenses to accrue. For example, because of the nature of how clinical trials are invoiced by clinical sites, especially outside of the U.S. where there is a significant time lag between the services provided by the clinical site and the time the clinical site bills us for their services, we must estimate such clinical site expenses on a monthly basis as clinical trial expenses. Although we believe our accrued liabilities reflect the best information available to us, our actual expenses could differ from our estimates.

Note Payable and Debt Issuance Costs

Note payable consists of our loan agreement with HC Royalty as lender under which we agreed to borrow $50.0 million in two $25.0 million tranches, or the HC Royalty Loan. We drew down the first tranche in the amount of $25.0 million in December 2012 and the second tranche in May 2013. The loan bears interest at an annual fixed rate of 10.75% of outstanding principal and quarterly interest payments are included in interest expense in our Condensed Consolidated Statements of Comprehensive Loss for the quarter ended September 30, 2013. Principal payments, when made, reduce our note payable balance. There is a synthetic royalty component based on net product revenues, including PROCYSBI, in a calendar year, and such royalty is payable quarterly. With respect to the first $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.25% of the first $25.0 million of PROCYSBI and future approved product net revenues for such calendar year, 3.0% of PROCYSBI and future approved product net revenues for such calendar year in excess of $25.0 million and not in excess of $50.0 million, and 1.0% of PROCYSBI and future approved product net revenues for such calendar year in excess of $50.0 million, payable quarterly. With respect to the second $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.0% of the first $25.0 million of PROCYSBI and future approved product net revenues for such calendar year, 3.0% of PROCYSBI and future approved product net revenues for such calendar year in excess of $25.0 million and not in excess of $50.0 million, and 1.0% of PROCYSBI and future approved product net revenues for such calendar year in excess of $50.0 million, payable quarterly.

As of September 30, 2013, approximately $0.8 million of royalty fees were accrued on net product revenues. We received marketing approval of PROCYSBI from the FDA on April 30, 2013 and commenced shipment of PROCYSBI in the U.S. during June 2013, and as a result, royalties became payable to HC Royalty based upon net revenues of PROCYSBI. The royalties are classified as interest expense in our Condensed Consolidated Statements

of Comprehensive Loss and accrued interest expense on our Condensed Consolidated Balance Sheets. We have not yet had sufficient revenue experience to estimate future revenues as of September 30, 2013. The loan and our obligation to make any payments shall terminate immediately when our payments received by HC Royalty equals $97.5 million, except that if, by December 20, 2014, net revenues for the immediately preceding four fiscal quarters exceed $100.0 million, then the loan and our obligation to make any payments shall terminate immediately when all payments received by HC Royalty from us equals $90.0 million. Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan using the effective interest method. The amortization of debt issuance costs is included in interest expense in our Condensed Consolidated Statements of Comprehensive Loss.

Goodwill and Intangible Assets and Related Impairment of Long-Lived Assets

We periodically evaluate our long-lived assets for indicators of possible impairment by comparison of the carrying amounts to future net undiscounted cash flows expected to be generated by such assets when events or changes in circumstances indicate the carrying amount of an asset may not be recoverable. Should an impairment exist, the impairment loss would be measured based on the excess carrying value of the asset over the asset's fair value or discounted estimates of future cash flows.

Goodwill represents the excess of the value of the purchase consideration over the identifiable assets acquired in the merger of our subsidiary with and into Raptor Pharmaceuticals Corp. in September 2009, or the 2009 Merger. Goodwill is reviewed annually, or when an indication of impairment exists. When an impairment analysis is performed, if deemed necessary, a write-down in valuation is recorded.

Intangible assets include the intellectual property and other rights relating to DR Cysteamine (currently developed as PROCYSBI and RP103) and to an out-license acquired in the 2009 Merger. Also included in intangible assets are milestone payments to UCSD that were payable upon approval of PROCYSBI by the FDA in the U.S. and by the EC in the EU. During the second quarter of 2013, we paid $750,000 upon FDA approval and during the third quarter of 2013, we accrued $500,000, both of which were capitalized as licensing milestones and are being amortized through 2027, the life of the licensed patents. All of the intangible assets related to PROCYSBI/RP103 are amortized using the straight-line method over the estimated useful life of 20 years or patent life, whichever is shorter. Intangible assets related to the out-license are amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents. During the quarter ended September 30, 2013, we did not identify any impairment losses.

We amortized $47,000 and $37,000 of intangible assets during the three months ended September 30, 2013 and August 31, 2012, respectively, and $134,000 and $109,000 during the nine months ended September 30, 2013 and August 31, 2012, respectively. These amounts relate to the amortization of the intangible assets discussed in the preceding paragraph.

Common Stock Warrant Liabilities

The warrants issued by us in our 2010 private placement contain a cash-out provision which may be triggered upon request by the warrant holders if we are acquired or upon the occurrence of certain other fundamental transactions involving us. This provision requires these warrants to be classified as liabilities and to be marked to market at each period-end commencing on August 31, 2010. The warrants issued by us in our December 2009 equity financing contain a conditional obligation that may require us to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under the Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 480, Distinguishing Liabilities from Equity, or ASC 480, a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, we have classified the warrants as liabilities and will mark them to fair value at each period-end. The common stock warrants are re-measured at the end of every reporting period with the change in value reported in our Condensed Consolidated Statements of Comprehensive Loss. Warrants which are recorded as liabilities that are exercised are re-measured and marked to market the day prior to exercise. Upon exercise of such warrants, the fair value of those warrants is reclassified to equity.

Income Taxes

Income taxes are recorded under the liability method, under which deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to affect taxable income. Valuation allowances are established when necessary to reduce deferred tax assets to the amount expected to be realized.

Our effective income tax rate is 0% for the three and nine months ended September 30, 2013 and we have determined that our effective tax rate for our fiscal year ended August 31, 2012 and the short tax year from September 1, 2012 to December 31, 2012 is 0%. Based on the weight of available evidence, including cumulative losses since inception and expected future losses, we have determined that it is more likely than not that the deferred tax asset amount will not be realized and therefore a full valuation allowance has been provided on our net deferred tax assets.

Utilization of our net operating loss, or NOL, carryovers may be subject to substantial annual limitation due to the ownership change rules under the Internal Revenue Code and similar state income tax law provisions including those related to the suspension and limitation of NOL carryovers for certain tax years. Such an annual limitation could result in the expiration of our NOL carryovers before utilization.

We account for income taxes under FASB ASC No. 740-10, Accounting for Uncertainty in Income Taxes. Under this approach, deferred tax assets and liabilities are recognized based on anticipated future tax consequences, using currently enacted tax laws attributed to temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts calculated for income tax purposes.

We recognize interest and/or penalties related to income tax matters as a component of income tax expense. As of September 30, 2013, there were no accrued interest or penalties related to uncertain tax positions. As of September 30, 2013, there were no unrecognized tax benefits for which the liability for such taxes were recognized as deferred liabilities.

We file U.S. Federal, California and other state income tax returns. In addition, we file income tax returns in France and the Netherlands. We are currently not subject to any income tax examinations. Due to our NOLs, generally all tax years remain open.

Stock-Based Compensation

Effective September 1, 2006, our stock-based compensation is accounted for in accordance with ASC Topic 718, Accounting for Compensation Arrangements, or ASC Topic 718 (previously listed as Statement of Financial Accounting Standards No. 123 (revised 2004), Share-Based Payment, or SFAS 123(R), and related interpretations. Under the fair value recognition provisions of this statement, share-based compensation cost is measured at the grant date based on the value of the award and is recognized as expense over the vesting period. Determining the fair value of share-based awards at the grant date requires judgment, including estimating future stock price volatility and employee stock option exercise behavior. If actual results differ significantly from these estimates, stock-based compensation expense and results of operations could be materially impacted.

For the quarter ended September 30, 2013, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 1.51%; 5 year expected life; 67.18% volatility; 2.5% turnover rate; and 0% dividend rate.

We based our Black-Scholes inputs on the following factors:

- the risk-free interest rate was based upon our review of current constant maturity treasury bill rates for five years;
- the expected life of five years was based upon our assessment of the ten-year term of the stock options issued, the fact that we have commercial sales and option holders have started to and will likely continue to exercise their stock options that are fully vested;
- the volatility was based on the actual annualized volatility of our common stock price as quoted on NASDAQ since the closing of our 2009 Merger on September 30, 2009;
- the turnover rate was based on our assessment of our historical employee turnover; and
- the dividend rate was based on our current decision to not pay dividends on our stock during our current stage.

If factors change and different assumptions are employed in the application of ASC Topic 718, the compensation expense recorded in future periods may differ significantly from what was recorded in the current period. See Note 7 of our condensed consolidated financial statements for a further discussion of our accounting for stock-based compensation.

We recognize as consulting expense the fair value of options granted to persons who are neither employees nor directors. Stock options issued to consultants are accounted for in accordance with the provisions of the FASB ASC

Topic 505-50, Equity-Based Payments to Non-Employees (previously listed as Emerging Issues Task Force, or EITF, Consensus No. 96-18, Accounting for Equity Instruments That Are Issued to Other Than Employees for Acquiring, or in Conjunction with Selling Goods or Services). The fair value of expensed options is based on the Black-Scholes option-pricing model assuming the same factors as stock-based compensation expense discussed above.

Recent Accounting Pronouncements

On February 5, 2013, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2013-02, Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income. This amendment requires an entity to present either parenthetically on the face of the financial statements or in the notes significant amounts reclassified from each component of accumulated other comprehensive income and the line item(s) affected by the reclassification. An entity would not need to show the statement of operations line item affected for certain components that are not required to be reclassified in their entirety to net income, such as amounts amortized into net periodic pension cost. For public companies, this amendment is effective for annual periods beginning after December 15, 2012, and for interim periods within those annual periods. Adoption of ASU No. 2013-02 did not impact our financial position or results of operations for the quarter and nine months ended September 30, 2013 and is not anticipated to have a significant effect on our financial reporting.

In July 2013, the FASB issued ASU 2013-11, Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists. ASU 2013-11 was issued to eliminate the diversity in practice in presentation of unrecognized tax benefits, and amends Accounting Standards Codification ("ASC") 740, "Income Taxes," to provide clarification of the financial statement presentation of an unrecognized tax benefit when a net operating loss carryforward, a similar tax loss, or a tax credit carryforward exists. According to the new guidance, unrecognized tax benefits will be netted against all available same-jurisdiction loss or other tax carryforward that would be utilized, rather than only being netted against carryforwards that are created by the unrecognized tax benefits. The revised guidance is effective for interim and annual periods beginning after December 15, 2013, with early adoption permitted. As this guidance relates to presentation only, the adoption of this guidance will not impact our financial position or results of operations. We do not expect the adoption to have a material impact on our financial statements.

Liquidity and Capital Resources

Capital Resource Requirements

As of September 30, 2013, we had $88.3 million in cash and cash equivalents, $24.6 million in current liabilities (of which $10.3 million represented the non-cash common stock warrant liability) and $74.7 million of net working capital.

Based upon our projected PROCYSBI net sales and planned operations, we believe that our cash and cash equivalents as of September 30, 2013 of $88.3 million will be sufficient to meet our projected operational requirements and obligations through at least the end of 2014.

The $50.0 million HC Royalty loan agreement executed on December 20, 2012, matures on December 31, 2019, and bears interest at an annual fixed rate of 10.75% and a variable royalty rate, tiered down, based on a percentage of PROCYSBI and future approved product net revenues. The loan is interest-only for the first two years. The proceeds from the loans will be used primarily to help fund the commercialization of PROCYSBI, advance our development programs and for general corporate purposes. See Note 4 – Notes Payable and Debt Issuance Costs for further information.

On April 30, 2012, we entered into a Sales Agreement with Cowen and Company, or Cowen, to sell shares of our common stock, with aggregate gross sales proceeds of up to $40.0 million, from time to time, through an "at the market" equity offering program under which Cowen acts as sales agent. In July 2013, we amended and restated the agreement which increased our ability to sell shares of our common stock up to an aggregate of $100.0 million. We pay a 3.0% commission to Cowen on any sales pursuant to the Amended and Restated Sales Agreement. Through September 30, 2013, we sold 7,599,474 shares at a weighted-average sales price of $7.08 per share for net proceeds of approximately $52.1 million. As of October 15, 2013, an aggregate of approximately $46.2 million remained available for future sales of our common stock under the Amended and Restated Sales Agreement.

As of September 30, 2013, Series A warrants to purchase up to 75,000 shares of our common stock were outstanding, all of which warrants were issued pursuant to a definitive securities purchase agreement, dated as of December 17, 2009. The outstanding Series A warrants are exercisable until December 22, 2014, at a per share exercise price of $2.45.

As of September 30, 2013, 768,359 shares (including the shares issuable upon exercise of the placement agent warrant described below) of our common stock warrants were outstanding, all of which warrants were issued pursuant to private placement purchase agreements, dated as of August 9, 2010. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share. The warrant that we issued to the placement agent for this private placement is exercisable for 97,952 shares of our common stock at an exercise price of $3.075 per share.

Future Funding Requirements

We may need to raise additional capital either through the sale of equity or debt securities (including convertible debt securities) to fund our operations and to, among other activities, commercialize PROCYSBI and to develop RP103 for various indications. Our future capital requirements may be substantial, and will depend on many factors, including:

- the success of our U.S. commercial launch of PROCYSBI including patient and revenue uptake;
- the cost of establishing the sales and marketing capabilities in the EU necessary to launch PROCYSBI in the EU in the first half of 2014;
- our ability to negotiate reimbursement and pricing of PROCYSBI in the EU;

- the successful launch of PROCYSBI in the EU;
- the cost of our manufacturing-related activities in support of PROCYSBI and RP103;
- the cost of activities related to the regulatory submission of PROCYSBI in Canada;
- the cost of additional clinical trials in order to obtain regulatory approvals for PROCYSBI in non-U.S. and non-EU countries;
- the timing and cost of our ongoing clinical programs for RP103, including: evaluating PROCYSBI in treatment-naïve cystinosis patients, and other supportive studies; evaluating RP103 as a potential treatment for Huntington's disease; and evaluating RP103 as a potential treatment for NAFLD;
- the cost of regulatory submissions, as well as the preparation, initiation and execution of clinical trials in potential new clinical indication using RP103;
- the cost of evaluating and potentially acquiring or in-licensing new drug compound(s) for potential clinical development;
- the cost of business development activities to identify, test and potentially license or acquire new therapeutic drug candidates; and
- the cost of filing, prosecuting and enforcing patent claims.

There can be no assurance that we will be successful in raising sufficient funds when needed. Additional financing may not be available in amounts or on terms satisfactory to us, or at all.

Commitments and Contingencies

We maintain several contracts with suppliers, contract manufacturers, research organizations, clinical organizations, drug labelers and distributors, and clinical sites, primarily to assist with clinical research and clinical and commercial manufacturing of PROCYSBI and clinical manufacturing of drug product for our HD and our NAFLD clinical collaborations. With the exception of the items listed below and updates as noted under Note 4 – Notes Payable and Debt Issuance Costs, our contractual obligations have not changed significantly during the quarter ended September 30, 2013 compared to those discussed in our Transition Report on Form 10-KT for the four month period ended December 31, 2012, filed with the SEC on March 14, 2013, as amended by Form 10-KT/A filed with the SEC on June 19, 2013.

On April 25, 2013, we executed a seven-year lease for a facility in Novato, California which we moved into at the end of June 2013 to accommodate personnel growth. We are obligated to make lease payments of $19,460 per month under this new lease from June 2013 through May 2014. On June 10, 2013, we amended the lease to add space to accommodate our research laboratory. We are obligated to make additional lease payments of $1,870 per month for the period June 2013 through May 2014 under this amendment. We plan to move to an adjacent facility when it becomes available in 2014. Rental expense for the larger adjacent facility will be higher than the rental expense for our current facility.

Off-Balance Sheet Arrangements

We do not have any outstanding derivative financial instruments, off-balance sheet guarantees, interest rate swap transactions or foreign currency contracts. We do not engage in trading activities involving non-exchange traded contracts.

Going Concern

Burr Pilger Mayer, Inc., Raptor Pharmaceutical Corp.'s independent registered public accounting firm, included a "going-concern" audit opinion on the consolidated financial statements for the four month period ended December 31, 2012 and for the period September 8, 2005 (inception) to December 31, 2012. The audit opinion reports substantial doubt about our ability to continue as a going concern due to significant operating losses since inception. We will need to raise additional capital and/or generate significant revenue at profitable levels to continue to operate as a going concern.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates and impacts our marketable securities. We do not have any derivative financial instruments.

Our market risks during the quarter ended September 30, 2013 have not materially changed from those discussed in our Transition Report on Form 10-KT for the four month period ended December 31, 2012, filed with the SEC on March 14, 2013, as amended by Form 10-KT/A filed with the SEC on June 19, 2013.

ITEM 4. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures. Based on that evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date are effective, at the reasonable assurance level, in ensuring that information required to be disclosed by us in the reports that we file or submit under the Securities Exchange Act of 1934, as amended, or the Exchange Act, is recorded, processed, summarized and reported within the time periods specified in the 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 us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can only provide reasonable assurance of achieving the desired control objectives, and in reaching a reasonable level of assurance, management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Changes in Internal Control over Financial Reporting

During the period covered by this Quarterly Report on Form 10-Q, there was no change that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II - OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

We are not currently subject to any material legal proceedings.

ITEM 1A. RISK FACTORS.

An investment in our common stock involves a high degree of risk. Before investing in our common stock, you should consider carefully the specific risks detailed in this "Risk Factors" section, together with all of the other information contained in this Quarterly Report on Form 10-Q. If any of these risks occur, our business, results of operations and financial condition could be harmed, the price of our common stock could decline, and you may lose part or all of your investment.

Risks Associated with Commercialization and Product Development

We currently depend entirely on the success of our lead drug, PROCYSBI. If PROCYSBI sales in the U.S. and the EU are not robust, our business, financial condition and results of operations will be adversely affected.

Sales of PROCYSBI will likely drive the trading price of our common stock. We launched commercial sales of PROCYSBI in the U.S. in June 2013 and do not have prior experience in commercializing therapeutics. If PROCYSBI sales are not robust, our stock price may not increase or could significantly decrease. The successful commercialization of PROCYSBI will depend on several factors, including:

- the successful launch of PROCYSBI in EU countries and other select territories;
- acceptance of PROCYSBI by physicians, parents, patients and cystinosis research/advocacy organizations including the conversion from the existing standard of care to PROCYSBI;
- coverage and reimbursement for PROCYSBI from commercial health plans and government health programs, which we refer to collectively as third-party payors;
- compliance with regulatory requirements including fulfilling any FDA and EC required post-approval commitments;
- provision of affordable out-of-pocket cost to patients and/or other programs to ensure patient access to PROCYSBI;
- approval by other country regulatory agencies of appropriate product labeling for PROCYSBI;
- agreements with wholesalers and distributors on commercially reasonable terms;
- manufacture and supply of adequate quantities of PROCYSBI to meet commercial demand; and
- development and maintenance of intellectual property protection for PROCYSBI.

If we fail to commercialize PROCYSBI at sufficient sales levels in the U.S. or successfully commercialize PROCYSBI in Europe within a reasonable time period, we may never become profitable and may be unable to sustain our business, and our business, financial condition and results of operations will be adversely affected.

Our ability to generate significant revenues from PROCYSBI is dependent upon market acceptance among physicians, patients, patient families, third-party payors and the healthcare community.

PROCYSBI may not attain market acceptance among physicians, patients, patient families, third-party payors or the healthcare community compared to the current standard of care. We believe that the degree of market acceptance and our ability to generate significant revenues from PROCYSBI will depend on a number of factors, including:

- availability and relative efficacy, safety and ease of administration of alternative treatments;
- the price of our product, both in absolute terms and relative to alternative treatments;
- timing of market introduction of our product as well as competitive drugs;
- efficacy, safety and the prevalence and severity of any side effects of PROCYSBI;
- identification of currently diagnosed and undiagnosed patients and continued projected growth of the cystinosis market;
- acceptance by patients, patient families and primary care and other specialists including conversion from the existing standard of care;
- the effect of current and future healthcare laws;
- availability of coverage and adequate reimbursement and pricing from third-party payors; and
- breadth of product labeling or product insert requirements of the FDA, EC or other regulatory authorities.

If PROCYSBI does not receive significant market acceptance among physicians, patients, patient families, third-party payors or the healthcare community, our ability to generate revenues from PROCYSBI will be severely affected.

If we are unable to expand the use of RP103, our proprietary delayed-release form of cysteamine (DR Cysteamine or RP103), and receive regulatory approval for any other indication, we may delay or cease some of our product development activities, which would adversely affect the long term value of RP103 and our growth prospects.

We must obtain and maintain appropriate pre-market approvals from regulatory agencies in each of the markets in which we intend to market our products. Once approved, we may only market our products for the specific uses that are reflected in the product's approved labeling. In the U.S., we are permitted to market RP103 only for the management of nephropathic cystinosis in adults and children six years and older under the brand name PROCYSBI. We are permitted to market PROCYSBI in the EU as an orphan medicinal product for the treatment of proven nephropathic cystinosis. We do not have approval of RP103 in any other market. A new drug application (NDA) submitted to the FDA or marketing authorization application (MAA) submitted to the European Commission must be supported by extensive clinical and preclinical data, as well as extensive information regarding chemistry, manufacturing and controls, to demonstrate the safety and efficacy of the applicable product candidate for the treatment of each individual indication.

Obtaining approval of an NDA, MAA or any other filing for marketing authorization in a foreign country is an extensive, expensive and uncertain process. The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. The FDA, EC or other regulatory authorities may delay, limit or deny approval of RP103 or our future drug product candidates for many reasons, including:

- the results of clinical trials may not meet the level of statistical or clinical significance required by regulatory authorities for approval;
- regulatory authorities may disagree with the number, design, size, conduct or implementation of our clinical trials;
- may not find the data from preclinical studies and clinical trials sufficient to demonstrate that a product candidate has

adequate clinical and other benefits or an adequate safety profile; or may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct additional trials;

- regulatory authorities may not accept data generated at our clinical trial sites;
- regulatory authorities may have difficulties scheduling an advisory committee meeting (or equivalent, if required) in a timely manner or the advisory committee may recommend against approval of our application or may recommend that the regulatory agency require, as a condition of approval, additional preclinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;
- regulatory authorities may require additional preclinical or clinical studies or other data prior to granting approval, and we may not be able to generate the required data on a timely basis, if at all;
- regulatory authorities may impose limitations on approved labeling, thus introducing reimbursement complications which may limit access for intended uses;

- regulatory authorities may identify deficiencies in the manufacturing processes or in the facilities of our third party suppliers and/or contract manufacturers, or may require us to manufacture additional validation batches or change our process or specifications;
- we may not be able to validate our manufacturing process to the satisfaction of the regulatory authorities, or they may not agree with our plan for potential retrospective validation; or
- regulatory authorities may change approval policies or adopt new regulations.

See also the risk factors titled "If we fail to obtain and maintain approval from regulatory authorities in international markets for PROCYSBI, RP103 and any future product candidates for which we have rights in international markets, our market opportunities will be limited and our business will be adversely impacted" and "If we fail to demonstrate safety or efficacy in our preclinical studies or clinical trials or keep to the terms of a product development program, our future business prospects for these drug product candidates will be materially adversely affected."

If we fail to gain regulatory approval for RP103 for other indications, we will have to delay or terminate some or all of our research product development programs and our business, financial condition and results of operations will be adversely affected.

We have no internal manufacturing experience with PROCYSBI and RP103. We expect to continue to rely on a single supplier for the active pharmaceutical ingredient and a single third-party manufacturer for the conversion to finished drug product. If we are unable to obtain an adequate supply of our drugs, our reputation would be harmed, our revenues would be delayed or diminished and our financial results would be adversely affected.

We do not currently manufacture PROCYSBI and RP103. We rely on single manufacturing sources for our cysteamine active pharmaceutical ingredient, or API, and finished products. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production to commercial requirements. Difficulties may arise related to production costs and yields, quality control, including stability of the product or product candidates and quality control testing, sourcing scarcities, resource constraints, equipment problems, shortages of qualified personnel, labor disputes, severe weather events, unstable political environments or financial difficulties at foreign facilities, as well as compliance with strictly enforced federal, state and foreign regulations.

We rely on one exclusive supplier for the API for PROCYSBI and RP103. A reduction or interruption in our supply of API from this supplier and of finished goods from our contract manufacturer, and efforts to identify and qualify alternative sources of API supply, could result in significant additional operating costs and delays in sales of PROCYSBI and in developing RP103 for HD and NAFLD. In addition, supply arrangements from alternative sources may not be available on acceptable economic terms, if at all.

We depend on our third-party suppliers and manufacturers for compliance with the FDA's cGMP requirements and other FDA requirements, Drug Enforcement Administration's regulations and other rules and regulations prescribed by applicable non-U.S. regulatory authorities. If we or our third-party suppliers and manufacturers fail to comply with applicable regulatory requirements, a regulatory agency may issue warning letters or untitled letters; seek an injunction or impose civil or criminal penalties or monetary fines; suspend or withdraw regulatory approval; suspend any ongoing clinical trials; refuse to approve pending applications or supplements to applications; suspend or impose restrictions on operations, including costly new manufacturing requirements; seize or detain products; or request that we initiate a product recall.

If any of these events were to occur, our reputation would be harmed, revenues from sales of our products would be delayed or diminished and our business, financial condition and results of operations would be adversely affected.

PROCYSBI is, and any other future product candidates, if approved, will be, subject to extensive and ongoing regulatory requirements and continued regulatory review, which may result in significant additional expense. Additionally, PROCYSBI and our future product candidates, if approved, may be subject to labeling and other restrictions or potential market withdrawal, and we may be subject to penalties and litigation if we fail to comply with regulatory requirements or experience problems with our products.

Our manufacturing processes, labeling, packaging, distribution, storage, adverse event reporting, dispensation, distribution, advertising, promotion and recordkeeping are subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, ongoing maintenance of product registration, as well as continued compliance with cGMPs (good manufacturing practices), GCPs (good clinical practices), GDPs (good distribution practices) and GLPs (good laboratory practices). If we do not comply with applicable regulations and requirements, the range of possible sanctions includes adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, withdrawal of a product's approval and enforcement actions, including injunctions and civil or criminal prosecution. In addition, if we or a regulatory agency discover previously unknown problems with PROCYSBI, such as adverse events of unanticipated severity or frequency, or identify data that suggest that PROCYSBI may present a risk to safety, the regulatory authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, our growth prospects and our operating results will be adversely affected.

In addition, any regulatory approvals that we obtain will be subject to limitations on the approved indicated uses for which the product may be marketed or the conditions of approval, or contain requirements for potentially costly post-market testing and surveillance to monitor the safety and efficacy of the product. The FDA and EC strictly regulate the promotional claims that may be made about prescription products and our product labeling, advertising and promotion is subject to continuing regulatory review. Physicians nevertheless may prescribe our product to their patients in a manner that is inconsistent with the approved label, or that is off-label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and if we are found to have improperly promoted off-label uses we may be subject to significant sanctions, civil and criminal fines and injunctions prohibiting us from engaging in specified promotional conduct.

In addition, engaging in improper promotion of our products for off-label uses in the U.S. can subject us to false claims litigation under federal and state statutes, which can lead to consent decrees, civil money penalties, restitution, criminal fines and imprisonment, and exclusion from participating in Medicare, Medicaid and other federal and state health care programs. These false claims statutes in the U.S. include the federal False Claims Act, which allows any individual to bring a lawsuit against a pharmaceutical company on behalf of the federal government alleging submission of false or fraudulent claims, or causing to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government prevails in the lawsuit, the individual will share in any fines or settlement funds. Growth in false claims litigation has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, agree to comply with burdensome reporting and compliance obligations, and be excluded from Medicare, Medicaid and other federal and state healthcare programs.

If serious adverse side effects become associated with PROCYSBI, our business will be harmed.

The prescribing information for PROCYSBI includes several warnings relating to observed adverse reactions of cysteamine bitartrate usage. These adverse reactions were not observed in our clinical trials supporting PROCYSBI's NDA and MAA, but required on our label due to our submission of a 505(b)(2) application in the U.S. and a hybrid

application in the EU. We expect to update adverse reactions listed in the prescribing information based on continued commercial use and additional clinical trials. If additional adverse reactions emerge, or if there is a pattern of severe or persistent previously observed side effects in the relevant patient populations, the FDA or other regulatory agencies could modify or revoke our marketing approval or require us to suspend production, or we may choose to withdraw PROCYSBI from the market. If this were to occur, we may be unable to obtain marketing approval in other indications. In addition, patients or their representatives may bring claims against us alleging serious adverse side effects or harm suffered as a result of use of PROCYSBI. Any such side effects or related claims could have a material adverse effect on our business, financial condition and results of operations.

See also the risk factor titled "We may be subject to product liability claims."

Pressure on drug product third-party payor coverage, reimbursement and pricing may impair our ability to be reimbursed for PROCYSBI and our other future product candidates at prices or on terms sufficient to provide a viable financial outcome.

Market acceptance and sales of PROCYSBI and any product candidates that we may develop will depend in large part on third-party payor coverage and reimbursement policies and may be affected by future healthcare reform measures in the U.S. as well as the EU and other key international markets. The continuing efforts of governmental and third-party payors to contain, reduce or shift the costs of healthcare through various means, including an increased emphasis on managed care and attempts to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, may result in downward pressure on product pricing, reimbursement and utilization, which may adversely affect our product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, drug coverage and reimbursement policies and pricing in general. Moreover, private health insurers and other third-party payors in the U.S. often follow the coverage and reimbursement policies of government payors, including the Medicare or Medicaid programs.

Beginning April 1, 2013, Medicare payments for all items and services under Part A and B, including drugs and biologicals, and most payments to plans under Medicare Part D were reduced by 2% under the sequestration (i.e., automatic spending reductions) required by the Budget Control Act of 2011, or BCA, as amended by the American Taxpayer Relief Act, or ATRA. The BCA requires sequestration for most federal programs, excluding Medicaid, Social Security and certain other programs, because Congress failed to enact legislation by January 15, 2012, to reduce federal deficits by $1.2 trillion over ten years. As long as BCA cuts remain in effect, they could adversely impact payment for PROCYSBI. In addition, other recent legislative changes that increase manufacturer liability for rebates and other payments under the 340B drug pricing program, the Medicaid Drug Rebate Program and the Medicare Part D prescription drug benefit also could impact our revenues. Further, payors also are increasingly considering new metrics as the basis for reimbursement rates, such as average sales price, or ASP, average manufacturer price, or AMP, or actual acquisition cost, or AAC. Although the intent of the changes to reimbursement methodologies generally is to limit payment increases, it is difficult to project the impact of these and other alternative reimbursement methodologies on the willingness of payors to cover PROCYSBI and any product candidates that we may develop. Although to-date PROCYSBI has been covered, we will not know whether third-party payors will continue to cover and reimburse PROCYSBI in the U.S. and whether third-party payors will cover and reimburse for our future products until we enter into payor negotiations. If reimbursement is not available or available only to limited levels, we may not be able to generate sufficient revenue to meet our operating costs or to achieve our revenue, cash flow breakeven or profitability goals in the timeframe that we expect, or at all.

Enacted and future legislation may increase the difficulty and cost for us to commercialize PROCYSBI or any other product candidate that we develop and affect the prices we may obtain.

In the U.S., there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that restrict or regulate post-approval activities, which may affect our ability to profitably sell PROCYSBI or any other product candidate for which we obtain marketing approval.

The Medicare Prescription Drug Improvement and Modernization Act of 2003, or MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for outpatient drug purchases by those covered by Medicare under a new Part D and introduced a new reimbursement methodology based on average sales prices for Medicare Part B physician-administered drugs. In addition, this legislation authorized Medicare Part D prescription drug plans to use formularies whereby they can limit the number of drugs that will be covered in any therapeutic class. As a result of this legislation and the expansion of federal coverage of drug products, there is additional pressure to contain and reduce costs. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own

reimbursement rates, and any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors. These cost reduction initiatives and other provisions of the MMA could decrease the coverage and reimbursement that we receive for any approved products, and could seriously harm our business.

In 2010, President Obama signed into law the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act of 2010 (together, the "Health Care Reform Law"), which is likely to continue the pressure on pharmaceutical pricing and a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Health Care Reform Law, among other things, revised the definition of AMP for reporting purposes, which could increase the amount of Medicaid drug rebates to states and extended the rebate program to beneficiaries enrolled in Medicaid managed care organizations. The Health Care Reform Law also imposed a significant annual fee on companies that manufacture or import branded prescription drug products and established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the U.S. The Health Care Reform Law also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or "donut hole." The Health Care Reform Law includes a provision to increase the Medicaid rebate for line extensions or reformulated drugs, which depending on how this provision is implemented could substantially increase our Medicaid rebate rate (in effect limiting reimbursement for these patients). These and other new provisions may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs. Please see the risk factor titled "Failure to comply with healthcare regulations may subject us to substantial penalties" for additional information.

Legislative and regulatory proposals also have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may subject us to more stringent product labeling and post-marketing testing and other requirements.

Failure to comply with healthcare regulations may subject us to substantial penalties.

Although we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, federal and state healthcare laws and regulations pertaining to fraud and abuse, physician payment transparency and privacy and security laws and regulations apply to us and our arrangements with healthcare providers, customers and other entities, including our marketing practices, educational programs and pricing policies. The laws that may affect our ability to operate as a commercial organization include:

- the federal healthcare programs' Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs, such as the Medicare and Medicaid programs;
- federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other federal third-party payors that are false or fraudulent;
- federal criminal laws that prohibit executing a scheme to defraud any federal healthcare benefit program or making false statements relating to healthcare matters;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information;
- the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and applicable manufacturers and group purchasing organizations to report annually to CMS ownership and investment interests held by physicians (as defined above) and their immediate family members and payments or

other "transfers of value" to such physician owners and their immediate family members. Manufacturers were required to begin data collection on August 1, 2013 and will be required to report such data to the government by March 31, 2014 and by the 90th calendar day of each year thereafter; and

- analogous state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers.

In addition, there has been a recent trend of increased federal and state regulation of payments made to physicians for marketing. Certain states mandate implementation of compliance programs and/or the tracking and reporting of gifts, compensation, and other remuneration to physicians. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increase the possibility that a healthcare company may violate one or more of the requirements.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available under such laws, it is possible that some of our business activities, including our relationships with physicians and other health care providers, some of whom recommend, purchase and/or prescribe our products, could be subject to challenge under one or more of such laws. While these arrangements were structured to comply with all applicable laws, if our operations are found to be in violation of any of the laws described above or any other laws and regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to market PROCYSBI, RP103 and other future drug candidates and adversely impact our financial results. The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. See also the risk factor titled "If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and future business prospects."

In addition, the Health Care Reform Law further strengthened these laws by amending the intent requirement of the federal anti-kickback and criminal health care fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the Health Care Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the false claims statutes. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and future business prospects.

We participate in the Medicaid Drug Rebate Program and other Federal and state government pricing programs in the U.S., and we may participate in additional government pricing programs in the future. These programs generally require us to pay rebates or provide discounts to government payers in connection with drugs, including PROCYSBI, that are dispensed to beneficiaries of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing and rebate calculations that we report on a monthly and quarterly basis to the government agencies that administer the programs. The terms, scope and complexity of these government pricing programs change frequently. Responding to current and future changes may increase our costs and the complexity of compliance will be time-consuming, and could have a material adverse effect on our results of operations.

Pricing and rebate calculations vary among products and programs. The calculations are complex and are often subject to interpretation by governmental or regulatory agencies and the courts.

In addition, the Office of Inspector General has recently increased its focus on the methodologies used by manufacturers to calculate AMP and BP to assess manufacturer compliance with reporting requirements under the

Medicaid Drug Rebate Program. We are liable for errors associated with our submission of pricing data and for overcharging government payors. For example, failure to submit monthly/quarterly AMP and BP data on a timely basis could result in a civil monetary penalty of $10,000 per day for each day the submission is late beyond the due date. Failure to make necessary disclosures and/or to identify overpayments could result in allegations against us under the Federal False Claims Act and other laws and regulations.

Unexpected refunds to the U.S. government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition and results of operations. In the event that CMS were to terminate our rebate agreement, no federal payments would be available under Medicaid or Medicare for our covered outpatient drugs.

Because the target patient populations for some of our drug product candidates, including PROCYSBI, are small, we must achieve significant market share and obtain relatively high per-patient prices for our products to achieve meaningful gross margins.

PROCYSBI and our clinical development of RP103 target diseases with small patient populations, including cystinosis and HD, respectively. A key component of the successful commercialization of a drug product for these indications includes identification of patients and a targeted prescriber base for the drug product. Due to small patient populations, we believe that we would need to have significant market penetration to achieve meaningful revenues and identifying patients and targeting the prescriber base are key to achieving significant market penetration. In addition, the per-patient prices at which we sell PROCYSBI (currently an estimated average of $250,000 per year in the U.S.) and RP103 for these indications will need to be relatively high in order for us to generate an appropriate return for the investment in these product development programs and achieve meaningful gross margins. There can be no assurance that we will be successful in achieving a sufficient degree of market penetration and/or obtaining or maintaining high per-patient prices for PROCYSBI and RP103 for diseases with small patient populations.

If we fail to obtain or maintain orphan drug exclusivity or regulatory exclusivity for some of our drug product candidates, our competitors may sell products to treat the same conditions or at greatly reduced prices and our revenues will be reduced.

As part of our business strategy, we intend to develop RP103 for additional indications and other drugs that may be eligible for FDA and EC orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, defined as a patient population of less than 200,000 in the U.S. , or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years, with an additional six months if for a pediatric indication. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective, a subsequent product is deemed clinically superior, or if the manufacturer is unable to assure sufficient quantity of the drug. Similar regulations are available from the EC with a 10-year period of market exclusivity.

Because the extent and scope of patent protection for some of our drug products may be particularly limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the orphan exclusivity period to maintain a competitive position. However, if we do not obtain orphan drug exclusivity for RP103 for the potential treatment of HD or other potential indications, or our future relevant drug products do not have strong patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced. Also, without strong patent protection, competitors may sell a generic version upon the expiration of orphan exclusivity, if our patent position is not upheld.

Even though we have been granted orphan drug designation prior to the approval of RP103 for the potential treatment of HD, and even if we obtain orphan drug designation for our future drug product candidates, we may not fulfill the criteria for exclusivity or we may not be the first to obtain marketing approval for any orphan indication. Further, even if we obtain orphan drug exclusivity for a particular product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care.

A breakthrough designation or fast track designation for our drug product candidates, if obtained, may not actually lead to a faster review process.

Under the Prescription Drug User Fee Act, the FDA has a goal of responding to NDAs within ten months of submission the filing date for standard review, but this timeframe is also often extended. In the future, we may seek approval of our drug candidates under programs designed to accelerate the FDA's review and approval of NDAs. For instance, a sponsor may seek FDA designation of a drug candidate as a "fast track product." Fast track products are those products intended for the treatment of a serious or life-threatening disease or condition and which demonstrate the potential to address unmet medical needs for such disease or condition. If fast track designation is obtained, the FDA may initiate review of sections of an NDA before the application is complete. This "rolling review" is available if the applicant provides and the FDA approves a schedule for the remaining information. In some cases, a fast track product may be approved on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Approvals of this kind typically include requirements for appropriate post-approval Phase IV clinical trials to validate the surrogate endpoint or otherwise confirm the effect of the clinical endpoint. In addition, the Food and Drug Administration Safety and Innovation Act, or FDASIA, which was enacted and signed into law in 2012, established a new category of drugs referred to as "breakthrough therapies," which are defined as drugs intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. In the future, we may request breakthrough designation or fast track designation from the FDA for our other drug product candidates, but we cannot assure you that we will obtain such designations. Moreover, even if we obtain breakthrough designation or fast track designation from the FDA, the designations do not guarantee FDA approval of our NDA, that the development program or review timeline will ultimately be shorter than if we had not obtained the designations, or that the FDA will not request additional information, including requesting additional clinical studies (although potentially a post-marketing requirement), during its review. Any request for additional information or clinical data could delay the FDA's timely review of our NDA.

We may not be successful in integrating our European operations with our U.S. operations.

In connection with the European commercial launch of PROCYSBI, we have expanded our operations in Europe where we expect to continue to add personnel. We may encounter difficulties successfully managing remotely a substantially larger and internationally diverse organization and may encounter delays in commercialization if we are not successful in integrating our international operations. Challenges related to managing international operations include the following:

- the potential strain on our financial and managerial controls and reporting systems and procedures;
- potential miscommunication between U.S. personnel and European personnel due to cultural and language differences;
- ability to operate within diverse individual country regulatory and statutory laws; and
- greater than anticipated costs of maintaining EU presence, in-country legal entities and related tax structures.

If we fail to obtain and maintain approval from regulatory authorities in international markets for PROCYSBI, RP103 and any future product candidates for which we have rights in international markets, our market opportunities will be limited and our business will be adversely impacted.

Sales of our products outside of the U.S. are subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approvals. Even if the FDA and EC grants marketing approval for a product candidate,

comparable regulatory authorities of foreign countries must also approve the manufacturing and marketing of our product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., including additional preclinical studies or clinical trials or manufacturing and control requirements. In many countries outside the U.S., a product candidate must be approved for reimbursement before it can be approved for sale in that country. In many cases, the price that we propose to charge for our products is also subject to approval by individual countries before we can launch our product candidates in those countries. Obtaining foreign regulatory approvals, complying with foreign regulatory requirements and gaining approved pricing and reimbursement could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries and regulatory approval in one country does not ensure approval in any other country, while a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others.

If we fail to demonstrate safety or efficacy in our preclinical studies or clinical trials or keep to the terms of a product development program, our future business prospects for these drug product candidates will be materially adversely affected.

The success of our development and commercialization efforts will be greatly dependent upon our ability to demonstrate safety and efficacy in preclinical studies and clinical trials. Preclinical studies involve testing in appropriate multiple non-human disease models to demonstrate efficacy and safety. Regulatory agencies evaluate these data carefully before they will approve clinical testing in humans. If certain preclinical data reveals potential safety issues or the results are inconsistent with an expectation of the drug product candidate's efficacy in humans, the regulatory agencies may require additional testing before allowing human clinical trials. This additional testing will increase program expenses and extend timelines. We may decide to suspend further testing on our drug product candidates or technologies if, in the judgment of our management and advisors, the preclinical test results do not support further development. There are many potential preclinical models to test for different disease states, and we could fail to choose the best preclinical model to determine proof of concept, safety and efficacy of our drug product candidates.

Following successful preclinical testing, drug product candidates must be tested in a clinical development program to provide data on safety and efficacy in humans prior to becoming eligible for product approval and licensure by regulatory agencies. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful. The clinical trial process may fail to demonstrate with statistical significance that our drug product candidates are safe for humans and effective for indicated uses. This failure would cause us to abandon a drug product candidate and may delay development of other drug product candidates. Any delay in, or termination of, our preclinical testing or clinical trials will delay the filing of our investigational new drug application, or IND, and NDA as applicable, with the FDA, MAA and Investigational Medicinal Product Dossier, or IMPD, with the EC or other regulatory agencies and, ultimately, our ability to commercialize our drug product candidates and generate product revenues. In addition, many of our clinical trials will involve small patient populations. Because of the small sample size, the results of these early clinical trials may not be indicative of future results. The failure to demonstrate safety or efficacy in our clinical trials would have a material adverse effect on our future business prospects, financial condition and results of operations.

We may be subject to product liability claims.

The nature of our business exposes us to potential liability risks inherent in the testing (including through human trials), manufacturing and marketing of drugs. PROCYSBI and our drug product candidates could potentially harm people or allegedly harm people and we may be subject to costly and damaging product liability claims. Many of the patients who participate in our current clinical trials and U.S. and EU cystinosis patients who may purchase PROCYSBI commercially are already critically ill or suffering from chronic debilitating diseases. The waivers we obtain in clinical trials may not be enforceable and may not protect us from liability or the costs of product liability litigation.

We may not be able to avoid significant liability if any product liability claim is brought against us. Although we currently carry product liability insurance, it may not be sufficient to cover future claims. If a successful product liability claim is brought against us and the amount of liability exceeds our insurance coverage, we may incur substantial charges that would adversely affect our business, financial condition and results of operations.

We rely on third parties for the distribution and pharmaceutical services of PROCYSBI in the U.S. and the EU.

We rely on a third party logistics provider and specialty pharmacy to distribute PROCYSBI to patients and to collect from insurance companies and government agencies in the U.S. and in the EU. Our ability to collect from the logistics provider is not only subject to such provider's credit worthiness but is also dependent, in part, on its ability to

arrange for full reimbursement from third party payors. The outsourcing of our distribution function is complex, and we may experience difficulties that could reduce, delay or stop shipments of PROCYSBI. If we encounter such distribution problems, and we are unable to quickly enter into a similar agreement with another specialty distributor on substantially similar terms, if at all, the distribution of PROCYSBI could become disrupted, resulting in reduced revenues, healthcare provider dissatisfaction and/or patient dissatisfaction which may harm our reputation and financial condition.

Our reliance on third parties may result in delays in completing, or a failure to complete, preclinical testing, clinical trials or regulatory marketing submissions.

In the course of product development, we engage and collaborate with a variety of external organizations to perform services essential to drug product development. The organizations which perform services may include, but are not limited to:

- governmental agencies, U.S. and international university laboratories;
- other biotechnology companies;
- contract manufacturing organizations;
- clinical research organizations;
- distribution and supply (logistics) service organizations;
- contract testing organizations;
- consultants or consulting organizations with specialized knowledge based expertise;
- intellectual property legal firms; and
- multiple other service organizations.

As a result of our engagement of these types of organizations to help us with our product development programs, many important aspects of our business are will be out of our direct control. Nevertheless, we are responsible for ensuring that each of our product development programs complies with applicable regulatory requirements, and our reliance on these organizations does not relieve us of our regulatory responsibilities. If any such organizations we engage in the future fail to perform their obligations under our agreements with them or fails to perform in a satisfactory manner, we may face delays in completing our development and commercialization processes for any of our drug product candidates. Furthermore, any loss or delay in obtaining contracts with such entities may also delay the completion of our clinical trials, regulatory filings and the potential market approval of our drug product candidates.

Specifically, we have and will continue to rely on third parties, such as contract research organizations and/or co-operative groups, to assist us in overseeing and monitoring clinical trials as well as to process the clinical results. If third parties fail to perform or to meet the applicable standards, this will result in delays in or failures to complete trials. A failure by us or such third parties to observe the terms of a product development program for any particular product candidate or to complete the clinical trials for a product candidate in the anticipated time frame could have significant negative repercussions on our business and financial condition.

In addition, our dependence on collaborative arrangements with third parties will subject us to a number of risks that could harm our ability to develop and commercialize products:

- collaborative arrangements might not be available on terms which are reasonably favorable to us, or at all;
- disagreements with partners may result in delays in the development and marketing of products, termination of collaboration agreements or time consuming and expensive legal action;
- agreement terms may be difficult or costly to enforce;
- partners may not allocate sufficient funds or resources to the development, promotion or marketing of our product candidates, or may not perform their obligations as expected;
- partners may choose to develop, independently or with other companies, alternative products or treatments, including products or treatments which compete with ours;
- agreements with partners may expire or be terminated without renewal, or partners may breach agreements with us;
- business combinations or significant changes in a partner's business strategy might adversely affect that partner's willingness or ability to complete its obligation to us; and
- the terms and conditions of the relevant agreements may no longer be suitable.

We cannot guarantee that we will be able to negotiate acceptable future collaboration agreements or that those currently in existence will make it possible for us to fulfill our objectives.

We depend on the support of key scientific and medical collaborators.

We must establish and maintain relationships with key opinion leaders, leading scientists and research institutions. We believe that such relationships are pivotal to establishing products using our technologies as a standard of care for their approved indications. Although we have various medical and scientific advisors and research collaborations, there is no assurance that our advisors and our research collaborators will continue to work with us or that we will be able to attract additional research partners. If we are not able to maintain existing or establish new clinical and scientific relationships to assist in our commercialization and research and development, we may not be able to successfully develop PROCYSBI, RP103 or our other drug product candidates.

We will continue to incur increased costs as a result of corporate governance and financial reporting laws and regulations and our management will continue to be required to devote substantial time to comply with such laws and regulations.

We face burdens relating to the recent trend toward stricter corporate governance and financial reporting standards. Legislation or regulations, such as the Sunshine Act, Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the Dodd-Frank Wall Street Reform and Consumer Protection Act, as well as other rules implemented by the FDA, the SEC and NASDAQ, follow the trend of imposing stricter corporate governance and financial reporting standards and have led to an increase in the costs of compliance, including substantial increases in consulting, auditing and legal fees. Our management and other personnel will need to devote a substantial amount of time to these requirements. New rules could make it more difficult or more costly for us to obtain certain types of insurance, including directors' and officers' liability insurance, and we may be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers. Failure to comply with these new laws and regulations may impact market perception of our financial condition and could materially harm our business.

In addition, the Sarbanes-Oxley Act requires, among other things, that we maintain effective internal control over financial reporting and disclosure controls and procedures. In particular, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. Our compliance with Section 404 will require that we incur substantial accounting and related expense and expend significant management efforts. We may need to hire additional accounting and financial staff to satisfy the ongoing requirements of Section 404. Moreover, if we are not able to comply with the requirements of Section 404, or we or our independent registered public accounting firm identifies deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by NASDAQ, the SEC or other regulatory authorities.

Our success depends on our ability to manage our projected growth.

Continued commercial sales of PROCYSBI in the U.S., the EU commercial launch of PROCYSBI, expansion of our commercial operations into other markets and the continuation of our clinical-stage programs and our current plans to in-license and acquire additional clinical-stage product candidates will require us to retain existing and add required new qualified and experienced personnel in all functional areas over the next several years. Also, if our preclinical pipeline diversifies through internal discoveries, or the acquisition or in-licensing of new molecules, we will need to hire additional scientists to supplement our existing scientific expertise over the next several years.

Our staff, financial resources, systems, procedures or controls may be inadequate to support our expanding operations and our management may be unable to take advantage of future market opportunities or manage successfully our relationships with third parties if we are unable to adequately manage our anticipated growth and the integration of

new personnel.

Our loan agreement with HC Royalty contains a number of restrictive covenants and other provisions, which, if violated, could result in the acceleration of the payment terms of our outstanding indebtedness, which could have an adverse impact on our business and financial condition.

In December 2012, we entered into a loan agreement with HealthCare Royalty Partners II, L.P., or HC Royalty, as lender, under which we agreed to borrow $50.0 million in two $25.0 million tranches, or the HC Royalty loan agreement. We drew down the first tranche in the amount of $25.0 million in December 2012 upon signing the HC Royalty loan agreement and we drew down the second tranche of $25.0 million in May 2013 as a result of our achievement of the milestone of U.S. approval of PROCYSBI. The HC Royalty loan agreement includes a number of affirmative and negative covenants, including the use of commercially reasonable efforts to exploit PROCYSBI and RP103 in specific markets and compliance with laws, as well as restrictions on mergers and sales of assets, incurrence of liens, incurrence of indebtedness and transactions with affiliates and other requirements. To secure the performance of our obligations under the HC Royalty loan agreement, we granted a security interest to HC Royalty in substantially all of our assets, the assets of our subsidiaries and a pledge of stock of certain of our subsidiaries. Our failure to comply with the terms of the HC Royalty loan agreement and related documents, the occurrence of a change of control of our Company or the occurrence of an uncured material adverse effect on our Company, or our wholly-owned subsidiary Raptor Pharmaceuticals, or the occurrence of certain other specified events, could result in an event of default under the HC Royalty loan agreement that, if not cured or waived, could result in the acceleration of the payment of all of our indebtedness to HC Royalty and interest thereon. Under the terms of the security agreement, in an event of default, the lender could potentially take possession of, foreclose on, sell, assign or grant a license to use our pledged collateral and assign and transfer the pledged stock of certain of our subsidiaries.

Credit risks from customers outside the U.S. may negatively affect our results of operations.

Any future sales of our products to government supported customers outside of the U.S. are likely to be subject to significant payment delays due to government funding and reimbursement practices, which will result in an increase in the length of time that we may have accounts receivable outstanding. For example, many governments in Europe are facing significant liquidity crises. If government reimbursement for future sales of PROCYSBI or our potential products in the EU is delayed or becomes unavailable, we may not be able to collect on amounts payable to us in reasonable time frames from such customers and our capital requirements will increase and our results of operations would be adversely affected.

Our business could be adversely affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates, foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets and business and economic conditions. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to increase the price of PROCYSBI or other future products due to the process by which healthcare providers are reimbursed.

The U.S. credit and capital markets have recently experienced historic dislocations and a massive liquidity crisis which have caused financing to be unavailable in many cases and, even if available, have caused the cost of prospective financings to significantly increase. These circumstances have had a material impact on liquidity in the debt and capital markets, making financing terms for borrowers or for companies seeking equity capital, for those companies that are able to find financing at all, less attractive. In many cases, financial conditions have resulted in the reduced availability or the unavailability of certain types of debt or equity financing. Continued uncertainty in the debt and equity markets may negatively impact our ability to access financing on favorable terms or at all. Federal legislation to deal with the current disruptions in the financial markets could have an adverse effect on our ability to raise other types of financing. In addition, our suppliers, manufacturers and other third parties important to our

business also may be negatively affected by market dislocations and disruptions, their businesses may be disrupted and this could adversely affect our business and results of operations.

Any product revenues could be reduced by imports from countries where our product candidates are available at lower prices.

Even though we have FDA approval of PROCYSBI, our sales in the U.S. may be reduced if PROCYSBI is imported into the U.S. from lower priced markets, whether legally or illegally. In the U.S., prices for pharmaceuticals are generally higher than in the bordering nations of Canada and Mexico. There have been proposals to legalize the import of pharmaceuticals from outside the U.S. If such legislation were enacted, our potential future revenues could be reduced.

Our future international sales and operating expenses will be subject to fluctuations in currency exchange rates.

If and when we launch PROCYSBI in the EU and in other countries outside the U.S., a portion of our business will be conducted in currencies other than our reporting currency, the U.S. dollar. We will recognize foreign currency gains or losses arising from our operations in the period in which we incur those gains or losses. As a result, currency fluctuations between the U.S. dollar and the currencies in which we do business will likely cause foreign currency translation gains and losses in the future. Because of the number of currencies that may be involved, the variability of currency exposures and the potential volatility of currency exchange rates, we may suffer significant foreign currency translation and transaction losses in the future due to the effect of exchange rate fluctuations.

Our future success depends, in part, on the continued services of our management team.

Our success is dependent in part upon the availability of our senior executive officers, including Christopher M. Starr, Ph.D., Chief Executive Officer; Julie Anne Smith, Chief Operating Officer; Georgia Erbez, Chief Financial Officer; Ted Daley, Chief Business Officer and Patrice Rioux, M.D., Ph.D., Chief Medical Officer. The loss or unavailability to us of any of these individuals or key research and development personnel, and particularly if lost to competitors, could have a material adverse effect on our business, prospects, financial condition and results of operations. We do not have key-man insurance on any of our employees.

There is no assurance that we will be able to retain key employees or consultants. There is intense competition for qualified scientists and managerial personnel from numerous pharmaceutical and biotechnology companies, as well as from academic and government organizations, research institutions and other entities. If key employees terminate their employment, or if insufficient numbers of qualified employees are retained, or are not available via recruitment, to maintain effective operations, our development activities might be adversely affected, management's attention might be diverted from managing our operations to hiring suitable replacements and our business might suffer. In addition, we might not be able to locate suitable replacements for any key employees that terminate their employment with us and we may not be able to offer employment to potential replacements on reasonable terms, which could negatively impact our product candidate development timelines and may adversely affect our future revenues and financial condition.

In addition to our employees, we rely and will continue to rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development strategy. All of our consultants and advisors will be employed by other employers or be self-employed, and will have commitments to or consulting or advisory contracts with other entities that may limit their availability to us.

If we do not achieve our projected development and commercialization goals in the time frames we expect and announce, the price of our common stock may decline.

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory, market launch and commercialization goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials, the submission of regulatory filings and product launch.

From time to time, we may publicly announce the estimated timing of some of these milestones. All of these milestones will be based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. For example, clinical trials may be delayed due to factors such as institutional review board, or IRB, approvals, qualification of clinical sites, scheduling conflicts with participating clinicians and clinical institutions and the rate of patient enrollment. In most circumstances, we rely on academic institutions, major medical institutions, governmental research organizations (U.S. or internationally based), clinical research organizations or contract manufacturing organizations to conduct, supervise or monitor some

or all aspects of clinical trials involving our product candidates. We will have limited control over the timing and other aspects of these clinical trials. See also the risk factor titled "Our reliance on third parties may result in delays in completing, or a failure to complete, preclinical testing, clinical trials or regulatory marketing submissions."

If we do not meet the milestones as publicly announced, or as projected by various security analysts who follow our Company, our stockholders or potential stockholders may lose confidence in our ability to meet overall product development and commercialization goals and, as a result, the price of our common stock may decline.

Our executive offices and laboratory facility are located near known earthquake fault zones, and the occurrence of an earthquake or other catastrophic disaster could cause damage to our facility and equipment, or that of our third-party manufacturers or single-source suppliers, which could materially impair our ability to continue our product development programs.

Our executive offices and laboratory facility are located in the San Francisco Bay Area near known earthquake fault zones and are vulnerable to significant damage from earthquakes. We and the contract manufacturers and our single-source suppliers of raw materials and critical services are also vulnerable to damage from other types of disasters, including fires, storms, floods, power losses and similar events. If such a disaster were to occur, our ability to continue our product development programs or product commercialization activities could be seriously, or potentially completely, impaired. The insurance we maintain may not be adequate to cover our losses resulting from disasters or other business interruptions.

Risks Related to Intellectual Property and Competition

If we are unable to protect our proprietary technology, we may not be able to compete as effectively and our business and financial prospects may be harmed.

Where possible, we seek patent protection for certain aspects of our technology. Patent protection may not be available for some of the drug product candidates we are developing. If we must spend extraordinary time and money protecting our patents, designing around patents held by others or licensing, potentially for large fees, patents or other proprietary rights held by others, our business and financial prospects may be harmed.

The patent positions of biopharmaceutical products are complex and uncertain.

We own or license issued U.S. and foreign patents and pending U.S. and foreign patent applications related to certain of our drug product candidates. However, these patents and patent applications do not ensure the protection of our intellectual property for a number of reasons, including the following:

- We do not know whether our patent applications will result in issued patents. For example, we may not have developed a method for treating a disease before others developed similar methods;
- Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us, or file patent applications before we do. Competitors may also claim that we are infringing on their patents and therefore cannot practice our technology as claimed under our patents, if issued.
- Competitors may also contest our patents, if issued, by showing the patent examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose that patent. As a Company, we have no meaningful experience with competitors interfering with our patents or patent applications;
- Enforcing patents is expensive and may absorb significant management time. Management would spend less time and resources on developing drug product candidates. The processes of defending patents and related intellectual property could increase our operating expenses and delay product programs; and
- Receipt of a patent may not provide practical protection. If we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent.

In addition, competitors also seek patent protection for their technology. Due to the number of patents in our field of technology, we cannot be certain that we do not infringe on those patents or that we will not infringe on patents granted in the future. If a patent holder believes our drug product candidate infringes on its patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe on their technology, we would face a number of issues, including the following:

- Defending a lawsuit takes significant time is typically very expensive;
- If a court decides that our drug product candidate infringes on the competitor's patent, we may have to pay substantial damages for past infringement;
- A court may prohibit us from selling or licensing the drug product candidate unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, we may have to pay substantial royalties or grant cross licenses to our patents; and
- Redesigning our drug product candidates so we do not infringe may not be possible or practical and could require substantial funds and time.

It is also unclear whether our trade secrets are adequately protected. While we use reasonable efforts to protect our trade secrets, our employees or consultants may unintentionally or willfully disclose our information to competitors. Enforcing a claim that someone else illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the U.S. are sometimes less willing to protect trade secrets. Our competitors may independently develop equivalent knowledge, methods and know-how. We may also support and collaborate in research conducted by government organizations, hospitals, universities or other educational institutions. These research partners may be unwilling to grant us any exclusive rights to technology or products derived from these collaborations prior to entering into the relationship. If we do not obtain required licenses or rights, we could encounter delays in our product development efforts while we attempt to design around other patents or even be prohibited from developing, manufacturing or selling drug product candidates requiring these licenses. There is also a risk that disputes may arise as to the rights to technology or drug product candidates developed in collaboration with other parties.

If we are limited in our ability to utilize acquired or licensed technologies, we may be unable to develop, out-license, market and sell our product candidates, which could prevent new product introductions and/or cause delayed new product introductions.

We have acquired and licensed certain proprietary technologies and plan to further license and acquire various patents and proprietary technologies owned by other parties. The agreements in place are critical to our product development programs. These agreements may be terminated, and all rights to the technologies and product candidates will be lost, if we fail to perform our obligations under these agreements and licenses in accordance with their terms including, but not limited to, our ability to fund all payments due under such agreements. Our inability to continue to maintain these technologies could adversely affect our business, financial condition and results of operations. In addition, our business strategy depends on the successful development of licensed and acquired technologies into commercial products, and, therefore, any limitations on our ability to utilize these technologies may impair our ability to develop, out-license or market and sell our product candidates, delay new product introductions, and/or adversely affect our reputation, any of which could have a material adverse effect on our business, financial condition and results of operations.

If the licensing agreements we entered into are terminated, we will lose the right to use or exploit our owned and licensed technologies.

We entered into a licensing agreement with UCSD for patents and know-how related to PROCYSBI and RP103 and a licensing agreement with Yeda Research and Development Company Limited, or Yeda, for patents originating from Weizmann Institute of Technology and Niigata University, related to use of transglutaminase inhibitors to treat

neurological diseases.

UCSD and Yeda may terminate their respective agreements with us upon the occurrence of certain events, including if we challenge the validity of any patents licensed under the respective agreements or if we materially breach our obligations to make certain payments and meet certain diligence milestones within specified time periods, and fail to remedy the breach within the permitted cure periods. Yeda may also terminate its agreement with us if we enter into certain liquidation proceedings. Although we are not currently in breach of these agreements, challenging any patents licensed under these agreements or involved in any liquidation proceedings, there is a risk that we may be in the future, giving UCSD and/or Yeda the right to terminate their respective agreements with us. We have the right to terminate these agreements at any time by giving prior written notice. If the UCSD or Yeda agreements are terminated by either party, we would lose certain of our rights relating to PROCYSBI and RP103 in the case of UCSD and would lose our rights to the Weizmann and Niigata patents in the case of Yeda. Under such circumstances, we would have no further right to use or exploit the patents, know-how and other intellectual property rights relating to those respective technologies. If this happens, we would be required to discontinue sales of PROCYSBI, we will have to delay or terminate some or all of our research and development programs, our financial condition and results of operations will be adversely affected, and we may have to cease our operations.

If our competitors succeed in developing products and technologies that are more effective than our own, or if scientific developments change our understanding of the potential scope and utility of our drug product candidates, then our technologies and future drug product candidates may be rendered less competitive.

We face significant competition from industry participants that are pursuing similar technologies that we are pursuing and are developing pharmaceutical products that are competitive with PROCYSBI or our drug product candidates. All of our large pharmaceutical competitors have greater capital resources, larger overall research and development staffs and facilities, and a longer history in drug discovery, development, regulatory approval, manufacturing and marketing than we do. With these additional resources and experience, our competitors may be able to respond to rapid and significant technological changes in the biotechnology and pharmaceutical industries faster than we can.

We expect that the technologies associated with biotechnology research and development will continue to develop rapidly. Our future success will depend in large part on our ability to maintain a competitive position with respect to these technologies. Rapid technological development, as well as new scientific developments, may result in our compounds, drug products, drug product candidates or processes becoming obsolete before we can recover any or all of the expenses incurred to develop them. For example, changes in our understanding of the appropriate population of patients who should be treated with a targeted therapy like we are developing may limit the drug's market potential if it is subsequently demonstrated that only certain subsets of patients should be treated with the targeted therapy.

If our agreements with employees, consultants, advisors, suppliers and corporate partners fail to protect our intellectual property, proprietary information or trade secrets, it could have a significant adverse effect on us.

We have taken steps to protect our intellectual property and proprietary technology, by entering into confidentiality agreements and intellectual property assignment agreements with our employees, consultants, advisors and corporate and educational institution partners. Such agreements may not be enforceable or may not provide meaningful protection for our trade secrets or other proprietary information in the event of unauthorized use or disclosure or other breaches of the agreements, and we may not be able to prevent such unauthorized disclosure. Monitoring unauthorized disclosure is difficult, and we do not know whether the steps we have taken to prevent such disclosure are, or will be, adequate. Furthermore, the laws of some foreign countries may not protect our intellectual property rights to the same extent as do the laws of the U.S.

Risks Related to Our Financial Position and Capital Requirements

Our commercialization efforts and clinical development programs will require substantial future funding which will impact our operational and financial condition.

Excluding PROCYSBI for cystinosis, it will take several years before we are able to develop our other drug product candidates into marketable drug products, if at all. The marketing and sales effort of PROCYSBI and our future approved products, our ability to gain adequate reimbursement, once products are approved for sale, and our product development programs will require substantial additional capital to successfully complete them, arising from costs to:

- conduct research, preclinical testing and human studies and clinical trials;
- establish or contract for pilot scale and commercial scale manufacturing processes and facilities;
- market and distribute PROCYSBI and our future approved products; and
- establish and develop quality control, manufacturing, regulatory, medical, distribution, marketing, sales, finance and administrative capabilities to support these programs.

Our future operating and capital needs will depend on many factors, including:

- the effectiveness of our commercialization activities;
- the scope and results of preclinical testing and human clinical trials;
- the pace of scientific progress in our research and development programs and the magnitude of these programs;
- our ability to obtain, and the time and costs involved in obtaining, regulatory approvals;
- the cost of manufacturing scale-up for new product candidates;
- our ability to prosecute, maintain and enforce, and the time and costs involved in preparing, filing, prosecuting, maintaining and enforcing, patent claims;
- competing technological and market developments;
- our ability to establish additional collaborations; and
- changes in our existing collaborations.

We base our outlook regarding the need for funds on many uncertain variables. Such uncertainties include the success of our commercial sales of PROCYSBI in the U.S. and EU, our efforts to commercialize our future approved products, the success of our research initiatives, regulatory approvals, the timing of events outside our direct control, such as negotiations with healthcare payors, potential strategic partners and other factors. In addition, certain product programs may require collaborative agreements with corporate partners with substantial assets and organizations to help with the very substantial funds required and the complex organizational resources required. Such agreements may require substantial time to complete and may not be available in the time frame desired or with acceptable financial terms, if at all. Any of these uncertain events can significantly change our cash requirements as they determine such one-time events as the receipt or payment of major milestones and other payments.

Significant additional funds from outside financing sources will be required to support our operations and if we are unable to obtain them on acceptable terms, we may be required to cease or reduce further development of PROCYSBI and our other drug product programs, to sell some or all of our technology or assets, to merge with another entity or to cease operations.

If we fail to obtain the capital necessary to fund our operations, our operational and financial results will be adversely affected.

As of September 30, 2013, we had an accumulated deficit of approximately $193.3 million. We expect to continue to incur losses for the foreseeable future and must obtain significant financing to fund our planned operations. Our recurring losses from operations to date 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 consolidated financial statements for the transitional four-month period ended December 31, 2012, with respect to this uncertainty. We will need to raise additional capital and/or generate significant revenue at profitable levels to continue to operate as a going concern. In addition, the perception that we may not be able to continue as a going concern may cause third parties to choose not to do business with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

In the future, we may need to sell equity or debt securities to raise additional funds to support, among other things, our development and commercialization programs. The sale of additional equity securities or convertible debt securities will result in additional dilution to our stockholders. Additional financing may not be available on a timely basis, in amounts or on terms satisfactory to us, or at all. We may be unable to raise additional capital due to a variety of factors, including our financial condition, the status of our research and development programs, the status of regulatory reviews for marketing approvals, the status of our commercialization activities, sales of PROCYSBI in the U.S., the execution of our launch of PROCYSBI in the EU, and the general condition of the financial markets. If we fail to raise additional financing when needed, we may have to delay or terminate some or all of our research and development programs, scale back our operations and/or reduce our commercial expenses for PROCYSBI. If such actions are required, our business, financial condition and results of operations will be adversely affected and our prospects may be significantly reduced.

Our cash flows and capital resources may be insufficient to make required payments on our indebtedness.

The required payments of principal and interest on our indebtedness under the HC Royalty Loan may require a substantial portion, or all, of our available cash to be dedicated to the service of these debt obligations. The loan bears interest at an annual fixed rate of 10.75% and a synthetic royalty based on the amount of PROCYSBI and other future approved product net revenues in a calendar year, and such royalty is payable quarterly. Principal payments under the HC Royalty Loan will become due beginning on the ninth quarterly payment date occurring after the date the second $25.0 million tranche was funded, or June 2015.

There is no assurance that our business will generate sufficient cash flow or that we will have capital resources in an amount sufficient to enable us to pay our indebtedness to HC Royalty. If our cash flows and capital resources are insufficient to fund these debt service obligations, we may be forced to reduce or delay product development, sales and marketing, and capital and other expenditures and we may be forced to restructure our indebtedness or raise additional capital through the issuance of equity or debt instruments. We cannot ensure that we will be able to refinance any of our indebtedness or raise additional capital on a timely basis, in sufficient amounts, on satisfactory terms or at all. In addition, the terms of the HC Royalty Loan may limit our ability to pursue any of these financing alternatives and these alternatives may not enable us to meet our scheduled debt service obligations. Failure to meet our debt service obligations may result in an event of default under the HC Royalty Loan, which would permit the lender to accelerate the payment of all of our indebtedness to HC Royalty and interest thereon, take possession of, foreclose on, sell, assign or grant a license to use, our pledged collateral and assign and transfer the pledged stock of our subsidiaries. This could have a material adverse impact on our financial condition and results of operations.

Risks Related to Our Common Stock

A substantial number of shares of our common stock are eligible for future sale in the public market, and the issuance or sale of equity, convertible or exchangeable securities in the market, or the perception of such future sales or issuances, could lead to a decline in the trading price of our common stock.

Any issuance of equity, convertible or exchangeable securities, including for the purposes of raising capital to fund our operations, financing acquisitions and the expansion of our business, will have a dilutive effect on our existing stockholders. In addition, the perceived market risk associated with the possible issuance of a large number of shares of our common stock or securities convertible or exchangeable into a large number of shares of our common stock could cause some of our stockholders to sell their common stock, thus causing the trading price of our common stock to decline. Subsequent sales of our common stock in the open market or the private placement of our common stock or securities convertible or exchangeable into our common stock could also have an adverse effect on the trading price of our common stock. If our common stock price declines, it will be more difficult for us to raise additional capital or we may be unable to raise additional capital at all.

We have entered into an Amended and Restated Sales Agreement with Cowen and Company, which, if utilized further, will create substantial dilution for our existing stockholders. The original Sales Agreement provided for at-the-market sales of our common stock with aggregate gross proceeds of up to $40,000,000. On July 3, 2013, we entered into an Amended and Restated Sales Agreement to increase the aggregate gross sales proceeds that may be raised pursuant to the agreement to $100,000,000 (of which approximately $38.3 million was already sold pursuant to the original Sales Agreement dated April 30, 2012). Sales in the at-the-market offering are being made pursuant to our prospectus supplement dated April 30, 2012, as amended by Amendment No. 2 dated July 3, 2013, which supplements our prospectus dated February 3, 2012, filed as part of the shelf registration statement that was declared effective by the SEC on February 3, 2012. As of October 15, 2013, an aggregate of approximately $46.2 million remained available for future sales of our common stock under the Amended and Restated Sales Agreement.

In connection with other collaborations, joint ventures, license agreements or future financings that we may enter into in the future, we may issue additional shares of common stock or other equity securities, and the value of the securities issued may be substantial and create additional dilution to our existing and future common stockholders.

Because we do not intend to pay any cash dividends on our common stock, investors will benefit from an investment in our common stock only if it appreciates in value. Investors seeking dividend income should not purchase shares of our common stock.

We have not declared or paid any cash dividends on our common stock since our inception. We anticipate that we will retain our future earnings, if any, to support our operations and to finance the growth and development of our business and do not expect to pay cash dividends in the foreseeable future. As a result, the success of an investment in our common stock will depend upon any future appreciation in the value of our common stock. There is no guarantee that our common stock will appreciate in value or even maintain its current price. Investors seeking dividend income should not invest in our common stock.

Our stock price is volatile, which could result in substantial losses over short periods of time for our stockholders. The trading volume in our common stock may be relatively small.

Our common stock is quoted on the NASDAQ Global Market. The trading price of our common stock has been and may continue to be volatile. Our operating performance, both financial and in the development of approved products, affects and will continue to significantly affect the market price of our common stock. We face a number of risks including those described in this Risk Factors section, which may negatively impact the price of our common stock.

The market price of our common stock also may be adversely impacted by broad market and industry fluctuations including general economic and technology trends, regardless of our operating performance. The NASDAQ Global Market has, from time to time, experienced extreme price and trading volume fluctuations, and the market prices of biopharmaceutical companies such as ours have been extremely volatile. Market prices for securities of pharmaceutical, biotechnology and other life sciences companies in a comparable stage to us have historically been particularly volatile and trading volume in such securities has often been relatively small. Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. The stock market also has periods during which industry segments, such as biotechnology, are in volatile swings of greater or lesser favor as investments. These swings in the investment in a sector (periods of net sales or purchases of equity securities) will directly affect the stock prices of many companies in the sector and, in particular, those companies that do not have conventional measures of financial and business health such as sales, earnings, growth rates, profitability and other measures.

These broad market fluctuations, during which our stage of company and our industry may experience a stronger degree of market sensitivity, will adversely affect the trading price of our common stock. In the past, following periods of volatility in the market resulting in substantial price declines of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation can result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

We can issue shares of preferred stock that may adversely affect the rights of a stockholder of our common stock.

Our certificate of incorporation authorizes us to issue up to 15,000,000 shares of preferred stock with designations, rights and preferences determined from time-to-time by our board of directors. Accordingly, our board of directors is empowered, without stockholder approval, to issue preferred stock with dividend, liquidation, conversion, voting or other rights superior to those of stockholders of our common stock.

Anti-takeover provisions under Delaware law, in our stockholder rights plan and in our certificate of incorporation and bylaws may prevent or complicate attempts by stockholders to change the board of directors or current management and could make a third-party acquisition of us difficult.

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law as currently in effect may make a change in control of our Company more difficult, even if a change in control may be beneficial to the stockholders. Our board of directors has the authority to issue up to 15,000,000 shares of preferred stock, none of which are issued or outstanding. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Our certificate of incorporation contains provisions that may enable our management to resist an unwelcome takeover attempt by a third party, including: a prohibition on actions by written consent of our stockholders; the fact that stockholder meetings must be called by our board of directors; and provisions requiring stockholders to provide advance notice of proposals. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, the board of directors approves the transaction. Our board of directors may use these provisions to prevent changes in the management and control of our Company. Also, under applicable Delaware law, our board of directors may adopt additional anti-takeover measures in the future.

We are a party to a stockholder rights plan, also referred to as a poison pill, which is intended to deter a hostile takeover of us by making such proposed acquisition more expensive and less desirable to the potential acquirer. The stockholder rights plan and our certificate of incorporation and bylaws, as amended, contain provisions that may discourage, delay or prevent a merger, acquisition or other change in control that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. These provisions could limit the price that investors might be willing to pay in the future for shares of our common stock.

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

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

None.

ITEM 5. OTHER INFORMATION

Beginning in fiscal 2006, we engaged Burr Pilger Mayer, Inc. ("BPM") as our independent registered public accounting firm. On November 4, 2013, BPM notified us that it had become aware of a violation of the independence standards of the SEC and BPM's internal policies arising from unauthorized purchases by a BPM employee of shares of our common stock. During the transition period of September 1, 2012 to December 31, 2012, the fiscal quarter ended March 31, 2013, and the fiscal quarter ended June 30, 2013, the employee executed transactions in our common stock and provided services to us related to our European corporate structure and the potential tax implications of operations outside the US but did not render audit services.

BPM has informed the Company that, based upon BPM's review of the facts and circumstances related to the now former employee's conduct, it has determined that BPM's objectivity and integrity with respect to the Company's audits were unaffected and that BPM's independence with respect to the audits remained unimpaired. As a result,

BPM is of the opinion that it remains independent. In accordance with the rules of the Public Company Accounting Oversight Board (PCAOB), BPM has confirmed to us in writing that the former employee's conduct did not impair BPM's independence with respect to the Company in accordance with the requirements of the SEC and the PCAOB. In discussions with the audit committee of our board of directors, BPM stated that, based upon the results of its internal review, it had concluded that the former employee did not provide audit services to the Company and the audit engagement team did not have any knowledge of the former employee's unauthorized trading activities specifically prohibited by BPM's policies and procedures.

Based upon BPM's conclusions and its representations to the Company, the audit committee has affirmed BPM's conclusions and noted that it has been informed by Company management that (i) they did not consult the former employee on the audit process or accounting treatment of any items appearing on the Company's financial statements and (ii) they were not aware that the former employee had engaged in prohibited trading activities.

Based upon the report by BPM and discussions with BPM, our audit committee has affirmed BPM's conclusion that, notwithstanding the actions of the former employee resulting in the violation of the SEC's auditor independence rule, BPM's capacity for objective and impartial judgment remains unimpaired and a reasonable investor would conclude that BPM's independence with respect to the Company has not been impaired. BPM has had preliminary conversations regarding this matter with, and BPM and the Company will be furnishing a detailed written analysis of this matter to, the Office of the Chief Accountant of the SEC for their review.

ITEM 6. EXHIBITS

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

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.

RAPTOR PHARMACEUTICAL CORP.

Date: November 7, 2013 By: /s/ Christopher M. Starr
Christopher M. Starr, Ph.D.
Chief Executive Officer
(Principal Executive Officer)

Date: November 7, 2013 By: /s/ Georgia Erbez
Georgia Erbez
Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit Index

Exhibit Number	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
2.1	Agreement and Plan of Merger and Reorganization, dated as of June 7, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc.	S-4 (333-136018)	07/25/2006	Annex A	
2.2	Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated as of August 25, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc.	S-4/A (333-136018)	08/25/2006	Annex A	
2.3	Agreement and Plan of Merger and Reorganization, dated July 27, 2009, by and among Raptor Pharmaceuticals Corp., TorreyPines Therapeutics, Inc., a Delaware corporation, and ECP Acquisition, Inc., a Delaware corporation	8-K	07/28/2009	2.3	
3.1	Certificate of Incorporation of Registrant	8-K	10/10/2006	3.1	
3.2(a)	Bylaws of Registrant	8-K	10/10/2006	3.2	
3.2(b)	Amendment to Bylaws of Registrant	10-K	03/29/2007	3.6	
3.2(c)	Amendment to Bylaws of the Registrant	8-K	05/14/2012	3.2	
3.3	Certificate of Amendment filed with the Secretary of State of the State of Nevada effecting an 8-for-1 reverse stock of Registrant's common stock and changing the name of Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc.	8-K	10/10/2006	3.3	
3.4	Articles of Conversion filed with the Secretary of State of the State of Nevada changing the state of incorporation of Registrant	8-K	10/10/2006	3.4	
3.5	Certificate of Conversion filed with the Secretary of State of the State of Delaware	8-K	10/10/2006	3.5	
3.6	Certificate of Amendment of Certificate of Incorporation of Registrant	8-K	10/05/2009	3.1	
3.7	Certificate of Merger between Raptor Pharmaceuticals Corp., ECP Acquisition, Inc. and TorreyPines Therapeutics, Inc.	8-K	10/09/2009	3.2	
4.1	Specimen common stock certificate of the Registrant	8-K	10/09/2009	4.7	
4.2(a)	Rights Agreement, dated as of May 13, 2005, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	8-K	05/16/2005	99.2	
4.2(b)	Amendment to Rights Agreement, dated as of June 7, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	8-K	06/12/2006	4.1	
4.2(c)	Amendment to Rights Agreement, dated as of October 3, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	10-K	03/29/2007	4.19	
4.2(d)	Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between Registrant and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company)	8-K	07/28/2009	2.3	

4.2(e)	Amendment to Rights Agreement, dated August 6, 2010, by and between Registrant and American Stock Transfer & Trust Company, LLC	8-K	08/10/2010	4.2
4.3	Form of Warrant issued to Oxford Financial and Silicon Valley Bank on September 27, 2005	10-K	03/29/2007	4.16
4.4	Form of Warrant issued to Comerica Bank on June 11, 2008	8-K	06/17/2008	4.1

4.5(a)*	Warrant to purchase common stock dated December 14, 2007 issued to Flower Ventures, LLC	10-QSB/A	04/15/2008	4.1	
4.5(b)*	Warrant Agreement Amendment, dated December 17, 2009, between the Registrant and Flower Ventures, LLC	10-QSB	04/09/2010	4.15	
4.6*	Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp.	8-K/A	05/28/2008	4.2	
4.7*	Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp.	8-K	08/25/2009	4.2	
4.8*	Form of Investor Warrants	8-K	12/18/2009	4.1	
4.9*	Form of Investor Warrants	8-K	08/10/2010	4.1	
4.8*	Placement Agent Warrant	8-K	08/13/2010	4.2	
10.1	2013 Plan Amendment to the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan	8-K	07/25/13	10.1	
31.1	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer				X
31.2	Certification of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer				X
32.1	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer, and Georgia Erbez, Chief Financial Officer, Secretary and Treasurer				X
101	The following materials from the Raptor Pharmaceutical Corp. Quarterly Report on Form 10-Q for the quarter ended September 30, 2013, formatted in Extensible Business Reporting Language (XBRL): (i) the Condensed Consolidated Balance Sheets; (ii) the Condensed Consolidated Statements of Comprehensive Loss; (iii) the Condensed Consolidated Statement of Stockholders' Equity; (iv) the Condensed Consolidated Statements of Cash Flows; and (v) related notes, tagged as blocks of text.				X

* The Raptor Pharmaceuticals Corp. warrants denoted by an asterisk have been converted into warrants of the Registrant and the exercise price of such warrants and number of shares of common stock issuable thereunder have been converted as described in Item 1.01 (under the section titled, "Background") of the Registrant's Current Report on Form 8-K, filed on October 5, 2009.