Raptor Pharmaceutical Corp
Form 10-Q
January 09, 2013
Table of Contents

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

FORM 10-Q

x QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended November 30, 2012
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.)

9 Commercial Blvd., Suite 200, Novato, CA 94949
(Address of principal executive offices) (Zip Code)

(415) 382-8111
(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 x No ¨
Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x 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 x
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 x

There were 52,424,649 shares of the registrant's common stock, par value $0.001, outstanding as of December 31, 2012.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
FORM 10-Q FOR THE QUARTER ENDED NOVEMBER 30, 2012
Table of Contents

Table of Contents

PART I - FINANCIAL INFORMATION

Item 1. Financial Statements.

Raptor Pharmaceutical Corp.
(A Development Stage Company)
Condensed Consolidated Balance Sheets
(In thousands, except per share data, or unless otherwise specified)

	November 30, 2012 (unaudited)	August 31, 2012 (1)
ASSETS		
Current assets:		
Cash and cash equivalents	$13,426	$23,580
Restricted cash	163	169
Short-term investments	22,056	15,307
Prepaid expenses and other	2,688	3,111
Total current assets	38,333	42,167
Intangible assets, net	2,168	2,205
Goodwill	3,275	3,275
Fixed assets, net	397	403
Deposits	26	105
Deferred offering costs	116	134
Debt issuance costs	161	0
Total assets	$44,476	$48,289
LIABILITIES AND STOCKHOLDERS' EQUITY		
Liabilities		
Current liabilities:		
Accounts payable	$1,846	$1,601
Accrued liabilities	2,972	2,652
Common stock warrant liability	16,238	17,266
Deferred rent	8	14
Capital lease liability – current	8	8
Total current liabilities	21,072	21,541
Capital lease liability - long-term	11	13
Total liabilities	21,083	21,554
Commitments and contingencies – see Note 7		
Stockholders' equity:		
Preferred stock, $0.001 par value per share, 15,000 shares authorized, zero shares issued and outstanding	0	0
Common stock, $0.001 par value per share, 150,000 shares authorized 52,098 and 50,568 shares issued and outstanding as at November 30, 2012 and August 31, 2012, respectively	52	51
Additional paid-in capital	153,454	143,380
Accumulated other comprehensive loss	(68)	(50)

Deficit accumulated during development stage	(130,045)	(116,646)
Total stockholders' equity	23,393	26,735
Total liabilities and stockholders' equity	$44,476	$48,289

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

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

Table of Contents

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

	For the three months ended November 30,		For the period from September 8, 2005 (inception) to November 30, 2012
	2012	2011	
Revenues:	$0	$0	$0
Operating expenses:			
General and administrative	6,365	2,336	37,941
Research and development	6,785	5,016	67,466
Total operating expenses	13,150	7,352	105,407
Loss from operations	(13,150)	(7,352)	(105,407)
Interest income	119	64	832
Interest expense	0	(1)	(119)
Foreign currency transaction gain	60	59	234
Realized gain on short-term investments	0	0	214
Unrealized loss on short-term investments	(64)	(35)	(66)
Adjustment to fair value of common stock warrants	(364)	(4,168)	(25,733)
Net loss	(13,399)	(11,433)	(130,045)
Other comprehensive loss			
Foreign currency translation adjustment	(18)	(8)	(68)
Comprehensive loss	$(13,417)	$(11,441)	$(130,113)
Net loss per share:			
Basic and diluted	$(0.26)	$(0.25)	
Weighted-average shares outstanding used to compute:			
Basic and diluted	51,557	45,623	

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

Table of Contents

Raptor Pharmaceutical Corp.
(A Development Stage Company)
Condensed Consolidated Statement of Stockholders' Equity
For the Three Months Ended November 30, 2012
(Unaudited)
(In thousands, except per share data, or unless otherwise specified)

	Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Deficit accumulated during development stage	Total
	Shares	Amount				
Balance at August 31, 2012	50,568	$ 51	$ 143,380	$ (50)	$ (116,646)	$ 26,735
Exercise of common stock warrants	327	0	956	0	0	956
Exercise of common stock options	50	0	127	0	0	127
Employee stock-based compensation expense	0	0	1,651	0	0	1,651
Consultant stock-based compensation expense	0	0	7	0	0	7
Reclassification of the fair value of warrant liabilities upon exercise	0	0	1,392	0	0	1,392
Issuance of common stock under an at-the-market sales agreement, net of commissions totaling $214	1,153	1	5,941	0	0	5,942
Foreign currency translation loss	0	0	0	(18)	0	(18)
Net loss	0	0	0	0	(13,399)	(13,399)
Balance at November 30, 2012	52,098	$ 52	$ 153,454	$ (68)	$ (130,045)	$ 23,393

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

Table of Contents

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

	For the three months ended November 30,		For the cumulative period from September 8, 2005 (inception) to November 30,
	2012	2011	2012
Cash flows from operating activities:			
Net loss	$(13,399)	$(11,433)	$(130,045)
Adjustments to reconcile net loss to net cash used in operating activities:			
Employee stock-based compensation exp.	1,651	917	9,489
Consultant stock-based compensation exp.	7	0	762
Fair value adjustment of common stock warrants	364	4,168	25,733
Amortization of intangible assets	37	36	733
Depreciation of fixed assets	32	9	598
Unrealized gain/(loss) on short-term investments	64	(18)	64
Write-off of intangible assets and other intellectual property	0	0	349
Amortization of capitalized finder's fee	0	0	102
Capitalized acquisition costs previously expensed	0	0	38
Changes in assets and liabilities:			
Prepaid expenses and other	423	86	(2,588)
Intangible assets	0	0	750
Deposits	79	0	(26)
Accounts payable	245	1,418	1,845
Accrued liabilities	320	(591)	2,290
Deferred rent	(6)	(3)	8
Net cash used in operating activities	(10,183)	(5,411)	(89,898)
Cash flows from investing activities:			
Purchase of fixed assets	(26)	(2)	(937)
Cash acquired in 2009 Merger	0	0	581
Change in restricted cash	6	1	(163)
Purchase of short-term investments	(6,813)	(30,000)	(52,120)
Sale of short-term investments	0	0	30,000
Net cash used in investing activities	(6,833)	(30,001)	(22,639)
Cash flows from financing activities:			

Proceeds from the sale of common stock	0	46,000	85,941
Proceeds from the sale of common stock under an equity line	0	0	11,640
Proceeds from the sale of common stock under an ATM agreement	6,156	0	13,843
Proceeds from the exercise of common stock warrants	956	1,957	21,867
Proceeds from the exercise of common stock options	127	39	662
Fundraising costs	(214)	(3,014)	(8,069)
Debt issuance costs	(161)	0	(161)
Deferred offering costs	18	0	36
Proceeds from the sale of common stock to initial investors	0	0	310
Proceeds from bridge loan	0	0	200
Repayment of bridge loan	0	0	(200)
Additions and payments on capital lease	(2)	(1)	(38)
Net cash provided by financing activities	6,880	44,981	126,031
Effect of exchange rates on cash and cash equivalents	(18)	(9)	(68)
Net increase (decrease) in cash and cash equivalents	(10,154)	9,560	13,426
Cash and cash equivalents, beginning of period	23,580	15,172	0
Cash and cash equivalents, end of period	$13,426	$24,732	$13,426

Table of Contents

Supplemental cash flow information:			
Interest paid	$0	$0	$17
Supplemental disclosure of non-cash financing activities:			
Warrants issued in connection with financing	$0	$0	$16,310
Initial fair value of warrants issued to placement agents in connection with financings	$0	$0	$209
Common stock and warrants issued in connection with reverse merger	$0	$0	$4,417
Common stock issued as fee for equity line	$0	$0	$828
Fair value of warrant liability reclassified to equity upon exercise	$1,392	$2,376	$19,380
Acquisition of equipment in exchange for capital lease	$0	$0	$48
Notes receivable issued in exchange for common stock	$0	$0	$110
Common stock issued for a finder's fee	$0	$0	$102
Common stock issued in asset purchase	$0	$0	$2,899

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

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

1. DESCRIPTION OF BUSINESS AND SIGNIFICANT ACCOUNTING POLICIES

Raptor Pharmaceutical Corp. (the "Company" or "Raptor"), a publicly-traded biotechnology company, seeks to research, manufacture, and commercialize medicines that improve life for patients with severe, rare disorders. Raptor currently has product candidates in pre-commercial and clinical development designed to potentially treat nephropathic cystinosis ("cystinosis"), Non-alcoholic Steatohepatitis ("NASH"), Huntington's Disease ("HD") and aldehyde dehydrogenase ("ALDH2") deficiency. Raptor's preclinical programs are based upon bioengineered novel drug candidates and drug-targeting platforms derived from the human receptor-associated protein and related proteins that are designed to target cancer and infectious diseases.

Basis of Presentation

The accompanying condensed consolidated financial statements reflect the results of operations of Raptor and have been prepared in accordance with the accounting principles generally accepted in the United States of America ("GAAP"). This Form 10-Q should be read in conjunction with the audited financial statements and accompanying notes in the Company's Annual Report on Form 10-K for the year ended August 31, 2012 ("2012 Form 10-K"). The Company's fiscal year end is August 31. On December 4, 2012, Raptor's board of directors approved a change in the Company's fiscal year end from August 31 to December 31. As a result of this change, the Company will file a transition report on Form 10-KT for the four-month period ended December 31, 2012. The Company expects to file the transition report on or before March 18, 2013.

The Company's condensed consolidated financial statements include the accounts of the Company's direct and indirect wholly owned subsidiaries. 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 November 30, 2012, the Company had accumulated losses of $130,045. Management expects to incur further losses for the foreseeable future. Management believes that the Company's cash, cash equivalents and short-term investments as of December 31, 2012 of approximately $58,000 (which includes $23,414 of proceeds, net of fees and commissions, received in December 2012 under a $50,000 loan agreement with HealthCare Royalty Partners ("HC Royalty")) will be sufficient to meet the Company's operating requirements and obligations into the fourth calendar quarter of 2013.

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.

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. and European banks as collateral for credit cards.

Short-term Investments

The Company invests in short-term investments in high credit-quality funds in order to obtain higher yields on its cash available for investment. Short-term investments consisted of:

	November 30, 2012	August 31,

		2012
Short-term duration government fund	$ 22,056	$15,307
Total short-term investments	$ 22,056	$15,307

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

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 as of November 30, 2012 and August 31, 2012. The investments are placed in financial institutions with strong credit ratings and management expects full recovery of the carrying amounts.

Functional Currency

The Company's consolidated functional currency is the U.S. dollar. Raptor Pharmaceuticals Europe B.V. ("BV") and RPTP European Holdings C.V. ("CV"), the Company's European subsidiary and Cayman-based subsidiary, respectively, use the European Euro as their functional currency. At each quarter end, BV'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 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 November 30 and August 31, 2012 are summarized as follows:

Assets	Level 1	Level 2	Level 3	November 30, 2012
Fair value of cash equivalents	$11,597	$0	$0	$ 11,597
Restricted cash	0	163	0	163
Short-term investments	22,056	0	0	22,056
Total	$33,653	$163	$0	$ 33,816
Liabilities				
Fair value of common stock warrants	$0	$0	$16,238	$ 16,238

Total	$0	$0	$16,238	$ 16,238

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

Assets	Level 1	Level 2	Level 3	August 31, 2012
Fair value of cash equivalents	$13,162	$0	$0	$13,162
Restricted cash	0	169	0	169
Short-term investments	15,307	0	0	15,307
Total	$28,469	$169	$0	$28,638
Liabilities				
Fair value of common stock warrants	$0	$0	$17,266	$17,266
Total	$0	$0	$17,266	$17,266

Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine (currently developed as RP103 and RP104) and to an out-license acquired in the 2009 Merger. The intangible assets related to RP103/RP104 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. 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.

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. An impairment analysis is performed, and if necessary, a resulting write-down in valuation is recorded.

Fixed Assets

Fixed assets, which mainly consist of leasehold improvements, 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.

As of August 31, 2012, the Company determined that the capitalized acquired in-process research and development cost of $900, representing the tezampanel and NGX 426 program acquired in its 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. As such, the Company expensed $900 as in-process research and development as part of research and development expense on its consolidated statements of comprehensive loss for the year ended August 31, 2012. During the three month period ended November 30, 2012, the Company did not identify any impairment losses.

10

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

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 the Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("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.

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 tax rate is 0% for income tax for the three month period ended November 30, 2012 and the Company expects that its effective tax rate fiscal year ended August 31, 2012 and the short calendar year from September 1, 2012 to December 31, 2012 will be 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.

On September 1, 2009, the Company adopted the provisions of ASC No. 740-10, Accounting for Uncertainty in Income Taxes ("ASC 740-10"). ASC 740-10 requires entities following GAAP to identify uncertain tax positions and disclose any potential tax liability on their financial statements using a two-step process, which includes recognition and measurement.

The Company's continuing practice is to recognize interest and/or penalties related to income tax matters as a component of income tax expense. As of November 30, 2012, there were no accrued interest or penalties related to

uncertain tax positions.

The Company files U.S. Federal, California, Georgia and North Carolina state income tax returns and Dutch income tax returns. The Company is currently not subject to any income tax examinations. Due to the Company's NOLs, generally all tax years remain open.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

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:

	November 30,	
	2012	2011
Warrants to purchase common stock	4,861	6,332
Options to purchase common stock	7,741	5,683
Total potentially dilutive securities	12,602	12,015

Net loss per share, basic and diluted, was $(0.26) and $(0.25) for the three month periods ended November 30, 2012 and 2011, respectively.

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.

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 6, Stock Option Plans, for further discussion of employee stock-based compensation.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

2. INTANGIBLE ASSETS AND GOODWILL

On December 14, 2007, the Company acquired the intellectual property and other rights to develop RP103/RP104 to treat various clinical indications from the University of California at San Diego ("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 $2,620, primarily based on the value of the Company's common stock and warrants issued to the Encode stockholders. Intangible assets originally recorded as a result of the 2009 Merger were $1,140 of which $900 was written off as of August 31, 2012 as discussed below.

Summary of intangible assets acquired as discussed above:

	November 30, 2012	August 31, 2012
Intangible asset (IP license for RP103/RP104) related to the Encode merger	$ 2,620	$2,620
Intangible assets (out-license) related to the 2009 Merger	240	240
Total intangible assets	2,860	2,860
Less accumulated amortization	(692)	(655)
Intangible assets, net	$ 2,168	$2,205

The intangible assets related to RP103/RP104 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. As of August 31, 2012, the Company determined that the capitalized acquired in-process research and development cost of $900, 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 $900 as in-process research and development as part of research and development expense on the Company's consolidated statements of comprehensive loss for the year ended August 31, 2012.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

During the three month periods ended November 30, 2012 and 2011 and the cumulative period from September 8, 2005 (inception) to November 30, 2012, the Company amortized $37, $36 and $733 (includes $42 related to NeuroTrans™ which was written off as of August 31, 2011), respectively, of intangible assets to research and development expense.

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

Amortization period	Amortization expense
Fiscal year ending August 31, 2013 – estimate	$ 146
Fiscal year ending August 31, 2014 – estimate	146
Fiscal year ending August 31, 2015 – estimate	146
Fiscal year ending August 31, 2016 – estimate	146
Fiscal year ending August 31, 2017 – estimate	146

Goodwill of $3,275 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 its fiscal year ended August 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 year ended August 31, 2012, the tezampanel/NGX426 asset was written off with a carrying value of $900 and during the year ended August 31, 2011, the NeuroTrans™ asset was written off with a carrying value of $108 due to the termination of a collaboration agreement.

3. FIXED ASSETS

Fixed assets consisted of:

Category	November 30, 2012	August 31, 2012	Estimated useful lives
Leasehold improvements	$ 146	$ 146	Shorter of life of asset or lease term
Office furniture	25	3	7 years
Laboratory equipment	594	569	5 years
Computer hardware and software	184	205	3 years
Capital lease equipment	26	26	Shorter of life of asset or lease term
Total at cost	975	949	
Less: accumulated depreciation	(578)	(546)	
Total fixed assets, net	$ 397	$ 403	

Depreciation expense for the three month periods ended November 30, 2012 and 2011 and the cumulative period from September 8, 2005 (inception) to November 30, 2012 was $32, $9 and $598, respectively. Accumulated depreciation on capital lease equipment was $8 and $6 as of November 30, 2012 and August 31, 2012, respectively.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

4. CAPITAL STRUCTURE

Preferred Stock

At November 30, 2012, the Company was authorized to issue 15,000 shares of $0.001 par value per share of preferred stock. There were no shares issued and outstanding. Dividends on the preferred stock will be paid when, and if, declared by the board of directors.

Common Stock

At November 30, 2012, the Company was authorized to issue 150,000 shares of $0.001 par value per share of common stock. Dividends on the common stock will be paid when, and if, declared by the board of directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held.

As of November 30, 2012 and August 31, 2012, there were 52,098 and 50,568 shares, respectively, of the Company's common stock issued and outstanding.

Common Stock Issuance Under At-The-Market ("ATM") Agreement

On April 30, 2012, the Company entered into an "At-the-Market" ("ATM") Sales Agreement, with Cowen and Company, LLC ("Cowen"), under which the Company may, at its discretion, sell its common stock with a sales value of up to a maximum of $40,000 through ATM sales on the NASDAQ Stock Market. Cowen is the sole sales agent for any sales made under the ATM for a 3% commission on gross proceeds. 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.

Sales in the ATM offering are being made pursuant to the prospectus supplement dated April 30, 2012, which supplements the Company's prospectus dated February 3, 2012, filed as part of the shelf registration statement that was declared effective by the SEC on February 3, 2012. Through November 30, 2012, the Company sold 2,660 shares under the ATM at a weighted-average selling price of $5.20 per share for net proceeds of $13,330.

Common Stock Warrants

During the three month period ended November 30, 2012, the Company received $956 from the exercise of warrants in exchange for the issuance of 327 shares of the Company's common stock. During the cumulative period from September 8, 2005 (inception) through November 30, 2012, the Company received $21,867 from the exercise of warrants in exchange for the issuance of an aggregate of 9,241 shares.

The table reflects the number of common stock warrants outstanding as of November 30, 2012:

	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 May / June 2008	433	$ 2.36	5/21/2013
Issued to placement agents in August 2009	65	$ 1.50	8/21/2014
TorreyPines warrants assumed in 2009 Merger	8	$ 80.86	*6/11/2013-9/26/2015
Issued to registered direct investors in Dec. 2009	680	$ 2.45	12/22/2014
Issued to private placement investors in Aug. 2010	3,344	$ 3.075	8/12/2015
Issued to placement agent in Aug. 2010	98	$ 3.075	8/12/2015
Total warrants outstanding	4,861	$ 3.02	*

*Weighted-average exercise price

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

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 warrants issued in the December 2009 and August 2010 equity financings using the following assumptions at November 30, 2012 and August 31, 2012:

	December 2009 equity financing Series A		August 2010 private placement Investors and placement agent	
	November 30, 2012	August 31, 2012	November 30, 2012	August 31, 2012
Fair value ($ millions)	2.6	2.9	13.6	14.4
Black-Scholes inputs:				
Stock price	$5.29	$4.97	$5.29	$4.97
Exercise price	$2.45	$2.45	$3.075	$3.075
Risk free interest rate	0.25%	0.22 %	0.34 %	0.30 %
Volatility	115 %	125 %	115 %	125 %
Expected term (years)	2.00	2.25	2.75	3.00
Dividend	0	0	0	0

Marked-to-Market
For the three month periods ended November 30, 2012 and 2011, and for the cumulative period from September 8, 2005 (inception) to November 30, 2012, 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 losses of $364, $4,168 and $25,733, 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 three month periods ended November 30, 2012 and 2011 (in millions):

	Three month periods ended November 30,	
	2012	2011
Fair value of December 2009 direct offering warrants (including placement agent warrants) at beginning of the fiscal years	$2.9	$5.9
December 2009 direct offering warrants exercised	(0.2)	(0.2)
Adjustment to mark to market common stock warrants	(0.1)	1.0
	2.6	6.7

December 2009 direct offering common stock warrant liability at fair value at November 30, 2012 and 2011		
Fair value of August 2010 private placement warrants (including broker warrants) at beginning of the fiscal years	14.4	17.7
August 2010 private placement warrants exercised	(0.8)	(1.7)
Adjustment to mark to market common stock warrants	0	2.7
August 2010 private placement common stock warrant liability at fair value at November 30, 2012 and 2011	13.6	18.7
Total warrant liability at November 30, 2012 and 2011	$16.2	$25.4

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

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. 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 for which we have estimated based upon the stage of the Company's 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 statement of comprehensive loss as other income or expense, respectively. The primary factors affecting the fair value of the warrant liability are the Company's stock price and volatility. In addition, the Black-Scholes option pricing model requires the input of highly subjective assumptions, and other reasonable assumptions could provide differing results. The Company's reported net loss was $13,399 for the three month period ended November 30, 2012. If the Company's November 30, 2012 closing stock price had been 10% lower, its net loss would have been approximately $1,939 lower. If the Company's November 30, 2012 closing stock price had been 10% higher, its net loss would have been approximately $1,961 higher. If the Company's November 30, 2012 volatility assumption had been 10% lower, its net loss would have been approximately $666 lower. If the Company's November 30, 2012 volatility assumption had been 10% higher, its net loss would have been approximately $622 higher.

5. ACCRUED LIABILITIES

Accrued liabilities consisted of:

	November 30, 2012	August 31, 2012
Clinical trial and related costs	$ 1,049	$841
Pre-commercial and other consulting	504	292
Accrued vacation and employee benefits	392	307
Accrued bonuses	374	746
Legal and patent fees	288	149
Salaries and wages	221	94
Auditing and tax preparation fees	110	108
Other	34	115
Total accrued liabilities	$ 2,972	$2,652

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

6. STOCK OPTION PLANS

During the three month period ended November 30, 2012, the Company received $127 from the exercise of stock options in exchange for the issuance of 50 shares of the Company's common stock. For the cumulative period from September 8, 2005 (inception) through November 30, 2012, the Company received $662 from the exercise of stock options resulting in the issuance of 290 shares of common stock.

Effective September 1, 2006, the Company began recording compensation expense associated with stock options and other forms of equity compensation in accordance with ASC 718. Prior to September 1, 2006, the Company accounted for stock options according to the provisions of APB Opinion No. 25, Accounting for Stock Issued to Employees, and related interpretations, and therefore no related compensation expense was recorded for awards granted with no intrinsic value. The Company adopted the modified prospective transition method provided for under ASC 718, and consequently has not retroactively adjusted results from prior periods. Under this transition method, compensation cost associated with stock options now includes: (i) quarterly amortization related to the remaining unvested portion of all stock option awards granted prior to September 1, 2006, based on the grant date value estimated in accordance with the original provisions of ASC 718; and (ii) quarterly amortization related to all stock option awards granted subsequent to September 1, 2006, based on the grant date fair value estimated in accordance with the provisions of ASC 718. In addition, the Company records consulting expense over the vesting period of stock options granted to consultants. The compensation expense for stock-based compensation awards includes an estimate for forfeitures and is recognized over the requisite service period of the options, which is typically the period over which the options vest, using the straight-line method. Employee stock-based compensation expense for the three month periods ended November 30, 2012 and 2011, respectively, and for the cumulative period from September 8, 2005 (inception) to November 30, 2012 was $1,651, $917 and $9,489, respectively, of which cumulatively $7,563 was included in general and administrative expense and $1,926 was included in research and development expense. No employee stock compensation costs were recognized for the period from September 8, 2005 (inception) to August 31, 2006, which was prior to the Company's adoption of ASC 718.

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
Three months ended November 30, 2011	1.02 %	6 years	121 %
Three months ended November 30, 2012	0.68 %	5 years	95 %

*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.

The Company recognizes as an expense the fair value of options granted to persons who are neither employees nor directors. The fair value of expensed options was based on the Black-Scholes option-pricing model assuming the same factors shown in the stock-based compensation expense table above. Stock-based compensation expense for consultants for the three month periods ended November 30, 2012 and 2011 and for the cumulative period from September 8, 2005 (inception) to November 30, 2012 was $7, zero and $762, respectively, of which cumulatively $147 was included in general and administrative expense and $615 was included in research and development

expense.

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

	Option shares	Weighted-average exercise price	Exercisable	Weighted-average fair value of options granted
Outstanding at August 31, 2012	6,125	$ 5.87	2,995	$ 3.37
Granted	1,666	$ 5.36	0	$ 3.84
Exercised	(50)	$ 2.53	0	$ 1.85
Outstanding at November 30, 2012	7,741	$ 5.78	3,320	$ 3.49

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

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

	Options outstanding and expected to vest November 30,		Options exercisable November 30,	
(In millions)	2012	2011	2012	2011
Intrinsic value	$7.0	$7.2	$5.2	$4.3
Number of options	7.7	5.7	3.3	2.1

There were 2,013 options available for grant as of November 30, 2012 under the 2010 Equity Incentive Plan, as amended (the "Plan"). Plan amendments 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 November 30, 2012, 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 (#)	Weighted-average remaining contractual life (yrs.)	Weighted-average exercise price ($)	Number of options exercisable (#)	Weighted-average exercise price ($)
$0 to $1.00	9	6.38	0.85	6	0.85
$1.01 to $2.00	79	6.55	1.78	74	1.76
$2.01 to $3.00	1,381	5.96	2.65	1,087	2.60
$3.01 to $4.00	1,764	8.03	3.50	1,193	3.52
$4.01 to $5.00	310	7.11	4.79	86	4.71
$5.01 to $6.00	3,807	9.23	5.27	758	5.14

$6.01 to $7.00	277	9.25	6.48	59	6.50
$7.01 to $8.00	70	9.21	7.75	13	7.75
$8.01 to $964.24	44	2.81	249.43	44	249.43
	7,741	8.35	5.78	3,320	6.87

At November 30, 2012, the total unrecognized compensation cost was $15,900. The weighted-average period over which it is expected to be recognized is approximately 3 years.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except per share data, or unless otherwise specified)

7. COMMITMENTS AND CONTINGENCIES

The Company maintained several contracts with drug labelers and distributors, research organizations, contract manufacturers, clinical organizations and clinical sites, primarily to assist with clinical research and clinical and commercial manufacturing for its cystinosis and HD programs and its NASH clinical collaboration. The Company's contractual obligations have not changed significantly during the three month period ended November 30, 2012 compared to its fiscal year ended August 31, 2012.

8. SUBSEQUENT EVENTS

On December 4, 2012, the Company changed its fiscal year end from August 31 to December 31 and it plans to file a transitional Annual Report on Form 10-KT in March 2013 with respect to the short year from September 1, 2012 to December 31, 2012.

On December 20, 2012, the Company signed a $50,000 Loan Agreement with HC Royalty and it had drawn the first tranche of proceeds (net of fees and commissions) of $23,414 under this agreement. The Company would receive an additional $25,000, before expenses, upon receiving U.S. Food and Drug Administration ("FDA") approval of RP103 for the treatment of cystinosis and other customary closing conditions. The loan bears interest at an annual fixed rate of 10.75% and a variable royalty rate, tiered down, based on a percentage of future net product sales. The loan is interest-only for the first two years.

On December 21, 2012, the Company received a notice from the FDA stating that the FDA would require additional time to complete its review of the Company's New Drug Application for RP103 for the potential treatment of cystinosis and that the initial Prescription Drug User Fee Act goal date has been extended from January 30, 2013 to April 30, 2013. No additional studies were requested by the FDA.

On December 28, 2012, the Company's wholly-owned operating subsidiaries, Raptor Therapeutics Inc. and Raptor Discoveries Inc. merged, with Raptor Therapeutics Inc. remaining as the surviving operating company. Concurrently, Raptor Therapeutics Inc. changed its name to Raptor Pharmaceuticals Inc.

Table of Contents

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

(In thousands, except per share data, or unless otherwise specified)

You should read the following discussion in conjunction with our condensed consolidated financial statements as of November 30, 2012, 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. and its direct and indirect wholly-owned subsidiaries, Raptor Pharmaceuticals Corp. (which was merged into us as of December 7, 2011), Raptor Discoveries Inc. (which was merged into Raptor Therapeutics Inc. as of December 28, 2012), or Raptor Discoveries, Raptor Therapeutics Inc. (which changed its name to Raptor Pharmaceuticals Inc. as of December 28, 2012), or Raptor Pharmaceuticals, Raptor European Products, LLC, RPTP European Holdings C.V. and Raptor Pharmaceuticals Europe B.V.

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, that plan for or anticipate the future. 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 facts, including our financial condition, future results of operations, projected revenues and expenses, business strategies, operating efficiencies or synergies, competitive positions, growth opportunities for existing intellectual properties, technologies, products, plans, and objectives of management, markets for our securities, and other matters, are about us and our industry that 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, wherever they occur, are necessarily estimates reflecting the best judgment of our senior management on the date on which they were made, or if no date is stated, as of the date of the filing made with the SEC in which such statements 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 That May Affect Future Results" in Part II, Item 1A of this Quarterly Report on Form 10-Q. Unless required by U.S. federal securities laws and the rules and regulations of the SEC, 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 to reflect later events or circumstances or to reflect the occurrence of unanticipated events or any other reason.

Plan of Operation and Overview

We are a development stage biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. Our initial focus is on developing our first product candidate, RP103, for the potential treatment for nephropathic cystinosis, or cystinosis, a rare genetic disorder. Cystinosis patients are at very high risk of experiencing life-threatening metabolic disorders, including kidney failure, severe gastrointestinal dysfunction and rickets as a result of an accumulation of the amino acid, cystine, in cells. As a result, cystinosis patients have a substantially reduced life span relative to unaffected individuals.

In July 2011, we announced that RP103 had met the sole primary endpoint in our Phase 3 clinical trial designed to evaluate RP103 as a treatment of cystinosis. In addition, we now report that 39 patients (rather than 38 as originally reported) were included in the evaluable data set with respect to this trial, which continues to meet its sole primary endpoint. Based on the 39 patients, on average, the peak white blood cell, or WBC, cystine level measured in patients treated with Cystagon® was 0.44 +/- 0.05 nmol 1/2 cystine/mg protein, compared to an average peak value of 0.51 +/- 0.05 nmol 1/2 cystine/mg protein for patients treated with RP103. The mean difference was 0.08 nmol 1/2 cystine/mg protein, with a 95.8% confidence interval of 0.01-0.15 (one sided [p=0.021]). As stipulated in the Statistical Analysis

Plan agreed upon with the FDA, the non-inferiority endpoint of the clinical trial would be achieved when the upper end of the confidence interval around the mean difference of WBC cystine levels did not exceed an absolute value of 0.3. The upper end of the confidence interval in the Phase 3 clinical trial was determined to be 0.15, thus achieving the non-inferiority endpoint. In addition, we now report that, on average, the total daily steady-state dose of RP103 in patients in the Phase 3 clinical trial was 84% of their established incoming dose of Cystagon.

In the first quarter of calendar 2012, we submitted a New Drug Application, or NDA, to the U.S. Food and Drug Administration, or FDA, requesting approval to market RP103 for the treatment of cystinosis. The FDA granted Standard Review designation for RP103 and assigned an initial user fee goal date of January 30, 2013, which the FDA has extended to April 30, 2013. Also in the first quarter of calendar 2012, we submitted a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, requesting approval to market RP103 for the treatment of cystinosis.

Table of Contents

Our three active clinical development programs utilize the same active pharmaceutical ingredient, cysteamine bitartrate. Cysteamine bitartrate was approved in 1994 as an orally available immediate-release powder in a capsule for the treatment of, and is the current standard of care, for cystinosis. We are reformulating cysteamine bitartrate to potentially improve the dose administration, safety and/or efficacy compared to existing treatment and repurposing cysteamine bitartrate for potential applications in new disease indications. Our proprietary delayed-release formulation, RP103, is a capsule containing enteric coated micro-beads of cysteamine bitartrate. We believe RP103 will require less frequent dosing and could reduce gastro-intestinal and other side effects compared to immediate-release cysteamine bitartrate for cystinosis patients. In addition to cystinosis, we are also testing RP103 for the potential treatment of Non-alcoholic Steatohepatitis, or NASH, and Huntington's Disease, or HD. RP104 is our enteric coated tablet formulation of cysteamine bitartrate in development. We have an exclusive worldwide license to delayed-release cysteamine from the University of California, San Diego, or UCSD, which is the basis for our proprietary formulations of cysteamine.

Our other clinical-stage product candidate is Convivia™, our proprietary oral formulation of 4-methylpyrazole, for the potential management of acetaldehyde toxicity due to alcohol consumption by individuals with aldehyde dehydrogenase, or ALDH2, deficiency, an inherited metabolic disorder.

Future Activities

Over the next fiscal year, we plan to conduct research and development and general and administrative activities including: pre-commercial launch preparation of RP103 for the treatment of cystinosis in the U.S. and EU, (including preparing commercial materials and coordinating drug supply) and, if approved by applicable regulatory authorities, conducting a commercial launch of RP103 in the U.S. and EU; supporting our ongoing extension study of RP103 in cystinosis until patients are converted onto commercial drug; conducting other supporting clinical studies of RP103 in cystinosis; supplying clinical material for our ongoing clinical trial of RP103 in HD; funding the collaboration and supplying clinical material in our ongoing Phase 2b clinical trial of RP103 in NASH; continuing business development of our preclinical product candidates; conducting research and development activities for in-licensed and newly discovered preclinical assets; supporting potential clinical trials of RP103 in malaria, Rett Syndrome, fibrosis and Parkinson's Disease (subject to potential external funding); and supporting associated facilities and administrative functions.

We plan to seek additional business development partners in Asia for our Convivia™ product candidate. We may also develop new preclinical, clinical and or commercial opportunities, including proprietary targets discovered in-house and in-licensed and acquired technologies.

Results of Operations

Three months ended November 30, 2012 and 2011

Revenue

To date, we have not generated any revenue from the sale of any products, and we do not expect to generate significant revenue unless or until we obtain marketing approval of RP103 for the treatment of cystinosis and commercialize the product.

General and Administrative Expenses

General and administrative expenses include pre-commercial, finance, executive and human resources compensation and benefits, pre-commercial expenses, such as reimbursement and marketing studies, corporate expenses, such as legal, tax and auditing fees, business development expenses, travel, board of director fees and expenses, investor relations expenses, intellectual property costs associated with filed (but not issued) patents, administrative consulting and allocated human resources and facilities costs. General and administrative expenses for the three-month period ended November 30, 2012 increased by $4,029 compared to the prior year's first fiscal quarter. The increase was primarily due to:

Reason for increase (decrease)	Increase (decrease)
	$ 2,290

Increase in pre-commercial expenses (in support of launch of RP103 for cystinosis) primarily for increased consulting services, staffing compensation, travel and legal	
Increase in finance and human resources infrastructure expenses	652
Decrease in allocated administrative costs to R&D due to change in allocation methodology	338
Other, net	749
Total increase Q1 FY 2013 versus Q1 FY 2012	$ 4,029

Table of Contents

Research and Development

Research and development expenses include medical, clinical, regulatory and scientists' compensation and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, commercial drug manufacturing costs prior to 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 for the three month period ended November 30, 2012 increased by approximately $1,769 over the prior year's first fiscal quarter primarily due to:

Reason for increase (decrease)	Increase (decrease)
Increased product manufacture of RP103 for cystinosis	$ 810
Increase in funding for NASH Phase 2b clinical trial	750
Salary increases and new hire salaries	324
Increase in preclinical services	250
Decrease in allocated administrative costs to R&D due to change in allocation methodology	(338)
Other, net	(27)
Total increase Q1 FY 2013 versus Q1 FY 2012	$ 1,769

Research and development expenses include the following: (in $ millions)

Major Program (stage of development)	Three month periods ended November 30, 2012	2011
RP103/RP104:		
Cystinosis (pre-commercial)	2.6	2.4
HD (clinical)	0.1	0.8
NASH (clinical)	0.8	0.6
Preclinical programs	0.1	0.1
Minor or inactive programs	0.2	0
R & D personnel and other costs not allocated to programs	3.0	1.1
Total research & development expenses	6.8	5.0

Major Program expenses recorded as general and administrative expenses: (in $ millions)

Major Program (stage of development)	Three month periods ended November 30, 2012	2011
RP103 for cystinosis (pre-commercial)	1.8	0.3

Preclinical programs	0.1	0.0

Additional major program expenses include expenses related to the preparation for the potential commercial launch of RP103 for the treatment of cystinosis and patent fees and patent expenses which were recorded as general and administrative expenses as these fees are to support patent applications (not issued patents).

Any of our major programs could be partnered for further development and/or could be accelerated, slowed or ceased due to scientific results or challenges in obtaining funding. We anticipate that we will need additional funding in order to pursue our plans beyond the fourth calendar quarter of 2013. 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 That May Affect Future Results" in Part II, Item 1A of this Quarterly Report on Form 10-Q.

Interest Income

Interest income for the three-month periods ended November 30, 2012 and 2011 was $119 and $64, respectively.

Interest Expense

Interest expense for the three-month periods ended November 30, 2012 and 2011 was nominal.

Table of Contents

Foreign Currency Transaction Gain

Foreign currency transaction gain for the three month periods ended November 30, 2012 and 2011 was nominal.

Unrealized Loss on Short-Term Investments

Unrealized loss on short-term investments represents the change in net asset value of the Company's short-term bond fund. The unrealized loss on short-term investments for the three month periods ended November 30, 2012 and 2011 was nominal.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of $364 for the three month period ended November 30, 2012 compared to a loss of $4,168 for the three month period ended November 30, 2011, representing an decrease of $3,804 resulting primarily from a smaller number of warrants outstanding during the three months ended November 30, 2012 compared to the three months ended November 30, 2011. These losses are non-cash.

Application of Critical Accounting Policies

Our condensed consolidated financial statements and accompanying notes are prepared in accordance with generally accepted accounting principles used in the U.S., 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. We believe the following critical accounting policies require us to make significant judgments and estimates in the preparation of our condensed consolidated financial statements.

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.

Functional Currency

Our consolidated functional currency is the U.S. dollar. Raptor Pharmaceuticals Europe B.V., or BV, and RPTP European Holdings C.V., or CV, our European subsidiary and Cayman-based subsidiary, respectively, use the European Euro as their functional currency. At each quarter end, BV'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 and CV's equity are adjusted for any translation gain or loss.

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.

Cash and Cash Equivalents

We consider all highly liquid investments with original maturities of three months or less, when purchased, to be cash equivalents. We maintain 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 our U.S. and European banks as collateral for credit cards.

Short-term Investments

We invest in short-term investments in high credit-quality funds in order to obtain higher yields on our cash available for investment. Such investments are not insured by the Federal Deposit Insurance Corporation. We completed an evaluation of our investments and determined that we did not have any other-than-temporary impairments as of November 30, 2012. The investments are placed in financial institutions with strong credit ratings and management expects full recovery of the carrying amounts.

Table of Contents

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.

Debt Issuance Costs

Debt issuance costs represent expenses incurred to obtain the debt we incurred under a loan agreement with HealthCare Royalty Partners, or HC Royalty, in December 2012. These costs are initially capitalized and will be amortized over the life of the loan.

Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine (currently developed as RP103 and RP104) and to an out-license acquired in the 2009 Merger. The intangible assets related to RP103/RP104 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. 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.

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. An impairment analysis is performed, and if necessary, a resulting write-down in valuation is recorded.

Fixed Assets

Fixed assets, which mainly consist of leasehold improvements, 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

We 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.

As of August 31, 2012, we determined that the capitalized acquired in-process research and development cost of $900, representing the tezampanel and NGX 426 program acquired in our 2009 Merger, was impaired due to our decision to discontinue development of this product candidate for thrombosis due to regulatory hurdles that would require significant expenditures which we chose not to prioritize for funding. As such, we expensed $900 as in-process research and development as part of research and development expense on our consolidated statements of comprehensive loss for the year ended August 31, 2012. During the three month period ended November 30, 2012, we did not identify any such impairment losses.

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 such warrants is reclassified to equity.

Table of Contents

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 tax rate is 0% for income tax for the three month period ended November 30, 2012 and we expect that our effective tax rate for our fiscal year ended August 31, 2012 and the short calendar year from September 1, 2012 to December 31, 2012 will be 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.

On September 1, 2009, we adopted the provisions of ASC No. 740-10, Accounting for Uncertainty in Income Taxes, or ASC 740-10. ASC 740-10 requires entities following GAAP to identify uncertain tax positions and disclose any potential tax liability on their financial statements using a two-step process, which includes recognition and measurement.

Our continuing practice is to recognize interest and/or penalties related to income tax matters as a component of income tax expense. As of November 30, 2012, there were no accrued interest or penalties related to uncertain tax positions.

We file U.S. Federal, California, Georgia and North Carolina state income tax returns and Dutch income tax returns. We are currently not subject to any income tax examinations. Due to our NOLs, generally all tax years remain open.

Research and Development

We are a development stage biotechnology company. 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 administrative 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.

In-Process Research and Development

Prior to September 1, 2009, we recorded in-process research and development expense for a product candidate acquisition where there was not more than one potential product or usage for the assets being acquired. Upon the adoption of the revised guidance on business combinations, effective September 1, 2009, the fair value of acquired in-process research and development is capitalized and tested for impairment at least annually. Upon completion of the research and development activities, the intangible asset is amortized into earnings over the related product's useful life. In-process research and development that is amortized or expensed is recorded as part of research and development expenses on our condensed consolidated statements of comprehensive loss. We review each product candidate acquisition to determine the existence of in-process research and development.

Comprehensive Loss

Components of comprehensive loss are reported in our 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.

Table of Contents

Stock-Based Compensation

In February 2010, our board of directors approved, and in March 2010 our stockholders approved, our 2010 Equity Incentive Plan, or the 2010 Plan, to grant up to an aggregate of 3,000 stock options or restricted stock or restricted stock units over the ten year life of the 2010 Plan. Our board of directors has determined not to make any new grants under any of our former plans, but rather under the 2010 Plan. The 2010 Plan allows for the granting of options to employees, directors and consultants. As of November 30, 2012, options to purchase 7,741 shares of our common stock were outstanding and 2,012 shares of our common stock remain available for future issuance under the 2010 Plan. Plan amendments allow for 50% accelerated vesting of unvested stock options upon a change of control as defined in the 2010 Plan, as amended. 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 2010 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 us 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 us or within 90 days' of such continuous service with us) or permanent disability, to eighteen (18) months from the date of termination of continuous service with us.

In May 2006, Raptor Pharmaceuticals Corp.'s stockholders approved the 2006 Equity Compensation Plan, as amended, referred to herein as the 2006 Plan. The 2006 Plan's term is ten years and allows for the granting of options to employees, directors and consultants. Effective as of the effective time of the 2009 Merger, we assumed the outstanding stock options of Raptor Pharmaceuticals Corp. granted under the 2006 Plan. Such assumed options are subject to the terms of the 2006 Plan and, in each case, are also subject to the terms and conditions of an incentive stock option agreement, non-qualified stock option agreement or other option award, as the case may be, issued under such 2006 Plan. Prior to the 2009 Merger, and subject to the 2009 Merger becoming effective, our board of directors adopted the 2006 Plan such that the 2006 Plan became an equity incentive plan of ours after the 2009 Merger. Typical option grants under the 2010 and 2006 Plans are for ten years with exercise prices at or above market price based on the last closing price as of the date prior to the grant date on the relevant stock market or exchange and vest over four years as follows: 6/48ths on the six month anniversary of the date of grant; and 1/48th per month thereafter.

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.

In March 2005, the FASB issued ASC Topic 718 (previously listed as Staff Accounting Bulletin No. 107), which offers guidance for what was previously referred to as SFAS 123(R). ASC Topic 718 was issued to assist preparers by simplifying some of the implementation challenges of SFAS 123(R) while enhancing the information that investors receive. ASC Topic 718 creates a framework that is premised on two overarching themes: (a) considerable judgment will be required by preparers to successfully implement SFAS 123(R), specifically when valuing employee stock options; and (b) reasonable individuals, acting in good faith, may conclude differently on the fair value of employee stock options. Key topics covered by ASC Topic 718 include valuation models, expected volatility and expected term. For the three month period ended November 30, 2012, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 0.68%; five year expected life; 95% 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 along with the fact that we are a development-stage company and our anticipation that option holders will exercise stock options when we are at a more mature stage of

development; the volatility was based on a combination of the actual annualized volatility of our common stock price as quoted on NASDAQ since the closing of our 2009 Merger on September 30, 2009 and of annualized volatility of peer companies; 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 at our current development 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 6 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.

Table of Contents

Liquidity and Capital Resources

Capital Resource Requirements

As of November 30, 2012, we had $35,482 in cash, cash equivalents, and short-term investments, $21,072 in current liabilities (of which $16,238 represented the non-cash common stock warrant liability) and $17,261 of net working capital.

We estimate that our cash, cash equivalents and short-term investments of $58,000 as of December 31, 2012, including $23,414 of proceeds, net of fees and commissions, received in December 2012 as the first tranche under a $50,000 loan agreement with HC Royalty, will be sufficient to meet our obligations into the fourth quarter of calendar 2013.

Under the terms of the HC Royalty loan agreement executed on December 20, 2012, we received the first $25,000 tranche of the loan at closing and we would receive an additional $25,000 upon FDA approval of RP103 for the treatment of cystinosis and satisfaction of other customary closing conditions. The loans, which mature on December 31, 2019, bear interest at an annual fixed rate of 10.75% and a variable royalty rate, tiered down, based on a percentage of future product sales. The loan is interest-only for the first two years. The proceeds for the loans will be used primarily to help fund the commercialization of RP103 for the treatment of cystinosis, advance our development programs and for general corporate purposes.

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 $40,000, from time to time, through an "at the market" equity offering program under which Cowen acts as sales agent. We pay a 3% commission to Cowen on any sales pursuant to this Sales Agreement. Through November 30, 2012, we sold 2,660 shares at a weighted-average sales price of $5.20 per share for net proceeds of $13,330.

As of November 30, 2012, Series A warrants to purchase up to 680 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 November 30, 2012, 3,442 shares (including 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 placement agent warrant that we issued to the placement agent for this private placement is for the purchase 98 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, develop and commercialize RP103 for the treatment of cystinosis and other indications. Our future capital requirements may be substantial, and will depend on many factors, including:

- the decisions of the FDA and EMA with respect to our applications for marketing approval of RP103 for the treatment of patients with cystinosis in the U.S. and the EU; the costs of activities related to the regulatory approval process; and the timing of approvals, if received;
- the cost of establishing the sales and marketing capabilities necessary to be prepared for a potential commercial launch of RP103 for the treatment of cystinosis, if approved;
- the timing and cost of our ongoing clinical programs for RP103, including: evaluating RP103 in treatment-naïve cystinosis patients, and other supportive studies; evaluating RP103 as a treatment for Huntington's Disease; and evaluating RP103 as a treatment for NASH;
- the cost of filing, prosecuting and enforcing patent claims;
- the costs of our manufacturing-related activities; and
- subject to receipt of marketing approval, the levels, timing and collection of revenue received from sales of RP103.

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 maintained several contracts with drug labelers and distributors, research organizations, contract manufacturers, clinical organizations and clinical sites, primarily to assist with clinical research and clinical and commercial manufacturing for our cystinosis and HD programs and our NASH clinical collaboration. Our contractual obligations have not changed significantly during the three month period ended November 30, 2012 compared to our fiscal year ended August 31, 2012.

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.

Table of Contents

Going Concern

Due to the uncertainty of our ability to meet our current operating and capital expenses, in their reports on our audited financial statements for the year ended August 31, 2012 and for the period September 8, 2005 (inception) to August 31, 2012, our independent registered public accounting firm, Burr Pilger Mayer, Inc., included an explanatory paragraph regarding substantial doubt about our ability to continue as a going concern. Our audited financial statements as of August 31, 2012 contain additional note disclosures describing the circumstances that led to this disclosure by our independent registered public accounting firm.

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 three months ended November 30, 2012 have not materially changed from those discussed in our Annual Report on Form 10-K for the year ended August 31, 2012, filed with the SEC on November 14, 2012.

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.

Changes in Internal Control over Financial Reporting

During the most recent fiscal quarter, there have not been any material changes in our internal control over financial reporting or in other factors that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Table of Contents

PART II - OTHER INFORMATION

Item 1. Legal Proceedings.

We are not subject to any material legal proceedings.

Item 1A. Risk Factors.

RISK FACTORS THAT MAY AFFECT FUTURE RESULTS

(In thousands, except per share data, or unless otherwise specified)

An investment in our securities involves a high degree of risk. Before you decide to invest in our securities, you should consider carefully all of the information in this Quarterly Report on Form 10-Q, including the risks and uncertainties described below, as well as other information included in or incorporated by reference into this Quarterly Report on Form 10-Q, particularly the specific risk factors discussed in the sections titled "Risk Factors" contained in our filings with the SEC pursuant to Sections 13(a), 13(c), 14 or 15(d) of the Exchange Act before deciding whether to invest in our securities. Any of these risks could have a material adverse effect on our business, prospects, financial condition and results of operations. In any such case, the trading price of our common stock could decline and you could lose all or part of your investment. You should also refer to the other information contained in this Quarterly Report on Form 10-Q, or incorporated herein by reference, including our financial statements and the notes to those statements, and the information set forth under the caption "Forward-Looking Statements." in Part I Item 2 of this Quarterly Report on Form 10-Q. The risks described below and contained in our other periodic reports are not the only ones that we face. Additional risks not presently known to us or that we currently deem immaterial may also adversely affect our business operations.

As of November 30, 2012, there were no material changes to the risk factors disclosed in our Annual Report on Form 10-K for the year ended August 31, 2012 that was filed with the SEC on November 14, 2012, except as set forth below:

Risks Associated with Product Development and Commercialization

We currently depend entirely on the success of our lead compound, RP103. We may not receive marketing approval for, or successfully commercialize, RP103 for any indication.

In addition to the disclosure contained in this risk factor contained in our Annual Report on Form 10-K for the year ended August 31, 2012, we note that the FDA has extended the Prescription Drug User Fee Act goal date for our RP103 NDA for the treatment of cystinosis from January 30, 2013 to April 30, 2013. There is no assurance that we will obtain regulatory approval for RP103 for the treatment of cystinosis in the U.S. by the date, or at all.

If we fail within a reasonable time period to gain approval for our lead drug product program, RP103 for the treatment of cystinosis, our financial results and financial condition will be adversely affected. In such a case, we will have to delay or terminate some or all of our research product development programs and may be forced to dramatically restructure or cease operations.

Our loan agreement with HC Royalty contains a number of restrictive covenants and other provisions, which, if violated, could result in the acceleration 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, or HC Royalty, as lender, under which we agreed to borrow $50,000 in two $25,000 tranches, or the HC Royalty Loan, and we have drawn the first tranche in the amount of $25,000. Our loan agreement with HC Royalty includes a variety of affirmative and negative covenants, including the use of commercially reasonable efforts to exploit 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, 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 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. Further, HC Royalty may terminate its commitment to fund the second $25,000 tranche upon the occurrence of any such event prior to the funding of such tranche.

Table of Contents

Risks Related to Our Financial Position and Capital Requirements

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

As of November 30, 2012, we had an accumulated deficit of $130,045. We expect to continue to incur losses for the foreseeable future and will have to raise substantial cash 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 year ended August 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 others to choose not to deal with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

As of November 30, 2012, our cash, cash equivalents and short term investments were $35,482. We believe our cash, cash equivalents and short-term investments as of December 31, 2012 of $58,000, including $23,414 we received with respect to the first tranche amount drawn down under our HC Royalty Loan in December 2012 will be sufficient to meet our projected operational requirements and obligations into the fourth quarter of calendar 2013.

There can be no assurance that we will be successful in raising sufficient funds when needed. If we are unable to obtain such additional capital when needed, we will be forced to reduce our expenditures or seek other corporate solutions.

In addition, under the HC Royalty Loan, HC Royalty has agreed to lend us $25,000 in a second tranche, provided that we have received FDA approval for RP103 for the treatment of cystinosis and other funding conditions are satisfied. There can be no assurance that we will receive such FDA approval or that such other conditions will be satisfied and, accordingly, there can be no assurance that we will be able to draw on the second $25,000 tranche under the HC Royalty Loan.

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 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, the execution of our potential launch of RP103 for cystinosis 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 pre-launch/launch expenses for RP103. If such actions are required, our financial condition and operating results will be adversely affected and our current value and potential future value 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 variable rate based on the amount of included product payments in a calendar year, and such interest is payable quarterly. Included product payments are the net revenues of our Company and our subsidiaries from existing and future products. Principal payments under the HC Royalty Loan will become due beginning on the ninth quarterly payment date occurring after the date the second $25,000 tranche is funded (if at all) and, in the case of the first tranche loan, in no event later than March 31, 2017.

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, on satisfactory terms or at all. In addition, the terms of the HC Royalty Loan may limit our ability to pursue any of these alternatives and these

alternative measures may not be successful and 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.

Table of Contents

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.
None.

Table of Contents

Item 6. Exhibits

Exhibit Index

(2)	Plan of acquisition, reorganization, arrangement, liquidation or succession
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. (incorporated by reference to Annex A to Registration Statement No. 333-136018 filed on July 25, 2006).
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. (incorporated by reference to Annex A to Amendment No. 1 to Registration Statement No. 333-136018 filed on August 25, 2006).
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 (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
2.4	Form of Voting Agreement between TorreyPines Therapeutics, Inc. and certain stockholders of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 99.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
2.5	Form of Voting Agreement between Raptor Pharmaceuticals Corp. and certain stockholders of TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
(3)(i), (ii)	Articles of incorporation; Bylaws
3.1	Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.2	Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.3	Certificate of Amendment filed with the Secretary of State of the State of Nevada effecting an 8-for-1 reverse stock of the Registrant's common stock and changing the name of the Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.3 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.4	Articles of Conversion filed with the Secretary of State of the State of Nevada changing the state of incorporation of the Registrant (incorporated by reference to Exhibit 3.4 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.5	Certificate of Conversion filed with the Secretary of State of the State of Delaware (incorporated by reference to Exhibit 3.5 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.6	

	Amendment to Bylaws of the Registrant (incorporated by reference to Exhibit 3.6 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
3.7	Charter Amendment for TorreyPines (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
3.8	Certificate of Merger between Raptor Pharmaceuticals Corp., ECP Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
3.9	Amendment to Bylaws of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on May 14, 2012).
(4)	Instruments defining the rights of security holders, including indentures
4.1	Specimen common stock certificate of the Registrant (incorporated by reference to Exhibit 4.7 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
4.2	Form of Warrant to Purchase Common Stock issued to previous holders of TPTX, Inc. redeemable convertible preferred stock in connection with the business combination between TorreyPines Therapeutics, Inc. and Axonyx, Inc. (incorporated by reference to Exhibit 4.2 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.3	Form of Registration Rights Agreement 1999 (incorporated by reference to Exhibit 4.4 to the Registrant's Annual Report on Form 10-KSB, filed on March 13, 2000).
4.4	Registration Rights Agreement dated as of January 8, 2004 between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on January 12, 2004).
4.5	Registration Rights Agreement dated as of May 3, 2004, between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on May 5, 2004).

Table of Contents

4.6 *	Form of Warrant issued to Comerica Bank on July 1, 2003 (incorporated by reference to Exhibit 4.14 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.7 *	Form of Warrant issued to Silicon Valley Bank on December 8, 2000 (incorporated by reference to Exhibit 4.15 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.8 *	Form of Warrant issued to Oxford Financial and Silicon Valley Bank on September 27, 2005 (incorporated by reference to Exhibit 4.16 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.9	Rights Agreement, dated as of May 13, 2005, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on May 16, 2005).
4.10	Amendment to Rights Agreement, dated as of June 7, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed on June 12, 2006).
4.11	Form of Warrant issued to Comerica Bank on June 11, 2008 (incorporated by reference to Exhibit 4.1 to the Registrant's Report on Form 8-K, filed on June 17, 2008).
4.12	Amendment to Rights Agreement, dated as of October 3, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.19 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.13	Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between TorreyPines and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company) (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
4.14	Amendment to Rights Agreement, dated August 6, 2010, by and between the Registrant and American Stock Transfer & Trust Company, LLC (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on August 10, 2010).
4.15	Warrant to purchase common stock dated December 14, 2007 issued to Flower Ventures, LLC (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April 15, 2008).
(31)	Section 302 Certification
31.1†	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director
31.2†	Certification of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer
(32)	Section 906 Certification
32.1†	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director, and of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer

101**	The following materials from the Raptor Pharmaceutical Corp. Quarterly Report on Form 10-Q for the quarter ended November 30, 2012, 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.

* The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.15 - 4.19 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.

** Furnished herewith and not "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended.

† Filed herewith.

Table of Contents

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.

By: /s/ Christopher M. Starr
Christopher M. Starr, Ph.D.
Chief Executive Officer and Director (Principal Executive Officer)
Date: January 9, 2013

By: /s/ Georgia Erbez
Georgia Erbez
Chief Financial Officer, Secretary and Treasurer (Principal Financial Officer and Principal Accounting Officer)
Date: January 9, 2013