Raptor Pharmaceutical Corp
Form 10-Q
August 07, 2014

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 June 30, 2014

OR

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

Commission File Number: 000-25571

RAPTOR PHARMACEUTICAL CORP.
(Exact name of registrant as specified in its charter)

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

7 Hamilton Landing, Suite 100, Novato, CA 94949
(Address of Principal Executive Offices)

(415) 408-6200
(Registrant's telephone number)

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

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 þ No o

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 o Accelerated filer þ Non-accelerated filer o Smaller reporting company o

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

At August 4, 2014, there were 62,742,871 shares of the registrant's common stock outstanding.

RAPTOR PHARMACEUTICAL CORP.

FORM 10-Q

TABLE OF CONTENTS

Table of Contents

PART I - FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS.

RAPTOR PHARMACEUTICAL CORP.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Unaudited)
(In thousands, except shares per share data)

	June 30, 2014	December 31, 2013
ASSETS		
Current assets:		
Cash and cash equivalents	$58,100	$83,052
Restricted cash	1,030	500
Accounts receivable	5,776	6,181
Inventories	8,350	3,000
Prepaid expenses and other	2,713	3,566
Total current assets	75,969	96,299
Noncurrent assets:		
Fixed assets, net	3,672	1,810
Intangible assets, net	3,093	3,213
Goodwill	3,275	3,275
Other assets	4,332	4,129
Total Assets	$90,341	$108,726
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$1,862	$5,264
Accrued liabilities	16,797	13,069
Common stock warrant liability	860	7,066
Deferred revenue	2,996	4,698
Capital lease liability – current	29	18
Note payable, current portion	2,500	-
Total current liabilities	25,044	30,115
Noncurrent liabilities:		
Note payable, net of current portion	47,500	50,000
Capital lease liability – long-term	63	41
Total liabilities	72,607	80,156
Stockholders' equity:		
Preferred stock, $0.001 par value per share, 15,000,000 shares authorized, zero shares issued and outstanding	-	-
Common stock, $0.001 par value per share, 150,000,000 shares authorized, 62,690,387 and 61,614,576 shares issued and outstanding at June 30, 2014 and December 31, 2013, respectively	63	62
Additional paid-in capital	250,836	234,286
Accumulated other comprehensive loss	(225)	(423)
Accumulated deficit	(232,940)	(205,355)
Total stockholders' equity	17,734	28,570

Total Liabilities and Stockholders' Equity	$90,341	$108,726

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

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(Unaudited)
(In thousands, except shares and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Revenues	$16,313	$21	$28,447	$21
Cost of sales	995	425	2,309	425
Gross profit (loss)	15,318	(404)	26,138	(404)
Operating expenses:				
Research and development	11,077	6,215	20,624	14,627
Selling, general and administrative	13,326	9,379	25,390	17,242
Total operating expenses	24,403	15,594	46,014	31,869
Loss from operations	$(9,085)	$(15,998)	$(19,876)	$(32,273)
Interest income	10	16	41	171
Interest expense	(3,497)	(1,075)	(6,476)	(1,801)
Foreign currency transaction gain (loss)	19	(1)	36	(35)
Loss on short-term investments	-	(22)	-	(129)
Adjustment to fair value of common stock warrants	(134)	(7,041)	(1,297)	(5,981)
Net loss before provision for income taxes	(12,687)	(24,121)	(27,572)	(40,048)
Provision for income taxes	(6)	-	(12)	-
Net Loss	$(12,693)	$(24,121)	$(27,584)	$(40,048)
Net loss per share:				
Basic and diluted	$(0.20)	$(0.43)	$(0.44)	$(0.73)
Weighted-average shares outstanding:				
Basic and diluted	62,652,079	56,227,770	62,414,793	54,977,350

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

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(Unaudited)
(In thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Net loss	$(12,693)	$(24,121)	$(27,584)	$(40,048)
Other comprehensive gain (loss):				
Foreign currency translation gain (loss)	48	(1)	197	(89)
Comprehensive Loss	$(12,645)	$(24,122)	$(27,387)	$(40,137)

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

Table of Contents

RAPTOR PHARMACEUTICAL CORP.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(Unaudited)
(In thousands)

	Six Months Ended June 30,	
	2014	2013
Cash flows from operating activities:		
Net loss	$(27,584)	$(40,048)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	5,650	3,597
Fair value adjustment of common stock warrants	1,297	5,981
Amortization of intangible assets	120	86
Depreciation of fixed assets	246	78
Realized gain on sale of fixed assets	-	(12)
Loss on short-term investments	-	129
Amortization of debt issuance cost	300	151
Changes in assets and liabilities:		
Accounts receivable	405	(26)
Inventories	(5,350)	(225)
Prepaid expenses and other assets	1,023	(6,561)
Accounts payable	(3,402)	(2,284)
Accrued liabilities	3,728	3,938
Deferred revenue	(1,702)	-
Net cash used in operating activities	(25,269)	(35,196)
Cash flows from investing activities:		
Net purchase of fixed assets	(2,056)	(521)
Purchase of short-term investments	-	(147)
Sale of short-term investments	-	22,114
Intangible assets	-	(750)
Change in restricted cash	(530)	(50)
Net cash (used in) provided by investing activities	(2,586)	20,646
Cash flows from financing activities:		
Proceeds from sale of common stock under an ATM agreement	-	22,367
Proceeds from the exercise of common stock warrants	1,826	6,706
Proceeds from the exercise of common stock options	1,611	7
Note payable	-	25,000
Debt issuance costs	(631)	(1,257)
Offering costs	(43)	89
Payments on capital lease	(19)	(18)
Net cash provided by financing activities	2,744	52,894
Effect of exchange rates on cash and cash equivalents	159	(89)
Net (decrease) increase in cash and cash equivalents	(24,952)	38,255
Cash and cash equivalents, beginning of period	83,052	36,313
Cash and Cash Equivalents, End of Period	$58,100	$74,568

Supplmental cash flow information:

Interest paid	$5,431	$1,647
Income taxes paid	$159	$-
Supplemental disclosure of non-cash financing activities:		
Fair value of warrant liability reclassified to equity upon excerise	$7,503	$8,918
Acquisition of equipment in exchange for capital lease	$-	$68

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

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

1. DESCRIPTION OF BUSINESS AND SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

The accompanying condensed consolidated financial statements reflect the financial position and results of operations of Raptor Pharmaceutical Corp. (the "Company" or "Raptor") and have been prepared in accordance with the accounting principles generally accepted in the United States of America ("GAAP") pursuant to the rules and regulations of the Securities and Exchange Commission (the "SEC"). Certain information and footnote disclosures have been condensed or omitted pursuant to such rules and regulations. The unaudited condensed consolidated financial statements have been prepared on the same basis as the annual financial statements and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for a fair presentation of the periods presented. The condensed consolidated balance sheet as of December 31, 2013 has been derived from our audited financial statements as of such date, but does not include all disclosures required by 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 December 31, 2013.

Raptor is a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. The Company's first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules ("PROCYSBI"), received marketing approval from the U.S. Food and Drug Administration ("FDA"), on April 30, 2013 for the management of nephropathic cystinosis in adults and children six years and older. The European equivalent, PROCYSBI® gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received marketing authorization on September 6, 2013 from the European Commission ("EC"), as an orphan medicinal product for the management of proven nephropathic cystinosis for marketing in the European Union ("EU"). PROCYSBI received seven years and 10 years of market exclusivity as an orphan drug in the U.S. and the EU, respectively. The Company commenced commercial sales of PROCYSBI in the U.S. in mid-June 2013 and in Germany in April 2014. For at least the near term, the Company's ability to generate revenues is entirely dependent upon sales of PROCYSBI in the U.S. for the management of nephropathic cystinosis in adults and children six years and older and in the EU for the management of proven nephropathic cystinosis.

Raptor's pipeline includes its proprietary delayed-release form of cysteamine, or RP103. Raptor currently has product candidates in clinical development designed to potentially treat Huntington's disease ("HD"), non-alcoholic steatohepatitis ("NASH"), Leigh syndrome and other mitochondrial disorders and aldehyde dehydrogenase deficiency ("ALDH2"). Raptor's preclinical programs are based upon bioengineered novel drug candidates that are designed to target cancer and other diseases.

The Company is subject to a number of risks, including: the level of commercial sales of PROCYSBI in the U.S. and Germany; the ability to successfully launch PROCYSBI in other international markets; the uncertainty of whether the Company's research and development efforts will result in expanded label for PROCYSBI and commercialization for RP103 in various indications or additional commercial products; competition from larger organizations; reliance on licensing the proprietary technology of others; dependence on key personnel; uncertain patent protection; and the need to raise capital through equity and/or debt financings. Funding may not be available when needed if at all or on terms acceptable to the Company. If the Company exhausts its cash reserves and is unable to obtain adequate financing, it may be required to curtail planned operating expenditures, including its development programs.

Basis of Presentation

The Company's condensed consolidated financial statements include the accounts of the Company's direct and indirect wholly owned subsidiaries, Raptor Pharmaceuticals Inc. and Raptor European Products, LLC, such subsidiaries incorporated in Delaware on August 1, 2007, and February 14, 2012, respectively, and Raptor Pharmaceuticals Europe B.V. (“BV”), Raptor Pharmaceuticals France SAS (“SAS”), Raptor Pharmaceuticals Germany GmbH (“GMBH”) and RPTP European Holdings C.V. (“CV”), domiciled in the Netherlands on December 15, 2009, in France on October 30, 2012, in Germany on October 16, 2013 and in the Cayman Islands on February 16, 2012, respectively. All inter-company accounts have been eliminated.

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

Use of Estimates

The preparation of financial statements in conformity with U.S. 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.

Functional Currency

The Company's consolidated functional currency is the U.S. dollar. BV, SAS, GMBH, and CV, the Company's Dutch, French, German and Cayman-based subsidiaries, respectively, use the European Euro as their functional currency. At each quarter end, each foreign subsidiary's balance sheet is translated into U.S. dollars based upon the quarter-end exchange rate, while their statements of operations are translated into U.S. dollars based upon an average exchange rate during the period.

Segment Information

The Company has determined that it operates in only one segment, as it only reports profit and loss information on an aggregate basis to its chief operating decision maker. The Company's long-lived assets maintained outside the U.S. are not material.

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 and for access to a value-added tax deferral program. As of June 30, 2014, the Company had $58.1 million in cash and cash equivalents, of which $4.7 million was held by its foreign subsidiaries.

Fair Value of Financial Instruments

The carrying amounts of certain of the Company's financial instruments including cash equivalents, restricted cash, accounts payable, accrued liabilities, note payable and capital lease liability approximate fair value due either to length of maturity or interest rates that approximate prevailing market rates. 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.

Revenue Recognition and Accounts Receivable

The Company recognizes revenue in accordance with the Financial Accounting Standards Board ("FASB") Accounting Standards Codification (“ASC”) 605, Revenue Recognition, when the following criteria have been met: persuasive evidence of an arrangement exists; delivery has occurred and risk of loss has passed; the seller's price to the buyer is fixed or determinable and collectability is reasonably assured. The Company determines that persuasive evidence of an arrangement exists based on written contracts that define the terms of the arrangements. Pursuant to

the contract terms, the Company determines when title to products and associated risk of loss has passed on to the customer. The Company assesses whether the fee is fixed or determinable based on the payment terms associated with the transaction and whether the sales price is subject to refund or adjustment. The Company assesses collectability based primarily on the customer's payment history and creditworthiness.

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

PROCYSBI is currently available for U.S. distribution from the Company's U.S. specialty pharmacy partner, the Accredo Health Group, Inc. ("Accredo") which is currently the Company's only U.S. customer and ships directly to patients. The Company's distributor in the EU is the Almac Group, Ltd. PROCYSBI is not available in U.S. retail pharmacies. Prior authorization of coverage by patients' commercial insurance plans, Raptor's patient assistance program ("PAP") or government payors is a prerequisite to the shipment of PROCYSBI to U.S. patients. Revenue is recognized in the U.S. once the product has been shipped by the specialty pharmacy to patients because the Company has not yet been able to reasonably estimate the third-party payor mix and resulting rebates based on its lack of sufficient historical data. Billings to the Company's distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenue by the Company until such shipments to patients occur. Revenue is currently recognized in the EU once the cash has been received as the Company has not yet been able to determine the payors' credit worthiness.

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

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

Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price, with cost determined on a first-in, first-out basis. Inventories are reviewed periodically to identify slow-moving inventory based on sales activity, both projected and historical, as well as product shelf-life. Prior to the approval of PROCYSBI by the FDA on April 30, 2013 and in Europe, prior to the approval by the EC on September 6, 2013, the Company recorded the purchase of raw materials and the manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to FDA and EC approval, the Company began capitalizing these costs and manufacturing overhead as commercial inventory. Upon launching PROCYSBI in mid-June 2013 in the U.S. and April 2014 in the EU, the Company began recognizing cost of sales. Cost of sales includes the cost of inventory sold or reserved; manufacturing, manufacturing overhead and supply chain costs; product shipping and handling costs; amortization of licensing approval milestone payments and licensing royalties payable to the University of California, San Diego ("UCSD").

The Company capitalizes inventory produced in preparation for product launches when positive results have been obtained for the clinical trials that the Company believes are necessary to support regulatory approval and the Company has determined it is probable that these capitalized costs will provide some future economic benefit in excess of capitalized costs. For these inventories, the Company also considers the expected approval date in assessing realizability. To the extent that inventory is expected to expire prior to being sold, the Company will write down the value of inventory. If actual results differ from those estimates, additional inventory write-offs may be required.

Impairment of Goodwill and Intangible Assets

Goodwill represents the excess of the purchase price over the fair value of tangible and identified intangible net assets of businesses acquired. Goodwill is not amortized, but is evaluated for impairment on an annual basis or more often when impairment indicators are present. The Company has one reporting unit. Therefore, the Company's consolidated net assets, including existing goodwill and other intangible assets, are considered to be the carrying value of the reporting unit. If the carrying value of the reporting unit is in excess of its fair value, an impairment may exist, and the Company must perform the second step of the analysis, in which the implied fair value of the goodwill is compared to its carrying value to determine the impairment charge, if any. If the estimated fair value of the reporting unit exceeds the carrying value of the reporting unit, goodwill is not impaired and no further analysis is required.

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

The Company makes judgments about the recoverability of purchased intangible assets with finite lives whenever events or changes in circumstances indicate that impairment may exist. Recoverability of purchased intangible assets with finite lives is measured by comparing the carrying amount of the asset to the future undiscounted cash flows the asset is expected to generate. Impairment, if any, is measured as the amount by which the carrying value exceeds the fair value of the impaired asset.

Fixed Assets

Fixed assets, which mainly consist of leasehold improvements, office furniture, manufacturing and lab equipment and computer hardware and software, 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.

Common Stock Warrant Liabilities

The Company issued warrants that contain conditional obligations that may require the Company to transfer cash to settle the warrants upon the occurrence of certain fundamental transactions. Therefore, the Company has classified the warrants as liabilities. The Company re-measures the liability at the end of every reporting period with the change in value reported in the Company's condensed consolidated statements of operations. At the exercise date, the fair values of these warrants are re-measured and reclassified to equity.

Note Payable and Debt Issuance Costs

Note payable consists of payments under the Company's December 2012 loan agreement with HealthCare Royalty Partners II, L.P. ("HC Royalty"), as lender, under which Raptor borrowed $50.0 million in two $25.0 million tranches received in December 2012 and May 2013. During the reporting period, the loan bore interest at an annual fixed rate of 10.75% of outstanding principal and included a synthetic royalty component based on net product sales, including PROCYSBI, in a calendar year. The fixed and royalty interest are recognized as interest expense as incurred. Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan to interest expense using the interest method.

Research and Development Costs

Research and development costs are charged to expense as incurred. Research and development expenses primarily include salaries and benefits for medical, clinical, regulatory, quality, pharmacovigilance and research personnel, preclinical studies, clinical trials and certain commercial drug manufacturing expenses prior to obtaining marketing approval.

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 loss per share is calculated by dividing net loss 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:

	Six Months Ended June 30,	
	2014	2013
Warrants to purchase common stock	334,764	2,212,615
Options to purchase common stock	9,952,407	8,335,534
Total Potentially Dilutive Securities	10,287,171	10,548,149

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

Comprehensive Loss

The components of comprehensive loss include net loss and foreign currency translation adjustments.

Stock Option Plan

Compensation costs related to the Company's stock option plans 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 compensation expense for stock-based compensation awards is reduced by an estimate for forfeitures.

The Company recognizes expense associated with stock options issued to third parties, including consultants, based upon the fair value of such awards on the date the options vest.

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. 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.

The Company identifies uncertain tax positions and discloses any potential tax liability on their financial statements. The Company recognizes interest and/or penalties related to income tax matters as a component of income tax expense. As of June 30 2014, there were no accrued uncertain tax positions or interest and penalties related to uncertain tax positions.

The Company files U.S. Federal, California, various other state and other income tax returns and various foreign country income tax returns. The Company is currently not subject to any income tax examinations. Due to the Company's net operating losses ("NOLs"), generally all tax years remain open.

Reclassifications

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

Recent Accounting Pronouncements

In May 2014, the FASB issued Accounting Standards Update (“ASU”) 2014-09, Revenue from Contracts with Customers, which outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The core principle of the revenue model is that “an entity recognizes revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be

entitled in exchange for those goods or services." In applying the revenue model to contracts within its scope, the Company will: identify the contract(s) with a customer, identify the performance obligations in the contract, determine the transaction price, allocate the transaction price to the performance obligations in the contract, and recognize revenue when (or as) the entity satisfies a performance obligation. This ASU is effective for interim and annual periods beginning after December 15, 2016 and early adoption is not permitted. The Company is currently evaluating this ASU to determine the impact adoption will have on its consolidated financial statements.

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

In June 2014, the FASB issued ASU 2014-12, Accounting for Share-Based Payments When the Terms of an Award Provide That a Performance Target Could Be Achieved after the Requisite Service Period. The ASU requires that a performance target that affects vesting, and that could be achieved after the requisite service period, be treated as a performance condition. A reporting entity should apply existing guidance in Topic 718 as it relates to awards with performance conditions that affect vesting to account for such awards. This ASU is effective for interim and annual periods beginning after December 15, 2015 and early adoption is permitted. The Company does not anticipate the adoption of this ASU will have a material impact on its consolidated financial statements.

2. FAIR VALUE MEASUREMENT

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 1 – Quoted market prices in active markets for identical assets or liabilities;

Level 2 – Inputs other than level one inputs that are either directly or indirectly observable; and

Level 3 – 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 June 30, 2014 and December 31, 2013 are summarized as follows:

(In thousands)

June 30, 2014	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents [1]	$51,744	$ -	$-	$51,744
Total	$51,744	$ -	$-	$51,744
Liabilities				
Common stock warrants	$-	$ -	$860	$860
Total	$-	$ -	$860	$860

December 31, 2013	Level 1	Level 2	Level 3	Total
Assets				
Cash equivalents [1]	$70,627	$ -	$-	$70,627
Total	$70,627	$ -	$-	$70,627

Liabilities				
Common stock warrants	$-	$ -	$7,066	$7,066
Total	$-	$ -	$7,066	$7,066

(1) Cash equivalents represent the fair value of the Company's investments in money market funds at June 30, 2014 and December 31, 2013.

Certain of the Company's common stock warrants are classified as liabilities and are, therefore, re-measured using the Black-Scholes option valuation model at the end of each reporting period with the change in value reported in the Company's condensed consolidated statements of operations.

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

The following table presents a reconciliation of the Company's recurring fair value measurements categorized within Level 3 of the fair value hierarchy (liability-classified common stock warrants):

(In thousands)	Six Months Ended June 30, 2014
Fair value as of December 31, 2013	$7,066
Change in fair value recognized in earnings	1,297
Exercises	(7,503)
Fair Value as of June 30, 2014	$860

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 fair value as of the reporting date is affected by Raptor's stock price as well as assumptions regarding a number of 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. The primary factors affecting the fair value of the warrant liability are the Company's stock price and volatility.

3. INTANGIBLE ASSETS AND GOODWILL

On December 14, 2007, the Company acquired the intellectual property and other rights to develop RP103 to treat various clinical indications from UCSD by way of a merger with Encode Pharmaceuticals, Inc., a privately held development stage company ("Encode"), which held the intellectual property license with UCSD. The fair value of the intangible assets at the time of acquisition was approximately $2.6 million.

Pursuant to the license agreement with UCSD, the Company is obligated to pay an annual maintenance fee until the commencement of commercial sales of any licensed products developed. The Company is also obligated to pay milestone payments upon the occurrence of certain events, royalties on net sales from products developed pursuant to the license agreement and a percentage of sublicense fees or royalties, if any. The Company is obligated to fulfill predetermined milestones within a specified number of years from the effective date of the license agreement, depending on the indication. To the extent that the Company fails to perform any of the obligations, UCSD may terminate the license or otherwise cause the license to become non-exclusive.

In April 2013, the Company received FDA approval of PROCYSBI (cysteamine bitartrate) delayed release capsules for the management of nephropathic cystinosis in adults and children 6 years and older. Subsequently, the Company announced that the EC had approved PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate) as an orphan medicinal product for the management of proven nephropathic cystinosis for marketing in the EU. In conjunction with these approvals, the Company paid milestone payments to UCSD during the second and third quarters of 2013 of $0.8 million and $0.5 million, respectively, pursuant to this license, which were capitalized as intangible assets.

A summary of intangibles acquired is as follows:

(In thousands)	Useful Life (Years)	June 30, 2014	December 31, 2013
Intangible asset (IP license for RP103) related to the Encode merger	20.0	$2,620	$ 2,620
Intangible assets (UCSD license - FDA and EC approval milestones)	20.0	1,250	1,250
Other intangible assets	16.0	240	240
Total intangible assets		4,110	4,110
Less accumulated amortization		(1,017)	(897)
Intangible Assets, Net		$3,093	$ 3,213

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

The intangible assets related to RP103 are being amortized over an 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. Other intangible assets are being amortized using the straight-line method over an estimated useful life of 16 years, which is the life of the intellectual property patents.

During the six months ended June 30, 2014 and year ended December 31, 2013, there was no intangible asset impairment recognized. During the three and six months ended June 30, 2014, the Company amortized approximately $37 thousand and $73 thousand, respectively, of intangible assets to research and development expense. During the three and six months ended June 30, 2013, the Company amortized approximately $50 thousand and $86 thousand, respectively, of intangible assets to research and development expense.

Amortization expense for intangible assets for each of the next five years is expected to be as follows:

(In thousands)	Amortization Expense
2014 (remaining 6 months)	$ 119
2015	238
2016	238
2017	238
2018	238

The Company tested the carrying value of goodwill for impairment as of December 31, 2013 and determined that there was no impairment.

4. INVENTORIES

Inventories consist of raw materials, work-in-process and finished goods related to the manufacture of PROCYSBI. Raw materials include the Company's active pharmaceutical ingredient ("API") for PROCYSBI. Work-in-process includes third party manufacturing and associated labor costs relating to the Company's personnel directly involved in the production process. Also included in inventories are raw materials and work-in-process that may be used for clinical trials, which are charged to research and development (“R&D”) expense when consumed.

Inventories are summarized as follows:

(In thousands)	June 30, 2014	December 31, 2013
Raw materials	$4,221	$ 2,469
Work-in-process	2,152	-
Finished goods	1,977	531
Total Inventories	$8,350	$ 3,000

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

5. FIXED ASSETS

Fixed assets consisted of:

(In thousands)	June 30, 2014	December 31, 2013	Estimated useful lives
Laboratory equipment	$1,485	$ 1,132	5 years
Assets under construction	1,354	102	-
Computer hardware and software	751	578	3 years
Office furniture	605	605	7 years
Leasehold improvements	288	-	Lease term
Capital lease equipment	110	68	Shorter of life of asset or lease term
Total at cost	4,593	2,485	
Less: accumulated depreciation	(921)	(675)	
Total Fixed Assets, Net	$3,672	$ 1,810	

Depreciation expense for the six months ended June 30, 2014 and 2013 was approximately $246 thousand and $78 thousand, respectively.

6. NOTE PAYABLE AND DEBT ISSUANCE COSTS

On December 20, 2012, the Company entered into a loan agreement with HC Royalty, as lender, under which it agreed to borrow $50.0 million in two $25.0 million tranches (the " Original HC Royalty Loan"). The Company received $23.4 million in net proceeds from the first tranche of the loan at closing in December 2012 and an additional $23.7 million in net proceeds in May 2013 from the second tranche upon FDA approval of PROCYSBI. The Company's loan agreement with HC Royalty included 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 the Company's obligations under the HC Royalty Loan, the Company granted a security interest to HC Royalty in substantially all of its assets, the assets of its domestic subsidiaries and a pledge of stock of certain of its domestic subsidiaries. The Company's failure to comply with the terms of the HC Royalty Loan and related documents, the occurrence of a change of control of the Company or the occurrence of an uncured material adverse effect on the Company or the occurrence of certain other specified events, would result in an event of default under the HC Royalty Loan that, if not cured or waived, could result in the acceleration of the payment of all of its indebtedness, as well as prepayment penalties, 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, the Company's pledged collateral and assign and transfer the pledged stock of certain of its subsidiaries.

During the reporting period, the loan bore interest at an annual fixed rate of 10.75%, payable quarterly. The loan also contains a synthetic royalty component based on net product revenues, including PROCYSBI, in a calendar year, and such royalty is payable quarterly. During the reporting period, with respect to the first $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bore a royalty rate of 6.25% of the first $25.0 million of product net revenues, 3.0% of product net revenues for such calendar year in excess of $25.0 million and up to $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly. During the reporting period, with respect to the second $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bore a royalty rate of 6.0% of the first $25.0 million of net revenues for such calendar year, 3.0% of product net revenues for such calendar year in excess of $25.0 million and up to $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly.

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

The Company received marketing approval of PROCYSBI from the FDA on April 30, 2013 and commenced shipment of PROCYSBI during June 2013, and as a result, royalties became payable to HC Royalty based upon net revenues of PROCYSBI. Interest expense on the loan for the three months ended June 30, 2014 and 2013 was $3.5 million and $1.1 million, respectively. Interest expense on the loan for the six months ended June 30, 2014 and 2013 was $6.5 million and $1.8 million, respectively.

During the reporting period, the loan and the Company's obligation to make payments thereunder would terminate immediately when all payments received by HC Royalty equaled $97.5 million. If, by December 20, 2014, net revenue for the immediately preceding four fiscal quarters exceeded $100.0 million, then the loan and the Company's obligation to make any payments would terminate immediately when all payments received by HC Royalty from the Company equaled $90.0 million. Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan to interest expense using the interest method.

Unamortized debt issuance costs totaled $3.1 million and $2.8 million as of June 30, 2014 and December 31, 2013, respectively. Amortization expense for the six months ended June 30, 2014 and 2013 was $0.3 million and $0.2 million, respectively.

7. ACCRUED LIABILITIES

Accrued liabilities consisted of:

(In thousands)	June 30, 2014	December 31, 2013
Personnel-related costs	$4,222	$ 4,443
Rebates and other sales deductions	3,562	2,325
Clinical trials and research and development costs	2,237	1,661
Royalty-based interest payable	2,000	1,255
License royalty payable	898	564
Manufacturing costs	536	294
Other	3,342	2,527
Total Accrued Liabilities	$16,797	$ 13,069

8. CAPITAL STRUCTURE

Preferred Stock

At June 30, 2014, the Company was authorized to issue 15,000,000 shares of $0.001 par value per share of preferred stock. There were no shares issued and outstanding.

Common Stock

At June 30, 2014, the Company was authorized to issue 150,000,000 shares of $0.001 par value per share of common stock. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held. As of June 30, 2014 and December 31, 2013, there were 62,690,387 and 61,614,576 shares, respectively, of the

Company's common stock issued and outstanding.

16

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)
Stockholder Rights Plan

Our stockholder rights plan entitles the holder of each outstanding share of common stock of the Company to one stock purchase right (a “Right”). Each Right entitles the registered holder to purchase from the Company one thousandth of a share of the Company’s Series A Participating Preferred Stock (the “Preferred Shares”) at a price of $15 per one one-thousandth of a Preferred Share (the “Purchase Price”), once the Rights become exercisable. The Rights will not be exercisable until the earlier of either (a) 10 days after the public announcement that a person, together with all affiliates or associates of such person, has become an “Acquiring Person” by obtaining beneficial ownership of 15% or more of the Company’s outstanding common stock, or (b) 10 business days (or a later date determined by the Board before any person or group becomes an Acquiring Person) after a person or group of affiliated or associated persons begins a tender or exchange offer which, if completed, would result in that person or group of affiliated or associated persons becoming an Acquiring Person. Each one one-thousandth of a share preferred stock, if issued, will have the same voting power as one one-hundred thirty-sixth ($1/136^{th}$) of a share of common stock and will entitle holders to a per share payment equal to the payment made on one one-hundred thirty-sixth ($1/136^{th}$) of a share of common stock, so that one full share of preferred stock would be entitled to receive a payment one one-hundred thirty-sixth (1/136th) of 1,000 times the per share payment to a share of common stock, provided that shares of the Company’s common stock are exchanged via merger, consolidation or a similar transaction. The Rights will expire on May 13, 2015 or on an earlier date if the Company redeems or exchanges the Rights.

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 $40.0 million through ATM offerings on the NASDAQ Stock Market. On July 3, 2013, the Company and Cowen amended and restated the Sales Agreement (the "Amended and Restated Sales Agreement") to increase the aggregate gross sales proceeds that may be raised to $100 million. Cowen is the sole sales agent for any sales made under the ATM for a 3.0% 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. During the six months ended June 30, 2014, there were no shares sold under the ATM. As of June 30, 2014, the Company had used approximately $53.8 million under the ATM.

Common Stock Warrants

During the six months ended June 30, 2014, the Company received approximately $1.8 million from the exercise of warrants in exchange for the issuance of 611,606 shares of the Company's common stock.

The number of common stock warrants outstanding as of June 30, 2014 were as follows:

	Number of Shares Exercisable	Exercise Price	Expiration Date
Issued in connection with Encode merger	233,309	$2.87	12/13/2015
TorreyPines warrants assumed in 2009 Merger	3,502	157.08	9/26/2015
Issued to registered direct investors in Dec. 2009	1	2.45	12/22/2014
Issued to placement agent in Aug. 2010	97,952	3.08	8/12/2015
Total Warrants Outstanding	334,764	$4.54	(1)

(1) Weighted average exercise price

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

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

A Black-Scholes option-pricing model was used to obtain the fair value of the warrant liabilities using the following assumptions at June 30, 2014 and December 31, 2013:

	December 2009 Equity Financing Series A		August 2010 Private Placement Investors and Placement Agent	
	June 30, 2014	December 31, 2013	June 30, 2014	December 31, 2013
Fair value (in thousands)	$-	$ 133	$860	$ 6,933
Black-Scholes inputs:				
Stock price	$11.55	$ 13.02	$11.55	$ 13.02
Exercise price	$2.45	$ 2.45	$3.08	$ 3.08
Risk free interest rate	0.07 %	0.13 %	0.29 %	0.33 %
Volatility	95.00%	95.00 %	95.00%	95.00 %
Expected term (years)	0.50	1.00	1.25	1.75
Dividend	-	-	-	-

9. STOCK OPTION PLANS

2010 Stock Incentive Plan

The Company's 2010 Stock Incentive Plan, as amended, provides for stock options, restricted shares or restricted share units to be granted to its employees, independent contractors, consultants or directors. During the three and six months ended June 30, 2014, the Company received approximately $0.5 million and $1.6 million, respectively, from the exercise of stock options. At June 30, 2014, there were 1,659,904 shares remaining available for issuance.

The Company recorded employee stock-based compensation expense as follows:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
(In thousands)	2014	2013	2014	2013
Cost of goods sold	$47	$-	$87	$-
Research and development	739	380	1,298	742
General and administrative	2,489	1,466	4,265	2,855
Total Stock-Based Compensation Expense	$3,275	$1,846	$5,650	$3,597

Table of Contents
RAPTOR PHARMACEUTICAL CORP.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
June 30, 2014
(Unaudited)

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:

	For the Three Months Ended June 30, 2014		For the Six Months Ended June 30, 2014	
	Option Shares	Weighted-average Exercise Price	Option Shares	Weighted-average Exercise Price
Beginning balance	9,584,383	$ 7.70	8,217,674	$ 6.05
Granted	597,988	8.40	2,383,718	13.17
Exercised	(91,528)	4.94	(464,205)	3.47
Canceled	(138,436)	11.39	(184,780)	14.69
Outstanding at June 30, 2014	9,952,407	7.72	9,952,407	7.72

10. COMMITMENTS AND CONTINGENCIES

The Company maintains 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 and distribution of PROCYSBI and clinical manufacturing of drug product for the Company's HD and NASH clinical collaborations. The Company's contractual obligations did not change significantly during the six months ended June 30, 2014 compared to those disclosed as of December 31, 2013.

11. SUBSEQUENT EVENTS

In July 2014, the Company agreed to sell $60 million aggregate principal amount of 8.0% convertible senior notes due 2019. These convertible notes require quarterly interest distributions at a fixed coupon rate equal of 8.0% until maturity or conversion, which will be no later than August 1, 2019. The convertible senior notes are convertible at the option of the holder at a conversion rate of 57.14 common shares per $1,000 principal amount of convertible senior notes at issuance (equivalent to a conversion price of $17.50 per common share), subject to adjustment in certain events. In addition, the convertible senior notes will automatically convert into shares of common stock if the price of the common stock is at or above 175% of the applicable conversion price over a 30 consecutive day period. Upon conversion of these convertible senior notes by a holder, the holder will receive shares of Raptor’s common stock.

In July 2014, the Company also entered into an amended and restated loan agreement with HC Royalty which revises the terms of the previous loan agreement dated as of December 20, 2012, between the Company and HC Royalty, and also provided for an additional $10 million in term loan funding. The interest rate has been revised to an annual fixed rate of 8.0%, compared to the original interest rate of 10.75%. The variable royalty rate under the amended and restated loan agreement has been revised to 8.0% on the first $50 million of revenue and 2.0% on revenue in excess of $50 million from the original rate of 12.25% on the first $25 million, 6.0% on the second $25 million of revenue, and 2.0% on revenue in excess of $50 million. The first payment of $3 million is due in June 2015. All term loans under the amended and restated loan agreement mature on March 31, 2020. The loan and the Company's obligation to make payments thereunder shall terminate immediately when all payments received by HC Royalty equal $120.0 million.

After deducting the commissions and offering costs, the Company received approximately $66 million of net proceeds from the convertible notes and amended and restated loan agreement.

Table of Contents

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

You should read the following discussion in conjunction with our condensed consolidated financial statements as of June 30, 2014, and the notes to such unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q. All references to "the Company," "we," "our" and "us" include the activities of Raptor Pharmaceutical Corp., Raptor Pharmaceuticals Inc. or Raptor Pharmaceuticals, Raptor European Products, LLC, RPTP European Holdings C.V., Raptor Pharmaceuticals Europe B.V., Raptor Pharmaceuticals France SAS and Raptor Pharmaceuticals Germany GmbH.

This Quarterly Report on Form 10-Q, including this "Management's Discussion and Analysis of Financial Condition and Results of Operations" section, contains "forward-looking statements," within the meaning of the Private Securities Litigation Reform Act of 1995.. In some cases, these statements can be identified by the use of terminology such as "believes," "expects," "anticipates," "plans," "may," "might," "will," "could," "should," "would," "projects," "predicts," "intends," "continues," "estimates," "potential," "opportunity" or the negative of these terms or other comparable terminology. All such statements, other than statements of historical 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, 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" 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 biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. On April 30, 2013, our first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules, or PROCYSBI, received marketing approval from the U.S. Food and Drug Administration, or FDA, for the management of nephropathic cystinosis in adults and children six years and older. On September 6, 2013, our European equivalent, PROCYSBI® gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received a Community or EU marketing authorization from the European Commission, or EC, as an orphan medicinal product for the management of proven nephropathic cystinosis. The EU marketing authorization allows us to commercialize PROCYSBI in the 28 Member States of the EU plus Norway, Liechtenstein, and Iceland (which are not EU Member States but are part of the European Free Trade Association, or EFTA). PROCYSBI received 7 years and 10 years of market exclusivity as an orphan drug in the U.S. and the EU, respectively. We commenced commercial sales of PROCYSBI in the U.S. in mid-June 2013 and in Germany in April 2014. With FDA approval of PROCYSBI and the commencement of commercial sales, we are no longer considered to be in the development stage.

Clinical Development Programs

Our three active clinical development programs utilize RP103, which contains the active pharmaceutical ingredient, cysteamine bitartrate. RP103 is our proprietary extended and delayed-release formulation capsule containing enteric coated microbeads of cysteamine bitartrate. Cysteamine bitartrate was approved in the U.S. in 1994 and the EU in 1997 as an orally available immediate-release powder in a capsule for the management of cystinosis. We have an exclusive worldwide license to delayed-release cysteamine bitartrate from the University of California, San Diego, or UCSD, which is the basis for our proprietary formulation of cysteamine. We currently have product candidates in clinical development designed to potentially treat Huntington's disease ("HD"), non-alcoholic steatohepatitis ("NASH"), and Leigh syndrome and other mitochondrial disorders.

Table of Contents

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.

Preclinical Product Candidates

Our preclinical programs, for which we may seek development partners in the future, include our cysteamine dioxygenase, or ADO, program and our HepTide™ program, for the potential treatment of hepatocellular carcinoma and other cancers susceptible to induced lysosomal storage.

Future Activities

Over the next fiscal year, our efforts will be focused on increasing sales of PROCYSBI in the U.S. and Germany; launching PROCYSBI in other countries in the EU; filing a New Drug Submission, or NDS, for cysteamine bitartrate delayed-release capsules with Health Canada in the second half of 2014; conducting a clinical trial to evaluate PROCYSBI in cystinosis patients that are cysteamine-naïve, as well as other supporting trials in underdeveloped markets; developing select global markets with significant numbers of known cystinosis patients; screening for undiagnosed and unidentified adult nephropathic cystinosis patients; supporting our regulatory pathways and/or clinical trials of RP103 for the potential treatment of HD, NASH, Leigh syndrome and mitochondrial disorders; preparing for potential clinical studies of RP103 in new therapeutic indications; supporting our novel preclinical programs; and identifying promising in-licensing candidates.

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 molecules discovered in-house and in-licensed and acquired technologies.

Results of Operations – Three and Six Months Ended June 30, 2014 and 2013

Revenue

For the three and six months ended June 30, 2014, we recognized $16.3 million and $28.4 million, respectively, in PROCYSBI net product sales. The first U.S. sales of PROCYSBI commenced in June 2013 and the launch of PROCYSBI in Germany commenced in April 2014. For the three months ended June 30, 2013, we recognized $21 thousand in PROCYSBI net product sales in the U.S.

Cost of Sales

Prior to FDA approval of PROCYSBI, our commercial manufacturing costs had been recorded as research and development expenses. As a result, our cost of sales for the next several quarters will reflect a lower average per unit cost of goods than will be recorded in the future. Cost of sales for the three and six months ended June 30, 2014 were $1.0 million and $2.3 million, respectively. Cost of sales primarily includes: raw materials and manufacturing costs for our commercial product PROCYSBI, amortization of licensing milestone payments, royalty fees due to UCSD on our net product sales and other indirect costs such as distribution, labeling, shipping and supplies. We began capitalizing commercial inventory costs upon FDA approval of PROCYSBI on April 30, 2013. Cost of sales for the three months ended June 30, 2013 were $0.4 million, as PROCYSBI became commercially available in the U.S. in June 2013.

Research and Development

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

Table of Contents

Research and development expenses increased approximately 78% to $11.1 million for the three months ended June 30, 2014 from $6.2 million during the three months ended June 30, 2013. The $4.9 million increase was primarily due to $1.9 million for increased staffing and personnel-related expenses for medical affairs, regulatory, pharmacovigilance activities; and $3.0 million to support projects and programs. Research and development expenses increased approximately 41% to $20.6 million for the six months ended June 30, 2014 from $14.6 million during the six months ended June 30, 2013. The increases were primarily due to an increase in staffing and associated salaries and benefits for medical, clinical, quality and regulatory personnel, as well as increased spending for preclinical studies, clinical trials and non-commercial drug manufacturing expenses.

Major program expenses recorded as research and development expenses:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
(In thousands)	2014	2013	2014	2013
RP103:				
Cystinosis (pre-commercial and extension)	$3,255	$3,015	$6,916	$7,357
HD (clinical)	335	161	838	374
NASH (clinical)	450	514	892	959
Preclinical programs	674	154	1,246	354
Other programs	605	247	1,258	501
R&D personnel and other costs not allocated to programs	5,758	2,124	9,474	5,082
Total Research and Development Expenses	$11,077	$6,215	$20,624	$14,627

Selling, General and Administrative Expenses

Selling, general and administrative expenses primarily include commercial expenses related to marketing and sales efforts in the U.S. and EU, including marketing and pricing studies, advertising, sales force commissions and other expenses, and market access support activities; commercial launch expenses for PROCYSBI, including patient support activities such as reimbursement assistance and establishing a customer relationship management system for our PROCYSBI sales team; intellectual property, legal and audit fees, finance, executive and commercial operations salaries and benefits; and other administrative and facilities costs.

Selling, general and administrative expenses increased approximately 42% to $13.3 million for the three months ended June 30, 2014 from $9.4 million in the three months ended June 30, 2013. The $3.9 million increase was primarily due to $3.2 million for increased staffing and personnel-related expenses to support commercial operations of PROCYSBI in the U.S., and for the launch of PROCYSBI in Germany, and $0.7 million of increased spending for external services to support commercial operations.

Selling, general and administrative expenses increased approximately 47% to $25.4 million for the six months ended June 30, 2014 from $17.2 million in the six months ended June 30, 2013. The $8.2 million increase was primarily due to $6.1 million for increased staffing and personnel-related expenses to support commercial operations for PROCYSBI in the U.S., for the establishment of our EU commercial headquarters and build out of our German commercial team in anticipation of the recent launch of PROCYSBI in Germany and $2.1 million of increased spending for external services to support commercial operations.

Interest Expense

Interest expense for the three months ended June 30, 2014 and 2013 was $3.5 million and $1.1 million, respectively. Interest expense for the six months ended June 30, 2014 and 2013 was $6.5 million and $1.8 million, respectively.

The increase in interest expense was due primarily to the $50.0 million loan agreement that we entered into with HealthCare Royalty Partners II, L.P., or HC Royalty, in December 2012. Interest expense for the six months ended June 30, 2014 also includes an interest expense royalty fee based on net sales for the quarter. We did not have significant product sales in the comparable period last year.

Table of Contents

Adjustment to the Fair Value of Common Stock Warrants

The adjustment to the fair value of common stock warrants was loss of $1.3 million for the six months ended June 30, 2014 compared to a loss of $6.0 million for the six months ended June 30, 2013. The loss for the six months ended June 30, 2014 was due primarily to the increase in the price of our stock through the dates that warrants were exercised during the quarter. The loss for the six months ended June 30, 2013 was due primarily to an increase in our stock price since December 31, 2012. At June 30, 2014, the remaining warrants outstanding were 334,764 compared to 2,212,615 at June 30, 2013.

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. 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 and results of operations.

Many of the following critical accounting policies require us to make significant judgments and estimates in the preparation of our consolidated financial statements.

Revenue Recognition and Accounts Receivable

We recognize revenue in accordance with the Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC 605, Revenue Recognition, when the following criteria have been met: persuasive evidence of an arrangement exists; delivery has occurred and risk of loss has passed; the seller's price to the buyer is fixed or determinable and collectability is reasonably assured. We determine that persuasive evidence of an arrangement exists based on written contracts that define the terms of the arrangements. Pursuant to the contract terms, we determine when title to products and associated risk of loss has passed onto the customer. We assess whether the fee is fixed or determinable based on the payment terms associated with the transaction and whether the sales price is subject to refund or adjustment. We assess collectability based primarily on the customer's payment history and creditworthiness.

PROCYSBI is currently available for U.S. distribution from our U.S. specialty pharmacy partner, the Accredo Health Group, Inc. (“Accredo”) which is currently our only U.S. customer and ships directly to patients. Our commercial launch in Germany commenced in April 2014, with the Almac Group, Ltd. as our distributor in the EU. PROCYSBI is not available in U.S. retail pharmacies. Prior authorization of coverage by patients' commercial insurance plans, our patient assistance program, or PAP, or government payors is a prerequisite to the shipment of PROCYSBI to U.S. patients. Revenue is recognized in the U.S. once the product has been shipped by the specialty pharmacy to patients because we have not yet been able to reasonably estimate the third-party payor mix and resulting rebates based on our lack of sufficient historical data. Billings to our distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenue by us until such shipments to patients occur. Revenue is currently recognized in the EU once the cash has been received as we have not yet been able to determine the payors’ credit worthiness.

We record revenue net of expected discounts, distributor fees, returns and rebates, including government rebates such as Medicare and Medicaid in the U.S. Allowances are recorded as a reduction of revenue at the time product sales are recognized. Allowances for government rebates and discounts are established based on the actual payor information, which is known in the U.S. and Germany at the time of shipment to patients, and the government-mandated discount

rates applicable to government-funded programs. The allowances are adjusted to reflect known changes in the factors that may impact such allowances in the quarter the changes are known.

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

Table of Contents

Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price, with cost determined on a first-in, first-out basis. Inventories are reviewed periodically to identify slow-moving inventory based on sales activity, both projected and historical, as well as product shelf-life. Prior to the approval of PROCYSBI by the FDA on April 30, 2013 and in Europe, prior to EC approval on September 6, 2013, we recorded the purchase of raw materials and the manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to FDA and EC approval, we began capitalizing these costs and manufacturing overhead as commercial inventory. Upon launching PROCYSBI in mid-June 2013 in the U.S. and April 2014 in the EU, we began recognizing cost of sales. Cost of sales includes the cost of inventory sold or reserved; manufacturing, manufacturing overhead and supply chain costs; product shipping and handling costs; amortization of licensing approval milestone payments and licensing royalties payable to UCSD.

We capitalize inventory produced in preparation for product launches when positive results have been obtained for the clinical trials that we believe are necessary to support regulatory approval and the we have determined it is probable that these capitalized costs will provide some future economic benefit in excess of capitalized costs. For these inventories, we also consider the expected approval date in assessing realizability. To the extent that inventory is expected to expire prior to being sold, we will write down the value of inventory. If actual results differ from those estimates, additional inventory write-offs may be required.

Note Payable

Note payable consists of payments under our December 2012 loan agreement with HC Royalty as lender, under which we borrowed $50.0 million in two $25.0 million tranches received in December 2012 and May 2013. During the reporting period, the loan bore interest at an annual fixed rate of 10.75% of outstanding principal and includes a synthetic royalty component based on net product sales, including PROCYSBI, in a calendar year. During the reporting period, with respect to the first $25.0 million tranche, for each calendar year, the loan bore a royalty rate of 6.25% of the first $25.0 million of product net revenues, 3.0% of product net revenues for such calendar year in excess of $25.0 million and up to $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly. During the reporting period, with respect to the second $25.0 million tranche, for each calendar year, the loan bore a royalty rate of 6.0% of the first $25.0 million of net revenues for such calendar year, 3.0% of product net revenues for such calendar year in excess of $25.0 million and up to $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly. The fixed and royalty interest are recognized as interest expense as incurred. The revenue royalty related interest may lead to significant fluctuations in interest expense from period to period.

Impairment of Goodwill and Intangible Assets

Goodwill represents the excess of the purchase price over the fair value of tangible and identified intangible net assets of businesses acquired. Goodwill is not amortized, but is evaluated for impairment on an annual basis or more often when impairment indicators are present. We have one reporting unit. Therefore, our consolidated net assets, including existing goodwill and other intangible assets, are considered to be the carrying value of the reporting unit. If the carrying value of the reporting unit is in excess of its fair value, an impairment may exist, and we must perform the second step of the analysis, in which the implied fair value of the goodwill is compared to its carrying value to determine the impairment charge, if any. If the estimated fair value of the reporting unit exceeds the carrying value of the reporting unit, goodwill is not impaired and no further analysis is required. We performed our goodwill impairment test as of December 31, 2013 and noted no impairment.

We make judgments about the recoverability of purchased intangible assets with finite lives whenever events or changes in circumstances indicate that impairment may exist. Recoverability of purchased intangible assets with finite lives is measured by comparing the carrying amount of the asset to the future undiscounted cash flows the asset

is expected to generate. Impairment, if any, is measured as the amount by which the carrying value exceeds the fair value of the impaired asset.

Assumptions and estimates about future values and remaining useful lives of our purchased intangible assets are complex and subjective. They can be affected by a variety of factors, including external factors such as industry and economic trends and internal factors such as changes in our business strategy and our internal forecasts.

Table of Contents

Common Stock Warrant Liabilities

The common stock warrants we issued in connection with certain fiscal year 2010 equity financings contain conditional obligations that may require us to transfer cash to settle the warrants upon the occurrence of certain fundamental transactions. Therefore, we have classified the warrants as liabilities. We re-measure the liability at the end of every reporting period with the change in value reported in our consolidated statements of operations. At the exercise date, the fair values of these warrants are re-measured and reclassified to equity.

We use the Black-Scholes option pricing model as our 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 our 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 our 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 operations as other income or expense, respectively. The primary factors affecting the fair value of the warrant liability are our stock price and volatility.

We reported a net loss of $12.7 million and $27.6 million and for the three and six months ended June 30, 2014, respectively. If our June 30, 2014 closing stock price had been 10% lower, our net loss would have been approximately $0.1 million lower. If our June 30, 2014 closing stock price had been 10% higher, our net loss would have been approximately $0.1 million higher.

A 10% increase or decrease of our volatility assumption for warrants at June 30, 2014 would not have had a material effect on our net loss due to the low number of warrants that remain outstanding at June 30, 2014.

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. 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. We intend to maintain the valuation allowance until sufficient positive evidence exists to support the reversal of the valuation allowance. Any decision to reverse part or all of the valuation allowance would be based on our estimate of future profitability.

We identify uncertain tax positions and record or disclose any resulting potential tax liability based upon whether the position is more likely than not sustainable upon examination. We consider proposed assessments by tax authorities, changes in facts and circumstances, issuance of new regulations or new case law and negotiations between tax authorities of different countries concerning our transfer prices or intellectual property transfers. As of June 30, 2014, we have identified no uncertain tax positions.

We file U.S. Federal, California, various other state and other income tax returns and various foreign country income tax returns. We are currently not subject to any income tax examinations. Due to our net operating losses, all tax years generally remain open in each jurisdiction.

Liquidity and Capital Resources

Capital Resource Requirements

As of June 30, 2014, we had approximately $58.1 million in cash and cash equivalents, approximately $25.0 million in current liabilities (of which approximately $0.9 million represented the common stock warrant liability, which is expected to be settled in shares) and approximately $50.9 million of net working capital.

Table of Contents

Additionally, in July 2014, we arranged a $70 million funding from HealthCare Royalty Partners (“HC Royalty”) and its affiliates. The funding includes $60 million in new convertible senior notes and an additional $10 million of funding pursuant to an amended and restated loan agreement. The convertibles notes require quarterly interest distributions at a fixed coupon rate equal of 8.0% until maturity or conversion, which will be no later than August 1, 2019. The convertible senior notes are convertible at the option of the holder at a conversion rate of 57.14 common shares per $1,000 principal amount of convertible senior notes at issuance (equivalent to a conversion price of $17.50 per common share), subject to adjustment in certain events. In addition, the convertible senior notes will automatically convert into shares of common stock if the price of the common stock is at or above 175% of the applicable conversion price over a 30 consecutive day period. Upon conversion of these convertible senior notes by a holder, the holder will receive shares Raptor’s common stock. After deducting the commissions and offering costs, we received approximately $66 million of net proceeds.

The company’s cash and cash equivalents, after the financing in July, are expected to be sufficient to fund operations through at least the first half of 2016, based on current operating plan assumptions.

The amended and restated loan agreement with HC Royalty revises the terms of the previous loan agreement dated as of December 20, 2012, between the Company and HC Royalty, and also provides for an additional $10 million in term loan funding. The base interest rate has been revised to an annual fixed rate of 8.0%, compared to the original interest rate of 10.75%. The variable royalty rate under the amended and restated loan agreement has been revised to 8.0% on the first $50 million of revenue and 2.0% of revenue in excess of $50 million from the original rate of 12.25% on the first $25 million and 6.0% on the second $25 million of revenue. All term loans under the amended and restated loan agreement mature on March 31, 2020.

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 million, from time to time through an "at the market," or ATM, 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. On July 3, 2013, we amended and restated the Sales Agreement to increase the aggregate gross sales proceeds that may be raised to $100 million. Cumulatively through June 30, 2014, we sold 7,599,474 shares under the ATM offerings at a weighted-average selling price of $7.08 per share for net proceeds of approximately $52.1 million. As of June 30, 2014, we had used approximately $53.8 million under the ATM.

Future Funding Requirements

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

the continuing sales of PROCYSBI in the U.S. and Germany, including patient uptake;

the ongoing costs of establishing the sales and marketing capabilities in the EU necessary to successfully launch PROCYSBI in Germany and other countries in the EU;

our ability to negotiate reimbursement and pricing of PROCYSBI in various countries in the EU;

the successful launch of PROCYSBI in other countries in the EU;

the cost of our manufacturing-related activities in support of PROCYSBI and RP103;

the cost of activities related to the regulatory submission of cysteamine bitartrate delayed-release capsules in Canada;

the cost of additional clinical trials in order to obtain regulatory approvals for PROCYSBI in non-U.S. and non-EU countries;

the timing and cost of our ongoing clinical programs for RP103, including: evaluating PROCYSBI in treatment-naïve cystinosis patients, and other supportive studies; evaluating RP103 as a potential treatment for HD; evaluating RP103 as a potential treatment for NASH; evaluating RP103 as a potential treatment for Leigh syndrome and other mitochondrial disorders;

the cost of regulatory submissions, as well as the preparation, initiation and execution of clinical trials in potential new clinical indication using RP103;

the cost of evaluating and potentially acquiring or in-licensing new drug compound(s) for potential clinical development;

the cost of business development activities to identify, test and potentially license or acquire new therapeutic drug candidates; and

the cost of filing, prosecuting and enforcing patent claims.

Table of Contents

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

Commitments and Contingencies

We maintain several contracts with drug labelers and distributors, research organizations, contract manufacturers, clinical organizations and clinical sites, primarily to assist with clinical research, clinical and commercial manufacturing of PROCYSBI and clinical manufacturing for our HD and our NASH clinical collaborations and our clinical study of RP103 in Leigh syndrome and other mitochondrial disorders. Our contractual obligations have not materially changed during the six months ended June 30, 2014 compared to those discussed in our Annual Report on Form 10-K for the year ended December 31, 2013, filed with the SEC on March 17, 2014.

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.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Our market risks during the six months ended June 30, 2014 have not materially changed from those discussed in our Annual Report on Form 10-K for the year ended December 31, 2013, filed with the SEC on March 17, 2014.

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. Our disclosure controls and procedures were designed to provide reasonable assurance of achieving our control objectives.

Changes in Internal Control over Financial Reporting

During the second quarter of 2014, there were no changes in our internal control over financial reporting that have materially affected or are reasonably likely to have a material impact on 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.

An investment in our common stock involves a high degree of risk. Before investing in our common stock, you should carefully consider the following risk factors and the additional risk factors included in our Annual Report on Form 10-K for the year ended December 31, 2013 filed with the Securities and Exchange Commission on March 17, 2014 (the "Annual Report"), together with all of the other information included in this Quarterly Report on Form 10-Q as well as our other publicly available filings with the SEC. If any of these risks occur, our business, results of operations and financial condition could be harmed, the price of our common stock could decline, and you may lose part or all of your investment. There have been no material changes to the risk factors included in our Annual Report except as noted below.

Risks Associated with Commercialization and Product Development

We currently depend entirely on the commercial success of our lead drug, PROCYSBI, for the management of nephropathic cystinosis.

PROCYSBI is our only product currently approved for marketing and, as a result, our operating results are substantially dependent on the commercial success of PROCYSBI, for which we commenced marketing in the U.S. in June 2013 and in Germany in April 2014. In the U.S., we are permitted to market PROCYSBI only for the management of nephropathic cystinosis in adults and children six years and older. In September 2013, we received marketing authorization from the EC, which allows us to commercialize PROCYSBI in the 28 Member States of the EU plus Norway, Liechtenstein and Iceland (which are not EU Member States but are part of the European Free Trade Association, or EFTA), for the treatment of proven nephropathic cystinosis; however, we only recently commenced our commercial launch of PROCYSBI in Germany and have not yet launched in any other country in the EU. We believe that the trading price of our common stock will be substantially affected by our results of operations and, in particular, net product sales of PROCYSBI. We do not have prior experience in commercializing therapeutics. If PROCYSBI sales do not meet expectations, our stock price may not increase or could significantly decrease. The successful commercialization of PROCYSBI will depend on several factors, including:

the success of our ongoing commercial launch of PROCYSBI in Germany;

the negotiation and agreement on an acceptable prices in EU countries and other select territories, and reimbursement at the country-specific price;

the successful commercial launch of PROCYSBI in the EU and other select territories;

acceptance of PROCYSBI by physicians, parents, patients and cystinosis research/advocacy organizations including the conversion from the existing standard of care to PROCYSBI;

coverage and reimbursement for PROCYSBI from commercial health plans and government health programs, which we refer to collectively as third-party payors;

compliance with regulatory requirements including fulfilling any FDA and EC required post-approval commitments;

provision of affordable out-of-pocket cost to patients and/or other programs to ensure patient access to PROCYSBI in the U.S.;

approval by regulatory agencies in other countries of appropriate product labeling for PROCYSBI;

agreements with wholesalers, distributors and pharmacies on commercially reasonable terms;

manufacture and supply of adequate quantities of PROCYSBI to meet commercial demand; and

development and maintenance of intellectual property protection for PROCYSBI.

Table of Contents

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

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

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

Beginning April 1, 2013, Medicare payments for all items and services under Part A and B, including drugs and biologicals, and most payments to plans under Medicare Part D were reduced by 2% under the automatic spending reductions, or sequestration, required by the Budget Control Act of 2011, or BCA, as amended by the American Taxpayer Relief Act, or ATRA. The BCA required sequestration for most federal programs, excluding Medicaid, Social Security and certain other programs, because Congress failed to enact legislation by January 15, 2012 to reduce federal deficits by $1.2 trillion over 10 years. As long as BCA cuts remain in effect, they could adversely impact payment for PROCYSBI. In addition, other recent legislative changes that increase manufacturer liability for rebates and other payments under the 340B drug pricing program, the Medicaid Drug Rebate Program and the Medicare Part D prescription drug benefit also could impact our revenues. See the risk factor in our Annual Report titled "Enacted and future legislation may increase the difficulty and cost for us to commercialize PROCYSBI or any other product candidate that we develop and affect the prices we may obtain."

Further, payors also are increasingly considering new metrics as the basis for reimbursement rates, such as average sales price, or ASP, average manufacturer price, or AMP, or actual acquisition cost, or AAC. Although the intent of the changes to reimbursement methodologies generally is to limit payment increases, it is difficult to project the impact of these and other alternative reimbursement methodologies on the willingness of payors to reimburse PROCYSBI and any product candidates that we may develop. Although to date PROCYSBI has been reimbursed in the U.S. and Germany, we do not know whether third-party payors will reimburse PROCYSBI in the other EEA countries or continue to reimburse PROCYSBI in the U.S. and whether third-party payors will reimburse RP103 and our future products for future commercial indications until we enter into payor negotiations. If coverage and reimbursement are not available or available only to limited levels, we may not be able to generate sufficient revenue to meet our operating costs or to achieve our revenue, cash flow breakeven or profitability goals in the timeframe that we expect, or at all.

As we expand our development and commercialization activities outside of the U.S., we will be subject to an increased risk of inadvertently conducting activities in a manner that violates the U.S. Foreign Corrupt Practices Act, or FCPA, and similar laws. If that occurs, we may be subject to civil or criminal penalties which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We are subject to the FCPA, which prohibits corporations and individuals from paying, offering to pay, or authorizing the payment of anything of value to any foreign government official, government staff member, political party, or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. We are also subject to the UK Bribery Act, which prohibits both domestic and international bribery, as well as bribery across both public and private sectors.

Table of Contents

In the course of establishing and expanding our commercial operations and seeking regulatory approvals outside of the U.S., we will need to establish and expand business relationships with various third parties, such as independent contractors, distributors, vendors, advocacy groups and physicians, and we will interact more frequently with foreign officials, including regulatory authorities and physicians employed by state-run healthcare institutions who may be deemed to be foreign officials under the FCPA, UK Bribery Act or similar laws of other countries that may govern our activities. Any interactions with any such parties or individuals where compensation is provided that are found to be in violation of such laws could result in substantial fines and penalties and could materially harm our business. Furthermore, any finding of a violation under one country's laws may increase the likelihood that we will be prosecuted and be found to have violated another country's laws. If our business practices outside the U.S. are found to be in violation of the FCPA, UK Bribery Act or other similar law, we may be subject to significant civil and criminal penalties which could have a material adverse effect on our business, financial condition and results of operations.

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

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

In the EU, the European Medicine Agency’s, or EMA's, Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU Community and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or the product would be a significant benefit to those affected). In addition, designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the medicinal product. An EU orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

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

generic version upon the expiration of orphan exclusivity, if our patent position is not upheld.

Even though we have been granted orphan drug designation in the U.S. and EU prior to the approval of RP103 for the potential treatment of HD, and even if we obtain orphan drug designation for our future drug product candidates, we may not fulfill the criteria for exclusivity or we may not be the first to obtain marketing approval for any orphan indication. Further, even if we obtain orphan drug exclusivity for a particular product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve a drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. The FDA can discontinue orphan drug exclusivity after it has been granted if the orphan drug cannot be manufactured in sufficient quantities to meet demand. Positive clinical trial results in any of our RP103 programs increase the risk that immediate-release cysteamine bitartrate may be used off-label in those indications in certain geographic areas due to immediate-release cysteamine bitartrate's lower cost and our 505(b)(2) filing status.

Table of Contents

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

We rely on a third-party logistics provider and specialty pharmacy to distribute PROCYSBI to patients in the U.S. and to pharmacies in Germany and to collect from insurance companies and government agencies in the U.S. and from pharmacies in the EU. Our ability to collect from the U.S. logistics provider is not only subject to such provider's credit worthiness but is also dependent, in part, on its ability to arrange for full reimbursement from third-party payors. The outsourcing of our distribution function is complex, and we may experience difficulties that could reduce, delay or stop shipments of PROCYSBI. If we encounter such distribution problems, and we are unable to quickly enter into a similar agreement with another specialty distributor on substantially similar terms, if at all, the distribution of PROCYSBI could become disrupted, resulting in reduced revenues, healthcare provider dissatisfaction and/or patient dissatisfaction which may harm our reputation and financial condition.

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

In December 2012, we entered into a loan agreement with HC Royalty as lender, under which we agreed to borrow $50.0 million in two $25.0 million tranches, or the original HC Royalty loan agreement. We drew down the first tranche in the amount of $25.0 million in December 2012 upon signing the original HC Royalty loan agreement and we drew down the second tranche of $25.0 million in May 2013 as a result of our achievement of the milestone of U.S. approval of PROCYSBI. On July 1, 2014, we entered into an amended and restated loan agreement with HC Royalty as lender, or the HC Royalty loan agreement, under which we borrowed from HC Royalty a third $10.0 million tranche on July 23, 2014. The HC Royalty loan agreement includes a number of affirmative and negative covenants, including the use of commercially reasonable efforts to exploit PROCYSBI and RP103 in specific markets and compliance with laws, as well as restrictions on mergers and sales of assets, incurrence of liens, incurrence of indebtedness and transactions with affiliates and other requirements. Our performance of our obligations under the HC Royalty loan agreement is secured by our grant of a security interest to HC Royalty in substantially all of our assets, the assets of our domestic subsidiaries and a pledge of stock of certain of our domestic 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 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. A default or material adverse effect or change of control would also trigger a prepayment penalty which would require us to pay a substantially higher amount due than the current balance of our loan.

Risks Related to Our Financial Position and Capital Requirements

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 agreement 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 8.0% and a synthetic royalty based on the amount of PROCYSBI and other future approved product net revenues in a calendar year, and such interest and royalty are payable quarterly. Principal payments under the HC Royalty loan agreement will become due beginning in June 2015.

Table of Contents

There is no assurance that our business will generate sufficient cash flow or that we will have capital resources in an amount sufficient to enable us to pay our indebtedness to HC Royalty. If our cash flows and capital resources are insufficient to fund these debt service obligations, we may be forced to reduce or delay product development, sales and marketing, and capital and other expenditures, and we may be forced to restructure our indebtedness or raise additional capital through the issuance of equity or debt instruments. We cannot ensure that we will be able to refinance any of our indebtedness or raise additional capital on a timely basis, in sufficient amounts, on satisfactory terms or at all. In addition, the terms of the HC Royalty loan agreement may limit our ability to pursue any of these financing alternatives and these alternatives may not enable us to meet our scheduled debt service obligations. Failure to meet our debt service obligations may result in an event of default under the HC Royalty loan agreement, 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. A default or material adverse effect or change of control would also trigger a prepayment penalty which would require us to pay a substantially higher amount due than the current balance of our loan. This could have a material adverse impact on our financial condition and results of operations.

Risks Related to Our Common Stock

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

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

We have entered into an Amended and Restated Sales Agreement with Cowen and Company, which, if utilized further, will create substantial dilution for our existing stockholders. The original Sales Agreement provided for at-the-market sales of our common stock with aggregate gross proceeds of up to $40.0 million. On July 3, 2013, we entered into an Amended and Restated Sales Agreement to increase the aggregate gross sales proceeds that may be raised pursuant to the agreement to $100.0 million. As of June 30, 2014, we had used approximately $53.8 million under the “at the market” offering program.

In July 2014, we issued $60.0 million aggregate principal amount of 8.0% convertible senior notes due 2019. The convertible senior notes are convertible at the option of the holder at a conversion rate of 57.14 shares of common stock per $1,000 principal amount of convertible senior notes, which is equivalent to an initial conversion price of $17.50, and is subject to adjustment in certain events. In addition, the convertible senior notes will automatically convert into shares of common stock if the price of the common stock is at or above 175% of the applicable conversion price over a 30 consecutive day period.

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

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

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES.

None.

Table of Contents

ITEM 4. MINE SAFETY DISCLOSURES.

None.

ITEM 5. OTHER INFORMATION.

None.

ITEM 6. EXHIBITS

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

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.

Date: August 7, 2014 By: /s/ Christopher M. Starr
Christopher M. Starr, Ph.D.
Chief Executive Officer and Director
(Principal Executive Officer)

Date: August 7, 2014 By: /s/ Georgia Erbez
Georgia Erbez
Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer and Principal Accounting Officer)

Table of Contents

Exhibit Index

Exhibit Number	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
2.1	Agreement and Plan of Merger and Reorganization, dated June 7, 2006, among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc.	S-4 (333-136018)	7/25/2006	Annex A	
2.2	Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated August 25, 2006, among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc.	S-4/A (333-136018)	8/25/2006	Annex A	
2.3	Agreement and Plan of Merger and Reorganization, dated July 27, 2009, among ECP Acquisition, Inc., Raptor Pharmaceuticals Corp. and TorreyPines Therapeutics, Inc.	8-K	7/28/2009	2.1	
3.1	Certificate of Incorporation of Registrant	8-K	10/10/2006	3.1	
3.2	Amended and Restated Bylaws of Registrant	8-K	2/26/2014	3.1	
3.3	Certificate of Amendment filed with the Secretary of State of the State of Nevada effecting an 8-for-1 reverse stock of Registrant's common stock and changing the name of Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc.	8-K	10/10/2006	3.3	
3.4	Articles of Conversion filed with the Secretary of State of the State of Nevada changing the state of incorporation of Registrant	8-K	10/10/2006	3.4	
3.5	Certificate of Conversion filed with the Secretary of State of the State of Delaware	8-K	10/10/2006	3.5	
3.6	Certificate of Amendment of Certificate of Incorporation of Registrant	8-K	10/5/2009	3.1	
3.7	Certificate of Merger of ECP Acquisition, Inc. with and into Registrant	8-K	10/5/2009	3.2	
4.1	Specimen common stock certificate of the Registrant	8-K/A	10/7/2009	4.7	
4.2(a)	Rights Agreement, dated as of May 13, 2005, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	8-K	5/16/2005	99.2	
4.2(b)	Amendment to Rights Agreement, dated as of June 7, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	8-K	6/12/2006	4.1	
4.2(c)	Amendment to Rights Agreement, dated as of October 3, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	10-K	3/29/2007	4.19	
4.2(d)	Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between Registrant and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company)	8-K	7/28/2009	2.3	
4.2(e)	Amendment to Rights Agreement, dated August 6, 2010, by and between Registrant and American Stock Transfer & Trust Company, LLC	8-K	8/10/2010	4.2	
4.3	Form of Warrant, dated September 27, 2005, issued to Oxford Financial and Silicon Valley Bank.	10-K	3/29/2007	4.16	
4.4*	Warrant, dated December 14, 2007, issued to Flower Ventures, LLC.	10-QSB**	4/15/2008	4.1	
4.5*	Warrant Agreement Amendment, dated December 17, 2009, between Flower Ventures, LLC and the Registrant.	10-Q	4/9/2010	4.15	

35

Table of Contents

31.1	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer	X
31.2	Certification of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer	X
32.1	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer, and Georgia Erbez, Chief Financial Officer, Secretary and Treasurer	X
101	The following materials from the Raptor Pharmaceutical Corp. Quarterly Report on Form 10-Q for the quarter ended June 30, 2014, formatted in Extensible Business Reporting Language (XBRL): (i) the Condensed Consolidated Balance Sheets; (ii) the Condensed Consolidated Statements of Operations; (iii) the Condensed Consolidated Statements of Comprehensive Loss; (iv) the Condensed Consolidated Statements of Cash Flows; and (v) related notes, tagged as blocks of text.	X

* The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.4 - 4.5 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.

** Incorporated by reference from the indicated filing of Raptor Pharmaceuticals Corp. rather than that of the Registrant