

Raptor Pharmaceutical Corp
Form 10-Q/A
May 12, 2014

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

FORM 10-Q/A
(Amendment No. 2)

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

For the quarterly period ended March 31, 2013

or

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

For the transition period from _____ to _____

Commission File Number: 000-25571

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

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

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

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

9 Commercial Blvd. Suite 200, Novato, CA 94949
(Former name, former address and former fiscal year, if changed since last report)

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

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

Edgar Filing: Raptor Pharmaceutical Corp - Form 10-Q/A

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

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

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

There were 62,616,859 shares of the registrant's common stock, par value \$0.001, outstanding as of April 30, 2014.

EXPLANATORY NOTE

As previously disclosed by Raptor Pharmaceutical Corp., or the “Company,” in its current report on Form 8-K filed with the U.S. Securities and Exchange Commission (the “Commission”) on January 22, 2014, the Company has engaged Grant Thornton LLP (“Grant Thornton”) to replace Burr Pilger Mayer, Inc. (“BPM”) to serve as the Company’s independent registered public accounting firm.

The Company is filing this Amendment No. 2 (the “Form 10-Q/A”) to its Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2013, filed with the Commission on May 8, 2013 (the “Form 10-Q”), as previously amended by Amendment No. 1 to Form 10-Q filed with the Commission on June 19, 2013, to include Grant Thornton’s review report with respect to the interim financial statements included in this Form 10-Q/A.

In addition to changes to the financial statements and notes thereto included in this Form 10-Q/A, which are to conform to the presentation of the 2013 financial statements included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2013 (filed with the Commission on March 17, 2014), the Company has amended (i) Part I, Item 2 to delete its previous disclosure relating to an explanatory paragraph regarding the Company’s ability to continue as a going concern because this explanatory paragraph was removed from BPM’s reissued reports for such periods due to changes in the Company’s circumstances, (ii) Part II, Item 4 to include an affirmative statement that the Company’s disclosure controls and procedures were designed to provide reasonable assurance of achieving its control objectives, and (iii) Part II, Item 6, to make changes to the exhibits.

Except as specifically noted above, this Form 10-Q/A does not modify or update disclosures in the Form 10-Q, and there have been no other material changes to the disclosures made in the Form 10-Q as of the filing of the Form 10-Q. Accordingly, except as specifically noted above, this Form 10-Q/A does not reflect events occurring after the filing of the Form 10-Q or modify or update any related or other disclosures.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

FORM 10-Q FOR THE QUARTER ENDED MARCH 31, 2013

Table of Contents

	Page
Part 1 - Financial Information	
<u>Report of Independent Registered Public Accounting Firm</u>	2
Item 1 <u>Financial Statements</u>	3
<u>Unaudited Condensed Consolidated Balance Sheets as of March 31, 2013 and December 31, 2012</u>	3
<u>Unaudited Condensed Consolidated Statements of Operations and Comprehensive Loss for the quarters ended March 31, 2013 and February 29, 2012 and the cumulative period from September 8, 2005 (inception) to March 31, 2013</u>	4
<u>Unaudited Condensed Consolidated Statement of Stockholders' Equity for the quarter ended March 31, 2013</u>	5
<u>Unaudited Condensed Consolidated Statements of Cash Flows for the quarters ended March 31, 2013 and February 29, 2012 and the cumulative period from September 8, 2005 (inception) to March 31, 2013</u>	6
<u>Notes to Condensed Consolidated Financial Statements</u>	7
Item 2 <u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	21
Item 3 <u>Quantitative and Qualitative Disclosures About Market Risk</u>	30
Item 4 <u>Controls and Procedures</u>	30
Part II - Other Information	
Item 1 <u>Legal Proceedings</u>	31
Item 1A <u>Risk Factors</u>	31
Item 2 <u>Unregistered Sales of Equity Securities and Use of Proceeds</u>	49
Item 3 <u>Defaults Upon Senior Securities</u>	49
Item 4 <u>Mine Safety Disclosures</u>	49
Item 5 <u>Other Information</u>	49
Item 6 <u>Exhibits</u>	49
<u>SIGNATURES</u>	50

Table of Contents

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

Board of Directors and Shareholders
Raptor Pharmaceutical Corp.

We have reviewed the accompanying condensed consolidated balance sheet of Raptor Pharmaceutical Corp. (a Delaware corporation) and subsidiaries (the "Company"), and the related condensed consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows, as of March 31, 2013 and for the three-month period then ended. These interim financial statements are the responsibility of the Company's management.

We conducted our review in accordance with the standards of the Public Company Accounting Oversight Board (United States). A review of interim financial information consists principally of applying analytical procedures and making inquiries of persons responsible for financial and accounting matters. It is substantially less in scope than an audit conducted in accordance with the standards of the Public Company Accounting Oversight Board (United States), the objective of which is the expression of an opinion regarding the financial statements taken as a whole. Accordingly, we do not express such an opinion.

Based on our review, we are not aware of any material modifications that should be made to the condensed consolidated interim financial statements referred to above for them to be in conformity with accounting principles generally accepted in the United States of America.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheet of the Company as of December 31, 2012, and the related consolidated statements of operations and comprehensive loss, stockholders' equity (deficit), and cash flows for the four month period then ended (not presented herein); and we expressed an unqualified opinion on those consolidated financial statements in our report dated March 17, 2014. In our opinion, the information set forth in the accompanying condensed consolidated balance sheet as of December 31, 2012, is fairly stated, in all material respects, in relation to the consolidated balance sheet from which it has been derived.

/s/ Grant Thornton LLP
San Francisco, California
March 17, 2014
2

Table of Contents

PART I - FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS. Raptor Pharmaceutical Corp.

(A Development Stage Company)

Condensed Consolidated Balance Sheets

(Unaudited)

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

	March 31, 2013	December 31, 2012
ASSETS		
Current assets:		
Cash and cash equivalents	\$36,288	\$36,313
Restricted cash	213	163
Short-term investments	22,123	22,096
Prepaid expenses and other	1,417	1,610
Total current assets	60,041	60,182
Intangible assets, net	2,120	2,156
Goodwill	3,275	3,275
Fixed assets, net	492	416
Deposits	220	26
Other assets	1,977	2,068
Total assets	\$68,125	\$68,123
LIABILITIES AND STOCKHOLDERS' EQUITY		
Liabilities		
Current liabilities:		
Accounts payable	\$2,716	\$4,599
Accrued liabilities	4,564	2,150
Common stock warrant liability	12,708	16,405
Deferred rent	0	6
Capital lease liability – current	9	8
Total current liabilities	19,997	23,168
Note payable	25,000	25,000
Capital lease liability - long-term	9	11
Total liabilities	45,006	48,179
Commitments and contingencies – see Note 8		
Stockholders' equity:		
Preferred stock, \$0.001 par value per share, 15,000,000 shares authorized, zero shares issued and outstanding	0	0
Common stock, \$0.001 par value per share, 150,000,000 shares authorized, 55,431,651 and 52,424,649 shares issued and outstanding at March 31, 2013 and December 31, 2012, respectively	55	52

Edgar Filing: Raptor Pharmaceutical Corp - Form 10-Q/A

Additional paid-in capital	175,132	155,945
Accumulated other comprehensive loss	(203)	(115)
Deficit accumulated during development stage	(151,865)	(135,938)
Total stockholders' equity	23,119	19,944
Total liabilities and stockholders' equity	\$68,125	\$68,123

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

3

Table of Contents

Raptor Pharmaceutical Corp.

(A Development Stage Company)

Condensed Consolidated Statements of Operations and Comprehensive Loss

(Unaudited)

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

	For the quarter ended		For the period from September 8, 2005 (inception) to
	March 31, 2013	February 29, 2012	March 31, 2013
Revenues:	\$0	\$0	\$0
Operating expenses:			
Research and development	8,412	3,971	78,056
General and administrative	7,863	2,452	48,410
Total operating expenses	16,275	6,423	126,466
Loss from operations	(16,275)	(6,423)	(126,466)
Interest income	155	107	1,028
Interest expense	(726)	(0)	(928)
Foreign currency transaction gain (loss)	(34)	18	253
Gain (loss) on short-term investments	(107)	120	41
Adjustment to fair value of common stock warrants	1,060	(7,814)	(25,793)
Net loss	(15,927)	(13,992)	(151,865)
Other comprehensive loss			
Foreign currency translation adjustment	(88)	(2)	(203)
Comprehensive loss	\$(16,015)	\$(13,994)	\$(152,068)
Net loss per share:			
Basic and diluted	\$(0.30)	\$(0.29)	
Weighted-average shares outstanding used to compute:			
Basic and diluted	53,713	47,967	

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

Table of Contents

Raptor Pharmaceutical Corp.

(A Development Stage Company)

Condensed Consolidated Statement of Stockholders' Equity

For the Quarter Ended March 31, 2013

(Unaudited)

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

	Common Shares	Common stock Amount	Additional paid- in capital	Accumulated other comprehensive loss	Deficit accumulated during development stage	Total
Balance at December 31, 2012	52,425	\$ 52	\$ 155,945	\$ (115)	\$ (135,938)	\$ 19,944
Exercise of common stock warrants	600	1	1,844	0	0	1,845
Exercise of common stock options	8	0	7	0	0	7
Employee stock-based compensation expense	0	0	1,748	0	0	1,748
Consultant stock-based compensation expense	0	0	4	0	0	4
Reclassification of the fair value of warrant liabilities upon exercise	0	0	2,636	0	0	2,636
Issuance of common stock under an at-the-market sales agreement, net of commissions of \$185	2,399	2	12,948	0	0	12,950
Foreign currency translation loss	0	0	0	(88)	0	(88)
Net loss	0	0	0	0	(15,927)	(15,927)
Balance at March 31, 2013	55,432	\$ 55	\$ 175,132	\$ (203)	\$ (151,865)	\$ 23,119

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

Table of Contents

Raptor Pharmaceutical Corp.

(A Development Stage Company)

Condensed Consolidated Statements of Cash Flows

(Unaudited)

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

	For the quarter ended		For the cumulative period from September 8, 2005 (inception) to
	March 31, 2013	February 29, 2012	March 31, 2013
Cash flows from operating activities:			
Net loss	\$(15,927)	\$(13,992)	\$(151,865)
Adjustments to reconcile net loss to net cash used in operating activities:			
Employee stock-based compensation expense	1,748	1,104	11,816
Consultant stock-based compensation expense	4	0	768
Fair value adjustment of common stock warrants	(1,060)	7,814	25,793
Amortization of intangible assets	36	37	782
Depreciation of fixed assets	36	9	644
Gain (loss) on short-term investments	107	(67)	171
Write-off of intangible assets and other intellectual property	0	0	1,249
Amortization of debt issuance costs	54	0	54
Changes in assets and liabilities:			
Prepaid expenses and other	193	(2,009)	(1,177)
Intangible assets	0	0	(150)
Deposits	(194)	0	(220)
Accounts payable	(1,883)	(1,178)	2,716
Accrued liabilities	2,414	717	3,884
Deferred rent	(6)	4	0
Net cash used in operating activities	(14,478)	(7,561)	(105,535)
Cash flows from investing activities:			
Purchase of fixed assets	(112)	(162)	(1,092)
Cash acquired in 2009 Merger	0	0	581
Change in restricted cash	(50)	(50)	(213)
Purchase of short-term investments	(134)	(156)	(52,294)
Sale of short-term investments	0	0	30,000
Net cash used in investing activities	(296)	(368)	(23,018)
Cash flows from financing activities:			
Proceeds from the sale of common stock	0	0	85,941

Edgar Filing: Raptor Pharmaceutical Corp - Form 10-Q/A

Proceeds from the sale of common stock under an equity line	0	(2)	4,201
Proceeds from the sale of common stock under an ATM agreement	12,949	0	26,318
Proceeds from the exercise of common stock warrants	1,845	2,820	24,598
Proceeds from the exercise of common stock options	7	67	734
Note payable	0	0	25,000
Debt issuance costs	(3)	0	(1,962)
Offering costs	40	0	(70)
Proceeds from the sale of common stock to initial investors	0	0	310
Payments on capital lease	(1)	(3)	(26)
Net cash provided by financing activities	14,837	2,884	165,044
Effect of exchange rates on cash and cash equivalents	(88)	2	(203)
Net increase (decrease) in cash and cash equivalents	(25)	(5,043)	36,288
Cash and cash equivalents, beginning of period	36,313	24,732	0
Cash and cash equivalents, end of period	\$36,288	\$19,689	\$36,288
Supplemental cash flow information:			
Interest paid	\$672	\$0	\$772
Supplemental disclosure of non-cash financing activities:			
Warrants issued in connection with financing	\$0	\$0	\$16,310
Initial fair value of warrants issued to placement agents in connection with financings	\$0	\$0	\$209
Common stock and warrants issued in connection with reverse merger	\$0	\$0	\$4,417
Common stock issued as fee for equity line	\$0	\$0	\$828
Fair value of warrant liability reclassified to equity upon exercise	\$2,636	\$6,613	\$22,969
Acquisition of equipment in exchange for capital lease	\$0	\$0	\$48
Notes receivable issued in exchange for common stock	\$0	\$0	\$110
Common stock issued for a finder's fee	\$0	\$0	\$102
Common stock issued in asset purchase	\$0	\$0	\$2,899

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

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

1. DESCRIPTION OF BUSINESS AND SIGNIFICANT ACCOUNTING POLICIES

Raptor Pharmaceutical Corp. (the “Company” or “Raptor”) is a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat rare, debilitating and often fatal diseases. Raptor’s first product, PROCYSBI™ (cysteamine bitartrate) delayed-release capsules received marketing approval from the U.S. Food and Drug Administration (“FDA”) on April 30, 2013 for the management of nephropathic cystinosis (“cystinosis”) in adults and children six years and older. Cystinosis is a rare metabolic lysosomal storage disease. The Company plans to launch PROCYSBI™ in the U.S. by the end of the second quarter of 2013. The Company anticipates receiving a determination from the European Medicines Agency (“EMA”) regarding Raptor’s application for marketing approval of PROCYSBI™ in the EU in the second half of 2013. Raptor’s pipeline also includes RP103 in Phase 2/3 development for Huntington's disease and RP103 in Phase 2b development for nonalcoholic fatty liver disease (“NAFLD”) in children. Raptor's preclinical programs are based upon novel drug candidates that are designed to treat primary liver cancer and various other diseases.

The Company is subject to a number of risks, including: the success of the U.S. launch of PROCYSBITM; the approval of PROCYSBI™ in the EU; the need to raise capital through equity and/or debt financings; the uncertainty of whether the Company's research and development efforts will result in successful commercial products; competition from larger organizations; reliance on licensing the proprietary technology of others; dependence on key personnel; uncertain patent protection; and dependence on corporate partners and collaborators. Funding may not be available when needed if at all or on terms acceptable to us. 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.

Change in Fiscal Year End

In December 2012, Raptor's board of directors approved a change in the Company's fiscal year end from August 31 to December 31, effective on December 31, 2012. The accompanying condensed consolidated financial statements cover the period from January 1, 2013 through March 31, 2013, representing the first quarter of Raptor's newly adopted fiscal year. The prior fiscal year quarter ended February 29, 2012 is reported on the basis of Raptor’s previous fiscal year cycle. As a result of the change in Raptor’s fiscal year end, the quarterly periods of Raptor’s newly adopted fiscal year do not coincide with the historical quarterly periods that Raptor had previously reported.

Basis of Presentation

The accompanying condensed consolidated financial statements reflect the results of operations of Raptor and have been prepared in accordance with the accounting principles generally accepted in the U.S. (“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, 2012 has been derived from the Company’s audited financial statements as of such date but does not include all disclosures required by GAAP. This Form 10-Q/A should be read in conjunction with the audited financial statements and accompanying notes in the Company's Transition Report on Form 10-KT/A for the four-month period ended December 31, 2012 as amended.

The Company's condensed consolidated financial statements include the accounts of the Company's direct and indirect wholly owned subsidiaries, Raptor Pharmaceuticals Inc., formerly known as Raptor Therapeutics Inc. which merged with Raptor Discoveries Inc. in December 2012 prior to changing its name and Raptor European Products, LLC, such subsidiaries incorporated in Delaware on August 1, 2007, and February 14, 2012, respectively, and Raptor Pharmaceuticals Europe B.V. (domiciled in the Netherlands and formed on December 15, 2009), Raptor Pharmaceuticals France SAS (formed in France on October 18, 2012) and RPTP European Holdings C.V. (located in the Grand Caymans and formed on February 16, 2012). All intercompany accounts have been eliminated.

Use of Estimates

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

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

Comprehensive Loss

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

Cash and Cash Equivalents

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

Short-term Investments

The Company invests in short-term investments in high credit-quality funds in order to obtain higher yields on its cash available for investment. Short-term investments as of March 31, 2013 and December 31, 2012 consisted of approximately \$22.1 million invested in a short duration government fund.

Such investments are not insured by the Federal Deposit Insurance Corporation. The Company completed an evaluation of its investments and determined that it did not have any other-than-temporary impairments as of March 31, 2013 and December 31, 2012.

Functional Currency

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

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 values 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.

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

Level one - Quoted market prices in active markets for identical assets or liabilities;

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

Level three - Unobservable inputs developed using estimates and assumptions, which are developed by the reporting entity and reflect those assumptions that a market participant would use.

8

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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 March 31, 2013 and December 31, 2012 are summarized as follows:

(In thousands)

Assets	Level 1	Level		March
		2	Level 3	31, 2013
Fair value of cash equivalents	\$33,013	\$ 0	\$0	\$ 33,013
Short-term investments	22,123	0	0	22,123
Total	\$55,136	\$ 0	\$0	\$ 55,136
Liabilities				
Fair value of common stock warrants	\$0	\$ 0	\$12,708	\$ 12,708
Total	\$0	\$ 0	\$12,708	\$ 12,708
Assets	Level 1	Level		December
		2	Level 3	31, 2012
Fair value of cash equivalents	\$35,069	\$ 0	\$0	\$ 35,069
Short-term investments	22,096	0	0	22,096
Total	\$57,165	\$ 0	\$0	\$ 57,165
Liabilities				
Fair value of common stock warrants	\$0	\$ 0	\$16,405	\$ 16,405
Total	\$0	\$ 0	\$16,405	\$ 16,405

See Note 5 for further discussion on the calculation of the fair value of the common stock warrant liability.

Intangible Assets

Intangible assets primarily include the intellectual property and other rights relating to DR Cysteamine (currently developed as PROCYSBI TM / RP103) and to an out-license acquired in a 2009 merger. The intangible assets related to PROCYSBI TM /RP103 are amortized using the straight-line method over the estimated useful life of 20 years, which is the life of the intellectual property patents. The 20-year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. The intangible assets related to the out-license are amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

Fixed Assets

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

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.

Impairment of Goodwill and Long-Lived 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.

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.

Accrued Liabilities

Accrued liabilities include estimates of certain expenses which the Company has not yet been invoiced and requires management's judgment in determining appropriate expenses to accrue. Although the Company believes its accrued liabilities reflect the best information available to it, the Company's actual expenses could differ from its estimates.

Common Stock Warrant Liabilities

The 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 consolidated statements of operations and comprehensive loss. At the exercise date, the fair values of these warrants are re-measured and reclassified to equity.

Deferred Offering Costs

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

Note Payable and Debt Issuance Costs

Note payable consists of the Company's loan agreement with HealthCare Royalty Partners II, L.P. ("HC Royalty"), as lender, under which Raptor agreed to borrow \$50.0 million in two \$25.0 million tranches. The Company received the first \$25 million tranche in December 2012, and the second tranche is expected by the end of May 2013. The loan bears interest at an annual fixed rate of 10.75% of outstanding principal and includes a synthetic royalty component based on sales of products, including PROCYSBI TM, 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 using the interest method.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

Research and Development Costs

Research and development costs are charged to expense as incurred. Research and development expenses include medical, clinical, regulatory and scientists' salaries and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, commercial drug manufacturing expenses prior to obtaining marketing approval, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated human resources and facilities expenses.

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 March 31, 2013, there were no liabilities recorded related to uncertain tax positions and no accrued interest or penalties related to uncertain tax positions.

The Company files U.S. Federal, California, various other state income and other 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.

Net Loss per Share

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

(In thousands)	March 31, 2013	February 29, 2012
Warrants to purchase common stock	3,963	5,277
Options to purchase common stock	7,885	5,842
Total potentially dilutive securities	11,848	11,119

Net loss per share, basic and diluted, was \$(0.30) and \$(0.29) for the quarters ended March 31, 2013 and February 29, 2012, respectively.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

Stock Option Plan

Compensation cost 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 on a straight-line basis 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 Company recognizes expense associated with stock options issued to third parties, including consultants, based upon the fair values of such awards on the dates the awards vest.

Reclassifications

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

2. INTANGIBLE ASSETS AND GOODWILL

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

Intangible assets originally recorded as a result of a 2009 merger were approximately \$1.1 million, of which \$0.9 million was written off to research and development expense during the year ended August 31, 2012, as discussed below.

A summary of intangible assets acquired is as follows:

(In thousands)	March 31, 2013	December 31, 2012
Intangible asset (IP license for PROCYSBITM/RP103) related to the Encode merger	\$2,620	\$ 2,620
Intangible assets (out-license) related to the 2009 Merger	240	240
Total intangible assets	2,860	2,860
Less accumulated amortization	(740)	(704)
Intangible assets, net	\$2,120	\$ 2,156

At August 31, 2012, the Company determined that the capitalized acquired in-process research and development asset carried at \$0.9 million, representing the tezampanel and NGX 426 program acquired in a 2009 merger, was fully impaired due to the Company's decision to discontinue development of this product candidate . During the quarter ended March 31, 2013, the Company did not identify any impairment losses.

12

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

During the quarters ended March 31, 2013 and February 29, 2012 and the cumulative period from September 8, 2005 (inception) to March 31, 2013, the Company amortized approximately \$36,000, \$37,000 and \$0.8 million, respectively, of intangible assets to research and development expense.

Amortization expense for intangible assets for the periods indicated is expected to be as follows:

Year ending December 31, (In thousands)	Amortization expense
2013	\$ 146
2014	146
2015	146
2016	146
2017	146

The Company tested the carrying value of goodwill for impairment as of the end of its transition period for the four month period ended December 31, 2012 and determined that there was no impairment.

3. FIXED ASSETS

Fixed assets consisted of:

Category (In thousands)	March 31, 2013	December 31, 2012	Estimated useful lives
Leasehold improvements	\$ 146	\$ 146	Shorter of life of asset or lease term
Office furniture	35	35	7 years
Laboratory equipment	664	593	5 years
Computer hardware and software	245	204	3 years
Capital lease equipment	27	27	Shorter of life of asset or lease term
Total at cost	1,117	1,005	
Less: accumulated depreciation	(625)	(589)	
Total fixed assets, net	\$492	\$ 416	

Depreciation expense for the quarters ended March 31, 2013 and February 29, 2012 and the cumulative period from September 8, 2005 (inception) to March 31, 2013 was approximately \$36,000, \$9,000 and approximately \$0.6 million, respectively. Accumulated depreciation on capital lease equipment was approximately \$10,000 and \$2,000 as of March 31, 2013 and December 31, 2012, respectively.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

4. NOTE PAYABLE AND DEBT ISSUANCE COSTS

Note payable consists of the Company's loan agreement with HC Royalty, as lender, under which the Company agreed to borrow \$50.0 million in two \$25.0 million tranches. As of March 31, 2013, the Company drew down the first tranche in the amount of \$25.0 million. With respect to the first \$25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.25% of the first \$25.0 million of PROCYSBI TM and future approved product net revenues for such calendar year, 3.0% of the PROCYSBI TM and future approved product net revenues for such calendar year in excess of \$25.0 million and below \$50.0 million, and 1.0% of the PROCYSBI TM and future approved product net revenues for such calendar year in excess of \$50.0 million, payable quarterly.

As of March 31, 2013, there was no royalty expense accrued or paid since the Company had no approved products at that time. Subsequent to March 31, 2013, as a result of marketing approval of PROCYSBI TM for cystinosis by the FDA, synthetic royalties will be payable to HC Royalty upon sales of PROCYSBI TM. As of March 31, 2013, the Company's note payable balance was \$25.0 million.

Unamortized debt issuance costs totaled \$1.9 million and \$2.0 million as of March 31, 2013 and December 31, 2012, respectively.

The loan and the Company's obligation to make any payments shall terminate immediately when all payments received by HC Royalty from the Company aggregate to \$97.5 million. If, by December 20, 2014, net revenues for the immediately preceding four fiscal quarters exceed \$100.0 million, then the loan and the Company's obligation to make any payments shall terminate immediately when all payments received by HC Royalty from the Company aggregate to \$90.0 million. The synthetic royalty will be accrued quarterly upon commencement of sales of PROCYSBI TM.

To secure the performance of its 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 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 agreement 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, 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 its 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, the Company's pledged collateral and assign and transfer the pledged stock of certain of its subsidiaries. Further, HC Royalty may terminate its commitment to fund the second \$25.0 million tranche upon the occurrence of any such event prior to the funding of such tranche.

5. CAPITAL STRUCTURE

Preferred Stock

At March 31, 2013, 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 March 31, 2013, 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 March 31, 2013 and December 31, 2012, there were 55,431,651 and 52,424,649 shares, respectively, of the Company's common stock issued and outstanding.

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

On April 30, 2012, the Company entered into an "At-the-Market" ("ATM") Sales Agreement, with Cowen and Company, LLC ("Cowen"), under which the Company may, at its discretion, sell its common stock with a sales value of up to a maximum of \$40.0 million through ATM sales on the NASDAQ Stock Market. 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.

Sales in the ATM offering are being made pursuant to the prospectus supplement dated April 30, 2012, as amended by Amendment No. 1 dated June 28, 2012, which supplements the Company's prospectus dated February 3, 2012, filed as part of the shelf registration statement that was declared effective by the Securities and Exchange Commission ("SEC") on February 3, 2012. Cumulatively through March 31, 2013, the Company sold 5,059,446 shares under the ATM at a weighted-average selling price of \$5.39 per share for net proceeds of approximately \$26.4 million.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

Common Stock Warrants

During the quarter ended March 31, 2013, the Company received approximately \$1.8 million from the exercise of warrants in exchange for the issuance of 600,000 shares of the Company's common stock. During the cumulative period from September 8, 2005 (inception) through March 31, 2013, the Company received approximately \$24.6 million from the exercise of warrants in exchange for the issuance of an aggregate of 10,137,866 shares.

The table reflects the number of common stock warrants outstanding as of March 31, 2013:

	Number of shares exercisable (In thousands)	Exercise price	Expiration date
Issued in connection with Encode merger	233	\$ 2.87	12/13/2015
Issued to placement agents in May / June 2008	433	\$ 2.36	5/21/2013
Issued to placement agents in August 2009	65	\$ 1.50	8/21/2014
TorreyPines warrants assumed in 2009 Merger	8	\$ 80.86	*6/11/2013-9/26/2015
Issued to registered direct investors in Dec. 2009	631	\$ 2.45	12/22/2014
Issued to private placement investors in Aug. 2010	2,495	\$ 3.075	8/12/2015
Issued to placement agent in Aug. 2010	98	\$ 3.075	8/12/2015
Total warrants outstanding	3,963	\$ 3.02	*

*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. A financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, the Company has classified the warrants from both financings as liabilities and will mark them to fair value at each period end.

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

	December 2009 equity financing Series A March		August 2010 private placement Investors and placement agent March	
	31, 2013	December 31, 2012	31, 2013	December 31, 2012
Fair value (dollars in millions)	\$2.5	\$ 2.6	\$10.2	\$ 13.8
Black-Scholes inputs:				
Stock price	\$5.85	\$ 5.85	\$5.85	\$ 5.85

Edgar Filing: Raptor Pharmaceutical Corp - Form 10-Q/A

Exercise price	\$2.45	\$ 2.45	\$3.075	\$ 3.075
Risk free interest rate	0.25 %	0.25 %	0.31 %	0.31 %
Volatility	95 %	100 %	95 %	112 %
Expected term (years)	1.75	2.00	2.25	2.50
Dividend	0	0	0	0

15

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

For the quarters ended March 31, 2013 and February 29, 2012, and for the cumulative period from September 8, 2005 (inception) to March 31, 2013, the Company recorded a gain of approximately \$1.0 million, losses of approximately \$7.8 million and \$25.8 million, respectively, in its condensed consolidated statements of operations and comprehensive loss from changes in the fair values of warrants.

Below is the activity of the warrant liabilities for the quarters ended March 31, 2013 and February 29, 2012:

	For the quarter ended	
	March 31, 2013	February 29, 2012
(In millions)		
Fair value of December 2009 direct offering warrants (including placement agent warrants) at beginning of the quarters ended March 31, 2013 and February 29, 2012	\$2.6	\$ 6.7
December 2009 warrants exercised	0	(4.1)
Adjustment to fair value of common stock warrants	(0.1)	2.2
December 2009 direct offering common stock warrant liability at fair value at March 31, 2013 and February 29, 2012	2.5	4.8
Fair value of August 2010 private placement warrants (including broker warrants) at beginning of the quarters ended March 31, 2013 and February 29, 2012	13.8	18.7
August 2010 warrants exercised	(2.6)	(2.5)
Adjustment to fair value of common stock warrants	(1.0)	5.6
August 2010 private placement common stock warrant liability at fair value at March 31, 2013 and February 29, 2012	10.2	21.8
Total warrant liability at March 31, 2013 and February 29, 2012	\$12.7	\$ 26.6

As discussed above, the Company uses the Black-Scholes option pricing model as its method of valuation for warrants that are subject to warrant liability accounting. The determination of the fair value as of the reporting date is affected by Raptor's stock price as well as assumptions regarding a number of highly complex and subjective variables which could provide differing variables. These variables include, but are not limited to, expected stock price volatility over the term of the security and risk-free interest rate. In addition, the Black-Scholes option pricing model requires the input of an expected life for the securities for which we have estimated based upon the stage of the Company's development. The fair value of the warrant liability is revalued each balance sheet date utilizing Black-Scholes valuation model computations with the decrease or increase in fair value being reported in the Condensed Consolidated Statement of Comprehensive Loss as other income or expense, respectively. The Company's reported net loss was approximately \$15.9 million for the quarter ended March 31, 2013. If the Company's March 31, 2013 closing stock price had been 10% lower, its net loss would have been approximately \$1.7 million lower. If the Company's March 31, 2013 closing stock price had been 10% higher, its net loss would have been approximately \$1.7 million higher. If the Company's March 31, 2013 volatility assumption had been 10% lower, its net loss would have been approximately \$0.5 million lower. If the Company's March 31, 2013 volatility assumption had been 10% higher, its net loss would have been approximately \$0.5 million higher.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

6. ACCRUED LIABILITIES

Accrued liabilities consisted of:

	March 31, 2013	December 31, 2012
(In thousands)		
Clinical trials and related costs	\$1,439	\$ 641
Pre-commercial and other consulting	993	167
Accrued bonuses	889	502
Accrued vacation and employee benefits	700	420
Legal and patent fees	417	44
Salaries and wages	38	322
Other	88	54
Total accrued liabilities	\$4,564	\$ 2,150

7. STOCK OPTION PLANS

Stock options are granted to recognize the contributions made by its employees, independent contractors, consultants and directors, to provide those individuals with additional incentive to devote themselves to the Company's future success and to improve its ability to attract, retain and motivate individuals upon whom its growth and financial success depends. Employee stock options generally vest over four years with a six-month cliff-vesting period. In general, all options are exercisable over a period not to exceed the contractual term of ten years from the date the stock options are granted at prices not less than the fair market value of the Company's common stock on the grant date. The Company has and may grant options with different vesting terms from time to time.

Employee stock-based compensation expense for the quarters ended March 31, 2013 and February 29, 2012, respectively, and for the cumulative period from September 8, 2005 (inception) to March 31, 2013 was as follows:

(In millions)	For the quarter ended March 31, 2013	For the quarter ended February 29, 2012	Cumulative Period from September 8, 2005 to March 31, 2013
Research and development	\$ 0.3	\$ 0.2	\$ 2.4
Selling, general and administrative	1.4	0.9	9.4
Total stock-based compensation expense	\$ 1.7	\$ 1.1	\$ 11.8

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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

Period*	Risk-free interest rate	Expected life of stock option	Annual volatility
Quarter ended February 29, 2012	1.12	% 5 years	122 %
Quarter ended March 31, 2013	0.82	% 5 years	68 %

*Dividend rate is 0% for all periods presented.

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

The Company recognizes as an expense the fair value of options granted to persons who are neither employees nor directors. The fair value of expensed options was based on the Black-Scholes option-pricing model assuming the same factors shown in the stock-based compensation expense table above. Stock-based compensation expense for consultants for the quarters ended March 31, 2013 and February 29, 2012 and for the cumulative period from September 8, 2005 (inception) to March 31, 2013 was approximately \$4,000, \$0 and \$0.8 million, respectively, of which cumulatively approximately \$0.1 million was included in general and administrative expense and approximately \$0.6 million was included in research and development expense for the cumulative period.

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

	Option shares (In thousands)	Weighted- average exercise price	Exercisable (In thousands)	Weighted- average fair value of options granted
Outstanding at December 31, 2012	7,791	\$ 5.79	3,494	\$ 3.48
Granted	130	5.39	0	3.04
Exercised	(8)	0.85	0	0.62
Canceled	(28)	13.94	0	4.25
Outstanding at March 31, 2013	7,885	5.76	4,031	3.47

There were approximately 1.8 million options available for grant as of March 31, 2013 under the 2010 Equity Incentive Plan, as amended (the "Plan").

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

As of March 31, 2013, the options outstanding under all of the Company's stock option plans consisted of the following:

Range of exercise prices	Options outstanding			Options vested and exercisable	
	Number of options outstanding and expected to vest (#, in thousands)	Weighted-average remaining contractual life (yrs.)	Weighted-average exercise price (\$)	Number of options exercisable (#, in thousands)	Weighted-average exercise price (\$)
\$0 to \$1.00	1	6.05	\$ 0.85	1	\$ 0.85
\$1.01 to \$2.00	79	6.22	1.78	75	1.76
\$2.01 to \$3.00	1,353	5.64	2.66	1,121	2.61
\$3.01 to \$4.00	1,764	7.65	3.50	1,334	3.52
\$4.01 to \$5.00	310	8.66	4.79	100	4.73
\$5.01 to \$6.00	3,989	8.94	5.28	1,256	5.23
\$6.01 to \$7.00	277	8.92	6.48	83	6.50
\$7.01 to \$8.00	70	8.88	7.75	19	7.75
\$8.01 to \$964.24	42	2.59	254.62	42	254.62
	7,885	8.01	5.76	4,031	6.48

At March 31, 2013, the total unrecognized compensation cost was approximately \$13.6 million. The weighted-average period over which it is expected to be recognized is approximately 3 years.

8. COMMITMENTS AND CONTINGENCIES

The Company maintains several contracts with 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 NAFLD clinical collaborations. The Company's contractual obligations did not change materially during the quarter ended March 31, 2013 compared to those disclosed as of December 31, 2012.

Table of Contents

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

9. SUBSEQUENT EVENTS

On April 30, 2013, the Company's lead product candidate, PROCYSBITM (cysteamine bitartrate) delayed-release capsules, received marketing approval from the FDA for the management of nephropathic cystinosis in adults and children six years and older. The Company plans to commercially launch PROCYSBI TM in the U.S. in the second quarter of 2013 and anticipates the Company's expenses will increase due to significant commercialization activities followed by the uptake of net revenues. As a result of drug approval, the Company will commence capitalizing commercial inventory on May 1, 2013. Due to the nature of the success rate of drug development, it is the Company's accounting policy to expense drug product manufacturing costs as research and development expenses until that drug product has received marketing approval in a significant market.

On April 25, 2013, the Company executed a seven year lease for facilities in Novato, California, which it will inhabit by June 30, 2013 to accommodate personnel growth. The Company will make lease payments of \$19,460 per month under this new lease which commences in June 2013 through May 2014. The Company plans to move to an adjacent facility which becomes available in 2014. Rental expense for the larger adjacent facility will be higher than the facility into which the Company will initially move.

On April 3, 2013, the Company executed two contracts with Accredo Health Group, Inc. ("Accredo") to provide the Company exclusive distribution and specialty pharmacy services for PROCYSBI TM to cystinosis patients in the U.S. Accredo will be Raptor's only significant customer of PROCYSBI TM in the U.S. Accredo will receive a distributor fee for warehousing and distribution of PROCYSBI TM along with a rebate for pharmacy services based upon customary terms. These costs will be captured in cost of sales upon commencement of services.

Table of Contents

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

You should read the following discussion in conjunction with our condensed consolidated financial statements as of March 31, 2013, and the notes to such condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q. All references to "the Company", "we", "our" and "us" include the activities of Raptor Pharmaceutical Corp., Raptor Pharmaceuticals Inc. or Raptor Pharmaceuticals, Raptor European Products, LLC, RPTP European Holdings C.V., Raptor Pharmaceuticals Europe B.V. and Raptor Pharmaceuticals France SAS.

This Quarterly Report on Form 10-Q, including this "Management's Discussion and Analysis of Financial Condition and Results of Operations" section, contains "forward-looking statements," within the meaning of the Private Securities Litigation Reform Act of 1995, that plan for or anticipate the future. In some cases, these statements can be identified by the use of terminology such as "believes," "expects," "anticipates," "plans," "may," "might," "will," "could," "should," "would," "projects," "predicts," "intends," "continues," "estimates," "potential," "opportunity" or the negative of these terms or other comparable terminology. All such statements, other than statements of historical facts, including our financial condition, future results of operations, projected revenues and expenses, business strategies, operating efficiencies or synergies, competitive positions, growth opportunities for existing intellectual properties, technologies, products, plans, and objectives of management, markets for our securities, and other matters, are about us and our industry that involve substantial risks and uncertainties and constitute forward-looking statements for the purpose of the safe harbor provided by Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements, wherever they occur, are necessarily estimates reflecting the best judgment of our senior management on the date on which they were made, or if no date is stated, as of the date of the filing made with the SEC in which such statements were made. You should not place undue reliance on these statements, which only reflect information available as of the date that they were made. We cannot give you any assurance that such forward-looking statements will prove to be accurate and such forward-looking events may not occur. Our business' actual operations, performance, development and results might differ materially from any forward-looking statement due to various known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled "Risk Factors" 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.

Change in Fiscal Year End

In December 2012, our board of directors approved a change in our fiscal year end from August 31 to December 31. The following discussions compare the unaudited periods from January 1 through March 31, 2013, the first quarter of our newly adopted fiscal year and our prior year's fiscal quarter from December 1, 2011 through February 29, 2012. The prior fiscal year quarter ended February 29, 2012 is reported on the basis of our previous fiscal year cycle. As a result of the change in our fiscal year end, the quarterly periods of our newly adopted fiscal year do not coincide with the historical quarterly periods that we had previously reported. We believe the comparative information provided for the three-month period ended February 29, 2012 provides a meaningful comparison to the three-month period ended March 31, 2013 and there are no factors, seasonal or otherwise, that materially impact the comparability of information or trends.

Plan of Operation and Overview

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

Our first product, PROCYSBITM (cysteamine bitartrate) delayed-release capsules, or PROCYSBITM, received marketing approval from the U.S. Food and Drug Administration, or FDA, on April 30, 2013 for the management of nephropathic cystinosis in adults and children six years and older. PROCYSBI TM (formerly known as RP103) capsules contain cysteamine formulated into innovative microspherized beads that are individually enteric coated providing a delayed-release pharmacokinetic profile. The enteric coating makes PROCYSBI™ pH sensitive, allowing the microbeads to bypass the stomach and be absorbed in the more alkaline environment of the proximal small intestine. PROCYSBI TM is dosed every twelve hours for the management of nephropathic cystinosis. We plan to commence the launch of PROCYSBI TM in the U.S. in the second quarter of 2013. In conjunction with the pending launch of PROCYSBI TM , we have initiated a dedicated call center, which will serve as an integrated resource for PROCYSBI TM prescription intake, third-party payor reimbursement adjudication, patient financial support and ongoing outreach for managing compliance. This call center, along with one specialty pharmacy and proactive physician and patient disease education initiatives, reflect our commitment to helping patients manage their disease. In Europe, we submitted a marketing authorization application, or MAA, to the European Medicines Agency, or EMA, in the first quarter of calendar 2012, requesting approval to market PROCYSBI TM for the management of nephropathic cystinosis in the EU. We anticipate a decision from the EMA in the second half of 2013.

Table of Contents

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

Commercializing PROCYSBITM in the U.S.;

Gaining regulatory approval of PROCYSBITM in the EU;

Negotiating reimbursement country by country within the EU and the launching of PROCYSBITM in those countries, if approved by the EMA;

Supporting our clinical trials that are evaluating PROCYSBITM in cystinosis patients that are cysteamine-naïve, as well as other supporting studies;

Supporting our clinical trials of RP103 for the potential treatment of Huntington's disease, or HD, and nonalcoholic fatty liver disease, or NAFLD, in children;

Continuing the development of our novel preclinical programs.

Nephropathic Cystinosis, or Cystinosis

Cystinosis is a rare, inherited condition that affects every cell of the body by causing the buildup of a protein building block called cystine. The buildup of cystine causes kidney and other problems throughout the body. These problems cause the body to lose too much sugar (glucose), proteins and salts (electrolytes) through urine and harm tissue.

Cystinosis may lead to slow body growth and small stature, weak bones, hypothyroidism, blindness, muscle weakness, pulmonary dysfunction and developing and worsening kidney failure.

It is estimated that there are currently about 500 people in the U.S. – 2,000 worldwide – diagnosed with this disorder. Cystinosis is classically associated with blond-haired, blue-eyed children of European descent, although all races and ethnic backgrounds may be affected. The disease tends to affect slightly more males than females, with a male-to-female ratio of 1.4 to 1.

Table of Contents

Results of Operations

Quarters ended March 31, 2013 and February 29, 2012

Revenue

On April 30, 2013, the FDA granted marketing approval in the U.S. for the sale of our first product, PROCYSBITM, for the management of nephropathic cystinosis. As such, through March 31, 2013, we have not yet generated revenues from the sale of PROCYSBI TM.

Research and Development

Research and development expenses include medical, clinical, regulatory and scientists' compensation and benefits, expenses associated with the manufacturing and testing of PROCYSBI TM in anticipation of our commercial launch in the U.S. (all drug product manufactured before drug approval is expensed as research and development expense), lab collaborations, preclinical studies, clinical trials, clinical trial materials, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated human resources and facilities expenses. Research and development expenses for the quarter ended March 31, 2013 increased by approximately \$4.4 million compared to the quarter ended February 29, 2012. The increase was primarily due to:

Reason for increase (In thousands)	Increase (decrease)
In anticipation of the U.S. launch of PROCYSBITM:	
Increased product manufacture of PROCYSBITM for cystinosis	\$ 2,653
Increase in consulting and external services	699
Build-up in staffing for medical affairs, quality and regulatory	556
Increase in clinical trial activity	420
Other, net	113
Total increase in research and development expenses for the quarter ended March 31, 2013 versus the quarter ended February 29, 2012	\$ 4,441

General and Administrative Expenses

General and administrative expenses include finance, executive and pre-commercial operations compensation and benefits; pre-commercial expenses, such as reimbursement and marketing studies, expenses associated with the commercial launch of PROCYSBI TM such as printing of physician and patient education materials, setting up RaptorCares.com to adjudicate insurance claims and provide patient support, and establishing a customer relationship management system for our PROCYSBI TM sales team; corporate costs, such as legal and auditing fees, business development expenses, travel, board of director fees and expenses, investor relations expenses, intellectual property expenses associated with filed (but not issued) patents, administrative consulting and allocated human resources and facilities costs. General and administrative expenses for the quarter ended March 31, 2013 increased by approximately \$5.4 million compared to the quarter ended February 29, 2012. The increase was primarily due to:

Reason for increase (In thousands)	Increase (decrease)
In anticipation of the U.S. launch of PROCYSBITM:	
Increase in staffing for pre-commercialization ramp-up	\$ 1,933
Increase in advertising and promotion work related to pre-commercialization	1,020

Edgar Filing: Raptor Pharmaceutical Corp - Form 10-Q/A

Increase in consulting and legal fees incurred for pre-commercialization activities	1,265
Increase in other marketing work incurred as part of pre-commercialization activities	648
Other, net	545
Total increase in general and administrative expenses for the quarter ended March 31, 2013 versus the quarter ended February 29, 2012	\$ 5,411

23

Table of Contents

Major program expenses recorded as research and development expenses:

Major Program (stage of development) (in thousands)	For the quarter ended		September 8, 2005
	March 31, 2013	February 29, 2012	(Inception) to March 31, 2013
PROCYSBITM: Cystinosis (pre-commercial)	\$4,179	\$ 2,249	\$ 41,587
RP103: HD (clinical)	212	190	3,265
RP103: NAFLD in children (clinical)	445	212	4,831
Preclinical programs	200	15	2,520
Minor or inactive programs	254	(11)	6,168
Research and development personnel and other costs not allocated to programs	3,122	1,316	19,685
Total research and development expenses	\$8,412	\$ 3,971	\$ 78,056

Major program expenses recorded as general and administrative expenses:

Major Program (stage of development) (in thousands)	For the quarter ended		September 8, 2005
	March 31, 2013	February 29, 2012	(Inception) to March 31, 2013
PROCYSBITM: Cystinosis (pre-commercial)	\$2,292	\$ 234	\$ 9,012
RP103: HD (clinical)	30	10	55
RP103: NAFLD in children (clinical)	46	21	301
Preclinical programs	18	15	450
Minor or inactive programs	112	47	1,209
Total program-related general and administrative expenses	\$2,498	\$ 327	\$ 11,027

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

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

Table of Contents

Interest Income

Interest income for the quarters ended March 31, 2013 and February 29, 2012 was approximately \$0.2 million and \$0.1 million, respectively.

Interest Expense

Interest expense for the quarters ended March 31, 2013 and February 29, 2012 was approximately \$0.7 million and nominal, respectively. The increase in interest expense was due to our debt facility we entered into in December 2012.

Foreign Currency Transaction Gain

Foreign currency transaction gain/loss for the quarters ended March 31, 2013 and February 29, 2012 was approximately a \$34,000 loss and \$18,000 gain, respectively.

Unrealized Gain/Loss on Short-Term Investments

Unrealized gain/loss on short-term investments represents the change in net asset value of our short-term bond fund. The unrealized loss on short-term investments for the quarter ended March 31, 2013 was approximately \$0.1 million compared to an unrealized gain of approximately \$0.1 million for the quarter ended February 29, 2012.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a gain of approximately \$1.1 million for the quarter ended March 31, 2013 compared to a loss of approximately \$7.8 million for the quarter ended February 29, 2012. The gain for the quarter ended March 31, 2013 was due primarily to a decrease in the volatility of our stock price. The loss in the comparable quarter ended February 29, 2012 was due primarily to an increase of \$1.46 per share in our stock price for which we were required to increase our warrant liability. These losses are non-cash.

Table of Contents

Application of Critical Accounting Policies

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

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

Use of Estimates

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

Fair Value of Financial Instruments

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

Accrued Liabilities

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

Note Payable and Debt Issuance Costs

Note payable consists of our loan agreement with HealthCare Royalty Partners, or HC Royalty, as lender. We agreed to borrow \$50.0 million in two \$25.0 million tranches, or the HC Royalty Loan. As of March 31, 2013, we have drawn the first tranche in the amount of \$25.0 million, which is stated at the borrowed amount. Due to the approval of PROCYSBI TM by the FDA on April 30, 2013, HC Royalty is required to fund the second tranche subject to the satisfaction of customary closing conditions. We are in the process of drawing down the second \$25.0 million tranche. The loan bears interest at an annual fixed rate of 10.75% of outstanding principal and quarterly interest payments are included in interest expense in our Condensed Consolidated Statements of Comprehensive Loss for the quarter ended March 31, 2013. Principal payments, when made, reduce our note payable balance. There is a synthetic royalty component based on sales of products in a calendar year, and such royalty is payable quarterly. With respect to the first \$25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.25% of the first \$25.0 million of PROCYSBI TM and future approved product net revenues for such calendar year, 3.0% of PROCYSBI TM and future approved product net revenues for such calendar year in excess of \$25.0

million and not in excess of \$50.0 million, and 1.0% of PROCYSBI TM and future approved product net revenues for such calendar year in excess of \$50.0 million, payable quarterly. With respect to the second \$25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.0% of the first \$25.0 million of PROCYSBI TM and future approved product net revenues for such calendar year, 3.0% of PROCYSBI TM and future approved product net revenues for such calendar year in excess of \$25.0 million and not in excess of \$50.0 million, and 1.0% of PROCYSBI TM and future approved product net revenues for such calendar year in excess of \$50.0 million, payable quarterly.

As of March 31, 2013, there was no royalty expense accrued or paid since we had no approved products at that time and future revenues were not yet estimable. Subsequent to quarter-end, we received marketing approval of PROCYSBI TM for nephropathic cystinosis by the FDA on April 30, 2013, and as a result, synthetic royalties will be payable to HC Royalty quarterly following commencement of sales of PROCYSBI TM. The loan and our obligation to make any payments shall terminate immediately when all payments received by HC Royalty from us equals \$97.5 million, except that if, by December 20, 2014, net revenues for the immediately preceding four fiscal quarters exceed \$100.0 million, then the loan and our obligation to make any payments shall terminate immediately when all payments received by HC Royalty from us equals \$90.0 million. Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan using the effective interest method. The amortization of debt issuance costs is included in interest expense in our Condensed Consolidated Statements of Comprehensive Loss. The synthetic royalty will be accrued quarterly upon commencement of sales of PROCYSBI TM.

Table of Contents

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

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

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

Intangible assets include the intellectual property and other rights relating to DR Cysteamine (currently developed as PROCYSBI TM and RP103) and to an out-license acquired in the 2009 Merger. The intangible assets related to PROCYSBI TM /RP103 are amortized using the straight-line method over the estimated useful life of 20 years, which is the life of the intellectual property patents. The 20-year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. The intangible assets related to the out-license are amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents. During the quarter ended March 31, 2013, we did not identify any impairment losses.

Common Stock Warrant Liabilities

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

Income Taxes

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

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

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

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

Our continuing practice is to recognize interest and/or penalties related to income tax matters as a component of income tax expense. As of March 31, 2013, there were no accrued interest or penalties related to uncertain tax positions.

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

Table of Contents

Stock-Based Compensation

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

For the quarter ended March 31, 2013, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 0.82%; five year expected life; 68% volatility; 2.5% turnover rate; and 0% dividend rate.

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

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

the expected life of five years was based upon our assessment of the ten-year term of the stock options issued along with the fact that we are a development-stage company and our anticipation that option holders will exercise stock options when we are at a more mature stage of development;

the volatility was based on a combination of the actual annualized volatility of our common stock price as quoted on NASDAQ since the closing of our 2009 Merger on September 30, 2009 and of annualized volatility of peer companies as reported in their respective SEC filings; and

the turnover rate was based on our assessment of our historical employee turnover; and the dividend rate was based on our current decision to not pay dividends on our stock at our current development stage.

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

We recognize as consulting expense the fair value of options granted to persons who are neither employees nor directors. Stock options issued to consultants are accounted for in accordance with the provisions of the FASB ASC Topic 505-50, Equity-Based Payments to Non-Employees (previously listed as Emerging Issues Task Force, or EITF, Consensus No. 96-18, Accounting for Equity Instruments That Are Issued to Other Than Employees for Acquiring, or in Conjunction with Selling Goods or Services). The fair value of expensed options is based on the Black-Scholes option-pricing model assuming the same factors as stock-based compensation expense discussed above.

Recent Accounting Pronouncements

None.

Table of Contents

Liquidity and Capital Resources

Capital Resource Requirements

As of March 31, 2013, we had approximately \$58.4 million in cash, cash equivalents and short-term investments, approximately \$20.0 million in current liabilities (of which approximately \$12.7 million represented the non-cash common stock warrant liability) and approximately \$40.0 million of net working capital.

We estimate that without regard to any other future sources of funds, our cash, cash equivalents and short-term investments of approximately \$58.4 million as of March 31, 2013 plus estimated net proceeds of approximately \$23.4 million we are in the process of drawing as the second tranche of our \$50.0 million loan agreement with HC Royalty, will be sufficient to meet our obligations into the second quarter of 2014.

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

On April 30, 2012, we entered into a Sales Agreement with Cowen and Company, or Cowen, to sell shares of our common stock, with aggregate gross sales proceeds of up to \$40.0 million, from time to time, through an "at the market" equity offering program under which Cowen acts as sales agent. We pay a 3.0% commission to Cowen on any sales pursuant to this Sales Agreement. Through March 31, 2013, we sold 5,059,446 shares at a weighted-average sales price of \$5.39 per share for net proceeds of approximately \$26.4 million. As of March 31, 2013, \$12.7 million of gross proceeds remains available for future sales under this Sales Agreement.

As of March 31, 2013, Series A warrants to purchase up to 631,250 shares of our common stock were outstanding, all of which warrants were issued pursuant to a definitive securities purchase agreement, dated as of December 17, 2009. The outstanding Series A warrants are exercisable until December 22, 2014, at a per share exercise price of \$2.45.

As of March 31, 2013, 2,592,424 shares (including the placement agent warrant described below) of our common stock warrants were outstanding, all of which warrants were issued pursuant to private placement purchase agreements, dated as of August 9, 2010. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of \$3.075 per share. The placement agent warrant that we issued to the placement agent for this private placement is for the purchase 97,952 shares of our common stock at an exercise price of \$3.075 per share.

Future Funding Requirements

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

the success of our U.S. commercial launch of PROCYSBI TM including patient and revenue uptake;
the cost of establishing the sales and marketing capabilities in the U.S. and EU necessary for the U.S. commercial launch of PROCYSBI TM and the potential EU launch, if approved;
the decisions of the EMA with respect to our application for marketing approval of PROCYSBI TM in the EU; the costs of activities related to the regulatory approval process; and the timing of approval, if received and the potential EU launch, if approved;

our ability to negotiate reimbursement and pricing of PROCYSBITM in the EU; if approved;
the cost of additional clinical trials in order to obtain regulatory approvals for PROCYSBITM in non-U.S. and non-EU countries;
the timing and cost of our ongoing clinical programs for RP103, including: evaluating PROCYSBITM in treatment-naïve cystinosis patients, and other supportive studies; evaluating RP103 as a potential treatment for Huntington's disease; and evaluating RP103 as a potential treatment for NAFLD;
the cost of filing, prosecuting and enforcing patent claims; and
the cost of our manufacturing-related activities in support of PROCYSBITM and RP103.

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

Table of Contents

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 and clinical and commercial manufacturing of PROCYSBI™ and clinical manufacturing of our HD and our NAFLD clinical collaborations. Our contractual obligations have not changed significantly during the quarter ended March 31, 2013 compared to those discussed in our Transition Report on Form 10-KT for the four month period ended December 31, 2012, filed with the SEC on March 14, 2013.

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

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

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 quarter ended March 31, 2013, we hired a Controller which increases our ability to implement additional internal control procedures. There have not been any other factors that have materially affected or are reasonably likely to have a negative 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 consider carefully the specific risks detailed in this "Risk Factors" section, together with all of the other information contained in this Quarterly Report on Form 10-Q. If any of these risks occur, our business, results of operations and financial condition could be harmed, the price of our common stock could decline, and you may lose part or all of your investment.

Risks Associated with Product Development and Commercialization

We currently depend entirely on the success of our lead drug, PROCYSBITM. If we do not successfully commercialize PROCYSBI TM or obtain EU approval of PROCYSBI TM our financial results and financial condition will be adversely affected.

On April 30, 2013, the U.S. Food and Drug Administration, or FDA, approved PROCYSBITM (cysteamine bitartrate) delayed-release capsules for the management of nephropathic cystinosis in adults and children six years and older, permitting us to commercially market and distribute PROCYSBI TM in the U.S. for this indication. The success of our commercial launch of PROCYSBI TM will likely drive our value as reflected in the trading price of our common stock. However, we do not have experience in commercializing therapeutics. If our PROCYSBI TM launch is not as robust as expected by analysts and investors, our value may not increase or could significantly decrease. We have not yet obtained marketing approval in the EU, where we believe there is a significant number of cystinosis patients. If we do not obtain marketing approval in the EU, our business prospects will decline. The successful commercialization of PROCYSBI TM will depend on several factors, including:

the successful launch of PROCYSBITM in the U.S., including, among other factors, identification of potential physician prescribers and potential patients for, and obtaining sales, of PROCYSBI TM ;

approval of PROCYSBITM in the EU for the management of nephropathic cystinosis by the EMA;

the successful launch of PROCYSBITM in the EU and other selected territories throughout the economically developed world, if approved;

effective communication distinguishing the safety and efficacy of PROCYSBITM from competitive products or alternative therapeutic regimes;

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

appropriate reimbursement for PROCYSBITM 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 required post-approval commitments;

provision of affordable out-of-pocket cost to patients and/or other programs to ensure patient access to PROCYSBITM;

approval by the EMA and other regulatory agencies of appropriate product labeling for PROCYSBITM;
execution and maintenance of agreements with wholesalers and distributors on commercially reasonable terms;
manufacture and supply of adequate quantities of PROCYSBITM to meet commercial demand;
development and maintenance of intellectual property protection for PROCYSBITM; and
execution of robust pre-launch, commercial launch and ongoing commercial operations and medical affairs' activities in support of marketing and sales requirements.

31

Table of Contents

If we fail to successfully commercialize PROCYSBITM at sufficient sales levels or gain EU approval of PROCYSBITM within a reasonable time period, we will be unable to sustain or grow our business and we may never become profitable, and our business, financial condition and results of operations will be adversely affected.

If we are unable to expand the use of RP103 and receive regulatory approval for any other indication, we may delay or cease some of our product development activities, which would adversely affect the longer term value of RP103 and our growth prospects.

We must obtain and maintain appropriate approvals from regulatory agencies in each of the markets in which we intend to market our products before we may market them. If we receive approvals for our products, we may only market our products for the specific uses that are reflected in the product's approved labeling. In the U.S., we are permitted to market RP103 for the management of nephropathic cystinosis in adults and children six years and older under the brand name PROCYSBITM, and we may not market RP103 in the U.S. for any other indication. We do not have approval of RP103 in any other market and we are not permitted to market RP103 in these markets until we obtain necessary regulatory approvals. To market a new drug in the U.S., we must submit to the FDA and obtain FDA approval of a new drug application, or NDA for each individual indication. To market a new drug in Europe, we must submit to the EMA or relevant regulatory authority in the designated Reference Member State and obtain approval of an MAA for each individual indication. An NDA or MAA must be supported by extensive clinical and preclinical data, as well as extensive information regarding chemistry, manufacturing and controls, to demonstrate the safety and efficacy of the applicable product candidate for the treatment of each individual indication.

We have additional product development programs in the clinical testing stage for the use of RP103 in HD and in NAFLD. These product development programs have not advanced to the stage of a submission for marketing approval to the FDA or EMA or to any other regulatory body in any other jurisdiction.

Obtaining approval of an NDA or MAA or any other filing for approval in a foreign country is an extensive, lengthy, expensive and uncertain process. The FDA, EMA or other regulatory authorities may reject a filing or delay, limit or deny approval of RP103 for many reasons, including:

the results of clinical trials may not meet the level of statistical significance or clinical significance required by the FDA, EMA and/or other regulatory authorities for approval;

the FDA, EMA or other regulatory authorities may disagree with the number, design, size, conduct or implementation of our clinical trials; may not find the data from preclinical studies and clinical trials sufficient to demonstrate that RP103 has adequate clinical and other benefits and an adequate safety profile; or may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct one or more additional trials;

the FDA, EMA or other regulatory authorities may not accept data generated at our clinical trial sites;

the FDA, EMA or other regulatory authorities may have difficulties scheduling an advisory committee meeting (if required) in a timely manner or the advisory committee may recommend against approval of our application or may recommend that the regulatory agency require, as a condition of approval, additional preclinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;

the FDA, EMA or other regulatory authorities may require additional preclinical or clinical studies or other data prior to granting approval, and we may not be able to generate the required data on a timely basis, if at all;

the FDA, EMA or other regulatory authorities may impose limitations on approved labeling of RP103, thus introducing reimbursement complications which may limit access for intended uses;

the FDA, EMA or other regulatory authorities may identify deficiencies in the manufacturing processes or in the facilities of our third party contract manufacturers, or may require us to manufacture additional validation batches or change our process or specifications;

we may not be able to validate our manufacturing process to the satisfaction of the FDA, EMA or other regulatory authorities, or they may not agree with our plan for concurrent validation; or

the FDA, EMA or other regulatory authorities may change approval policies or adopt new regulations.

Despite regulatory guidelines, we cannot reliably predict if or when any of the drug product candidates we are developing or intend to develop will be approved for marketing. If we fail to gain regulatory approval for RP103 for other indications, our financial results and financial condition will be adversely affected. In such a case, we will have to delay or terminate some or all of our research product development programs and may be forced to dramatically restructure or cease operations.

Table of Contents

PROCYSBITM and our other future product candidates will be subject to labeling and other restrictions or potential market withdrawal, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products.

Any regulatory approvals that we obtain for PROCYSBITM or our product candidates will also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval. The FDA strictly regulates the promotional claims that may be made about prescription products and our product labeling, advertising and promotion is subject to regulatory requirements and continuing regulatory review. Physicians may nevertheless prescribe our product to their patients in a manner that is inconsistent with the approved label, or off-label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant sanctions. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

In addition, if the FDA, EMA or other regulatory authorities approve a product candidate, the manufacturing processes, labeling, packaging, distribution, storage, adverse event reporting, dispensation, distribution, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements will include submissions of safety and other post-marketing information and reports, ongoing maintenance of product registration, as well as continued compliance with cGMPs (good manufacturing practices), GCPs (good clinical practices), and GLPs (good laboratory practices). If we do not comply with the applicable regulations and requirements, the range of possible sanctions includes issuance of adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications and enforcement actions, including injunctions and civil or criminal prosecution. The FDA, EMA and other international regulatory agencies can withdraw a product's approval, including PROCYSBITM, under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues.

If serious adverse side effects are associated with PROCYSBITM, our business could be harmed.

The FDA-approved prescribing information for PROCYSBITM for the management of nephropathic cystinosis includes several warnings relating to observed adverse reactions. With commercial use and additional clinical trials, we expect to continue to update adverse reactions listed in the prescribing information. If additional adverse reactions emerge, or a pattern of severe or persistent previously observed side effects is observed in the relevant patient populations, the FDA or other regulatory agencies could modify or revoke marketing approval or we may choose to withdraw PROCYSBITM from the market. If this were to occur, we may be unable to obtain marketing approval in additional indications. In addition, if patients receiving PROCYSBITM were to suffer harm as a result of their use of the product, these patients or their representatives may bring claims against us. These claims, or the mere threat of these claims, could have a material adverse effect on our business and results of operations.

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

Market acceptance and sales of PROCYSBITM and any product candidates that we may develop will depend in large part on global reimbursement policies and may be affected by future healthcare reform measures in the U.S. as well as the EU and other key international markets. The continuing efforts of governmental and third-party payors to contain, reduce or shift the costs of healthcare through various means may harm our business. Successful commercialization of our products will depend in part on the availability of governmental and third-party private payor reimbursement for the therapeutic value of our products. For example, in many foreign markets, the pricing or profitability of healthcare

products is subject to government control. In the U.S., there has been, and we expect there will continue to be, a number of federal and state proposals to implement similar government price control. The implementation or even the announcement of any of these legislative or regulatory proposals or reforms could harm our business, by reducing the prices we are able to charge for our products, reducing the reimbursement rates for our products and increasing governmental rebates, impeding our ability to achieve profitability, raise capital or form collaborations. In particular, in the U.S., private health insurers and other third-party payors often follow the coverage and reimbursement policies of government payors, including the Medicare or Medicaid programs. In many European countries, patients are unlikely to use prescription drugs that are not reimbursed by their governments. Further, the increased emphasis on managed healthcare in the U.S. and on country and regional pricing and reimbursement controls in the EU will put additional downward pressure on product pricing, reimbursement and usage, which may adversely affect our product sales and results of operations. In the U.S., EU and other significant or potentially significant markets for our product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. 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. For PROCYSBI™, we will not know what reimbursement rates will be until we enter into payor negotiations. For our other product candidates, we will not know what the reimbursement rates will be until we obtain regulatory approval and then launch and enter into payor negotiations. If we are unable to obtain sufficiently high reimbursement rates for our products, they will not be commercially viable.

Table of Contents

Even with U.S. approval of PROCYSBITM, our ability to generate revenues from PROCYSBITM will be subject to attaining significant market acceptance among physicians, patients, patient families, healthcare payors and the healthcare community.

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

availability and relative efficacy and safety of therapeutic alternatives;

the price of our products, both in absolute terms and relative to alternative treatments;

timing of market introduction of our products as well as competitive drugs;

efficacy and safety and real-world patient and physician experience with PROCYSBITM;

identification of currently diagnosed and undiagnosed patients and continued projected growth of the cystinosis market;

prevalence and severity of any side effects;

acceptance by patients, patient families, primary care specialists and key specialists including conversion from the existing standard of care;

potential or perceived advantages or disadvantages of our products compared to alternative treatments, including safety, efficacy, cost of treatment and relative convenience and ease of administration;

strength of sales, marketing, market access, medical affairs and distribution support;

the effect of current and future healthcare laws;

availability of coverage and adequate reimbursement and pricing from government and other third-party payors; and

breadth of product labeling or product insert requirements of the FDA, EMA or other regulatory authorities.

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

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

PROCYSBITM and our clinical development of RP103 target diseases with small patient populations, including cystinosis and HD. A key component of the successful commercialization of a drug product for these indications includes identification of patients and a targeted prescriber base for the drug product. Due to small patient populations, we believe that we would need to have significant market penetration to achieve meaningful revenues and identifying patients and targeting the prescriber base are key to achieving significant market penetration. In addition, the per-patient prices at which we sell PROCYSBITM and RP103 for these indications will need to be relatively high in order for us to generate an appropriate return for the investment in these product development programs and achieve

meaningful gross margins. There can be no assurance that we will be successful in achieving a sufficient degree of market penetration and/or obtaining high per-patient prices for PROCYSBI™ and RP103 for diseases with small patient populations.

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

As part of our business strategy, we intend to develop some 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. 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 half year 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 or if the manufacturer is unable to assure sufficient quantity of the drug. Similar regulations are available from the EMA with a 10-year period of market exclusivity.

34

Table of Contents

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 exclusivity period under Orphan Drug Act designation to maintain a competitive position. If we do not obtain orphan drug exclusivity for PROCYSBI™ or RP103 under the Hatch-Waxman Act, PROCYSBI™ or RP103 are eligible for a 3-year regulatory exclusivity period as a reformulated version of a previously approved drug substance for which clinical studies that are essential for approval have been conducted in addition to the half year pediatric exclusivity, as applicable. However, if we do not obtain orphan drug exclusivity for our drug products that 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.

Even though we have been granted orphan drug designation prior to the approval of PROCYSBITM for the potential treatment of cystinosis and 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 renewed exclusivity for PROCYSBI™ since the active ingredient in PROCYSBI™ had been granted exclusivity in the 1990s by another company 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 product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

The priority review for our drug product candidates, if obtained, may not actually lead to a faster review process.

In the future, we may request six month priority review from the FDA and EMA for RP103 for HD and our other drug product candidates; however, the FDA and EMA may not grant it. Without priority review, the FDA and EMA review timeline could be at least 10 to 12 months. Under the FDA policies, a drug candidate is eligible for priority review from the time a complete NDA is accepted for filing, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A lengthier review process will delay revenue from the sale of products and will increase the capital necessary to fund these product development programs. Obtaining priority review from the FDA does not guarantee FDA approval of our NDA or that the FDA will not request additional information, including requesting additional clinical studies, during its review. Any request for additional information or clinical data could delay the FDA's timely review of our NDA.

Even though we have obtained U.S. regulatory approval for PROCYSBITM, we will be subject to ongoing regulatory obligations, oversight and continued regulatory review, which may result in significant additional expense.

Although we received U.S. marketing approval for PROCYSBITM, approval of PROCYSBITM in the EU could contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials and extraordinary requirements for surveillance to monitor the safety and efficacy of the drug product. Post-marketing studies and/or post-market surveillance may suggest that a product causes undesirable side effects which present an increased risk to the patient. If we or a regulatory agency discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or identify data that suggest that one of our approved products may present a risk to safety, the regulatory authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is delayed or withdrawn, our growth prospects and our operating results will be adversely affected.

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

Sales of PROCYSBITM, RP103 and our other product candidates outside of the U.S. will be subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approvals. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries, including the EMA, must also approve the manufacturing and marketing of our product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., including additional preclinical studies or clinical trials or manufacturing and control requirements. In many countries outside the U.S., a product candidate must be approved for reimbursement before it can be approved for sale in that country. In many cases, the price that we propose to charge for our products is also subject to approval by individual countries before we can launch our product candidates in those countries. Obtaining foreign regulatory approvals, complying with foreign regulatory requirements and gaining approved pricing and reimbursement could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries and regulatory approval in one country does not ensure approval in any other country, while a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others.

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

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

Table of Contents

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

The use of any of our drug product candidates in clinical trials or the commercialization of PROCYSBITM may expose us to liability claims.

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

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

We have no internal manufacturing experience and expect to continue to rely on a single third-party manufacturer to produce drug products that adequately support our commercial sales of PROCYSBI TM, our clinical trials of RP103 and potential commercial sales of RP103 if approved for other indications. If we fail to adequately supply PROCYSBI TM to commercial patients or RP103 for patients in clinical trials, our reputation would be harmed, our revenues could be delayed and our financial results could be adversely affected.

We do not currently manufacture PROCYSBITM and RP103 and do not currently plan to develop the internal capacity to do so. We rely on a single manufacturer of the delayed release capsules as finished products of PROCYSBI TM and RP103. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production to commercial requirements. These problems include difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Our third-party manufacturer and key suppliers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes, severe weather events, unstable political environments at foreign facilities or financial difficulties. If this manufacturer or key suppliers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to successfully launch PROCYSBI TM, or develop or launch any other product candidate based on RP103 would be jeopardized.

In addition, we rely on one exclusive supplier for the active pharmaceutical ingredient, or API, for PROCYSBITM and RP103. While we work closely with this supplier, along with our exclusive finished goods supplier, to ensure continuity of supply while maintaining high quality and reliability, we cannot guarantee that these efforts will be successful. A reduction or interruption in our supply of API from this supplier and finished goods from our contract manufacturer, and efforts to identify and qualify alternative sources of API supply, could result in significant additional operating costs and delays in commercializing PROCYSBI TM and in developing RP103 for HD and NAFLD. In addition, supply arrangements from alternative sources may not be available on acceptable economic terms, if at all.

36

Table of Contents

In addition, our manufacturers and suppliers are subject to the FDA's current cGMP requirements and other FDA requirements, Drug Enforcement Administration's regulations and other rules and regulations prescribed by non-U.S. regulatory authorities. We depend on our third party suppliers and manufacturers for compliance with these requirements, and they may not be able to do so. If we or our third party suppliers and manufacturers fail to comply with applicable regulatory requirements, a regulatory agency may issue warning letters or untitled letters; seek an injunction or impose civil or criminal penalties or monetary fines; suspend or withdraw regulatory approval; suspend any ongoing clinical trials; refuse to approve pending applications or supplements to applications filed by us; suspend or impose restrictions on operations; including costly new manufacturing requirements; seize or detain products; or request that we initiate a product recall. The occurrence of any of these regulatory actions or penalties may inhibit our ability to commercialize our product and generate revenue.

We rely on third parties for the distribution and pharmaceutical services of PROCYSBITM in the U.S. If these third parties fail to perform under our agreements with them, it may harm our reputation and may result in reduced revenues which may harm our financial condition.

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

Our reliance on third parties may result in delays in completing, or a failure to complete, preclinical testing, clinical trials or regulatory marketing submissions if they fail to perform under our agreements with them.

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

governmental agencies, U.S. and international university laboratories;

other biotechnology companies;

contract manufacturing organizations;

clinical research organizations;

distribution and supply (logistics) service organizations;

testing organizations;

consultants or consulting organizations with specialized knowledge based expertise;

intellectual property legal firms; and

multiple other service organizations.

If we engage these organizations to help us with our product development programs, many important aspects of this process have been and will be out of our direct control. If any of these organizations we engage in the future fail to perform their obligations under our agreements with them or fail to perform in a satisfactory manner, we may face delays in completing our development and commercialization processes for any of our drug product candidates. Furthermore, any loss or delay in obtaining contracts with such entities may also delay the completion of our clinical trials, regulatory filings and the potential market approval of our drug product candidates.

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

37

Table of Contents

Our dependence on collaborative arrangements with other independent parties will subject us to a number of risks that could harm our ability to develop and commercialize products:

collaborative arrangements might not be available on terms which are reasonably favorable to us, or at all;

disagreements with partners may result in delays in the development and marketing of products, termination of collaboration agreements or time consuming and expensive legal action;

agreement terms may be difficult or costly to enforce:

we cannot control the amount and timing of resources partners devote to product candidates or their prioritization of product candidates, and partners may not allocate sufficient funds or resources to the development, promotion or marketing of our product candidates, or may not perform their obligations as expected;

partners may choose to develop, independently or with other companies, alternative products or treatments, including products or treatments which compete with ours;

agreements with partners may expire or be terminated without renewal, or partners may breach collaboration agreements with us;

business combinations or significant changes in a partner's business strategy might adversely affect that partner's willingness or ability to complete its obligation to us; and

the terms and conditions of the relevant agreements may no longer be suitable.

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

If we do not obtain the support of new, and maintain the support of existing, key scientific and medical collaborators, it may be difficult to develop PROCYSBI™, RP103 and new products and establish those products as a standard of care for various indications.

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

Government health care reform could increase our costs, which could adversely affect our financial condition and results of operations.

Our industry is highly regulated and changes in law may adversely impact our business, operations or financial results. The Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Affordability Reconciliation Act of 2010, or the PPACA, is a sweeping measure intended to expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program.

Several provisions of the new law, which have varying effective dates, may affect us, including our costs. For example, the PPACA increased the Medicaid rebate rate, revised the definition of "average manufacturer price" for reporting purposes, which could further increase the amount of the Medicaid drug rebates paid to states, and extended the rebate program to beneficiaries enrolled in Medicaid managed care organizations. The PPACA also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or "donut hole." The law also established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the U.S. The PPACA includes a provision to increase the Medicaid rebate for line extensions or reformulated drugs (NDA Type 3) priced higher than the original drug. Depending on the final regulations this could substantially increase our Medicaid rebate rate (in effect limiting reimbursement for these patients) if we participate in the Medicaid Rebate Program. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with healthcare practitioners. Although the U.S. Supreme Court recently upheld most of the PPACA, it remains unclear whether there will be any changes made to certain provisions of PPACA through acts of Congress at some point in the future. In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, the President signed into law the Budget Control Act of 2011, which may result in such changes as aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. The full impact on our business of PPACA and the Budget Control Act is uncertain. We cannot predict whether other legislative changes will be adopted, if any, or how such changes would affect the pharmaceutical industry generally and specifically the commercialization of PROCYSBI™

Table of Contents

We are or may be subject to various healthcare regulations, and if we fail to comply with such regulations, we could face substantial penalties.

The laws that may affect our ability to operate as a commercial organization include:

the federal healthcare programs' Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs;

federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;

federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information;

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

U.S. and European reporting requirements detailing interactions with and payments to healthcare providers.

If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to market PROCYSBI™, RP103 and other future drug candidates and adversely impact our financial results.

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

We face burdens relating to the recent trend toward stricter corporate governance and financial reporting standards. Legislation or regulations such as the Sunshine Act, Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the Dodd-Frank Wall Street Reform and Consumer Protection Act, as well as other rules implemented by the FDA, the SEC and NASDAQ, follow the trend of imposing stricter corporate governance and financial reporting standards and have led to an increase in the costs of compliance, including substantial increases in consulting, auditing and legal fees. New rules could make it more difficult or more costly for us to obtain certain types of insurance, including directors' and officers' liability insurance, and we may be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers. Failure to comply with these new laws and regulations may impact market perception of our financial condition and could materially harm our business. Additionally, it is unclear what additional laws or regulations may develop, and we cannot predict the ultimate impact of any future changes in law. Our management and other personnel will need to devote a substantial amount of time to these requirements.

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

Table of Contents

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

Successful implementation of the U.S. commercial launch of PROCYSBITM, the potential EU commercial launch of PROCYSBI TM (if approved in the EU), the continuation of our clinical-stage programs and our current plans to in-license and acquire additional clinical-stage product candidates will require us to retain existing and add required new qualified and experienced personnel in the commercial, regulatory, manufacturing, quality, program management, clinical and medical areas over the next several years. Also, as our preclinical pipeline diversifies through internal discoveries, or the acquisition or in-licensing of new molecules, we will need to hire additional scientists to supplement our existing scientific expertise over the next several years.

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

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

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

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

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

the potential strain on our financial and managerial controls and reporting systems and procedures;

potential miscommunication between U.S. personnel and European personnel due to cultural and language differences;

ability to operate within diverse individual country regulatory and statutory laws; and
greater than anticipated costs of maintaining EU presence, in-country legal entities and related tax structures.

40

Table of Contents

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

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

Our business could be adversely affected by macroeconomic conditions.

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

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

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

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

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

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

Table of Contents

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

Our success is dependent in part upon the availability of our senior executive officers, including Christopher M. Starr, Ph.D., Chief Executive Officer; Julie Anne Smith, Chief Operating Officer; Georgia Erbez, Chief Financial Officer; Ted Daley, Chief Business Officer and Patrice Rioux, M.D., Ph.D., Chief Medical Officer. The loss or unavailability to us of any of these individuals or key research and development personnel, and particularly if lost to competitors, could have a material adverse effect on our business, prospects, financial condition and operating results. We have no key-man insurance on any of our employees.

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

If we do not achieve our projected development and commercialization goals in the time frames we expect and announce, the credibility of our management and our organizational competence may be adversely affected.

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

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

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

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

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

Table of Contents

Risks Related to Intellectual Property and Competition

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

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

The patent positions of biopharmaceutical products are complex and uncertain.

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

We do not know whether our patent applications will result in issued patents. For example, we may not have developed a method for treating a disease before others developed similar methods;

Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us, or file patent applications before we do. Competitors may also claim that we are infringing on their patents and therefore cannot practice our technology as claimed under our patents, if issued. Competitors may also contest our patents, if issued, by showing the patent examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose that patent. As a Company, we have no meaningful experience with competitors interfering with our patents or patent applications;

Enforcing patents is expensive and may absorb significant management time. Management would spend less time and resources on developing drug product candidates. The processes of defending patents and related intellectual property could increase our operating expenses and delay product programs; and

Receipt of a patent may not provide much practical protection. If we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent.

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

Defending a lawsuit takes significant time is typically very expensive;

If a court decides that our drug product candidate infringes on the competitor's patent, we may have to pay substantial damages for past infringement;

A court may prohibit us from selling or licensing the drug product candidate unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, we may have to pay substantial royalties or grant cross licenses to our patents; and

Redesigning our drug product candidates so we do not infringe may not be possible or practical and could require substantial funds and time.

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

Table of Contents

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

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

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

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

UCSD and Yeda may terminate their respective agreements with us upon the occurrence of certain events, including if we enter into certain bankruptcy proceedings or if we materially breach our payment obligations and fail to remedy the breach within the permitted cure periods. Although we are not currently involved in any bankruptcy proceedings or in breach of these agreements, there is a risk that we may be in the future, giving UCSD and Yeda the right to terminate their respective agreements with us. We have the right to terminate these agreements at any time by giving prior written notice. If the UCSD or Yeda agreements are terminated by either party, we would lose our rights to PROCYSBI TM and RP103 in the case of UCSD and would lose our rights to the Weizmann and Niigata patents in the case of Yeda. Under such circumstances, we would have no further right to use or exploit the patents, copyrights or trademarks in those respective technologies. If this happens, we would not be able to sell PROCYSBI TM, we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected, and we may have to cease our operations.

Companies and universities, including those that have licensed product candidates to us for research, clinical development and marketing, are sophisticated competitors that could develop similar products to compete with our products which could reduce our future revenues.

Licensing our product candidates from other companies, universities or individuals does not always prevent them from developing non-identical but competitive products for their own commercial or research purposes, or from pursuing patent protection in areas that are competitive with us. While we seek patent protection for all of our owned and licensed product candidates, our licensors or assignors or other research organizations who created these product candidates are experienced scientists and business people who may continue to do research and development and seek patent protection in the same areas that led to the discovery of the product candidates that are licensed or assigned to us. By virtue of the previous research that led to the discovery of the drugs or product candidates that they licensed or assigned to us, these companies, universities or individuals may be able to develop and out-license or market competitive products in less time than might be required to develop a product with which they have no prior experience and may reduce our future revenues from such product candidates. In some instances, information published in the scientific literature can provide insights which could enable development of viable competitive

product candidates on an accelerated time frame.

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

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

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

44

Table of Contents

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

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

Risks Related to Our Financial Position and Capital Requirements

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

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

conduct research, preclinical testing and human studies and clinical trials;

establish or contract for pilot scale and commercial scale manufacturing processes and facilities;

market and distribute PROCYSBITM and our future approved products; and

establish and develop quality control, manufacturing, regulatory, medical, distribution, marketing, sales, finance and administrative capabilities to support these programs.

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

the effectiveness of our commercialization activities;

the scope and results of preclinical testing and human clinical trials;

the pace of scientific progress in our research and development programs and the magnitude of these programs;

our ability to obtain, and the time and costs involved in obtaining, regulatory approvals;

the cost of manufacturing scale-up for new product candidates;

our ability to prosecute, maintain and enforce, and the time and costs involved in preparing, filing, prosecuting, maintaining and enforcing, patent claims;

competing technological and market developments;

our ability to establish additional collaborations; and

changes in our existing collaborations.

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

Table of Contents

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

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

As of March 31, 2013, we had an accumulated deficit of approximately \$151.9 million. We expect to continue to incur losses for the foreseeable future and must obtain significant financing to fund our planned operations. Our recurring losses from operations to date raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the transitional four-month period ended December 31, 2012, with respect to this uncertainty. We will need to raise additional capital and/or generate significant revenue at profitable levels to continue to operate as a going concern. In addition, the perception that we may not be able to continue as a going concern may cause third parties to choose not to do business with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

We believe that without regard to any other future sources of funds, our cash, cash equivalents and short-term investments as of March 31, 2013 of approximately \$58.4 million, plus estimated net proceeds of approximately \$23.4 million that we expect to receive in May 2013 with respect to the second tranche under our HC Royalty Loan agreement, will be sufficient to meet our projected operational requirements and obligations into the second quarter of 2014.

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

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

The required payments of principal and interest on our indebtedness under the HC Royalty Loan may require a substantial portion, or all, of our available cash to be dedicated to the service of these debt obligations. The loan bears interest at an annual fixed rate of 10.75% and a synthetic royalty based on the amount of PROCYSBI™ and other future approved product net revenues in a calendar year, and such royalty is payable quarterly. Principal payments under the HC Royalty Loan will become due beginning on the ninth quarterly payment date occurring after the date the second \$25.0 million tranche is funded and, in the case of the first tranche loan, in no event later than March 31, 2017.

There is no assurance that our business will generate sufficient cash flow or that we will have capital resources in an amount sufficient to enable us to pay our indebtedness to HC Royalty. If our cash flows and capital resources are insufficient to fund these debt service obligations, we may be forced to reduce or delay product development, sales

and marketing, and capital and other expenditures and we may be forced to restructure our indebtedness or raise additional capital through the issuance of equity or debt instruments. We cannot ensure that we will be able to refinance any of our indebtedness or raise additional capital on a timely basis, in sufficient amounts, on satisfactory terms or at all. In addition, the terms of the HC Royalty Loan may limit our ability to pursue any of these financing alternatives and these alternatives may not enable us to meet our scheduled debt service obligations. Failure to meet our debt service obligations may result in an event of default under the HC Royalty Loan, which would permit the lender to accelerate the payment of all of our indebtedness to HC Royalty and interest thereon, take possession of, foreclose on, sell, assign or grant a license to use, our pledged collateral and assign and transfer the pledged stock of our subsidiaries. This could have a material adverse impact on our financial condition and results of operations.

46

Table of Contents

Risks Related to Our Common Stock

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

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

In addition, we have an at-the-market sales agreement with Cowen and Company which, as of March 31, 2013, allows us to sell up to an additional \$12.7 million worth of stock, which, if utilized further, will create substantial dilution for our existing stockholders.

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

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

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

Table of Contents

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

Our common stock is quoted on the NASDAQ Global Market. The trading price of our common stock has been and may continue to be volatile. Our operating performance, both financial and in the development of approved products, does and will continue to significantly affect the market price of our common stock. We face a number of risks including those described herein, which may negatively impact the price of our common stock. Some of the factors that may cause the market price of our common stock to fluctuate include:

the perceived success or failure of our launch of PROCYSBITM;

the decision by the EMA of our MAA for PROCYSBITM in the EU;

the results of ongoing preclinical studies and planned early stage clinical trials of our preclinical drug candidates;

the results and timing of regulatory reviews relating to our drug candidates;

failure of any of our drug candidates, if approved, to achieve commercial success and, in particular, the rate of market penetration and sales growth in the launch period;

the results of our current and any future clinical trials of our current drug candidates;

issues in manufacturing our drug candidates or any approved products;

the entry into, or termination of, key agreements, including key strategic alliance agreements;

failure to meet security analysts' and investors' expectations;

the initiation of, material developments in, or conclusion of litigation to enforce or defend any of our intellectual property rights;

general and industry-specific economic conditions that may affect our product program expenditures;

the results of clinical trials conducted by others on drugs that would compete with our drug candidates;

the loss of key employees;

the introduction by others of technological innovations or new commercial products or development of product programs which have a direct negative competitive impact on our products or product development programs;

changes in estimates or recommendations by securities analysts, if any, who cover our common stock or influence the level of investor confidence in our sector of the equity market;

future sales of our common stock or exercise of common stock warrants or options;

changes in the structure of health care payment systems; and

period-to-period fluctuations in our financial results.

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

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

48

Table of Contents

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

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

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

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

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

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

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES.

None.

ITEM 4. MINE SAFETY DISCLOSURES.

None.

ITEM 5. OTHER INFORMATION.

None.

ITEM 6. EXHIBITS

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

49

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.

Signatures	Title	Date
/s/ Christopher M. Starr		May 12, 2014
Christopher M. Starr, Ph.D.	Chief Executive Officer and Director (Principal Executive Officer)	
/s/ Georgia Erbez		May 12, 2014
Georgia Erbez	Chief Financial Officer, Secretary and Treasurer (Principal Financial Officer and Principal Accounting Officer)	

Table of Contents

Exhibit Index

- (2) Plan of acquisition, reorganization, arrangement, liquidation or succession
- 2.1† Agreement and Plan of Merger and Reorganization, dated as of June 7, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Registration Statement No. 333-136018 filed on July 25, 2006).
- 2.2† Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated as of August 25, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Amendment No. 1 to Registration Statement No. 333-136018 filed on August 25, 2006).
- 2.3† Agreement and Plan of Merger and Reorganization, dated July 27, 2009, by and among Raptor Pharmaceuticals Corp., TorreyPines Therapeutics, Inc., a Delaware corporation, and ECP Acquisition, Inc., a Delaware corporation (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
- (3)(i),(ii) Articles of incorporation; Bylaws
- 3.1† Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
- 3.2† Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on February 26, 2014).
- 3.3† Certificate of Amendment filed with the Secretary of State of the State of Nevada effecting an 8-for-1 reverse stock of the Registrant's common stock and changing the name of the Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.3 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
- 3.4† Articles of Conversion filed with the Secretary of State of the State of Nevada changing the state of incorporation of the Registrant (incorporated by reference to Exhibit 3.4 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
- 3.5† Certificate of Conversion filed with the Secretary of State of the State of Delaware (incorporated by reference to Exhibit 3.5 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
- 3.6† Charter Amendment for TorreyPines (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 5, 2009).
- 3.7† Certificate of Merger between Raptor Pharmaceuticals Corp., ECP Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 5, 2009).

Table of Contents

(4) Instruments defining the rights of security holders, including indentures

- 4.1† Specimen common stock certificate of the Registrant (incorporated by reference to Exhibit 4.7 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
- 4.2† Form of Warrant issued to Oxford Financial and Silicon Valley Bank on September 27, 2005 (incorporated by reference to Exhibit 4.16 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.3† Form of Warrant issued to Comerica Bank on June 11, 2008 (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed on June 17, 2008).
- 4.4† Rights Agreement, dated as of May 13, 2005, between Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on May 16, 2005).
- 4.5(a)† Amendment to Rights Agreement, dated as of June 7, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed on June 12, 2006).
- 4.5(b)† Amendment to Rights Agreement, dated as of October 3, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.19 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.5(c)† Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between TorreyPines and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company) (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
- 4.5(d)† Amendment to Rights Agreement, dated August 6, 2010, by and between the Registrant and American Stock Transfer & Trust Company, LLC (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on August 10, 2010).
- 4.6*† Warrant to purchase common stock dated December 14, 2007 issued to Flower Ventures, LLC (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April 15, 2008).
- 4.7*† Warrant Agreement Amendment, dated December 17, 2009, between the Registrant and Flower Ventures, LLC (incorporated by reference to Exhibit 4.15 to Registrant's Quarterly Report on Form 10-Q, filed on April 9, 2010)
- 4.8*† Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K/A, filed on May 28, 2008).
- 4.9*† Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on August 25, 2009).
- 4.10† Form of Investor Warrants (incorporated by reference to Exhibit 10.1 on Registrant's Current Report on Form 8-K filed on December 18, 2009).

4.11 Form of Investor Warrants (incorporated by reference to Exhibit 4.1 on Registrant's Current Report on Form 8-K filed on August 10, 2010).

4.12 Placement Agent Warrant (incorporated by reference to Exhibit 4.2 on Registrant's Current Report on Form 8-K filed on August 13, 2010).

52

Table of Contents

(10) Material contracts

Loan Agreement, dated as of December 20, 2012, by and among Registrant, HealthCare Royalty Partners II, L.P. and the Guarantors party thereto. (This exhibit 10.1 supersedes in its entirety exhibit 10.38 to Registrant's Transition Report on Form 10-KT filed on March 14, 2013.)

(15) Letter regarding Unaudited Interim Financial Information

15.1 Awareness Letter of Grant Thornton LLP, Independent Registered Public Accounting Firm

(31) Section 302 Certification

31.1 Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director

31.2 Certification of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer

(32) Section 906 Certification

32.1 Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director, and of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer

101 The following materials from the Raptor Pharmaceutical Corp. Quarterly Report on Form 10-Q/A for the quarter ended March 31, 2013, formatted in Extensible Business Reporting Language (XBRL): (i) the Condensed Consolidated Balance Sheets; (ii) the Condensed Consolidated Statements of Comprehensive Loss; (iii) the Condensed Consolidated Statement of Stockholders' Equity; (iv) the Condensed Consolidated Statements of Cash Flows; and (v) related notes, tagged as blocks of text.

* The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.6 - 4.9 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.

** Certain information omitted pursuant to a request for confidential treatment filed with and granted by the SEC.

† Previously filed.