Raptor Pharmaceutical Corp
Form 10-Q
July 10, 2012
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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

x QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended May 31, 2012
or

¨ TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to
Commission File Number: 000-25571

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

Delaware	86-0883978
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
9 Commercial Blvd., Suite 200, Novato, CA 94949	
(Address of principal executive offices) (Zip Code)	

(415) 382-8111
(Registrant's telephone number, including area code)
(Former name, former address and former fiscal year, if changed since last report)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934, during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes x No ¨
Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x No ¨
Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller

reporting company" in Rule 12b-2 of the Exchange Act.:

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

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

There were 49,128,366 shares of the registrant's common stock, par value $0.001, outstanding as of June 28, 2012.

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PART I - FINANCIAL INFORMATION
Item 1. Financial Statements.
Raptor Pharmaceutical Corp.
(A Development Stage Company)
Condensed Consolidated Balance Sheets

	May 31, 2012 (unaudited)	August 31, 2011 (1)
ASSETS		
Current assets:		
Cash and cash equivalents	$17,760,689	$15,172,086
Restricted cash	168,876	114,468
Short-term investments	25,381,868	-
Prepaid expenses and other	2,446,670	415,944
Total current assets	45,758,103	15,702,498
Intangible assets, net	3,141,417	3,250,917
Goodwill	3,275,403	3,275,403
Fixed assets, net	316,057	76,997
Deposits	104,906	104,906
Deferred offering costs	119,592	151,783
Total assets	$52,715,478	$22,562,504
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Liabilities		
Current liabilities:		
Accounts payable	$1,912,321	$847,137
Accrued liabilities	3,006,495	2,249,254
Common stock warrant liability	19,137,907	23,575,294
Deferred rent	20,101	24,136
Capital lease liability - current	7,873	3,953
Total current liabilities	24,084,697	26,699,774
Capital lease liability - long-term	15,556	9,778
Total liabilities	24,100,253	26,709,552
Commitments and contingencies		
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value, 15,000,000 shares authorized, zero shares issued and outstanding	-	-
Common stock, $0.001 par value, 150,000,000 shares authorized 49,086,807 and 35,569,188 shares issued and outstanding as at May 31, 2012 and August 31, 2011, respectively	49,089	35,569
Additional paid-in capital	135,005,481	73,817,083
Accumulated other comprehensive income (loss)	(15,840)	1,904

Deficit accumulated during development stage	(106,423,505)	(78,001,604)
Total stockholders' equity (deficit)	28,615,225	(4,147,048)
Total liabilities and stockholders' equity (deficit)	$52,715,478	$22,562,504

(1) Derived from the Company's audited consolidated financial statements as of August 31, 2011.
The accompanying notes are an integral part of these condensed consolidated financial statements.

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Raptor Pharmaceutical Corp.
(A Development Stage Company)
Condensed Consolidated Statements of Comprehensive Loss
(Unaudited)

	For the three months ended	
	May 31, 2012	May 31, 2011
Revenues:	$-	$-
Operating expenses:		
General and administrative	4,104,552	1,733,218
Research and development	6,019,680	3,901,651
Total operating expenses	10,124,232	5,634,869
Loss from operations	(10,124,232)	(5,634,869)
Interest income	90,806	12,116
Interest expense	(878)	(486)
Foreign currency transaction gain (loss)	44,582	(1,910)
Unrealized gain on short-term investments	55,556	-
Adjustment to fair value of common stock warrants	6,937,282	(14,641,775)
Net loss	(2,996,884)	(20,266,924)
Other comprehensive income (loss)		
Foreign currency translation adjustment	(10,861)	1,910
Comprehensive loss	$(3,007,745)	$(20,265,014)
Net loss per share:		
Basic and diluted	$(0.06)	$(0.62)
Weighted-average shares outstanding used to compute:		
Basic and diluted	48,954,000	32,594,450

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

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Raptor Pharmaceutical Corp.
(A Development Stage Company)
Condensed Consolidated Statements of Comprehensive Loss
(Unaudited)

	For the nine months ended		For the period from September 8, 2005 (inception) to
	May 31, 2012	May 31, 2011	May 31, 2012
Revenues:	$-	$-	$ -
Operating expenses:			
General and administrative	8,893,258	4,565,829	25,747,281
Research and development	15,007,330	10,266,027	54,244,620
Total operating expenses	23,900,588	14,831,856	79,991,901
Loss from operations	(23,900,588)	(14,831,856)	(79,991,901)
Interest income	262,398	31,348	634,838
Interest expense	(1,595)	(1,484)	(117,725)
Foreign currency transaction gain	122,267	89	151,129
Unrealized gain on short-term investments	140,716	-	140,716
Adjustment to fair value of common stock warrants	(5,045,099)	(18,558,182)	(27,240,562)
Net loss	(28,421,901)	(33,360,085)	(106,423,505)
Other comprehensive gain (loss)			
Foreign currency translation adjustment	(17,744)	7,459	(15,840)
Comprehensive loss	$(28,439,645)	$(33,352,626)	$ (106,439,345)
Net loss per share:			
Basic and diluted	$(0.60)	$(1.06)	
Weighted-average shares outstanding used to compute:			
Basic and diluted	47,514,195	31,536,829	

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

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Raptor Pharmaceutical Corp.
(A Development Stage Company)
Condensed Consolidated Statement of Stockholders' Equity (Deficit)
For the Nine Months Ended May 31, 2012
(Unaudited)

	Common stock		Additional paid-	Accumulated other comprehensive	Deficit accumulated during development	
	Shares	Amount	in capital	income (loss)	stage	Total
Balance at August 31, 2011	35,569,188	$35,569	$73,817,083	$ 1,904	$(78,001,604)	$(4,147,048)
Exercise of common stock warrants	1,831,078	1,833	5,011,815	-	-	5,013,648
Exercise of common stock options	122,541	123	266,173	-	-	266,296
Employee stock-based compensation expense	-	-	3,219,416	-	-	3,219,416
Consultant stock-based compensation expense	-	-	46,289	-	-	46,289
Reclassification of the fair value of warrant liabilities upon exercise	-	-	9,482,486	-	-	9,482,486
Issuance of common stock in a follow-on public offering at $4.00 per share purchase price, net of fundraising costs totaling $3,166,146	11,500,000	11,500	42,820,702	-	-	42,832,202
Issuance of common stock under an at-the-market sales agreement, net of commissions totaling $10,564	64,000	64	341,517	-	-	341,581
Foreign currency translation loss	-	-	-	(17,744)	-	(17,744)
Net loss	-	-	-	-	(28,421,901)	(28,421,901)
Balance at May 31, 2012	49,086,807	$49,089	$135,005,481	$ (15,840)	$(106,423,505)	$28,615,225

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

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Raptor Pharmaceutical Corp.
(A Development Stage Company)
Condensed Consolidated Statements of Cash Flows
(unaudited)

	For the nine months ended		For the cumulative period from September 8, 2005 (inception) to May 31, 2012
	May 31, 2012	May 31, 2011	
Cash flows from operating activities:			
Net loss	$(28,421,901)	$(33,360,085)	$ (106,423,505)
Adjustments to reconcile net loss to net cash used in operating activities:			
Employee stock-based compensation exp.	3,219,416	1,541,888	6,570,778
Consultant stock-based compensation exp.	46,289	38,016	729,611
Fair value adjustment of common stock warrants	5,045,099	18,558,182	27,240,562
Amortization of intangible assets	109,500	115,125	660,458
Depreciation of fixed assets	35,534	58,182	536,307
Unrealized gain on short-term investments	(140,717)	-	(140,717)
Write-off of intangible assets and other intellectual property	-	-	348,750
Amortization of capitalized finder's fee	-	-	102,000
Capitalized acquisition costs previously expensed	-	-	38,000
Changes in assets and liabilities:			
Prepaid expenses and other	(2,030,726)	94,824	(2,347,232)
Intangible assets	-	-	(150,000)
Deposits	-	(2,000)	(104,907)
Accounts payable	1,065,184	507,303	1,912,321
Accrued liabilities	757,241	(112,498)	2,325,769
Deferred rent	(4,035)	24,356	19,996
Net cash used in operating activities	(20,319,116)	(12,536,707)	(68,681,809)
Cash flows from investing activities:			
Purchase of fixed assets	(274,594)	(29,989)	(821,120)
Cash acquired in 2009 Merger	-	-	581,391
Increase in restricted cash	(54,408)	(114,282)	(168,876)
Purchase of short-term investments	(30,241,151)	-	(30,241,151)
Sale of short-term investments	5,000,000	-	5,000,000
Net cash used in investing activities	(25,570,153)	(144,271)	(25,649,756)
Cash flows from financing activities:			
Proceeds from the sale of common stock	46,000,000	-	85,941,278
Proceeds from the sale of common stock under an equity line	-	6,747,778	11,639,568
Proceeds from the sale of common stock under an ATM agreement	352,145	-	352,145

Proceeds from the exercise of common stock warrants	5,013,648	2,300,838	20,911,147
Proceeds from the exercise of common stock options	266,296	8,828	434,904
Fundraising costs	(3,026,579)	(8,182)	(7,353,543)
Deferred offering costs	(119,592)	-	(119,592)
Proceeds from the sale of common stock to initial investors	-	-	310,000
Proceeds from bridge loan	-	-	200,000
Repayment of bridge loan	-	-	(200,000)
Additions and payments on capital lease	9,698	(3,572)	(7,813)
Net cash provided by financing activities	48,495,616	9,045,690	112,108,094
Foreign currency translation gain (loss)	(17,744)	7,459	(15,840)
Net increase (decrease) in cash and cash equivalents	2,588,603	(3,627,829)	17,760,689
Cash and cash equivalents, beginning of period	15,172,086	16,953,524	-
Cash and cash equivalents, end of period	$17,760,689	$13,325,695	$ 17,760,689

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Supplemental disclosure of non-cash financing activities:			
Warrants issued in connection with financing	$-	$-	$16,310,414
Initial fair value of warrants issued to placement agents in connection with financings	$-	$-	$208,660
Common stock and warrants issued in connection with reverse merger	$-	$-	$4,417,046
Common stock issued as fee for equity line	$-	$352,500	$827,637
Fair value of warrant liability reclassified to equity upon exercise	$9,482,486	$1,485,643	$17,988,157
Acquisition of equipment in exchange for capital lease	$12,943	$-	$48,077
Notes receivable issued in exchange for common stock	$-	$-	$110,000
Common stock issued for a finder's fee	$-	$-	$102,000
Common stock issued in asset purchase	$-	$-	$2,898,624

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

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RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(1) NATURE OF OPERATIONS AND BUSINESS RISKS

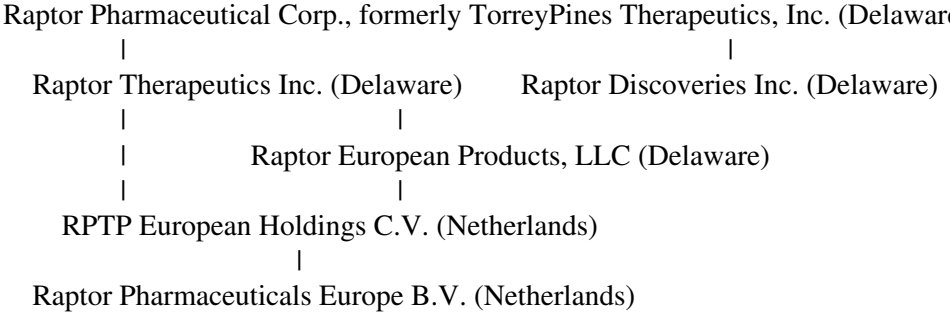
The accompanying condensed consolidated financial statements reflect the results of operations of Raptor Pharmaceutical Corp. (the "Company" or "Raptor") and have been prepared in accordance with the accounting principles generally accepted in the United States of America ("GAAP"). The Company's fiscal year end is August 31.

On July 28, 2009, the Company and ECP Acquisition, Inc., a Delaware corporation, the Company's then wholly-owned subsidiary ("merger sub"), entered into an Agreement and Plan of Merger and Reorganization (the "2009 Merger Agreement"), with Raptor Pharmaceuticals Corp., a Delaware corporation ("RPC"). On September 29, 2009, on the terms and subject to the conditions set forth in the 2009 Merger Agreement, pursuant to a stock-for-stock reverse triangular merger (the "2009 Merger"), merger sub was merged with and into RPC and RPC survived the 2009 Merger as a wholly-owned subsidiary of the Company. Immediately prior to the 2009 Merger and in connection therewith, the Company effected a 1-for-17 reverse stock split of its common stock and changed its corporate name from "TorreyPines Therapeutics, Inc." to "Raptor Pharmaceutical Corp."

As a result of the 2009 Merger and in accordance with the 2009 Merger Agreement, each share of RPC's common stock outstanding immediately prior to the effective time of the 2009 Merger was converted into the right to receive 0.2331234 shares of the Company's common stock, on a post 1-for-17 reverse-split basis. Each option and warrant to purchase RPC's common stock outstanding immediately prior to the effective time of the 2009 Merger was assumed by the Company at the effective time of the 2009 Merger, with each share of such common stock underlying such options and warrants being converted into the right to receive 0.2331234 shares of the Company's common stock, on a post 1-for-17 reverse split basis, rounded down to the nearest whole share of the Company's common stock. Following the 2009 Merger, each such option or warrant has an exercise price per share of the Company's common stock equal to the quotient obtained by dividing the per share exercise price of such common stock subject to such option or warrant by 0.2331234, rounded up to the nearest whole cent.

Immediately following the effective time of the 2009 Merger, RPC's stockholders (as of immediately prior to the 2009 Merger) owned approximately 95% of the Company's outstanding common stock and the Company's stockholders (as of immediately prior to the 2009 Merger) owned approximately 5% of the Company's outstanding common stock. RPC, the Company's wholly-owned subsidiary, was the "accounting acquirer," and for accounting purposes, the Company was deemed as having been "acquired" in the 2009 Merger. The Board of Directors and officers that managed and operated RPC immediately prior to the effective time of the 2009 Merger became the Company's Board of Directors and officers. Additionally, following the effective time of the 2009 Merger, the business conducted by RPC immediately prior to the effective time of the 2009 Merger became primarily the business conducted by the Company. In December 2011, RPC merged into Raptor Pharmaceutical Corp.

The following reflects the Company's current, post-2009 Merger corporate structure (jurisdiction of incorporation/registration):



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RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

Raptor, a publicly-traded biotechnology company, seeks to research, manufacture, and commercialize medicines that improve life for patients with severe, rare disorders. Raptor currently has product candidates in clinical development designed to potentially treat nephropathic cystinosis, Non-alcoholic Steatohepatitis ("NASH"), Huntington's Disease ("HD"), aldehyde dehydrogenase deficiency ("ALDH2"), and thrombotic disorder. Raptor's preclinical programs are based upon bioengineered novel drug candidates and drug-targeting platforms derived from the human receptor-associated protein and related proteins that are designed to target cancer and infectious diseases.
The Company is subject to a number of risks, including: the need to raise capital through equity and/or debt financings; the uncertainty 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. See the section titled "Risk Factors That May Affect Future Results" included elsewhere in this Quarterly Report on Form 10-Q.
(2) SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES
(a) Basis of Presentation
The Company's condensed consolidated financial statements include the accounts of the Company's direct and indirect wholly owned subsidiaries, Raptor Discoveries Inc., Raptor Therapeutics Inc. and Raptor European Products, LLC, such subsidiaries incorporated in Delaware on September 8, 2005 (date of inception), August 1, 2007, and February 14, 2012, respectively, and Raptor Pharmaceuticals Europe B.V. and RPTP European Holdings C.V., incorporated/registered in the Netherlands on December 15, 2009 and February 16, 2012, respectively. All inter-company accounts have been eliminated. The Company's condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. Through May 31, 2012, the Company had accumulated losses of approximately $106.4 million. Management expects to incur further losses for the foreseeable future. Management believes that the Company's cash, cash equivalents and short term investments as of May 31, 2012 of approximately $43.1 million will be sufficient to meet the Company's operating requirements and obligations through the first calendar quarter of 2013. Until the Company can generate sufficient levels of cash from its operations, the Company expects to continue to finance future cash needs primarily through proceeds from equity or debt financings and collaborative agreements with corporate partners or through a business combination with a company that has such financing in order to be able to sustain its operations until the Company can achieve profitability and positive cash flows, if ever. The Company cannot assure that such financing or transaction will be available on acceptable terms, or at all. The uncertainty of this situation raises substantial doubt about the Company's ability to continue as a going concern. The accompanying condensed consolidated financial statements do not include any adjustments that might result from the failure to continue as a going concern.
The Company's independent registered public accounting firm has audited the Company's consolidated financial statements for the years ended August 31, 2011 and 2010 and for the period from September 8, 2005 (inception) to August 31, 2011. The November 14, 2011 audit opinion included a paragraph indicating substantial doubt as to the Company's ability to continue as a going concern due to the fact that the Company is in the development stage and has not generated any revenue or sustained operating profits to date.

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RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(b) Use of Estimates
The preparation of financial statements in conformity with GAAP requires management to make certain estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the dates of the condensed consolidated financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.
(c) Functional Currency
The Company's consolidated functional currency is the U.S. dollar. Raptor Pharmaceuticals Europe B.V. ("BV"), the Company's European subsidiary, uses the European Euro as its functional currency. At each quarter end, BV's balance sheet is translated into U.S. dollars based upon the quarter-end exchange rate, while its statement of operations is translated into U.S. dollars based upon an average of the Euro's value between the beginning and end date of the reporting period. BV's equity is adjusted for any translation gain or loss.
(d) Fair Value of Financial Instruments
The carrying amounts of certain of the Company's financial instruments including cash and cash equivalents, restricted cash, prepaid expenses, accounts payable, accrued liabilities and capital lease liability approximate fair value due either to length of maturity or interest rates that approximate prevailing market rates unless otherwise disclosed in these condensed consolidated financial statements. The warrant liability is carried at fair value which is determined using the Black-Scholes option valuation model at the end of each reporting period.
(e) 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.
(f) Short-term Investments
The Company invests in short-term investments in high credit-quality funds in order to obtain higher yields on its idle cash. Short-term investments consisted of:

	May 31, 2012	August 31, 2011
Adjustable-rate government fund	$15,215,961	$ -
Ultra short-term income fund	10,165,907	-
Total short-term investments	$25,381,868	$ -

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 May 31, 2012. The investments are placed in financial institutions with strong credit ratings and management expects full recovery of the carrying amounts.
(g) 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.

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(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(h) Intangible Assets
Intangible assets include the intellectual property and other rights relating to DR Cysteamine (currently developed as RP103 and RP104), to an out-license acquired in the 2009 Merger and the rights to tezampanel and NGX 426 (oral tezampanel) also acquired in the 2009 Merger (tezampanel and oral tezampanel are referred to as tezampanel hereafter). The intangible assets related to RP103/RP104 are amortized using the straight-line method over the estimated useful life of 20 years, which is the life of the intellectual property patents. The 20-year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. The intangible assets related to the out-license will be amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents. The intangible assets related to tezampanel, which has been classified as in-process research and development, will not be amortized until development is completed, but will be tested annually for impairment.
(i) Goodwill
Goodwill represents the excess of the value of the purchase consideration over the identifiable assets acquired in the 2009 Merger. Goodwill is reviewed annually, or when an indication of impairment exists. An impairment analysis is performed, and if necessary, a resulting write-down in valuation is recorded.
(j) 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.
(k) Impairment of Long-Lived Assets
The Company evaluates its long-lived assets for indicators of possible impairment by comparison of the carrying amounts to future net undiscounted cash flows expected to be generated by such assets when events or changes in circumstances indicate the carrying amount of an asset may not be recoverable. Should an impairment exist, the impairment loss would be measured based on the excess carrying value of the asset over the asset's fair value or discounted estimates of future cash flows. During the nine months ending May 31, 2012, the Company has not identified any such impairment losses.
(l) Common Stock Warrant Liabilities
The warrants issued by the Company in the 2010 private placement contain a cash-out provision which may be triggered upon request by the warrant holders if the Company is acquired or upon the occurrence of certain other fundamental transactions involving the Company. This provision requires these warrants to be classified as liabilities and to be marked to market at each period-end commencing on August 31, 2010. The warrants issued by the Company in its December 2009 equity financing contain a conditional obligation that may require the Company to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under the Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 480, Distinguishing Liabilities from Equity ("ASC 480"), a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, the Company has classified the warrants as liabilities and will mark them to fair value at each period-end. The common stock warrants are re-measured at the end of every reporting period with the change in value reported in the Company's condensed consolidated statements of comprehensive loss. Warrants which are recorded as liabilities that are exercised are re-measured and marked to market the day prior to exercise. Upon exercise of such warrants, the fair value of such warrants is reclassified to equity.

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RAPTOR PHARMACEUTICAL CORP.
(A Development Stage Company)
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(m) Income Taxes

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

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

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

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

The Company's continuing practice is to recognize interest and/or penalties related to income tax matters as a component of income tax expense. As of May 31, 2012, there was no accrued interest and penalties related to uncertain tax positions.

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

As disclosed in the Company's Form 8-K dated April 19, 2012, the Company entered into a Platform Contribution Transaction Agreement (the "Agreement") with its wholly-owned indirect subsidiary, RPTP European Holdings C.V., relating to certain intellectual property of the Company. To date, the Company has not recorded any transactions related to such Agreement.

(n) Research and Development

The Company is a development stage biotechnology company. Research and development costs are charged to expense as incurred. Research and development expenses include medical, clinical, regulatory and scientists' salaries and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, commercial drug manufactured 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 executive, human resources and facilities expenses. Research and development expenses are offset by contra-expenses, which are reimbursements of research and development expenses received either from research collaborators or from government grants or tax rebates.

(o) In-Process Research and Development

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

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(p) 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:

	May 31, 2012	May 31, 2011
Warrants to purchase common stock	5,187,772	9,425,017
Options to purchase common stock	6,095,435	3,589,940
Total potentially dilutive securities	11,283,207	13,014,957

Net loss per share, basic and diluted, was $(0.06) and $(0.62) for the three month periods ended May 31, 2012 and 2011, respectively. Net loss per share, basic and diluted, was $(0.60) and $(1.06) for the nine month periods ended May 31, 2012 and 2011, respectively.

(q) Comprehensive Loss

Components of comprehensive loss are reported in the Company's condensed consolidated statements of comprehensive loss in the period in which they are recognized. The components of comprehensive loss include net loss and foreign currency translation adjustments.

(r) Stock Option Plan

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

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(s) Recent Accounting Pronouncements

In December 2010, the FASB issued Accounting Standards Update ("ASU") 2010-28 , Intangibles - Goodwill and Other (Topic 350): When to Perform Step 2 of the Goodwill Impairment Test for Reporting Units with Zero or Negative Carrying Amounts ("ASU 2010-28"). ASU 2010-28 modifies Step 1 of the goodwill impairment test for reporting units with zero or negative carrying amounts and requires the Company to perform Step 2 if it is more likely than not that a goodwill impairment may exist. ASU 2010-28 is effective for fiscal years and interim periods within those years, beginning after December 15, 2010. Early adoption is not permitted. The Company adopted these standards on September 1, 2011 and has determined that ASU 2010-28 had no material impact on its condensed consolidated financial statements for the three and nine month periods ended May 31, 2012, because there was no requirement to perform Step 2 due to the Company's positive carrying amount.

In December 2010, the FASB issued ASU 2010-29, Business Combinations (Topic 805): Disclosure of Supplementary Pro Forma Information for Business Combinations ("ASU 2010-29"). ASU 2010-29 is an update that addresses diversity in practice about the interpretation of the pro forma revenue and earnings disclosure requirements for business combinations if the entity presents comparative financial statements and expands the required disclosures to include a description of the nature and amount of material, nonrecurring pro forma adjustments directly attributable to the business combination included in the reported pro forma revenue and earnings. This standard is effective prospectively for business combinations for which the acquisition dates are on or after the beginning of the first annual reporting period beginning on or after December 15, 2010. Early adoption is permitted. The Company adopted these standards on September 1, 2011; however, since there were no business combinations during the three and nine month periods ended May 31, 2012, ASU 2010-29 had no material impact on the Company's financial disclosure. However, the provision will impact the financial disclosures of any business combinations in the future.

In May 2011, the FASB issued ASU 2011-04, Fair Value Measurement (Topic 820): Amendments to Achieve Common Fair Value Measurement and Disclosure Requirements in GAAP and IFRSs ("ASU 2011-04"). ASU 2011-04 is intended to result in convergence between GAAP and International Financial Reporting Standards ("IFRS") requirements for measurement of and disclosures about fair value. The amendments are not expected to have a significant impact on companies applying GAAP. Key provisions of the amendment include: a prohibition on grouping financial instruments for purposes of determining fair value, except when an entity manages market and credit risks on the basis of the entity's net exposure to the group; an extension of the prohibition against the use of a blockage factor to all fair value measurements (that prohibition currently applies only to financial instruments with quoted prices in active markets); and a requirement that for recurring Level 3 fair value measurements, entities disclose quantitative information about unobservable inputs, a description of the valuation process used and qualitative details about the sensitivity of the measurements. In addition, for items not carried at fair value but for which fair value is disclosed, entities will be required to disclose the level within the fair value hierarchy that applies to the fair value measurement disclosed. ASU 2011-04 is effective for interim and annual periods beginning after December 15, 2011. The Company has adopted these standards on March 1, 2012 and has determined that ASU 2011-04 did not have a material impact on its condensed consolidated financial statements.

In June 2011, the FASB issued ASU 2011-05, Comprehensive Income (Topic 220): Presentation of Comprehensive Income ("ASU 2011-05"). ASU 2011-05 will require companies to present the components of net income and other comprehensive income either as one continuous statement or as two consecutive statements. It eliminates the option to present components of other comprehensive income as part of the statement of changes in stockholders' equity. The standard does not change the items which must be reported in other comprehensive income, how such items are measured or when they must be reclassified to net income. This standard is effective for interim and annual periods beginning after December 15, 2011. The Company early adopted these standards as of August 31, 2011. Because ASU 2011-05 impacts presentation only, it had no effect on the Company's condensed consolidated financial statements or on its financial condition for the three and nine month periods ended May 31, 2012.

In September 2011, the FASB issued ASU 2011-08, Intangibles - Goodwill and Other (Topic 350): Testing Goodwill for Impairment ("ASU 2011-08"), which permits an entity to first assess qualitative factors to determine whether it is more likely than not that the fair value of a reporting unit is less than its carrying amount as a basis for determining whether it is necessary to perform the two-step goodwill impairment test. Because the Company has only one reporting unit, which has a fair value higher than its carrying amount, adoption of ASU 2011-08 did not have a material impact on the Company's condensed consolidated financial statements for the three and nine month periods ended May 31, 2012.

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(3) INTANGIBLE ASSETS AND GOODWILL

On December 14, 2007, the Company acquired the intellectual property and other rights to develop RP103/RP104 to treat various clinical indications from the University of California at San Diego ("UCSD") by way of a merger with Encode Pharmaceuticals, Inc., a privately held development stage company ("Encode"), which held the intellectual property license with UCSD. The intangible assets acquired in the merger with Encode were recorded at approximately $2.6 million, primarily based on the value of the Company's common stock and warrants issued to the Encode stockholders.

Intangible assets recorded as a result of the 2009 Merger were approximately $1.1 million as discussed in Note 8 below.

Summary of intangibles acquired as discussed above:

	May 31, 2012	August 31, 2011
Intangible asset (IP license for RP103/RP104) related to the Encode merger	$2,620,000	$2,620,000
Intangible assets (out-license) related to the 2009 Merger	240,000	240,000
In-process research and development (IP license for tezampanel) related to the 2009 Merger	900,000	900,000
Total intangible assets	3,760,000	3,760,000
Less accumulated amortization	(618,583)	(509,083)
Intangible assets, net	$3,141,417	$3,250,917

The intangible assets related to RP103/RP104 are being amortized monthly over 20 years, which are the lives of the intellectual property patents and the estimated useful life. The 20 year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. The intangible assets related to the out-license will be amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents. The intangible assets related to tezampanel, which has been classified as in-process research and development, will not be amortized until the product is developed. During the three month periods ended May 31, 2012 and 2011, the Company amortized $36,500 and $18,741, respectively, of intangible assets to research and development expense. During the nine month periods ended May 31, 2012 and 2011 and the cumulative period from September 8, 2005 (inception) to May 31, 2012, the Company amortized $109,500, $115,125 and $660,458 (included $41,875 related to NeuroTrans™ which was written off as of August 31, 2011), respectively, of intangible assets to research and development expense.

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

Amortization period	Amortization expense
Fiscal year ending August 31, 2012 - estimate	$ 146,000
Fiscal year ending August 31, 2013 - estimate	146,000
Fiscal year ending August 31, 2014 - estimate	146,000
Fiscal year ending August 31, 2015 - estimate	146,000
Fiscal year ending August 31, 2016 - estimate	146,000

Goodwill of $3,275,403 represents the excess of total consideration recorded for the 2009 Merger over the value of the assets assumed. The Company tested the carrying value of goodwill for impairment as of its fiscal year ended August 31, 2011 and determined that there was no impairment. Intangibles are tested for impairment whenever events indicate that their carrying values may not be recoverable. During the year ended August 31, 2011, the NeuroTrans™ asset was written off with a carrying value of $108,250 due to the termination of a collaboration agreement.

(4) FIXED ASSETS

Fixed assets consisted of:

Category	May 31, 2012	August 31, 2011	Estimated useful lives
Leasehold improvements	$145,903	$ 124,763	Shorter of life of asset or lease term
Office furniture	3,188	3,188	7 years
Laboratory equipment	503,704	285,346	5 years
Computer hardware and software	153,381	131,229	3 years
Capital lease equipment	26,674	13,730	Shorter of life of asset or lease term
Total at cost	832,850	558,256	
Less: accumulated depreciation	(516,793)	(481,259)	
Total fixed assets, net	$316,057	$ 76,997	

Depreciation expense for the three month periods ended May 31, 2012 and 2011 was $17,570 and $18,742, respectively. Depreciation expense for the nine month periods ended May 31, 2012 and 2011 and the cumulative period from September 8, 2005 (inception) to May 31, 2012 was $35,534, $58,182 and $536,307, respectively. Accumulated depreciation on capital lease equipment was $3,501 and zero as of May 31, 2012 and August 31, 2011, respectively.

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(5) FAIR VALUE MEASUREMENT

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

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

Determining which category an asset or liability falls within the hierarchy requires significant judgment. The Company evaluates its hierarchy disclosures each quarter. Assets and liabilities measured at fair value on a recurring basis at May 31, 2012 and August 31, 2011 are summarized as follows:

Assets	Level 1	Level 2	Level 3	May 31, 2012
Fair value of cash equivalents	$17,145,998	$-	$-	$ 17,145,998
Restricted cash	-	168,876	-	168,876
Short-term investments	25,381,868	-	-	25,381,868
Total	$42,527,866	$168,876	$-	$ 42,696,742
Liabilities				
Fair value of common stock warrants	$-	$-	$19,137,907	$ 19,137,907
Total	$-	$-	$19,137,907	$ 19,137,907

Assets	Level 1	Level 2	Level 3	August 31, 2011
Fair value of cash equivalents	$13,855,813	$-	$-	$ 13,855,813
Restricted cash	-	114,468	-	114,468
Total	$13,855,813	$114,468	$-	$ 13,970,281
Liabilities				
Fair value of common stock warrants	$-	$-	$23,575,294	$ 23,575,294
Total	$-	$-	$23,575,294	$ 23,575,294

Cash equivalents and short-term investments represent the fair value of the Company's investment in four money markets and two short-term bond funds, respectively, as of May 31, 2012 and three money market accounts as of August 31, 2011. As of May 31, 2012, the fair value of the Company's common stock warrant liability decreased resulting primarily from decrease in warrants outstanding due to warrants exercised, offset by an increase in the Company's common stock price compared to the stock price as of August 31, 2011.

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Marked-to-Market
The common stock warrants issued in the Company's August 2010 private placement and the Company's December 2009 equity financing are classified as liabilities under ASC 480 and are, therefore, re-measured using the Black-Scholes option valuation model at the end of every reporting period with the change in value reported in the Company's condensed consolidated statements of comprehensive loss.
For the three and nine months ended May 31, 2012 and 2011 and for the cumulative period from September 8, 2005 (inception) to May 31, 2012, as a result of the marking-to-market of the warrant liability at quarter-end and the day prior to the exercise of warrants subject to warrant liability accounting, the Company recorded a gain of approximately $6.9 million, losses of $5.0 million, $14.6 million, $18.6 million and $27.2 million, respectively, in the line item adjustment to fair value of common stock warrants in its condensed consolidated statements of comprehensive loss. See Note 9 for further discussion on the calculation of the fair value of the warrant liability.
Below is the activity of the warrant liabilities (in millions):

	Nine Month Periods Ended May 31,	
	2012	2011
Fair value of December 2009 direct offering warrants (including placement agent warrants) at beginning of the fiscal years	$ 5.9	$ 5.8
December 2009 direct offering warrants exercised	(4.8)	(1.4)
Adjustment to mark to market common stock warrants	2.1	7.1
December 2009 direct offering common stock warrant liability at fair value at May 31, 2012 and 2011	3.2	11.5
Fair value of August 2010 private placement warrants (including broker warrants) at beginning of the fiscal years	17.7	9.9
August 2010 private placement warrants exercised	(4.7)	-
Adjustment to mark to market common stock warrants	2.9	11.5
August 2010 private placement common stock warrant liability at fair value at May 31, 2012 and 2011	15.9	21.4
Total warrant liability at May 31, 2012 and 2011	$ 19.1	$ 32.9

(6) ACCRUED LIABILITIES
Accrued liabilities consisted of:

	May 31, 2012	August 31, 2011
Clinical trial costs	$1,304,985	$1,177,859
Accrued vacation and employee benefits	360,686	142,678
Accrued bonuses	445,595	478,619

Salaries and wages	174,948	125,069
Legal fees	158,312	164,761
Consulting-general and administrative	152,141	18,085
Patent costs	96,523	2,969
Milestone fee	250,000	-
Clinical trial materials	-	125,256
Other	63,305	13,958
Total accrued liabilities	$3,006,495	$2,249,254

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(7) STOCK OPTION PLANS

Effective September 1, 2006, the Company began recording compensation expense associated with stock options and other forms of equity compensation in accordance with ASC 718. Prior to September 1, 2006, the Company accounted for stock options according to the provisions of APB Opinion No. 25, Accounting for Stock Issued to Employees, and related interpretations, and therefore no related compensation expense was recorded for awards granted with no intrinsic value. The Company adopted the modified prospective transition method provided for under ASC 718, and consequently has not retroactively adjusted results from prior periods. Under this transition method, compensation cost associated with stock options now includes: (i) quarterly amortization related to the remaining unvested portion of all stock option awards granted prior to September 1, 2006, based on the grant date value estimated in accordance with the original provisions of ASC 718; and (ii) quarterly amortization related to all stock option awards granted subsequent to September 1, 2006, based on the grant date fair value estimated in accordance with the provisions of ASC 718. In addition, the Company records consulting expense over the vesting period of stock options granted to consultants. The compensation expense for stock-based compensation awards includes an estimate for forfeitures and is recognized over the requisite service period of the options, which is typically the period over which the options vest, using the straight-line method. Employee stock-based compensation expense for the three month periods ended May 31, 2012 and 2011 was $1,198,378 and $362,327, respectively. Employee stock-based compensation expense for the nine month periods ended May 31, 2012 and 2011, respectively, and for the cumulative period from September 8, 2005 (inception) to May 31, 2012 was $3,219,416, $1,541,888 and $6,570,778, respectively, of which cumulatively $5,261,406 was included in general and administrative expense and $1,309,372 was included in research and development expense. No employee stock compensation costs were recognized for the period from September 8, 2005 (inception) to August 31, 2006, which was prior to the Company's adoption of ASC 718.

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

Period*	Risk-free interest rate	Expected life of stock option	Annual volatility
September 8, 2005 (inception) to August 31, 2006**	5%	10 years	100%
Year ended August 31, 2007	4 to 5%	8 years	100%
Year ended August 31, 2008	2 to 3.75%	8 years	109 to 128%
Year ended August 31, 2009	1.5 to 3.2%	7 years	170 to 240%
Year ended August 31, 2010	2.1 to 3.1%	6 to 7 years	55 to 245%
Year ended August 31, 2011	1.6 to 2.4%	6 years	88 to 116%
Three months ended November 30, 2011	1.2%	6 years	121%
Three months ended February 29, 2012	1.12%	5 years	122%
Three months ended May 31, 2012	0.89%	5 years	124%

* Dividend rate is 0% for all periods presented.

** Stock-based compensation expense was recorded on the condensed consolidated statements of operations and statements of comprehensive loss commencing on the effective date of ASC 718, September 1, 2006. Prior to September 1, 2006, stock-based compensation was reflected only in the footnotes to the condensed consolidated statements of operations, with no effect on the condensed consolidated statements of operations, per the guidelines of APB Opinion No. 25. Consultant stock-based compensation expense has been recorded on the condensed consolidated statements of operations and statements of comprehensive loss since inception.

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If factors change and different assumptions are employed in the application of ASC 718, the compensation expense recorded in future periods may differ significantly from what was recorded in the current period.
The Company recognizes as an expense the fair value of options granted to persons who are neither employees nor directors. The fair value of expensed options was based on the Black-Scholes option-pricing model assuming the same factors shown in the stock-based compensation expense table above. Stock-based compensation expense for consultants for the three months ended May 31, 2012 and 2011 was $46,289 and $1,007, respectively. Stock-based compensation expense for consultants for the nine months ended May 31, 2012 and 2011 and for the cumulative period from September 8, 2005 (inception) to May 31, 2012 was $46,289, $38,016 and $729,611, respectively, of which cumulatively $147,295 was included in general and administrative expense and $582,316 was included in research and development expense.
A summary of the activity in the 2010 Equity Incentive Plan, the 2006 Equity Compensation Plan, as amended and the Company's other stock option plans, is as follows:

	Option shares	Weighted-average exercise price	Exercisable	Weighted-average fair value of options granted
Outstanding at September 8, 2005	-	$ -	-	$ -
Granted	580,108	$ 2.64	-	$ 2.47
Exercised	-	$ -	-	$ -
Canceled	-	$ -	-	$ -
Outstanding at August 31, 2006	580,108	$ 2.64	4,010	$ 2.47
Granted	107,452	$ 2.56	-	$ 2.31
Exercised	(3,381)	$ 2.57	-	$ 2.40
Canceled	-	-	-	$ -
Outstanding at August 31, 2007	684,179	$ 2.63	273,236	$ 2.45
Granted	223,439	$ 2.27	-	$ 2.21
Exercised	-	$ -	-	$ -
Canceled	-	$ -	-	$ -
Outstanding at August 31, 2008	907,618	$ 2.54	600,837	$ 2.39
Granted	81,595	$ 1.13	-	$ 1.04
Exercised	-	$ -	-	$ -
Canceled	-	$ -	-	$ -
Outstanding at August 31, 2009	989,213	$ 2.42	826,303	$ 2.40
Granted	302,772	$ 2.29	160,605	$ 1.24
Assumed in the 2009 Merger	161,044	$ 114.12	158,475	$ 2.63
Exercised	(37,881)	$ 1.69	-	$ 1.49
Canceled	(23,860)	$ 142.42	-	$ 2.00
Outstanding at August 31, 2010	1,391,288	$ 14.25	1,089,248	$ 1.87

Granted	2,231,790	$ 3.39	834,624	$ 2.54
Exercised	(39,302)	$ 2.44	-	$ 2.02
Canceled	(3,221)	$ 1,088.33	-	$ -
Outstanding at August 31, 2011	3,580,555	$ 6.64	1,881,349	$ 2.30
Granted	2,119,905	$ 5.13	-	$ 4.45
Exercised	(17,485)	$ 2.27	-	$ 1.97
Canceled	(477)	$ 15.81	-	$ 0.05
Outstanding at November 30, 2011	5,682,498	$ 6.09	2,051,680	$ 3.14
Granted	190,000	$ 6.87	-	$ 5.73
Exercised	(26,128)	$ 2.55	-	$ 1.96
Canceled	(4,522)	$ 429.54	-	$ -
Outstanding at February 29, 2012	5,841,848	$ 5.80	2,222,773	$ 3.25
Granted	345,015	$ 6.01	-	$ 5.02
Exercised	(78,928)	$ 2.03	-	$ 1.49
Canceled	(12,500)	$ 6.96	-	$ 6.96
Outstanding at May 31, 2012	6,095,435	$ 5.87	2,678,872	$ 3.34

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The weighted-average intrinsic values of stock options outstanding and expected to vest and stock options exercisable as of May 31, 2012 and 2011 were approximately $8.5 million, $5.3 million, $7.8 million and $4.1 million, respectively (representing 6.1 million, 2.6 million, 3.3 million and 1.5 million shares, respectively).
There were 1,230,993 options available for grant as of May 31, 2012 under the 2010 Equity Incentive Plan, as amended (the "Plan"), which was approved by the Company's Board of Directors as of February 2, 2010 and approved by its stockholders on March 9, 2010. On April 7, 2011, the Company's stockholders passed amendments to the Plan which allow for an increase of the grant pool based upon 5% of the Company's common stock outstanding as of April 7, 2011, August 31, 2011 and August 31, 2012 up to an aggregate maximum increase of 6,000,000 shares. The April 7 and August 31, 2011 replenishments added 1,629,516 and 1,778,459 shares, respectively, available for grant under the Plan. The amendments also allow for 50% accelerated vesting of unvested stock options upon a change of control as defined in the Plan. In September 2011, the Company's Board of Directors approved an amended and restated form of award agreement under the Plan, which will be used for awards granted on or after September 22, 2011. The amended and restated award agreement, subject to the terms of any applicable employment agreement, extends the termination date of the awards granted under the Plan that are vested as of such termination date due to (a) an employee's or a non-employee director's retirement at age 62 or older which employee or non-employee director has at least five (5) years of continuous service with the Company prior to such retirement, (b) the termination of a non-employee director's board membership for reasons other than for cause or retirement and (c) an employee's or a non-employee director's death (during his or her continuous service with the Company or within 90 days' of such continuous service with the Company) or permanent disability, to eighteen (18) months from the date of termination of continuous service with the Company. No further grants will be made under any previous or assumed stock option plans. As of May 31, 2012, the options outstanding under all of the Company's stock option plans consisted of the following:

	Options outstanding			Options exercisable	
Range of exercise prices	Number of options outstanding and expected to vest (#)	Weighted-average remaining contractual life (yrs.)	Weighted-average exercise price ($)	Number of options exercisable (#)	Weighted-average exercise price ($)
$0 to $1.00	9,472	6.88	0.85	1,457	0.85
$1.01 to $2.00	81,735	7.03	1.76	70,068	1.74
$2.01 to $3.00	1,465,586	6.34	2.65	1,077,895	2.59
$3.01 to $4.00	1,765,023	9.54	3.50	983,702	3.53
$4.01 to $5.00	87,412	7.77	4.58	86,172	4.71
$5.01 to $6.00	2,294,905	9.36	5.16	408,291	5.13
$6.01 to $7.00	277,515	9.76	6.48	7,500	6.67
$7.01 to $8.00	70,000	9.71	7.75	-	-
$8.01 to $964.24	43,787	3.29	249.94	43,787	249.94
	6,095,435	8.36	5.87	2,678,872	7.42

At May 31, 2012, the total unrecognized compensation cost was approximately $12.1 million. The weighted-average period over which it is expected to be recognized is 3 years.

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(8) ISSUANCE OF COMMON STOCK

As of May 31, 2012, there were 49,086,807 shares of the Company's common stock outstanding.

ISSUANCE OF COMMON STOCK PURSUANT TO COMMON STOCK WARRANT EXERCISES AND STOCK OPTION EXERCISES

During the three and nine month periods ended May 31, 2012, the Company received approximately $237,000 and $5.0 million from the exercise of warrants in exchange for the issuance of approximately 90,000 and 1.8 million shares of the Company's common stock respectively. During the cumulative period from September 8, 2005 (inception) through May 31, 2012, the Company received approximately $20.9 million from the exercise of warrants in exchange for the issuance of an aggregate of 6.9 million shares.

During the three and nine month periods ended May 31, 2012, the Company received approximately $160,000 and $266,000 from the exercise of stock options in exchange for the issuance of approximately 79,000 and 123,000 shares of the Company's common stock, respectively. For the cumulative period from September 8, 2005 (inception) through May 31, 2012, the Company received approximately $435,000 from the exercise of stock options resulting in the issuance of approximately 203,000 shares of common stock.

ISSUANCE OF COMMON STOCK PURSUANT TO AN ASSET PURCHASE AGREEMENT WITH CONVIVIA, INC.

On October 18, 2007, the Company purchased certain assets of Convivia, including intellectual property, know-how and research reports related to a product candidate targeting liver ALDH2 deficiency, a genetic metabolic disorder. The Company hired Convivia's chief executive officer and founder, Thomas E. (Ted) Daley, as President of clinical development. In exchange for the assets related to the ALDH2 deficiency program, the Company issued to Convivia 46,625 shares of its restricted, unregistered common stock, an additional 46,625 shares of its restricted, unregistered common stock to a third party in settlement of a convertible loan between the third party and Convivia, and another 8,742 shares of restricted, unregistered common stock in settlement of other obligations of Convivia. Mr. Daley, as the former sole stockholder of Convivia (now dissolved), may earn additional shares of the Company based on certain triggering events or milestones related to the development of Convivia assets (referred to as Convivia™). In addition, Mr. Daley may earn cash bonuses based on the same triggering events pursuant to his employment agreement. In January 2008, Mr. Daley earned a $30,000 cash bonus pursuant to his employment agreement for executing the Patheon formulation agreement for manufacturing Convivia™. In March 2008, Mr. Daley earned a $10,000 cash bonus pursuant to his employment agreement and was issued 23,312 shares of common stock valued at $56,000 based on the execution of an agreement to supply the Company with the active pharmaceutical ingredient for Convivia™ pursuant to the asset purchase agreement.

In October 2008, Mr. Daley was issued 23,312 shares of restricted common stock valued at $27,000 and earned a $30,000 cash bonus (pursuant to Mr. Daley's employment agreement) pursuant to the fulfillment of a clinical milestone. In July 2010, the Company issued 11,656 shares of its restricted common stock valued at $35,551 and paid a $10,000 cash bonus to Mr. Daley as a result of the execution of the license agreement with Uni Pharma for the development of Convivia™ in Taiwan. As discussed above, in aggregate, the Company has issued to Mr. Daley 58,280 shares of Raptor's common stock valued at $118,551 and paid $70,000 in cash bonuses related to Convivia™ milestones along with another $20,000 in cash bonuses related to employment milestones pursuant to Mr. Daley's employment agreement. Pursuant to ASC 730, the accounting guidelines for expensing research and development costs, the Company has expensed the value of the stock issued in connection with this asset purchase (except for milestone bonuses, which are expensed as compensation expense) as in-process research and development expense under research and development expenses in the amount of $240,625 on its consolidated statement of operations for the year ended August 31, 2008.

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MERGER OF RAPTOR THERAPEUTICS INC. AND ENCODE PHARMACEUTICALS, INC.

On December 14, 2007, the Company entered into a Merger Agreement (the "Encode Merger Agreement"), dated as of the same date, by and between the Company, its subsidiary, Raptor Therapeutics Inc. and Encode. Pursuant to the Encode Merger Agreement, a certificate of merger was filed with the Secretary of State of the State of Delaware and Encode was merged with and into Raptor Therapeutics Inc. The existence of Encode ceased as of the date of the Encode Merger Agreement. Pursuant to the Encode Merger Agreement and the certificate of merger, Raptor Therapeutics Inc., as the surviving corporation, continued as a wholly-owned subsidiary of the Company. Under the terms of and subject to the conditions set forth in the Encode Merger Agreement, the Company issued 802,946 shares of restricted, unregistered shares of the Company's common stock, par value $.001 per share (the "Common Stock") to the stockholders of Encode (the "Encode Stockholders"), options ("Company Options") to purchase 83,325 shares of Common Stock to the option holders of Encode (the "Encode Option Holders"), and warrants ("Company Warrants") to purchase 256,034 restricted, unregistered shares of Common Stock to the warrant holders of Encode (the "Encode Warrant Holders", and together with the Encode Stockholders and Encode Option Holders, the "Encode Security Holders"), as of the date of the Encode Merger Agreement. Such Common Stock, Company Options to purchase Common Stock, and Company Warrants to purchase Common Stock combine for an aggregate amount of 1,142,305 shares of Common Stock issuable to the Encode security holders as of the closing of the merger with Encode. The purchase price was valued at $2.6 million, which was reflected as intangible assets on the Company's consolidated balance sheet as of August 31, 2008, primarily based on the value of the Company's common stock and warrants issued to Encode Stockholders. The Encode Security Holders are eligible to receive up to an additional 559,496 shares of Common Stock, Company Options and Company Warrants to purchase Common Stock in the aggregate based on certain triggering events related to regulatory approval of RP103/RP104, an Encode product program described below, if completed within the five year anniversary date of the Encode Merger Agreement. The Company recorded this transaction as an asset purchase rather than a business combination, as Encode had not commenced planned principal operations at the time of the merger, such as generating revenues from its drug product candidate.

As a result of the merger with Encode, the Company received the exclusive worldwide license to RP103/RP104 (the "License Agreement"), developed by clinical scientists at the UCSD, School of Medicine. RP103/RP104 is a proprietary enterically coated formulation of cysteamine bitartrate, a cystine depleting agent currently approved by the U.S. Food and Drug Administration ("FDA"). Cysteamine bitartrate is prescribed for the management of the genetic disorder known as nephropathic cystinosis ("cystinosis"), a lysosomal storage disease. The active ingredient in RP103/RP104 has also demonstrated potential in studies as a treatment for other metabolic and neurodegenerative diseases, such as Huntington's Disease and NASH.

In consideration of the grant of the license, the Company will be obligated to pay an annual maintenance fee until it begins commercial sales of any products developed pursuant to the License Agreement. In addition to the maintenance fee, the Company will be obligated to pay during the life of the License Agreement: milestone payments ranging from $20,000 to $750,000 for orphan indications and from $80,000 to $1,500,000 for non-orphan indications upon the occurrence of certain events, if ever; royalties on commercial net sales from products developed pursuant to the License Agreement ranging from 1.75% to 5.5%; a percentage of sublicense fees ranging from 25% to 50%; a percentage of sublicense royalties; and a minimum annual royalty commencing the year the Company begins commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, the Company is obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 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. Cumulatively, Raptor has expensed $930,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, Huntington's Disease and NASH and on regulatory filings in cystinosis. In

March 2012, the Company filed its Marketing Authorization Application ("MAA") with the European Medicines Agency ("EMA"), as well as its New Drug Application ("NDA") with the U.S. Food and Drug Administration ("FDA"), for RP103 for the potential treatment of cystinosis. In conjunction with the achievement of MAA/NDA filing milestone, the Company paid $250,000 to UCSD pursuant to this license. Future milestones of $500,000 and $750,000 will be payable if the MAA and NDA for cystinosis are approved, respectively, which the Company anticipates may occur in the first half of calendar 2013.

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ISSUANCES OF COMMON STOCK AND WARRANTS IN CONNECTION WITH THE SALE OF UNITS IN PRIVATE PLACEMENTS

During the period from May 21, 2008 through June 27, 2008, Raptor entered into a Securities Purchase Agreement, as amended (the "2008 Private Placement Purchase Agreement"), with 11 investors for the private placement of units of the Company, each unit comprised of one share of Raptor's Common Stock and one warrant to purchase one half of one share of Raptor's Common Stock, at a purchase price of $2.14 per unit. Pursuant to the 2008 Private Placement Purchase Agreement, the Company sold an aggregate of 4,662,468 shares of Common Stock for aggregate gross proceeds of $10.0 million and issued to the investors warrants, exercisable for two years from the initial closing, which entitle the investors to purchase up to an aggregate of 2,331,234 shares of Common Stock of the Company and have an exercise price of either $3.22 or $3.86 per share, depending on when such warrants are exercised, if at all, and were valued at approximately $3.0 million (using the following Black-Scholes pricing model assumptions: risk-free interest rate 2%; expected term 2 years and annual volatility 121.45%).

In connection with the May/June 2008 private placement, the Company issued warrants and a cash fee to placement agents to compensate them for placing investors into the financing. Placement agents were issued warrants exercisable for 7% of Common Stock issued and issuable under the warrants issued to investors as part of the financing units and a cash fee based upon the proceeds of the sale of the units of the private placement. In connection with the sale of units, the Company issued placement agent warrants to purchase 489,559 shares of Raptor's Common Stock at an exercise price of $2.36 per share for a five year term (valued at approximately $960,000 using the following Black-Scholes pricing model assumptions: risk-free interest rate 2%; expected term 5 years and annual volatility 121.45%) and cash fees to placement agents totaling $700,000. Of the placement agents compensated, Limetree Capital was issued warrants to purchase 438,890 shares of Raptor's Common Stock and cash commission of $627,550. One of the Company's Board members served on the board of Limetree Capital.

On April 29, 2009, in order to reflect current market prices, Raptor notified the holders of warrants purchased in the May/June 2008 private placement that the Company was offering, in exchange for such warrants, new warrants to purchase its common stock at an exercise price of $1.29 per share, but only to the extent such exchange of the original warrants and exercise of the new warrants, including the delivery of the exercise price, occurred on or prior to July 17, 2009. The new warrants were valued at approximately $2.3 million based on the following Black-Scholes pricing model assumptions: risk-free interest rate 0.55%; expected term 1 year and annual volatility 231.97%. The warrants that were not exchanged prior to or on July 17, 2009 retained their original exercise prices of $3.86 per share and original expiration date of May 21, 2010. The Company received $2,614,500 of proceeds from warrant exercises that resulted in the issuance of 2,031,670 shares of Raptor's common stock pursuant to the exchange described above.

On August 21, 2009, Raptor entered into a securities purchase agreement with four investors for the private placement of units of the Company at a purchase price of $1.37 per unit, each unit comprised of one share of Raptor's common stock, par value $0.001 per share and one warrant to purchase one half of one share of Raptor's common stock. Pursuant to the securities purchase agreement, the Company sold an aggregate of 1,738,226 units to the investors for aggregate gross proceeds of $2,386,000. The 1,738,226 units are comprised of an aggregate of 1,738,226 shares of common stock and warrants to purchase up to 869,113 shares of Raptor's common stock valued at $1.0 million (using the following Black-Scholes pricing model assumptions: risk-free interest rate 1.11%; expected term 2 years and annual volatility 240.29%). The warrants, exercisable for two years from the closing, entitle the investors to purchase, in the aggregate, up to 869,113 shares of Raptor's common stock and have an exercise price of either $2.57 until the first anniversary of issuance or $3.22 per share after the first anniversary of issuance.

In connection with the August 2009 private placement, the Company issued warrants and a cash fee to Limetree Capital as its sole placement agent to compensate it for placing investors into the financing. Limetree Capital was issued warrants exercisable for 7% of common stock issued and issuable under the warrants issued to investors as part of the financing units and a 3.5% cash fee based upon the proceeds of the sale of the units of the August 2009 private placement. Limetree Capital was issued a five-year warrant to purchase 129,733 shares of Raptor's Common Stock at an exercise price of $1.50 per share (valued at approximately $171,000 using the following Black-Scholes pricing

model assumptions: risk-free interest rate 2.58%; expected term 5 years and annual volatility 240.29%) and cash commission of $59,360.

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2009 MERGER AND NASDAQ LISTING

On September 29, 2009, the Company, formerly known as TorreyPines Therapeutics, Inc. ("TorreyPines") and RPC completed a reverse merger. The Company changed its name to "Raptor Pharmaceutical Corp." and commenced trading on September 30, 2009 on the NASDAQ Capital Market under the ticker symbol "RPTP."

In connection with the exchange of shares in the merger, immediately after the effective time of such merger, RPC and the Company's stockholders owned 95% and 5% of the outstanding shares of the combined company, respectively. RPC stockholders received (as of immediately prior to such merger) 17,881,300 shares of the combined company's common stock in exchange for the 76,703,147 shares of RPC's common stock outstanding immediately prior to the closing of the merger. On September 29, 2009, immediately prior to the effective time of such merger, the Company's Board of Directors, with the consent of RPC's Board of Directors, acted to effect a reverse stock split of the issued and outstanding shares of the Company's common stock such that every 17 shares of the Company's common stock outstanding immediately prior to the effective time of the merger would represent one share of the Company's common stock. Due to the reverse stock split implemented by the Company, the 15,999,058 shares of the Company's common stock outstanding immediately prior to the closing of the merger became 940,863 shares of the combined company's common stock.

In connection with the merger and subject to the same conversion factor as the RPC common stock (.2331234), the combined company assumed all of RPC's stock options and warrants outstanding at the time of the merger. The combined company also retained the Company's stock options and warrants outstanding at the merger, subject to the same adjustment factor as described above to give effect to the 1 for 17 reverse split.

The combined company is headquartered in Novato, California and is managed by Christopher M. Starr, Ph.D., as Chief Executive Officer and director, Todd C. Zankel, Ph.D., as Chief Scientific Officer, Kim R. Tsuchimoto as Chief Financial Officer, Ted Daley, as President of clinical development and Patrice P. Rioux., M.D., Ph.D., as Chief Medical Officer of clinical development.

There were a number of factors on which RPC's Board of Directors relied in approving the 2009 Merger. The primary reason for RPC's Board of Directors' decision to merge with TorreyPines was the benefit anticipated from the additional liquidity expected from having a NASDAQ trading market on which the combined company's common stock could be listed, in addition to having access to an expanded pipeline of product candidates across a wider spectrum of diseases and markets.

The liquidity benefit is the primary factor behind the goodwill recognized in the transaction (see below). The goodwill is expected to be fully deductible for tax purposes. Below is a breakdown of the assets acquired and liabilities assumed in the merger described herein (in millions, except for %):

Asset Allocation	Value	%
Cash and equivalents	$0.58	13
Other current assets	0.10	2
Accrued liabilities	(0.68)	(15)
Intangible assets:		
In-process research and development	0.90	20
Licenses	0.24	6
Total identifiable assets	1.14	26
Plus goodwill	3.28	74

Total net assets acquired	$4.42	100

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ISSUANCES OF COMMON STOCK AND WARRANTS IN CONNECTION WITH THE SALE OF UNITS IN A REGISTERED DIRECT OFFERING

On December 17, 2009, the Company entered into a Placement Agent Agreement with Ladenburg Thalmann & Co. Inc. as placement agent (the "2009 Placement Agent"), relating to the issuance and sale to the Direct Offering Investors (as defined below) pursuant to a registered direct offering (the "Direct Offering") of up to 3,747,558 units (the "Units"), consisting of (i) 3,747,558 shares of the Company's common stock, (ii) warrants to purchase an aggregate of up to 1,873,779 shares of the Company's common stock (and the shares of common stock issuable from time to time upon exercise of such warrants) (the "Series A Warrants"), and (iii) warrants to purchase an aggregate of up to 1,873,779 shares of the Company's common stock (and the shares of common stock issuable from time to time upon exercise of such warrants) (the "Series B Warrants," and collectively with the Series A Warrants, the "Investor Warrants").

The 2009 Placement Agent received a placement fee equal to 6.5% of the gross cash proceeds to the Company from the Direct Offering of the Units or $487,183 (excluding any consideration that may be paid in the future upon exercise of the Warrants), a warrant to purchase up to an aggregate of 74,951 shares of the Company's common stock at $2.50 per share (valued at approximately $52,000 using the following Black-Scholes pricing model assumptions: risk-free interest rate 2.23%; expected term 5 years and annual volatility 49.28%) and $25,000 in out-of-pocket accountable expenses. The warrant issued to Ladenburg has the same terms and conditions as the Investor Warrants except that the exercise price is 125% of the public offering price per share or $2.50 per share, and the expiration date is five years from the effective date of the Registration Statement.

In connection with the Direct Offering, following execution of the Placement Agent Agreement, the Company also entered into a definitive securities purchase agreement (the "Direct Offering Purchase Agreement"), dated as of December 17, 2009, with 33 investors set forth on the signature pages thereto (collectively, the "Direct Offering Investors") with respect to the Direct Offering of the Units, whereby, on an aggregate basis, the Direct Offering Investors agreed to purchase 3,747,558 Units for a negotiated purchase price of $2.00 per Unit, amounting to gross proceeds of approximately $7.5 million and net proceeds after commissions and expenses of approximately $6.2 million. Each Unit consists of one share of the Company's common stock, one Series A Warrant exercisable for 0.5 of a share of the Company's common stock and one Series B Warrant exercisable for 0.5 of a share of the Company's common stock. The shares of the Company's common stock and the Warrants were issued separately. The Series A Warrants were exercisable during the period beginning one hundred eighty (180) days after the date of issue and ending on the fifth (5th) anniversary of the date of issue. The Series B Warrants were exercisable during the period beginning one hundred eighty (180) days after the date of issue and ending on the eighteen (18) month anniversary of the date of issue. The Investor Warrants have a per share exercise price of $2.45. At closing of the financing, the Series A Warrants were valued at $1.3 million (using the following Black-Scholes pricing model assumptions: risk-free interest rate 2.23%; expected term 5 years and annual volatility 49.28%) and the Series B Warrants were valued at $0.5 million (using the following Black-Scholes pricing model assumptions: risk-free interest rate 0.56%; expected term 18 months and annual volatility 49.28%). Based on the underlying terms of the Investor Warrants and Placement Agent Warrants, the Investor Warrants and the Placement Agent Warrants are classified as a liability, as discussed further below in Note 9.

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ISSUANCES OF COMMON STOCK IN CONNECTION WITH AN EQUITY LINE

On April 16, 2010, the Company signed a purchase agreement with Lincoln Park Capital Fund, LLC ("LPC"), together with a registration rights agreement, whereby LPC agreed to purchase up to $15.0 million of the Company's common stock over a 25 month period. Under the registration rights agreement, the Company agreed to file a registration statement related to the transaction with the U.S. Securities and Exchange Commission ("SEC") covering the shares that have been issued or may be issued to LPC under the purchase agreement. Such registration statement was declared effective by the SEC on May 7, 2010. Post-effective amendments to such registration statement were filed on November 23, 2010 and December 1, 2010, which amended registration statement was declared effective by the SEC on December 1, 2010. Post-effective amendments to such amended registration statement were filed on October 11, 2011 and October 14, 2011 on Form S-3, which amended registration statement was declared effective by the SEC on October 21, 2011. After May 7, 2010, the Company had the right over a 25-month period to sell its shares of common stock to LPC in amounts of $100,000 to up to $1 million per sale, depending on certain conditions as set forth in the purchase agreement, up to the aggregate amount of $15.0 million. The purchase agreement may be terminated by the Company at any time at its discretion without any cost to the Company.

The purchase price of the shares issued to LPC under the purchase agreement is based on the prevailing market prices of the Company's shares at the time of sale without any fixed discount. The Company controlled the timing and amount of any sales of shares to LPC. LPC did not have the right or the obligation to purchase any shares of the Company's common stock on any business day that the purchase price of the Company's common stock was below $1.50 per share.

In consideration for entering into the purchase agreement (the "LPC Purchase Agreement"), the Company issued to LPC 145,033 shares of common stock valued at $246,556 (recorded as deferred offering costs on the Company's condensed consolidated balance sheet and amortized over the usage of the equity line) as a commitment fee and was required to issue up to an additional 217,549 shares of its common stock pro rata as LPC purchases the $15.0 million of the Company's common stock over the 25-month period. Since inception, the Company sold 4,186,038 shares to LPC at a weighted-average price of $2.78 and paid commitment fees to LPC in the form of 168,929 shares (in addition to the 145,033 shares issued as the initial commitment fee), valued at $581,081. The Company issued an aggregate of 4,500,000 shares (including shares issued to LPC as commitment fees) to LPC pursuant to the LPC Purchase Agreement and does not plan to issue or register additional shares under such agreement.

2010 PRIVATE PLACEMENT

On August 9, 2010, the Company entered into a securities purchase agreement with 23 investors set forth on the signature pages thereto (the "U.S. Investors") and a separate securities purchase agreement with a certain Canadian investor (the "Canadian Investor") and together with the U.S. Investors, the "2010 Private Placement Investors") set forth on the signature pages thereto (collectively, the "2010 Private Placement Purchase Agreements"), for the private placement (the "2010 Private Placement") of the Company's common stock and warrants to purchase its common stock, at a purchase price of $3.075 per unit, with each unit comprised of one share of common stock and a warrant to purchase one share of common stock. JMP Securities LLC (the "2010 Placement Agent") served as the Company's placement agent in the 2010 Private Placement.

The closing of this private placement occurred on August 12, 2010. The Company issued and sold an aggregate of 4,897,614 units, comprised of 4,897,614 shares of common stock and warrants to purchase up to 4,897,614 shares of its common stock for gross proceeds of approximately $15.1 million. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share. At closing of the 2010 Private Placement, the warrants issued to investors were valued at approximately $7.8 million (using the following Black-Scholes pricing model assumptions: risk-free interest rate 1.74%; expected term 5 years and annual volatility 85.14%). As the placement agent for the 2010 Private Placement, the 2010 Placement Agent was issued one warrant to purchase 97,952 shares of the Company's common stock (valued at approximately $0.2 million, based upon the same Black-Scholes inputs as the

investor warrants), paid a cash commission of $978,911 and reimbursed for certain of its expenses incurred in connection with the 2010 Private Placement.

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In connection with the 2010 Private Placement, on August 12, 2010, the Company entered into a registration rights agreement with the 2010 Private Placement Investors, pursuant to which the Company filed with the SEC a registration statement related to the 2010 Private Placement covering the resale of the common stock issued to the 2010 Private Placement Investors under the 2010 Private Placement Purchase Agreements and the shares of common stock that will be issued to the 2010 Private Placement Investors upon exercise of the warrants, including the warrant issued to the 2010 Placement Agent. Such registration statement was declared effective on August 31, 2010. Post-effective amendments to such registration statement were filed on November 23, 2010 and December 1, 2010, which amended registration statement was declared effective by the SEC on December 1, 2010. A post-effective amendment to such amended registration statement was filed on October 11, 2011 on Form S-3, which amended registration statement was declared effective by the SEC on October 21, 2011.

2011 FOLLOW-ON PUBLIC OFFERING

On September 13, 2011, the Company closed an underwritten public offering of shares of the Company's common stock at a price to the public of $4.00 per share. The shares sold in the offering included 10.0 million shares of common stock plus an additional 1.5 million shares of common stock pursuant to the exercise by the underwriters of the over-allotment option the Company granted to them. Total gross proceeds to the Company in the offering (including in connection with the sale of the shares of common stock pursuant to the exercise of the over-allotment option) totaled $46.0 million, before underwriting discounts and commissions. The offering resulted in net proceeds to the Company of approximately $42.8 million after deduction of underwriting discounts of 6% and other offering expenses paid by the Company.

ISSUANCES OF COMMON STOCK IN CONNECTION WITH AN AT-THE-MARKET COMMON STOCK SALES PROGRAM

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 million through ATM sales on the NASDAQ Stock Market. Cowen acts as sole sales agent for any sales made under the ATM for a 3% commission on gross proceeds. The common stock is being sold at prevailing market prices at the time of the sale of common stock, and, as a result, prices may vary.

Sales in the ATM offering are being made pursuant to the prospectus supplement dated April 30, 2012, which supplements the Company's prospectus dated February 3, 2012, filed as part of the shelf registration statement that was declared effective by the SEC on February 3, 2012. Through May 31, 2012, the Company sold 64,000 shares under the ATM at a weighted-average selling price of $5.50 per share for net proceeds (after 3% commission to Cowen) of $341,581.

The following is a summary of common stock outstanding as of May 31, 2012:

Transaction	Date of Issuance	Common Stock Issued
Founders' shares	Sep. 2005	1,398,742
Seed round	Feb. 2006	466,247
PIPE concurrent with reverse merger	May 2006	1,942,695
Shares issued in connection with reverse merger	May 2006	3,100,541
Warrant exercises	Jan. 2007 - May 2012	6,881,196
Stock option exercises	Mar. 2007 - May 2012	203,104
Loan finder's fee	Sep. 2007	46,625
Convivia asset purchase	Oct. 2007 -Jun. 2010	160,272

Encode merger RP103/RP104 asset purchase	Dec. 2007	802,946
Shares issued pursuant to consulting agreement	May 2008	2,040
2008 private placement	May/Jun. 2008	4,662,468
Warrant exercises from warrant exchange	Jun./Jul. 2009	2,031,670
2009 private placement	Aug. 2009	1,738,226
Shares issued in connection with reverse merger	Sep. 2009	940,863
2009 registered direct financing	Dec. 2009	3,747,558
Shares issued to equity line investor (incl. fee shares)	Apr. 2010 - Feb. 2011	4,500,000
2010 private placement	Aug. 2010	4,897,614
2011 follow-on public offering	Sep. 2011	11,500,000
Shares issued pursuant to ATM agreement	May 2012	64,000
Total shares of common stock outstanding		49,086,807

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(9) WARRANTS
The table reflects the number of common stock warrants outstanding as of May 31, 2012:

	Number of shares exercisable	Exercise price	Expiration date
Issued in connection with Encode merger	233,309	$ 2.87	12/13/2015
Issued to placement agents in May / June 2008	432,649	$ 2.36	5/21/2013
Issued to placement agents in August 2009	65,000	$ 1.50	8/21/2014
TorreyPines warrants assumed in 2009 Merger	8,140	$ 80.86 *	6/11/2013-9/26/2015
Issued to registered direct investors in Dec. 2009	756,250	$ 2.45	12/23/2014
Issued to private placement investors in Aug. 2010	3,594,472	$ 3.075	8/11/2015
Issued to placement agent in Aug. 2010	97,952	$ 3.075	8/11/2015
Total warrants outstanding	5,187,772	$ 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. Under ASC 480, a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, the Company has classified the warrants from both financings as liabilities and will mark them to fair value at each period end.
A Black-Scholes option-pricing model was used to obtain the fair value of the warrants issued in the December 2009 and August 2010 equity financings using the following assumptions at May 31, 2012 and August 31, 2011:

	December 2009 equity financing Series A		August 2010 equity financing investors and placement agent	
	May 31, 2012	August 31, 2011	May 31, 2012	August 31, 2011
Fair value ($ millions)	3.2	5.9	15.9	17.7
Black-Scholes inputs:				
Stock price	$5.37	$ 4.73	$ 5.37	$ 4.73
Exercise price	$2.45	$ 2.45	$ 3.075	$ 3.075
Risk free interest rate	0.31 %	0.38 %	0.35 %	0.70 %
Volatility	123.5%	116.4 %	123.5 %	116.4 %
Expected term (years)	2.50	3.25	3.25	4.00
Dividend	0	0	0	0

For the three and nine month periods ended May 31, 2012 and 2011, and for the cumulative period from September 8, 2005 (inception) to May 31, 2012, as a result of the marking-to-market of the warrant liability at quarter-end and the day prior to the exercise of warrants subject to warrant liability accounting, the Company recorded a gain of approximately $6.9 million, losses of approximately $5.0 million, $14.6 million, $18.6 million and $27.2 million, respectively, in the line item adjustment to fair value of common stock warrants in its condensed consolidated statements of comprehensive loss. See Note 5 for further discussion on the marking-to-market of the warrant liability.

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(10) COMMITMENTS AND CONTINGENCIES

CONTRACTUAL OBLIGATIONS WITH THOMAS E. DALEY (ASSIGNEE OF THE DISSOLVED CONVIVIA, INC.)

Pursuant to the terms of the asset purchase agreement the Company entered into with Convivia, Inc. and Thomas E. Daley for the purchase of intellectual property related to its 4-MP product candidate program (the "Asset Purchase Agreement"), Mr. Daley will be entitled to receive the following, if at all, in such amounts and only to the extent certain future milestones are accomplished by the Company (or any of its subsidiaries thereof), as set forth below:

23,312 shares of Raptor's restricted, unregistered Common Stock within fifteen (15) days after the Company enters into a manufacturing license or other agreement to produce any product that is predominantly based upon or derived from any assets purchased from Convivia ("Purchased Assets") in quantity ("Product") if such license agreement is executed within one (1) year of execution of the Asset Purchase Agreement or, if thereafter, 11,656 shares of Raptor's restricted, unregistered Common Stock. Should the Company obtain a second such license or agreement for a Product, Mr. Daley will be entitled to receive 11,656 shares of the Company's restricted, unregistered Common Stock within 30 days of execution of such second license or other agreement. In January 2008, Mr. Daley earned a $30,000 cash bonus pursuant to his employment agreement for executing the Patheon formulation agreement for manufacturing Convivia™. On March 31, 2008, the Company issued 23,312 shares of Raptor's Common Stock valued at $56,000 to Mr. Daley pursuant to this milestone reflecting the execution of an agreement to supply the active pharmaceutical ingredient for Convivia™, combined with the execution of a formulation agreement to produce the oral formulation of Convivia™. In July 2010, the Company issued 11,656 shares of its restricted common stock valued at $35,551 and paid a $10,000 cash bonus to Mr. Daley as a result of the execution of the license agreement with Uni Pharma for the development of Convivia™ in Taiwan.

23,312 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days after it receives its first patent allowance on any patents which constitute part of the Purchased Assets in any one of certain predetermined countries (each, a "Major Market").

11,656 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days after the Company receives its second patent allowance on any patents which constitute part of the Purchased Assets different from the patent referenced in the immediately preceding paragraph above in a Major Market.

23,312 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days of completing predetermined benchmarks in a Major Market by the Company or its licensee of the first Phase 2 human clinical trial for a Product ("Successful Completion") if such Successful Completion occurs within one (1) year of execution of the Asset Purchase Agreement or, if thereafter, 11,656 shares of the Company's restricted, unregistered Common Stock within thirty (30) days of such Successful Completion. In October 2008, the Company issued 23,312 shares of Raptor's Common Stock valued at $27,000 and a $30,000 cash bonus (pursuant to Mr. Daley's employment agreement) to Mr. Daley pursuant to the fulfillment of this milestone.

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11,656 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days of a Successful Completion in a Major Market by the Company's or its licensee of the second Phase 2 human clinical trial for a Product (other than the Product for which a distribution is made under the immediately preceding paragraph).
23,312 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days after the Company or its licensee applies for approval to market and sell a Product in a Major Market for the indications for which approval is sought ("Marketing Approval").
11,656 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days after the Company or its licensee applies for Marketing Approval in a Major Market (other than the Major Market for which a distribution is made under the immediately preceding paragraph above).
46,625 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days after the Company or its licensee obtains the first Marketing Approval for a Product from the applicable regulatory agency in a Major Market.
23,312 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days after the Company or its licensee obtains Marketing Approval for a Product from the applicable regulatory agency in a Major Market (other than the Major Market for which a distribution is made under the immediately preceding paragraph above).
As discussed above, in aggregate, the Company has issued to Mr. Daley, 58,280 shares of Raptor's common stock valued at $118,551 and paid $70,000 in cash bonuses related to Convivia™ milestones along with another $20,000 in cash bonuses related to employment milestones pursuant to Mr. Daley's employment agreement.
CONTRACTUAL OBLIGATIONS WITH FORMER ENCODE STOCKHOLDERS AND UCSD RELATING TO THE ACQUISITION OF THE DR CYSTEAMINE (RP103 AND RP104) LICENSE
As a result of the merger between Raptor Therapeutics Inc. and Encode, as discussed in Note 8 above, the Encode Security Holders are eligible to receive up to an additional 559,496 shares of Raptor's common stock, Company Options and Company Warrants to purchase Raptor's common stock in the aggregate based on certain triggering events related to regulatory approval of RP103/RP104, an Encode product program, if completed within the five year anniversary date of the merger agreement.
Also as a result of the merger, the Company will be obligated to pay an annual maintenance fee to UCSD for the exclusive license to develop RP103/RP104 for certain indications of $15,000 until it begins commercial sales of any products developed pursuant to the License Agreement. In addition to the maintenance fee, the Company will be obligated to pay during the life of the License Agreement: milestone payments ranging from $20,000 to $750,000 for orphan indications and from $80,000 to $1,500,000 for non-orphan indications upon the occurrence of certain events, if ever; royalties on commercial net sales from products developed pursuant to the License Agreement ranging from 1.75% to 5.5%; a percentage of sublicense fees ranging from 25% to 50%; a percentage of sublicense royalties; and a minimum annual royalty commencing the year the Company begins commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, the Company is obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 years from the effective date of the License Agreement, depending on the indication. In addition, the Company is obligated to, among other things, secure $1.0 million in funding prior to December 18, 2008 (which the Company has fulfilled by raising $10.0 million in its May/June 2008 private placement) and annually spend at least $200,000 for the development of products (which, as of its fiscal years ended August 31, 2011, 2010 and 2009 by spending approximately $11.3 million, $6.2 million and $4.1 million, respectively, on such programs) pursuant to the License Agreement. Cumulatively, the Company has expensed $930,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, Huntington's Disease and NASH and on regulatory filings in cystinosis. In March 2012, the Company filed its MAA with the EMA, as well as its NDA with the FDA for RP103 for the potential treatment of cystinosis. In conjunction with the achievement of MAA/NDA filing milestone, the Company paid $250,000 to UCSD pursuant to this license. Future milestones of $500,000 and $750,000 will be payable if the MAA and NDA for cystinosis are approved,

respectively, which the Company anticipates may occur in the first half of calendar 2013.
To the extent that the Company fails to perform any of its obligations under the License Agreement, then UCSD may terminate the license or otherwise cause the license to become non-exclusive.

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OFFICE LEASES

In March 2006, the Company entered into a lease for the Company's executive offices and research laboratory in Novato, California and expanded the lease on January 26, 2007. Base monthly payments were subject to annual rent increase of between 3% to 5%, based on the Consumer Price Index ("CPI") and annual adjustments to base operating expenses. In October 2010, the Company executed a lease addendum to the Novato lease for an additional 3,100 square feet ($5,309 per month) starting in April 2011. In February 2012, the Company executed a second addendum to the Novato lease for an additional 1,636 square feet ($2,879.47 per month) starting in March 2012. Effective April 1, 2010, the Company's monthly base rent including base operating expenses was $10,826. Effective April 11, 2011, the Company's monthly base including base operating expenses increased to $16,135 with an adjustment for CPI and operating expenses in April 2012. Effective March 1, 2012, the monthly base including base operating expenses increased to $19,014, with an adjustment for CPI. The Novato lease expires in March 2013. In January 2010, the Company entered into a one-year lease for administrative offices in San Mateo, California for $2,655 per month. The Company anticipates continuing the San Mateo lease on a monthly basis.

During the three month periods ended May 31, 2012 and 2011, the Company's rent expense was $63,779 and $53,901, respectively. During the nine month periods ended May 31, 2012 and 2011 and the cumulative period from September 8, 2005 (inception) to May 31, 2012, the Company's rent expense was $178,285, $154,417 and $905,365, respectively.

The minimum future lease payments under this operating lease assuming a 3% CPI increase per year are as follows:

Period	Amount
June 1, 2012 to August 31, 2012	$58,755
Fiscal year ending August 31, 2013	137,094

CAPITAL LEASE

On August 31, 2011, the Company leased a photocopier which is subject to a 39-month lease at $387 per month. On March 30, 2012, the Company leased another photocopier which is subject to a 39-month lease at $365 per month. The future lease payments under the corresponding capital leases are as follows:

Period	Amount
June 1, 2012 to August 31, 2012	$2,256
Year ending August 31, 2013	9,027
Year ending August 31, 2014	9,027
Year ending August 31, 2015	5,177
Total future capital lease payments	25,487
Less interest	(2,058)
Total current and long-term capital lease liability	$23,429

Interest rate on the capital leases are 6% based on the lessor's implicit rates of return.

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CONTRACT/CLINICAL RESEARCH AGREEMENTS

During the three and nine month periods ended May 31, 2012, the Company maintained several contracts with research organizations, clinical organizations and clinical sites, primarily to assist with clinical research for its cystinosis program and its NASH clinical collaboration. The future commitments pursuant to clinical research agreements are estimated as follows:

Period	Amount
June 1, 2012 to August 31, 2012	$1,011,399
Fiscal year ending August 31, 2013	5,931,571
Fiscal year ending August 31, 2014	2,152,937

STORAGE AND CLINICAL DISTRIBUTION AGREEMENT

During the three and nine month periods ended May 31, 2012, the Company maintained an agreement with a company that stores and distributes clinical materials for Raptor's cystinosis, Huntington's Disease and NASH clinical trials. The future commitments pursuant to this agreement are estimated as follows:

Period	Amount
June 1, 2012 to August 31, 2012	$157,687
Fiscal year ending August 31, 2013	601,100
Fiscal year ending August 31, 2014	202,600

FORMULATION / MANUFACTURING AGREEMENTS

In April 2008, the Company executed an agreement with a contract manufacturing organization to formulate and manufacture RP103 for its cystinosis and Huntington's Disease programs and subsequently, for its NASH program. The costs are invoiced to the Company in installments throughout the formulation and manufacturing process. In November 2010, the Company executed a supply agreement with a contract manufacturer for the active pharmaceutical ingredient of RP103. The future commitments pursuant to these contracts related to both clinical and near-term commercial manufacturing are estimated as follows:

Period	Amount
June 1, 2012 to August 31, 2012	$2,703,681
Fiscal year ending August 31, 2013	10,577,151
Fiscal year ending August 31, 2014	8,328,598

(11) QUALIFYING THERAPEUTIC DISCOVERY PROJECT GRANT

In October 2010, the Company was awarded a tax grant under the U.S. Government's Qualifying Therapeutic Discovery Project for five of its research programs including its cystinosis, Huntington's Disease and NASH clinical programs and its HepTide™ and WntTide™ preclinical cancer research programs. The Company was granted an aggregate of approximately $1.1 million for all five programs of which, as of August 31, 2011, it had received approximately $874,000. The Company recorded the $874,000 of proceeds as a contra-research and development expense during the first two quarters of fiscal 2011. The Company records the contra-expense upon deposit of the grant proceeds. During the three and nine months ended May 31, 2012, the Company received approximately $162,000 pursuant to the government program funding guidelines and the remaining balance of approximately $36,000 was drawn but was returned to the government in March 2012 along with an additional $28,000 as recapture tax because the Company had not incurred the amount originally estimated as qualified expenses for its WntTide™ program, which was the basis

for the program funding.

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(12) SUBSEQUENT EVENTS

On June 13, 2012, the Company announced that the FDA accepted for filing of the Company's NDA for its investigational drug candidate RP103, for the potential treatment of nephropathic cystinosis. The FDA granted Standard Review designation for RP103 and has assigned the user fee goal date of January 30, 2013.

On June 18, 2012, the Company announced that enrollment was complete in its Phase 2/3 clinical trial of RP103 for the potential treatment of Huntington's Disease. The 96-patient, Phase 2/3 clinical trial is being conducted under a collaboration agreement with The Centre Hospitalier Universitaire d'Angers ("CHU d'Angers"), with 8 active clinical sites throughout France. The clinical trial is an 18-month, double-blind, placebo-controlled study to be followed by an open-label extension study with all patients taking RP103 for up to an additional 18 months. The primary end point of the clinical trial is based upon the Unified Huntington's Disease Rating Scale ("UHDRS"). Blood levels of brain-derived neurotrophic factor ("BDNF") are being measured as a secondary endpoint. Under the collaboration agreement with CHU d'Angers, Raptor supplies RP103 and placebo capsules for the clinical trial and open-label extension study in exchange for regulatory and commercial rights to the clinical trial results. Clinical expenses of the study are covered by a grant from the French government (PHRC 2004-03bis CYST-HD). Interim results of this study following 18 months of treatment are expected to be announced in the first half of calendar 2014.

On June 25, 2012, the Company announced the dosing of a first patient in its Phase 2b juvenile clinical trial evaluating the safety and potential efficacy of RP103 as a potential treatment of non-alcoholic steatohepatitis ("NASH"), an advanced form of non-alcoholic fatty liver disease ("NAFLD").

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

FORWARD-LOOKING STATEMENTS

In this Quarterly Report on Form 10-Q, in other filings with the Securities and Exchange Commission, or the SEC, and in press releases and other public statements by our officers throughout the year, we make or will make statements that plan for or anticipate the future. These "forward-looking statements," within the meaning of the Private Securities Litigation Reform Act of 1995, include statements about our future business plans and strategies, as well as other statements that are not historical in nature. These forward-looking statements are based on our current expectations. 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," "anticipates," "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. Our business' actual operations, performance, development and results might differ materially from any forward-looking statement due to various known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled "Risk Factors That May Affect Future Results" in Part II, Item 1A of this Quarterly Report on Form 10-Q and including, but not limited to, the following:

- our need for, and our ability to obtain, additional funds;
- our ability to successfully obtain adequate drug pricing and successful commercialization of our drug products;
- uncertainties relating to clinical trials and regulatory reviews;
- our dependence on a limited number of therapeutic compounds and formulations of these compounds;
- the early stage of the products we are developing;
- the acceptance of any of our future products by physicians and patients;
- competition and dependence on collaborative partners;
- loss of key management or scientific personnel;
- our ability to obtain adequate intellectual property protection and to enforce these rights;
- our ability to avoid infringement of the intellectual property rights of others; and
- the other factors and risks described under the section captioned "Risk Factors That May Affect Future Results" in Part II, Item 1A of this Quarterly Report on Form 10-Q, as well as other factors not identified therein.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, the factors discussed in this Quarterly Report on Form 10-Q, in other filings with the SEC and in press releases and other public statements by our officers throughout the year, could cause actual results or outcomes to differ materially and/or adversely from those expressed in any forward-looking statements made by us or on our behalf, and therefore we

cannot guarantee future results, levels of activity, performance or achievements and you should not place undue reliance on any such forward-looking statements. We cannot give you any assurance that such forward-looking statements will prove to be accurate and such forward-looking events may not occur. In light of the significant uncertainties inherent in such forward-looking statements, you should not regard the inclusion of this information as a representation by us or any other person that the results or conditions described in those statements or our objectives and plans will be achieved.

All subsequent forward-looking statements attributable to us or any person acting on our behalf are expressly qualified in their entirety by the cautionary statements contained or referred to herein. 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.

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MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion in conjunction with our condensed consolidated financial statements as of May 31, 2012, and the notes to such condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q. All references to "the Company", "we", "our" and "us" include the activities of Raptor Pharmaceutical Corp. and its wholly-owned subsidiaries, Raptor Pharmaceuticals Corp. (which was merged into us as of December 7, 2011), Raptor Discoveries Inc., or Raptor Discoveries, Raptor Therapeutics Inc., or Raptor Therapeutics, Raptor European Products, LLC, RPTP European Holdings C.V. and Raptor Pharmaceuticals Europe B.V. This "Management's Discussion and Analysis of Financial Condition and Results of Operations" section contains forward-looking statements. Please see "Forward-Looking Statements" for a discussion of the uncertainties, risks and assumptions associated with these statements. Our actual results and the timing of certain events could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including those discussed below and elsewhere in this Quarterly Report on Form 10-Q, particularly under the heading "Risk Factors That May Affect Future Results".

Overview

Our goal is to research, develop and commercialize proprietary prescription medicines that improve life for patients with severe, rare disorders. Our product portfolio includes both candidates from our proprietary drug targeting platforms and in-licensed and acquired product candidates.

Our current pipeline includes three clinical development programs, which we are actively developing. We also have two other clinical-stage product candidates, one of which we are seeking additional product development partners in Asia. In addition, we have three preclinical product candidates for which we are also seeking development partners.

Clinical Development Programs

Our three active clinical development programs are based on an existing therapeutic compound, cysteamine bitartrate, that we are reformulating and repurposing for potential improvement in dose administration, safety and/or efficacy in nephropathic cystinosis and for potential application in new disease indications. We are developing two formulations of cysteamine bitartrate: RP103 and RP104. RP103 is our proprietary delayed-release formulation of cysteamine bitartrate microbeads in capsules, which we believe may require less frequent dosing and could reduce gastro-intestinal side effects compared to immediate-release cysteamine bitartrate, which is the current standard of care for nephropathic cystinosis. RP104 is a delayed-release formulation of cysteamine bitartrate in tablets that we intend to develop for the potential treatment of non-alcoholic steatohepatitis, or NASH, a metabolic disorder of the liver. We received the exclusive worldwide license to RP103/RP104 for use in the field of human therapeutics for metabolic and neurologic disorders, developed by clinical scientists at the University of California, San Diego, or UCSD, School of Medicine through the 2007 merger of our clinical subsidiary and Encode Pharmaceuticals, Inc., formerly a privately-held development company.

RP103 for Nephropathic Cystinosis

Nephropathic cystinosis is an inherited error of metabolism estimated to affect a population of 2,000 patients worldwide, including 500 in the U.S. and 800 in Europe. Cystinosis is usually diagnosed in the first year of life and requires lifelong therapy. In early childhood, these patients exhibit poor growth, vision problems (photophobia) and specific kidney problems (called Fanconi syndrome) that result in increased urination, thirst, and dehydration. Without treatment, cystine crystals accumulate in tissues and organs, including the kidneys, brain, liver, thyroid, pancreas, muscles and eyes. Left untreated, the disease can be fatal by the first decade of life. Additional complications include muscle wasting, poor growth, difficulty swallowing, diabetes and hypothyroidism.

Studies have shown that cysteamine therapy may delay and/or prevent kidney transplant and other clinical manifestations of the disease. The goal of cysteamine treatment of nephropathic cystinosis is to reduce cystine levels in cells. However, patient compliance is challenging due to frequent dosing and gastrointestinal side effects. Treatment with immediate-release cysteamine bitartrate, Cystagon®, the current standard of care, requires the drug to be taken strictly every six hours, including a middle-of-the-night dose. In a recent survey of 37 patients and caregivers conducted at a June 2011 conference hosted by the Cystinosis Research Foundation, or CRF, 63% of patients indicated that the burden of nighttime dosing rated a 9 on a scale from 1 to 10. This was the most significant

compliance burden noted by patients in the survey. In addition to the dosing challenges associated with Cystagon®, side effects include gastrointestinal distress, nausea and vomiting, beyond those normally experienced as a result of the disease itself. We believe patients are engaging in frequent, concomitant and chronic use of proton-pump inhibitors, or PPIs, to reduce the gastrointestinal side effects. As a result, we believe that the required dosing regimen coupled with these adverse side effects is resulting in poor patient compliance, with approximately 70% to 80% of patients failing to comply with prescribed dosing, which in turn is resulting in inadequate disease control.

We are developing RP103 to address the compliance issues associated with Cystagon®. The primary goal of the RP103 formulation is to reduce dosing to once every 12 hours. We believe that by reducing dosing regimen compliance will increase, patients will be able to have an uninterrupted night's sleep, and parents and schools will not have to address drug administration during school hours. We also believe the RP103 formulation can improve gastrointestinal tolerability and reduce PPI use.

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Pivotal Phase 3 Clinical Trial. In July 2011, we announced that RP103 had met the primary endpoint in its Phase 3 clinical trial for the treatment of nephropathic cystinosis. The primary endpoint of the trial was non-inferiority of RP103 compared to Cystagon® in fully Cystagon®-compliant patients, as measured by white blood cell, or WBC, cystine levels, which was the established efficacy surrogate biomarker. We also reported that there were no unexpected serious safety concerns experienced by patients in the trial attributable to RP103. This pivotal Phase 3 clinical trial was designed as an outpatient study of the pharmacodynamics, pharmacokinetics, safety and tolerability of RP103 compared to Cystagon® in nephropathic cystinosis patients. The clinical trial was conducted at eight clinical research centers in the U.S. and Europe. The protocol for our Phase 3 clinical trial was a result of two rounds of discussion with the U.S. Food and Drug Administration, or FDA, under a Special Protocol Assessment, or SPA. In order to timely commence our Phase 3 clinical trial in June 2010, we did not finalize the SPA process with the FDA; however, our protocol design incorporated the FDA comments.

Of the 43 patients randomized, 41 patients completed the Phase 3 protocol, of which 38 were included in the evaluable data set, 3 not being fully compliant with the protocol while on Cystagon®. The age range of study participants was 6 to 26 years, with 87% of patients below 16 years of age. On average, the peak WBC cystine level measured in patients treated with Cystagon® was 0.54 +/- 0.05 nmol 1/2 cystine/mg protein, compared to an average peak value of 0.62 +/- 0.05 nmol 1/2 cystine/mg protein for patients treated with RP103. The mean difference was 0.08 nmol 1/2 cystine/mg protein, with a 95.8% confidence interval of 0.00-0.16 (one sided p=0.021). The non-inferiority endpoint of the clinical trial would be achieved when the upper end of the confidence interval around the mean difference of WBC cystine levels did not exceed an absolute value of 0.3. The upper end of the confidence interval in the Phase 3 clinical trial was determined to be 0.16, thus achieving the non-inferiority endpoint.

In addition to achieving the primary endpoint, patients in the study received a lower average daily dose of RP103, compared to Cystagon®. The starting dose of RP103 for patients in the Phase 3 clinical trial was initially set at 70% of their established dose of Cystagon®. The protocol allowed for a single RP103 dose increase of 25%, based on intermediate WBC cystine results, to reflect the current standard of care in establishing appropriate dosing of Cystagon® in nephropathic cystinosis patients. Approximately one-third of patients remained at 70% of their starting Cystagon® dose throughout the study. The remaining two-thirds of the patients had their RP103 dose increased. On average, the total daily, steady-state dose of RP103 in patients in the Phase 3 clinical trial was 82% of their established, incoming dose of Cystagon®.

Extension Study. All patients who completed our pivotal Phase 3 clinical trial of RP103 for the potential treatment of nephropathic cystinosis were given the opportunity to enroll in a planned voluntary extension study in which they would continue to be treated with RP103 and would make regular clinic visits to monitor WBC cystine levels and collect long-term safety and quality of life data. Of the 40 patients who entered the extension study after completing the Phase 3 clinical trial, 38 are currently still enrolled. All of these 38 patients have now been taking RP103 in the extension study for at least 6 months, with some patients having been in the extension study for as long as 22 months. We included at least 6 months of safety data for all Phase 3 completers who elected to enroll in the extension study with our New Drug Application, or NDA, and Marketing Authorization Application, or MAA, filings. We plan to keep the extension study open to all enrolled patients until RP103 becomes locally commercially available.

Based on the positive results of our Phase 3 clinical trial and on the findings of our RP103 bioequivalence study, which demonstrated similar drug exposure whether administered in whole capsule or sprinkled onto applesauce, the U.S. and E.U. regulatory agencies approved our expanded enrollment in the extension study to include patients who did not qualify for the Phase 3 clinical trial. These patients include children one to six years old and patients who have undergone a kidney transplant. Eighteen additional patients are enrolled in the expanded extension study.

NDA/MAA Submission. Based on the results from our pivotal Phase 3 clinical trial and the extension study, we submitted applications for marketing approval of RP103 for the potential treatment of nephropathic cystinosis with both the FDA and the European Medicines Agency, or EMA. In March 2012, the EMA validated our MAA for RP103 for the potential treatment of nephropathic cystinosis. Validation of the MAA confirms that the submission is sufficiently complete for the EMA to begin its formal review process. We anticipate a decision from the EMA in the first half of calendar 2013. In June 2012, the FDA accepted for filing our NDA for our investigational drug candidate RP103, for the potential treatment of nephropathic cystinosis. The FDA granted Standard Review designation for RP103 and has assigned the user fee goal date (upon which we anticipate a decision by the FDA) of January 30, 2013.

Future milestones payments of $500,000 and $750,000 will be payable to UCSD if the MAA and NDA for nephropathic cystinosis are approved, respectively.

Preparation for Potential Commercial Launch. In anticipation of approval for RP103, we have begun building our commercial infrastructure both in the U.S. and in the E.U. to launch RP103 for the potential treatment of nephropathic cystinosis. We recently announced the appointment of Henk Doude van Troostwijk as our General Manager of European Commercial Operations. Mr. Doude van Troostwijk is responsible for building and managing our commercial operations in the E.U., initially focusing on the potential launch and subsequent marketing of RP103 for nephropathic cystinosis in anticipation of the EMA's approval of our MAA. We anticipate additional hiring in Europe as well as the U.S. in preparation for the potential commercialization of RP103 for nephropathic cystinosis. Upon regulatory approval of RP103 in the E.U., our initial plan is to focus on launching in Germany, France and the U.K., followed by other E.U. countries. We also have been working with rare disease organizations in both the U.S. and the E.U. to gain support of our efforts to market RP103 for nephropathic cystinosis, an orphan indication. Our medical team plans to evaluate potential future studies and assessments that could aid in the reimbursement process in Europe.

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In addition to hiring personnel, we are in negotiations with a reimbursement hub in the U.S., which, in conjunction with other vendors, will handle early patient education initiatives, benefits investigations, co-pay assistance, pharmacovigilance, product complaints, named patient distribution, commercial distribution, labeling and specialty pharmacy services. The goal of early patient education and benefits investigation is to be able to convert patients to commercial drug as soon as we obtain regulatory approval of RP103 in the U.S.

RP103 for Huntington's Disease

In 2008, we received FDA orphan drug designation for cysteamine formulations, including RP103, for the potential treatment of Huntington's Disease, or HD. HD is a rare hereditary condition caused by a defective gene. This gene makes an abnormal protein which leads to the degeneration of certain nerve cells in the brain. Adult-onset HD, the most common form of this disorder, usually appears in patients who are in their early 30s or 40s.

There are few treatment options for HD. Drugs that are available only help minimize some of the symptoms such as the uncontrollable movements and mood swings associated with HD. HD patients are believed to be deficient in brain-derived neurotrophic factor, or BDNF. In preclinical studies, cysteamine has shown the potential to slow the progression of HD by increasing the levels and intracellular transport of BDNF in mice and non-human primates. Centre Hospitalier Universitaire, or CHU d'Angers in France is currently conducting a Phase 2/3 clinical trial of RP103 designed to investigate potential mechanism of cysteamine in HD patients, using BDNF as a biomarker of potential efficacy. The trial commenced in October 2010, with full enrollment in June 2012. Eight clinical sites in France have enrolled 96 patients in a placebo-controlled, 18-month trial, followed by an open label trial with all placebo patients rolling onto RP103 and all non-placebo patients continuing on RP103 for up to another 18 months. The primary endpoint of the trial will be based upon the Unified Huntington's Disease Rating Scale, or UHDRS. Blood levels of brain-derived neurotrophic factor are being measured as a secondary endpoint. Under the collaboration agreement with CHU d'Angers, we supply RP103 and placebo capsules for the clinical trial and open-label extension study in exchange for regulatory and commercial rights to the clinical trial results. Clinical expenses of the study are covered by a grant from the French government. Interim results of this study following 18 months of treatment are expected to be announced in the first half of calendar 2014.

RP104 for NASH

NASH is a progressive liver disease, with a 25% incidence in obese patients. Approximately 2% to 5% of the U.S. population is afflicted with this disease, which can cause cirrhosis, liver failure and end-stage liver disease. The incidence of NASH is increasing in the U.S. adolescent population. Currently, we are not aware of any therapeutic options for NASH. The disease is generally managed with lifestyle changes such as diet, exercise and weight reduction.

Cysteamine is a precursor of the potent liver anti-oxidant glutathione, or GSH, and increasing GSH has the potential to reverse NASH-related liver damage. GSH itself does not enter easily into cells, even when given in large doses. However, GSH precursors, such as cysteamine, enter into cells and have been shown to be effective in the treatment of certain conditions by preventing significant GSH depletion. We are currently investigating the use of RP103 for the potential treatment of NASH, while we continue formulation development on a tablet formulation of delayed release cysteamine, RP104, intended for future NASH studies.

Our Phase 2a clinical trial of RP103 for the potential treatment of NASH showed a marked decline in alanine aminotransferase, or ALT, levels during the treatment period of 26 weeks with 7 of 11 juvenile patients achieving a greater than 50% reduction and 6 of 11 reduced to within normal range. Aspartate aminotransferase, or AST, levels were also improved, with patients averaging 41% reduction by the end of the treatment phase. The reduction in liver enzymes was largely sustained during the 6 month post-treatment monitoring phase. Other important liver function markers showed positive trends. Levels of cytokeratin 18, a potential marker of disease activity in Non-alcoholic Fatty Liver Disease, or NAFLD, decreased by an average of 45%. Adiponectin levels increased by an average of 35% during the treatment period. Reduced adiponectin levels are thought to be a marker of the pathogenesis and progression of NASH.

The Phase 2a trial results were consistent with ALT and AST reductions seen in patients that achieve a 10% weight loss. Body Mass Index did not change significantly during both the treatment and post-treatment phases in our Phase 2a clinical trial.

In this Phase 2a clinical trial, clinical investigators used a prototype of RP103 which demonstrated a favorable safety profile, with mean gastrointestinal symptom scores of 1.1 at baseline and 0.7 after 6 months of treatment using a rating system in which the maximum score of 14 indicates most severe gastrointestinal symptoms.

On June 25, 2012, we announced the dosing of a first patient in our Phase 2b juvenile clinical trial evaluating the safety and potential efficacy of RP103 as a potential treatment of NASH, an advanced form of NAFLD. This clinical trial is being conducted pursuant to a Cooperative Research and Development Agreement, or CRADA, that we entered into with the National Institute of Diabetes and Digestive and Kidney Diseases, or NIDDK, part of the National Institutes of Health, on December 15, 2011.

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The trial, called Cysteamine Bitartrate Delayed-Release for the Treatment of Non-alcoholic Fatty Liver Disease in Children, or CyNCh, is expected to enroll a total of 160 pediatric participants at ten U.S. centers in the NIDDK-sponsored NASH Clinical Research Network. NIDDK and we are sharing the costs to conduct the CyNCh clinical trial. The primary objective of this randomized, multicenter, double-blind, placebo-controlled Phase 2b clinical trial is to evaluate whether 52 weeks of treatment with RP103 in children reverses damage caused by NASH as measured by changes in NAFLD Activity Score, or NAS, a histological rating scale of disease activity. Secondary endpoints will include blood markers for liver health including ALT and AST as well as safety and tolerability. We anticipate potential data release in connection with the Phase 2b clinical trial in the first half of calendar 2014.

Other Clinical-Stage Product Candidates

Convivia™ for ALDH2 Deficiency

We are developing Convivia, our proprietary oral formulation of 4-methylpyrazole, or 4-MP, for the potential treatment of acetaldehyde toxicity resulting from ALDH2 deficiency. Sometimes referred to as ethanol intolerance or "Asian flush," ALDH2 deficiency is an inherited metabolic disorder affecting 40% to 50% of East Asian populations. ALDH2 deficiency impairs the activity of the liver enzyme ALDH2, the second enzyme of the primary metabolic pathway for ethanol and other alcohols. The result is an accumulation of acetaldehyde, a carcinogenic intermediate in the metabolism of ethanol, in blood and tissues of affected persons who drink alcoholic beverages. In recurrent drinkers, this disorder has been associated with increased risks of digestive tract cancers and other serious health problems. In addition to these long-term serious health risks, elevated acetaldehyde levels lead to immediate and unpleasant symptoms including facial flushing, tachycardia, or rapid heart rate, headache, nausea and dizziness. We are developing Convivia to potentially lower systemic acetaldehyde levels and reduce symptoms associated with alcohol intake by ALDH2-deficient individuals.

In 2008, we completed a Phase 2a clinical trial of Convivia taken concomitantly with alcohol, at a clinical research center in Honolulu, Hawaii. This study demonstrated that at all dose levels tested the active ingredient in Convivia reduced tachycardia, which is commonly experienced by ALDH2 deficient people who drink. The study also demonstrated that the active ingredient in Convivia reduced peak acetaldehyde levels and total acetaldehyde exposure in a subset of the study participants who possess specific genetic variants of the liver ADH and ALDH2 enzymes estimated to occur in about 15% to 20% of East Asians.

We own the intellectual property portfolio pertaining to Convivia, including method of use and formulation patents filed by us. In June 2010, we entered into an exclusive agreement with Uni Pharma Co., Ltd. to commercialize Convivia in Taiwan. Uni Pharma is responsible for clinical development, registration and commercialization of Convivia in Taiwan; however, we continue to seek pharmaceutical companies in other Asian countries to potentially license Convivia.

Tezampanel for Anti-Platelet Therapy

Thrombosis is a major cause of morbidity and mortality in the U.S. In addition to deep vein thrombosis and pulmonary embolus, thrombotic mechanisms predominate as the basis for both heart attack and stroke. During thrombosis, platelets become activated, a process involving a cascade of signaling factors, ultimately leading to aggregation and the formation of a solid mass, the thrombus, within blood vessels.

In addition to such well-known platelet signaling molecules as thromboxane A2 (blocked by aspirin) and adenosine diphosphate (blocked by Plavix), researchers have recently demonstrated the release of glutamate by platelets during platelet activation. Glutamate release by a platelet acts to stimulate release of glutamate from other platelets, potentiating aggregation and the formation of the thrombus. Released glutamate acts by binding cell surface glutamate receptors expressed on platelets themselves. One particular type of the glutamate receptor is important in platelet activation, the AMPA receptor. Compounds that specifically activate the AMPA receptor can increase platelet activation. Conversely, compounds that inhibit the AMPA receptor decrease platelet activation.

A potent inhibitor of the AMPA receptor is tezampanel, a molecule developed by Eli Lilly and Company and licensed to us. Tezampanel has been shown to inhibit human platelet activation, subsequent human platelet aggregation, and thrombosis in mice. Tezampanel has been extensively tested in Phase I clinical trials in other unrelated indications and has been demonstrated to be safe over a wide range of doses, without any serious adverse events and without any major abnormal laboratory tests. Human pharmacokinetics of tezampanel, are well characterized. We are planning a Phase I clinical trial in healthy volunteers to determine the efficacy of tezampanel in blocking platelet activation and

aggregation, which we anticipate will commence by the end of calendar 2012.

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Preclinical Product Candidates

Our preclinical platforms consist of targeted therapeutics for the potential treatment of multiple indications, including liver diseases, neurodegenerative diseases and breast cancer. We are seeking development partners for these programs. These preclinical programs include the following:

• Our receptor-associated protein, or RAP, platform consists of: HepTide™ for the potential treatment of primary liver cancer and other liver diseases; and NeuroTrans™ to potentially deliver therapeutics across the blood-brain barrier for treatment of a variety of neurological diseases.

• Our mesoderm development protein, or Mesd, platform consists of WntTide™ for the potential treatment of breast cancer.

Future Activities

Over the next 12 months, we plan to conduct research and development and general and administrative activities including: commercial preparation and drug supply for the potential launch of RP103 for the potential treatment of nephropathic cystinosis in the U.S. and E.U.; supporting our ongoing extension study of RP103 in nephropathic cystinosis until patients are converted onto commercial drug; conducting other supporting clinical studies of RP103 in nephropathic cystinosis; supplying clinical material for our ongoing clinical trial of RP103 in HD; funding the collaboration and supplying clinical material in our ongoing Phase 2b clinical trial of RP103 in NASH; funding a potential Phase 1 clinical trial of tezampanel as a potential anti-platelet agent (anticipated to commence in the second half of calendar 2012); continuing business development of our preclinical product candidates; research and development of in-licensed and newly discovered preclinical assets; supporting potential clinical trials in malaria and Parkinson's Disease (if foundation funding is obtained); and supporting associated facilities and administrative functions. We plan to seek additional business development partners for our Convivia product candidate in Asia. We may also develop new preclinical and clinical opportunities, including proprietary targets discovered in-house and in-licensed and acquired technologies.

IP Protection for RP103 for Nephropathic Cystinosis and Other Indications

Our composition and method of use patents. We have an exclusive worldwide license from UCSD to issued and pending patents covering composition of matter, or COM, method of use, or MOU, and composition of use, or COU, for RP103, a Delayed Release form of cysteamine bitartrate, to treat nephropathic cystinosis and other therapeutic indications. U.S. Patent No. 8,129,433 (expires 2027), which applications are pending in European and other countries, represents a COM patent, which covers the composition comprising cysteamine and any material that provides increased delivery to the small intestine and composition comprising enterically coated cysteamine. U.S. Patent No. 8,026,284 (expires 2027), which applications are pending in European and other countries, represents a MOU patent, which covers method of administering cysteamine composition that increases delivery to small intestine, at dosing schedule less than four times daily, including two times daily and contains pharmacokinetic claims. European Appl. 07762690.1 (expires 2027) represents a COU patent and has allowed claims to composition comprising enteric cysteamine/cystamine for treating nephropathic cystinosis two times a day.

Our cysteamine intellectual property to treat metabolic and neurodegenerative conditions. In addition, our UCSD license includes U.S. Patent No. 7,994,226 (expires 2028), an MOU patent which covers cysteamine and related compounds for the potential treatment of NASH. Our exclusive worldwide license from the Weizmann Institute includes U.S. Patent Nos. 6,794,414 and 6,355,690, an MOU patent which covers the use of transglutaminase inhibitors (a class of molecules which includes cysteamine) to treat HD and other neurodegenerative diseases mediated by transglutaminase, or other diseases associated with CAG repeat expansion.

Recent Developments

On April 16, 2012, we announced that one of our wholly-owned subsidiaries, Raptor Therapeutics Inc., or Raptor Therapeutics, entered into that certain Intellectual Property Platform Contribution Transaction License Agreement with RPTP European Holdings, C.V., or RPCV, which is 99% owned by Raptor Therapeutics and 1% owned by Raptor European Products, LLC, a wholly-owned subsidiary of Raptor Therapeutics. Pursuant to the agreement, RPCV was granted a perpetual, royalty-free, exclusive license, with the right to grant sublicenses, to the intellectual property rights relating to all proprietary products or services relating to the use of cysteamine, and any salts thereof, to treat nephropathic cystinosis and other indications, and other related sources of revenue (the Raptor Products and Services), to make, use and sell Raptor Products and Services, within all countries except the U.S. In addition, RPCV

was granted a perpetual, royalty-free, non-exclusive license, with the right to grant sublicenses, to make, or have made, improvements, modifications and/or enhancements to any and all inventions, methods, updates, adaptations, know-how, technical data, trade secrets, functional or detailed design specifications, designs and enhancements that relate to the Raptor Products and Services within all countries except the U.S. In consideration for the licenses granted to RPCV under the agreement, RPCV will make certain platform contribution transaction payments to Raptor Therapeutics up to a specified completion date in amounts to be agreed upon by the parties on a quarterly basis pending an independent analysis of the value of the relevant intellectual property rights.

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On April 30, 2012, we entered into an "At-the-Market", or ATM, Sales Agreement, with Cowen and Company, LLC, or Cowen, pursuant to which we may, at our discretion, sell our common stock with a sales value of up to an aggregate maximum of $40 million through ATM sales on The NASDAQ Global Market. Cowen acts as sole sales agent for any sales made under the ATM for a 3% commission on gross proceeds. Our common stock sold through the ATM is sold at prevailing market prices at the time of sale. Therefore, sales prices under the ATM may vary. Sales under the ATM are made pursuant to our prospectus supplement dated April 30, 2012, which supplements our prospectus dated February 3, 2012, filed as part of our shelf registration statement that was declared effective by the SEC on February 3, 2012. From inception through June 28, 2012, we have sold 99,500 shares under the ATM at a weighted-average selling price of $5.50 per share for net proceeds (after 3% commission to Cowen) of $530,601.

In May 2012, we acquired exclusive rights to intellectual property related to cysteamine and related compounds in the potential treatment of parasitic diseases, including malaria, from McGill University, or McGill, in Montreal, Canada. The McGill patent covers the use of cysteamine and related compounds in the potential treatment of malaria in combination with artemisinin, the current standard of care. Researchers at McGill reported that, in mouse models of malaria, the combination reduced parasite levels in red blood cells and improved survival rates compared to artemisinin alone.

In June 2012, we acquired exclusive rights to cysteamine and related compounds for the potential treatment of Parkinson's Disease from Université Laval, or Laval, Quebec, Canada. Our agreement with Laval provides exclusive rights to technology related to the use of cysteamine and related compounds to potentially modify the progression of Parkinson's Disease. Researchers at Laval reported that administration of cystamine (an oxidized form of cysteamine) in an animal model of Parkinson's Disease showed signs of preventing neuron loss and rescuing neurons undergoing a degenerative process. Signs of restoration of neuronal loss and partial reversal of behavioral impairments were also observed.

Application of Critical Accounting Policies

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

Use of Estimates

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

Functional Currency

Our consolidated functional currency is the U.S. dollar. Raptor Pharmaceuticals Europe B.V., or BV, our European subsidiary, uses the European Euro as its functional currency. At quarter end, BV's balance sheet is translated into U.S. dollars based upon the quarter end exchange rate, while its statement of operations is translated into U.S. dollars based upon an average of the Euro's value between the beginning and end date of the reporting period. BV's equity is adjusted for any translation gain or loss.

Fair Value of Financial Instruments

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

Cash and Cash Equivalents

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

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Short-term Investments

We invest in short-term investments in high credit-quality funds in order to obtain higher yields on our idle cash. Such investments are not insured by the Federal Deposit Insurance Corporation. We completed an evaluation of our investments and determined that we did not have any other-than-temporary impairments as of May 31, 2012. The investments are placed in financial institutions with strong credit ratings and management expects full recovery of the carrying amounts.

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.

Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine (currently developed as RP103 and RP104), the rights to tezampanel and rights to an out-license acquired in the 2009 Merger. The intangible assets related to RP103/RP104 are being 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 will be amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents. The intangible assets related to tezampanel, which has been classified as in-process research and development, will not be amortized until development is completed, but will be tested annually for impairment.

Goodwill

Goodwill represents the excess of the value of the purchase consideration over the identifiable assets acquired in the 2009 Merger. Goodwill is reviewed annually, or when an indication of impairment exists, to determine if any impairment analysis and resulting write-down in valuation is necessary.

Fixed Assets

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

Impairment of Long-Lived Assets

We evaluate our long-lived assets for indicators of possible impairment by comparison of the carrying amounts to future net undiscounted cash flows expected to be generated by such assets when events or changes in circumstances indicate the carrying amount of an asset may not be recoverable. Should an impairment exist, the impairment loss would be measured based on the excess carrying value of the asset over the asset's fair value or discounted estimates of future cash flows. During the nine months ended May 31, 2012, we have not identified any such impairment losses.

Common Stock Warrant Liabilities

The warrants issued by us in the 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, a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, we have classified the warrants as liabilities and will mark them to fair value at each period end. The common stock warrants are re-measured at the end of every reporting period with the change in value reported in our condensed consolidated statements of comprehensive loss. Warrants which are recorded as liabilities that are exercised are re-measured and marked to market the day prior to exercise. Upon exercise of such warrants, the fair value of such warrants is reclassified to equity.

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.

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Our effective tax rate is 0% for income tax for the nine months ended May 31, 2012 and we expect that our effective tax rate for the full year 2012 will be 0%. Based on the weight of available evidence, including cumulative losses since inception and expected future losses, we have determined that it is more likely than not that the deferred tax asset amount will not be realized and therefore a valuation allowance has been provided on net deferred tax assets. Utilization of our net operating loss may be subject to substantial annual limitation due to the ownership change limitations provided by the Internal Revenue Code and similar state provisions. Such an annual limitation could result in the expiration of the net operating loss before utilization.

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

Our continuing practice is to recognize interest and/or penalties related to income tax matters as a component of income tax expense. As of May 31, 2012, there was no accrued interest and/or penalties related to uncertain tax positions.

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

As disclosed in our Form 8-K dated April 19, 2012, we entered into a Platform Contribution Transaction Agreement, or the Agreement, with our wholly-owned indirect subsidiary, RPTP European Holdings C.V., relating to certain intellectual property of ours. To date, we have not recorded any transactions related to such Agreement.

Research and Development

We are a development stage biotechnology company. Research and development costs are charged to expense as incurred. Research and development expenses include medical, clinical, regulatory and scientists' salaries and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, commercial manufacturing costs prior to drug 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 executive, human resources and facilities expenses. Research and development expenses are offset by contra-expenses, which are reimbursements of research and development expenses received either from research collaborators or from government grants or tax rebates.

In-Process Research and Development

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

Comprehensive Loss

Components of comprehensive loss are reported in our condensed consolidated statements of comprehensive loss in the period in which they are recognized. The components of comprehensive loss include net loss and foreign currency translation adjustments.

Stock-Based Compensation

In February 2010, our Board of Directors approved, and in March 2010 our stockholders approved, our 2010 Equity Incentive Plan, or the 2010 Plan, to grant up to an aggregate of 3,000,000 stock options or restricted stock or restricted stock units over the ten year life of the 2010 Plan. Our Board of Directors has determined not to make any new grants under any of our former plans, but rather under the 2010 Plan. The 2010 Plan allows for the granting of options to employees, directors and consultants. On April 7, 2011, our stockholders passed amendments to the 2010 Plan which allow for an increase of the grant pool based upon 5% of our common stock outstanding as of April 7, 2011, August 31, 2011 and August 31, 2012 up to an aggregate maximum increase of 6,000,000 shares. The April 7, 2011 and August 31, 2011 increases added 1,629,516 and 1,778,459 shares, respectively, available for grant under the

2010 Plan. As of May 31, 2012, options to purchase 6,095,435 shares of our common stock were outstanding and 1,230,993 shares of our common stock remain available for future issuance under the 2010 Plan. The amendments also allow for 50% accelerated vesting of unvested stock options upon a change of control as defined in the 2010 Plan, as amended. In September 2011, our Board of Directors approved an amended and restated form of award agreement to the 2010 Plan, which will be used for awards granted on or after September 22, 2011. The amended and restated award agreement, subject to the terms of any applicable employment agreement, extends the termination date of the awards granted under the 2010 Plan that are vested as of such termination date due to (a) an employee's or a non-employee director's retirement at age 62 or older which employee or non-employee director has at least five (5) years of continuous service with us prior to such retirement, (b) the termination of a non-employee director's board membership for reasons other than for cause or retirement and (c) an employee's or a non-employee director's death (during his or her continuous service with us or within 90 days' of such continuous service with us) or permanent disability, to eighteen (18) months from the date of termination of continuous service with us.

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

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

In March 2005, the FASB issued ASC Topic 718 (previously listed as Staff Accounting Bulletin No. 107), which offers guidance for what was previously referred to as SFAS 123(R). ASC Topic 718 was issued to assist preparers by simplifying some of the implementation challenges of SFAS 123(R) while enhancing the information that investors receive. ASC Topic 718 creates a framework that is premised on two overarching themes: (a) considerable judgment will be required by preparers to successfully implement SFAS 123(R), specifically when valuing employee stock options; and (b) reasonable individuals, acting in good faith, may conclude differently on the fair value of employee stock options. Key topics covered by ASC Topic 718 include valuation models, expected volatility and expected term. For the three month period ended May 31, 2012, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 0.89%; 5 year expected life; 123.52% 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 the actual volatility of our common stock price as quoted on NASDAQ since the closing of our 2009 Merger on September 30, 2009; the turnover rate was based on our assessment of our historical employee turnover; and the dividend rate was based on our current decision to not pay dividends on our stock 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.

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Results of Operations
Three months ended May 31, 2012 and 2011
General and Administrative Expenses
General and administrative expenses include finance and executive compensation and benefits for personnel performing pre-commercial and administrative functions, pre-commercial expenses, such as reimbursement and marketing studies, corporate costs, such as legal, tax and auditing fees, business development expenses, travel, board of director fees and expenses, investor relations expenses, intellectual property costs associated with filed (but not issued) patents, administrative consulting and allocated human resources and facilities costs. General and administrative expenses for the three-month period ended May 31, 2012 increased by approximately $2.4 million compared to the prior year's third fiscal quarter. The increase was primarily due to:

Reason for increase	Increase in $ thousands
Expenses not in Q3 FY2011:	
Commercial operations requirements RP103 for cystinosis:	
Pre-commercial consulting services	498
Tax study and advisory fees related to EU headquarters	426
Q3 accrual for annual performance bonus based on assessment of performance to date	127
Salary and benefit increases and new finance and commercial operations personnel	378
Stock-based compensation expense, employees and directors (non-cash)	669
Board expansion from 5 to 8 members, retainer fees and expenses	70
Increased legal fees due to in-licensing negotiations	194
Other, net	9
Total increase Q3 FY2012 versus Q3 FY2011	2,371

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Research and Development

Research and development expenses include medical, clinical, regulatory and scientists' compensation and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, commercial drug manufacturing costs prior to marketing approval, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated executive, human resources and facilities expenses. Research and development expenses for the three month period ended May 31, 2012 increased by approximately $2.1 million over the prior year's third fiscal quarter primarily due to:

Reason for increase (decrease)	Increase (decrease) in $ thousands
R&D compensation	
Salary increases and new hire compensation	102
Stock-based compensation expense, employees (non-cash)	167
Q3 accrual for annual performance bonus based on assessment of performance to-date	102
Regulatory consulting for NDA/MAA preparation	102
Reduction in Phase 3 cystinosis trial expense partially offset by extension study	(197)
Increased product manufacture of RP103 and RP104 for cystinosis, HD, NASH	1,331
Lower reimbursement from collaboration partner for clinical materials for Convivia	116
Milestone payment for filing the MAA with the EMA on RP103 for cystinosis	250
Preclinical studies	163
Other, net	(18)
Total increase Q3 FY2012 versus Q3 FY2011	2,118

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

	Three month periods ended	
Major Program (stage of development)	May 31, 2012	May 31, 2011
RP103/RP104 - All indications (clinical/pre-commercial)	4.4	3.0
Preclinical programs	0.4	-
R&D personnel and other costs not allocated to programs	1.2	0.9
Total research and development expenses	6.0	3.9

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

Major Program (stage of development)	Three month periods ended May 31, 2012	May 31, 2011
RP103/RP104 - All indications (clinical and pre-commercial)	0.8	0.4
Preclinical programs	0.1	0.0

Additional major program expenses include patent fees and patent expenses which were recorded as general and administrative expenses as these fees are to support patent applications (not issued patents) and expenses related to the preparation for commercial launch of RP103 for the treatment of cystinosis (approximately $217,000, $605,000 and $1,075,000 for the three and nine month periods ended May 31, 2012 and the cumulative period from September 8, 2006 (inception) to May 31, 2012, respectively).

Any of our major programs could be partnered for further development and/or could be accelerated, slowed or ceased due to scientific results or challenges in obtaining funding. We anticipate that we will need additional funding in order to pursue our plans beyond the first calendar quarter of 2013. In addition, the timing and costs of development of our programs beyond the next 12 months are highly uncertain and difficult to estimate. See risks and other factors described under the section captioned "Risk Factors That May Affect Future Results" in Part II, Item 1A of this Quarterly Report on Form 10-Q.

Current Status of Major Programs

Please refer to the subsection titled "Overview" under this "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this Quarterly Report on Form 10-Q for a detailed discussion of each of our major programs. In summary, RP103/RP104 is being developed in cystinosis, NASH and HD. In July 2011, we announced that our Phase 3 clinical trial of RP103 for the treatment of cystinosis met its sole primary clinical endpoint and in November 2009, we released data from our Phase 2b clinical trial. In March 2012, we filed for marketing approval of RP103 for cystinosis in both the U.S. and in the E.U. and anticipate regulatory decisions in the first half of calendar 2013 and have begun development of commercial infrastructure in anticipation of drug launch. In May 2010, we presented the data from our NASH Phase 2a clinical trial and have signed a collaborative agreement with the NIH for a Phase 2b clinical trial, which commenced in June 2012 with potential data release in the first half of calendar 2014. We continue to work on the formulation of RP104 as a delayed-released compressed tablet of cysteamine bitartrate for future NASH studies. In October 2010, our collaborator commenced a Phase 2/3 clinical trial of RP103 in HD patients. The Phase 2/3 HD clinical trial was fully enrolled in June 2012 with potential data release in the first half of calendar 2014.

Our Convivia™ product candidate for ALDH2 for alcohol intolerance completed its initial clinical study in 2008 and in June 2010, we licensed Convivia™ to Uni Pharma for further clinical and commercial development in Taiwan. We continue to seek other potential partners for Convivia™ in other East Asian countries where the largest potential Convivia™ market exists.

We are preparing for a Phase 1 clinical trial for the potential treatment of thrombotic disorder but plan to eventually out-license our tezampanel product candidate. HepTide™ will be undergoing further preclinical proof of concept studies and WntTide™ and NeuroTrans™ are being considered for potential out-licensing for further development. All preclinical product candidates will require further study prior to moving into a clinical phase of development.

Interest Income

Interest income for the three-month periods ended May 31, 2012 and 2011 was nominal.

Interest Expense

Interest expense for the three-month periods ended May 31, 2012 and 2011 was nominal.

Foreign Currency Transaction Gain (Loss)

Foreign currency transaction gain and loss and for the three-month periods ended May 31, 2012 and 2011 was nominal.

Unrealized Gain on Short-Term Investments

Unrealized gain on short-term investments represents the change in net asset value of the Company's two short-term bond funds. The unrealized gain on short-term investments for the three-month periods ended May 31, 2012 and 2011 was nominal.

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Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a gain of approximately $6.9 million for the three month period ended May 31, 2012 compared to a loss of approximately $(14.6) million for the three month period ended May 31, 2011, representing an increase of approximately $21.5 million resulting from higher stock prices of our common stock, as well as lower number of warrants outstanding during the three months ended May 31, 2012 compared to the three months ended May 31, 2011. These gains/losses are non-cash.

Nine months ended May 31, 2012 and 2011

General and Administrative Expenses

General and administrative expenses include finance and executive compensation and benefits for personnel performing pre-commercial and administrative functions, pre-commercial expenses, such as reimbursement and marketing studies, corporate costs, such as legal, tax and auditing fees, business development expenses, travel, Board of Director fees and expenses, investor relations expenses, intellectual property costs associated with filed (but not issued) patents, administrative consulting and allocated human resources and facilities costs. General and administrative expenses for the nine month period ended May 31, 2012 increased by approximately $4.3 million compared to the same period of the prior year. The increase was primarily due to:

Reason for increase (decrease)	Increase (decrease) in $ thousands
Expenses not in Q3-YTD FY2011:	
Commercial operations requirements RP103 for cystinosis:	
Pre-commercial consulting services	1,013
Tax study and advisory fees related to EU headquarters	564
Q3 YTD accrual for annual performance bonus based on assessment of performance to date	257
Audit fee increase primarily due to internal control attestation in FY2011 audit	103
Salary and benefit increases and new finance and commercial operations personnel	695
Stock-based compensation expense, employees and directors (non-cash)	1,371
Board expansion from 5 to 8 members, retainer fees and expenses	226
Legal fees due to in-licensing of intellectual property	196
Increased executive and human resource costs allocated to R&D due to higher headcount	(302)
Other, net	204
Total increase Q3-YTD FY2012 versus Q3-YTD FY2011	4,327

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Research and Development

Research and development expenses include medical, clinical, regulatory and scientists' compensation and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, commercial drug manufacturing costs prior to marketing approval, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated executive, human resources and facilities expenses. Research and development expenses for the nine month period ended May 31, 2012 increased by approximately $4.7 million over the same period of the prior year primarily due to:

Reason for increase (decrease)	Increase (decrease) in $ thousands
Increased product manufacture of RP103 and RP104 for cystinosis, HD, NASH	2,802
Tax grants for preclinical and clinical programs not available	820
Increased executive and human resource costs allocated to R&D due to higher headcount R&D compensation	302
Salary increases and new hire compensation	290
Stock-based compensation expense, employees (non-cash)	307
Q3-YTD accrual for annual performance bonus based on assessment of performance to-date	201
Preclinical studies	358
Reduction in Phase 3 cystinosis trial expense partially offset by extension study	(861)
Net increase in milestone expenses paid, primarily for the RP103 MAA and NASH IND filings	280
Regulatory consulting for NDA/MAA preparation	126
Other, net	116
Total increase Q3-YTD FY2012 versus Q3-YTD FY2011	4,741

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

		Nine month periods ended	
Major Program (stage of development)	Cumulative through May 31, 2012	May 31, 2012	May 31, 2011
RP103/RP104 - All indications (clinical/pre-commercial)	32.7	10.8	7.6
Convivia™ (clinical)	2.5	-	0.1
Preclinical programs	2.8	0.5	0.1
Minor or inactive programs	1.1	-	-
R&D personnel and other costs not allocated to programs	15.1	3.7	2.5
Total research and development expenses	54.2	15.0	10.3

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

		Nine month periods ended
Major Program (stage of development)	Cumulative through	

	May 31, 2012	May 31, 2012	May 31, 2011
RP103/RP104 - All indications (clinical and pre-commercial)	2.5	1.4	0.6
Convivia™ (clinical)	0.3	0.1	0.1
Preclinical programs	0.8	0.1	0.1

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

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Any of our major programs could be partnered for further development and/or could be accelerated, slowed or ceased due to scientific results or challenges in obtaining funding. We anticipate that we will need additional funding in order to pursue our plans beyond the first calendar quarter of 2013. In addition, the timing and costs of development of our programs beyond the next 12 months are uncertain and difficult to estimate. See risks and other factors described under the section captioned "Risk Factors That May Affect Future Results" in Part II, Item 1A of this Quarterly Report on Form 10-Q.

Interest Income

Interest income increased by approximately $231,000 for the nine months ended May 31, 2012 compared to the same period of the prior fiscal year due to the purchase of short-term investments in October 2011.

Interest Expense

Interest expense for the nine months ended May 31, 2012 and 2011 was nominal.

Foreign Currency Transaction Gain

Foreign currency transaction gain for the nine months ended May 31, 2012 and 2011 was approximately a $122,000 gain and a nominal gain, respectively.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of approximately $(5.0) million for the nine months ended May 31, 2012 compared to a loss of approximately $(18.6) million for the nine months ended May 31, 2011, a decrease in loss of approximately $13.6 million resulting from the lower number of warrants outstanding, as well as a decrease in the price of our common stock during the nine months ended May 31, 2012 compared to the nine months ended May 31, 2011. These losses are non-cash.

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Liquidity and Capital Resources

Capital Resource Requirements

As of May 31, 2012, we had approximately $43.1 million in cash, cash equivalents and short-term investments, approximately $24.1 million in current liabilities (of which $19.1 million represented the non-cash common stock warrant liability) and approximately $21.7 million of net working capital.

We believe our cash, cash equivalents and short-term investments as of May 31, 2012 will be sufficient to meet our obligations through the first calendar quarter of 2013.

Our recurring losses from operations raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the year ended August 31, 2011 with respect to this uncertainty. We may need to generate significant revenue or raise additional capital to continue to operate as a going concern beyond the first calendar quarter of 2013. In addition, the perception that we may not be able to continue as a going concern may cause others to choose not to deal with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

The sale of additional securities is likely to result in additional dilution to our stockholders. Additional financing may not be available when needed in amounts or on terms satisfactory to us or at all. We may be unable to raise additional financing due to a variety of factors, including our financial condition, the status of our research and development programs, 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, our financial condition and operating results may be adversely affected and we may have to scale back our operations.

In December 2009, we entered into a definitive securities purchase agreement, or the Direct Offering Purchase Agreement, dated as of December 17, 2009, with 33 investors (collectively, the Direct Offering Investors) with respect to the sale of units, whereby, on an aggregate basis, the investors agreed to purchase 3,747,558 Units for a negotiated purchase price of $2.00 per unit for aggregate gross proceeds of approximately $7.5 million. Each unit consists of one share of our common stock, one Series A Warrant exercisable for 0.5 of a share of our common stock and one Series B Warrant exercisable for 0.5 of a share of our common stock. The shares of our common stock and the warrants were issued separately. The Series A Warrants exercisable for an aggregate 1,873,779 shares of our common stock were exercisable commencing on June 20, 2010 and ending December 22, 2014. The Series B Warrants exercisable for an aggregate 1,873,779 shares of our common stock were exercisable commencing on June 20, 2010 and ended June 22, 2011. The investor warrants have a per share exercise price of $2.45. In connection with this offering we paid a placement agent cash compensation equal to 6.5% of the gross proceeds or $487,183 plus a five-year warrant at an exercise price of $2.50 per share for the purchase of up to 74,951 shares of our common stock. As of May 31, 2012, 1,117,529 shares of our common stock have been issued upon exercise of the Series A Warrants, 1,873,779 shares of our common stock have been issued upon exercise of the Series B Warrants and 74,951 shares of our common stock have been issued upon exercise of the placement agent warrants. As of May 31, 2012, Series A warrants to purchase up to 756,250 shares of our common stock were outstanding.

On August 9, 2010, we entered into the 2010 Private Placement Purchase Agreements with the 2010 Private Placement Investors for the private placement of our common stock and warrants to purchase our common stock, at a purchase price of $3.075 per unit, with each unit comprised of one share of common stock and a warrant to purchase one share of common stock. We issued and sold an aggregate of 4,897,614 units, comprised of an aggregate of 4,897,614 shares of common stock and warrants to purchase up to 4,897,614 shares of our common stock for gross proceeds of approximately $15.1 million. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share. The placement agent for the 2010 Private Placement was issued one warrant to purchase 97,952 shares of our common stock at an exercise price of $3.075 per share, paid a cash commission of $978,911 and reimbursed for certain of its expenses incurred in connection with the 2010 Private Placement. As of May 31, 2012, 1,303,142 shares of our common stock have been issued upon exercise of the warrants. As of May 31, 2012, warrants to purchase up to 3,692,424 shares (including the placement agent warrants) of our common stock were outstanding.

On September 13, 2011, we closed an underwritten public offering of shares of our common stock at a price to the public of $4.00 per share. The shares sold in the offering included 10.0 million shares of our common stock plus an

additional 1.5 million shares of common stock pursuant to the exercise by the underwriters of the over-allotment option we granted to them. Total gross proceeds to us in the offering (including in connection with the sale of the shares of common stock pursuant to the exercise of the over-allotment option) were $46.0 million, before underwriting discounts and commissions. The offering resulted in net proceeds to us of approximately $42.8 million after deduction of underwriting discounts of 6% and other offering expenses paid by us.

On April 30, 2012, we entered into a Sales Agreement with Cowen and Company, LLC, or Cowen, to sell shares of our common stock, with aggregate gross sales proceeds of up to $40,000,000, from time to time, through an "at the market" equity offering program under which Cowen will act as sales agent. We pay a 3% commission to Cowen on any sales pursuant to this Sales Agreement. As of June 28, 2012, we sold an aggregate of 99,500 shares at a weighted-average exercise price of $5.50 per share for net proceeds of $530,601.

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There can be no assurance that we will be able to obtain funds required for our continued operation. There can be no assurance that additional financing will be available to us or, if available, that it can be obtained on commercially reasonable terms. If we are not able to obtain financing on a timely basis, we will not be able to meet our obligations as they become due and we will be forced to scale down or perhaps even cease the operation of our business. This also may be the case if we become insolvent or if we breach our licensing agreements with UCSD, Washington University or Yeda, or due to non-payment (and we do not cure our non-payment within the stated cure period). If this happens, we would lose all the rights to RP103 and RP104 licensed to us by UCSD, all rights to Mesd licensed to us by Washington University and the rights licensed to us by Yeda, depending on which agreement is breached.

We anticipate that we will not be able to generate revenues from the sale of products until we obtain regulatory approval for our lead drug product candidate, further develop our other drug product candidates and obtain the necessary regulatory approvals to market our future drug product candidates, which could take seven months or more for our lead product candidate and several years or more for our other product candidates, if we are able to do so at all. Accordingly, our cash flow projections are subject to numerous contingencies and risk factors beyond our control, including successfully developing our drug product candidates, market acceptance of our drug product candidates including critical pricing, competition from well-funded competitors, and our ability to manage our expected growth. It is likely that for a couple of years, we will not be able to generate internal positive cash flow from the sales of our drug product candidates sufficient to meet our operating cash flow and capital expenditure requirements.

There is substantial doubt about our ability to continue as a going concern as the continuation of our business is dependent upon obtaining further long-term financing, the obtaining of regulatory approvals for our product candidates, the successful development of our drug product candidates and related technologies, the successful and sufficient market acceptance of any product offerings that we may introduce and, finally, the achievement of a profitable level of operations. The issuance of additional equity securities by us is likely to result in a significant dilution in the equity interests of our current stockholders. Obtaining commercial loans, assuming those loans would be available, including on acceptable terms, will increase our liabilities and future cash commitments.

Research and Development Activities

We plan to conduct further research and development, seek to support several clinical trials for RP103, improve upon our internal discovery molecules and in-licensed technology and continue business development efforts for our preclinical and other clinical-stage product candidates in the next 12 months. We plan to conduct research and development activities by our own laboratory staff and also by engaging contract research organizations, clinical research organizations and contract manufacturing organizations. We also plan to incur costs for the production of our clinical study drug candidates, RP103 and RP104, for commercial production of RP103 for cystinosis until marketing approval, clinical trials, clinical and medical advisors and consulting and collaboration fees. We anticipate that our research and development costs will increase during the next 12 months primarily due to the build-up of inventory of RP103 for cystinosis prior to marketing approval in anticipation of drug launch and the addition of our Phase 2 clinical trial in NASH.

General and Administrative Activities

General and administrative costs in the next 12 months will consist primarily of commercial and pre-commercial activities in anticipation of approval and launch of RP103 for cystinosis, legal, tax and accounting fees, patent legal fees, investor relations expenses, board fees and expenses, insurance, rent and facility support expenses. We anticipate that general and administrative expenses will increase primarily due to the commercial and pre-commercial efforts required to prepare for the commercial launch of RP103 in cystinosis in both the U.S. and the E.U.

Officer and Employee Compensation

We presently have 30 full time employees and one part-time employee. Of the 30 full-time employees, 16 are in general and administrative (including 5 U.S.- and 2 E.U.-based commercial operations employees) and 14 are in research and development functions. Based on our current plan, over the next 12 month period, we plan to add personnel in the areas of sales and marketing, regulatory, clinical, medical affairs and quality. We also plan to supplement our human resources needs through consultants and contractors as needed. We anticipate that our compensation expense will increase significantly during the next 12 months due to the addition of employees primarily in support of commercial operations in anticipation of launching RP103 for cystinosis in both the U.S. and

the E.U. Officer and employee compensation is recorded on our condensed consolidated statements of comprehensive loss as either research and development expenses or general and administrative expenses based upon the functions served by the officers and employees.

Capital Expenditures

In the next 12 months, relatively minor capital expenditures will be made for lab equipment and office furniture.

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Contractual Obligations with Thomas E. Daley (assignee of the dissolved Convivia, Inc.)

Pursuant to the terms of the asset purchase agreement, or the Asset Purchase Agreement, that we entered into with Convivia, Inc. and Thomas E. Daley, pursuant to which we purchased intellectual property related to our 4-MP product candidate program known as Convivia™, Mr. Daley will be entitled to receive various payments in the form of our restricted common stock and cash, if at all, in such amounts and only to the extent certain future milestones are accomplished by us. See Note 10 Commitments and Contingencies for further details in our condensed consolidated financial statements located elsewhere in this Quarterly Report on Form 10-Q.

Contractual Obligations with Former Encode Security Holders

Pursuant to the terms of the merger agreement, or the Encode Merger Agreement, that we entered into with Encode Pharmaceuticals, Inc. and Nicholas Stergis in December 2007, former Encode Security Holders will be entitled to receive the following, if at all, in such amounts and only to the extent certain future milestones are accomplished by us, as set forth below:

- Restricted, unregistered common stock, stock options to purchase our common stock, and warrants to purchase our common stock in an amount equal to, in the aggregate, 116,562 shares of our common stock upon the receipt by it at any time prior to the fifth-year anniversary of the Encode Merger Agreement of approval to market and sell a product for the treatment of cystinosis predominantly based upon and derived from the assets acquired from Encode, or Cystinosis Product, from the applicable regulatory agency (e.g., FDA and European Agency for the Evaluation of European Medical Products, or EMA) in a given major market in the world.

- Restricted, unregistered common stock, stock options to purchase our common stock, and warrants to purchase our common stock in an amount equal to 442,934 shares of our common stock upon the receipt by us at any time prior to the fifth anniversary of the Encode Merger Agreement of approval to market and sell a product, other than a Cystinosis Product, predominantly based upon and derived from the assets acquired from Encode, from the applicable regulatory agency (e.g., FDA and EMA) in a given major market in the world.

If within five years from the date of the Encode Merger Agreement, there occurs a transaction or series of related transactions that results in the sale of all or substantially all of the assets acquired from Encode other than to our affiliate in such case where such assets are valued at no less than $2.5 million, the former Encode stockholders will be entitled to receive, in the aggregate, restricted, unregistered common stock, stock options to purchase our common stock, and warrants to purchase our common stock in an amount equal to 559,496 shares of common stock, less the aggregate of all milestone payments previously made or owing, if any.

To the extent that future milestones as described above are accomplished by us within five years from the effective time of the merger with Encode, we will be obligated to file a registration statement within 90 days covering such Encode stockholder's portion of such respective future restricted, unregistered common stock issued relating to such milestone payment.

Contractual Obligations with UCSD

As a result of the merger of Raptor Therapeutics Inc. and Encode, we received the exclusive worldwide license to RP103/RP104, or the License Agreement for use in the field of human therapeutics for metabolic and neurologic disorders, developed by clinical scientists at the UCSD, School of Medicine. RP103/RP104 is a proprietary, delayed-release, enteric-coated formulation of cysteamine bitartrate, a cystine depleting agent currently approved by the FDA. Cysteamine bitartrate is prescribed for the management of the genetic disorder known as cystinosis, a lysosomal storage disease. The active ingredient in RP103/RP104 has also demonstrated potential in studies as a treatment for other metabolic and neurodegenerative diseases, such as HD and NASH.

In consideration of the grant of the license, prior to the merger, Encode paid an initial license fee and we are obligated to pay an annual maintenance fee of $15,000 until we begin commercial sales of any products developed pursuant to the License Agreement. In addition to the maintenance fee, we are obligated to pay during the life of the License Agreement: milestone payments ranging from $20,000 to $750,000 for orphan indications and from $80,000 to $1,500,000 for non-orphan indications upon the occurrence of certain events, if ever; royalties on commercial net

sales from products developed pursuant to the License Agreement ranging from 1.75% to 5.5%; a percentage of sublicense fees ranging from 25% to 50%; a percentage of sublicense royalties; and a minimum annual royalty commencing the year we begin commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, we are obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 years from the effective date of the License Agreement, depending on the indication. In addition, we are obligated to, among other things, annually spend at least $200,000 for the development of products-which as of August 31, 2011, 2010 and 2009 we satisfied by spending approximately $11.3 million, $6.2 million and $4.1 million, respectively, on such programs -pursuant to the License Agreement. To date, we have paid $930,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, HD and in NASH and regulatory filings in cystinosis. In March 2012, we filed our MAA with the EMA as well as our NDA with the FDA for RP103 for the potential treatment of cystinosis, a milestone which we paid $250,000 to UCSD pursuant to this license. To the extent that we fail to perform any of our obligations under the License Agreement, UCSD may terminate the license or otherwise cause the license to become non-exclusive. Future milestones of $500,000 and $750,000 will be payable if the MAA and NDA for cystinosis are approved, respectively, which we anticipate may occur in the first half of calendar 2013.

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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.

Reverse Acquisition

We have treated the 2009 Merger as a reverse acquisition and the reverse acquisition is accounted for as a recapitalization.

For accounting purposes, Raptor Pharmaceuticals Corp. is considered the accounting acquirer in the reverse acquisition. The historical financial statements reported in this Quarterly Report on Form 10-Q and in future periods are and will be those of Raptor Pharmaceuticals Corp. (merged into Raptor Pharmaceutical Corp. effective December 7, 2011) consolidated with its subsidiaries and with us, its parent, Raptor Pharmaceutical Corp. (formerly TorreyPines Therapeutics, Inc.). Earnings per share for periods prior to the reverse merger have been restated to reflect the number of equivalent shares received by former stockholders.

Going Concern

Due to the uncertainty of our ability to meet our current operating and capital expenses, in their reports on our audited financial statements for the years ended August 31, 2011, 2010, 2009, 2008, 2007 and for the period September 8, 2005 (inception) to August 31, 2006, our independent registered public accounting firm, Burr Pilger Mayer, Inc., included an explanatory paragraph regarding substantial doubt about our ability to continue as a going concern. Our financial statements contain additional note disclosures describing the circumstances that led to this disclosure by our independent registered public accounting firm.

New Accounting Pronouncements

In December 2010, the FASB issued Accounting Standards Update ("ASU") 2010-28 , Intangibles - Goodwill and Other (Topic 350): When to Perform Step 2 of the Goodwill Impairment Test for Reporting Units with Zero or Negative Carrying Amounts ("ASU 2010-28"). ASU 2010-28 modifies Step 1 of the goodwill impairment test for reporting units with zero or negative carrying amounts and requires the company to perform Step 2 if it is more likely than not that a goodwill impairment may exist. ASU 2010-28 is effective for fiscal years and interim periods within those years, beginning after December 15, 2010. Early adoption is not permitted. We adopted these standards on September 1, 2011 and have determined that ASU 2010-28 has no material impact on our condensed consolidated financial statements for the three and nine month periods ended May 31, 2012, because there was no requirement to perform Step 2 due to our positive carrying amount.

In December 2010, the FASB issued ASU 2010-29, Business Combinations (Topic 805): Disclosure of Supplementary Pro Forma Information for Business Combinations ("ASU 2010-29"). ASU 2010-29 is an update that addresses diversity in practice about the interpretation of the pro forma revenue and earnings disclosure requirements for business combinations if the entity presents comparative financial statements and expands the required disclosures to include a description of the nature and amount of material, nonrecurring pro forma adjustments directly attributable to the business combination included in the reported pro forma revenue and earnings. This standard is effective prospectively for business combinations for which the acquisition dates are on or after the beginning of the first annual reporting period beginning on or after December 15, 2010. Early adoption is permitted. We adopted these standards on September 1, 2011, however since there were no business combinations during the three and nine month periods ended May 31, 2012, ASU 2010-29 had no material impact on our financial disclosure, however, the provision will impact the financial disclosures of any business combinations in the future.

In May 2011, the FASB issued ASU 2011-04, Fair Value Measurement (Topic 820): Amendments to Achieve Common Fair Value Measurement and Disclosure Requirements in U.S. GAAP and IFRSs ("ASU 2011-04"). ASU 2011-04 is intended to result in convergence between U.S. GAAP and International Financial Reporting Standards ("IFRS") requirements for measurement of and disclosures about fair value. The amendments are not expected to have a significant impact on companies applying U.S. GAAP. Key provisions of the amendment include: a prohibition on grouping financial instruments for purposes of determining fair value, except when an entity manages market and credit risks on the basis of the entity's net exposure to the group; an extension of the prohibition against the use of a blockage factor to all fair value measurements (that prohibition currently applies only to financial instruments with quoted prices in active markets); and a requirement that for recurring Level 3 fair value measurements, entities

disclose quantitative information about unobservable inputs, a description of the valuation process used and qualitative details about the sensitivity of the measurements. In addition, for items not carried at fair value but for which fair value is disclosed, entities will be required to disclose the level within the fair value hierarchy that applies to the fair value measurement disclosed. ASU 2011-04 is effective for interim and annual periods beginning after December 15, 2011. We adopted these standards on March 1, 2012 and have determined that ASU 2011-04 did not have a material impact on our condensed consolidated financial statements.

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In June 2011, the FASB issued ASU 2011-05, Comprehensive Income (Topic 220): Presentation of Comprehensive Income ("ASU 2011-05"). ASU 2011-05 will require companies to present the components of net income and other comprehensive income either as one continuous statement or as two consecutive statements. It eliminates the option to present components of other comprehensive income as part of the statement of changes in stockholders' equity. The standard does not change the items which must be reported in other comprehensive income, how such items are measured or when they must be reclassified to net income. This standard is effective for interim and annual periods beginning after December 15, 2011. We early adopted these standards as of August 31, 2011. Because ASU 2011-05 impacts presentation only, it had no effect on our condensed consolidated financial statements or on our financial condition for the three and nine month periods ended May 31, 2012.

In September 2011, the FASB issued ASU 2011-08, Intangibles - Goodwill and Other (Topic 350): Testing Goodwill for Impairment ("ASU 2011-08"), which permits an entity to first assess qualitative factors to determine whether it is more likely than not that the fair value of a reporting unit is less than its carrying amount as a basis for determining whether it is necessary to perform the two-step goodwill impairment test. Because we have only one reporting unit, which has a fair value higher than our carrying amount, adoption of ASU 2011-08 did not have a material impact on our condensed consolidated financial statements for the three and nine month periods ended May 31, 2012.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Market risk represents the risk of changes in the value of market risk sensitive instruments caused by fluctuations in interest rates, foreign exchange rates and commodity prices. We are exposed to various market risks that may arise from adverse changes in market rates and prices, such as interest rates and foreign exchange fluctuations. We do not enter into derivatives or other financial instruments for trading or speculative purposes.

Foreign Exchange Risk

A majority of our assets and liabilities are maintained in the United States in U.S. dollars and a majority of our expenditures are transacted in U.S. dollars. We are subject to foreign exchange risk for the operations of BV which uses the European Euro as its functional currency. We do not believe that a hypothetical one percentage point fluctuation in the U.S. dollar exchange rate would materially affect our consolidated financial position, results from operations or cash flows as of May 31, 2012. We do not currently hedge our foreign currency transactions.

Interest Rate Risk

We are subject to interest rate risks associated with fluctuations in interest rates. In October 2011, we invested in two $15 million short-term bond funds with the goal of increasing yield on our idle cash. Approximately $19.2 million remained in the money market account yielding approximately .04% per year. The two short-term bond funds include one that exclusively invests in government securities and the other invests in a combination of government and other securities, both funds have historical annual yields of over 2%. Both bond funds pay dividends and provide their net asset value of their assets on a daily basis with daily liquidity. The change in net asset value is recorded on our statements of comprehensive loss as unrealized gain or loss on short-term investments. We completed an evaluation of our investments and determined that we did not have any other-than-temporary impairments as of May 31, 2012. The investments are placed in financial institutions with strong credit ratings and management expects full recovery of the carrying amounts. A hypothetical one percentage point decline in interest rates would not have materially affected our consolidated financial position, results of operations or cash flows as of May 31, 2012.

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Item 4. Controls and Procedures

As of May 31, 2012, we performed an evaluation of the effectiveness of our disclosure controls and procedures that are designed to ensure that the information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act, is accumulated and communicated to our management, including our principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Based on our evaluation, our management, including our Chief Executive Officer and Chief Financial Officer, has concluded that our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act) as of May 31, 2012, are effective at a reasonable assurance level.

Our management, under the supervision of our Chief Executive Officer and Chief Financial Officer, is responsible for establishing and maintaining adequate internal control over our financial reporting, as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act. Our internal control over financial reporting is defined as a process designed by, or under the supervision of, our Chief Executive Officer and Chief Financial Officer, and effected by our Board of Directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP. Internal control over financial reporting includes policies and procedures that: (i) pertain to the maintenance of records that in reasonable detail accurately and fairly reflect our transactions and asset dispositions; (ii) provide reasonable assurance that transactions are recorded as necessary to permit the preparation of our financial statements in accordance with GAAP, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and (iii) provide reasonable assurance regarding the prevention or timely detection of unauthorized acquisition, use or disposition of assets that could have a material effect on our financial statements.

Due to its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation under the framework in Internal Control-Integrated Framework, our management concluded that our internal control over financial reporting was effective as of May 31, 2012.

Changes in Internal Control over Financial Reporting

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

PART II - OTHER INFORMATION

Item 1. Legal Proceedings.

We know of no material, active or pending legal proceedings against us, or any of our property, and we are not involved as a plaintiff in any material proceedings or pending litigation. There are no proceedings in which any of our directors, officers or affiliates, or any registered or beneficial stockholders are an adverse party or have a material interest adverse to us.

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Item 1A. Risk Factors.

RISK FACTORS THAT MAY AFFECT FUTURE RESULTS

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

Certain Risks Related to Our Business

If we fail to obtain the capital necessary to fund our operations, our financial results, financial condition and our ability to continue as a going concern will be adversely affected and we will have to delay or terminate some or all of our product development programs.

Our condensed consolidated financial statements as of May 31, 2012 have been prepared assuming that we will continue as a going concern. As of May 31, 2012, we had an accumulated deficit of approximately $106.4 million. We expect to continue to incur losses for the foreseeable future and will have to raise substantial cash to fund our planned operations. Our recurring losses from operations to date raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the year ended August 31, 2011, with respect to this uncertainty. We will need to raise additional capital and/ or generate significant revenue at profitable levels to continue to operate as a going concern. In addition, the perception that we may not be able to continue as a going concern may cause others to choose not to deal with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

As of May 31, 2012, our cash, cash equivalents and short term investments were approximately $43.1 million. We believe our cash, cash equivalents and short-term investments as of May 31, 2012 will be sufficient to meet our obligations through the first calendar quarter of 2013. There can be no assurance that we will be successful in raising sufficient equity funds when needed. If we are unable to obtain such additional capital when needed, we will be forced to reduce our expenditures or seek other corporate solutions.

In addition, in the future, we may need to sell equity or debt securities to raise additional funds. The sale of additional equity securities will result in additional dilution to our stockholders. Additional financing may not be available in amounts or on terms satisfactory to us or at all. We may be unable to raise additional capital due to a variety of factors, including our financial condition, the status of our research and development programs, the status of regulatory reviews for marketing approvals, the status of our commercialization activities, the execution of our potential launch of RP103 for cystinosis and the general condition of the financial markets. If we fail to raise additional financing when needed, we may have to delay or terminate some or all of our research and development programs, scale back our operations and/or reduce our pre-launch/launch expenses for RP103, our financial condition and operating results will be adversely affected and our potential future value may be significantly diminished.

If we obtain additional capital, we expect to continue to spend substantial amounts of capital on our operations for the foreseeable future. The amount of additional capital we will need depends on many factors, including:

- the progress, timing and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems and to build, acquire, or contract for manufacturing capabilities;

- the time and cost necessary to build commercial infrastructure, launch product candidates into the marketplace and successfully commercialize our product candidates, if approved;
- the time and cost necessary to respond to technological and market developments; and
- any changes made to, or new developments in, our existing collaborative, licensing and other corporate relationships or any new collaborative, licensing and other commercial relationships that we may establish.

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Moreover, our fixed expenses such as rent, collaboration and license payments and other contractual commitments are substantial and will likely increase in the future. These fixed expenses are likely to increase because we expect to enter into:

- additional licenses and collaborative agreements;
- contracts for manufacturing, clinical and preclinical research, regulatory services, consulting, maintenance and administrative services; and
- additional contracts for expanded facilities.

We are a late development stage company that has not generated any product sales or other revenues to date and have a limited operating history. Our lead product candidate is under FDA and EMA review for potential marketing approval in 2013. One of our drug product candidates is in a Phase 2/3 trial. A second candidate is in a Phase 2b clinical trial. Other product candidates are in the concept stage and have not undergone significant testing in preclinical studies or any testing in clinical trials. Moreover, we cannot be certain that our research and development efforts will be successful or, if successful, that our drug product candidates will ever be approved for sale, or will generate commercially viable revenue levels. We have a limited relevant operating history upon which an evaluation of our performance and prospects can be made. As a company, we have not launched a product candidate. We are subject to all of the business risks associated with a new enterprise, including, but not limited to, risks of unforeseen capital requirements, failure of drug product candidates either in preclinical testing, clinical trials, regulatory reviews or commercialization, failure to establish business relationships and competitive disadvantages against larger and more established companies.

We may not be able to obtain regulatory approval for our drug product candidates, which would adversely affect our financial results and financial condition and we would have to delay or terminate some or all of our research product development programs.

We are not permitted to market RP103 or any of our other product candidates in the U.S. until we obtain regulatory approval from the FDA. 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. To market a new drug in Europe, we must submit to the applicable regulatory authority in the designed Reference Member State and obtain approval of, a Marketing Authorization Application, or MAA. 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 effectiveness of the applicable product candidate.

In March 2012, we submitted an NDA to the FDA and an MAA to the EMA seeking approval to market our investigational drug candidate, Cysteamine Bitartrate Delayed-release Capsules (RP103), for the potential treatment of nephropathic cystinosis. The FDA has assigned the user fee goal date of January 30, 2013 for the RP103 NDA. Our MAA for RP103 is under review by the EMA. We anticipate a decision from the EMA in the first half of calendar 2013. However, there is no assurance that we will obtain regulatory approval for RP103 for the potential treatment of nephropathic cystinosis in the U.S. or the E.U. Other than RP103 for the potential treatment of nephropathic cystinosis, none of our other drug product candidates have been submitted for marketing approval. Despite regulatory guidelines, we cannot reliably predict if or when any of the drug product candidates we intend to develop will be approved for marketing. If we fail to gain approval for our drug product candidates, our financial results and financial condition will be adversely affected, 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.

Even if we obtain FDA approval for our product candidates, we will be subject to ongoing FDA obligations and continued regulatory review, which may result in significant additional expense. Additionally, any of our product candidates, if approved by the FDA, could be subject to labeling and other restrictions and market withdrawal, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products, which may adversely affect the value of our Company and our operating results.

Any regulatory approvals that we obtain for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or 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 product candidate. In addition, if the FDA approves a product candidate, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage,

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, registration, as well as continued compliance with cGMPs or GCPs, and 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 and comparable international regulatory agencies can withdraw a product's approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues. 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 data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government 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, the value of our Company and our operating results will be adversely affected.

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

Sales of our 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, compliance with foreign regulatory requirements and approved pricing 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.

Even if we receive regulatory approval for RP103 for the potential treatment of nephropathic cystinosis, our ability to generate revenues from RP103 will be subject to attaining significant market acceptance among physicians, patients and healthcare payers.

RP103 for the potential treatment of nephropathic cystinosis, if approved, may not attain market acceptance among physicians, patients, healthcare payers or the medical community. We believe that the degree of market acceptance and our ability to generate revenues from RP103 if marketing approval is obtained will depend on a number of factors, including:

- timing of market introduction of our products as well as competitive drugs;
- efficacy and safety of our products;
- identification of patients and continued projected growth of the nephropathic cystinosis market;
- prevalence and severity of any side effects;
- acceptance by patients, primary care specialists and key specialists;
- potential or perceived advantages or disadvantages of our products over alternative treatments, including cost of treatment and relative convenience and ease of administration;
- strength of sales, marketing and distribution support;
- the price of our products, both in absolute terms and relative to alternative treatments;
- the effect of current and future healthcare laws;
- availability of coverage and adequate reimbursement and pricing from government and other third- party payers; and
- product labeling or product insert requirements of the FDA or other regulatory authorities.

If RP 103 for the potential treatment of nephropathic cystinosis does not receive significant market acceptance among physicians, patients, healthcare payers or the medical community, our ability to generate revenues from this drug product may be severely affected.

Our current business plan is highly dependent upon our ability to successfully execute on our sales and marketing strategy for the commercialization of RP103. If we are not able to successfully execute on our sales and marketing strategy, we may not be able to generate significant product revenues, and our stock price may decline.

Our strategy is to build a fully-integrated biopharmaceutical company focused on the development of RP103 and a robust pipeline. We may not be able to successfully market or commercialize RP103 for the potential treatment of nephropathic cystinosis. If we are unable to successfully implement our commercial plans and drive adoption of RP 103 by patients and physicians through our sales, marketing and commercialization efforts, we will not be able to generate sustainable revenues from product sales. In addition, we will lose revenue if our marketing activities are restricted, if coverage, pricing or reimbursement is limited, or if alternative treatments for nephropathic cystinosis gain commercial acceptance. The patient population with nephropathic cystinosis disease is not large. Therefore, opportunities for future sales growth will be limited and will depend on patient identification. Because our current

business plan is highly dependent on the commercialization of RP103 for the potential treatment of nephropathic cystinosis, negative trends in revenue from this product could have an adverse effect on our results of operations and cause the value of our common stock to decline.

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Because the target patient populations for some of our orphan drug products are small, we must achieve significant market share and obtain relatively high per-patient prices for our products to achieve meaningful profitability and to generate an appropriate return for the investment in the associated product development programs.

Our clinical development of RP103 targets diseases with small patient populations, including nephropathic cystinosis and HD. A key component of the successful commercialization of a drug product for these indications includes identification of patients for the drug product. If we are successful in obtaining regulatory approval to market RP103 for a disease with a small patient population and, in the case of HD, successful in developing this product candidate for that indication, we will need to identify patients and market RP103 for these indications in the U.S. and Europe, at a minimum, to achieve significant market penetration. In addition, the per-patient prices at which we sell 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 profitability. There can be no assurance that we will be successful in identifying patients or obtaining high per-patient prices for our product candidates that target diseases with small patient populations.

Pressure on drug product pricing and third-party coverage and reimbursement may impair our ability to raise capital, form collaborations and, if any of our product candidates become marketable, sell such products or to sell them on terms sufficient to provide a viable financial outcome.

The continuing efforts of governmental and third-party payers to contain or reduce the costs of healthcare through various means may harm our business. For example, in some foreign markets, the pricing or profitability of healthcare products is subject to government control. In the U.S., there have been, and we expect there will continue to be, a number of federal and state proposals to implement similar government control. The implementation or even the announcement of any of these legislative or regulatory proposals or reforms could harm our business, if any of our product candidates become marketable, by reducing the prices we are able to charge for our products, impeding our ability to achieve profitability, raise capital or form collaborations.

Market acceptance and sales of any of our product candidates that we may develop will depend in large part on global reimbursement policies and may be affected by future healthcare reform measures, both in the U.S. and other key international markets. Successful commercialization of our products will depend in part on the availability of governmental and third-party payer reimbursement for the therapeutic value and cost of our products. In particular, in the U.S., private health insurers and other third-party payers often follow the coverage and reimbursement policies of government payors, including the Medicare or Medicaid programs, and in many European countries, patients are unlikely to use prescription drugs that are not reimbursed by their governments. In the U.S., E.U. and other significant or potentially significant markets for our product candidates, government authorities and third-party payers 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. Further, the increased emphasis on managed healthcare in the U.S. and on country and regional pricing and reimbursement controls in the E.U. will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our product sales, and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, drug coverage and reimbursement policies and pricing in general. For our product candidates, we will not know what the reimbursement rates will be until we are ready to market the product and we actually negotiate the rates. If we are unable to obtain sufficiently high reimbursement rates for our products, they may not be commercially viable.

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. 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. Most recently, on August 2, 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.

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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 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; and
- 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.

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 our products after receiving regulatory approval and impact our financial results.

If we are not able to develop our drug product candidates, we would have to terminate or delay some of our research product development programs and our financial results and financial condition will be adversely affected.

There are many reasons why we may fail in our efforts to develop our drug product candidates. These include:

- the possibility that preclinical testing or clinical trials may show that our drug product candidates are ineffective and/or cause harmful side effects sufficient to prevent marketing;
- our drug product candidates may prove to be too expensive to manufacture or administer to patients;
- even if we believe our preclinical and clinical data demonstrate the safety and effectiveness of our products, regulatory authorities may disagree and deem these data insufficient to support approval;
- our drug product candidates may otherwise fail to receive necessary regulatory approvals from the FDA or foreign regulatory authorities in a timely manner, or at all;
- our drug product candidates, if approved, may not be produced in commercial quantities;
- our drug product candidates, if approved, may not achieve required pricing or commercial acceptance;
- our drug product candidates may not gain acceptable pricing in the diverse markets, U.S. and international, in which we plan to conduct commercialization activities. For our small patient population indications, favorable pricing in multiple markets is essential to a financially viable product;
- regulatory or governmental authorities may apply restrictions to our drug product candidates, which could adversely affect their commercial success; and
- the proprietary rights of other parties may prevent us or our potential collaborative partners from marketing our drug product candidates.

If we fail to successfully develop our drug product candidates, our financial results and financial condition will be adversely affected, 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.

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If we fail to demonstrate efficacy in our preclinical studies, clinical trials, regulatory submissions and product commercialization activities, our future business prospects, financial condition and operating results 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 efficacy in preclinical studies and clinical trials. Preclinical studies involve testing drug product candidates in appropriate 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.

Moreover, success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and we cannot be sure that the results of later clinical trials will replicate the results of prior clinical trials and preclinical testing. The clinical trial process may fail to demonstrate 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 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. 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.

If any of our future clinical development drug product candidates become the subject of problems, including those related to, among others:

- efficacy or safety concerns with the drug product candidates, even if not justified;
- unexpected side-effects;
- regulatory proceedings subjecting the drug product candidates to potential recall;
- publicity affecting doctor prescription or patient use of the drug product candidates;
- pressure from competitive products; or
- introduction of more effective treatments;

our ability to sustain our development programs will become critically compromised. For example, efficacy or safety concerns may arise, whether or not justified, that could lead to the suspension or termination of our clinical programs. Each clinical phase is designed to test attributes of drug product candidates and problems that might result in the termination of the entire clinical plan can be revealed at any time throughout the overall clinical program. The failure to demonstrate efficacy in our clinical trials would have a material adverse effect on our future business prospects, financial condition and operating results.

Completion of clinical tests depends on, among other things, the number of patients available for testing, which is a function of many factors, including the number of patients with the relevant conditions, the nature of the clinical testing, the proximity of patients to clinical testing centers, the eligibility criteria for tests as well as competition with other clinical testing programs involving the same patient profile but different treatments. We will 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 and manage test requests, which may result in delays or failure to complete trials, if the third parties fail to perform or to meet the applicable standards. 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.

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If we do not achieve our projected development and commercialization goals in the time frames we announce and expect, the credibility of our management and our technology may be adversely affected and, as a result, the price of our common stock may decline and our reputation among potential collaborators may also decline.

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

From time to time, we may publicly announce the expected 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, including for clinical trials, due to factors such as delays, scheduling conflicts with participating clinicians and clinical institutions and the rate of patient enrollment. In certain circumstances we will rely on academic 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 less control over the timing and other aspects of these clinical trials than if we conducted them entirely on our own.

If we do not meet the milestones as publicly announced, our stockholders or potential stockholders may lose confidence in our ability to meet these milestones and, as a result, the price of our common stock may decline.

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

With respect to most of our drug product candidates, it will take several years before we are able to develop them into marketable drug product candidates, if at all. The marketing and sales effort of our products, our ability to gain adequate reimbursement, if 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 pilot scale and commercial scale manufacturing processes and facilities;
- market and distribute our products; and
- establish and develop quality control, regulatory, medical, manufacturing, 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;
- 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 our products, if approved, the success of our research initiatives, regulatory approvals, the timing of events outside our direct control such as negotiations with potential strategic partners and other factors. In additional, 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, with acceptable financial terms, if at all. Any of these uncertain events can significantly change our cash requirements as they determine such one-time events as the receipt or payment of major milestones and other payments.

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

entity or to cease operations.

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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, and/or adversely affect our reputation, any of which could have a material adverse effect on our business, prospects, financial condition, and operating results.

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, in which case 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.

We entered into: a licensing agreement with UCSD for RP103/RP104; a licensing agreement with Washington University for mesoderm development protein, or Mesd; 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, Washington University 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, Washington University 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, Washington University or Yeda agreements are terminated by either party, we would lose our rights to RP103/RP104, in the case of UCSD, would lose our rights to the Mesd technology, in the case of the Washington University agreement, 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 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.

If we fail to compete successfully with respect to acquisitions, product or technology licenses, joint venture and other collaborative opportunities, we may be limited in our ability to develop our current or future drug product candidates.

Our competitors compete with us to attract established biotechnology and pharmaceutical companies or organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. Collaborations include licensing proprietary technology from, and other relationships with, academic research institutions and government supported research or healthcare organizations. If our competitors successfully enter into partnering arrangements or license agreements with academic research institutions or governmental research organizations, we will then be precluded from pursuing those specific opportunities. Since each of these opportunities is unique, we may not be able to find a substitute. Other companies have already begun many drug development programs, which may target diseases that we are also targeting, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities. Universities and public and private research institutions also compete with us or seek appropriate research collaborations with us or our competitors. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our drug product candidates. We will attempt to license this proprietary technology, if available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to

compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products.

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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. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to pass through price increases on to our future customers due to the process by which healthcare providers are reimbursed for our future products by the government.

The U.S. credit and capital markets have recently experienced historic dislocations and liquidity disruptions 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 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.

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 establish products using our technologies as a standard of care for various indications, which may limit our revenue growth and profitability and could have a material adverse effect on our business, prospects, financial condition and operating results.

We will need to establish relationships with additional leading scientists and research institutions. We believe that such relationships are pivotal to establishing products using our technologies as a standard of care for various indications. Although we have established a Medical and Scientific Advisory Board and research collaborations, there is no assurance that our Advisory Board members 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 scientific relationships to assist in our research and development, we may not be able to successfully develop our drug product candidates.

If the manufacturers or suppliers upon whom we rely fail to produce in the volumes and quality that we require on a timely basis, or to comply with stringent regulations applicable to them, we may face delays in the development and commercialization of, or be unable to meet demand for, our products, if any, and may lose time in the development process and lose potential revenues.

We do not currently manufacture our drug product candidates and do not currently plan to develop the capacity to do so. 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. 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 manufacturers and key suppliers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes, unstable political environments at foreign facilities or financial difficulties. If these manufacturers or key suppliers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to timely launch any potential product candidate, if approved, would be jeopardized.

In addition, all manufacturers and suppliers of pharmaceutical products must comply with cGMP requirements enforced by the FDA, through its facilities inspection program. The FDA is likely to conduct inspections of our third party manufacturer and key supplier facilities as part of their review of any of our NDAs. If our third party manufacturers and key suppliers are not in compliance with cGMP requirements, it may result in a delay of approval, particularly if these sites are supplying single source ingredients required for the manufacture of any potential product. These cGMP requirements include quality control, quality assurance and the maintenance of records and

documentation. Furthermore, regulatory qualifications of manufacturing facilities are applied on the basis of the specific facility being used to produce supplies. As a result, if one of the manufacturers that we rely on shifts production from one facility to another, the new facility must go through a complete regulatory qualification and be approved by regulatory authorities prior to being used for commercial supply. Our manufacturers may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. A failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall, or withdrawal of product approval. If the safety of any quantities supplied is compromised due to our third party manufacturer's or key supplier's failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products.

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If we fail to obtain or maintain orphan drug 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. 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.

Because the extent and scope of patent protection for some of our drug products is 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 our drug products that do not have 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 for RP103 for the potential treatment of cystinosis and the potential treatment of HD, and even if we obtain orphan drug designation for our future drug product candidates, due to the uncertainties associated with developing pharmaceutical products, 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.

There are many difficult challenges associated with developing proteins that can be used to transport therapeutics across the blood-brain barrier.

Our RAP technology has a potential clinical use as a drug transporter through the blood-brain barrier. However, we do not know that our technology will work or work safely. Many groups and companies have attempted to solve the critical medical challenge of developing an efficient method of transporting therapeutic proteins from the blood stream into the brain. Unfortunately, these efforts to date have met with little success due in part to a lack of adequate understanding of the biology of the blood-brain barrier and to the enormous scientific complexity of the transport process itself. In the research and development of our RAP technology, we will certainly face many of the same issues that have caused these earlier attempts to fail. It is possible that:

- we or any future collaborator/licensee will not be able to produce enough RAP drug product candidates for testing;
- the pharmacokinetics, or where the drug distributes in the body, of our RAP drug product candidates will preclude sufficient binding to the targeted receptors on the blood-brain barrier;
- the targeted receptors are not transported across the blood-brain barrier;
- other features of the blood-brain barrier, apart from the cells, block access molecules to brain tissue after transport across the cells;
- the targeted receptors are expressed on the blood-brain barrier at densities insufficient to allow adequate transport of our RAP drug product candidates into the brain;
- targeting of the selected receptors induces harmful side-effects which prevent their use as drugs; or
- that we or our future collaborator/licensee's RAP drug product candidates cause unacceptable side-effects.

Any of these conditions may preclude the use of RAP or RAP fusion compounds from potentially treating diseases affecting the brain.

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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 our drug product candidates. Many 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 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.

Our reliance on third parties, such as collaborators (including government agencies such as the U.S. National Institutes of Health and a French academic healthcare agency), university laboratories, contract manufacturing organizations, contract or clinical research organizations and consulting organizations, 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.

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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 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 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.

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

Even if we obtain FDA approval to market our potential products in the U.S., our sales in the U.S. may be reduced if our products are 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.

The use of any of our drug product candidates in clinical trials or the commercialization of our drug products in the future 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 our drug product candidates. While we are in clinical stage testing, 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 are already critically ill or suffering from chronic debilitating diseases when they enter a trial. 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 a $5.0 million clinical product liability insurance policy, it may not be sufficient to cover future claims.

In addition, the product liability insurance that we will need to obtain in connection with the commercial sales of our product candidates, if and when they receive regulatory approval, may not be sufficient or available in meaningful amounts or at a reasonable cost. Furthermore, while we continue to take precautionary steps, 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 currently do not have any clinical or product liability claims or threats of claims filed against us.

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 our Chief Executive Officer, Dr. Christopher M. Starr, our Chief Scientific Officer, Dr. Todd C. Zankel, our Chief Financial Officer, Kim R. Tsuchimoto, Ted Daley, the President of our clinical development subsidiary, and Dr. Patrice P. Rioux, Chief Medical Officer of our clinical development subsidiary. 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 employees are retained 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, or that are terminated from, 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.

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Our success depends on our ability to manage our growth.

With the potential commercial launch of RP103 for cystinosis, the continued progress of our clinical-stage programs and with our plan to in-license and acquire additional clinical-stage product candidates, we will be required to retain existing and add required new experienced personnel in the commercial, regulatory, 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.

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

In connection with the potential commercial launch of RP103 for nephropathic cystinosis, we have expanded our operations in the E.U. and 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 with individual country regulatory and statutory laws;
- delayed communication due to time zone differences between the U.S. and Europe;
- creating a cohesive branding and corporate presence between the U.S. and European offices;
- the potential impairment of relationships with employees and suppliers as a result of any integration of new personnel; and
- greater than anticipated costs of maintaining E.U. presence and related Dutch tax structure.

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 third-party manufacturers with whom we contract and our single-source suppliers of raw materials are also vulnerable to damage from other types of disasters, including fires, floods, power loss and similar events. If any disaster were to occur, our ability to continue our product development programs, 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.

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We will incur increased costs as a result of recently enacted and proposed changes in laws and regulations and our management will 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 have led to an increase in the costs of compliance for companies similar to us, including 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.

If we fail to establish and maintain collaborations or if our partners do not perform, we may be unable to develop and commercialize our product candidates, which may adversely affect our future revenues and financial condition.

We have entered into collaborative arrangements with third parties to develop and/or commercialize product candidates. Additional collaborations might be necessary in order for us to fund our research and development activities and third-party manufacturing arrangements, seek and obtain regulatory approvals and successfully commercialize existing and future product candidates. If we fail to maintain the existing collaborative arrangements held by us or fail to enter into additional collaborative arrangements, the number of product candidates from which we could receive future revenues would decline.

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

- collaborative arrangements might not be available on terms which are reasonably favorable to us;
- 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;
- outside of agreement terms (which may be different or costly to enforce, if enforceable), 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 their obligations 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.

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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 time of our management. Management would spend less time and resources on developing drug product candidates, which 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 and can be 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 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.

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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.

Our ability to utilize the benefits of our net operating loss carryforwards and other tax attributes if we generate income, could be substantially limited if we experience an "ownership change" within the meaning of Section 382 of the Internal Revenue Code.

Under Sections 382 and 383 of the Internal Revenue Code, as amended, limitations may apply to the use by a "loss corporation" of certain tax attributes including net operating loss carryforwards, capital loss carryforwards, unrealized built-in losses and tax credits in the event an "ownership change" occurs for tax purposes. In general, an "ownership change" would occur if there is a cumulative change in the ownership of our common stock of more than 50 percentage points by one or more "5% shareholders" during a three-year period. In the event of an "ownership change," the tax attributes from prior periods that may be used to offset our taxable income (if we generate taxable income in the future) in each year after the "ownership change" will be subject to an annual limitation. In general, the annual limitation is equal to the product of the fair market value of our common stock on the date of the "ownership change" and the "long term tax exempt rate" (which is published monthly by the Internal Revenue Service), subject to specified adjustments. This limitation could accelerate our cash tax payments if we generate income and could result in a significant portion of our deferred tax asset expiring before we could fully utilize them.

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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 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 may be more difficult for us to, or we may be unable to, raise additional capital.

As of May 31, 2012, we had warrants outstanding related to the assumption of warrants from our Encode merger, issuance of warrants related to our May/June 2008 private placement, issuance of warrants related to our August 2009 private placement, the assumption of warrants pursuant to the 2009 Merger, issuance of warrants related to our December 2009 registered direct offering and issuance of warrants related to our August 2010 private placement. See Note 9 in our condensed consolidated financial statements located in this quarterly report on Form 10-Q for the quarter ended May 31, 2012 for further discussion regarding our common stock warrants.

As of May 31, 2012, there were (i) outstanding warrants to purchase 5,187,772 shares of our common stock at a weighted-average exercise price of $3.02 per share (ii) outstanding options to purchase 5,945,988 shares of our common stock outstanding under our 2010 and 2006 Raptor stock option plans at a weighted-average exercise price of $4.10, (iii) options to purchase 149,447 shares of our common stock outstanding under our TorreyPines Therapeutics stock option plans at a weighted-average exercise price of $76.26 and (iv) 1,230,993 shares of our common stock available for future stock option grants issued under our 2010 Raptor stock option plan. The shares issuable under our stock option plans will be available for immediate resale in the public market. The shares issuable under the warrants are available for immediate resale in the public market. The market price of our common stock could decline as a result of such resales due to the increased number of shares available for sale in the market.

	Number of shares exercisable	Exercise price	Expiration date
Issued in connection with Encode merger	233,309	$ 2.87	12/13/2015
Issued to placement agents in May/June 2008	432,649	$ 2.36	5/21/2013
Issued to placement agents in August 2009	65,000	$ 1.50	8/21/2014
TorreyPines warrants assumed in 2009 Merger	8,140	$ 80.86*	6/11/2013-9/26/2015
Issued to registered direct investors in Dec. 2009	756,250	$ 2.45	12/22/2014
Issued to private placement investors in Aug. 2010	3,594,472	$ 3.075	8/12/2015
Issued to placement agent in Aug. 2010	97,952	$ 3.075	8/12/2015
Total warrants outstanding	5,187,772	$ 3.02*	

* Weighted-average exercise price

Our executive officers and our Board of Directors own, in the aggregate, 1,720,642 shares, or approximately 3.5% of our outstanding common stock as of May 31, 2012. Sales of a substantial number of shares of our common stock by such officers and directors in the public trading market, whether in a single transaction or a series of transactions, or the perception that these sales may occur, could also have a significant effect on volatility and the trading price of our common stock. Future milestone payments, as more fully set forth under "Contractual Obligations with Thomas E. Daley (assignee of the dissolved Convivia, Inc.)" under Note 10 Commitments and Contingencies in our condensed consolidated financial statements and "Contractual Obligations with Former Encode Security Holders" section located in this quarterly report on Form 10-Q for the quarter ended May 31, 2012, in connection with our acquisition of the

Convivia assets and merger with Encode will result in dilution. We may be required to make additional contingent payments of up to 699,369 shares of our common stock, in the aggregate, under the terms of our acquisition of Convivia assets and merger with Encode, based on milestones related to certain future marketing and development approvals obtained with respect to Convivia and Encode product candidates. The issuance of any of these shares will result in further dilution to our existing stockholders.

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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 or liquidity 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 or liquidity should not invest in our common stock.

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

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.

The market price of our common stock also may be adversely impacted by broad market and industry fluctuations regardless of our operating performance, including general economic and technology trends. The NASDAQ Global Market has, from time to time, experienced extreme price and trading volume fluctuations, and the market prices of biopharmaceutical development companies such as ours have been extremely volatile. Market prices for securities of early-stage pharmaceutical, biotechnology and other life sciences companies have historically been particularly volatile and trading in such securities has often been limited. Some of the factors that may cause the market price of our common stock to fluctuate include:

- the results and timing of regulatory reviews relating to the approval of our drug candidates;
- failure of any of our drug candidates, if approved, to achieve commercial success;
- 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;
- difference in security analysts' and investors' expectations;
- the results of ongoing preclinical studies and planned early stage clinical trials of our preclinical drug candidates;
- 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;
- 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.

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Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock. In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation can result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

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

Our certificate of incorporation authorizes us to issue up to 15.0 million 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 15.0 million 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.

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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.

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Item 6. Exhibits

Exhibit Index

(2)	Plan of acquisition, reorganization, arrangement, liquidation or succession
2.1	Agreement and Plan of Merger and Reorganization, dated as of June 7, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Registration Statement No. 333-136018 filed on July 25, 2006).
2.2	Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated as of August 25, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Amendment No. 1 to Registration Statement No. 333-136018 filed on August 25, 2006).
2.3	Agreement and Plan of Merger and Reorganization, dated July 27, 2009, by and among Raptor Pharmaceuticals Corp., TorreyPines Therapeutics, Inc., a Delaware corporation, and ECP Acquisition, Inc., a Delaware corporation (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
2.4	Form of Voting Agreement between TorreyPines Therapeutics, Inc. and certain stockholders of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 99.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
2.5	Form of Voting Agreement between Raptor Pharmaceuticals Corp. and certain stockholders of TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
(3)(i), (ii)	Articles of incorporation; Bylaws
3.1	Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.2	Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.3	Certificate of Amendment filed with the Secretary of State of the State of Nevada effecting an 8-for-1 reverse stock of the Registrant's common stock and changing the name of the Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.3 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.4	Articles of Conversion filed with the Secretary of State of the State of Nevada changing the state of incorporation of the Registrant (incorporated by reference to Exhibit 3.4 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
3.5	Certificate of Conversion filed with the Secretary of State of the State of Delaware (incorporated by reference to Exhibit 3.5 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).

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

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4.5	Registration Rights Agreement dated as of May 3, 2004, between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on May 5, 2004).
4.6	Form of Warrant issued to Comerica Bank on July 1, 2003 (incorporated by reference to Exhibit 4.14 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.7	Form of Warrant issued to Silicon Valley Bank on December 8, 2000 (incorporated by reference to Exhibit 4.15 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.8	Form of Warrant issued to Oxford Financial and Silicon Valley Bank on September 27, 2005 (incorporated by reference to Exhibit 4.16 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.9	Rights Agreement, dated as of May 13, 2005, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on May 16, 2005).
4.10	Amendment to Rights Agreement, dated as of June 7, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed on June 12, 2006).
4.11	Form of Warrant issued to Comerica Bank on June 11, 2008 (incorporated by reference to Exhibit 4.1 to the Registrant's Report on Form 8-K, filed on June 17, 2008).
4.12	Amendment to Rights Agreement, dated as of October 3, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.19 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
4.13	Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between TorreyPines and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company) (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
4.14	Amendment to Rights Agreement, dated August 6, 2010, by and between the Registrant and American Stock Transfer & Trust Company, LLC (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on August 10, 2010).
4.15 *	Warrant to purchase common stock dated December 14, 2007 issued to Flower Ventures, LLC (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April 15, 2008).
4.16 *	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 10Q, filed on April 9, 2010).
4.17 *	Warrant to purchase common stock dated December 14, 2007 issued to ICON Partners, LP (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April

15, 2008).

4.18 *	Form of Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on May 22, 2008).
4.19 *	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).
4.20 *	Form of Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on August 25, 2009).
4.21 *	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).
4.22	Form of Senior Debt Indenture of the Registrant (incorporated by reference to Exhibit 4.10 to the Registrant's Registration Statement on Form S-3, filed on October 7, 2009).
4.23	Form of Subordinated Debt Indenture of the Registrant (incorporated by reference to Exhibit 4.11 to the Registrant's Registration Statement on Form S-3, filed on October 7, 2009).
4.24	Form of Investor Warrants (incorporated by reference to Exhibit 4.1 on Registrant's Current Report on Form 8-K filed on December 18, 2009).
4.25	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).
4.26	Placement Agent Warrant (incorporated by reference to Exhibit 4.2 on Registrant's Current Report on Form 8-K filed on August 13, 2010).

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4.27	Reference is made to Exhibits 3.1 through 3.8.
(10)	Material Contracts
10.1†**#	Employment Agreement dated April 15, 2012 between Raptor Pharmaceuticals Europe B.V. and Henk Doude van Troostwijk.
10.2†**	Intellectual Property Platform Contribution Transaction License Agreement, dated April 16, 2012, between RPTP European Holdings, C.V. and Raptor Therapeutics Inc.
10.3	Sales Agreement, dated as of April 30, 2012, between Raptor Pharmaceutical Corp. and Cowen and Company, LLC. (incorporated by reference to Exhibit 1.1 on Registrant's Current Report on Form 8-K filed on May 1, 2012).
(31)	Section 302 Certification
31.1†	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director
31.2†	Certification of Kim R. Tsuchimoto, 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 Kim R. Tsuchimoto, Chief Financial Officer, Secretary and Treasurer
101***	The following materials from the Raptor Pharmaceutical Corp. Quarterly Report on Form 10-Q for the quarter ended May 31, 2012, formatted in Extensible Business Reporting Language (XBRL): (i) the Condensed Consolidated Balance Sheets; (ii) the Condensed Consolidated Statements of Comprehensive Loss; (iii) the Condensed Consolidated Statement of Stockholders' Equity (Deficit); (iv) the Condensed Consolidated Statements of Cash Flows; and (v) related notes, tagged as blocks of text.

*	The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.15 - 4.21 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 separately with the SEC.
***	Furnished herewith and not "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended.
#	Indicates a management contract or compensatory plan or arrangement.
†	Filed herewith.

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language). Users of this data are advised that, pursuant to Rule 406T of Regulation S-T, the interactive data file is deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, is deemed not filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is otherwise not subject to liability under these sections.

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

RAPTOR PHARMACEUTICAL CORP.

By: /s/ Christopher M. Starr
Christopher M. Starr, Ph.D.
Chief Executive Officer and
Director
(Principal Executive Officer)
Date: July 10, 2012

By: /s/ Kim R. Tsuchimoto
Kim R. Tsuchimoto
Chief Financial Officer,
Secretary and Treasurer
(Principal Financial Officer
and Principal Accounting
Officer)
Date: July 10, 2012

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Exhibit Index

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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).
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4.14	Amendment to Rights Agreement, dated August 6, 2010, by and between the Registrant and American Stock Transfer & Trust Company, LLC (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on August 10, 2010).
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4.25	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).

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4.26	Placement Agent Warrant (incorporated by reference to Exhibit 4.2 on Registrant's Current Report on Form 8-K filed on August 13, 2010).
4.27	Reference is made to Exhibits 3.1 through 3.8.
(10)	Material Contracts
10.1†**#	Employment Agreement dated April 15, 2012 between Raptor Pharmaceuticals Europe B.V. and Henk Doude van Troostwijk.
10.2†**	Intellectual Property Platform Contribution Transaction License Agreement, dated April 16, 2012, between RPTP European Holdings, C.V. and Raptor Therapeutics Inc.
10.3	Sales Agreement, dated as of April 30, 2012, between Raptor Pharmaceutical Corp. and Cowen and Company, LLC. (incorporated by reference to Exhibit 1.1 on Registrant's Current Report on Form 8-K filed on May 1, 2012).
(31)	Section 302 Certification
31.1†	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director
31.2†	Certification of Kim R. Tsuchimoto, 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 Kim R. Tsuchimoto, Chief Financial Officer, Secretary and Treasurer
101***	The following materials from the Raptor Pharmaceutical Corp. Quarterly Report on Form 10-Q for the quarter ended May 31, 2012, formatted in Extensible Business Reporting Language (XBRL): (i) the Condensed Consolidated Balance Sheets; (ii) the Condensed Consolidated Statements of Comprehensive Loss; (iii) the Condensed Consolidated Statement of Stockholders' Equity (Deficit); (iv) the Condensed Consolidated Statements of Cash Flows; and (v) related notes, tagged as blocks of text.

*	The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.15 - 4.21 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 separately with the SEC.
***	Furnished herewith and not "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended.
#	Indicates a management contract or compensatory plan or arrangement.
†	Filed herewith.

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language). Users of this data are advised that, pursuant to Rule 406T of Regulation S-T, the interactive data file is deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, is deemed not filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is otherwise not subject to liability under these sections.