

Raptor Pharmaceutical Corp  
Form 10-Q  
July 13, 2011

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549  
FORM 10-Q

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

For the quarterly period ended May 31, 2011

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  
(State or other jurisdiction of incorporation  
or organization)

86-0883978  
(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  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 (§

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232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

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

Large accelerated  
filer

Accelerated filer

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

Smaller reporting  
company

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

There were 34,595,103 shares of the registrant's common stock, \$.001 par value per share, outstanding at July 5, 2011.

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RAPTOR PHARMACEUTICAL CORP.

FORM 10-Q FOR THE QUARTER ENDED MAY 31, 2011

Table of Contents

	Page
Part 1 - Financial Information	
Item 1	
Financial Statements	
Condensed Consolidated Balance Sheets as of May 31, 2011 (unaudited) and August 31, 2010	2
Unaudited Condensed Consolidated Statements of Operations for the three month periods ended May 31, 2011 and 2010	3
Unaudited Condensed Consolidated Statements of Operations for the nine month periods ended May 31, 2011 and 2010 and the cumulative period from September 8, 2005 (inception) to May 31, 2011	4
Unaudited Condensed Consolidated Statements of Cash Flows for the nine month periods ended May 31, 2011 and 2010 and the cumulative period from September 8, 2005 (inception) to May 31, 2011	5
Notes to Condensed Consolidated Financial Statements	6
Management's Discussion and Analysis of Financial Condition and Results of Operations	31
Quantitative and Qualitative Disclosures About Market Risk	53
Item 2	
Item 3	
Item 4	
Controls and Procedures	53
Part II - Other Information	
Item 1	
Legal Proceedings	53
Item 1A	
Risk Factors	54
Item 2	
Unregistered Sales of Equity Securities and Use of Proceeds	57
Item 3	
Defaults Upon Senior Securities	58
Item 4	
(Removed and Reserved)	58
Item 5	
Other Information	58
Item 6	
Exhibits	59
SIGNATURES	61

## PART I – FINANCIAL INFORMATION

## Item 1. Financial Statements.

Raptor Pharmaceutical Corp.  
(A Development Stage Company)  
Condensed Consolidated Balance Sheets

ASSETS	May 31, 2011 (unaudited)	August 31, 2010 (1)
Current assets:		
Cash and cash equivalents	\$ 13,325,695	\$ 16,953,524
Restricted cash	114,282	-
Prepaid expenses and other	191,074	285,898
Total current assets	13,631,051	17,239,422
Intangible assets, net	3,397,417	3,512,542
Goodwill	3,275,403	3,275,403
Fixed assets, net	65,056	93,249
Deposits	104,906	102,906
Deferred offering costs	-	166,015
Total assets	\$ 20,473,833	\$ 24,389,537
<b>LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)</b>		
Liabilities		
Current liabilities:		
Accounts payable	\$ 1,144,624	\$ 637,321
Accrued liabilities	1,017,312	1,129,810
Common stock warrant liability	32,852,755	15,780,216
Deferred rent	27,029	2,673
Capital lease liability – current	3,106	4,865
Total current liabilities	35,044,826	17,554,885
Capital lease liability - long-term	-	1,811
Total liabilities	35,044,826	17,556,696
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, 33,127,556 and 30,076,758 shares issued and	33,128	30,077

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outstanding as at May 31, 2011 and August 31, 2010,  
respectively

Additional paid-in capital	59,563,190	47,617,449
Accumulated other comprehensive loss	(395)	(7,854)
Deficit accumulated during development stage	(74,166,916)	(40,806,831)
Total stockholders' equity (deficit)	(14,570,993)	6,832,841
Total liabilities and stockholders' equity (deficit)	\$ 20,473,833	\$ 24,389,537

(1) Derived from the Company's audited consolidated financial statements as of August 31, 2010.

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

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

For the three month periods from  
March 1, 2011 to May 31,

	2011	2010
Revenues:	\$ -	\$ -
Operating expenses:		
General and administrative	1,733,218	938,113
Research and development	3,901,651	2,176,658
Total operating expenses	5,634,869	3,114,771
Loss from operations	(5,634,869)	(3,114,771)
Interest income	12,116	5,489
Interest expense	(486)	(814)
Foreign currency transaction loss	-	-
Adjustment to fair value of common stock warrants	(14,641,775)	(4,345,251)
Net loss	\$ (20,265,014)	\$ (7,455,347)
Loss per share from operations:		
Basic and diluted	\$ (0.17)	\$ (0.14)
Net loss per share:		
Basic and diluted	\$ (0.62)	\$ (0.33)
Weighted average shares outstanding used to compute:		
Basic and diluted	32,594,450	22,842,875

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

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

	For the nine month periods from		For the cumulative period from
	September 1, 2010 to May 31, 2011	September 1, 2009 to May 31, 2010	September 8, 2005 (inception) to May 31, 2011
Revenues:	\$ -	\$ -	\$ -
Operating expenses:			
General and administrative	4,565,829	2,926,960	15,242,217
Research and development	10,266,027	6,271,997	34,474,391
In-process research and dev.	-	-	240,625
Total operating expenses	14,831,856	9,198,957	49,957,233
Loss from operations	(14,831,856)	(9,198,957)	(49,957,233)
Interest income	31,348	15,897	358,952
Interest expense	(1,484)	(2,649)	(115,371)
Foreign currency transaction gain (loss)	89	-	(368)
Adjustment to fair value of common stock warrants	(18,558,182)	(5,388,641)	(24,452,896)
Net loss	(33,360,085)	\$ (14,574,350)	\$ (74,166,916)
Loss per share from operations:			
Basic and diluted	\$ (0.47)	\$ (0.44)	
Net loss per share:			
Basic and diluted	\$ (1.06)	\$ (0.69)	
Weighted average shares outstanding used to compute:			
Basic and diluted	31,536,829	20,999,659	

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





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

	For the nine month periods from		For the cumulative
	September 1, 2010	September 1, 2009 to	period from
	to May 31, 2011	May 31, 2010	September 8, 2005
			(inception)
			to May 31, 2011
Cash flows from operating activities:			
Net loss	\$ (33,360,085)	\$ (14,574,350)	\$ (74,166,917)
Adjustments to reconcile net loss to net cash used in operating activities:			
Employee stock-based compensation expense	1,541,888	140,857	2,973,646
Consultant stock-based compensation expense	38,016	75,405	523,957
Fair value adjustment of common stock warrants	18,558,182	5,388,641	24,452,896
Amortization of intangible assets	115,125	113,875	512,583
Depreciation of fixed assets	58,182	55,026	481,363
In-process research and development	-	-	240,625
Amortization of capitalized finder's fee	-	-	102,000
Capitalized acquisition costs previously expensed	-	-	38,000
Changes in assets and liabilities:			
Prepaid expenses and other	94,824	75,933	(91,636)
Intangible assets	-	-	(150,000)
Deposits	(2,000)	(2,700)	(104,907)
Accounts payable	507,303	191,699	1,144,624
Accrued liabilities	(112,498)	(816,996)	336,586
Deferred rent	24,356	1,081	26,924
Net cash used in operating activities	(12,536,707)	(9,351,529)	(43,680,256)
Cash flows from investing activities:			
Purchase of fixed assets	(29,989)	(14,400)	(527,095)
Cash acquired in 2009 Merger	-	581,395	581,391
Increase in restricted cash	(114,282)	-	(114,282)
Net cash provided by (used in) investing activities	(144,271)	566,995	(59,986)
Cash flows from financing activities:			

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Proceeds from the sale of common stock	-	7,495,116	39,941,278
Proceeds from the sale of common stock under an equity line	6,747,778	2,399,976	11,647,729
Proceeds from the exercise of common stock warrants	2,300,838	56,018	9,285,357
Proceeds from the exercise of common stock options	8,828	50,060	81,549
Fundraising costs	(8,182)	(1,430,488)	(4,183,362)
Proceeds from the sale of common stock to initial investors	-	-	310,000
Proceeds from bridge loan	-	-	200,000
Repayment of bridge loan	-	-	(200,000)
Principal payments on capital lease	(3,572)	(3,022)	(16,219)
Net cash provided by financing activities	9,045,690	8,567,660	57,066,332
Foreign currency translation gain (loss)	7,459	-	(395)
Net increase (decrease) in cash and cash equivalents	(3,627,829)	(216,874)	13,325,695
Cash and cash equivalents, beginning of period	16,953,524	3,701,787	-
Cash and cash equivalents, end of period	\$ 13,325,695	\$ 3,484,913	\$ 13,325,695
Supplemental disclosure of non-cash financing activities:			
Warrants issued in connection with financing	\$ -	\$ 1,916,011	\$ 16,310,414
Common stock and warrants issued in connection with reverse merger	\$ -	\$ 4,417,046	\$ 4,417,046
Common stock issued as fee for equity line	\$ 352,500	\$ 363,331	\$ 827,637
Fair value of warrant liability reclassified to equity upon exercise	\$ 1,485,643	\$ -	\$ 1,485,643
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
Amortization of direct offering costs	\$ -	\$ -	\$ 156,400

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





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(f/k/a Bennu Pharmaceuticals Inc.) (f/k/a Raptor Pharmaceutical Inc.)  
(merged with TPTX, Inc. on August 30, 2010)  
|  
Raptor Pharmaceuticals Europe B.V. (Netherlands)

-6-

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RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

Raptor is a publicly-traded biotechnology company dedicated to speeding the delivery of new treatment options to patients by enhancing existing therapeutics through the application of highly specialized drug targeting platforms and formulation expertise. The Company focuses on underserved patient populations where it can have the greatest potential impact. Raptor's clinical division advances clinical-stage product candidates towards marketing approval and commercialization. Raptor's clinical programs include DR Cysteamine for the potential treatment of nephropathic cystinosis, non-alcoholic steatohepatitis ("NASH"), and Huntington's Disease. Raptor also has Convivia™ for the potential treatment of aldehyde dehydrogenase ("ALDH2") deficiency, a clinical stage product candidate for which it has out-licensed to a Taiwanese firm and continues to seek additional Asian out-licenses or to form a development partnership franchise in Asia where ALDH2 deficiency is prevalent. The Company is also developing tezampanel in a planned Phase 1 study for the potential treatment of thrombotic disorder.

Raptor's preclinical division bioengineers novel drug candidates and drug-targeting platforms derived from the human receptor-associated protein ("RAP") and related proteins. Raptor's preclinical programs target cancer, neurodegenerative disorders and infectious diseases. The HepTide™ program is designed to utilize engineered RAP-based peptides conjugated to drugs to target delivery to the liver to potentially treat primary liver cancer and other liver diseases. NeuroTrans™ represents engineered RAP peptides created to target receptors in the brain and is currently, in collaboration with Roche, undergoing preclinical evaluation for their ability to enhance the transport of therapeutics across the blood-brain barrier. WntTide™ is based upon Mesd and Mesd peptides that the Company is studying in a preclinical breast cancer model for WntTide™'s potential inhibition of Wnt signaling through LRP5, which may block cancers dependent on signaling through LRP5 or LRP6.

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 Pharmaceuticals Corp., Raptor Discoveries Inc., and Raptor Therapeutics Inc., such subsidiaries incorporated in Delaware on May 5, 2006, September 8, 2005 (date of inception), and August 1, 2007, respectively, and Raptor Pharmaceuticals Europe B.V. incorporated in the Netherlands on December 15, 2009. 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, 2011, the Company had accumulated losses of approximately \$74.2 million. Management expects to incur further losses for the foreseeable future. Management believes that the Company's cash and cash equivalents as of June 30, 2011 of approximately \$15.7 million will be sufficient to meet the Company's obligations into January 2012. The Company plans to raise equity funds by end of the third calendar quarter of 2011 and continues to review strategic partnerships and collaborations as a potential means to fund its preclinical and clinical programs beyond January 2012. 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, loans 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.

-7-

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RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

On September 29, 2009, upon the closing of the merger with RPC (as discussed further in the Note 9, Issuance of Common Stock), RPC's stockholders exchanged each share of RPC's common stock into .2331234 shares of the post-merger company and the exercise prices and stock prices were divided by .2331234 to reflect the post-merger equivalent stock prices and exercise prices. Therefore, all shares of common stock and exercise prices of common stock options and warrants are reported in these condensed consolidated financial statements on a post-merger basis.

The Company's independent registered public accounting firm has audited the Company's consolidated financial statements for the years ended August 31, 2010 and 2009. The November 22, 2010 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 to date.

(b) Use of Estimates

The preparation of financial statements in conformity with United States generally accepted accounting principles 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., (the "BV"), the Company's European subsidiary, records its functional currency as the European Euro. At quarter-end the 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. The 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) Segment Reporting

The Company has determined that it operates in two operating segments, preclinical development and clinical development. Operating segments are components of an enterprise for which separate financial information is available and are evaluated regularly by the Company in deciding how to allocate resources and in assessing performance. The Company's chief executive officer assesses the Company's performance and allocates its resources.



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Below is a break-down of the Company's net loss and total assets by operating segment:

For the three months ended May 31,						
	2011			2010		
	Preclinical	Clinical	Total	Preclinical	Clinical	Total
Net loss	\$ (2,938,749)	\$ (17,326,265)	\$ (20,265,014)	\$ (1,873,835)	\$ (5,581,512)	\$ (7,455,347)
Total assets	3,588,228	16,885,605	20,473,833	2,689,609	8,167,950	10,857,559

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

For the nine months ended May 31,

	2011			2010		
	Preclinical	Clinical	Total	Preclinical	Clinical	Total
Net loss	\$ (5,895,442)	\$ (27,464,643)	\$ (33,360,085)	\$ (3,902,752)	\$ (10,671,598)	\$ (14,574,350)
Total assets	3,588,228	16,885,605	20,473,833	2,689,609	8,167,950	10,857,559

(f) 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. The Company has not experienced any losses on these investments. Restricted cash represents compensating balances required by our U.S. and European banks as collateral for credit cards.

(g) Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine, to the RAP technology, 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 DR Cysteamine and the RAP technology 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.

(h) 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.

(i) 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.

(j) 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. The Company has not identified any such impairment losses to date.

(k) 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

-9-

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RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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 operations. Warrants which are recorded as liabilities that are exercised are re-measured and marked to market the day prior to exercise and such value is recorded as adjustment to fair value of common stock warrants with an offset to additional paid in capital.

(l) 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.

(m) 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, 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.

(n) 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. The Company reviews each product candidate acquisition to determine the existence of in-process research and development.

(o) 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

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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,	
	2011	2010
Warrants to purchase common stock	9,425,017	5,543,738
Options to purchase common stock	3,589,940	1,390,353
Total potentially dilutive securities	13,014,957	6,934,091

(p) 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

-10-

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

period. The fair value of the equity award granted is estimated on the date of the grant. The Company previously applied Accounting Principles Board (“APB”) Opinion No. 25, Accounting for Stock Issued to Employees, and related interpretations and provided the required pro forma disclosures required by SFAS No. 123, Accounting for Stock-Based Compensation. 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 (“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). See Note 8, Stock Option Plans, for further discussion of employee stock-based compensation.

(q) Recent Accounting Pronouncements

In December 2010, the FASB issued 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 will adopt these standards on September 1, 2011 and is currently assessing the impact on its condensed consolidated financial statements.

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. The Company will adopt these standards on March 1, 2012 and is currently assessing the impact on its condensed consolidated financial statements.

In June 2011, 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 will adopt these standards on September 1, 2012. Because ASU

2011-05 impacts presentation only, it will have no effect on the Company's condensed consolidated financial statements or on its financial condition.

(3) INTANGIBLE ASSETS AND GOODWILL

On January 27, 2006, BioMarin Pharmaceutical Inc. ("BioMarin") assigned the intellectual property and other rights relating to the RAP technology to the Company. As consideration for the assignment of the RAP technology, BioMarin will receive milestone payments based on certain financing and regulatory triggering events. No other consideration was paid for this assignment. The Company has recorded \$150,000 of intangible assets on the condensed consolidated balance sheets as of May 31, 2011 and August 31, 2010 based on the estimated fair value of its agreement with BioMarin.

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

On December 14, 2007, the Company acquired the intellectual property and other rights to develop DR Cysteamine 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 9 below.

Summary of intangibles acquired as discussed above:

Intangible asset (IP license) related to the Encode merger	\$	2,620,000
Intangible asset related to NeuroTrans™ purchase from BioMarin		150,000
Intangible assets (out-license) related to the 2009 Merger		240,000
In-process research and development (IP license) related to the 2009 Merger		900,000
Total intangible assets		3,910,000
Less accumulated amortization		(512,583)
Intangible assets, net	\$	3,397,417

The intangible assets related to DR Cysteamine and NeuroTrans™ are being amortized monthly over 20 years, which are the life 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 and nine months ended May 31, 2011 and 2010 and the cumulative period from September 8, 2005 (inception) to May 31, 2011, the Company amortized \$18,741, \$58,182, \$38,375, \$113,875, and \$512,583, respectively, of intangible assets to research and development expense.

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

Amortization period	Amortization expense
September 8, 2005 (inception) to August 31, 2006 – actual	\$ 4,375
Fiscal year ended August 31, 2007 – actual	7,500
Fiscal year ended August 31, 2008 – actual	94,833
Fiscal year ended August 31, 2009 – actual	138,500
Fiscal year ended August 31, 2010 – actual	152,250



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Fiscal year ending August 31, 2011 – estimate	153,500
Fiscal year ending August 31, 2012 – estimate	153,500
Fiscal year ending August 31, 2013 – estimate	153,500
Fiscal year ending August 31, 2014 – estimate	153,500
Fiscal year ending August 31, 2015 – estimate	153,500

Goodwill of \$3,275,404 represents the excess of total consideration recorded for the 2009 Merger over the value of the assets assumed. In October 2010, the Company reviewed the carrying value of goodwill for impairment as of its fiscal year ended August 31, 2010 and determined that there was no impairment. For the three and nine months ended May 31, 2011, there were no indications of impairment of goodwill. Intangibles are tested for impairment whenever events indicate that their carrying values may not be recoverable. There were no indications of impairment of intangible assets during the three and nine months ended May 31, 2011.

**RAPTOR PHARMACEUTICAL CORP.**  
(A Development Stage Company)

**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**

**(4) FIXED ASSETS**

Fixed assets consisted of:

Category	May 31, 2011	August 31, 2010	Estimated useful lives
Leasehold improvements	\$ 124,763	\$ 119,773	Shorter of life of asset or lease term
Office furniture	3,188	3,188	7 years
Laboratory equipment	277,303	277,303	5 years
Computer hardware and software	119,841	94,842	3 years
Capital lease equipment	14,006	14,006	Shorter of life of asset or lease term
Total at cost	539,101	509,112	
Less: accumulated depreciation	(474,045)	(415,863)	
Total fixed assets, net	\$ 65,056	\$ 93,249	

Depreciation expense for the three and nine months ended May 31, 2011 and 2010 and the cumulative period from September 8, 2005 (inception) to May 31, 2011 was \$18,742, \$58,182, \$19,041, \$55,026 and \$481,363, respectively. Accumulated depreciation on capital lease equipment was \$11,492 and \$3,951 as of May 31, 2011, and August 31, 2010, respectively.

**(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, 2011 and August 31, 2010 are summarized as follows:

Assets	Level 1	Level 2	Level 3	May 31, 2011
	\$11,388,325	\$ —	\$ —	\$11,388,325

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Fair value of cash equivalents				
Restricted cash	—	114,282	—	114,282
Total	\$11,388,325	\$ 114,282	\$ —	\$11,502,607

Liabilities

Fair value of common stock warrants	\$ —	\$ —	\$32,852,755	\$32,852,755
Total	\$ —	\$ —	\$32,852,755	\$32,852,755

Assets	Level 1	Level 2	Level 3	August 31, 2010
Fair value of cash equivalents	\$16,509,186	\$ —	\$ —	\$16,509,186
Total	\$16,509,186	\$ —	\$ —	\$16,509,186

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

Liabilities

Fair value of common stock warrants	\$	—	\$	—	\$15,780,216	\$15,780,216
Total	\$	—	\$	—	\$15,780,216	\$15,780,216

Cash equivalents represent the fair value of the Company's investment in four and two money market accounts as of May 31, 2011, and August 31, 2010, respectively.

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 operations.

For the three and nine months ended May 31, 2011 and 2010, 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 losses of \$14.6 million, \$18.6 million, \$4.3 million and \$5.4 million, respectively, in the line item adjustment to fair value of common stock warrants in its condensed consolidated statement of operations. See Note 10 for further discussion on the calculation of the fair value of the warrant liability.

	Warrant liability in millions
Fair value of December 2009 direct offering warrants (including broker warrants) at fiscal year ended August 31, 2010	\$ 5.8
Adjustment to mark to market common stock warrants at quarter ended November 30, 2010	2.3
Adjustment to mark to market common stock warrants at quarter ended February 28, 2011	(1.0)
Adjustment to mark to market common stock warrants at quarter ended May 31, 2011	4.4
December 2009 direct offering common stock warrant liability at fair value on May 31, 2011	11.5
Fair value of August 2010 private placement warrants (including broker warrants) at fiscal year ended August 31, 2010	9.9
Adjustment to mark to market common stock warrants at quarter ended November 30, 2010	3.5
Adjustment to mark to market common stock warrants at quarter ended February 28, 2011	(0.8)
	8.8

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Adjustment to mark to market common stock warrants at quarter ended	
May 31, 2011	
August 2010 private placement common stock warrant liability at fair value on May 31, 2011	21.4
Total warrant liability at May 31, 2011	\$ 32.9

(6) ACCRUED LIABILITIES

Accrued liabilities consisted of:

	May 31, 2011	August 31, 2010
Clinical trial costs	\$ 604,493	\$ 280,918
Accrued vacation and employee benefits	117,935	79,077
Consulting - general and administrative	89,846	19,304
Salaries and wages	67,487	88,024
Patent costs	64,061	8,956
Clinical trial materials	28,151	50,000
Legal fees	26,135	182,890
Auditing and tax preparation fees	19,204	33,245
Clinical milestone payment due to UCSD	-	200,000
Accrued bonuses	-	184,021
Other	-	3,375
Total accrued liabilities	\$ 1,017,312	\$ 1,129,810

-14-

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(7) COMPREHENSIVE LOSS

The following table shows the computation of total comprehensive loss:

	Three months ended May 31,		Nine months ended May 31,	
	2011	2010	2011	2010
Net loss	\$(20,265,014)	\$(7,455,347)	\$(33,360,085)	\$(14,574,350)
Foreign currency translation adjustments	1,910	-	7,459	-
Total comprehensive loss	\$(20,263,104)	\$(7,455,347)	\$(33,352,626)	\$(14,574,350)

Other comprehensive loss includes gains (losses) on the translation of foreign currency denominated financial statements. Adjustments resulting from these translations are accumulated and reported as a component of other comprehensive income in the stockholders' equity section of the balance sheet.

(8) 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 Accounting Principles Board ("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 and nine months ended May 31, 2011 and 2010 and for the cumulative period from September 8, 2005 (inception) to May 31, 2011 was \$362,327, \$1,541,888, \$87,852, \$140,857 and \$2,973,646, respectively, of which cumulatively \$2,394,969 was included in general and administrative expense and \$578,677 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.



RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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

Period*	Risk-free Interest rate	Expected life of stock option	Annual volatility	Annual turnover rate
September 8, 2005 (inception) to August 31, 2006**	5%	10 years	100%	0%
Quarter ended November 30, 2006	5%	8 years	100%	10%
Quarter ended February 28, 2007	5%	8 years	100%	10%
Quarter ended May 31, 2007	5%	8 years	100%	10%
Quarter ended August 31, 2007	4%	8 years	100%	10%
Quarter ended November 30, 2007	3.75%	8 years	109%	10%
Quarter ended February 29, 2008	2%	8 years	119%	10%
Quarter ended May 31, 2008	2%	8 years	121%	10%
Quarter ended August 31, 2008	2.5%	8 years	128%	10%
Quarter ended November 30, 2008	1.5%	7 years	170%	10%
Quarter ended February 28, 2009	2.0%	7 years	220%	10%
Quarter ended May 31, 2009	2.6%	7 years	233%	10%
Quarter ended August 31, 2009	3.2%	7 years	240%	10%
Quarter ended November 30, 2009	3.0%	7 years	245%	10%
Quarter ended February 28, 2010	3.1%	7 years	55%	10%
Quarter ended May 31, 2010	3.1%	7 years	77%	2.5%
Quarter ended August 31, 2010	2.07%	6 years	85%	2.5%
Quarter ended November 30, 2010	1.64%	6 years	88%	2.5%
Quarter ended February 28, 2011	2.42%	6 years	90%	2.5%
Quarter ended May 31, 2011	2.38%	6 years	96%	2.5%

\* Dividend rate is 0% for all periods presented.

\*\*



Stock-based compensation expense was recorded on the condensed consolidated statements of operations 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 since inception.

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.

During the three months ended May 31, 2010, the Company changed its volatility calculation to reflect its historical trading commencing on September 30, 2009, which is the date that the 2009 Merger was consummated and the Company's common stock started trading on NASDAQ. The Company originally estimated volatility based upon historical volatility commencing in June 2006, when it first began trading on the Over-the-Counter Bulletin Board. The Company changed the volatility assumptions to better reflect its anticipated trading on NASDAQ. During the three months ended May 31, 2010, the Company analyzed its actual historical turnover rate and concluded that 2.5% was a more accurate estimate of future turnover rate on an annual basis.

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 and nine months ended May 31, 2011 and 2010 and for the cumulative period from September 8, 2005 (inception) to May 31, 2011 was \$1,007, \$38,016, \$4,721, \$75,405 and \$523,957, respectively, of which cumulatively \$147,295 was included in general and administrative expense and \$376,662 was included in research and development expense.

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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	1,750,680	\$ 3.36	335,859	\$ 0.15
Exercised	—	—	—	—
Canceled	(1,102)	\$ 1,292.00	—	—
Outstanding at November 30, 2010	3,140,866	\$ 7.73	1,424,005	\$ 2.11

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Granted	130,000	\$ 3.54	10,000	\$ 2.66
Exercised	(3,835)	\$ 2.30	—	—
Canceled	(1,724)	\$ 1,075.76	—	—
Outstanding at February 28, 2011	3,265,307	\$ 7.01	1,537,971	\$ 2.02
Granted	325,000	\$ 3.33	—	\$ 2.58
Exercised	-	—	—	—
Canceled	(367)	\$ 614.72	—	—
Outstanding at May 31, 2011	3,589,940	\$ 6.61	1,736,667	\$ 2.08

The weighted average intrinsic values of stock options outstanding and expected to vest and stock options exercisable as of May 31, 2011 and 2010 were \$7,770,104, \$4,090,703, \$1,255,298 and \$854,835, respectively.

There were 2,121,064 options available for grant as of May 31, 2011 under the 2010 Equity Incentive Plan (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 a replenishment 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 replenishment of 6,000,000 shares. The April 7, 2011 replenishment added 1,629,516 shares available

**RAPTOR PHARMACEUTICAL CORP.**  
(A Development Stage Company)

**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**

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, as amended. No further grants will be made under any previous or assumed stock option plans. As of May 31, 2011, the options outstanding under all of the Company's stock option plans consisted of the following:

Range of exercise prices	Options outstanding			Options exercisable		
	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	34,969	7.88	.85	18,212	0.85	
\$1.01 to \$2.00	85,773	8.00	1.71	58,076	1.71	
\$2.01 to \$3.00	1,590,356	7.21	2.56	1,003,513	2.56	
\$3.01 to \$4.00	1,767,924	9.38	3.53	548,063	3.53	
\$4.01 to \$5.00	62,104	7.52	4.58	59,989	4.58	
\$5.01 to \$964.24	48,814	3.90	264.16	48,814	264.16	
	3,589,940	8.40	6.61	1,736,667	10.44	

At May 31, 2011, the total unrecognized compensation cost was approximately \$4.2 million. The weighted-average period over which it is expected to be recognized is 3 years.

**(9) ISSUANCE OF COMMON STOCK**

As of May 31, 2011, there were 33,127,556 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 months ended May 31, 2011, the Company received \$1,743,882 and \$2,300,838, respectively, from the exercise of warrants in exchange for the issuance of 712,238 and 933,858 shares, respectively, of the Company's common stock, respectively. During the cumulative period from September 8, 2005 (inception) through May 31, 2011, the Company received approximately \$9.2 million from the exercise of warrants in exchange for the issuance of an aggregate of 4,675,616 shares.

During the three and nine months ended May 31, 2011, the Company received zero and \$8,828, respectively, from the exercise of stock options in exchange for the issuance of zero and 3,835 shares, respectively, of the Company's common stock. For the cumulative period from September 8, 2005 (inception) through May 31, 2011, the Company received \$81,549 from the exercise of stock options resulting in the issuance of 45,096 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 its clinical division. 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. 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 ConviviaTM. 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 ConviviaTM pursuant to the asset purchase agreement.

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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<sup>TM</sup> in Taiwan. 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 in the amount of \$240,625 on its consolidated statement of operations for the year ended August 31, 2008.

MERGER OF RAPTOR'S CLINICAL DEVELOPMENT SUBSIDIARY 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 clinical development subsidiary 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 the Company's clinical development subsidiary. 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, the Company's clinical development subsidiary, 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 optionholders of Encode (the "Encode Optionholders"), and warrants ("Company Warrants") to purchase 256,034 restricted, unregistered shares of Common Stock to the warrantholders of Encode (the "Encode Warrantholders", and together with the Encode Stockholders and Encode Optionholders, the "Encode Securityholders"), 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 Securityholders as of the closing of the merger with Encode. The purchase price was valued at \$2.6 million, which is 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 Securityholders 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 DR Cysteamine, 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 DR Cysteamine (the "License Agreement"), developed by clinical scientists at the UCSD, School of Medicine. DR Cysteamine 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 DR Cysteamine 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 \$470,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, Huntington’s Disease and NASH.

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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

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 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 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 serves 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.

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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 the clinical division and Patrice P. Rioux., M.D., Ph.D., as Chief Medical Officer of the clinical division.

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 has been assigned to the Company’s clinical segment and 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 (millions)	%
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Cash and equivalents	\$	0.58	13
Other current assets		0.10	2
Accrued liabilities		(0.68)	(15)
Intangible assets:			
In-process research & 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

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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 will be 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 will be 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 liability, as

discussed further below in Note 10.

#### 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 has agreed to purchase up to \$15 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. A post-effective amendment to such registration statement was filed on November 23, 2010 and was declared effective by the SEC on December 1, 2010. The Company has the right over a 25-month period to sell its shares of common stock to LPC in amounts of \$100,000 to up to \$1,000,000 per sale, depending on certain conditions as set forth in the purchase agreement, up to the aggregate amount of \$15 million. The purchase agreement may be terminated by the Company at any time at its discretion without any cost to the Company.

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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 controls the timing and amount of any sales of shares to LPC. LPC does 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 is 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 balance sheet and amortized over the usage of the equity line) as a commitment fee and is required to issue up to an additional 217,549 shares of its common stock pro rata as LPC purchases the \$15 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 has 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.

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. A post-effective amendment to such registration statement was filed on November 23, 2010 and was declared effective by the SEC on December 1, 2010.

## RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

The following is a summary of common stock outstanding as of May 31, 2011:

Transaction	Date of Issuance	Common Stock Issued
Founders' shares	Sept. 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. – Nov. 2007	1,513,359
Stock option exercises	Mar. 2007	3,380
Loan finder's fee	Sept. 2007	46,625
Convivia asset purchase	Oct. 2007 – June 2010	160,272
Encode merger DR Cysteamine asset purchase	Dec. 2007	802,946
Shares issued pursuant to consulting agreement	May 2008	2,040
PIPE — initial tranche	May 2008	1,030,405
PIPE — second tranche	May 2008	69,937
PIPE — third tranche	June 2008	3,562,126
Warrant exercises from warrant exchange PIPE	June/July 2009	2,031,670
Warrant exercises	August 2009	1,738,226
Shares issued in connection with reverse merger	Sept. 2009 – May 2011	1,130,594
Stock option exercises	September 2009	940,863
	October 2009 – Feb. 2011	41,716
Registered direct financing	December 2009	3,747,558
Shares issued to equity line investor (incl. fee shares)	April 2010 – Feb. 2011	4,500,000
2010 private placement	August 2010	4,897,614
Total shares of common stock outstanding		33,127,556

## (10) WARRANTS

The table reflects the number common stock warrants outstanding as of May 31, 2011:



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	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	453,578	\$ 2.36	5/21/2013
Issued to PIPE investors in August 2009	635,990	\$ 3.22	8/21/2011
Issued to placement agents in August 2009	129,733	\$ 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	1,025,000	\$ 2.45	6/22/2011
Issued to registered direct investors in Dec. 2009	1,868,750	\$ 2.45	12/23/2014
Issued to placement agent in Dec. 2009	74,951	\$ 2.50	12/23/2014
Issued to private placement investors in Aug. 2010	4,897,614	\$ 3.075	8/11/2015
Issued to placement agent in Aug. 2010	97,952	\$ 3.075	8/11/2015
Total warrants outstanding	9,425,017	\$ 2.89*	

\* Average exercise price

## RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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 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 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, 2011 and August 31, 2010:

	December 2009 equity financing				August 2010 private placement			
	Series A		Series B		Placement agent		Investors and placement agent	
	At May 31, 2011	At August 31, 2010	At May 31, 2011	At August 31, 2010	At May 31, 2011	At August 31, 2010	At May 31, 2011	At August 31, 2010
Fair value (\$ millions)	8.0	3.7	3.2	2.0	0.3	0.1	21.4	9.9
Black-Scholes inputs:								
Stock price	\$5.54	\$2.98	\$5.54	\$2.98	\$5.54	\$2.98	\$5.54	\$2.98
Exercise price	\$2.45	\$2.45	\$2.45	\$2.45	\$2.50	\$2.50	\$3.075	\$3.075
Risk free interest rate	1.11%	1.36%	0.04%	0.24%	1.11%	1.36%	1.57%	1.74%
Volatility	95.6%	85.1%	95.6%	85.1%	95.6%	85.1%	95.6%	85.1%
Expected term (years)	3.50	4.25	0.06	0.75	3.50	4.25	4.25	5.0
Dividend	0	0	0	0	0	0	0	0

For the three and nine months ended May 31, 2011 and 2010, as a result of the marking-to-market of the warrant liability at quarter-end and the day prior to the exercise of warrants subject to warrant liability accounting, the Company recorded losses of approximately \$14.6 million, \$18.6 million, \$4.3 million and \$5.4 million, respectively, in the line item adjustment to fair value of common stock warrants in its condensed consolidated statements of operations. See Note 5 for further discussion on the marking-to-market of the warrant liability.

(11) COMMITMENTS AND CONTINGENCIES

CONTRACTUAL OBLIGATIONS WITH BIOMARIN

Pursuant to the terms of the asset purchase agreement the Company entered into with BioMarin for the purchase of intellectual property related to the Company's receptor-associated protein ("RAP") based technology (including NeuroTrans™), the Company is obligated to make the following milestone payments to BioMarin upon the achievement of the following events:

\$50,000 (paid by the Company in June 2006) within 30 days after the Company receives total aggregate debt or equity financing of at least \$2,500,000;

\$100,000 (paid by the Company in June 2006) within 30 days after the Company receives total aggregate debt or equity financing of at least \$5,000,000;

\$500,000 upon the Company's filing and acceptance of an investigational new drug application for a drug product candidate based on the NeuroTrans™ product candidate;

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

\$2,500,000 upon the Company's successful completion of a Phase 2 human clinical trial for a drug product candidate based on the NeuroTrans™ product candidate;

\$5,000,000 upon on the Company's successful completion of a Phase 3 human clinical trial for a drug product candidate based on the NeuroTrans™ product candidate;

\$12,000,000 within 90 days of the Company's obtaining marketing approval from the FDA or other similar regulatory agencies for a drug product candidate based on the NeuroTrans™ product candidate;

\$5,000,000 within 90 days of the Company's obtaining marketing approval from the FDA or other similar regulatory agencies for a second drug product candidate based on the NeuroTrans™ product candidate;

\$5,000,000 within 60 days after the end of the first calendar year in which the Company's aggregated revenues derived from drug product candidates based on the NeuroTrans™ product candidate exceed \$100,000,000; and

\$20,000,000 within 60 days after the end of the first calendar year in which the Company's aggregated revenues derived from drug product candidates based on the NeuroTrans™ product candidate exceed \$500,000,000.

In addition to these milestone payments, the Company is also obligated to pay BioMarin a royalty at a percentage of the Company's aggregated revenues derived from drug product candidates based on the NeuroTrans™ product candidate. On June 9, 2006, the Company made a milestone payment in the amount of \$150,000 to BioMarin because the Company raised \$5,000,000 in its May 25, 2006 private placement financing. If the Company becomes insolvent or if the Company breaches its asset purchase agreement with BioMarin due to non-payment and the Company does not cure its non-payment within the stated cure period, all of the Company's rights to the RAP technology (including NeuroTrans™) will revert back to BioMarin.

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 ConviviaTM. 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 ConviviaTM, combined with the execution of a formulation agreement to produce the oral formulation of ConviviaTM. 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 ConviviaTM 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.

RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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.

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 above).

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, 58,281 shares of Raptor's common stock valued at \$118,551 and paid \$70,000 in cash bonuses related to Convivia<sup>TM</sup> 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 LICENSE**

As a result of the merger between the Company's clinical subsidiary and Encode, as discussed in Note 9 above, the Encode Securityholders 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 DR Cysteamine, 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 DR Cysteamine 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 million in funding prior to December 18, 2008 (which the Company has

## RAPTOR PHARMACEUTICAL CORP.

(A Development Stage Company)

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

fulfilled by raising \$10 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, 2010 and 2009, the Company has fulfilled by spending approximately \$6.2 million and \$4.1 million, respectively, on such programs) pursuant to the License Agreement. Cumulatively, the Company has expensed \$470,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, Huntington's Disease and NASH. 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.

## 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 April 1, 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. Effective April 1, 2010, the Company's monthly base rent including base operating expenses were \$10,826 and effective April 11, 2011, the Company's monthly base including base operating expenses is \$16,135 with an adjustment for CPI and operating expenses in April 2012. 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 and nine months ended May 31, 2011 and 2010 and the cumulative period from September 8, 2005 (inception) to May 31, 2011, the Company's rent expense was \$53,901, \$154,417, \$38,811, \$106,955 and \$673,348, respectively.

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

Period	Amount
June 1, 2011 to August 31, 2011	\$ 48,406
Fiscal year ending August 31, 2012	196,043
Fiscal year ending August 31, 2013	116,335

## CAPITAL LEASE

In September 2008, the Company leased a photocopier, which is subject to a 39-month lease at \$469 per month. The future lease payments under the capital lease are as follows:

Period	Amount
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June 1, 2011 to August 31, 2011	\$	1,406
September 1, 2011 to December 31, 2011		1,875
Total future capital lease payments		3,281
Less interest		(175)
Total current and long-term capital lease liability	\$	3,106

Interest rate on the capital lease is 17% based on the lessor's implicit rate of return.

CONTRACT/CLINICAL RESEARCH AGREEMENTS

During the three and nine months ended May 31, 2011, the Company maintained several contracts with research and clinical organizations and clinical sites, consultants to research drug pricing in the E.U., develop research assays, and to assist with clinical research for Raptor's cystinosis program.

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

The future commitments pursuant to research agreements are as follows:

Period	Amount
June 1, 2011 to August 31, 2011	\$ 1,391,201
Fiscal year ending August 31, 2012	1,156,370
Fiscal year ending August 31, 2013	289,588

STORAGE AND CLINICAL DISTRIBUTION AGREEMENT

During the three and nine months ended May 31, 2011, the Company maintained an agreement with a company that stores and distributes clinical materials for Raptor's cystinosis and Huntington's Disease trials. The future commitments pursuant to this agreement are as follows:

Period	Amount
June 1, 2011 to August 31, 2011	\$ 22,000
Fiscal year ending August 31, 2012	326,000
Fiscal year ending August 31, 2013	32,000

FORMULATION / MANUFACTURING AGREEMENTS

In April 2008, the Company executed an agreement with a contract manufacturing organization to formulate and manufacture DR Cysteamine for its cystinosis and Huntington's Disease programs. 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 agreement of DR Cysteamine. In July 2010, the Company executed a manufacturing agreement to provide tezampanel study drug for the Company's thrombosis program. The future commitments pursuant to these contracts are as follows:

Period	Amount
June 1, 2011 to August 31, 2011	\$ 1,045,957
Fiscal year ending August 31, 2012	2,792,876
Fiscal year ending August 31, 2013	444,773

(12) 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 (non-alcoholic steatohepatitis) clinical programs and its HepTide<sup>TM</sup> and WntTide<sup>TM</sup> preclinical cancer research programs. The Company was granted an aggregate of approximately \$1.1 million for all five programs of which, as of May 31, 2011, it had received approximately \$874,000. The Company recorded the \$194,000 and \$680,000 of proceeds as a contra-research and development expense in its preclinical and clinical development division,

respectively, during the first two quarters of fiscal 2011. The Company records the contra-expense upon deposit of the grant proceeds. The balance of the award of approximately \$198,000 is expected to be received by the Company in September 2012, pursuant to the government program funding guidelines.

(13) SUBSEQUENT EVENTS

From June 1 through June 22, 2011 (the expiration date of the Series B Warrants), the Company issued 150,000 shares of the Company's common stock pursuant to the exercise of Series A Warrants for \$367,500 in gross proceeds to the Company and 1,025,000 shares of the Company's common stock pursuant to the exercise of the Series B Warrants for \$2,511,250 in gross proceeds to the Company; such Series A and Series B Warrants were issued in connection with the Company's registered direct offering completed in December 2009 discussed in Note 9. The Company also issued 74,951 shares of the Company's common stock pursuant to the exercise of Placement Agent Warrants issued to the Placement Agent in the December 2009 registered direct offering for \$187,378 in gross proceeds to the Company. Total aggregate proceeds from warrant exercises related to the December 2009 registered direct offering from June 1 through June 22, 2011 was approximately \$3.1 million.

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," "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;
- 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.

## 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, 2011, 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., Raptor Discoveries Inc., or Raptor Discoveries, Raptor Therapeutics Inc., or Raptor Therapeutics, 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

We believe that we are building a balanced pipeline of drug candidates that may expand the reach and benefit of existing therapeutics. 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 Asian business development partners but are not actively developing, and we have three preclinical product candidates we are developing, two of which are based upon our proprietary drug-targeting platforms.

### Clinical Development Programs

Our three active clinical development programs are based on an existing therapeutic that we are reformulating for potential improvement in safety and/or efficacy and for application in new disease indications. These clinical development programs include the following:

- DR Cysteamine for the potential treatment of nephropathic cystinosis, or cystinosis, a rare genetic disorder;
- DR Cysteamine for the potential treatment of non-alcoholic steatohepatitis, or NASH, a metabolic disorder of the liver; and
- DR Cysteamine for the potential treatment of Huntington's Disease, or HD, an inherited neurodegenerative disorder.

### Other Clinical-Stage Product Candidates

Our other clinical-stage product candidates include:

- Convivia™ for the potential management of acetaldehyde toxicity due to alcohol consumption by individuals with aldehyde dehydrogenase, or ALDH2 deficiency, an inherited metabolic disorder; and
- Tezampanel, a glutamate receptor antagonist for the potential treatment of thrombosis disorder.

### Preclinical Product Candidates

Our preclinical platforms consist of targeted therapeutics, which we are developing for the potential treatment of multiple indications, including liver diseases, neurodegenerative diseases and breast cancer. 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 (licensed to Hoffman – La Roche).
- 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 activities based upon our DR Cysteamine clinical and pre-commercial programs, our tezampanel anti-thrombotic program and continued development of our preclinical product candidates. We also plan to seek additional Asian business development partners for our Convivia™ product candidate. We may also develop future in-licensed technologies and acquired technologies.

A brief summary of our primary objectives in the next 12 months for our research and development activities is provided below. We will need to raise additional funds to pursue our primary objectives during the next 12 months. There can be no assurances that our research and development activities will be successful or that we will obtain additional funding. In addition, if we do not raise additional funds, we may not be able to continue as a going concern. . If we cannot raise sufficient additional funds, programs and activities may have to be delayed , deferred, or otherwise managed at low levels of expenditures.

#### Clinical Development Programs

We develop clinical-stage drug product candidates which are: internally discovered therapeutic candidates based on our novel drug delivery platforms and in-licensed or purchased clinical-stage products which may be new chemical entities in mid-to-late stage clinical development, currently approved drugs with potential efficacy in additional indications, and treatments that we could repurpose or reformulate as potentially more effective or convenient treatments for a drug's currently approved indications.

#### Lead Clinical Development Program: Development of DR Cysteamine for the Potential Treatment of Nephropathic Cystinosis or Cystinosis

Our DR Cysteamine product candidate is a proprietary delayed-release, enteric-coated microbead formulation of cysteamine bitartrate contained in a gelatin capsule. We are investigating DR Cysteamine for the potential treatment of cystinosis.

Immediate-release cysteamine bitartrate, a cystine-depleting agent, is currently the only U.S. Food and Drug Administration, or FDA, and the European Medicines Agency, or EMA, approved drug to treat cystinosis, a rare genetic disease. Immediate-release cysteamine has been reported to be effective at preventing or delaying kidney failure and other serious health problems in cystinosis patients. However, we believe that patient compliance is challenging due to the requirement for every six-hour dosing and gastrointestinal side effects. Our DR Cysteamine for the potential treatment of cystinosis is designed to mitigate some of these difficulties. It is expected to be dosed twice daily, compared to the current every-six-hour dosing schedule. In addition, DR Cysteamine is designed to pass through the stomach and deliver the drug directly to the small intestine, where it is more easily absorbed into the bloodstream and may result in fewer gastrointestinal side effects. The EMA and FDA granted orphan drug designation for DR Cysteamine for the treatment of cystinosis in 2010 and 2006, respectively.

In June 2009, we commenced our Phase 2b clinical trial of DR Cysteamine in cystinosis, in which we enrolled nine cystinosis patients with histories of compliance using the currently available immediate-release form of cysteamine bitartrate. The clinical trial, which was conducted at the University of California at San Diego, or UCSD, evaluated safety, tolerability, pharmacokinetics and pharmacodynamics of a single dose of DR Cysteamine in patients. In November 2009, we released the data from the study which indicated improved tolerability and the potential to reduce total daily dosage and administration frequency compared to immediate-release cysteamine bitartrate.

On June 28, 2010, we commenced our Phase 3 clinical trial, designed as a multi-center, randomized, crossover, outpatient study of the safety, tolerability, pharmacokinetics, or PK, and pharmacodynamics, or PD, of every 12-hour DR Cysteamine compared to immediate-release, every 6-hour cysteamine bitartrate in cystinosis patients. The design of our Phase 3 clinical trial is a result of discussions with the FDA under a Special Protocol Assessment, or SPA, process by which the FDA provided significant guidance on trial protocol design, clinical endpoints, and statistical analyses. The SPA process resulted in concurrence on trial design but due to time constraints, we started the Phase 3 study without a formal SPA agreement in place. The primary endpoint of our study is the steady-state white blood cell, or WBC, cystine levels of patients taking DR Cysteamine compared to immediate-release cysteamine bitartrate.



Secondary endpoints are the safety and tolerability of DR Cysteamine and the comparability of steady-state PK of DR Cysteamine and immediate-release cysteamine bitartrate in cystinosis patients. The Phase 3 trial was conducted at nine sites in North America and Europe.

As of June 3, 2011, we completed our Phase 3 clinical trial with a total of 41 patients, of which 40 patients voluntarily enrolled in an extension study in which patients continue on our DR Cysteamine treatment for up to two years. We anticipate that our top-line Phase 3 clinical trial data will be available by the end of July 2011 and we are in the pre-commercial planning stage in anticipation of potential positive trial outcomes and potential drug approval in 2012. If DR Cysteamine is approved by the FDA and EMA, we are currently planning to commercialize DR Cysteamine in the U.S. and E.U. by ourselves. However, we may enter into marketing partnerships for certain markets outside of the U.S. and E.U.

#### Development of DR Cysteamine for the Potential Treatment of Non-Alcoholic Steatohepatitis or NASH

In October 2008, we commenced a clinical trial in collaboration with UCSD to investigate a prototype formulation of DR Cysteamine for the treatment of NASH in juvenile patients. In May 2010, we presented positive Phase 2a clinical trial results from our pilot study of delayed-release cysteamine bitartrate in 11 adolescent patients with NASH, a progressive form of liver disease believed to affect 5% to 11% of the U.S. population. The results were presented at the Digestive Disease Week 2010 conference in New Orleans, Louisiana on May 2, 2010. Our open-label Phase 2a clinical trial was conducted under a collaboration agreement with UCSD at UCSD's General Clinical Research Center. Eligible patients with baseline levels of the

liver enzymes alanine transaminase, or ALT, and aspartate aminotransferase, or AST, that were at least twice that of normal levels, were enrolled to receive twice-daily, escalating oral doses of up to 1,000 mg of delayed-release cysteamine bitartrate (a prototype of our DR Cysteamine) for six months, followed by a six-month post-treatment monitoring period. Patients showed a marked decline in ALT levels during the treatment period, with 7 of 11 patients achieving a greater than 50% reduction and 6 of 11 reduced to within normal range. AST levels also saw significant improvements 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. Body Mass Index, or BMI, did not change significantly during both the treatment and post-treatment phases. Delayed-release cysteamine bitartrate demonstrated a strong, 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.

There are no currently approved drug therapies for NASH, and patients are advised to make lifestyle changes such as diet, exercise and weight reduction to manage the disease. DR Cysteamine may provide a potential treatment option for patients with NASH.

Although NASH is most common in insulin-resistant obese adults with diabetes and abnormal serum lipid profiles, its prevalence is increasing among juveniles as obesity rates rise within this patient population. Although most patients are asymptomatic and feel healthy, NASH causes decreased liver function and can lead to cirrhosis, liver failure and end-stage liver disease.

We are currently working with our clinical trial material manufacturer to provide an enteric coated compressed tablet formulation of DR Cysteamine for our next potential clinical trial in NASH and are preparing an IND submission in 2011 in anticipation of such clinical trial. Although it is our intention to continue the clinical development of DR Cysteamine in NASH, we are currently not funded for, and therefore do not have a timetable for, the initiation of a Phase 2b clinical trial. We are in early stages of discussions to co-develop or partner the clinical development of DR Cysteamine in NASH.

#### Development of DR Cysteamine for the Potential Treatment of Huntington's Disease or HD

Huntington's Disease, or HD, is a fatal, inherited degenerative neurological disease affecting about 30,000 people in the U.S. and a comparable number of people in Europe. We are not aware of any treatment for HD other than therapeutics that minimize symptoms such as the uncontrollable movements and mood swings resulting from HD. We are collaborating with a French governmental institution, CHU d' Angers, on a Phase 2 clinical trial investigating DR Cysteamine in HD patients, which began in October 2010. We are providing the clinical trial materials for the study, which is sponsored by CHU d' Angers and funded in part by a grant from the French government. Eight clinical sites in France are being set up by CHU d' Angers for a 96 patient, placebo-controlled, 18-month trial, followed by an open-label trial with all placebo patients rolling onto DR Cysteamine and all non-placebo patients continuing on DR Cysteamine for up to another 18 months. The primary end point of the trial will be based upon the Unified Huntington's Disease Rating Scale, or UHDRS. We were granted Orphan Drug Designation in the U.S. by the FDA for cysteamine as a potential treatment for HD in 2008 and are in the process of applying for Orphan Drug Designation in the E.U.

In June 2010, we acquired an exclusive worldwide license to intellectual property related to the potential treatment of Huntington's Disease from the Weizmann Institute of Science in Israel and Niigata University in Japan. The Weizmann and Niigata patents cover the use of transglutaminase inhibitors, a class of molecules chemically similar to

cysteamine, in the potential treatment of Huntington's Disease and other neurological disorders. These patents add to our portfolio of intellectual property related to our programs utilizing DR Cysteamine.

#### Other Clinical-Stage Product Candidates

##### Convivia™ for Liver Aldehyde Dehydrogenase, or ALDH2, Deficiency

Convivia™ is our proprietary oral formulation of 4-methylpyrazole, or 4-MP, intended for the potential treatment of acetaldehyde toxicity resulting from alcohol consumption in individuals with ALDH2 deficiency, which is an inherited disorder of the body's ability to breakdown ethanol, commonly referred to as alcohol intolerance. 4-MP is presently marketed in the U.S. and E.U. in an intravenous form as an anti-toxin. Convivia™ is designed to lower systemic levels of acetaldehyde (a carcinogen) and reduce symptoms, such as tachycardia and flushing, associated with alcohol consumption by ALDH2-deficient individuals.

Convivia™ is a capsule designed to be taken approximately 30 minutes prior to consuming an alcoholic beverage.

In 2008, we completed a Phase 2a dose escalation clinical trial of oral 4-MP with ethanol in ALDH2 deficient patients. The study results demonstrated that the active ingredient in Convivia™ significantly reduced heart palpitations (tachycardia), which are commonly experienced by ALDH2 deficient people who drink, at all dose levels tested. The study also found that the 4-MP significantly 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. We believe that this subset represents approximately one-third of East Asian populations.

In June 2010, we entered into an exclusive agreement with Uni Pharma Co., Ltd., or Uni Pharma, to commercialize Convivia™ in Taiwan. Under terms of the agreement, we will grant to Uni Pharma an exclusive license under all relevant patent applications, trademarks and future patents controlled by us to market Convivia™ in Taiwan, with an option to expand the license to South Korea under the same terms. Uni Pharma will register Convivia™ for drug licensure for existing indications and will conduct a clinical trial and register Convivia™ for acetaldehyde toxicity resulting from ALDH2 deficiency. Uni Pharma will be responsible for marketing and sales activities for the commercialization of Convivia™ in the markets covered under the license agreement. We continue to seek potential partners in other Asian countries to continue clinical development of Convivia™ in those countries.

#### Tezampanel for the Potential Treatment of Thrombotic Disorder

Thrombosis is a major cause of morbidity and mortality in the United States. In addition to deep vein thrombosis, or DVT, and pulmonary embolus, or PE, 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, potentially leading to 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 particularly potent inhibitor of the AMPA receptor is tezampanel, a molecule developed by Eli Lilly and licensed to us. Tezampanel has been shown to inhibit human platelet activation, subsequent human platelet aggregation, and thrombosis in a preclinical model. The inventors of this novel technology are Dr. Charles Lowenstein and Dr. Craig Morrell, both are currently at the University of Rochester in New York. A patent addressing the use of glutamate receptor antagonists as anti-platelet agents was assigned to Johns Hopkins University and exclusively licensed to us.

Tezampanel has been tested in Phase I clinical trials. The drug candidate 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. In collaboration with Dr. Lowenstein and Dr. Morrell, we are preparing for a Phase I clinical trial in healthy volunteers, subject to regulatory reviews and approval, currently

anticipated to commence by the end of 2011, to determine the initial indications of pharmacological activity of tezampanel in blocking platelet activation and aggregation.

#### Preclinical Product Candidates

We are also developing a drug-targeting platform based on the proprietary use of RAP and Mesd. We believe that these proteins may have therapeutic applications in cancer, infectious diseases and neurodegenerative diseases, among others.

These applications are based on the assumption that our targeting molecules can be engineered to bind to a selective subset of receptors with restricted tissue distribution under particular conditions of administration. We believe these selective tissue distributions can be used to deliver drugs to the liver or to other tissues, such as the brain.

In addition to selectively transporting drugs to specific tissues, selective receptor binding constitutes a means by which receptor function might be specifically controlled, either through modulating its binding capacity or its prevalence on the cell surface. Mesd is being engineered for this latter application.

#### HepTide™ for Hepatocellular Carcinoma or HCC and Other Liver Diseases

Drugs currently used to treat primary liver cancer are often toxic to other organs and tissues. We believe that the pharmacokinetic behavior of RAP (i.e., the determination of the fate or disposition of RAP once administered to a living organism) may diminish the non-target toxicity and increase the on-target efficacy of attached therapeutics.

In preclinical studies of our radio-labeled HepTide™ (a variant of RAP), HepTide™, our proprietary drug-targeting peptide was shown to distribute predominately to the liver. Radio-labeled HepTide™, which was tested in a preclinical research model of HCC at the National Research Council in Winnipeg, Manitoba, Canada, showed 4.5 times more delivery to the liver than the radio-labeled control. Another study of radio-labeled HepTide™ in a non-HCC preclinical model, showed 7 times more delivery to the liver than the radio-labeled control, with significantly smaller amounts of radio-labeled HepTide™ delivery to other tissues and organs.

HCC is caused by the malignant transformation of hepatocytes, epithelial cells lining the vascular sinusoids of the liver, or their progenitors. HepTide™ has been shown to bind to lipoprotein receptor-related protein, or LRP1, receptors on hepatocytes. We believe that the pharmacokinetics and systemic toxicity of a number of potent anti-tumor agents may be controlled in this way.

There are additional factors that favor the suitability of RAP as an HCC targeting agent:

- RAP is captured by hepatocytes with efficiency, primarily on first-pass.
- Late-stage HCC is perfused exclusively by the hepatic artery, while the majority of the liver is primarily perfused through the portal vein.

Studies have shown that the RAP receptor, LRP1, is well expressed on human HCC and under-expressed on non-cancerous, but otherwise diseased, hepatocytes. Also, LRP1 expression is maintained on metastasized HCC. These factors will favor delivery of RAP peptide-conjugated anti-tumor agents to tumor cells, whether in the liver or at metastasized sites.

We are evaluating conjugates between HepTide™ and other molecules for testing in vitro and in appropriate preclinical models for the potential treatment of HCC and other liver diseases.

#### NeuroTrans™ for the Potential Treatment of Diseases Affecting the Brain

Hundreds of known genetic and neurodegenerative diseases affect the brain. Drugs often have difficulty reaching these disease-affected areas because the brain has evolved a protective barrier, commonly referred to as the blood-brain barrier.

Part of the solution to the medical problem of neurodegenerative diseases is the creation of effective brain targeting and delivery technologies. One of the most obvious ways of delivering therapeutics to the brain is via the brain's extensive vascular network. Treating these diseases by delivering therapeutics into the brain in a minimally invasive way, including through a natural receptor mediated transport mechanism called transcytosis, is a vision shared by many researchers and clinicians in the neuroscience and neuromedical fields.

NeuroTrans™ is our proprietary RAP-based technology program to research the delivery of therapeutics across the blood-brain barrier. We believe our NeuroTrans™ platform may provide therapies that will be safer, less intrusive and more effective than current approaches in treating a wide variety of brain disorders.

In preclinical studies, NeuroTrans™ has been conjugated to a variety of protein drugs, including enzymes and growth factors, without interfering with the function of either fusion partner. Studies indicate that radio-labeled NeuroTrans™ may be transcytosed across the blood-brain barrier and that fusions between NeuroTrans™ and therapeutic proteins may be manufactured economically. Experiments conducted in collaboration with Stanford University in 2008 support the NeuroTrans™ peptide's ability to enhance the transport of cargo molecules into the cells that line the blood-brain barrier.

In June 2009, we entered into a collaboration and licensing agreement with F. Hoffman — La Roche Ltd. and Hoffman—La Roche Inc., or Roche, to evaluate therapeutic delivery across the blood-brain barrier utilizing NeuroTrans™. Under the terms of the agreement, Roche has funded studies of select molecules attached to NeuroTrans™. The agreement provides Roche with an exclusive worldwide license to NeuroTrans™ for use in the delivery of diagnostic and therapeutic molecules across the blood-brain barrier. Roche's and our scientists are actively collaborating on the project. We have received an initial upfront payment for the collaboration to cover our portion of the initial studies, and may earn development milestone payments (which get passed through to BioMarin) and royalties in exchange for the licensing of NeuroTrans™ to Roche.

#### WntTide™ for the Potential Treatment of Cancer

Human Mesd is a natural inhibitor of the receptor LRP6. LRP6 has recently been shown to play a role in the progression of some breast tumors. Studies in the laboratory of Professor Guojun Bu, one of our scientific advisors, formerly at the Washington University in St. Louis Medical School support the potential of Mesd and related peptides to target these tumors. These molecules and applications are licensed to us from Washington University.

WntTide™ is our proprietary, Mesd-based peptide that we are developing as a potential therapeutic to inhibit the growth and metastasis of tumors over-expressing LRP5 or LRP6. We have licensed the use of Mesd from Washington University for the potential treatment of cancer and bone density disorders.

In April 2009, Washington University conducted a preclinical study of WntTide™ in a breast cancer model which showed tumor inhibition. The results of this study were presented at the 2nd Annual Wnt Conference in Washington, D.C. in June 2009 and have been published in the peer-reviewed publication, the Proceedings of the National Academy of Sciences, on March 1, 2010. The paper, titled "LRP6 Overexpression Defines a Class of Breast Cancer Subtype and Is a Target for Therapy," presented results that support the potential efficacy of WntTide™ as a targeted treatment for triple-negative breast cancers, a particularly aggressive and difficult-to-treat indication for recurrent and metastatic disease. Abnormal Wnt activation, found in 40% to 60% of breast cancers, is often associated with triple-negative breast cancers. We are currently evaluating WntTide™ in a preclinical breast cancer model to inhibit the Wnt-signaling pathway designed to block cancers dependent upon signaling through LRP6, as well as other IND enabling studies.

#### Other Development Areas

##### Securing Additional and Complementary Technology Licenses from Others

We plan to establish additional research collaborations with prominent universities and research labs currently working on the development of potential targeting molecules, and to secure licenses from these universities and labs for technology resulting from the collaboration. No assurances can be made regarding our ability to establish such collaborations over the next 12 months, or at all. We intend to focus our in-licensing and product candidate acquisition activities on identifying complementary therapeutics, therapeutic platforms that offer a number of therapeutic targets, and clinical-stage therapeutics based on existing approved drugs in order to create proprietary reformulations to improve safety and efficacy or to expand such drugs' clinical indications through additional clinical trials. We may obtain these products through collaborations, joint ventures or through merger and/or acquisitions with other biotechnology/pharmaceutical companies.

#### Strategic Acquisitions

Reverse Merger with Raptor Pharmaceuticals Corp.



In July 2009, we, and our then wholly-owned subsidiary ECP Acquisition, Inc., a Delaware corporation, or merger sub, entered into an Agreement and Plan of Merger and Reorganization, or the 2009 Merger Agreement, with Raptor Pharmaceuticals Corp., a Delaware corporation. On September 29, 2009, on the terms and subject to the conditions set forth in the 2009 Merger Agreement, merger sub was merged with and into Raptor Pharmaceuticals Corp. and Raptor Pharmaceuticals Corp. survived such merger as our wholly-owned subsidiary. This merger is referred to herein as the 2009 Merger. Immediately prior to the 2009 Merger and in connection therewith, we effected a 1-for-17 reverse stock split of our common stock and changed our corporate name to “Raptor Pharmaceutical Corp.”

As of immediately following the effective time of the 2009 Merger, Raptor Pharmaceuticals Corp.’s stockholders (as of immediately prior to the 2009 Merger) owned approximately 95% of our outstanding common stock and our stockholders owned approximately 5% of our outstanding common stock, in each case without taking into account any of our or Raptor Pharmaceuticals Corp.’s shares of common stock, respectively, that were issuable pursuant to outstanding options or warrants of ours or Raptor Pharmaceuticals Corp., respectively, outstanding as of the effective time of the 2009 Merger. Although Raptor Pharmaceuticals Corp. became our wholly-owned subsidiary, Raptor Pharmaceuticals Corp. was the “accounting acquirer” in the

2009 Merger and its board of directors and officers manage and operate the combined company. Our common stock currently trades on the NASDAQ Capital Market under the ticker symbol, "RPTP."

#### Purchase of Convivia™

In October 2007, prior to the 2009 Merger, Raptor Pharmaceuticals Corp. purchased certain assets of Convivia, Inc., or Convivia, including intellectual property, know-how and research reports related to a product candidate targeting liver ALDH2 deficiency, a genetic metabolic disorder. Raptor Pharmaceuticals Corp. hired Convivia's chief executive officer and founder, Thomas E. (Ted) Daley, as the President of its clinical development division. In exchange for the assets related to the ALDH2 deficiency program, what we now call Convivia™, RPC issued to Convivia 46,625 shares of our common stock, an additional 46,625 shares of our common stock to a third party in settlement of a convertible loan between the third party and Convivia, and another 8,742 shares of our common stock in settlement of other obligations of Convivia. Mr. Daley, as the former sole stockholder of Convivia, may earn additional shares of our common stock based on certain triggering events or milestones related to the development of the Convivia assets. 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 as a result of the milestone of our execution of a formulation agreement for manufacturing Convivia™ with Patheon. In March 2008, Raptor Pharmaceuticals Corp. issued to Mr. Daley 23,312 shares of our common stock pursuant to the Convivia purchase agreement as a result of the milestone of our execution of an agreement to supply us with the active pharmaceutical ingredient for Convivia™ and two \$10,000 cash bonuses pursuant to his employment agreement for reaching his six-month and one-year employment anniversaries. In October 2008, RPC issued to Mr. Daley 23,312 shares of our common stock valued at \$27,000 and a \$30,000 cash bonus as a result of fulfilling a clinical milestone. In July 2010, we issued 11,656 shares of our 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.

#### Purchase of DR Cysteamine

In December 2007, prior to the 2009 Merger, through a merger between Encode Pharmaceuticals, Inc., or Encode, and Raptor Therapeutics, RPC purchased certain assets, including the clinical development and commercial rights to DR Cysteamine. Under the terms of and subject to the conditions set forth in the merger agreement, RPC issued 802,946 shares of its common stock to the stockholders of Encode, or Encode Stockholders, options, or Encode Options, to purchase up to, in the aggregate, 83,325 shares of its common stock to the optionholders of Encode, or Encode Optionholders, and warrants, or Encode Warrants, to purchase up to, in the aggregate, 256,034 shares of its common stock to the warrantholders of Encode, or Encode Warrantholders, and together with the Encode Stockholders and Encode Optionholders, referred to herein collectively as the Encode Securityholders, as of the date of such agreement. The Encode Securityholders are eligible to receive up to an additional 559,496 shares of our common stock, Encode Options and Encode Warrants to purchase our common stock in the aggregate based on certain triggering events related to regulatory approval of DR Cysteamine, an Encode product program, if completed within the five year anniversary date of the merger agreement.

As a result of the Encode merger, we received the exclusive worldwide license to DR Cysteamine, referred to herein as the License Agreement, developed by clinical scientists at the UCSD School of Medicine. In consideration of the grant of the license, 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, among other things, to spend annually at least \$200,000 for the development of products (which we satisfied, as of August 31, 2010 and 2009 by spending approximately \$6.2 million and \$4.1 million, respectively, on such programs) pursuant to the License Agreement. Cumulatively, we have expensed \$470,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, Huntington's Disease and NASH. 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.

#### Application of Critical Accounting Policies

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

-37-

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

#### Functional Currency

Our consolidated functional currency is the U.S. dollar. Raptor Pharmaceuticals Europe B.V. or BV, our European subsidiary, records its functional currency as the European Euro. At quarter-end the 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 between the beginning and end of the reporting period. The 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. We have not experienced any losses on these investments. Restricted cash represents compensating balances required by our U.S. and European banks as collateral for credit cards.

#### Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine, to the RAP technology, to an out-license acquired in the 2009 Merger and the rights to tezampanel, also acquired in the 2009 Merger. The intangible assets related to DR Cysteamine and the RAP technology 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.

#### Goodwill

Goodwill represents the excess of the value of the purchase consideration over the identifiable assets acquired in the 2009 Merger. Goodwill will be 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. We have not identified any such impairment losses to date.

### Common Stock Warrant Liabilities

The warrants issued in our 2010 private placement, or 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 our Company. This provision requires these warrants to be classified as liabilities and will be marked to market at each period end commencing on August 31, 2010. The warrants we issued in our December 2009 direct offering, or the Direct Offering, contain a conditional obligation that may require us to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under the Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 480, Distinguishing Liabilities from Equity, or ASC 480, a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, we have classified the warrants issued in the Direct Offering as liabilities and will mark them to fair value at each period end. The warrants to purchase our common stock are re-measured at the end of every reporting period with the change in value reported in our condensed consolidated statements of operations. Warrants which are recorded as liabilities that are exercised are re-measured and marked to market the day prior to exercise and such value is recorded as adjustment to fair value of common stock warrants with an offset to additional paid in capital.

### 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.

### Research and Development

We are an early development stage 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, 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. We review each product candidate acquisition to determine the existence of in-process research and development.

### 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's term is ten years and 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 a replenishment 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 replenishment of 6,000,000 shares. The April 7, 2011 replenishment added 1,629,516 shares available for grant 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 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 718 (previously listed as Statement of Financial Accounting Standards, or SFAS, No. 123 (revised 2004), Share-Based Payment), 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 718 (previously listed as Staff Accounting Bulletin, or SAB, No. 107, or SAB 107), which offers guidance for what was previously referred to as SFAS 123(R). ASC 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 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 718 include valuation models, expected volatility and expected term.

For the three months ended May 31, 2011, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 2.38%; 6 year expected life; 96% 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 seven and five years (average); the expected life of six 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 the company is 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 718, the compensation expense recorded in future periods may differ significantly from what was recorded in the current period. See Note 8 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, or ASC 505-50 (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.





## Results of Operations

Three months ended May 31, 2011 and 2010

## General and Administrative Expenses

General and administrative expenses include finance, executive and pre-commercial compensation and benefits, corporate costs, such as legal 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 and pre-commercial consulting and allocated human resources and facilities costs. General and administrative expenses for the three months ended May 31, 2011 increased by approximately \$814,000 compared to the same period of the prior fiscal year. The increase was primarily due to:

Reason for Variance	Variance in \$ Thousands
Consulting in Q3 2011 related to commercial planning for potential launch of DR Cysteamine for cystinosis and business development consulting	310
Amortization of new stock option grants in Q1 2011 to employees and directors (non-cash)	221
Decrease in executive costs allocated to R&D due to lower Q3 2011 executive costs versus Q3 2010	166
Increase in Delaware franchise taxes due to higher book value per share tax basis	123
Patent fees in Q3 2011 higher due to the increase in size of patent portfolio in clinical compared to Q3 2010 partially offset by decreased preclinical patent activity	56
Q3 2011 401(K) match not given in Q3 2010	44
Recruiting fee for VP, Quality Operations	35
Office expenses due to new employees commencing July 2010	31
Increase in travel in Q3 2011 due to attendance at more conferences and the additional travel of new VP, Commercial Operations	31
Tax preparation services incurred earlier than in FY 2010 and increase in audit fees	30
Increase in human resources costs allocated to R&D due to increase in R&D headcount	(82)
Decrease in bonuses due to March 2010 bonuses not repeated in Q3 2011	(107)
Decrease in legal costs due to S-1 preparation in Q3 2010 not repeated in Q3 2011	(128)
Other various, net	84
General and Administrative variance three months ended May 31, 2011 vs. May 31, 2010	814



## 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, 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.

Research and development expenses for the three months ended May 31, 2011 increased by approximately \$1.7 million over the same period of the prior year primarily due to:

Reason for Variance	Variance in \$ Thousands
DR Cysteamine Phase 3 and extension trial clinical study costs - trial commenced in June 2010	1,850
Reduction of R&D consulting due to the hiring of a clinical operations director in March 2010 and regulatory senior manager in July 2010	(164)
Decrease in executive costs allocated to R&D due to lower Q3 2011 executive costs versus Q3 2010	(166)
Other various, net	205
Research and Development variance three months ended May 31, 2011 vs. May 31, 2010	1,725

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

Major Program (stage of development)	Estimated next 12 months	Cumulative through May 31, 2011	Three months ended May 31,	
			2011	2010
DR Cysteamine – All				
Indications (clinical)	11.1	18.8	3.0	1.3
Convivia <sup>TM</sup> (clinical)	(0.1)	2.3	-	-
HepTide <sup>TM</sup> (preclinical)	0.1	1.5	-	-
NeuroTrans <sup>TM</sup> (preclinical)	-	0.4	-	0.1
WntTide <sup>TM</sup> (preclinical)	0.3	0.3	-	-
Minor or Inactive Programs	0.2	1.0	-	-
R & D Personnel and Other Costs Not Allocated to Programs	4.6	10.2	0.9	0.8
Total Research & Development Expenses	16.2	34.5	3.9	2.2



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

Major Program (stage of development)	Estimated next 12 months	Cumulative through May 31, 2011	Three months ended May 31,	
			2011	2010
DR Cysteamine – All Indications (clinical)	4.65	0.93	0.43	0.03
Convivia™ (clinical)	0.05	0.26	0.02	0.02
HepTide™ (preclinical)	0.05	0.34	0.00	0.01
NeuroTrans™ (preclinical)	0.05	0.22	0.00	0.02
WntTide™ (preclinical)	0.06	0.17	0.01	0.02

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 DR Cysteamine for the potential treatment of cystinosis (\$302,000, \$364,000 and \$4.5 million for the three months ended May 31, 2011, the cumulative period from inception to May 31, 2011 and estimated for the next 12 months).

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 for and beyond the next 12 months. In addition, the timing and costs of development of our programs beyond the next 12 months is highly uncertain and difficult to estimate. See the section titled “Risk Factors that may Affect Future Results” of this Quarterly Report on Form 10-Q for further discussion about the risks and uncertainties pertaining to drug development.

#### Current Status of Major Programs

Please refer to the subsection titled, “Future Activities” 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, DR Cysteamine is being developed in cystinosis, NASH and HD. In November 2009, we released data from our Phase 2b clinical trial and in June 2010, we completed our Phase 3 clinical trial to study DR Cysteamine in cystinosis patients. We anticipate release of the top-line Phase 3 data by the end of July 2011 and we are in the pre-commercial planning stage in anticipation of potential positive trial outcomes and potential drug approval in 2012. In May 2010, we presented the data from our NASH Phase 2a clinical trial and are reformulating the drug product candidate for a potential Phase 2b trial in 2011. In October 2010, our collaborator commenced a Phase 2 clinical trial of DR Cysteamine in HD patients. By the end of 2011, we anticipate that our collaborator at Rochester University will commence a Phase 1 clinical trial of tezampanel for the potential treatment of thrombotic disorder.

Our Convivia™ product candidate completed its initial clinical study in 2008 and in June 2010, we licensed Convivia™ to Uni Pharma for further clinical development in Taiwan, with an option to develop Convivia™ in South Korea. We continue to seek other potential partners for Convivia™ in other Asian countries where its potential market exists. NeuroTrans™ is currently being studied under a collaboration agreement with Roche.

HepTide™ will be undergoing further preclinical proof of concept studies and WntTide™ is being considered for potential out-licensing for further development. All preclinical product candidates will require further study prior to potentially moving into a clinical phase of development.

Interest Income

Interest income for the three months ended May 31, 2011 and 2010 were nominal.

Interest Expense

Interest expense for the three months ended May 31, 2011 and 2010 were nominal.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of approximately \$(14.6) million in Q3 2011 versus a loss of approximately \$(4.3) million in Q3 2010, an increase in loss of approximately \$10.3 million, due to the increase in stock price of our common stock during the three months ended May 31, 2011 versus the three months ended May 31, 2010. These losses are non-cash.