

Raptor Pharmaceutical Corp  
Form 10-Q  
April 09, 2010

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

or

For the quarterly period ended February 28, 2010  
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 22,580,365 shares of the registrant's common stock, \$.001 par value per share, outstanding at April 4, 2010.

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RAPTOR PHARMACEUTICAL CORP.

FORM 10-Q FOR THE QUARTER ENDED FEBRUARY 28, 2010

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## PART I – FINANCIAL INFORMATION

## Item 1. Financial Statements.

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

| ASSETS   | February 28, 2010<br>(unaudited) | August 31, 2009<br>(1) |
|--|----------------------------------|------------------------|
| Current assets:  |                                  |                        |
| Cash and cash equivalents  | \$ 4,572,919                     | \$ 3,701,787           |
| Prepaid expenses and other   | 149,234                          | 107,054                |
| Total current assets   | 4,722,153                        | 3,808,841              |
| Intangible assets, net   | 3,589,292                        | 2,524,792              |
| Goodwill   | 3,275,403                        | -                      |
| Fixed assets, net  | 113,699                          | 144,735                |
| Deposits   | 100,206                          | 100,206                |
| Total assets   | \$ 11,800,753                    | \$ 6,578,574           |
| <b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>  |                                  |                        |
| Liabilities  |                                  |                        |
| Current liabilities:   |                                  |                        |
| Accounts payable   | \$ 949,749                       | \$ 613,577             |
| Accrued liabilities  | 520,013                          | 451,243                |
| Common stock warrant liability   | 2,959,400                        | -                      |
| Deferred rent  | 1,097                            | -                      |
| Capital lease liability – current  | 4,475                            | 4,117                  |
| Total current liabilities  | 4,434,734                        | 1,068,937              |
| Capital lease liability - long-term  | 4,345                            | 6,676                  |
| Total liabilities  | 4,439,079                        | 1,075,613              |
| Commitments and contingencies  |                                  |                        |
| Stockholders' equity:  |                                  |                        |
| 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 22,580,365 and 17,857,555 shares issued and outstanding as at February 28, 2010 and August 31, 2009, respectively | 22,580                           | 17,858                 |

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|  |               |              |
|--|---------------|--------------|
| Additional paid-in capital                   | 36,337,280    | 27,364,286   |
| Deficit accumulated during development stage | (28,998,186)  | (21,879,183) |
| Total stockholders' equity                   | 7,361,674     | 5,502,961    |
| Total liabilities and stockholders' equity   | \$ 11,800,753 | \$ 6,578,574 |

(1) Derived from the Company's audited consolidated financial statements as of August 31, 2009.

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

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

|   | For the three month periods from         |  |
|---|--|--|
|   | December 1, 2009 to<br>February 28, 2010 | December 1, 2008 to<br>February 28, 2009 |
| Revenues:   | \$ -                                     | \$ -                                     |
| Operating expenses:                                     |  |  |
| General and administrative                              | 981,272                                  | 604,574                                  |
| Research and development                                | 2,162,004                                | 1,653,854                                |
| Total operating expenses                                | 3,143,276                                | 2,258,428                                |
| Loss from operations                                    | (3,143,276)                              | (2,258,428)                              |
| Interest income   | 7,145                                    | 8,185                                    |
| Interest expense  | (811)                                    | (595)                                    |
| Adjustment to fair value of<br>common                   |  |  |
| stock warrants  | (1,043,389)                              | -  |
| Net loss  | \$ (4,180,331)                           | \$ (2,250,838)                           |
| Net loss per share:                                     |  |  |
| Basic and diluted                                       | \$ (0.19)                                | \$ (0.16)                                |
| Weighted average shares<br>outstanding used to compute: |  |  |
| Basic and diluted                                       | 21,622,108                               | 14,087,658                               |

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



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

|  | For the six month periods from               |   | For the cumulative period  |
|--|--|---|--|
|  | September 1,<br>2009 to February<br>28, 2010 | September 1, 2008 to<br>February 28, 2009 | from<br>September 8, 2005<br>(inception) to<br>February 28, 2010 |
| Revenues:  | \$ -   | \$ -                                      | \$ -   |
| Operating expenses:                                  |  |   |  |
| General and administrative                           | 1,988,848                                    | 1,264,263                                 | 8,945,088  |
| Research and development                             | 4,095,339                                    | 3,474,253                                 | 18,969,623   |
| In-process research and dev.                         | -  | -   | 240,625  |
| Total operating expenses                             | 6,084,187                                    | 4,738,516                                 | 28,155,336   |
| Loss from operations                                 | (6,084,187)                                  | (4,738,516)                               | (28,155,336)   |
| Interest income                                      | 10,409                                       | 29,963                                    | 312,312  |
| Interest expense                                     | (1,836)                                      | (1,281)                                   | (111,773)  |
| Adjustment to fair value of common stock warrants    | (1,043,389)                                  | -   | (1,043,389)  |
| Net loss   | \$(7,119,003)                                | \$ (4,709,834)                            | \$ (28,998,186)  |
| Net loss per share:                                  |  |   |  |
| Basic and diluted                                    | \$ (0.35)                                    | \$ (0.33)                                 |  |
| Weighted average shares outstanding used to compute: |  |   |  |
| Basic and diluted                                    | 20,062,776                                   | 14,081,218                                |  |

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





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

|   | For the six month periods from |                      | For the cumulative    |
|---|--------------------------------|----------------------|-----------------------|
|   | September 1, 2009              |                      | period from September |
|   | to February 28,                | September 1, 2008 to | 8, 2005               |
|   | 2010                           | February 28, 2009    | (inception) to        |
|   |                                |                      | February 28, 2010     |
| Cash flows from operating activities:                                       |                                |                      |                       |
| Net loss  | \$ (7,119,003)                 | \$ (4,709,834)       | \$ (28,998,186)       |
| Adjustments to reconcile net loss to net cash used in operating activities: |                                |                      |                       |
| Employee stock-based compensation exp.                                      | 53,005                         | 225,291              | 1,268,032             |
| Consultant stock-based compensation exp.                                    | 70,680                         | 22,795               | 478,294               |
| Fair value adjustment of common stock warrants                              | 1,043,389                      | -                    | 1,043,389             |
| Amortization of intangible assets   | 75,500                         | 69,249               | 320,708               |
| Depreciation of fixed assets  | 35,986                         | 45,766               | 386,926               |
| 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  | 57,257                         | (4,602)              | (49,796)              |
| Intangible assets   | -                              | -                    | (150,000)             |
| Deposits  | -                              | -                    | (100,206)             |
| Accounts payable  | 336,172                        | 213,429              | 949,748               |
| Accrued liabilities   | (612,061)                      | (47,648)             | (160,713)             |
| Deferred rent   | 1,097                          | 183                  | 992                   |
| Net cash used in operating activities                                       | (6,057,978)                    | (4,185,371)          | (24,630,187)          |
| Cash flows from investing activities:                                       |                                |                      |                       |
| Purchase of fixed assets  | (3,303)                        | (6,075)              | (479,653)             |
| Cash acquired in 2009 Merger  | 581,395                        | -                    | 581,395               |
| Net cash provided by (used in) investing activities                         | 578,092                        | (6,075)              | 101,742               |

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Cash flows from financing activities:

|   |              |              |              |
|---|--------------|--------------|--------------|
| Proceeds from the sale of common stock                      | 7,495,116    | -            | 24,881,116   |
| Proceeds from the exercise of common stock warrants         | 56,020       | -            | 6,565,520    |
| Proceeds from the exercise of common stock options          | 6,348        | -            | 15,048       |
| Fundraising costs   | (1,204,493)  | (35,848)     | (2,659,816)  |
| 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                         | (1,973)      | (1,621)      | (10,504)     |
| Net cash provided by (used in) financing activities         | 6,351,018    | (37,469)     | 29,101,364   |
| Net increase (decrease) in cash and cash equivalents        | 871,132      | (4,228,915)  | 4,572,919    |
| Cash and cash equivalents, beginning of period              | 3,701,787    | 7,546,912    | -            |
| Cash and cash equivalents, end of period                    | \$ 4,572,919 | \$ 3,317,997 | \$ 4,572,919 |

Supplemental disclosure of non-cash financing activities:

|  |              |           |              |
|--|--------------|-----------|--------------|
| Warrants issued in connection with financing                       | \$ 1,916,011 | \$ -      | \$ 8,549,583 |
| Common stock and warrants issued in connection with reverse merger | \$ 4,415,403 | \$ -      | \$ 4,415,403 |
| Acquisition of equipment in exchange for capital lease             | \$ -         | \$ 14,006 | \$ 21,403    |
| Notes receivable issued in exchange for common stock               | \$ -         | \$ -      | \$ 110,000   |
| Common stock issued for a finder's fee                             | \$ -         | \$ -      | \$ 102,000   |
| Common stock issued in asset purchase                              | \$ -         | \$ -      | \$ 2,898,624 |

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



RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(1) NATURE OF OPERATIONS AND BUSINESS RISKS

The accompanying condensed consolidated financial statements reflect the results of operations of Raptor Pharmaceutical Corp. (the “Company” or “Raptor”) and have been prepared in accordance with the accounting principles generally accepted in the United States of America.

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

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

Immediately following the effective time of the 2009 Merger, RPC’s (as of immediately prior to the 2009 Merger) stockholders owned approximately 95% of the Company’s outstanding common stock and the Company’s (as of immediately prior to the 2009 Merger) stockholders owned approximately 5% of the Company’s outstanding common stock.

RPC, the Company’s wholly-owned subsidiary, was the “accounting acquirer,” and for accounting purposes, the Company was deemed as having been “acquired” in the 2009 Merger. The board of directors and officers that managed and operated RPC immediately prior to the effective time of the 2009 Merger became the Company’s board of directors and officers. Additionally, following the effective time of the 2009 Merger, the business conducted by RPC immediately prior to the effective time of the 2009 Merger became primarily the business conducted by the Company.

The following reflects the Company’s current, post 2009 Merger corporate structure (State of Incorporation):

Raptor Pharmaceutical Corp., formerly TorreyPines Therapeutics, Inc. (Delaware)

| |  
TPTX, Inc. (Delaware) Raptor Pharmaceuticals Corp. (Delaware)

| |  
Raptor Therapeutics Inc. (Delaware) Raptor Discoveries Inc. (Delaware)  
(f/k/a Bennu Pharmaceuticals Inc.) (f/k/a Raptor Pharmaceutical Inc.)

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 preclinical division bioengineers novel drug candidates and drug-targeting platforms derived from the human receptor-associated protein (“RAP”) and related proteins, while its 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 two clinical stage product candidates in which the Company is seeking to out-license or form a development partnership: Convivia™ for the potential

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

treatment of aldehyde dehydrogenase (“ALDH2”) deficiency; and Tezampanel and NGX426, a non-opioid solution designed to treat chronic pain. Raptor’s preclinical programs target cancer, neurodegenerative disorders and infectious diseases. HepTide™ is designed to utilize engineered RAP-based peptides conjugated to drugs to target delivery to the liver to potentially treat primary liver cancer and hepatitis. NeuroTrans™ represents engineered RAP peptides created to target receptors in the brain and are 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. Raptor is also examining Tezampanel and NGX426, for the treatment of thrombotic disorder. The Company’s fiscal year end is August 31.

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 proprietary technology of others; dependence on key personnel; uncertain patent protection; and dependence on corporate partners and collaborators.

See the section titled “Risk Factors” in Part II Item 1A of 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 wholly owned subsidiaries, Raptor Pharmaceuticals Corp., Raptor Discoveries Inc., Raptor Therapeutics Inc., and TPTX, Inc., such subsidiaries incorporated in Delaware on May 5, 2006, September 8, 2005 (date of inception), August 1, 2007 and April 24, 2000, respectively. All inter-company accounts have been eliminated. The Company’s condensed consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. Through February 28, 2010, the Company had accumulated losses of approximately \$29.0 million. Management expects to incur further losses for the foreseeable future. Management believes that the Company’s cash and cash equivalents at February 28, 2010 will be sufficient to meet the Company’s obligations into the third calendar quarter of 2010. The Company is currently in the process of negotiating strategic partnerships and collaborations in order to fund its preclinical and clinical programs into 2011. The Company is also reviewing a few equity and debt financing proposals to provide potential funding beginning in the second calendar quarter of 2010. If the Company is not able to obtain funds that provide significant additional capital for it in the next two months, either through a strategic transaction or through the sale of its equity or debt, it may not be able to continue as a going concern. 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.

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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, 2009 and 2008. The October 27, 2009 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.

Management plans to seek additional debt and/or equity financing for the Company through private or public offerings or through a business combination or strategic partnership, but it 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 consolidated financial statements do not include any adjustments that might result from the failure to continue as a going concern.



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

**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS**

(b) Use of Estimates

The preparation of financial statements in conformity with 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 consolidated financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

(c) Fair Value of Financial Instruments

The carrying amounts of certain of the Company's financial instruments including cash and cash equivalents, prepaid expenses, accounts payable, accrued liabilities and capital lease liability approximate fair value due to their short maturities.

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

|              | For the three month periods ended February 28, |                |                |              |                |                |
|--------------|--|----------------|----------------|--------------|----------------|----------------|
|              | 2010   |                |                | 2009         |                |                |
|              | Preclinical                                    | Clinical       | Total          | Preclinical  | Clinical       | Total          |
| Net loss     | \$ (973,941)                                   | \$ (3,206,390) | \$ (4,180,331) | \$ (808,819) | \$ (1,442,019) | \$ (2,250,838) |
| Total assets | 829,051  | 10,971,702     | 11,800,753     | 945,233      | 5,354,210      | 6,299,443      |

|              | For the six month periods ended February 28, |                |                |                |                |                |
|--------------|--|----------------|----------------|----------------|----------------|----------------|
|              | 2010   |                |                | 2009           |                |                |
|              | Preclinical                                  | Clinical       | Total          | Preclinical    | Clinical       | Total          |
| Net loss     | \$ (1,966,258)                               | \$ (5,152,745) | \$ (7,119,003) | \$ (1,605,161) | \$ (3,104,673) | \$ (4,709,834) |
| Total assets | 829,051                                      | 10,971,702     | 11,800,753     | 945,233        | 5,354,210      | 6,299,443      |

(e) Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less to be cash equivalents.

(f) Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine, to the RAP technology and to the out-license and the rights to NGX 426 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 NGX 426, which has been classified as in-process research and development, will not be amortized until development is completed.

(g) 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.

RAPTOR PHARMACEUTICAL CORP.  
(A Development Stage Company)

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(h) 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.

(i) 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.

(j) Common Stock Warrant Liabilities

The warrants the Company issued in its December 2009 equity financing contain a conditional obligation that may require the Company to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under 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, the Company has classified the warrants as liabilities and will mark them to fair value at each period end.

(k) Marking-to-Market

The common stock warrants issued in the Company's December 2009 equity financing are classified as liabilities under ASC 480 and are, therefore, re-measured at the end of every reporting period with the change in value reported in its condensed consolidated statements of operations.

(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 an early development stage company. Research and development costs are charged to expense as incurred. Research and development expenses include scientists' salaries, 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.

(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.

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(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 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:

|                                       | 2010      | February 28,<br>2009 |
|---------------------------------------|-----------|----------------------|
| Warrants to purchase common stock     | 5,843,302 | 3,090,814            |
| Options to purchase common stock      | 1,191,534 | 954,227              |
| Total potentially dilutive securities | 7,034,836 | 4,045,041            |

(p) Stock Option Plan

Effective September 1, 2006, the Company adopted the provisions of Financial Accounting Standards Board (“FASB”) Accounting Standards Codification (“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 2006 Equity Incentive Plan, as amended. Under ASC 718, compensation cost is measured at the grant date based on the fair value of the equity instruments awarded and is recognized over the period during which an employee is required to provide service in exchange for the award, or the requisite service period, which is usually the vesting period. The fair value of the equity award granted is estimated on the date of the grant. The Company 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 September 2006, the FASB issued ASC Topic 820, Fair Value Measurements (“ASC 820”) (previously listed as SFAS No. 157, Fair Value Measurements). ASC 820 defines fair value, establishes a framework for measuring fair value in accordance with generally accepted accounting principles, and expands disclosures about fair value measurements. ASC 820 does not require any new fair value measurements; rather, it applies under other accounting pronouncements that require or permit fair value measurements. The provisions of ASC 820 are to be applied prospectively as of the beginning of the fiscal year in which it is initially applied, with any transition adjustment recognized as a cumulative-effect adjustment to the opening balance of retained earnings. The provisions of ASC 820

are effective for fiscal years beginning after November 15, 2007; therefore, the Company adopted ASC 820 as of September 1, 2008 for financial assets and liabilities. In accordance with FASB Staff Position 157-2, Effective Date of ASC 820, the Company adopted the provisions of ASC 820 for its non-financial assets and non-financial liabilities on September 1, 2009 and has determined that it had no material impact on the Company's results for the three and six months ended February 28, 2010. See Note 5, Fair Value Measurement, regarding the disclosure of the Company's value of its cash equivalents.

In February 2007, the FASB issued ASC Topic 825, Financial Instruments, ("ASC 825") (previously SFAS 159, The Fair Value Option for Financial Assets and Financial Liabilities, Including an Amendment of FASB Statement No. 115), which permits the measurement of many financial instruments and certain other asset and liabilities at fair value on an instrument-by-instrument basis (the fair value option). The guidance is applicable for fiscal years beginning after November 15, 2007; therefore, the Company adopted ASC 825 as of September 1, 2008. The Company has determined that ASC 825 had no material impact on its financial results for the three and six months ended February 28, 2010.

In June 2007, the EITF reached a consensus on ASC Topic 730, Research and Development, ("ASC 730") (previously EITF No. 07-3, Accounting for Nonrefundable Advance Payments for Goods or Services to Be Used in Future Research and Development Activities). ASC 730 specifies the timing of expense recognition for non-refundable advance payments for goods or services that will be used or rendered for research and development activities. ASC 730 was effective for fiscal years beginning

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after December 15, 2007, and early adoption is not permitted; therefore, the Company adopted ASC 730 as of September 1, 2008. The Company has determined that ASC 730 had no material impact on its financial results for the three and six months ended February 28, 2010.

In December 2007, the EITF reached a consensus on ASC Topic 808, Collaborative Agreement (“ASC 808”) (previously EITF 07-01, Accounting for Collaborative Arrangements). ASC 808 discusses the appropriate income statement presentation and classification for the activities and payments between the participants in arrangements related to the development and commercialization of intellectual property. The sufficiency of disclosure related to these arrangements is also specified. ASC 808 is effective for fiscal years beginning after December 15, 2008. As a result, ASC 808 is effective for the Company as of September 1, 2009. Based upon the nature of the Company’s business, ASC 808 could have a material impact on the Company’s financial position and consolidated results of operations in future years, but had no material impact for the three and six months ended February 28, 2010.

In December 2007, the FASB issued ASC Topic 805, Business Combinations, (“ASC 805”) (previously SFAS 141(R)) and FASB ASC Topic 810, Consolidation (“ASC 810”) (previously SFAS 160, Noncontrolling Interests in Consolidated Financial Statements, an amendment of ARB No. 51). These statements will significantly change the financial accounting and reporting of business combination transactions and non-controlling (or minority) interests in consolidated financial statements. ASC 805 requires companies to: (i) recognize, with certain exceptions, 100% of the fair values of assets acquired, liabilities assumed, and non-controlling interests in acquisitions of less than a 100% controlling interest when the acquisition constitutes a change in control of the acquired entity; (ii) measure acquirer shares issued in consideration for a business combination at fair value on the acquisition date; (iii) recognize contingent consideration arrangements at their acquisition-date fair values, with subsequent changes in fair value generally reflected in earnings; (iv) with certain exceptions, recognize pre-acquisition loss and gain contingencies at their acquisition-date fair values; (v) capitalize in-process research and development assets acquired; (vi) expense, as incurred, acquisition-related transaction costs; (vii) capitalize acquisition-related restructuring costs only if the criteria in ASC Topic 420, Exit and Disposal Cost Obligations (previously SFAS No. 146, Accounting for Costs Associated with Exit or Disposal Activities), are met as of the acquisition date; and (viii) recognize changes that result from a business combination transaction in an acquirer’s existing income tax valuation allowances and tax uncertainty accruals as adjustments to income tax expense. ASC 805 is required to be adopted concurrently with ASC 810 and is effective for business combination transactions for which the acquisition date is on or after the beginning of the first annual reporting period beginning on or after December 15, 2008 (the Company’s fiscal 2010). Early adoption of these statements is prohibited. The Company believes the adoption of these statements will have a material impact on significant acquisitions completed after September 1, 2009. See Note 9 which reflects the accounting treatment of the 2009 Merger utilizing these provisions.

In March 2008, the FASB issued ASC Topic 815, Derivatives and Hedging (“ASC 815”) (previously SFAS No. 161, Disclosures about Derivative Instruments and Hedging Activities). This statement will require enhanced disclosures about derivative instruments and hedging activities to enable investors to better understand their effects on an entity’s financial position, financial performance and cash flows. It is effective for financial statements issued for fiscal years and interim periods beginning after November 15, 2008, with early application encouraged. The Company adopted ASC 815 on December 1, 2008 and has determined that ASC 815 had no material impact on its financial results for the three and six months ended February 28, 2010.

In May 2008, the FASB released ASC Topic 470, Debt (“ASC 470”) (previously FASB Staff Position APB 14-1 Accounting For Convertible Debt Instruments That May Be Settled in Cash Upon Conversion (Including Partial Cash Settlement)), which alters the accounting treatment for convertible debt instruments that allow for either mandatory or optional cash settlements. ASC 470 specifies that issuers of such instruments should separately account for the liability and equity components in a manner that will reflect the entity’s nonconvertible debt borrowing rate when interest cost is recognized in subsequent periods. Furthermore, it would require recognizing interest expense in prior periods pursuant to retrospective accounting treatment. ASC 470 is effective for financial statements issued for fiscal years beginning after December 15, 2008; therefore, the Company adopted ASC 470 as of September 1, 2009. The Company has determined that ASC 470 had no material impact on its condensed consolidated financial statements for the three and six months ended February 28, 2010.

In June 2008, the FASB issued FASB ASC Topic 815, Derivatives and Hedging (“ASC 815”) (previously EITF 07-5, Determining Whether an Instrument (or an Embedded Feature) is Indexed to an Entity's Own Stock). ASC 815 requires entities to evaluate whether an equity-linked financial instrument (or embedded feature) is indexed to its own stock by assessing the instrument’s contingent exercise provisions and settlement provisions. Instruments not indexed to their own stock fail to meet the scope exception of SFAS No. 133, Accounting for Derivative Instruments and Hedging Activities, paragraph 11(a), and should be classified as a liability and marked-to-market. The statement is effective for fiscal years beginning after December 15, 2008 and



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interim periods within those fiscal years and is to be applied to outstanding instruments upon adoption with the cumulative effect of the change in accounting principle recognized as an adjustment to the opening balance of retained earnings. The Company adopted ASC 815 as of September 1, 2009 and has determined that ASC 815 had no material impact on the Company's condensed consolidated statement of operations for the three and six months ended February 28, 2010.

In April 2008, the FASB issued ASC Topic 350, Intangibles – Goodwill and Other (“ASC 350”) (previously FSP SFAS No. 142-3, Determination of the Useful Life of Intangible Assets). ASC 350 provides guidance with respect to estimating the useful lives of recognized intangible assets acquired on or after the effective date and requires additional disclosure related to the renewal or extension of the terms of recognized intangible assets. ASC 350 is effective for fiscal years and interim periods beginning after December 15, 2008. The Company adopted ASC 350 as of September 1, 2009 and has determined that ASC 350 had no material impact on the Company's condensed consolidated financial statements for the three and six months ended February 28, 2010.

In May 2009, the FASB issued ASC Topic 855, Subsequent Events (“ASC 855”) (previously SFAS No. 165, Subsequent Events). ASC 855 establishes general standards of accounting for and disclosure of events that occur after the balance sheet date but before financial statements are issued or are available to be issued. ASC 855 defines the period after the balance sheet date during which management of a reporting entity should evaluate events or transactions that may occur for potential recognition or disclosure in the financial statements, and the circumstances under which an entity should recognize events or transactions occurring after the balance sheet date in its financial statements. ASC 855 is effective for fiscal years and interim periods ending after June 15, 2009. The Company adopted ASC 855 as of August 31, 2009 and anticipates that the adoption will impact the accounting and disclosure of future transactions. The Company's management has evaluated and disclosed subsequent events from the balance sheet date of February 28, 2010 through the day before the date that these condensed consolidated financial statements were included in the Company's Quarterly Report on Form 10-Q and filed with the Securities and Exchange Commission (“SEC”).

ASC Topic 825, Financial Instruments, (“ASC 825”) (previously FSP FAS 107-1 and APB 28-1 amends FASB Statement No. 107, Disclosures about Fair Value of Financial Instruments), to require disclosures about the fair value of financial instruments for interim reporting periods of publicly traded companies as well as in annual financial statements. ASC 825 also amends APB Opinion No. 28, Interim Financial Reporting, to require those disclosures in summarized financial information at interim reporting periods. The adoption of ASC 825 did not have a material impact on the Company's condensed consolidated financial statements for the three and six months ended February 28, 2010.

In June 2009, the FASB issued SFAS No. 167, Amendments to FASB Interpretation No. 46(R) (“SFAS 167”), which has not yet been codified in the ASC. The amendments include: (i) the elimination of the exemption for qualifying special purpose entities, (ii) a new approach for determining who should consolidate a variable-interest entity, and (iii) changes to when it is necessary to reassess who should consolidate a variable-interest entity. This statement is effective for fiscal years beginning after November 15, 2009, and for interim periods within that first annual reporting period. The Company is currently evaluating the impact of this standard, however, it does not expect SFAS 167 will

have a material impact on its condensed consolidated financial statements.

In June 2009, the FASB issued ASC Topic 105, Generally Accepted Accounting Standards (“ASC 105”) (previously SFAS No. 168, The FASB Accounting Standards Codification™ and the Hierarchy of Generally Accepted Accounting Principles, a replacement of FASB Statement No. 162) (the “Codification”). The Codification, which was launched on July 1, 2009, became the single source of authoritative nongovernmental U.S. GAAP, superseding existing FASB, American Institute of Certified Public Accountants, EITF and related literature. The Codification eliminates the GAAP hierarchy contained in ASC 105 and establishes one level of authoritative GAAP. All other literature is considered non-authoritative. ASC 105 is effective for financial statements issued for interim and annual periods ending after September 15, 2009. The Company adopted ASC 105 as of September 1, 2009; however, references to both current GAAP and the Codification are included in this filing. The Company has determined that this provision had no material impact on its condensed consolidated financial statements for the three and six months ended February 28, 2010.

In June 2009, the FASB issued ASC Topic 860, Transfers and Servicing (Statement No. 166, Accounting for Transfers of Financial Assets — an amendment of FASB Statement No. 140) (“ASC 860”). The guidance removes the concept of a qualifying special purpose entity and changes the requirements for derecognizing financial assets. Many types of transferred financial assets that would have been derecognized previously are no longer eligible for derecognition. The guidance is effective for financial

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statements issued for fiscal years and interim periods beginning after November 15, 2009, and early adoption is prohibited. The guidance applies prospectively to transfers of financial assets occurring on or after the effective date. The Company is currently assessing the impact of ASC 860 and does not expect the adoption of this guidance to have a material impact on its condensed consolidated financial statements.

In January 2010, FASB issued Accounting Standards Update (“ASU”) 2010-06, Fair Value Measurements and Disclosures (Topic 820): Improving Disclosures about Fair Value Measurements (“ASU 2010-6”). The ASU amends Subtopic 820-10 with new disclosure requirements and clarification of existing disclosure requirements. New disclosures required include the amount of significant transfers in and out of levels 1 and 2 fair value measurements and the reasons for the transfers. In addition, the reconciliation for level 3 activity will be required on a gross rather than net basis. The ASU provides additional guidance related to the level of disaggregation in determining classes of assets and liabilities and disclosures about inputs and valuation techniques. The amendments are effective for annual or interim reporting periods beginning after December 15, 2009, except for the requirement to provide the reconciliation for level 3 activity on a gross basis, which will be effective for fiscal years beginning after December 15, 2010. The Company is currently assessing the impact of ASU 2010-6 and does not expect the adoption of this guidance to have a material impact on its condensed consolidated financial statements.

### (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 consolidated balance sheets as of February 28, 2010 and August 31, 2009 based on the estimated fair value of its agreement with BioMarin.

On December 14, 2007, the Company acquired the intellectual property and other rights to develop DR Cysteamine to treat various indications from the University of California at San Diego (“UCSD”) by way of a merger with Encode Pharmaceuticals, Inc. (“Encode”), a privately held research and development company, which held the intellectual property license with UCSD. The intangible assets, recorded at approximately \$2.6 million acquired in the merger with Encode, were primarily based on the value of the Company’s common stock and warrants issued to the Encode stockholder.

Intangible assets recorded as a result of the 2009 Merger were approximately \$1.1 million as discussed in Note 9 below.

|  |    |           |
|--|----|-----------|
| Intangible asset (IP license) related to the Encode merger, gross                  | \$ | 2,620,000 |
| Intangible asset related to NeuroTrans™ purchase from BioMarin, gross              |    | 150,000   |
| Intangible assets (out-license) related to the 2009 Merger, gross                  |    | 240,000   |
| In-process research and development (IP license) related to the 2009 Merger, gross |    | 900,000   |

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|                               |    |           |
|-------------------------------|----|-----------|
| Total gross intangible assets |    | 3,910,000 |
| Less accumulated amortization |    | (320,708) |
| Intangible assets, net        | \$ | 3,589,292 |

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 NGX 426, which has been classified as in-process research and development, will not be amortized until the product is developed. During the three and six months ended February 28, 2010 and 2009 and the cumulative period from September 8, 2005 (inception) to February 28, 2010, the Company amortized \$38,375, \$75,500, \$34,625, \$69,249 and \$320,708, respectively, of intangible assets to research and development expense.

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

| Amortization period                                       | Amortization expense |
|---|----------------------|
| September 8, 2005 (inception) to August 31, 2006 – actual | \$ 4,375             |
| Fiscal year ending August 31, 2007 – actual               | 7,500                |
| Fiscal year ending August 31, 2008 – actual               | 94,833               |
| Fiscal year ending August 31, 2009 – actual               | 138,500              |
| Fiscal year ending August 31, 2010 – estimate             | 141,000              |
| 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              |

(4) FIXED ASSETS

Fixed assets consisted of:

| Category                       | February 28, 2010 | August 31, 2009 | Estimated useful lives                 |
|--------------------------------|-------------------|-----------------|--|
| Leasehold improvements         | \$ 113,422        | \$ 113,422      | 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 | 83,740            | 80,437          | 3 years                                |
| Capital lease equipment        | 14,006            | 14,006          | Shorter of life of asset or lease term |
| Total at cost                  | 491,659           | 488,356         |  |
| Less: accumulated depreciation | (377,960)         | (343,621)       |  |

|                         |    |         |    |         |
|-------------------------|----|---------|----|---------|
| Total fixed assets, net | \$ | 113,699 | \$ | 144,735 |
|-------------------------|----|---------|----|---------|

Depreciation expense for the three and six months ended February 28, 2010 and 2009 and the cumulative period from September 8, 2005 (inception) to February 28, 2010 was \$18,817, \$35,986, \$24,087, \$45,766 and \$386,926, respectively. Accumulated depreciation on capital lease equipment was \$6,105 and \$3,951 as of February 28, 2010 and August 31, 2009, 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.

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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 February 28, 2010 and August 31, 2009 are summarized as follows:

|                                     |                    |             |                    |             | February 28, 2010  |
|-------------------------------------|--------------------|-------------|--------------------|-------------|--------------------|
| Assets                              | Level 1            | Level 2     | Level 3            |             |                    |
| Fair value of cash equivalents      | \$4,329,813        | \$ —        | \$ —               | \$ —        | \$4,329,813        |
| <b>Total</b>                        | <b>\$4,329,813</b> | <b>\$ —</b> | <b>\$ —</b>        | <b>\$ —</b> | <b>\$4,329,813</b> |
| <b>Liabilities</b>                  |                    |             |                    |             |                    |
| Fair value of common stock warrants | \$ —               | \$ —        | \$2,959,400        |             | \$2,959,400        |
| <b>Total</b>                        | <b>\$ —</b>        | <b>\$ —</b> | <b>\$2,959,400</b> |             | <b>\$2,959,400</b> |

|                                |                     |             |             |             | August 31, 2009     |
|--------------------------------|---------------------|-------------|-------------|-------------|---------------------|
| Assets                         | Level 1             | Level 2     | Level 3     |             |                     |
| Fair value of cash equivalents | \$ 3,515,353        | \$ —        | \$ —        | \$ —        | \$ 3,515,353        |
| <b>Total</b>                   | <b>\$ 3,515,353</b> | <b>\$ —</b> | <b>\$ —</b> | <b>\$ —</b> | <b>\$ 3,513,353</b> |

Cash equivalents represent the fair value of the Company's investment in two money market accounts as of February 28, 2010 and August 31, 2009.

#### Marking-to-Market

The common stock warrants issued in the Company's December 2009 equity financing are classified as liabilities under ASC 480 and are, therefore, re-measured at the end of every reporting period with the change in value reported in its condensed consolidated statements of operations.

On February 28, 2010, as a result of the marking-to-market of the warrant liability, the Company recorded a loss of \$1.0 million in the line item adjustment to fair value of common stock warrants in its condensed consolidated statement of operations.

Warrant liability  
in millions

|  |    |      |  |
|--|----|------|--|
| Fair value at issuance date on December 23, 2009 | \$ | 1.92 |  |
|  |    | 1.04 |  |

Adjustment to mark to market common  
stock warrants at February 28, 2010

|  |    |      |
|--|----|------|
| Common stock warrant liability at fair<br>value on February 28, 2010 | \$ | 2.96 |
|--|----|------|



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(6) ACCRUED LIABILITIES

Accrued liabilities consisted of:

|  | February 28, 2010 | August 31, 2009 |
|--|-------------------|-----------------|
| Salaries and benefits and other obligations related to 2009 Merger | \$ 165,520        | \$ —            |
| Legal fees   | 146,742           | 195,552         |
| Accrued vacation   | 66,701            | 38,109          |
| Salaries and wages   | 60,065            | 10,500          |
| Patent costs   | 40,000            | 57,351          |
| Consulting — research and development                              | 19,003            | 19,720          |
| Auditing and tax preparation fees                                  | 13,070            | 21,000          |
| 2009 Merger joint proxy/prospectus                                 | —                 | 109,011         |
| Other  | 8,912             | -               |
| Total accrued liabilities  | \$ 520,013        | \$ 451,243      |

(7) IN-PROCESS RESEARCH AND DEVELOPMENT

On October 17, 2007, the Company purchased certain assets of Convivia, Inc. (“Convivia”), including intellectual property, know-how and research reports related to a product candidate targeting liver ALDH2 deficiency, a genetic metabolic disorder. The Company issued an aggregate of 101,991 shares of its restricted, unregistered common stock to the seller and other third parties in settlement of the asset purchase. Pursuant to ASC Topic 730, Research and Development (previously Financial Accounting Standard (“FAS”) 2 Paragraph 11(c), Intangibles Purchased From Others), the Company has expensed the value of the common stock issued in connection with this asset purchase as in-process research and development expense. The amount expensed was based upon the closing price of Raptor’s common stock on the date of the closing of the asset purchase transaction of \$2.359 per share multiplied by the aggregate number of shares of Raptor common stock issued or 101,991 for a total expense of \$240,625 recorded on Raptor’s consolidated statement of operations during the year ended August 31, 2008.

(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, as interpreted by 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 six months ended February 28, 2010 and 2009 and for the cumulative period from September 8, 2005 (inception) to February 28, 2010 was \$27,202, \$53,005, \$108,773, \$225,291 and \$1,268,032, respectively, of which cumulatively \$1,074,773 was included in general and administrative expense and \$193,259 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.

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

| Period*  | Risk-free<br>interest rate | Expected<br>life of<br>stock<br>option | Annual<br>volatility | Annual<br>turnover<br>rate |
|--|----------------------------|--|----------------------|----------------------------|
| September 8, 2005<br>(inception) to August 31,<br>2006** | 5%                         | 10 years                               | 100%                 | 0%                         |
| Quarter ended November<br>30, 2006                       | 5%                         | 8 years                                | 100%                 | 10%                        |
| Quarter ended February 28,<br>2007                       | 5%                         | 8 years                                | 100%                 | 10%                        |
| Quarter ended May 31,<br>2007                            | 5%                         | 8 years                                | 100%                 | 10%                        |
| Quarter ended August 31,<br>2007                         | 4%                         | 8 years                                | 100%                 | 10%                        |
| Quarter ended November<br>30, 2007                       | 3.75%                      | 8 years                                | 109%                 | 10%                        |
| Quarter ended February 29,<br>2008                       | 2%                         | 8 years                                | 119%                 | 10%                        |
| Quarter ended May 31,<br>2008                            | 2%                         | 8 years                                | 121%                 | 10%                        |
| Quarter ended August 31,<br>2008                         | 2.5%                       | 8 years                                | 128%                 | 10%                        |
| Quarter ended November<br>30, 2008                       | 1.5%                       | 7 years                                | 170%                 | 10%                        |
| Quarter ended February 28,<br>2009                       | 2.0%                       | 7 years                                | 220%                 | 10%                        |
|  | 2.6%                       | 7 years                                | 233%                 | 10%                        |

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|                                    |      |         |      |     |
|------------------------------------|------|---------|------|-----|
| Quarter ended May 31,<br>2009      |      |         |      |     |
| Quarter ended August 31,<br>2009   | 3.2% | 7 years | 240% | 10% |
| Quarter ended November<br>30, 2009 | 3.0% | 7 years | 245% | 10% |
| Quarter ended February 28,<br>2010 | 3.1% | 7 years | 55%  | 10% |

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

\*\* Stock-based compensation expense was recorded on the 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 consolidated statements of operations, with no effect on the consolidated statements of operations, per the guidelines of APB Opinion No. 25. Consultant stock-based compensation expense has been recorded on the 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 month period ended February 28, 2010, the Company changed its volatility calculation to reflect its historical trading commencing on September 30, 2009, which is the date the Company merged and started trading on NASDAQ. The Company originally calculated volatility of its stock for Black-Scholes purposes 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.

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 six months ended February 28, 2010 and 2009 and for the cumulative period from September 8, 2005 (inception) to February 28, 2010 was \$5,480, \$70,680, \$9,802, \$22,795 and \$478,293, respectively, of which cumulatively \$118,919 was included in general and administrative expense and \$359,374 was included in research and development expense.



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

|                                     | Option shares | Weighted<br>average<br>exercise price | Exercisable | Weighted average<br>fair value of<br>options granted |
|-------------------------------------|---------------|---------------------------------------|-------------|--|
| Outstanding at September 8,<br>2005 | —             | —                                     | —           | —  |
| Granted                             | 580,108       | \$ 2.64                               | —           | \$ 2.47  |
| Exercised                           | —             | —                                     | —           | —  |
| Canceled                            | —             | —                                     | —           | —  |
| Outstanding at August 31,<br>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,<br>2007   | 684,179       | \$ 2.63                               | 273,236     | \$ 2.45  |
| Granted                             | 223,439       | \$ 2.27                               | —           | \$ 2.21  |
| Exercised                           | —             | —                                     | —           | —  |
| Canceled                            | —             | —                                     | —           | —  |
| Outstanding at August 31,<br>2008   | 907,618       | \$ 2.54                               | 600,837     | \$ 2.39  |
| Granted                             | 81,595        | \$ 1.13                               | —           | \$ 1.04  |
| Exercised                           | —             | —                                     | —           | —  |
| Canceled                            | —             | —                                     | —           | —  |
| Outstanding at August 31,<br>2009   | 989,213       | \$ 2.42                               | 826,303     | \$ 2.28  |
| Granted                             | 50,590        | \$ 3.43                               | 34,959      | \$ 2.26  |
| Assumed in the 2009<br>Merger       | 161,044       | \$ 114.12                             | 158,475     | \$ 2.63  |
| Exercised                           | (2,115)       | \$ 2.24                               | —           | \$ 2.24  |
| Canceled                            | (5,606)       | \$ 376.53                             | —           | \$ 2.63  |
| Outstanding at November<br>30, 2009 | 1,193,126     | \$ 17.30                              | 1,109,737   | \$ 2.34  |
| Granted                             | -             | -                                     | -           | -  |
| Exercised                           | (850)         | \$ 1.88                               | -           | \$ 1.76  |
| Canceled                            | (742)         | \$ 1,559.50                           | -           | \$ 2.63  |
|                                     | 1,191,534     | \$ 16.35                              | 1,035,521   | \$ 2.34  |

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Outstanding at February 28,  
2010

The weighted average intrinsic values of stock options outstanding and expected to vest and stock options exercisable as of February 28, 2010 and 2009 were \$107,997, \$46,195, \$3,750 and zero, respectively.

There were 2,949,410 options available for grant as of February 28, 2010 under the 2006 Equity Compensation Plan, as amended, under the stock option plans assumed in the 2009 Merger, and under the 2010 Equity Incentive 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. As of February 28, 2010, the options outstanding consisted of the following:

| Range of<br>exercise prices | Number of<br>options<br>outstanding<br>(#) | Options outstanding  |   | Options exercisable                     |   |
|-----------------------------|--|--|---|---|---|
|                             |  | Weighted<br>average<br>remaining<br>contractual<br>life (yrs.) | Weighted<br>average<br>exercise<br>price (\$) | Number of<br>options<br>exercisable (#) | Weighted<br>average<br>exercise price<br>(\$) |
| \$0 to \$1.00               | 34,969                                     | 9.13   | .85   | 7,285                                   | 0.85  |
| \$1.01 to \$2.00            | 75,769                                     | 8.69   | 1.55  | 45,897                                  | 1.54  |
| \$2.01 to \$3.00            | 872,473                                    | 6.57   | 2.56  | 812,484                                 | 2.58  |
| \$3.01 to \$4.00            | 94,146                                     | 9.62   | 3.64  | 59,178                                  | 3.84  |
| \$4.01 to \$5.00            | 62,104                                     | 9.63   | 4.57  | 58,604                                  | 4.59  |
| \$5.01 to \$1,564           | 52,073                                     | 5.00   | 316.43  | 52,073                                  | 316.43  |
|                             | 1,191,534                                  | 6.96   | 16.35   | 1,035,521                               | 18.49   |

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At February 28, 2010, the total unrecognized compensation cost was approximately \$253,000. The weighted average period over which it is expected to be recognized is 2.75 years.

(9) ISSUANCE OF COMMON STOCK

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

During the six month period ended February 28, 2010, the Company received \$56,020 from the exercise of a warrant issued to a placement agent in the May/June 2008 private placement in exchange for the issuance of 23,744 shares of the Company's common stock and the Company issued 7,680 shares of its common stock resulting from a cashless exercise of a warrant issued in 2007 in connection with the purchase of DR Cysteamine. During the cumulative period from September 8, 2005 (inception) through February 28, 2010, the Company received \$6.566 million from the exercise of warrants in exchange for the issuance of an aggregate of 3,576,454 shares.

During the six month period ended February 28, 2010, the Company received \$6,348 from the exercise of stock options in exchange for 2,965 shares of the Company's common stock. For the cumulative period from September 8, 2005 (inception) through February 28, 2010, the Company received \$15,044 from the exercise of stock options resulting in the issuance of 6,345 shares of common stock. Total common stock outstanding as of February 28, 2010 was 22,580,365 shares.

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. 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. 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 condensed 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 Pharmaceuticals, Inc. ("Encode"), a privately held development stage company. 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

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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 (“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. To-date, Raptor has paid \$270,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis and in NASH.

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 "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 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.

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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.

#### 2009 MERGER AND NASDAQ LISTING

On September 29, 2009, the Company, formerly known as TorreyPines Therapeutics, Inc. ("TorreyPines") and Raptor Pharmaceuticals Corp. ("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, C.P.A., 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 merger, including having access to an expanded pipeline of product candidates and having development capabilities across a wider spectrum of diseases and

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markets. Another 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. This 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) | %      |
|-----------------------------------|------------------|--------|
| 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      |
| <br>Total identifiable assets     | <br>1.14         | <br>26 |
| Plus Goodwill                     | 3.28             | 74     |
| Total net assets acquired         | \$ 4.42          | 100    |

Acquisition costs incurred by the Company related to the merger were approximately \$0.6 million and were expensed as incurred. If the reverse merger had occurred on September 1, 2008, the Company's revenues would have increased by approximately \$1.5 million from fees earned by TorreyPines from the sale one of its programs in the quarter ended December 31, 2008, for total pro forma revenues of \$1.5 million for the six months ended February 28, 2009. Net loss would have increased by approximately \$2.5 million due to an increase of revenues of \$1.5 million described above offset by \$3.1 million of loss on impairment of purchased patents recognized by TorreyPines during the period plus \$0.9 million in transaction costs and costs associated with obligations owed to the TorreyPines employees for a pro forma net loss of \$7.2 million (or \$(0.51) per share) for the six month period ended February 28, 2009. For the three month period ended February 28, 2009 the Company's revenues would have remained zero and the Company's net loss and net loss per share would have remained \$(2.3) million or \$(0.16) per share. If the reverse merger had occurred on September 1, 2009, the Company's revenues would have remained zero for the six months ended February 28, 2010. Net loss for the six months ended February 28, 2010 would have increased by approximately \$0.3 million due to the transaction costs which were accrued during the fiscal year ended August 31, 2009, for a pro forma net loss of \$7.4 million or \$(0.37) per share. For the three month period ended February 28, 2010 the Company's revenues would have remained zero and the Company's net loss and net loss per share would have remained \$(4.2) million or \$(0.19) per share.

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 "Placement Agent"), relating to the issuance and sale to the Investors (as defined below)

pursuant to a registered direct offering (the “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 Placement Agent for the Offering received a placement fee equal to 6.5% of the gross cash proceeds to the Company from the 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 the Placement Agent 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 Offering, following execution of the Placement Agent Agreement, the Company also entered into a definitive securities purchase agreement (the “Purchase Agreement”), dated as of December 17, 2009, with 33 investors set forth

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on the signature pages thereto (collectively, the “Investors”) with respect to the Offering of the Units, whereby, on an aggregate basis, the Investors agreed to purchase 3,747,558 Units for a negotiated purchase price of \$2.00 per Unit, amounting to gross proceeds of approximately \$7.5 million and estimated net proceeds after commissions and expenses of approximately \$6.9 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. 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 are classified as liability, as discussed further below in Note 10.

The following is a summary of common stock outstanding as of February 28, 2010:

| Transaction                                     | Date                  | Common Stock<br>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 – Nov. 2008 | 148,616                |
| 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         | June/July 2009        | 2,031,670              |
| PIPE  | August 2009           | 1,738,226              |
| Warrant exercises                               | September 2009        | 31,424                 |
| Shares issued in connection with reverse merger | September 2009        | 940,863                |



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|   |                             |            |
|---|-----------------------------|------------|
| Stock option exercises                      | October 2009 – Jan.<br>2010 | 2,965      |
| Registered direct financing                 | December 2009               | 3,747,558  |
| Total shares of common stock<br>outstanding |                             | 22,580,365 |

(10) WARRANTS

The table reflects the number common stock warrants outstanding as of February 28, 2010:

|   | Number of<br>shares<br>exercisable | Exercise price  | Expiration date    |
|---|------------------------------------|-----------------|--------------------|
| Issued in lieu of deferred<br>legal fees              | 13,987                             | \$ 2.57         | 2/13/2011          |
| Issued in connection with<br>Encode merger            | 233,309                            | \$ 2.87         | 12/13/2015         |
| Issued to PIPE investors in<br>May / June 2008        | 299,564                            | \$ 3.86         | 5/21/2010          |
| Issued to placement agents<br>in May / June 2008      | 465,816                            | \$ 2.36         | 5/21/2013          |
| Issued to PIPE investors in<br>August 2009            | 869,113                            | \$ 2.57/\$3.22* | 8/21/2011          |
| Issued to placement agents<br>in August 2009          | 129,733                            | \$ 1.50         | 8/21/2014          |
| TorreyPines warrants<br>assumed in 2009 Merger        | 9,271                              | \$ 87.71**      | 7/1/2010 9/26/2015 |
| Issued to registered direct<br>investors in Dec. 2009 | 1,873,779                          | \$ 2.45         | 6/22/2011          |
| Issued to registered direct<br>investors in Dec. 2009 | 1,873,779                          | \$ 2.45         | 12/23/2014         |
| Issued to placement agent<br>in Dec. 2009             | 74,951                             | \$ 2.50         | 12/23/2014         |
| Total warrants outstanding                            | 5,843,302                          | \$ 2.66**       |                    |

\* First year exercisable at \$2.57; second year exercisable at \$3.22

\*\* Average exercise price

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The warrants the Company issued in its December 2009 equity financing contain a conditional obligation that may require the Company to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under 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, the Company has classified the warrants 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 using the following assumptions:

| Black-Scholes<br>inputs    | Series A at<br>issuance<br>December<br>23, 2009 | Series A at<br>quarter<br>ended<br>February<br>28, 2010 | Series B at<br>issuance<br>December<br>23, 2009 | Series B at<br>quarter ended<br>February 28,<br>2010 | Placement<br>agent at<br>issuance<br>December<br>23, 2009 | Placement<br>agent at<br>quarter ended<br>February 28,<br>2010 |
|----------------------------|---|---|---|--|---|--|
| Stock price                | \$1.89  | \$2.27  | \$1.89  | \$2.27   | \$1.89  | \$2.27   |
| Exercise price             | \$2.45  | \$2.45  | \$2.45  | \$2.45   | \$2.50  | \$2.50   |
| Risk free interest<br>rate | 2.23%   | 2.39%   | 0.56%   | 0.57%  | 2.23%   | 2.39%  |
| Volatility                 | 49.28%  | 55.03%  | 49.28%  | 55.03%   | 49.28%  | 55.03%   |
| Expected term in<br>years  | 5   | 4.75  | 1.5   | 1.25   | 5   | 4.75   |
| Dividend                   | 0   | 0   | 0   | 0  | 0   | 0  |

(11) COMMITMENTS AND CONTINGENCIES

CONTRACTUAL OBLIGATIONS WITH BIOMARIN

Pursuant to the terms of the asset purchase agreement the Company entered into with BioMarin Pharmaceutical Inc. (“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 Raptor 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 Raptor 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;

\$2,500,000 upon the Company's successful completion of a Phase II 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 III 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.

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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 ("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, 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. On March 31, 2008, the Company issued 23,312 shares of Raptor's Common Stock valued at \$56,000 to Mr. Daley pursuant to this milestone reflecting the execution of an agreement to supply the active pharmaceutical ingredient for Convivia™, combined with the execution of a formulation agreement to produce the oral formulation of Convivia™.

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

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

23,312 shares of the Company's restricted, unregistered Common Stock within fifteen (15) days of completing predetermined benchmarks in a Major Market by the Company or its licensee of the first phase II 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 II 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.

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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, 46,625 shares of Raptor's common stock valued at \$83,000 and paid \$30,000 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 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 year ended August 31, 2009, the Company has fulfilled by spending approximately \$4.1 million on such programs) pursuant to the License Agreement. To-date, the Company has paid \$270,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis and in 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.

CONTRACTUAL OBLIGATIONS TO TPTX, INC. EMPLOYEES

Pursuant to the documents related to the 2009 Merger, including amended employment agreements with the TPTX, Inc. employees, who were former executives of TorreyPines prior to the merger, the Company was obligated to pay such former executives their salaries, benefits and other obligations through February 28, 2010, which obligations

were extended through mid-April 2010. The remaining obligations as of February 28, 2010 are approximately \$166,000 in the aggregate.

#### OFFICE LEASES

In March 2006, the Company entered into a lease for the Company's executive offices and research laboratory in Novato, California. Base monthly payments were \$5,206 per month subject to annual rent increase of between 3% to 5%, based on the Consumer Price Index ("CPI"). In March 2006, the Company paid \$20,207 as a security deposit on this lease, which expired in March 2009. Effective April 1, 2007, the Company leased additional office space adjoining the existing leased space, increasing the Company's base rent to \$9,764 per month without extending the term of the original lease. The original lease allows for one three-year extension at the market rate and up to \$18,643 in reimbursement for tenant improvements. In June 2008, the Company's rent increased to \$10,215, reflecting a CPI increase of 3% plus an increase in operating costs for the period from April 1, 2008 to March 31, 2009. In September 2008, the Company executed a lease addendum replacing the one three-year extension with two two-year extensions commencing on April 1, 2009 and renegotiated the first two-year extension base rent to \$10,068 with an adjustment after the first year for CPI between 3% (minimum) and 5% (maximum). In January 2010, the Company entered into a one year lease for a administrative offices in San Mateo, California for \$2,655 per month. During the three and six month periods ended February 28, 2010 and 2009 and the cumulative period from September 8, 2005 (inception) to February 28, 2010, the Company paid \$34,597, \$68,144, \$30,736, \$62,473 and \$436,539, respectively, in rent.

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NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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

| Period                              | Amount    |
|-------------------------------------|-----------|
| Fiscal year ending August 31, 2010  | \$ 79,164 |
| September 1, 2010 to March 31, 2011 | 87,408    |

#### CAPITAL LEASE

In June 2006, the Company leased a photocopier machine for 36 months at \$242 per month. There was no purchase option at the end of the lease. Based on the fair value and estimated useful life of the photocopier and the life of the lease and the photocopier, the Company has accounted for the lease as a capital lease. In September 2008, the Company replaced the originally leased photocopier with a new photocopier which is subject to a 39-month lease at \$469 per month. There were no penalties imposed for cancelling the original lease.

The future lease payments under the capital lease are as follows:

| Period  | Amount   |
|---|----------|
| Fiscal year ending August 31, 2010                  | \$ 2,812 |
| Fiscal year ending August 31, 2011                  | 5,625    |
| September 1, 2011 to December 31, 2011              | 1,875    |
| Total future capital lease payments                 | 10,312   |
| Less interest                                       | (1,492)  |
| Total current and long-term capital lease liability | \$ 8,820 |

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

#### CONTRACT/CLINICAL RESEARCH AGREEMENTS

During the three month period ended February 28, 2010, the Company maintained several contracts with research and clinical organizations to develop research assays and to assist with clinical research for Raptor's cystinosis program.

The future commitments pursuant to the research agreement are as follows:

| Period                                | Amount       |
|---------------------------------------|--------------|
| March 1, 2010 through August 31, 2010 | \$ 1,246,491 |
| September 1, 2010 to August 31, 2011  | 700,618      |

#### STORAGE AND CLINICAL DISTRIBUTION AGREEMENT

During the three month period ended February 28, 2010, the Company maintained an agreement with a company that stores and distributes clinical materials for Raptor's cystinosis trial. The future commitments pursuant to this



agreement are as follows:

| Period                                |    | Amount  |
|---------------------------------------|----|---------|
| March 1, 2010 through August 31, 2010 | \$ | 55,598  |
| Fiscal year ending August 31, 2011    |    | 111,196 |
| Fiscal year ending August 31, 2012    |    | 83,397  |

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NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

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. Also in July 2008, the Company executed a supply agreement with a contract manufacturer for the active pharmaceutical agreement of DR Cysteamine. The future commitments pursuant to these contracts are as follows:

| Period                                |    | Amount    |
|---------------------------------------|----|-----------|
| March 1, 2010 through August 31, 2010 | \$ | 1,364,783 |
| Fiscal year ending August 31, 2011    |    | 245,777   |
| Fiscal year ending August 31, 2012    |    | 67,439    |

(12) SUBSEQUENT EVENTS

The Company's management has evaluated and disclosed subsequent events from the balance sheet date of February 28, 2010 through the day before the date that the condensed consolidated financial statements were included in Company's Quarterly Report on Form 10-Q and filed with the SEC.



Item 2. Management's Discussion and Analysis 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 operation, 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," 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;
- 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;
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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” 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

### Overview

You should read the following discussion in conjunction with our condensed consolidated financial statements as of February 28, 2010, 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., TPTX, Inc., Raptor Discoveries Inc. (f/k/a Raptor Pharmaceutical Inc.), or Raptor Discoveries, and Raptor Therapeutics Inc. (f/k/a Bennu Pharmaceuticals Inc.), or Raptor Therapeutics. 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.”

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 three other clinical-stage product candidates, for which we are seeking business development partners but are not actively developing, and we have four preclinical product candidates we are developing, three 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.

### Other Clinical-Stage Product Candidates

We have three clinical-stage product candidates for which we are seeking partners:

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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 and NGX426, non-opioids for the potential treatment of migraine, acute pain, and chronic pain.

#### 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 platforms include the following:

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

We are also examining our glutamate receptor antagonists, tezampanel and NGX426, for the potential treatment of thrombosis disorder.

## Future Activities

Over the next 12 months, we plan to conduct research and development activities based upon our DR Cysteamine clinical programs and continued development of our preclinical product candidates. We also plan to seek business development partners for our Convivia™ product candidate and Tezampanel and NGX426. 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. There can be no assurances that our research and development activities will be successful. Our plans for research and development activities over the next 12 months can only be implemented if we are successful in raising significant funds during this period. If we do not raise significant additional funds, we may not be able to continue as a going concern.

## 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.

### 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.

We believe that 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 EMEA, approved drug to treat cystinosis, a rare genetic disease. Immediate-release cysteamine is effective at preventing or delaying kidney failure and other serious health problems in cystinosis patients. However, patient compliance is challenging due to the requirement for frequent 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 FDA granted orphan drug designation for DR Cysteamine for the treatment of cystinosis in 2006.

In June 2009, we commenced our Phase IIb 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. We had a post-Phase IIb meeting with the FDA at the end of January 2010, resulting in the FDA requesting that we submit a special protocol assessment application, or SPA, that would allow the FDA to review and approve the Phase III protocol in advance of conducting the pivotal Phase III trial. We submitted our SPA to the FDA in early March 2010 and anticipate a definitive response from the FDA in the coming weeks. While we await feedback from the FDA on our SPA, we are setting up several clinical sites in the U.S. and E.U. for our pivotal Phase III clinical study in



cystinosis patients which we anticipate will commence in the second quarter of 2010. While we plan to commercialize DR Cysteamine in the U.S. by ourselves, we are actively negotiating with a potential development partner for DR Cysteamine for markets outside of the U.S.

#### 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 October 2009, we announced positive findings from the completed treatment phase of this open-label Phase IIa clinical trial. At the completion of the initial six-month treatment phase, the study achieved the primary endpoint: mean blood levels of alanine aminotransferase, or ALT, a common biomarker for NASH, were reduced by over 50%. Additionally, over half of the study participants had achieved normalized ALT levels by the end of the treatment phase.

There are no currently approved drug therapies for NASH, and patients are limited to 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.

The NASH trial entails six months of treatment followed by a six-month post-treatment monitoring period. Eligible patients with baseline ALT and aspartate aminotransferase or AST measurements at least twice that of normal levels were enrolled to receive twice-daily, escalating oral doses of up to 1,000 mg of DR Cysteamine. The trial, now completed, had enrolled eleven NASH patients between 11-18 years old. No major adverse events were reported during the six-month treatment phase. Final data are being presented by UCSD clinicians in May 2010 at a clinical conference. We are in early stages of discussions to co-develop or partner DR Cysteamine for the potential treatment of NASH and are reviewing the trial design for the next potential clinical trial 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 institution, CHU d' Angers, on a Phase II clinical trial investigating DR Cysteamine in HD patients, anticipated to begin in the second quarter of 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.

#### Other Clinical-Stage Product Candidates

We have three clinical-stage product candidates for which we are seeking partners.

#### Convivia™ for Liver Aldehyde Dehydrogenase 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 IIa 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. We are actively seeking corporate partnerships with pharmaceutical companies in selected Asian countries to continue clinical development of Convivia™ in those countries. We are currently negotiating a license agreement with a company that plans on developing Convivia™ in Taiwan and Korea.

#### Tezampanel and NGX426 for the Potential Treatment of Migraine and Pain

Tezampanel and NGX426, the oral prodrug of tezampanel, are what we believe to be first-in-class compounds that may represent novel treatments for both pain and non-pain indications. Tezampanel and NGX426 are receptor antagonists that target and inhibit a specific group of receptors—the AMPA and kainate glutamate receptors—found in the brain and other tissues. While normal glutamate production is essential, excess glutamate production, either through injury or disease, has been implicated in a number of diseases and disorders. Published data show that during a migraine, increased levels of glutamate activate AMPA and kainate receptors, result in the transmission of pain and, in many patients, the development of increased pain sensitivity. By acting at both the AMPA and kainate receptor sites to competitively block the binding of glutamate, tezampanel and NGX426 have the potential to treat a number of diseases and disorders. These include chronic pain, such as migraine and neuropathic pain, muscle spasticity and a condition known as central sensitization, a persistent and acute sensitivity to pain.

Results of a Phase IIb clinical trial of tezampanel were released in October 2007. In the trial, a single dose of tezampanel given by injection was statistically significant compared to placebo in treating acute migraine headache. This was the sixth Phase II trial in which tezampanel has been shown to have analgesic activity. Based on a review of the Phase II data, the FDA previously agreed that tezampanel may move forward into a Phase III program for acute migraine.

In December 2008, results of NGX426 in a human experimental model of cutaneous pain, hyperalgesia and allodynia demonstrated a statistically significant reduction in spontaneous pain, hyperalgesia and allodynia compared to placebo following injections of capsaicin (i.e., chili oil) under the skin. In February 2009, results from a Phase 1 multiple dose trial of NGX426 showed that the compound is safe and well-tolerated in healthy male and female subjects when dosed once daily for five consecutive days.

In November 2009, we announced the presentation of clinical trial data on NGX426 at the 12th International Conference on the Mechanisms and Treatment of Neuropathic Pain. The results of the study led by Mark Wallace, M.D., Professor of Clinical Anesthesiology at the Center for Pain Medicine of the University of California at San Diego, suggested that NGX426 has the potential to be effective in a variety of neuropathic pain states, which are caused by damage to or dysfunction of the peripheral or central nervous system rather than stimulation of pain receptors.

We are currently seeking out-licensing partners for the migraine and pain programs and no development costs will be incurred for further development of these indications.

#### 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 and Hepatitis C

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 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 a chemotherapeutic for testing in vitro and in appropriate preclinical models for the potential treatment of HCC.

We are also evaluating conjugates between HepTide™ and an antiviral agent for testing in vitro and in appropriate preclinical models for the potential treatment of hepatitis C.

#### 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 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, 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. We are currently planning another breast tumor preclinical model study with researchers at Washington University in the continued development of WntTide™.

#### Tezampanel and NGX426 for the Potential Treatment of Thrombotic Disorder

Research conducted at Johns Hopkins University, or JHU, by Craig Morrell, D.V.M., Ph.D., and Charles Lowenstein, M.D. demonstrated the importance of glutamate release in promoting platelet activation and thrombosis. Research shows that platelets treated with an AMPA/kainate receptor antagonist such as tezampanel or NGX426 are more resistant to glutamate-induced aggregation than untreated platelets. This identifies the AMPA/kainate receptors on platelets targeted by tezampanel or NGX426 as a new antithrombotic target with a different mechanism of action than Plavix®, aspirin or tPA. We have licensed the intellectual property of Tezampanel and NGX 426 for the treatment of thrombotic disorder from JHU and are in discussions with potential collaborators regarding the development of this product candidate.

## 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 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 such 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 ConviviaTM

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 ConviviaTM, Raptor Pharmaceuticals Corp. issued to Convivia 200,000 shares of its common stock, an additional 200,000 shares of its common stock to a third party in settlement of a convertible loan between the third party and Convivia, and another 37,500 shares of its 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 100,000 shares of its 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, Raptor Pharmaceuticals Corp. issued to Mr. Daley 100,000 shares of its common stock valued at \$27,000 and a \$30,000 cash bonus as a result of fulfilling a clinical milestone. Due to the 2009 Merger, the 200,000, 200,000, 37,500, 100,000 and 100,000 shares Raptor Pharmaceuticals Corp., respectively, described above, became 46,625, 46,625, 8,742, 23,312 and 23,312 shares of our common stock, respectively.

#### Purchase of DR Cysteamine

In December 2007, prior to the 2009 Merger, through a merger between Encode Pharmaceuticals, Inc., or Encode, and Raptor Therapeutics, Raptor Pharmaceuticals Corp. purchased certain assets, including the clinical development rights to DR Cysteamine. Under the terms of and subject to the conditions set forth in the merger agreement, Raptor Pharmaceuticals Corp. issued 3,444,297 shares of its common stock to the stockholders of Encode, or Encode Stockholders, options, or Encode Options, to purchase up to, in the aggregate, 357,427 shares of its common stock to the optionholders of Encode, or Encode Optionholders,

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and warrants, or Encode Warrants, to purchase up to, in the aggregate, 1,098,276 shares of its common stock to the warrant holders of Encode, or Encode Warrant holders, and together with the Encode Stockholders and Encode Option holders, referred to herein collectively as the Encode Securityholders, as of the date of such agreement. Due to the 2009 Merger, the 3,444,296 shares of Raptor Pharmaceuticals Corp.'s common stock, the 357,427 Encode Options and 1,098,276 Encode Warrants, respectively, became 802,946 shares of our common stock, Encode Options to purchase 83,325 shares of our common stock and Encode Warrants to purchase 256,034 shares of our common stock, respectively. 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, 2009 by spending approximately \$4.1 million on such programs) pursuant to the License Agreement. To-date, we have paid \$270,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis and in 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 consolidated financial statements is critical to an understanding of our consolidated financial position.

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

#### Fair Value of Financial Instruments

The carrying amounts of certain of our financial instruments including cash and cash equivalents, prepaid expenses, accounts payable, accrued liabilities and capital lease liability approximate fair value due to their short maturities.

#### Cash and Cash Equivalents

We consider all highly liquid investments with original maturities of three months or less to be cash equivalents.

### Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine, to the RAP technology and to the out-license and the rights to NGX 426 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 NGX 426, which has been classified as in-process research and development, will not be amortized until development is completed.

### 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 we issued in our December 2009 equity financing contain a conditional obligation that may require us to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under Accounting Standards Codification or ASC Topic 480, Distinguishing Liabilities from Equity, or ASC 480, a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, we have classified the warrants as liabilities and will mark them to fair value at each period end.

#### Marking-to-Market

The common stock warrants issued in the our December 2009 equity financing are classified as liabilities under ASC 480 and are, therefore, re-measured at the end of every reporting period with the change in value reported in our condensed consolidated statements of operations.

#### 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 scientists' salaries, 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.

### 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.

### 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 new 2010 Plan.

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 it 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 Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or 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 SEC 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 month period ended February 28, 2010, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 3.1%; 7 year expected life; 55% volatility; 10% 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 years; the expected life 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 the over the counter bulletin board; the turnover rate was based on our assessment of our size and the minimum potential for employee turnover at our current development-stage; 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

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.

On November 10, 2005, the FASB issued ASC 718 (previously listed as FASB Staff Position, or FSP, No. FAS 123(R)-3 Transition Election Related to Accounting for Tax Effects of Share-Based Payment Awards). ASC 718 provides a practical transition election related to the accounting for the tax effects of share-based payment awards to employees, as an alternative to the transition guidance for the additional paid-in capital pool, or the APIC pool. The alternative transition method includes simplified methods to establish the beginning balance of the APIC pool related to the tax effects of employee stock-based compensation, and to determine the subsequent impact on the APIC pool and statements of cash flows of the tax effects of employee stock-based compensation awards that are outstanding upon adoption of ASC 718. The guidance in ASC 718 is effective after November 10, 2005. We may take up to one year from the later of adoption of ASC 718 or the effective date of this section of ASC 718 to evaluate our available transition alternatives and make our one-time election. We have elected the “short-form” method to calculating excess tax benefits available for use in offsetting future tax shortfalls in accordance with ASC 718.

## Results of Operations

Three months ended February 28, 2010 and 2009

## General and Administrative

General and administrative expenses (including officer and employee compensation allocated to general and administrative expenses) for the three months ended February 28, 2010 increased by approximately \$377,000 compared to the same period of the prior year. The increase was primarily due to:

| Reason for Variance  | Variance in<br>\$ Thousands |
|--|-----------------------------|
| Additional proxy expenses regarding 2009 Merger                                | 178                         |
| Patent application expenses for preclinical product candidates                 | 133                         |
| Expenses related to drug stability and maintenance of records for pain program | 67                          |
| Legal expenses for clinical trial agreements and licenses                      | 63                          |
| Additional administrative consulting for Dutch subsidiary                      | 36                          |
| Additional accounting and professional fees regarding 2009 Merger              | 23                          |
| Travel for clinical business development                                       | 11                          |
| Additional rent and office expenses for satellite office                       | 11                          |
| Benefits for CMO hired in April 2009   | 11                          |
| New Board member fees appointed in September 2009                              | 10                          |
| Decrease in preclinical travel   | (4)                         |
| Reduction of preclinical administrative consulting                             | (5)                         |
| Assets fully depreciated in 2009   | (6)                         |
| Reduction of D&O insurance limits  | (7)                         |
| No recruiting fees in Q1 2010  | (7)                         |
| Ending of benefits for employee voluntary termination in Oct. 2009             | (14)                        |
| Reduction of clinical patent expenses  | (14)                        |
| Increase in overhead allocation to R&D   | (41)                        |
| Three-year stock options no longer expensed                                    | (68)                        |
| General and Administrative variance Quarter to Date                            |                             |
| Second Quarter   |                             |
| 2010 vs. Second Quarter 2009   | 377                         |

## Research and Development

Research and development expenses (including officer and employee compensation allocated to research and development) for the three months ended February 28, 2010 increased by approximately \$508,000 over the prior year primarily due to:

| Reason for Variance   | Variance in \$<br>Thousands |
|---|-----------------------------|
| Manufacture of DR Cysteamine for cystinosis and Huntington's clinical trials  | 250                         |
| CRO fees incurred preparing for Phase III cystinosis trial and for close out of Phase IIb cystinosis clinical trial | 247                         |
|   | 146                         |



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|   |      |
|---|------|
| Clinical costs for Phase IIb cystinosis trial and preparing for Phase III cystinosis trial    |      |
| Hiring of CMO in April 2009   | 85   |
| Increase in allocation of G&A overhead expenses to R&D  | 41   |
| Travel by CMO hired in April 2009   | 27   |
| NeuroTrans issued patent costs  | 17   |
| Three-year stock options no longer expensed   | (16) |
| Employee voluntarily terminated in Oct. 2009  | (22) |
| Reduction of preclinical R&D consultants  | (27) |
| No HepTide or WntTide materials in 2010   | (67) |
| No HepTide or WntTide preclinical studies in 2010   | (78) |
| Reduction of R&D consultants replaced by CMO and Director of Program Mgmt.                    | (95) |
| Research and Development variance Quarter to Date Second Quarter 2010 vs. Second Quarter 2009 | 508  |

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

| Major Program (stage of development)                      | Estimated next 12 months | Cumulative through February 28, 2010 | Three month periods ended February 28, |            | Six month periods ended February 28, |            |
|---|--------------------------|--------------------------------------|--|------------|--------------------------------------|------------|
|   |                          |                                      | 2010                                   | 2009       | 2010                                 | 2009       |
| DR Cysteamine – All Indications (clinical)                | 7.0                      | 7.7                                  | 1.5                                    | 1.0        | 2.7                                  | 1.8        |
| Convivia™ (clinical)                                      | 0.1                      | 2.3                                  | 0.1                                    | 0.1        | 0.2                                  | 0.3        |
| HepTide™ (preclinical)                                    | -                        | 1.6                                  | -                                      | 0.1        | -                                    | 0.2        |
| NeuroTrans™ (preclinical)                                 | -                        | 0.3                                  | -                                      | -          | -                                    | 0.1        |
| WntTide™ (preclinical)                                    | -                        | 0.4                                  | -                                      | -          | 0.1                                  | 0.1        |
| Minor or Inactive Programs                                | -                        | 0.7                                  | -                                      | 0.1        | 0.1                                  | 0.1        |
| R & D Personnel and Other Costs Not Allocated to Programs | 2.0                      | 5.9                                  | 0.5                                    | 0.4        | 1.0                                  | 0.9        |
| <b>Total Research &amp; Development Expenses</b>          | <b>9.1</b>               | <b>19.0</b>                          | <b>2.2</b>                             | <b>1.7</b> | <b>4.1</b>                           | <b>3.5</b> |

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

| Major Program (stage of development)       | Estimated next 12 months | Cumulative through February 28, 2010 | Three month periods ended February 28, |      | Six month periods ended February 28, |      |
|--|--------------------------|--------------------------------------|--|------|--------------------------------------|------|
|  |                          |                                      | 2010                                   | 2009 | 2010                                 | 2009 |
| DR Cysteamine – All Indications (clinical) | 0.06                     | 0.22                                 | 0.02                                   | 0.04 | 0.03                                 | 0.08 |
| Convivia™ (clinical)                       | 0.01                     | 0.12                                 | -                                      | -    | 0.03                                 | -    |
| HepTide™ (preclinical)                     | 0.04                     | 0.32                                 | 0.09                                   | -    | 0.15                                 | -    |
| NeuroTrans™ (preclinical)                  | 0.03                     | 0.16                                 | -                                      | 0.02 | 0.02                                 | 0.02 |
| WntTide™ (preclinical)                     | 0.01                     | 0.10                                 | 0.04                                   | -    | 0.04                                 | -    |

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).

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 will need significant additional funding in order to pursue our plans for 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 Part II Item 1A of this Quarterly Report on Form 10-Q titled “Risk Factors” for further discussion about the risks and uncertainties pertaining to drug development.

#### Current Status of Major Programs

Please refer to the section titled, “Future Activities” above in 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 IIb clinical trial to study DR Cysteamine in cystinosis

patients. In October 2009, we announced the data from the treatment portion of our NASH Phase IIa clinical trial. Our NASH clinical trial (post-treatment phase) is complete and data will be presented by clinicians in May 2010. We anticipate studying DR Cysteamine in a Phase III clinical trial in cystinosis patients and a Phase IIa clinical trial in HD patients in the second quarter of 2010.

Our Convivia™ product candidate completed its initial clinical study in 2008 and we are in negotiations with a potential development partner for further development of our Convivia™ product candidate in Asia where its potential market exists. We are seeking to out-license our Tezampanel and NGX426 product candidates and no development costs will be incurred for the pain indication. 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 decreased by \$1,040 for the three months ended February 28, 2010 compared to the same period of the prior year due to a decrease in money market balances.

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## Interest Expense

Interest expenses for the three months ended February 28, 2010 and 2009 were nominal.

## Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants increased by \$(1.04) million for the three months ended February 28, 2010 compared to the same period of the prior year due to the fact that there was no warrant liability recorded in the prior year.

## Six months ended February 28, 2010 and 2009

## General and Administrative

General and administrative expenses (including officer and employee compensation allocated to general and administrative expenses) for the six months ended February 28, 2010 increased by approximately \$725,000 compared to the same period of the prior year. The increase was primarily due to:

| Reason for Variance   | Variance in \$<br>Thousands |
|---|-----------------------------|
| Additional proxy expenses regarding 2009 Merger   | 214                         |
| Patent application expenses related to preclinical product candidates                           | 196                         |
| Expenses related to drug stability and maintenance of records for pain program                  | 171                         |
| Additional accounting and professional fees regarding 2009 Merger                               | 102                         |
| Legal expenses for clinical trial agreements and licenses                                       | 90                          |
| NASDAQ fees related to 2009 Merger  | 80                          |
| Consulting fee related to 2009 Merger   | 53                          |
| Transfer agent fees related to 2009 Merger  | 39                          |
| Additional administrative consulting for financial statement footnotes                          | 28                          |
| Benefits for CMO hired April 2009   | 17                          |
| Franchise taxes increase due to increased assets  | 12                          |
| New Board member fees appointed in September 2009   | 10                          |
| Additional travel for business development  | 9                           |
| Additional bank fees for credit card charges  | 5                           |
| Additional rent and expenses for satellite office   | 4                           |
| Increase in overhead allocation to R&D  | (8)                         |
| Decrease in preclinical travel  | (11)                        |
| Assets fully depreciated in 2009  | (12)                        |
| Reduction of D&O insurance limits   | (13)                        |
| Ending of benefits for employee terminated in Oct. 2009   | (14)                        |
| Reduction of clinical patent expenses   | (16)                        |
| No recruiting fees in Q1 2010   | (42)                        |
| No bonuses in Q1 2010   | (67)                        |
| Three-year stock options no longer expensed   | (122)                       |
| General and Administrative variance Year to Date Second Quarter<br>2010 vs. Second Quarter 2009 | 725                         |

Research and Development

Research and development expenses (including officer and employee compensation allocated to research and development) for the six months ended February 28, 2010 increased by approximately \$621,000 over the prior year primarily due to:

| Reason for Variance   | Variance in \$<br>Thousands |
|---|-----------------------------|
| Manufacture of DR Cysteamine for cystinosis and Huntington's clinical trials  | 5                           |
| Clinical costs of Phase IIb cystinosis trial and preparing for Phase III cystinosis trial                           | 3                           |
| CRO fees incurred preparing for Phase III cystinosis trial and for close out of Phase IIb cystinosis clinical trial | 2                           |
| Hiring of CMO in April 2009   | 1                           |
| Assay development for DR Cysteamine in 2010   | 1                           |
| Travel by CMO hired in April 2009   | 1                           |
| NeuroTrans issued patent costs  | 1                           |



|  |       |
|--|-------|
| Increase in allocation of G&A overhead expenses to R&D                                     | 8     |
| Three-year stock options no longer expensed  | (29)  |
| No HepTide or WntTide preclinical studies in 2010  | (39)  |
| Employee voluntarily terminated in Oct. 2009   | (41)  |
| Reduction of preclinical R&D consultants   | (46)  |
| No preclinical studies of DR Cysteamine in 2010  | (116) |
| No HepTide or WntTide materials in 2010  | (157) |
| No milestone payments to UCSD for DR Cysteamine in 2010                                    | (180) |
| Reduction of R&D consultants replaced by CMO and Director of Program Mgmt.                 | (222) |
| Research and Development variance Year to Date Second Quarter 2010 vs. Second Quarter 2009 | 621   |

#### Interest Income

Interest income decreased by \$19,554 for the six months ended February 28, 2010 compared to the same period of the prior year due to the decrease in money market balances.

#### Interest Expense

Interest expenses for the six months ended February 28, 2010 and 2009 were nominal.

#### Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants increased by \$(1.04) million for the six months ended February 28, 2010 compared to the same period of the prior year due to the fact that there was no warrant liability recorded in the prior year.

#### Liquidity and Capital Resources

##### Capital Resource Requirements

As of February 28, 2010, we had approximately \$4.6 million in cash, approximately \$4.4 million in current liabilities and approximately \$0.2 million of net working capital. Our forecasted average monthly cash expenditures for the next twelve months are approximately \$0.95 million.

We believe our cash and cash equivalents at February 28, 2010 will be sufficient to meet our obligations into the third calendar quarter of 2010. We are currently in the process of negotiating strategic partnerships and collaborations in order to fund our preclinical and clinical programs into 2011. We are also reviewing a few equity and debt financing proposals to provide potential funding beginning in the second calendar quarter of 2010. If we do not enter into any such partnership or collaboration agreement or equity or debt offering which provides significant additional capital for us in the next two months, we will be forced to scale down our expenditures as described herein or possibly cease operations.

Our recurring losses from operations and our accumulated deficit raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory

paragraph in its report on our consolidated financial statements for the year ended August 31, 2009 with respect to this uncertainty. We will need to generate significant revenue or raise additional capital to continue to operate as a going concern. In addition, the perception that we may not be able to continue as a going concern may cause others to choose not to deal with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

In April 2009, in order to reflect then-current market prices, Raptor Pharmaceuticals Corp. notified the holders of warrants purchased in the May/June 2008 private placement that it was offering, in exchange for such warrants, new warrants to purchase its common stock at an exercise price of \$0.30 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 warrants that were not exchanged prior to or on July 17, 2009 retained their original exercise prices of \$0.90 per share and original expiration date of May 21, 2010. Raptor Pharmaceuticals Corp. received approximately \$2.6 million of proceeds from warrant exercises that resulted in the issuance of 8,715,000 shares of its common stock pursuant to the exchange described above. On a post-2009 Merger basis, the warrants that were not exchanged prior to or on July 17, 2009 are warrants to purchase shares of our common stock at an exercise price of \$3.86 per share and continue to have an expiration date of May 21, 2010, and the 8,715,000 shares of Raptor Pharmaceuticals Corp.'s common stock described above are 2,031,670 shares of our common stock.

In August 2009, Raptor Pharmaceuticals Corp. entered into a Securities Purchase Agreement with four investors for the private placement of units at a purchase price of \$0.32 per unit. Each unit was comprised of one share of its common stock, par



value \$0.001 per share and one warrant to purchase one half of one share of its common stock. At the closing, Raptor Pharmaceuticals Corp. sold an aggregate of 7,456,250 units to the investors, comprised of an aggregate of 7,456,250 shares of its common stock and warrants to purchase up to in the aggregate, 3,728,125 shares of its common stock, for aggregate gross proceeds of \$2,386,000. The investor warrants, exercisable for two years from the closing, had an exercise price of \$0.60 per share

during the first year and \$0.75 per share during the second year, depending on when such investor warrants were exercised, if at all. On a post-2009 Merger basis, the 7,456,250 shares of Raptor Pharmaceuticals Corp.'s common stock described above are 1,738,226 shares of our common stock and the two-year warrants are warrants to purchase up to, in the aggregate, 869,113 shares of our common stock and have an exercise price of \$2.57 per share during the first year and \$3.22 per share during the second year, depending on when such investor warrants are exercised, if at all.

In December 2009, we entered into a definitive securities purchase agreement, or the Purchase Agreement, dated as of December 17, 2009, with 33 investors (collectively, the Investors) with respect to the sale of units, whereby, on an aggregate basis, the investors agreed to purchase 3,747,558 Units for a negotiated purchase price of \$2.00 per unit for aggregate gross proceeds of approximately \$7.5 million. Each unit consists of one share of our common stock, one Series A Warrant exercisable for 0.5 of a share of our common stock and one Series B Warrant exercisable for 0.5 of a share of our common stock. The shares of our common stock and the warrants were issued separately. The Series A Warrants 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. In connection with this offering we paid a placement agent cash compensation equal to 6.5% of the gross proceeds or \$487,183 plus a five-year warrant at an exercise price of \$2.50 for the purchase of up to 74,951 shares of our common stock.

There can be no assurance that we will be able to obtain funds required for our continued operation. There can be no assurance that additional financing will be available to us or, if available, that it can be obtained on commercially reasonable terms. If we are not able to obtain financing on a timely basis, we will not be able to meet our obligations as they become due and we will be forced to scale down or perhaps even cease the operation of our business. This also may be the case if we become insolvent or if we breach our asset purchase agreement with BioMarin or our licensing agreements with Washington University and UCSD due to non-payment (and we do not cure our non-payment within the stated cure period). If this happens, we would lose all rights to the RAP technology assigned to us by BioMarin and/or the rights to Mesd licensed to us by Washington University and/or the rights to DR Cysteamine licensed to us by UCSD, depending on which agreement is breached. If we lose our rights to the intellectual property related to the RAP technology purchased by us from BioMarin, our agreement with Roche would likely be terminated and any milestone or royalty payments from Roche to us would thereafter cease to accrue.

We will need to raise significant long-term financing in order to implement our 12 month operating plan. If we are able to raise significant additional funding within the next two months, for the next 12 months we intend to expend a total of approximately \$11.4 million to implement our operating plan of researching and developing our DR Cysteamine clinical programs, our RAP based platform, our licensed technologies, as well as continuing business development efforts for our other clinical-stage product candidates. Specifically, we estimate our operating expenses and working capital requirements for the next 12 months to be as follows:

| Estimated spending for the next 12 months:         | In millions |
|--|-------------|
| Research and development activities                | \$ 7.5      |
| Research and development compensation and benefits | 1.6         |

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|  |    |      |
|--|----|------|
| General and administrative activities                |    | 1.3  |
| General and administrative compensation and benefits |    | 1.0  |
| Capital expenditures                                 |    | -    |
| Total estimated spending for the next 12 months      | \$ | 11.4 |

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

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

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### Research and Development Activities

We plan to conduct further research and development, seeking to support several clinical trials for DR Cysteamine, improve upon our RAP-based and in-licensed technology and continue business development efforts for our other clinical-stage product candidates in the next 12 months. We plan to conduct research and development activities by our own laboratory staff and also by engaging contract research organizations, clinical research organizations and contract manufacturing organizations. We also plan to incur costs for the production of our clinical study drug candidate, DR Cysteamine, clinical trials, clinical and medical advisors and consulting and collaboration fees. Assuming we obtain additional long-term financing, we anticipate our research and development costs for the next 12 months, excluding in-house research and development compensation, will be approximately \$7.5 million. We will need to scale down our research and development plans and expenses detailed herein in the 12 months if we are not able to raise significant additional funding over the next two months as detailed in the section above titled, "Capital Resource Requirements."

### Officer and Employee Compensation

In addition to the three officers of TPTX, Inc., we currently employ five executive officers. Obligations to the TPTX, Inc. officers end in mid-April 2010, at which time such officers will no longer be employed by us. We also have one permanent scientific staff member, three permanent clinical development staff members and one permanent finance staff member. Assuming we obtain significant additional long-term financing, we anticipate spending up to approximately \$2.6 million in officer and employee compensation during the next 12 months, of which \$1.6 million is allocated to research and development expenses and \$1.0 million is allocated to general and administrative expenses. Cash received as a result of the 2009 Merger is sufficient to cover the TPTX, Inc. personnel expense obligations through mid-April 2010. We will need to scale down our officer and employee compensation expenses detailed herein in the next 12 months if we are not able to raise significant additional funding over the next two months as detailed in the section above titled, "Capital Resource Requirements."

### General and Administrative

Assuming we obtain additional long-term financing, we anticipate spending approximately \$1.3 million on general and administrative costs in the next 12 months. These costs will consist primarily of legal and accounting fees, patent legal fees, investor relations expenses, board fees and expenses, insurance, rent and facility support expenses, excluding finance and administrative compensation. We will need to scale down our general and administrative plans and expenses detailed herein in the next 12 months if we are not able to raise significant additional funding over the next two months as detailed in the section above titled, "Capital Resource Requirements."

### Capital Expenditures

We anticipate spending approximately \$20,000 in the next 12 months on capital expenditures for lab equipment and office furniture. We will need to scale down our capital expenditures detailed herein in the next 12 months if we are not able to raise significant additional funding over the next two months as detailed in the section above titled, "Capital Resource Requirements."

### Contractual Obligations with BioMarin

Pursuant to the terms of the asset purchase agreement we entered into with BioMarin for the purchase of intellectual property related to our RAP based technology (including NeuroTrans™), we are obligated to make the following milestone payments to BioMarin upon the achievement of the following events:

- \$50,000 (paid by us in June 2006) within 30 days after we receive total aggregate debt or equity financing of at least \$2,500,000;
- \$100,000 (paid by us in June 2006) within 30 days after we receive total aggregate debt or equity financing of at least \$5,000,000;
- \$500,000 upon our filing and acceptance of an investigational new drug application for a drug product candidate based on our NeuroTrans™ product candidate;
- \$2,500,000 upon our successful completion of a Phase II human clinical trial for a drug product candidate based on our NeuroTrans™ product candidate;
- \$5,000,000 upon our successful completion of a Phase III human clinical trial for a drug product candidate based on our NeuroTrans™ product candidate;

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- \$12,000,000 within 90 days of our obtaining marketing approval from the FDA or other similar regulatory agencies for a drug product candidate based on our NeuroTrans™ product candidate;
- \$5,000,000 within 90 days of our obtaining marketing approval from the FDA or other similar regulatory agencies for a second drug product candidate based on our NeuroTrans™ product candidate;
- \$5,000,000 within 60 days after the end of the first calendar year in which our aggregated revenues derived from drug product candidates based on our NeuroTrans™ product candidate exceed \$100,000,000; and
- \$20,000,000 within 60 days after the end of the first calendar year in which our aggregated revenues derived from drug product candidates based on our NeuroTrans™ product candidate exceed \$500,000,000.

In addition to these milestone payments, we are also obligated to pay BioMarin a royalty at a percentage of our aggregated revenues derived from drug product candidates based on our NeuroTrans™ product candidate. On June 9, 2006, we made a milestone payment in the amount of \$150,000 to BioMarin because we raised \$5,000,000 in our May 25, 2006 private placement financing. If we become insolvent or if we breach our asset purchase agreement with BioMarin due to non-payment and we do not cure our non-payment within the stated cure period, all of our rights to 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, or the Asset Purchase Agreement, that we entered into with Convivia, Inc. and Thomas E. Daley, pursuant to which we purchased intellectual property related to our 4-MP product candidate program, 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 us, as set forth below:

- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days after we enter into a manufacturing license or other agreement to produce any product that is predominantly based upon or derived from any assets purchased from Convivia, or Purchased Assets, in quantity, referred to as 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 our restricted, unregistered common stock. Should we obtain a second such license or agreement for a Product, Mr. Daley will be entitled to receive 11,656 shares of our restricted, unregistered common stock within 30 days of execution of such second license or other agreement. On March 31, 2008, Raptor Pharmaceuticals Corp. issued 100,000 shares of its common stock valued at \$56,000 to Mr. Daley pursuant to this milestone reflecting the execution of an agreement to supply the active pharmaceutical ingredient for Convivia<sup>TM</sup>, combined with the execution of a formulation agreement to produce the oral formulation of Convivia<sup>TM</sup>. Due to the 2009 Merger, the 100,000 shares Raptor Pharmaceuticals Corp. described above became 23,312 shares of our common stock.
- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days after we receive our first patent allowance on any patents which constitute part of the Purchased Assets in any one of certain predetermined countries, or a Major Market.
- 11,656 shares of our restricted, unregistered common stock within fifteen (15) days after we receive our second patent allowance on any patents which constitute part of the Purchased Assets different from the patent referenced in the immediately preceding bullet point above in a Major Market.
- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days of completion of predetermined benchmarks in a Major Market by us or our licensee of the first phase II human clinical trial for a Product, or 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 our restricted, unregistered common stock within thirty (30) days of such Successful Completion. In October 2008, Raptor Pharmaceuticals Corp. issued 100,000 shares of its 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. Due to the 2009 Merger, the 100,000 shares Raptor Pharmaceuticals Corp. described above became 23,312 shares of our common stock.

- 11,656 shares of our restricted, unregistered common stock within fifteen (15) days of a Successful Completion in a Major Market by us or our licensee of the second phase II human clinical trial for a Product (other than the Product for which a distribution is made under the immediately preceding bullet point above).

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- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days after we or our licensee applies for approval to market and sell a Product in a Major Market for the indications for which approval is sought, or Marketing Approval.
- 11,656 shares of our restricted, unregistered common stock within fifteen (15) days after we or our licensee applies for Marketing Approval in a Major Market (other than the Major Market for which a distribution is made under the immediately preceding bullet point above).
- 46,625 shares of our restricted, unregistered common stock within fifteen (15) days after we or our licensee obtains the first Marketing Approval for a Product from the applicable regulatory agency in a Major Market.
- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days after we or our 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 bullet point above).

As discussed above, in aggregate, Raptor Pharmaceuticals Corp. issued to Mr. Daley, 200,000 shares of its common stock valued at \$83,000 and paid \$30,000 in cash bonuses related to Convivia™ milestones along with another \$20,000 in cash bonuses related to employment milestones pursuant to Mr. Daley's employment agreement. Due to the 2009 Merger, such 200,000 shares Raptor Pharmaceuticals Corp. described above became 46,625 shares of our common stock.

#### Contractual Obligations with Former Encode Securityholders

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

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

the world.

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

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

Pursuant to the terms of the Encode Merger Agreement, an Encode stockholder was granted the right to demand the registration of its portion of the initial restricted, unregistered common stock issued to it in connection with the execution of the Encode Merger Agreement at any time following 140 days from the closing date of the merger with Encode and prior to the expiration of the fourth anniversary of the Encode Merger Agreement. To the extent that future milestones as described above are accomplished by us within five years from the effective time of the merger with Encode, we will be obligated to file a registration statement within 90 days covering such Encode stockholder's portion of such respective future restricted, unregistered common stock issued relating to such milestone payment.

#### Contractual Obligations with UCSD

As a result of the merger of our clinical subsidiary and Encode, we received the exclusive worldwide license to DR Cysteamine, or License Agreement for use in the field of human therapeutics for metabolic and neurologic disorders, developed by clinical scientists at the UCSD, School of Medicine. DR Cysteamine is a proprietary, delayed-release, enteric-coated microbead formulation of cysteamine bitartrate, a cystine depleting agent currently approved by the FDA. Cysteamine bitartrate is



prescribed for the management of the genetic disorder known as cystinosis, a lysosomal storage disease. The active ingredient in DR Cysteamine has also demonstrated potential in studies as a treatment for other metabolic and neurodegenerative diseases, such as HD and NASH.

In consideration of the grant of the license, prior to the merger, Encode paid an initial license fee and we will be 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 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 we begin commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, we are obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 years from the effective date of the License Agreement, depending on the indication. In addition, we are obligated to, among other things, annually spend at least \$200,000 for the development of products—which, as of August 31, 2009, we had spent approximately \$4.1 million on such programs—pursuant to the License Agreement. To-date, we have paid \$270,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis and in 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.

#### Contractual Obligations to TPTX, Inc. Employees

Pursuant to the documents related to the 2009 Merger, including amended employment agreements with the TPTX, Inc. employees, who were former executives of TorreyPines prior to the 2009 Merger, we were obligated to pay such former executives their salaries, benefits and other obligations through February 28, 2010, which obligations were extended through mid-April 2010. The remaining obligations as of February 28, 2010 are approximately \$166,000 in the aggregate.

#### Off-Balance Sheet Arrangements

We do not have any outstanding derivative financial instruments, off-balance sheet guarantees, interest rate swap transactions or foreign currency contracts. We do not engage in trading activities involving non-exchange traded contracts.

#### Reverse Acquisition

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

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

#### Going Concern

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

#### New Accounting Pronouncements

In September 2006, ASC Topic 820, Fair Value Measurements, or ASC 820 (previously listed as the FASB issued SFAS No. 157, Fair Value Measurements). ASC 820 defines fair value, establishes a framework for measuring fair value in accordance with generally accepted accounting principles, and expands disclosures about fair value measurements. ASC 820 does not require any new fair value measurements; rather, it applies under other accounting pronouncements that require or permit fair value measurements. The provisions of ASC 820 are to be applied prospectively as of the beginning of the fiscal year in which it is initially applied, with any transition adjustment recognized as a cumulative-effect adjustment to the opening balance of retained earnings. The provisions of ASC 820 are effective for fiscal years beginning after November 15, 2007; therefore, we adopted ASC 820 as of September 1, 2008 for financial assets and liabilities. In accordance with FASB Staff Position 157-2, Effective Date of ASC 820, we adopted the provisions of ASC 820 for our non-financial assets and non-financial liabilities on September 1, 2009 and have determined that it had no material impact on our results for the three and six months ended February 28, 2010. See Note 5, Fair Value Measurements, in our condensed consolidated financial statement footnotes, regarding the disclosure of the value of our cash equivalents.

In February 2007, the FASB issued ASC Topic 825, Financial Instruments, or ASC 825 (previously SFAS 159, The Fair Value Option for Financial Assets and Financial Liabilities, Including an Amendment of FASB Statement No. 115), which permits the measurement of many financial instruments and certain other asset and liabilities at fair value on an instrument-by-instrument basis (the fair value option). The guidance is applicable for fiscal years beginning after November 15, 2007; therefore, we adopted ASC 825 as of September 1, 2008. We have determined that ASC 825 had no material impact on our financial results for the three and six months ended February 28, 2010.

In June 2007, the EITF reached a consensus on ASC Topic 730, Research and Development, or ASC 730 (previously EITF No. 07-3, Accounting for Nonrefundable Advance Payments for Goods or Services to Be Used in Future Research and Development Activities). ASC 730 specifies the timing of expense recognition for non-refundable advance payments for goods or services that will be used or rendered for research and development activities. ASC 730 was effective for fiscal years beginning after December 15, 2007, and early adoption is not permitted; therefore, we adopted ASC 730 as of September 1, 2008. We have determined that ASC 730 had no material impact on our financial results for the three and six months ended February 28, 2010.

In December 2007, the EITF reached a consensus on ASC Topic 808, Collaborative Agreement, or ASC 808 (previously EITF 07-01, Accounting for Collaborative Arrangements). ASC 808 discusses the appropriate income statement presentation and classification for the activities and payments between the participants in arrangements related to the development and commercialization of intellectual property. The sufficiency of disclosure related to these arrangements is also specified. ASC 808 is effective for fiscal years beginning after December 15, 2008. As a result, ASC 808 is effective for us as of September 1, 2009. Based upon the nature of our business, ASC 808 could have a material impact on our financial position and consolidated results of operations in future years, but had no material impact for the three and six months ended February 28, 2010.

In December 2007, the FASB issued ASC Topic 805, Business Combinations, or ASC 805 (previously SFAS 141(R) and FASB ASC Topic 810, Consolidation, or ASC 810 (previously SFAS 160, Noncontrolling Interests in Consolidated Financial Statements, an amendment of ARB No. 51). These statements will significantly change the financial accounting and reporting of business combination transactions and non-controlling (or minority) interests in consolidated financial statements. ASC 805 requires companies to: (i) recognize, with certain exceptions, 100% of the fair values of assets acquired, liabilities assumed, and non-controlling interests in acquisitions of less than a 100% controlling interest when the acquisition constitutes a change in control of the acquired entity; (ii) measure acquirer shares issued in consideration for a business combination at fair value on the acquisition date; (iii) recognize contingent consideration arrangements at their acquisition-date fair values, with subsequent changes in fair value generally reflected in earnings; (iv) with certain exceptions, recognize pre-acquisition loss and gain contingencies at their acquisition-date fair values; (v) capitalize in-process research and development assets acquired; (vi) expense, as incurred, acquisition-related transaction costs; (vii) capitalize acquisition-related restructuring costs only if the criteria in ASC Topic 420, Exit and Disposal Cost Obligations, (previously SFAS No. 146, Accounting for Costs Associated with Exit or Disposal Activities), are met as of the acquisition date; and (viii) recognize changes that result from a business combination transaction in an acquirer's existing income tax valuation allowances and tax uncertainty accruals as adjustments to income tax expense. ASC 805 is required to be adopted concurrently with ASC 810 and is effective for business combination transactions for which the acquisition date is on or after the beginning of the first annual reporting period beginning on or after December 15, 2008 (our fiscal 2010). Early adoption of these statements is prohibited. We believe the adoption of these statements will have a material impact on significant acquisitions completed after September 1, 2009. See Note 9 to our condensed consolidated financial statements, which reflects the accounting treatment of our 2009 Merger utilizing these provisions.

In March 2008, the FASB issued ASC Topic 815, Derivatives and Hedging, or ASC 815 (previously SFAS No. 161, Disclosures about Derivative Instruments and Hedging Activities). This statement will require enhanced disclosures about derivative instruments and hedging activities to enable investors to better understand their effects on an entity's financial position, financial performance, and cash flows. It is effective for financial statements issued for fiscal years

and interim periods beginning after November 15, 2008, with early application encouraged. We adopted ASC 815 on December 1, 2008 and have determined that ASC 815 had no material impact on our financial results for the three and six months ended February 28, 2010.

In May 2008, the FASB released ASC Topic 470, Debt, or ASC 470 (previously FSP APB 14-1 Accounting For Convertible Debt Instruments That May Be Settled in Cash Upon Conversion (Including Partial Cash Settlement)), which alters the accounting treatment for convertible debt instruments that allow for either mandatory or optional cash settlements. ASC 470 specifies that issuers of such instruments should separately account for the liability and equity components in a manner that will reflect the entity's nonconvertible debt borrowing rate when interest cost is recognized in subsequent periods. Furthermore, it would require recognizing interest expense in prior periods pursuant to retrospective accounting treatment. ASC 470 is effective for financial statements issued for fiscal years beginning after December 15, 2008; therefore, we adopted ASC 470 as of September 1, 2009. We have determined that ASC 470 had no material impact on our condensed consolidated financial statements for the three and six months ended February 28, 2010.

In June 2008, the FASB issued FASB ASC Topic 815, Derivatives and Hedging, or ASC 815 (previously EITF 07-5, Determining Whether an Instrument (or an Embedded Feature) is Indexed to an Entity's Own Stock). ASC 815 requires entities to evaluate whether an equity-linked financial instrument (or embedded feature) is indexed to its own stock by assessing the

instrument's contingent exercise provisions and settlement provisions. Instruments not indexed to their own stock fail to meet the scope exception of SFAS No. 133, Accounting for Derivative Instruments and Hedging Activities, paragraph 11(a), and should be classified as a liability and marked-to-market. The statement is effective for fiscal years beginning after December 15, 2008 and interim periods within those fiscal years and is to be applied to outstanding instruments upon adoption with the cumulative effect of the change in accounting principle recognized as an adjustment to the opening balance of retained earnings. We adopted ASC 815 as of September 1, 2009 and have determined that ASC 815 had no material impact on our condensed consolidated statement of operations for the three and six months ended February 28, 2010.

In April 2008, the FASB issued ASC Topic 350, Intangibles – Goodwill and Other, or ASC 350 (previously FSP SFAS No. 142-3, Determination of the Useful Life of Intangible Assets). ASC 350 provides guidance with respect to estimating the useful lives of recognized intangible assets acquired on or after the effective date and requires additional disclosure related to the renewal or extension of the terms of recognized intangible assets. ASC 350 is effective for fiscal years and interim periods beginning after December 15, 2008. We adopted ASC 350 as of September 1, 2009 and have determined that ASC 350 had no material impact on our condensed consolidated financial statements for the three and six months ended February 28, 2010.

In May 2009, the FASB issued ASC Topic 855, Subsequent Events, or ASC 855 (previously SFAS No. 165, Subsequent Events). ASC 855 establishes general standards of accounting for and disclosure of events that occur after the balance sheet date but before financial statements are issued or are available to be issued. ASC 855 defines the period after the balance sheet date during which management of a reporting entity should evaluate events or transactions that may occur for potential recognition or disclosure in the financial statements, and the circumstances under which an entity should recognize events or transactions occurring after the balance sheet date in its financial statements. ASC 855 is effective for fiscal years and interim periods ending after June 15, 2009. We adopted ASC 855 as of August 31, 2009 and anticipate that the adoption will impact the accounting and disclosure of future transactions. Our management has evaluated and disclosed subsequent events from the balance sheet date of February 28, 2010 through the day before the date that these condensed consolidated financial statements were included in our Quarterly Report on Form 10-Q and filed with the SEC.

ASC Topic 825, Financial Instruments, or ASC 825 (previously FSP FAS 107-1 and APB 28-1 amends FASB Statement No. 107, Disclosures about Fair Value of Financial Instruments), to require disclosures about the fair value of financial instruments for interim reporting periods of publicly traded companies as well as in annual financial statements. This ASC 825 also amends APB Opinion No. 28, Interim Financial Reporting, to require those disclosures in summarized financial information at interim reporting periods. The adoption of ASC 825 did not have a material impact on our condensed consolidated financial statements for the three and six months ended February 28, 2010.

In June 2009, the FASB issued SFAS No. 167, Amendments to FASB Interpretation No. 46(R), or SFAS 167, which has not yet been codified in the ASC. The amendments include: (i) the elimination of the exemption for qualifying special purpose entities, (ii) a new approach for determining who should consolidate a variable-interest entity, and (iii) changes to when it is necessary to reassess who should consolidate a variable-interest entity. This statement is effective for fiscal years beginning after November 15, 2009, and for interim periods within that first annual reporting period. We are currently evaluating the impact of this standard, however, we do not expect SFAS 167 will have a material impact on our condensed consolidated financial statements.

In June 2009, the FASB issued ASC Topic 105, Generally Accepted Accounting Standards, or ASC 105 (previously SFAS No. 168, The FASB Accounting Standards Codification<sup>TM</sup> and the Hierarchy of Generally Accepted Accounting Principles, a replacement of FASB Statement No. 162), or the Codification. The Codification, which was launched on July 1, 2009, became the single source of authoritative nongovernmental U.S. GAAP, superseding existing FASB, American Institute of Certified Public Accountants, EITF and related literature. The Codification

eliminates the GAAP hierarchy contained in ASC 105 and establishes one level of authoritative GAAP. All other literature is considered non-authoritative. ASC 105 is effective for financial statements issued for interim and annual periods ending after September 15, 2009. We adopted ASC 105 as of September 1, 2009 however, references to both current GAAP and the Codification are included in this filing. We have determined that this provision had no material impact on our condensed consolidated financial statements for the three and six months ended February 28, 2010.

In June 2009, the FASB issued ASC Topic 860, Transfers and Servicing (Statement No. 166, Accounting for Transfers of Financial Assets — an amendment of FASB Statement No. 140), or ASC 860. The guidance removes the concept of a qualifying special purpose entity and changes the requirements for derecognizing financial assets. Many types of transferred financial assets that would have been derecognized previously are no longer eligible for derecognition. The guidance is effective for financial statements issued for fiscal years and interim periods beginning after November 15, 2009, and early adoption is prohibited. The guidance applies prospectively to transfers of financial assets occurring on or after the effective date. We are currently assessing the impact of ASC 860 and do not expect the adoption of this guidance to have a material impact on our condensed consolidated financial statements.

In January 2010, FASB issued Accounting Standards Update, or ASU, 2010-06, Fair Value Measurements and Disclosures (Topic 820): Improving Disclosures about Fair Value Measurements, or ASU 2010-6. The ASU amends Subtopic 820-10 with new disclosure requirements and clarification of existing disclosure requirements. New disclosures required include the amount of significant transfers in and out of levels 1 and 2 fair value measurements and the reasons for the transfers. In addition, the reconciliation for level 3 activity will be required on a gross rather than net basis. The ASU provides additional guidance related to the level of disaggregation in determining classes of assets and liabilities and disclosures about inputs and valuation techniques. The amendments are effective for annual or interim reporting periods beginning after December 15, 2009, except for the requirement to provide the reconciliation for level 3 activity on a gross basis, which will be effective for fiscal years beginning after December 15, 2010. We are currently assessing the impact of ASU 2010-6 and do not expect the adoption of this guidance to have a material impact on our condensed consolidated financial statements.

### Item 3: Quantitative And Qualitative Disclosures About Market Risk

#### Interest Rate Market Risk

Our exposure to market risk for changes in interest rates relates primarily to our investment portfolio. By policy, we place our investments with highly rated credit issuers and limit the amount of credit exposure to any one issuer. As stated in our policy, we seek to improve the safety and likelihood of preservation of our invested funds by limiting default risk and market risk.

We mitigate default risk by investing in high credit quality securities and by positioning our portfolio to respond appropriately to a significant reduction in a credit rating of any investment issuer or guarantor. The portfolio includes only marketable securities with active secondary or resale markets to ensure portfolio liquidity.

As of February 28, 2010, our investment portfolio does not include any investments with significant exposure to the subprime mortgage market issues. Based on our investment portfolio, which consists 100% of money market accounts, and interest rates at February 28, 2010, we believe that a 100 basis point decrease in interest rates could result in a potential loss of future interest income of approximately \$43,000 annually; however such a decrease would have no effect on the fair value of the money market principal balances.

Of our total consolidated cash and cash equivalent balance of approximately \$4.6 million as of February 28, 2010, our money market balances represent \$4.3 million, or 93%.

Our debt obligations consist of our capital lease to finance our photocopier, which carries a fixed imputed interest rate and, as a result, we are not exposed to interest rate market risk on our capital lease obligations. The carrying value of our capital lease obligation approximates its fair value at February 28, 2010.

### Item 4. CONTROLS AND PROCEDURES

#### Conclusion Regarding Effectiveness of Disclosure Controls and Procedures

As of February 28, 2010, we performed an evaluation of the effectiveness of our disclosure controls and procedures that are designed to ensure that the information required to be disclosed in the reports that we file or submit under Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is

accumulated and communicated to our management, including our principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Based on our evaluation, our management, including our Chief Executive Officer and Chief Financial Officer, has concluded that our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Exchange Act) as of February 28, 2010, were not effective at a reasonable assurance level solely with respect to the amendment to Form 8-K regarding new Item 5.07 (Submission of Matters to a Vote of Security Holders) that became effective on February 28, 2010. We held our annual meeting of stockholders on March 9, 2010 and filed a Current Report on Form 8-K with the SEC disclosing the results of such annual meeting of stockholders on April 7, 2010.

#### Changes in Internal Control Over Financial Reporting

During the most recent fiscal quarter, other than the addition of two bank accounts controlled by the former TorreyPines employees, which continue to be controlled by the former TorreyPines employees, there have not been any significant changes in our internal control over financial reporting or in other factors that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.



## PART II - OTHER INFORMATION

### Item 1. Legal Proceedings.

Several lawsuits were filed against us (as TorreyPines) in February 2005 in the U.S. District Court for the Southern District of New York asserting claims under Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 thereunder on behalf of a class of purchasers of our common stock during the period from June 26, 2003, through and including February 4, 2005, referred to as the class period. Dr. Marvin S. Hausman, M.D., a former director and a former Chief Executive Officer, and Dr. Gosse B. Bruinsma, M.D., also a former director and a former Chief Executive Officer, were also named as defendants in the lawsuits. These actions were consolidated into a single class action lawsuit in January 2006. On April 10, 2006, the class action plaintiffs filed an amended consolidated complaint. We filed our answer to that complaint on May 26, 2006. Our motion to dismiss the consolidated amended complaint was filed on May 26, 2006 and was submitted to the court for a decision in September 2006. On March 31, 2009 the U.S. District Court for the Southern District of New York dismissed the proceedings. On April 24, 2009, an appeal was filed with the United States Court of Appeals for the Second Circuit by the class action plaintiffs. Our response to such appeal was filed on October 23, 2009. On March 23, 2010, the United States Court of Appeals for the Second Circuit dismissed the plaintiffs' complaint and upheld the original dismissal of the complaint by the U.S. District Court for the Southern District of New York. We do not anticipate any additional action on this claim, however, if an appeal is filed by the plaintiffs to the United States Supreme Court and if such appeal is successful, it could burden the Company with additional liability above and beyond the insurance coverage provided under the insurance policy that we currently maintain.

Other than as described above, we know of no material, active or pending legal proceedings against us, nor are we involved as a plaintiff in any material proceedings or pending litigation. There are no proceedings in which any of our directors, officers or affiliates, or any registered or beneficial stockholders are an adverse party or have a material interest adverse to us.

### Item 1A. Risk Factors.

#### RISK FACTORS THAT MAY AFFECT FUTURE RESULTS

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

Risks Related to Our Business

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

Our condensed consolidated financial statements as of February 28, 2010 have been prepared assuming that we will continue as a going concern. As of February 28, 2010, we had an accumulated deficit of approximately \$29.0 million. We expect to continue to incur losses for the foreseeable future and will have to raise substantial cash to fund our planned operations. Our recurring losses from operations and our stockholders' deficit raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the year ended August 31, 2009, with respect to this uncertainty. We will need to generate significant revenue or raise additional capital to continue to operate as a going concern. In addition, the perception that we may not be able

to continue as a going concern may cause others to choose not to deal with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

We believe our cash and cash equivalents at February 28, 2010 will be sufficient to meet our obligations into the third calendar quarter of 2010. We are currently in the process of negotiating strategic partnerships and collaborations in order to fund our preclinical and clinical programs into 2011. We are also reviewing a few equity and debt financing proposals to provide potential funding beginning in the second calendar quarter of 2010. If we do not enter into any such partnership or collaboration agreement or equity offering, either of which will result in significant additional capital for us in the next two months, we will be forced to scale down our expenditures as described herein or possibly cease operations. We will need to sell equity or debt securities to raise significant additional funds. The sale of additional securities is likely to result in additional dilution to our stockholders. Additional financing may not be available in amounts or on terms satisfactory to us or at all. We may be unable to raise additional financing due to a variety of factors, including our financial condition, the status of our research and development programs, and the general condition of the financial markets. If we fail to raise significant additional financing, we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected and we may have to cease our operations.

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

- the progress, timing and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the time and cost necessary to respond to technological and market developments; and
- any changes made or new developments in our existing collaborative, licensing and other corporate relationships or any new collaborative, licensing and other commercial relationships that we may establish.

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

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

- financing facilities.

We are an early development stage company and have not generated any revenues to date and have a limited operating history. Many of our drug product candidates are in the concept stage and have not undergone significant testing in preclinical studies or any testing in clinical trials. Moreover, we cannot be certain that our research and development efforts will be successful or, if successful, that our drug product candidates will ever be approved for sale or generate commercial revenues. We have a limited relevant operating history upon which an evaluation of our performance and prospects can be made. We are subject to all of the business risks associated with a new enterprise, including, but not limited to, risks of unforeseen capital requirements, failure of drug product candidates either in preclinical testing or in clinical trials, failure to establish business relationships, and competitive disadvantages against larger and more established companies.

The current disruptions in the financial markets could affect our ability to obtain financing on favorable terms (or at all).

The U.S. credit markets have recently experienced historic dislocations and liquidity disruptions which have caused financing to be unavailable in many cases and, even if available, have caused the cost of prospective financings to increase. These circumstances have materially impacted liquidity in the debt markets, making financing terms for borrowers able to find financing less attractive, and in many cases have resulted in the unavailability of certain types of debt financing. Continued uncertainty in

the debt and equity markets may negatively impact our ability to access financing on favorable terms or at all. In addition, Federal legislation to deal with the current disruptions in the financial markets could have an adverse affect on our ability to raise other types of financing.

Even if we are able to develop our drug product candidates, we may not be able to receive regulatory approval, or if approved, we may not be able to generate significant revenues or successfully commercialize our products, which would adversely affect our financial results and financial condition and we would have to delay or terminate some or all of our research product development programs.

All of our drug product candidates are at an early stage of development and will require extensive additional research and development, including preclinical testing and clinical trials, as well as regulatory approvals, before we can market them. Since our inception in 1997, and since Raptor Pharmaceuticals Corp. began operations in 2005, both companies have dedicated substantially all of their resources to the research and development of their technologies and related compounds. All of our compounds currently are in preclinical or clinical development, and none have been submitted for marketing approval. Our preclinical compounds may not enter human clinical trials on a timely basis, if at all, and we may not develop any product candidates suitable for commercialization. We cannot predict if or when any of the drug product candidates we intend to develop will be approved for marketing. There are many reasons that we may fail in our efforts to develop our drug product candidates. These include:

- the possibility that preclinical testing or clinical trials may show that our drug product candidates are ineffective and/or cause harmful side effects;
- our drug product candidates may prove to be too expensive to manufacture or administer to patients;
- our drug product candidates may fail to receive necessary regulatory approvals from the FDA or foreign regulatory authorities in a timely manner, or at all;
- our drug product candidates, if approved, may not be produced in commercial quantities or at reasonable costs;
- our drug product candidates, if approved, may not achieve commercial acceptance;
- regulatory or governmental authorities may apply restrictions to our drug product candidates, which could adversely affect their commercial success; and
- the proprietary rights of other parties may prevent us or our potential collaborative partners from marketing our drug product candidates.

If we fail to develop our drug product candidates, our financial results and financial condition will be adversely affected, we will have to delay or terminate some or all of our research product development programs and may be forced to cease operations.

If we are limited in our ability to utilize acquired or licensed technologies, we may be unable to develop, out-license, market and sell our product candidates, which could cause delayed new product introductions, and/or adversely affect our reputation, any of which could have a material adverse effect on our business, prospects, financial condition, and operating results.

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

If the purchase or licensing agreements we entered into are terminated, we will lose the right to use or exploit our owned and licensed technologies, in which case we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected and we may have to cease our operations.

We entered into an asset purchase agreement with BioMarin Pharmaceutical Inc., or BioMarin, for the purchase of intellectual property related to the receptor-associated protein, or RAP, technology, a licensing agreement with Washington University for mesoderm development protein, or Mesd, and a licensing agreement with UCSD for DR Cysteamine. BioMarin, Washington University and UCSD may terminate their respective agreements with us upon the occurrence of certain events, including if we enter into certain bankruptcy proceedings or if we materially breach our payment obligations and fail to remedy the breach within the permitted cure periods. Although we are not currently involved in any bankruptcy proceedings or in breach of these agreements, there is a risk that we may be in the future, giving BioMarin, Washington University and UCSD the right to terminate their respective agreements with us. We have the right to terminate these agreements at any time by giving prior written notice. If the BioMarin, Washington University or UCSD agreements are terminated by either party, we would be forced to assign back to BioMarin, in the case of the BioMarin agreement, all of our rights, title and interest in and to the intellectual property related to the RAP technology, would lose our rights to the Mesd technology, in the case of the Washington University agreement and would lose our rights to DR Cysteamine, in the case of UCSD. Under such circumstances, we would have no further right to use or exploit the patents, copyrights or trademarks in those respective technologies. If this happens, we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected, and we may have to cease our operations. If we lose our rights to the intellectual property related to the RAP technology purchased by us from BioMarin, our agreement with Roche regarding the evaluation of therapeutic delivery across the blood-brain barrier utilizing NeuroTrans™ would likely be terminated and any milestone or royalty payments from Roche to us would thereafter cease to accrue.

If we fail to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop our drug product candidates.

Our competitors compete with us to attract established biotechnology and pharmaceutical companies or organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. Collaborations include licensing proprietary technology from, and other relationships with, academic research institutions. If our competitors successfully enter into partnering arrangements or license agreements with academic research institutions, we will then be precluded from pursuing those specific opportunities. Since each of these opportunities is unique, we may not be able to find a substitute. Other companies have already begun many drug development programs, which may target diseases that we are also targeting, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities.

Universities and public and private research institutions also compete with us. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our drug product candidates. We will attempt to license this proprietary technology, if available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products.

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

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

From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones will be based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. If we do not meet these milestones as publicly announced, our stockholders may lose confidence in our ability to meet these milestones and, as a result, the price of our common stock may decline.



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

It will take several years before we are able to develop marketable drug product candidates, if at all. Our product development programs will require substantial additional capital to successfully complete them, arising from costs to:

- conduct research, preclinical testing and human studies;
- establish pilot scale and commercial scale manufacturing processes and facilities; and
- establish and develop quality control, regulatory, marketing, sales, finance and administrative capabilities to support these programs.

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

- the pace of scientific progress in our research and development programs and the magnitude of these programs;
- the scope and results of preclinical testing and human clinical trials;
- our ability to obtain, and the time and costs involved in obtaining regulatory approvals;
- our ability to prosecute, maintain, and enforce, and the time and costs involved in preparing, filing, prosecuting, maintaining and enforcing patent claims;
- competing technological and market developments;
- our ability to establish additional collaborations;
- changes in our existing collaborations;
- the cost of manufacturing scale-up; and
- the effectiveness of our commercialization activities.

We base our outlook regarding the need for funds on many uncertain variables. Such uncertainties include the success of our research initiatives, regulatory approvals, the timing of events outside our direct control such as negotiations with potential strategic partners and other factors. Any of these uncertain events can significantly change our cash requirements as they determine such one-time events as the receipt or payment of major milestones and other payments.

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

Uncertainties regarding healthcare reform and third-party reimbursement may impair our ability to raise capital, form collaborations and if any of our product candidates become marketable, sell such products.

The continuing efforts of governmental and third-party payers to contain or reduce the costs of healthcare through various means may harm our business. For example, in some foreign markets, the pricing or profitability of healthcare products is subject to government control. In the United States, there have been, and we expect there will continue to be, a number of federal and state proposals to implement similar government control. The implementation or even the announcement of any of these legislative or regulatory proposals or reforms could harm our business if any of our product candidates become marketable by reducing the prices we or our partners are able to charge for our products (if marketable), impeding our ability to achieve profitability, raise capital or form collaborations. In addition, the availability of reimbursement from third-party payers determines, in large part, the demand for healthcare products in the United States and elsewhere. Examples of such third-party payers are government and private insurance plans. Significant uncertainty exists as to the reimbursement status of newly approved healthcare products and third-party payers are increasingly challenging the prices charged for medical products and services. If we succeed in bringing one or more products to the market, reimbursement from third-party payers may not be available or may not be sufficient to allow us to sell such products on a competitive or profitable basis.

If we fail to demonstrate efficacy in our preclinical studies and clinical trials our future business prospects, financial condition and operating results will be materially adversely affected.

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

Moreover, success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and we cannot be sure that the results of later clinical trials will replicate the results of prior clinical trials and preclinical testing. The clinical trial process may fail to demonstrate that our drug product candidates are safe for humans and effective for indicated uses. This failure would cause us to abandon a drug product candidate and may delay development of other drug product candidates. Any delay in, or termination of, our preclinical testing or clinical trials will delay the filing of our investigational new drug application, or IND, and new drug application, or NDA, as applicable, with the FDA and, ultimately, our ability to commercialize our drug product candidates and generate product revenues. In addition, some of our clinical trials will involve small patient populations. Because of the small sample size, the results of these early clinical trials may not be indicative of future results. Following successful preclinical testing, drug product candidates will need to be tested in a clinical development program to provide data on safety and efficacy prior to becoming eligible for product approval and licensure by regulatory agencies. From first clinical trial through product approval can take at least eight years, on average in the U.S.

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

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

our ability to sustain our development programs will become critically compromised. For example, efficacy or safety concerns may arise, whether or not justified, that could lead to the suspension or termination of our clinical programs.

Each clinical phase is designed to test attributes of drug product candidates and problems that might result in the termination of the entire clinical plan can be revealed at any time throughout the overall clinical program. The failure to demonstrate efficacy in our clinical trials would have a material adverse effect on our future business prospects, financial condition and operating results.

If we do not obtain the support of new, and maintain the support of existing, key scientific collaborators, it may be difficult to establish products using our technologies as a standard of care for various indications, which may limit our revenue growth and profitability and could have a material adverse effect on our business, prospects, financial condition and operating results.

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

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

We do not currently manufacture our drug product candidates and do not currently plan to develop the capacity to do so. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up initial production. These problems include difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Our third-party manufacturers and key suppliers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes, unstable political environments at foreign facilities or financial difficulties. If these manufacturers or key suppliers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to timely launch any potential product candidate, if approved, would be jeopardized.

In addition, all manufacturers and suppliers of pharmaceutical products must comply with cGMP requirements enforced by the FDA, through its facilities inspection program. The FDA is likely to conduct inspections of our third party manufacturer and key supplier facilities as part of their review of any of our NDAs. If our third party manufacturers and key suppliers are not in compliance with cGMP requirements, it may result in a delay of approval, particularly if these sites are supplying single source ingredients required for the manufacture of any potential product. These cGMP requirements include quality control, quality assurance and the maintenance of records and documentation. Furthermore, regulatory qualifications of manufacturing facilities are applied on the basis of the specific facility being used to produce supplies. As a result, if a manufacturer for us shifts production from one facility to another, the new facility must go through a complete regulatory qualification and be approved by regulatory authorities prior to being used for commercial supply. Our manufacturers may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. A failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall, or withdrawal of product approval. If the safety of any quantities supplied is compromised due to a our third party manufacturer's or key supplier's failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products.

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

As part of our business strategy, we intend to develop some drugs that may be eligible for FDA and European Union, or EU, orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, defined as a patient population of less than 200,000 in the U.S. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. Similar regulations are available in the EU with a 10-year period of market exclusivity.

Because the extent and scope of patent protection for some of our drug products is particularly limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs,

we plan to rely on the exclusivity period under Orphan Drug Act designation to maintain a competitive position. If we do not obtain orphan drug exclusivity for our drug products that do not have patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced.

Even though we have obtained orphan drug designation for DR Cysteamine for the potential treatment of nephropathic cystinosis, the potential treatment of HD and the potential treatment of Batten Disease and even if we obtain orphan drug designation for our future drug product candidates, due to the uncertainties associated with developing pharmaceutical products, we may not be the first to obtain marketing approval for any orphan indication. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

The fast-track designation for our drug product candidates, if obtained, may not actually lead to a faster review process and a delay in the review process or in the approval of our products will delay revenue from the sale of the products and will increase the capital necessary to fund these product development programs.

Although we have received Orphan Drug Designations from the FDA as described above, our drug product candidates may not receive an FDA fast-track designation or priority review. Without fast-track designation, submitting an NDA and getting through the regulatory process to gain marketing approval is a lengthy process. Under fast-track designation, the FDA may initiate review of sections of a fast-track drug's NDA before the application is complete. However, the FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, the fast-track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process. Under the FDA policies, a drug candidate is eligible for priority review, or review within a six-month time frame from the time a complete NDA is accepted for filing, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A fast-track designated drug candidate would ordinarily meet the FDA's criteria for priority review. The fast-track designation for our drug product candidates, if obtained, may not actually lead to a faster review process and a delay in the review process or in the approval of our products will delay revenue from the sale of the products and will increase the capital necessary to fund these product development programs.

Because the target patient populations for some of our products are small, we must achieve significant market share and obtain high per-patient prices for our products to achieve profitability.

Our clinical development of DR Cysteamine targets diseases with small patient populations, including nephropathic cystinosis and HD. If we are successful in developing DR Cysteamine and receive regulatory approval to market DR Cysteamine for a disease with a small patient population, the per-patient prices at which we could sell DR Cysteamine for these indications are likely to be relatively high in order for us to recover our development costs and achieve profitability. We believe that we will need to market DR Cysteamine for these indications worldwide to achieve significant market penetration of this product.

We may not be able to market or generate sales of our products to the extent anticipated.

Assuming that we are successful in developing our drug product candidates and receive regulatory clearances to market our products, our ability to successfully penetrate the market and generate sales of those products may be limited by a number of factors, including the following:

- Certain of our competitors in the field have already received regulatory approvals for and have begun marketing similar products in the U.S., the EU, Japan and other territories, which may result in greater physician awareness of their products as compared to ours.
- Information from our competitors or the academic community indicating that current products or new products are more effective than our future products could, if and when it is generated, impede our market penetration or decrease our future market share.

- Physicians may be reluctant to switch from existing treatment methods, including traditional therapy agents, to our future products.
- The price for our future products, as well as pricing decisions by our competitors, may have an effect on our revenues.
- Our future revenues may diminish if third-party payers, including private healthcare coverage insurers and healthcare maintenance organizations, do not provide adequate coverage or reimbursement for our future products.



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

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

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

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

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

We face significant competition from industry participants that are pursuing similar technologies that we are pursuing and are developing pharmaceutical products that are competitive with our drug product candidates. Nearly all of our industry competitors have greater capital resources, larger overall research and development staffs and facilities, and a longer history in drug discovery and development, obtaining regulatory approval and pharmaceutical product manufacturing and marketing than we do. With these additional resources, our competitors may be able to respond to the rapid and significant technological changes in the biotechnology and pharmaceutical industries faster than we can. Our future success will depend in large part on our ability to maintain a competitive position with respect to these

technologies. Rapid technological development, as well as new scientific developments, may result in our compounds, drug product candidates or processes becoming obsolete before we can recover any of the expenses incurred to develop them. For example, changes in our understanding of the appropriate population of patients who should be treated with a targeted therapy like we are developing may limit the drug's market potential if it is subsequently demonstrated that only certain subsets of patients should be treated with the targeted therapy.

Our reliance on third parties, such as collaborators, university laboratories, contract manufacturing organizations and contract or clinical research organizations, may result in delays in completing, or a failure to complete, preclinical testing or clinical trials if they fail to perform under our agreements with them.

In the course of product development, we may engage university laboratories, other biotechnology or companies or contract or clinical manufacturing organizations to manufacture drug material for us to be used in preclinical and clinical testing and collaborators and contract or clinical research organizations to conduct and manage preclinical studies and clinical trials. If we engage these organizations to help us with our preclinical and clinical programs, many important aspects of this process have been and will be out of our direct control. If any of these organizations we may engage in the future fail to perform their obligations under our agreements with them or fail to perform preclinical testing and/or clinical trials in a satisfactory manner, we may face delays in completing our clinical trials, as well as commercialization of any of our drug product candidates. Furthermore, any loss

or delay in obtaining contracts with such entities may also delay the completion of our clinical trials, regulatory filings and the potential market approval of our drug product candidates.

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

Licensing our product candidates from other companies, universities or individuals does not always prevent them from developing non-identical but competitive products for their own commercial purposes, nor from pursuing patent protection in areas that are competitive with us. While we seek patent protection for all of our owned and licensed product candidates, our licensors or assignors who created these product candidates are experienced scientists and business people who may continue to do research and development and seek patent protection in the same areas that led to the discovery of the product candidates that they licensed or assigned to us. By virtue of the previous research that led to the discovery of the drugs or product candidates that they licensed or assigned to us, these companies, universities, or individuals may be able to develop and market competitive products in less time than might be required to develop a product with which they have no prior experience and may reduce our future revenues from such product candidates.

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

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

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

The nature of our business exposes us to potential liability risks inherent in the testing, manufacturing and marketing of our drug product candidates. While we are in clinical stage testing, our drug product candidates could potentially harm people or allegedly harm people and we may be subject to costly and damaging product liability claims. Some of the patients who participate in clinical trials are already critically ill when they enter a trial. The waivers we obtain may not be enforceable and may not protect us from liability or the costs of product liability litigation. Although we currently carry a \$3 million clinical product liability insurance policy, it may not be sufficient to cover future claims. We currently do not have any clinical or product liability claims or threats of claims filed against us.

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

Our success is dependent in part upon the availability of our senior executive officers, including our Chief Executive Officer, Dr. Christopher M. Starr, our Chief Scientific Officer, Dr. Todd C. Zankel, our Chief Financial Officer, Kim R. Tsuchimoto, Ted Daley, the President of our clinical development subsidiary and Dr. Patrice P. Rioux, Chief Medical Officer of our clinical development subsidiary. The loss or unavailability to us of any of these individuals or key research and development personnel, and particularly if lost to competitors, could have a material adverse effect on our business, prospects, financial condition, and operating results. We have no key-man insurance on any of our employees. There is intense competition for qualified scientists and managerial personnel from numerous pharmaceutical and biotechnology companies, as well as from academic and government organizations, research

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

Our success depends on our ability to manage our growth.

If we are able to raise significant additional financing, we expect to continue to grow, which could strain our managerial, operational, financial and other resources. With the addition of our clinical-stage programs and with our plan to in-license and acquire additional clinical-stage product candidates, we will be required to retain experienced personnel in the regulatory, clinical and medical areas over the next several years. Also, as our preclinical pipeline diversifies through the acquisition or in-licensing of new molecules, we will need to hire additional scientists to supplement our existing scientific expertise over the next several years.

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

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

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

We will incur increased costs as a result of recently enacted and proposed changes in laws and regulations and our management will be required to devote substantial time to comply with such laws and regulations.

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

In addition, the Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. In particular, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. Our compliance with Section 404 will require that we incur substantial accounting and related expense and expend

significant management efforts. In the future, we may need to hire additional accounting and financial staff to satisfy the ongoing requirements of Section 404. Moreover, if we are not able to comply with the requirements of Section 404, or we or our independent registered public accounting firm identifies deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by NASDAQ, the SEC or other regulatory authorities.

We may be required to suspend, repeat or terminate our clinical trials if they do not meet regulatory requirements, the results are negative or inconclusive, or if the trials are not well designed, which may result in significant negative repercussions on our business and financial condition.

Before regulatory approval for any potential product can be obtained, we must undertake extensive clinical testing on humans to demonstrate the tolerability and efficacy of the product, both on our own terms, and as compared to the other principal drugs on the market that have the same therapeutic indication. We cannot provide assurance that we will obtain authorization to permit product candidates that are already in the preclinical development phase to enter the human clinical testing phase. In addition, we cannot provide assurance that any authorized preclinical or clinical testing will be completed successfully within any specified time period by us, or without significant additional resources or expertise to those originally expected to be necessary. We cannot provide assurance that such testing will show potential products to be safe and efficacious or that any such product will be approved for a specific indication. Further, the results from preclinical studies and early clinical trials may not be indicative of the results that will be obtained in later-stage clinical trials. In addition, we or regulatory authorities may suspend clinical trials at any time on the basis that the participants are being exposed to unacceptable health risks.

Completion of clinical tests depends on, among other things, the number of patients available for testing, which is a function of many factors, including the number of patients with the relevant conditions, the nature of the clinical testing, the proximity of patients to clinical testing centers, the eligibility criteria for tests as well as competition with other clinical testing programs involving the same patient profile but different treatments. We will rely on third parties, such as contract research organizations and/or co-operative groups, to assist us in overseeing and monitoring clinical trials as well as to process the clinical results and manage test requests, which may result in delays or failure to complete trials, if the third parties fail to perform or to meet the applicable standards. A failure by us or such third parties to keep to the terms of a product program development for any particular product candidate or to complete the clinical trials for a product candidate in the envisaged time frame could have significant negative repercussions on our business and financial condition.

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

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

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

- collaborative arrangements might not be on terms favorable to us;
- disagreements with partners may result in delays in the development and marketing of products, termination of collaboration agreements or time consuming and expensive legal action;

- we cannot control the amount and timing of resources partners devote to product candidates or their prioritization of product candidates, and partners may not allocate sufficient funds or resources to the development, promotion or marketing of our product candidates, or may not perform their obligations as expected;
- partners may choose to develop, independently or with other companies, alternative products or treatments, including products or treatments which compete with ours;
- agreements with partners may expire or be terminated without renewal, or partners may breach collaboration agreements with us;
- business combinations or significant changes in a partner's business strategy might adversely affect that partner's willingness or ability to complete their obligations to us; and
- the terms and conditions of the relevant agreements may no longer be suitable.

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



We may not complete our clinical trials in the time expected, which could delay or prevent the commercialization of our products, which may adversely affect our future revenues and financial condition.

Although for planning purposes we forecast the commencement and completion of clinical trials, the actual timing of these events can vary dramatically due to factors such as delays, scheduling conflicts with participating clinicians and clinical institutions and the rate of patient enrollment. Clinical trials involving our product candidates may not commence nor be completed as forecasted. In certain circumstances we will rely on academic institutions or clinical research organizations to conduct, supervise or monitor some or all aspects of clinical trials involving our product candidates. We will have less control over the timing and other aspects of these clinical trials than if we conducted them entirely on our own. These trials may not commence or be completed as we expect. They may not be conducted successfully. Failure to commence or complete, or delays in, any of our planned clinical trials could delay or prevent the commercialization of our product candidates and harm our business and may adversely affect our future revenues and financial condition.

If we fail to keep pace with rapid technological change in the biotechnology and pharmaceutical industries, our product candidates could become obsolete, which may adversely affect our future revenues and financial condition.

Biotechnology and related pharmaceutical technology have undergone and are subject to rapid and significant change. We expect that the technologies associated with biotechnology research and development will continue to develop rapidly. Our future will depend in large part on our ability to maintain a competitive position with respect to these technologies. Any compounds, products or processes that we develop may become obsolete before we recover any expenses incurred in connection with developing such products, which may adversely affect our future revenues and financial condition.

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

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

The patent positions of biopharmaceutical products are complex and uncertain.

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

- We do not know whether our patent applications will result in issued patents. For example, we may not have developed a method for treating a disease before others developed similar methods.
- Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us. Competitors may also claim that we are infringing on their patents and therefore cannot practice our technology as claimed under our patents, if issued. Competitors may also contest our patents, if issued, by showing the patent

examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose that patent. As a company, we have no meaningful experience with competitors interfering with our patents or patent applications.

- Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing drug product candidates, which could increase our operating expenses and delay product programs.
- Receipt of a patent may not provide much practical protection. If we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent.

- In addition, competitors also seek patent protection for their technology. Due to the number of patents in our field of technology, we cannot be certain that we do not infringe on those patents or that we will not infringe on patents granted in the future. If a patent holder believes our drug product candidate infringes on its patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe on their technology, we would face a number of issues, including the following:
  - Defending a lawsuit takes significant time and can be very expensive.
  - If a court decides that our drug product candidate infringes on the competitor's patent, we may have to pay substantial damages for past infringement.
- A court may prohibit us from selling or licensing the drug product candidate unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, we may have to pay substantial royalties or grant cross licenses to our patents.
- Redesigning our drug product candidates so we do not infringe may not be possible or could require substantial funds and time.

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

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

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

United States.

#### Risks Related to Our Common Stock

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

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

In addition, future sales of substantial amounts of our currently outstanding common stock in the public market, or the perception that such sales could occur, could adversely affect prevailing trading prices of our common stock, and could impair our ability to raise capital through future offerings of equity or equity-related securities. We cannot predict what effect, if any, future sales of our common stock, or the availability of shares for future sales, will have on the trading price of our common stock.

In May and June 2008, prior to our merger with Raptor Pharmaceuticals Corp. in 2009, pursuant to a securities purchase agreement for a private placement of units, Raptor Pharmaceuticals Corp. issued to investors in such private placement, 20,000,000 shares of its common stock and two-year warrants to purchase up to, in the aggregate, 10,000,000 shares of its common stock and to placement agents in such private placement, five-year warrants to purchase up to, in the aggregate, 2,100,000 shares of its common stock. On a post-merger basis, the 20,000,000 shares of Raptor Pharmaceuticals Corp.'s common stock, the two-year warrants to purchase up to, in the aggregate, 10,000,000 shares of Raptor Pharmaceuticals Corp.'s common stock and the five-year warrants to purchase up to, in the aggregate, 2,100,000 shares of Raptor Pharmaceuticals Corp.'s common stock, respectively, would be 4,662,468 shares of our common stock, two-year warrants to purchase up to, in the aggregate, 2,331,234 shares of our common stock and the five-year warrants to purchase up to, in the aggregate, 489,559 shares of our common stock, respectively.

In April 2009, in order to reflect then-current market prices, Raptor Pharmaceuticals Corp. notified the holders of warrants purchased in the May/June 2008 private placement that it was offering, in exchange for such warrants, new warrants to purchase its common stock at an exercise price of \$0.30 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 warrants that were not exchanged prior to or on July 17, 2009 retained their original exercise prices of \$0.90 per share and original expiration date of May 21, 2010. On a post-merger basis, the warrants that were not exchanged prior to or on July 17, 2009 would be warrants to purchase shares of our common stock at an exercise price of \$3.86 per share and would continue to have an expiration date of May 21, 2010. Raptor Pharmaceuticals Corp. received approximately \$2.6 million of proceeds from warrant exercises that resulted in the issuance of 8,715,000 shares of its common stock pursuant to the exchange described above. On a post-merger basis, the 8,715,000 shares of Raptor Pharmaceuticals Corp.'s common stock would be 2,031,670 shares of our common stock.

In August 2009, pursuant to a securities purchase agreement for a private placement of units, Raptor Pharmaceuticals Corp. issued to investors in such private placement, 7,456,250 shares of its common stock and two-year warrants to purchase up to, in the aggregate, 3,728,125 shares of its common stock and to placement agents in such private placement, a five-year warrant to purchase up to, in the aggregate, 556,500 shares of its common stock. On a post-merger basis, the 7,456,250 shares of Raptor Pharmaceuticals Corp.'s common stock, the two-year warrants to purchase up to, in the aggregate, 3,728,125 shares of Raptor Pharmaceuticals Corp.'s common stock and the five-year warrants to purchase up to, in the aggregate, 556,500 shares of Raptor Pharmaceuticals Corp.'s common stock, respectively, would be 1,738,226 shares of our common stock, two-year warrants to purchase up to, in the aggregate, 869,113 shares of our common stock and the five-year warrants to purchase up to, in the aggregate, 129,733 shares of our common stock, respectively.

In December 2009, we entered into a definitive securities purchase agreement or the Purchase Agreement, dated as of December 17, 2009, with 33 investors set forth on the signature pages thereto, collectively, the Investors, with respect to the offering of Units, whereby, on an aggregate basis, the Investors agreed to purchase 3,747,558 Units for a negotiated purchase price of \$2.00 per Unit for aggregate gross proceeds of approximately \$7.5 million. Each Unit

consists of one share of our common stock, one Series A Warrant exercisable for 0.5 of a share of our common stock and one Series B Warrant exercisable for 0.5 of a share of our common stock. Units will not be issued or certificated. The shares of our common stock and the Warrants will be 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. In connection with this offering we paid a placement agent cash compensation equaled to 6.5% of the gross proceeds or \$487,183 plus a a five-year warrant at an exercise price of \$2.50 for the purchase of up to 74,951 shares of our common stock, on the same terms as the investor warrants described above.

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

As of December 23, 2009, after the financing described in the previous paragraph, there were 22,579,515 shares of our common stock outstanding. We also had outstanding as of December 23, 2009 warrants that are exercisable to purchase an aggregate of 5,843,302 shares of our common stock at a weighted average exercise price of \$2.66 per share. On October 13, 2009, we filed a registration statement registering the resale of up to an aggregate of 5,557,865 shares of our common stock (including common stock issuable under warrants). Such registration statement was declared effective by the SEC on November 12, 2009.

In addition to our outstanding warrants, as of December 23, 2009, there were (i) options to purchase 1,037,688 shares of our common stock outstanding under our 2006 Raptor Pharmaceutical Equity Incentive Plan at a weighted-average exercise price of \$2.47, (ii) options to purchase 158,475 shares of our common stock outstanding under our 2006 TorreyPines Therapeutics Equity Incentive Plan at a weighted-average exercise price of \$114.12, (iii) 355,557 shares of our common stock available for issuance under our 2006 Raptor Pharmaceutical Equity Incentive Plan (of which all such shares are subject to approval by our stockholders at our 2010 Annual Meeting of stockholders) and (iv) 852,547 shares of our common stock available for issuance under our 2006 TorreyPines Therapeutics Equity Incentive Plan. The shares issuable under our equity incentive plans will be available for immediate resale in the public market. The shares issuable under the warrants are available for immediate resale in the public market. The market price of our common stock could decline as a result of such resales due to the increased number of shares available for sale in the market.

Our executive officers and directors are subject to lock-up agreements pursuant to the Purchase Agreement executed in our December 2009 financing. Each lock-up agreement is for a period of 90 days commencing on December 18, 2009, and represent 1,728,022 shares, or 7.7% of our outstanding common stock as of December 23, 2009 (taking into account the 3,747,558 shares of common stock sold in the December 2009 financing). The lock-up period expired in March 2010, which means that these stockholders have the ability to sell a substantial number of shares of common stock in the public market in a short period of time. Sales of a substantial number of shares of common stock in the public trading market, whether in a single transaction or a series of transactions, or the perception that these sales may occur, could also have a significant effect on volatility and the trading price of our common stock.

Future milestone payments, as more fully set forth under “Contractual Obligations with Thomas E. Daley (as assignee of the dissolved Convivia, Inc.)” and “Contractual Obligations with Former Encode Securityholders” discussed in certain of our periodic filings with the SEC relating to our acquisition of the Convivia assets and merger with Encode will result in dilution. We may be required to make additional contingent payments of up to 664,400 shares of our common stock, in the aggregate, under the terms of our acquisition of Convivia assets and merger with Encode, based on milestones related to certain future marketing and development approvals obtained with respect to Convivia and Encode product candidates. The issuance of any of these shares will result in further dilution to our existing stockholders.

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

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

Our stock price is volatile, which could result in substantial losses for our stockholders, and the trading in our common stock may be limited.

Our common stock is quoted on The Nasdaq Capital Market. The trading price of our common stock has been and may continue to be volatile. Our operating performance does and will continue to significantly affect the market price of our common stock. We face a number of risks including those described herein, which may negatively impact the price of our common stock.



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

- the results of our current and any future clinical trials of our drug candidates;
- the results of ongoing preclinical studies and planned clinical trials of our preclinical drug candidates;
- the entry into, or termination of, key agreements, including key strategic alliance agreements;
- the results and timing of regulatory reviews relating to the approval of our drug candidates;
- the initiation of, material developments in, or conclusion of litigation to enforce or defend any of our intellectual property rights;
- failure of any of our drug candidates, if approved, to achieve commercial success;
- general and industry-specific economic conditions that may affect our research and development expenditures;
- the results of clinical trials conducted by others on drugs that would compete with our drug candidates;
- issues in manufacturing our drug candidates or any approved products;
- the loss of key employees;
- the introduction of technological innovations or new commercial products by our competitors;
- Changes in estimates or recommendations by securities analysts, if any, who cover our common stock;
- future sales of our common stock;
- Changes in the structure of health care payment systems; and

- period-to-period fluctuations in our financial results.

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

Our stock is a penny stock. Trading of our stock may be restricted by the SEC's penny stock regulations and the FINRA's sales practice requirements, which may limit a stockholder's ability to buy and sell our stock.

Our common stock is a penny stock. The SEC has adopted Rule 15c-9 which generally defines "penny stock" to be any equity security that has a market price less than \$5.00 per share or an exercise price of less than \$5.00 per share, subject to certain exceptions. Our securities are covered by the penny stock rules, which impose additional sales practice requirements on broker-dealers who sell to persons other than established customers and institutional accredited investors. The penny stock rules require a broker-dealer, prior to a transaction in a penny stock not otherwise exempt from the rules, to deliver a standardized risk disclosure document in a form prepared by the SEC which provides information about penny stocks and the nature and level of risks in the penny stock market. The broker-dealer also must provide the customer with current bid and offer quotations for the penny stock, the compensation of the broker-dealer and its salesperson in the transaction and monthly account statements showing the market value of each penny stock held in the customer's account. The bid and offer quotations, and the broker-dealer and salesperson

compensation information, must be given to the customer orally or in writing prior to effecting the transaction and must be given to the customer in writing before or with the customer's confirmation. In addition, the penny stock rules require that prior to a transaction in a penny stock not otherwise exempt from these rules, the broker-dealer must make a special written determination that the penny stock is a suitable investment for the purchaser and receive the purchaser's written agreement to the transaction. These disclosure requirements may have the effect of reducing the level of trading activity in the secondary market for the stock that is subject to these penny stock rules. Consequently, these penny stock rules may affect the ability of broker-dealers to trade our securities. We believe that the penny stock rules discourage investor interest in and limit the marketability of our common stock.

In addition to the "penny stock" rules promulgated by the SEC, the Financial Industry Regulatory Authority, or FINRA, has adopted rules that require that in recommending an investment to a customer, a broker-dealer must have reasonable grounds for believing that the investment is suitable for that customer. Prior to recommending speculative low priced securities to their non-institutional customers, broker-dealers must make reasonable efforts to obtain information about the customer's financial status, tax status, investment objectives and other information. Under interpretations of these rules, the FINRA believes that there is a high probability that speculative low priced securities will not be suitable for at least some customers. The FINRA requirements make it more difficult for broker-dealers to recommend that their customers buy our common stock, which may limit your ability to buy and sell our stock.

An adverse determination, if any, in the class action suit in which we are a defendant, or our inability to obtain or maintain directors' and officers' liability insurance, could have a material adverse effect on us.

A class action securities lawsuit was filed against us, as described in the section titled, "Legal Proceedings" in certain of our periodic reports that we file with the SEC. On March 23, 2010, the United States Court of Appeals for the Second Circuit dismissed the plaintiffs' complaint and upheld the original dismissal of the complaint by the U.S. District Court for the Southern District of New York. We do not anticipate any additional action on this claim, however, if an appeal is filed by the plaintiffs to the United States Supreme Court and if such appeal is successful, it could burden the Company with additional liability above and beyond the insurance coverage provided under the insurance policy that we currently maintain as further described below. Furthermore, regardless of the outcome, we may incur significant defense costs, and the time and attention of our key management may be diverted from normal business operations. If we are ultimately required to pay significant defense costs, damages or settlement amounts, such payments could materially and adversely affect our operations and results. We have purchased liability insurance, however, if any costs or expenses associated with the litigation exceed the insurance coverage, we may be forced to bear some or all of these costs and expenses directly, which could be substantial and may have an adverse effect on our business, financial condition, results of operations and cash flows. In any event, publicity surrounding the lawsuits and/or any outcome unfavorable to us could adversely affect our reputation and stock price. The uncertainty associated with substantial unresolved lawsuits could harm our business, financial condition and reputation. We have certain obligations to indemnify our officers and directors and to advance expenses to such officers and directors. Although we have purchased liability insurance for our directors and officers, if our insurance carriers should deny coverage, or if the indemnification costs exceed the insurance coverage, we may be forced to bear some or all of these indemnification costs directly, which could be substantial and may have an adverse effect on our business, financial condition, results of operations and cash flows. If the cost of the liability insurance increases significantly, or if this insurance becomes unavailable, we may not be able to maintain or increase our levels of insurance coverage for our directors and officers, which could make it difficult to attract or retain qualified directors and officers.

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

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

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law as currently in effect may make a change in control of our Company more difficult, even if a change in control would be beneficial to the stockholders. Our board of directors has the authority to issue up to 15,000,000 shares of preferred stock, none of which are issued or outstanding. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Our charter contains provisions that may enable our management to resist an unwelcome takeover attempt by a third party, including:

a prohibition on actions by written consent of our stockholders; the fact that stockholder meetings must be called by our board of directors; and provisions requiring stockholders to provide advance notice of proposals. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, the board of directors approves the transaction. Our board of directors may use these provisions to prevent changes in the management and control of our Company. Also, under applicable Delaware law, our board of directors may adopt additional anti-takeover measures in the future.

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

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. (Removed and Reserved).

Item 5. Other Information.

None.



Item 6. Exhibits

Exhibit Index

|              |  |  |
|--------------|--|--|
| (2)          |  | Plan of acquisition, reorganization, arrangement, liquidation or succession  |
| 2.1          |  | Agreement and Plan of Merger and Reorganization, dated as of June 7, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Registration Statement No. 333-136018 filed on July 25, 2006).   |
| 2.2          |  | Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated as of August 25, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Amendment No. 1 to Registration Statement No. 333-136018 filed on August 25, 2006).  |
| 2.3          |  | Agreement and Plan of Merger and Reorganization, dated July 27, 2009, by and among Raptor Pharmaceuticals Corp., TorreyPines Therapeutics, Inc., a Delaware corporation, and ECP Acquisition, Inc., a Delaware corporation (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).                          |
| (3)(i), (ii) |  | Articles of incorporation; Bylaws  |
| 3.1          |  | Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).   |
| 3.2          |  | Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).   |
| 3.3          |  | Certificate of Amendment filed with the Secretary of State of the State of Nevada effecting an 8-for-1 reverse stock of the Registrant's common stock and changing the name of the Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.3 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006). |
| 3.4          |  | Articles of Conversion filed with the Secretary of State of the State of Nevada changing the state of incorporation of the Registrant (incorporated by reference to Exhibit 3.4 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).  |
| 3.5          |  | Certificate of Conversion filed with the Secretary of State of the State of Delaware (incorporated by reference to Exhibit 3.5 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).   |
| 3.6          |  | Amendment to Bylaws of the Registrant (incorporated by reference to Exhibit 3.6 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).  |
| 3.7          |  | Charter Amendment for TorreyPines (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).   |
| 3.8          |  | Certificate of Merger between Raptor Pharmaceuticals Corp. and ECP Acquisition, Inc. TorreyPines (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).  |
| (4)          |  | Instruments defining the rights of security holders, including indentures  |
| 4.1          |  | Specimen common stock certificate of the Registrant (incorporated by reference to Exhibit 4.7 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).   |
| 4.2          |  | Form of Warrant to Purchase Common Stock issued to previous holders of TPTX, Inc. redeemable convertible preferred stock in connection with the  |

- business combination between TorreyPines Therapeutics, Inc. and Axonyx, Inc. (incorporated by reference to Exhibit 4.2 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.3 Form of Registration Rights Agreement 1999 (incorporated by reference to Exhibit 4.4 to the Registrant's Annual Report on Form 10-KSB, filed on March 13, 2000).
- 4.4 Registration Rights Agreement dated as of January 8, 2004 between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on January 12, 2004).
- 4.5 Registration Rights Agreement dated as of May 3, 2004, between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on May 5, 2004).
- 4.6 Form of Warrant issued to Comerica Bank on July 1, 2003 (incorporated by reference to Exhibit 4.14 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.7 Form of Warrant issued to Silicon Valley Bank on December 8, 2000 (incorporated by reference to Exhibit 4.15 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.8 Form of Warrant issued to Oxford Financial and Silicon Valley Bank on September 27, 2005 (incorporated by reference to Exhibit 4.16 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.9 Rights Agreement, dated as of May 13, 2005, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on May 16, 2005).
- 4.10 Amendment to Rights Agreement, dated as of June 7, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed on June 12, 2006).
- 4.11 Form of Warrant issued to Comerica Bank on June 11, 2008 (incorporated by reference to Exhibit 4.1 to the Registrant's Report on Form 8-K, filed on June 17, 2008).
- 4.12 Amendment to Rights Agreement, dated as of October 3, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.19 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.13 Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between TorreyPines and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company) (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
- 4.14 \* Warrant to purchase common stock dated December 14, 2007 issued to Flower Ventures, LLC (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April 15, 2008).
- 4.15 \* Warrant Agreement Amendment, dated December 17, 2009.
- 4.16 \* Warrant to purchase common stock dated December 14, 2007 issued to ICON Partners, LP (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April 15, 2008).
- 4.17 \*



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- 4.18 \* Form of Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on May 22, 2008).
- 4.19 \* Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K/A, filed on May 28, 2008).
- 4.20 \* Form of Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on August 25, 2009).
- 4.20 \* Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on August 25, 2009).
- 4.21 Reference is made to Exhibits 3.1 through 3.6.
- (31) Section 302 Certification
- 31.1 Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director
- 31.2 Certification of Kim R. Tsuchimoto, Chief Financial Officer, Secretary and Treasurer

(32) Section 906 Certification

32.1 Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director, and of Kim R. Tsuchimoto, Chief Financial Officer, Secretary and Treasurer

\* The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.14 - 4.20 have been converted into warrants of the Registrant and the exercise price of such warrants and number of shares of common stock issuable thereunder have been converted as described in Item 1.01 (under the section titled, "Background") of the Registrant's Current Report on Form 8-K, filed on October 5, 2009.

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SIGNATURES

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

RAPTOR PHARMACEUTICAL CORP.

By: /s/ Christopher M. Starr  
Christopher M. Starr, Ph.D.  
Chief Executive Officer and Director  
(Principal Executive Officer)  
Date: April 9, 2010

By: /s/ Kim R. Tsuchimoto  
Kim R. Tsuchimoto  
Chief Financial Officer, Secretary and Treasurer  
(Principal Financial Officer and Principal Accounting Officer)  
Date: April 9, 2010

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Exhibit Index

|              |  |
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| 2.2          | Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated as of August 25, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Amendment No. 1 to Registration Statement No. 333-136018 filed on August 25, 2006).  |
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| 4.2          |  |

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- Form of Warrant to Purchase Common Stock issued to previous holders of TPTX, Inc. redeemable convertible preferred stock in connection with the business combination between TorreyPines Therapeutics, Inc. and Axonyx, Inc. (incorporated by reference to Exhibit 4.2 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.3 Form of Registration Rights Agreement 1999 (incorporated by reference to Exhibit 4.4 to the Registrant's Annual Report on Form 10-KSB, filed on March 13, 2000).
- 4.4 Registration Rights Agreement dated as of January 8, 2004 between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on January 12, 2004).
- 4.5 Registration Rights Agreement dated as of May 3, 2004, between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on May 5, 2004).
- 4.6 Form of Warrant issued to Comerica Bank on July 1, 2003 (incorporated by reference to Exhibit 4.14 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.7 Form of Warrant issued to Silicon Valley Bank on December 8, 2000 (incorporated by reference to Exhibit 4.15 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.8 Form of Warrant issued to Oxford Financial and Silicon Valley Bank on September 27, 2005 (incorporated by reference to Exhibit 4.16 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.9 Rights Agreement, dated as of May 13, 2005, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on May 16, 2005).
- 4.10 Amendment to Rights Agreement, dated as of June 7, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed on June 12, 2006).
- 4.11 Form of Warrant issued to Comerica Bank on June 11, 2008 (incorporated by reference to Exhibit 4.1 to the Registrant's Report on Form 8-K, filed on June 17, 2008).
- 4.12 Amendment to Rights Agreement, dated as of October 3, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.19 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.13 Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between TorreyPines and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company) (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
- 4.14 \* Warrant to purchase common stock dated December 14, 2007 issued to Flower Ventures, LLC (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April 15, 2008).
- 4.15 \* Warrant Agreement Amendment, dated December 17, 2009.
- 4.16 \* Warrant to purchase common stock dated December 14, 2007 issued to ICON Partners, LP (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April

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- 15, 2008).
- 4.17 \* Form of Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on May 22, 2008).
- 4.18 \* Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K/A, filed on May 28, 2008).
- 4.19 \* Form of Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on August 25, 2009).
- 4.20\* Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on August 25, 2009).
- 4.21 Reference is made to Exhibits 3.1 through 3.6.
- (31) Section 302 Certification
- 31.1 Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director.
- 31.2 Certification of Kim R. Tsuchimoto, Chief Financial Officer, Secretary and Treasurer.
- (32) Section 906 Certification
- 32.1 Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director, and of Kim R. Tsuchimoto, Chief Financial Officer, Secretary and Treasurer.
- \* The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.14 - 4.20 have been converted into warrants of the Registrant and the exercise price of such warrants and number of shares of common stock issuable thereunder have been converted as described in Item 1.01 (under the section titled, "Background") of the Registrant's Current Report on Form 8-K, filed on October 5, 2009.