Raptor Pharmaceutical Corp
Form 10-K
November 22, 2010

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

[X] ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended August 31, 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 000-50720

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

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

9 Commercial Blvd., Suite 200, Novato, CA 94949
(Address of principal executive offices)

(415) 382-8111
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

| Title of Each Class | Name of Each Exchange on Which Registered |
|---|---|
| Common Stock, $.001 par value | The NASDAQ Capital Market |
| Preferred Share Purchase Rights | |

Securities registered under Section 12(g) of the Act:

None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes o No x

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes o No x

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. o

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

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

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

Indicate the number of shares outstanding of each of the registrant's classes of common stock, as of the latest practicable date: 30,213,378 shares common stock, par value $0.001, outstanding as of November 5, 2010. The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of February 26, 2010 (the last business day of the registrant's most recently completed second quarter) was $44.2 million.

The documents incorporated by reference are as follows:

None.

RAPTOR PHARMACEUTICAL CORP.

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PART I

FORWARD-LOOKING STATEMENTS

In this Annual Report on Form 10-K, 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," "e "opportunity" or the negative of these terms or other comparable terminology. All such statements, other than statements of historical facts, including our financial condition, future results of operations, projected revenues and expenses, business strategies, operating efficiencies or synergies, competitive positions, growth opportunities for existing intellectual properties, technologies, products, plans, and objectives of management, markets for our securities, and other matters, are about us and our industry that involve substantial risks and uncertainties and constitute forward-looking statements for the purpose of the safe harbor provided by Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements, wherever they occur, are necessarily estimates reflecting the best judgment of our senior management on the date on which they were made, or if no date is stated, as of the date of the filing made with the SEC in which such statements were made. You should not place undue reliance on these statements, which only reflect information available as of the date that they were made. Our business' actual operations, performance, development and results might differ materially from any forward-looking statement due to various known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled "Risk Factors," 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;

· 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 Annual Report on Form 10-K, 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 date of this Annual Report on Form 10-K to reflect later events or circumstances or to reflect the occurrence of unanticipated events or any other reason.

ITEM 1: BUSINESS

All discussions in this Annual Report on Form 10-K regarding our common stock, our stock price, our stock options and warrants to purchase our common stock have been converted to their equivalent post-merger number shares and equivalent post-merger stock prices and exercises prices. See page 3 for further discussion about our Reverse Merger with Raptor Pharmaceuticals Corp.

Overview

We believe that we are building a balanced pipeline of drug candidates that may expand the reach and benefit of existing therapeutics. Our product portfolio includes both candidates from our proprietary drug targeting platforms and in-licensed and acquired product candidates.

Our current pipeline includes three clinical development programs which we are actively developing. We also have 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:

- 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 other liver diseases; and NeuroTrans™

to potentially deliver therapeutics across the blood-brain barrier for treatment of a variety of neurological diseases.

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

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 in the section titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations.” There can be no assurances that our research and development activities will be successful. In addition, if we do not raise additional funds, we may not be able to continue as a going concern.

Strategic Acquisitions

Reverse Merger with Raptor Pharmaceuticals Corp., or RPC

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, or RPC. 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 RPC and RPC 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, RPC's stockholders (as of immediately prior to such 2009 Merger) owned approximately 95% of our outstanding common stock and our stockholders (as of immediately prior to such 2009 Merger) owned approximately 5% of our outstanding common stock, in each case without taking into account any of our or RPC's shares of common stock, respectively, that were issuable pursuant to outstanding options or warrants of ours or RPC, respectively, outstanding as of the effective time of the 2009 Merger. Although RPC became our wholly-owned subsidiary, RPC was the "accounting acquirer" in the 2009 Merger and its board of directors and officers manage and operate the combined company. Our common stock currently trades on the NASDAQ Capital Market under the ticker symbol, "RPTP."

Purchase of Convivia™

In October 2007, prior to the 2009 Merger, RPC 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. RPC hired Convivia's chief executive officer and founder, Thomas E. (Ted) Daley, as the President of its clinical development division. In exchange for the assets related to the ALDH2 deficiency program, what we now call Convivia™, RPC issued to Convivia 46,625 shares of our common stock, an additional 46,625 shares of our common stock to a third party in settlement of a convertible loan between the third party and Convivia, and another 8,742 shares of our common stock in settlement of other obligations of Convivia. Mr. Daley, as the former sole stockholder of Convivia, may earn additional shares of our common stock based on certain triggering events or milestones related to the development of the Convivia assets. In addition, Mr. Daley may earn cash bonuses based on the same triggering events pursuant to his employment agreement. In January 2008, Mr. Daley earned a $30,000 cash bonus pursuant to his employment agreement as a result of the milestone of our execution of a formulation agreement for manufacturing Convivia™ with Patheon. In March 2008, RPC issued to Mr. Daley 23,312 shares of our common stock pursuant to the Convivia purchase agreement as a result of the milestone of our execution of an agreement to supply us with the active pharmaceutical ingredient for Convivia™ and two $10,000 cash bonuses pursuant to his employment agreement for reaching his six-month and one-year employment anniversaries. In October 2008, RPC issued to Mr. Daley 23,312 shares of our common stock valued at $27,000 and a $30,000 cash bonus as a result of fulfilling a clinical milestone. In July 2010, we issued 11,656 shares of our restricted common stock valued at $35,551and paid a $10,000 cash bonus to Mr. Daley as a result of the execution of the license agreement with Uni Pharma for the development of ConviviaTM in Taiwan.

Purchase of DR Cysteamine

In December 2007, prior to the 2009 Merger, through a merger between Encode Pharmaceuticals, Inc., or Encode, and Raptor Therapeutics, RPC purchased certain assets, including the clinical development and commercial rights to DR Cysteamine. Under the terms of and subject to the conditions set forth in the merger agreement, RPC issued 802,946

shares of our common stock to the stockholders of Encode, or Encode Stockholders, options, or Encode Options, to purchase up to, in the aggregate, 83,325 shares of our common stock to the optionholders of Encode, or Encode Optionholders, and warrants, or Encode Warrants, to purchase up to, in the aggregate, 256,034 shares of our common stock to the warrantholders of Encode, or Encode Warrantholders, and together with the Encode Stockholders and Encode Optionholders, referred to herein collectively as the Encode Securityholders, as of the date of such agreement. The Encode Securityholders are eligible to receive up to an additional 559,496 shares of our common stock, Encode Options and Encode Warrants to purchase our common stock in the aggregate based on certain triggering events related to regulatory approval of DR Cysteamine, an Encode product program, if completed within the five year anniversary date of the merger agreement.

As a result of the Encode merger, we received the exclusive worldwide license to DR Cysteamine, referred to herein as the License Agreement, developed by clinical scientists at the University of California at San Diego, or UCSD, School of Medicine. In consideration of the grant of the license, we are obligated to pay an annual maintenance fee of $15,000 until we begin commercial sales of any products developed pursuant to the License Agreement. In addition to the maintenance fee, we are obligated to pay during the life of the License Agreement: milestone payments ranging from $20,000 to $750,000 for orphan indications and from $80,000 to $1,500,000 for non-orphan indications upon the occurrence of certain events, if ever; royalties on commercial net sales from products developed pursuant to the License Agreement ranging from 1.75% to 5.5%; a percentage of sublicense fees ranging from 25% to 50%; a percentage of sublicense royalties; and a minimum annual royalty commencing the

year we begin commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, we are obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 years from the effective date of the License Agreement, depending on the indication. In addition, we are obligated, among other things, to spend annually at least $200,000 for the development of products (which we satisfied, as of August 31, 2010 and 2009 by spending approximately $6.2 million and $4.1 million, respectively, on such programs) pursuant to the License Agreement. To-date, we have accrued $470,000 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.

Company History

Corporate Structure

We were initially incorporated in Nevada on July 29, 1997 as Axonyx Inc. In October 2006, Axonyx Inc. and its then-wholly-owned subsidiary completed a reverse merger, business combination with TorreyPines Therapeutics, Inc., reincorporated in Delaware and changed our corporate name to “TorreyPines Therapeutics, Inc.”

On September 29, 2009, we and a wholly-owned subsidiary completed a reverse merger, business combination with RPC pursuant to which RPC became our wholly-owned subsidiary. Immediately prior to such time, we changed our corporate name to “Raptor Pharmaceutical Corp.” After such merger, our common stock began trading on the NASDAQ Capital Market and currently trades under the ticker symbol “RPTP.” 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.

RPC was incorporated in the State of Nevada on April 1, 2002 under the name of Highland Clan Creations Corp., or HCCC. On June 9, 2006, HCCC merged with RPC which was incorporated on May 5, 2006 in Delaware. As a result, HCCC was reincorporated from the State of Nevada to the State of Delaware and changed its corporate name to “RPC”. HCCC was a publicly traded company quoted on the OTC Bulletin Board and upon such merger, its common stock traded on the OTC Bulletin Board under the ticker “RPTP.” Our principal executive office is located at 9 Commercial Blvd., Suite 200, Novato, CA 94949. Our phone number is (415) 382-8111.

On May 25, 2006, RPC acquired 100% of the outstanding capital stock of Raptor Discoveries (f/k/a Raptor Pharmaceutical Inc.) (incorporated in Delaware on September 8, 2005), a development-stage research and development company and on June 9, 2006, RPC disposed of its former wholly-owned subsidiary, Bodysentials Health & Beauty Inc., which sold nutritional milkshakes and drinks on the Internet. On August 1, 2007, RPC formed Raptor Therapeutics Inc. (f/k/a Bennu Pharmaceuticals Inc.) as its wholly-owned subsidiary for the purpose of developing clinical-stage drug product candidates through to commercialization.

Financing History of RPC

Initial Investors

On May 25, 2006, in exchange for all of the outstanding common stock of Raptor Pharmaceutical Inc. (now known as Raptor Discoveries Inc.), RPC issued 1,864,987 shares of our common stock to the-then Raptor Pharmaceutical Inc. stockholders including 699,370 shares of our common stock to each of Christopher M. Starr, Ph.D., and Todd C.

Zankel, Ph.D., our Chief Executive Officer and Chief Scientific Officer, respectively, 233,123 shares of our common stock to Erich Sager, a member of our board of directors and 233,123 shares of our common stock to an unrelated third party. These initial stockholders of Raptor Pharmaceutical Inc. purchased common stock of Raptor Pharmaceutical Inc. when it was a privately held company for the following amounts of proceeds: Dr. Starr $5,000; Dr. Zankel $5,000; Mr. Sager $100,000 and the unrelated third party $200,000

$5 Million Financing and the 2006 Reverse Merger

Pursuant to an agreement dated March 8, 2006, with HCCC, on May 25, 2006, RPC closed a $5 million financing concurrent with a reverse merger. As part of that agreement, HCCC loaned RPC $0.2 million to be repaid with accrued interest upon the earlier of six months or the closing of the financing. Also, the agreement stated that pending the closing of at least a $3.5 million financing, HCCC would be obligated to issue 186,499 units as fees to a placement agent and $30,000 in commissions to an investment broker. In the financing HCCC sold 1,942,695 units of RPC at $2.57 per unit. Each such unit consisted of one share of our common stock and one common stock purchase warrant exercisable for one share of our common stock at $2.57 per share. The warrants were exercisable for 18 months and expired on November 25, 2007. Gross proceeds from the financing were $5 million and net proceeds after the repayment of the $0.2 million loan plus interest and the deduction of commissions and legal fees totaled approximately $4.6 million. Prior to the warrants expiring, RPC received $3,895,000 in gross proceeds from the

exercise of warrants in exchange for 1,513,359 shares of our common stock.

Issuance of Common Stock Pursuant to Stock Option Exercises

Since inception, we and RPC have received $72,722 from the exercise of stock options resulting in the issuance of 41,262 shares of common stock. Our common stock outstanding as of November 5, 2010 was 30,213,378 shares.

RPC's 2008 and 2009 Private Placements and Warrant Exchange

During May and June 2008, prior to the 2009 Merger, RPC, issued an aggregate of 4,662,468 units of its securities, each unit comprised of one share of our common stock and one warrant to purchase one half of one share of our common stock, at a unit purchase price of $2.15 per unit, in a private placement with various accredited investors. The warrants, exercisable for two years from closing of such private placement, as initially issued, entitled such investors to purchase up to an aggregate of 2,331,234 shares of RPC's common stock at an exercise price of $3.22 per share during the first year and $3.86 per share during the second year. In connection with this private placement, RPC issued placement agents warrants to purchase in the aggregate 489,559 shares of our common stock at an exercise price of $2.36 per share for a five year term and it paid to such placement agents cash fees totaling $700,000. Such placement agent warrants contained a cashless (net exercise) feature that allows its holders, under certain circumstances, to exercise such warrants without making any cash payment. Of the placement agents compensated, Limetree Capital was issued warrants to purchase 438,890 shares of our common stock and was paid cash commissions of $627,550. Erich Sager, one of our board members, serves on the board of directors of Limetree Capital and is a founding partner thereof.

In July 2009, prior to the 2009 Merger, RPC closed a warrant exchange offer with those investor-warrant holders who were holders of the warrants to purchase its common stock issued in connection with its May and June 2008 private placement, as described above, of the right to exchange such warrants and subscribe for new warrants to purchase shares of RPC's common stock at an exercise price of $1.29 per share (to the extent such new warrants were exercised (in whole or in part) on or before July 17, 2009). Pursuant to such warrant exchange, new warrants were exercised for an aggregate amount of 2,031,670 shares of our common stock which resulted in aggregate proceeds to RPC of $2,614,500.

In August 2009, prior to the 2009 Merger, RPC issued an aggregate of 1,738,226 units of our securities, each unit comprised of one share of our common stock and one warrant to purchase one half of one share of our common stock, at a unit purchase price of $1.37 per unit, in a private placement with various accredited investors. The warrants, exercisable for two years from closing of such private placement, as initially issued, entitled such investors to purchase up to an aggregate of 869,113 shares of our common stock at an exercise price of $2.57 per share during the first year and $3.22 per share during the second year. In connection with this private placement, RPC issued Limetree Capital, the placement agent in such private placement, warrants to purchase in the aggregate 129,733 shares of our common stock at an exercise price of $1.50 per share for a five year term and it paid to such placement agent cash fees totaling $59,360. Such placement agent warrants contained a cashless (net exercise) feature that allows its holders, under certain circumstances, to exercise such warrants without making any cash payment.

We filed a registration statement with the SEC, covering the resale of 5,557,865 shares of our common stock, including common stock issuable upon the exercise of the warrants, on October 13, 2009. Such registration statement covers certain of our common stock as described above.

Post-Merger Financings

Registered Direct Offering

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

Ladenburg received a placement fee equal to 6.5% of the gross cash proceeds to us from the Direct Offering of the Units or $487,183 (excluding any consideration that may be paid in the future upon exercise of the Warrants), a warrant to purchase up to an aggregate of 74,951 shares of our common stock at $2.50 per share (valued at approximately $52,000 using the following Black-Scholes pricing model assumptions: risk-free interest rate 2.23%; expected term 5 years and annual volatility 49.28%) and

$25,000 in out-of-pocket accountable expenses. The warrant issued to Ladenburg has the same terms and conditions as the Investor Warrants except that the exercise price is 125% of the public offering price per share or $2.50 per share, and the expiration date is five years from the effective date of that certain shelf registration statement on Form S-3 (Registration No. 333-162374) which was declared effective by the SEC on November 5, 2009.

In connection with the Direct Offering, following execution of the Placement Agent Agreement, we also entered into a definitive securities purchase agreement, or the Direct Offering Purchase Agreement, dated as of December 17, 2009, with 33 investors set forth on the signature pages thereto, collectively referred to as Direct Offering Investors, with respect to the Direct Offering of the Units, whereby, on an aggregate basis, the Direct Offering Investors agreed to purchase 3,747,558 Units for a negotiated purchase price of $2.00 per Unit, amounting to gross proceeds of approximately $7.5 million and estimated net proceeds after commissions and expenses of approximately $6.2 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 are 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 are 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 on our consolidated financial statements.

Equity Line Facility with Lincoln Park Capital Fund, LLC, or LPC

On April 16, 2010, we executed a purchase agreement, or the LPC Purchase Agreement, and a registration rights agreement, or the LPC Registration Rights Agreement, with LPC. Under the LPC Purchase Agreement, LPC is obligated to purchase from us up to $15 million of our common stock, from time to time over a twenty-five (25) month period. The issuance of our common stock to LPC under the LPC Purchase Agreement is exempt from registration under the Securities Act in reliance on Section 4(2) of the Securities Act, as the transaction did not involve a public offering.

Pursuant to the LPC Registration Rights Agreement, we filed a registration statement on April 23, 2010 with the SEC, for 4.5 million shares of our common stock covering the shares that have been issued or may be issued to LPC under the LPC Purchase Agreement. The registration statement was declared effective on May 7, 2010. Thereafter, over approximately 25 months, generally we have the right to direct LPC to purchase up to $15,000,000 of our common stock in amounts up to $100,000 as often as every two business days under certain conditions. We can also accelerate the amount of our common stock to be purchased under certain circumstances. No sales of shares may occur at a purchase price below $1.50 per share. The purchase price of the shares will be based on the market prices of our shares at the time of sale as computed under the LPC Purchase Agreement without any fixed discount. We may at any time in our sole discretion terminate the LPC Purchase Agreement without fee, penalty or cost upon one business days notice. We issued 145,033 shares of our common stock to LPC as a commitment fee for entering into the agreement, and we are obligated to issue up to 217,549 shares pro rata as LPC purchases up to $15,000,000 of our common stock as directed by us.

The 4.5 million shares that we registered consist of 4,137,418 shares that we have or may sell to LPC, 145,033 shares we issued as a commitment fee, and 217,549 shares that we have or are obligated to issue to LPC as a commitment fee pro rata as up to $15 million of our common stock is purchased by LPC.

Cumulatively, as of November 5, 2010, we have sold approximately 2.2 million shares under the equity line, raising approximately $4.9 million in gross proceeds to us. See the section titled “Purchase of Equity Securities and Affiliated Purchasers” in the Annual Report on Form 10-K for additional details. We may direct LPC to purchase up to an additional $10.1 million of shares of our common stock under the LPC Purchase Agreement over the next 21 months, generally in amounts of up to $100,000 every 2 business days. The selling price of our common stock to LPC will have to average at least $5.14 per share for us to receive the maximum proceeds of $15 million under the LPC Purchase Agreement. Assuming a purchase price of $1.50 per share (the minimum price of the common stock) and the purchase by LPC of the 1,966,620 shares left under the LPC Purchase Agreement plus the proceeds from the 2,170,798 shares purchased by LPC to-date, proceeds to us would only be approximately $7.8 million unless we choose to register more than 4,137,418 shares for sale to LPC under the LPC Purchase Agreement, which, subject to the approval of our board of directors, we have the right, but not the obligation, to do. In the event we elect to issue more than the 4.5 million shares of our common stock registered under a certain registration statement with the SEC, we must first register under the Securities Act, any additional shares we may elect to sell to LPC before we can sell such additional shares, which could cause substantial dilution to our stockholders. In addition, in the event that we decide to issue more than 4.5 million

shares, i.e., greater than 19.99% of our outstanding shares of common stock as of the date of the LPC Purchase Agreement, we would first be required to seek stockholder approval in order to be in compliance with the NASDAQ Capital Market rules.

2010 Private Placement

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

The closing of this private placement occurred on August 12, 2010. We issued and sold an aggregate of 4,897,614 units, comprised of 4,897,614 shares of common stock and warrants to purchase up to 4,897,614 shares of our common stock for gross proceeds of approximately $15.1 million. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share. As the placement agent for the 2010 Private Placement, the Placement Agent was issued one warrant to purchase 97,952 shares of our common stock, paid a cash commission of $978,911 and reimbursed for certain of its expenses incurred in connection with the 2010 Private Placement.

In connection with the 2010 Private Placement, on August 12, 2010, we entered into a registration rights agreement, or the 2010 Private Placement Registration Rights Agreement, with the 2010 Private Placement Investors, pursuant to which we filed with the SEC a registration statement covering the resale of the common stock issued to the 2010 Private Placement Investors under the 2010 Private Placement Purchase Agreements and the shares of common stock that will be issued to the 2010 Private Placement Investors upon exercise of the warrants, including the warrant issued to the Placement Agent. Such registration statement was declared effective on August 31, 2010.

Our securities offered and sold under the 2010 Private Placement Purchase Agreements to the 2010 Private Placement Investors were offered and sold in reliance upon exemptions from registration under the Securities Act in reliance on Section 4(2) of the Securities Act and Rule 506 of Regulation D promulgated thereunder, as transactions by an issuer not involving a public offering.

Proprietary Rights

We purchased from BioMarin Pharmaceutical Inc., or BioMarin, the intellectual property owned by BioMarin for the research and development of the RAP technologies, including two patents, two pending patent applications and two provisional patent applications in review in the U.S., and countries in Europe and Asia and two trademarks for NeuroTrans™. Subsequent to the purchase from BioMarin, we have filed four additional patent applications for our RAP technologies. As of November 10, 2010, we have 16 patent applications under prosecution in the U.S. and internationally. Two of these applications relate to cysteamine, seven relate to our ConviviaTM program and the remaining seven cover our RAP platform. Four patents have been allowed in the U.S. relating to our RAP platform: US 7,700,554 expires in 2022; US 7,560,431 expires in 2023; US 7,569,544 expires in 2023 and US 7,829,537 expires in 2023, and another was allowed in Japan, Australia and Europe which expires in 2022. All other applications are awaiting examination in a variety of countries. We also entered into an exclusive worldwide license agreement with Washington University for our Mesd program for the treatment of cancer and bone diseases. We fund

the prosecution of a patent application covering this technology, which entered national phase in the U.S. and internationally in November 2009. In December 2007, we acquired an exclusive worldwide license agreement to pending patent applications from UCSD relating to our DR Cysteamine program. In March 2008, we amended our license with UCSD to add exclusive worldwide rights to develop DR Cysteamine for the potential treatment of NASH. Through the 2009 Merger, we have a license from Eli Lilly & Co. for the intellectual property related to tezampanel and NGX426 for pain indications and a license of tezampanel and NGX426 for the treatment of thrombotic disorder from JHU. We fund the prosecution of a patent covering this technology, which entered national phase in the U.S. in August 2009. In June 2010, we acquired an exclusive worldwide license to two issued patents related to the treatment of Huntington's Disease and other neurological disorders, from the Weizmann Institute of Science in Israel and Niigata University in Japan. These two patents, which expire in 2019, cover the use of transglutaminase inhibitors, a class of molecules chemically similar to cysteamine.

Regulatory Exclusivities

Orphan Drug Designation

We have been granted access to an Orphan Drug Designation from the U.S. Food and Drug Administration, or FDA, for use of DR Cysteamine to potentially treat cystinosis and the use of Cysteamine to potentially treat HD and Batten Disease. The Orphan Drug Act of 1983 generally provides incentives, including marketing exclusivity and tax benefits, to companies that undertake development and marketing of products to treat relatively rare diseases, which are defined as diseases for which fewer than 200,000 persons in the U.S. would be likely to receive the treatment. A drug that receives orphan drug status may receive up to seven years of exclusive marketing in the U.S. for that indication. Equivalent European regulations may give us ten years of marketing exclusivity for that indication in Europe. DR Cysteamine has been granted Orphan Drug Designation by the FDA and the European Medicines Agency, or EMA. If we fail to maintain orphan drug exclusivity for some of our drug product candidates, our competitors may sell products to treat the same conditions and our results of operations and revenues will be affected.

Competition

Cystinosis

The only pharmaceutical product currently approved by the FDA and the EMA, to treat cystinosis that we are aware of is Cystagon® (rapid release cysteamine bitartrate capsules), marketed in the U.S. by Mylan Pharmaceuticals, and by Recordati and Swedish Orphan International in markets outside of the U.S. Cystagon® was approved by FDA in 1994 and is the standard of care for cystinosis treatment.

While we believe that our DR Cysteamine formulation will be well received in the market due to what we believe will be reduced dose frequency and improved tolerability, if we receive marketing approval, we anticipate that Cystagon® will remain a well-established competitive product which may retain many patients, especially those for whom the dose schedule and tolerability do not pose significant problems.

We are not aware of any pharmaceutical company with an active program to develop an alternative therapy for cystinosis. There are companies developing and/or marketing products to treat symptoms and conditions related to, or resulting from cystinosis, but none developing products to treat the underlying metabolic disorder. Academic researchers in the U.S. and Europe are pursuing potential cures for cystinosis through gene therapy and stem cell therapy, as well as pro-drug approaches as alternatives to cysteamine bitartrate for cystinosis treatment. The development timeline for these approaches is many years.

Huntington’s Disease

We are not aware of any currently available treatment alternatives for HD, although there are products available such as Haldol, Klonopin and Xenazine to treat uncontrollable movements and mood swings that result from the disease. There are several pharmaceutical companies pursuing potential cures and treatments for HD, as well as numerous academic- and foundation-sponsored research efforts.

Companies with HD product candidates in development include Medivation, Inc., Amarin, Eli Lilly & Co. and Pfizer. Several other companies have drug candidates in preclinical development. Additionally, nutritional supplements including creatinine and coenzyme Q10 have been investigated as potential treatments for HD. The Huntington Study

Group sponsors numerous studies of potential therapies for HD, including coenzyme Q10 and the antibiotic minocycline.

NASH

We are not aware of any currently available treatment options for NASH. Weight loss, healthy diet, abstinence from alcohol and increased physical activity are typically suggested to slow the onset of NASH. There are numerous therapies being studied for NASH, including anti-oxidants (Vitamin E, betaine, Moexipril from Univasc), insulin sensitizing agents (Actos® from Takeda Pharmaceuticals for type 2 diabetes, in an ongoing Phase 3 study for NASH sponsored by University of Texas) and drugs to improve blood flow (Trental® from Aventis for treatment of intermittent claudication, which is reported to have failed to meet endpoints in a Phase 2 study for NASH). Gilead Sciences is developing a pan-caspase inhibitor for NASH. Other products being

studied for NASH include Byetta from Amylin, in an ongoing Phase 2/3 study for NASH; and siliphos, or milk thistle, in a UCSD Phase 2 study for NASH.

ALDH2 Deficiency

ALDH2 deficiency affects hundreds of millions of people worldwide and is especially prevalent in East Asian populations. The association of this metabolic disorder with serious health risks, including liver diseases and digestive tract cancers, has been documented in numerous peer-reviewed studies over the last 10 years. We are not aware of any pharmaceutical products currently approved for this indication, either in the U.S. or internationally. However, given the size of the potential patient population and the emerging awareness of this disorder as a serious health risk, we expect there are or will be other pharmaceutical companies, especially those with commercial operations in Asian countries, developing products to treat the symptoms of this condition. Many of these competitors may have greater resources, and existing commercial operations in the Asian countries which we expect will be the primary markets for this product.

Additionally, there are non-pharmaceutical products available such as supplements and traditional remedies, especially in some Asian countries, which are claimed to be effective in reducing the symptoms associated with ALDH2 deficiency and other physical reactions to ethanol consumption. Although we are not aware of any study which has demonstrated the efficacy of such non-pharmaceutical alternatives, these products may compete with our ALDH2 deficiency product candidate if it is approved for marketing.

Migraine

Triptans are the most commonly prescribed drugs for the treatment of moderate to severe migraine. There are currently seven triptans approved for use and Imitrex®, marketed by GlaxoSmithKline, dominates the market. Other triptans are: Zomig®, Maxalt®, Amerge®, Frova™, Axert®, and Relpax®. According to PhRMA's 2008 report, Medicines in Development for Neurologic Disorders, there are more than 30 companies seeking to develop compounds to treat migraine and pain disorders or to obtain additional indications to broaden the use of currently approved pain relieving prescription medications. This list includes most of the large pharmaceutical companies such as Abbott Laboratories, AstraZeneca, Eisai, Elan, Eli Lilly, GlaxoSmithKline, Merck, Pfizer, and Wyeth Pharmaceuticals as well as small and mid-sized biotechnology companies.

Pain

In the neuropathic pain market, we would compete with companies such as Pfizer, marketing Neurontin and Lyrica®, and Eli Lilly, marketing Cymbalta® in addition to opioids approved for treating neuropathic pain, off-label uses of products to treat neuropathic pain and generic products. Given the size of the neuropathic pain market, approximately $3.5 billion in 2006 and expected to double by 2016, it is likely that most of the large pharmaceutical companies as well as many biotechnology companies will look to develop compounds to treat neuropathic pain. Since the licensing of tezampanel, Eli Lilly has continued development of more potent and specific molecules (e.g., iGluR5 antagonists) targeting the same receptors as tezampanel and NGX424 and based on the same chemistry (i.e., tetrahydroisoquinoline moiety) as tezampanel and NGX424. Eli Lilly's third generation candidate is currently in Phase 2 studies for osteoarthritis and peripheral neuropathy.

Primary Liver Cancer

Surgical resection of the primary tumor or liver transplantation remains the only curative options for HCC patients. The acute and tragic nature of this aggressive cancer and the widely preserved unmet medical need continues to attract a significant level of interest in finding ways of treating this disease. For example, there are currently over 140 ongoing clinical trials actively recruiting patients with HCC listed in the ClinicalTrials.gov website. Many of these trials are designed to evaluate ways of locally administering chemotherapeutics or various ways of performing surgical resections of the tumors. One drug that was approved in 2007 for treatment of inoperable HCC is currently the standard-of-care for this disease due to its claims of enhancing overall survival time. This enhancement was determined to be minimal in the study population and to be even smaller within the Asian population of inoperable HCC patients. We believe that a number of biotechnology and pharmaceutical companies may have internal programs targeting the development of new therapeutics that may be useful in treating HCC in the future.

Brain Delivery

We believe we will be competing with other pharmaceutical and biotechnology companies that provide, or are attempting to develop product candidates to provide, remedies and treatments for brain and neurodegenerative diseases.

Three approaches are primarily used to solve the problem of reaching the brain with therapeutic compounds:

· Neurosurgery or invasive techniques.

· Pharmacological techniques, which include less than 2% of currently available drugs.

· Physiologically based techniques, such as transcytosis.

Invasive techniques include bone marrow transplants or implants of polymers with drugs imbedded in the material for slow release. These implants extend from the skull surface to deep into brain tissue sites and use a permeation enhancer. Mannitol induced osmotic shock that creates leaks in the blood-brain barrier allowing intravenous administered chemotherapeutics into the brain is used in the treatment of brain tumors, but is not appropriate for administration of drugs for chronic therapies. Companies active in developing treatments based on these invasive technologies include Alza Corporation, Ethypharm, Guilford Pharmaceuticals, Medtronic Inc., Neurotech, and Sumitomo Pharmaceutical.

Other invasive procedures utilize catheter-based delivery of the drug directly into the brain. This technique has proven useful in the treatment of brain tumors, but has not been successful in distributing drugs throughout the entire brain. Amgen Inc. recently conducted clinical trials for the treatment of Parkinson’s disease using intrathecal delivery through the use of various catheter/pump techniques.

The physiological route is a popular approach to cross the blood-brain barrier via lipid mediated free diffusion or by facilitated transport. This is the most common strategy used for the development of new neuropharmaceuticals, but has experienced limited success as it requires that the drug have sufficient lipophilic or fat-soluble properties so that it can pass through lipid membranes. The current method of delivery by this route, however, is nonspecific to the brain and side effects are common since most organs are exposed to the drug. Furthermore, many of the potential lipophilic therapeutic molecules are substrates for the blood-brain barrier’s multi-drug resistant proteins, which actively transport the therapeutic agent back into the blood. Consequently, large doses need to be used so that sufficient amounts of the drug reach the brain. These high doses can result in significant side effects as the drug is delivered to essentially all tissues of the body, which is extremely inefficient. Companies and organizations that are developing treatments based on various physiological approaches include Angiochem, AramGen Technology, to-BBB, Xenoport Inc., Bioasis, Oregon Health and Science University Neuro-oncology, Xenova Group Ltd., d-Pharm, Neurochem Inc., and Vasogen Inc.

Thrombotic Disorder

A number of anti-platelet drugs are already available on the market. These include the ADP receptor antagonist Plavix, the cyclooxygenase (and hence thromboxane) inhibitor, aspirin, and injectable integrin (IIb/IIIA) blockers such as Integrelin. Each drug has strengths and weaknesses (which predominantly involve excess bleeding). Since anti-thrombotic drugs are a multi-billion dollar market, it is likely that a large number of companies have additional therapies in development.

Because, many of our competitors have greater capital resources and larger overall research and development staffs and facilities, than us, there can be no assurances that we will be successful in competing in the areas discussed above. See the section under “Risk Factors” titled, “If our competitors succeed in developing products and technologies that are more effective than ours, 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.”

Government Regulations of the Biotechnology Industry

Regulation by governmental authorities in the U.S. and foreign countries is a significant factor in the development, manufacture, and expected marketing of our drug product candidates and in our ongoing research and development activities. The nature and extent to which such regulation will apply to us will vary depending on the nature of any drug product candidates developed. We anticipate that all of our drug product candidates will require regulatory approval by governmental agencies prior to commercialization.

In particular, human therapeutic products are subject to rigorous preclinical and clinical testing and other approval procedures of the FDA and similar regulatory authorities in other countries. Various federal statutes and regulations also govern or influence testing, manufacturing, safety, labeling, storage, and record-keeping related to such products and their marketing. The process of obtaining these approvals and the subsequent compliance with the appropriate federal statutes and regulations requires substantial time and financial resources. Any failure by us or our collaborators to obtain, or any delay in obtaining, regulatory approval could adversely affect the marketing of any of our drug product candidates, our ability to receive product revenues, and our liquidity and capital resources.

The FDA's Modernization Act codified the FDA's policy of granting "fast track" review of certain therapies targeting "orphan" indications and other therapies intended to treat severe or life threatening diseases and having potential to address unmet medical needs. Orphan indications are defined by the FDA as having a prevalence of less than 200,000 patients in the U.S. We anticipate that certain genetic diseases and primary liver cancer which could potentially be treated using our technology could qualify for fast track review under these revised guidelines. There can be no assurances, however, that we will be able to obtain fast track designation and, even with fast track designation, it is not guaranteed that the total review process will be faster or that approval will be obtained, if at all, earlier than would be the case if the drug product candidate had not received fast-track designation.

Before obtaining regulatory approvals for the commercial sale of any of our products under development, we must demonstrate through preclinical studies and clinical trials that the product is safe and efficacious for use in each target indication. The results from preclinical studies and early clinical trials might not be predictive of results that will be obtained in large-scale testing. Our clinical trials might not successfully demonstrate the safety and efficacy of any product candidates or result in marketable products.

In order to clinically test, manufacture, and market products for therapeutic use, we will have to satisfy mandatory procedures and safety and effectiveness standards established by various regulatory bodies. In the U.S., the Public Health Service Act and the Federal Food, Drug, and Cosmetic Act, as amended, and the regulations promulgated thereunder, and other federal and state statutes and regulations govern, among other things, the testing, manufacture, labeling, storage, record keeping, approval, advertising, and promotion of our current and proposed product candidates. Product development and approval within this regulatory framework takes a number of years and involves the expenditure of substantial resources.

The steps required by the FDA before new drug products may be marketed in the U.S. include:

· completion of preclinical studies;

· the submission to the FDA of a request for authorization to conduct clinical trials on an investigational new drug application, or IND, which must become effective before clinical trials may commence;

· adequate and well-controlled Phase 1, Phase 2 and Phase 3 clinical trials to establish and confirm the safety and efficacy of a drug candidate;

· submission to the FDA of a new drug application, or NDA, for the drug candidate for marketing approval; and

· review and approval of the NDA by the FDA before the product may be shipped or sold commercially.

In addition to obtaining FDA approval for each product, each product manufacturing establishment must be registered with the FDA and undergo an inspection prior to the approval of an NDA. Each manufacturing facility and its quality control and manufacturing procedures must also conform and adhere at all times to the FDA's cGMP regulations. In addition to preapproval inspections, the FDA and other government agencies regularly inspect manufacturing facilities for compliance with these requirements. If, as a result of these inspections, the FDA determines that any equipment, facilities, laboratories or processes do not comply with applicable FDA regulations and conditions of product approval, the FDA may seek civil, criminal, or administrative sanctions and/or remedies against us, including the suspension of the manufacturing operations. Manufacturers must expend substantial time, money and effort in the area of production and quality control to ensure full technical compliance with these standards.

Preclinical testing includes laboratory evaluation and characterization of the safety and efficacy of a drug and its formulation. Preclinical testing results are submitted to the FDA as a part of an IND which must become effective prior to commencement of clinical trials. Clinical trials are typically conducted in three sequential phases following submission of an IND. Phase 1 represents the initial administration of the drug to a small group of humans, either patients or healthy volunteers, typically to test for safety (adverse effects), dosage tolerance, absorption, distribution, metabolism, excretion and clinical pharmacology, and, if possible, to gain early evidence of effectiveness. Phase 2 involves studies in a small sample of the actual intended patient population to assess the efficacy of the drug for a specific indication, to determine dose tolerance and the optimal dose range and to gather additional information relating to safety and potential adverse effects. Once an investigational drug is found to have some efficacy and an acceptable safety profile in the targeted patient population, Phase 3 studies are initiated to further establish clinical safety and efficacy of the therapy in a broader sample of the general patient population, in order to determine the overall risk-benefit ratio of the drug and to provide an adequate basis for any physician labeling. During all clinical studies, we must adhere to Good Clinical Practice, or GCP, standards. The results of the research and product development, manufacturing, preclinical studies, clinical studies and related information are submitted in an NDA to the FDA.

The process of completing clinical testing and obtaining FDA approval for a new drug is likely to take a number of years and require the expenditure of substantial resources. If an application is submitted, there can be no assurance that the FDA will review and approve the NDA. Even after initial FDA approval has been obtained, further studies, including post-market studies, might be required to provide additional data on safety and will be required to gain approval for the use of a product as a treatment for clinical indications other than those for which the product was initially tested and approved. Also, the FDA will require post-market reporting and might require surveillance programs to monitor the side effects of the drug. Results of post-marketing programs might limit or expand the further marketing of the products. Further, if there are any modifications to the drug, including changes in indication, manufacturing process, labeling or a change in manufacturing facility, an NDA supplement might be required to be submitted to the FDA.

The rate of completion of any clinical trials will be dependent upon, among other factors, the rate of patient enrollment. Patient enrollment is a function of many factors, including the size of the patient population, the nature of the trial, the availability of alternative therapies and drugs, the proximity of patients to clinical sites and the eligibility criteria for the study. Delays in planned patient enrollment might result in increased costs and delays, which could have a material adverse effect on us.

We do not know whether our IND for future products or the protocols for any future clinical trials will be accepted by the FDA. We do not know if our clinical trials will begin or be completed on schedule or at all. Even if completed, we do not know if these trials will produce clinically meaningful results sufficient to support an application for marketing approval. The commencement of our planned clinical trials could be substantially delayed or prevented by several factors, including:

· a limited number of, and competition for, suitable patients with particular types of disease for enrollment in clinical trials;

· delays or failures in obtaining regulatory clearance to commence a clinical trial;

· delays or failures in obtaining sufficient clinical materials;

· delays or failures in reaching agreement on acceptable clinical trial agreement terms or clinical trial protocols with prospective sites; and

· delays or failures in obtaining Institutional Review Board, or IRB, approval to conduct a clinical trial at a prospective site.

The completion of our clinical trials could also be substantially delayed or prevented by several factors, including:

· slower than expected rates of patient recruitment and enrollment;

· failure of patients to complete the clinical trial;

· unforeseen safety issues;

· lack of efficacy during clinical trials;

· inability or unwillingness of patients or medical investigators to follow our clinical trial protocols;

· inability to monitor patients adequately during or after treatment; and

· regulatory action by the FDA for failure to comply with regulatory requirements.

Failure to comply with applicable FDA requirements may result in a number of consequences that could materially and adversely affect us. Failure to adhere to approved trial standards and GCPs in conducting clinical trials could cause the FDA to place a clinical hold on one or more studies which would delay research and data collection necessary for product approval. Noncompliance with GCPs could also have a negative impact on the FDA's evaluation of an NDA. Failure to adhere to GMPs and other applicable requirements could result in FDA enforcement action and in civil and criminal sanctions, including but not limited to fines, seizure of product, refusal of the FDA to approve product approval applications, withdrawal of approved applications, and prosecution.

Whether or not FDA approval has been obtained, approval of a product by regulatory authorities in foreign countries must be obtained prior to the commencement of marketing of the product in those countries. The requirements governing the conduct of clinical trials and product approvals vary widely from country to country, and the time required for approval might be longer or shorter than that required for FDA approval. Although there are some procedures for unified filings for some European countries, in general, each country at this time has its own procedures and requirements. There can be no assurance that any foreign approvals would be obtained.

In most cases, if the FDA has not approved a drug product candidate for sale in the U.S., the drug product candidate may be exported for sale outside of the U.S. only if it has been approved in any one of the following: the European Union, Canada, Australia, New Zealand, Japan, Israel, Switzerland and South Africa. Specific FDA regulations govern this process.

In addition to the regulatory framework for product approvals, we and our collaborative partners must comply with federal, state, and local laws and regulations regarding occupational safety, laboratory practices, the use, handling and disposition of radioactive materials, environmental protection and hazardous substance control, and other local, state, federal and foreign regulation. All facilities and manufacturing processes used by third parties to produce our drug candidates for clinical use in the United States must conform with cGMPs. These facilities and practices are subject to periodic regulatory inspections to ensure compliance with cGMP requirements. Their failure to comply with applicable regulations could extend, delay, or cause the termination of clinical trials conducted for our drug candidates. The impact of government regulation upon us cannot be predicted and could be material and adverse. We cannot accurately predict the extent of government regulation that might result from future legislation or administrative action.

Medical and Scientific Advisory Board

Our Medical and Scientific Advisory Board members work with our management team in the planning, development and execution of scientific and business strategies. The advisory board is composed of experienced academic and industry leaders with diverse expertise and knowledge in a variety of areas, including drug discovery, translational research, drug development, and business development. The following describes the background of our Medical and Scientific Advisory Board.

Stephen C. Blacklow, M.D., Ph.D. Over the last ten years, Dr. Blacklow's research team has achieved international recognition both for their mechanistic and structural studies of proteins of the LDL receptor family, and for their work

on the structure and function of human Notch proteins. Recently, Dr. Blacklow's team determined the structure of a RAP d3- receptor complex by X-ray crystallography. Dr. Blacklow graduated from Harvard College summa cum laude in 1983, and received his M.D. and Ph.D. in bioorganic chemistry from Harvard University in 1991. Dr. Blacklow is a board-certified pathologist and an Associate Professor of Pathology at Harvard Medical School where he is the Director of the Harvard M.D.-Ph.D. program, basic sciences track. He has directed a research laboratory at the Brigham and Women's Hospital, a teaching affiliate of the Harvard Medical School, since 1998, and he will be joining the Department of Cancer Biology at the Dana Farber Cancer Institute in 2010.

Guojun Bu, Ph.D., is a molecular and cell biologist and a leader in the field of the LDL receptor family. Dr. Bu obtained his undergraduate degree from the Beijing Normal University in China. He then studied biochemistry and molecular biology in the Department of Biochemistry at Virginia Tech where he received his Ph.D. Dr. Bu moved to the Washington University School of Medicine for a postdoctoral training in cell biology where he later became a member of the faculty. He is currently Professor of Pediatrics, and of Cell Biology and Physiology in the Department of Neuroscience at the Mayo Clinic in Jacksonville, Florida. Among the numerous awards that he has received, Dr. Bu has been an Established Investigator of the American Heart Association and a recipient of a Zenith Fellows Award from the Alzheimer's Association. He currently serves as an Editorial Board member for the Journal of Biological Chemistry and Journal of Lipid Research, and is the Editor-in-Chief of Molecular Neurodegeneration.

Ranjan Dohil, M.D., is Professor of Pediatrics at the University of California, San Diego, within the Division of Gastroenterology, Hepatology and Nutrition. An interest in childhood acid-peptic disorders led Dr. Dohil to study patients with cystinosis taking cysteamine. He has published the results of a number of studies trying to better understand the pharmacokinetics of cysteamine with the intent of developing a new formulation of cysteamine that would result in an improved quality of life for patients with cystinosis. Dr. Dohil also has a research interest in eosinophilic esophagitis, a condition that over the past few years has increased in incidence. Within this field, his work has led to the development of a treatment that is becoming more widely used. Dr. Dohil undertook his medical training at the University of Wales College of Medicine in Cardiff, U.K. He has served as a physician in many hospitals over his career including the University Hospital of Wales in Cardiff, U.K., the British Columbia's Children's Hospital in Vancouver, Canada and at St. Bartholemew and The London Medical School.

Jerry Schneider, M.D. is Research Professor of Pediatrics and Dean for Academic Affairs Emeritus at the University of California, San Diego (UCSD) School of Medicine. He also serves as a member of the Board of Directors and Chair of the Scientific Review Board for the Cystinosis Research Foundation. Over the course of his distinguished career, Dr. Schneider has been actively involved in the study of metabolic diseases. An expert on the diagnosis and treatment of cystinosis, Dr. Schneider has published over 150 papers on cystinosis and related subjects over the past 40 years. Since 1969 he has been associated with the UCSD School of Medicine in both academic and research capacities. Dr. Schneider earned his M.D. from Northwestern University. He received postgraduate training at Johns Hopkins University, the National Institutes of Health (NIH), and the Centre de Genetique Moleculaire, Gif-sur-Yvette, France. He was also a Guggenheim Fellow and a Fogarty Senior Fellow at the Imperial Cancer Research Fund Laboratories in London, England.

Legal Proceedings

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.

Research and Development

We are a research and development company and our plan is to focus our efforts in the discovery, research, preclinical and clinical development of our RAP based platforms, complementary technologies and clinical drug candidates to provide therapies that we believe will be safer, less intrusive, and more effective than current approaches in treating a wide variety of brain disorders and neurodegenerative diseases, genetic disorders and cancer. During the period from September 8, 2005 (inception of Raptor Pharmaceuticals Corp.) to August 31, 2010, we incurred approximately $24.2 million ($9.3 million and $6.6 million for the years ended August 31, 2010 and 2009, respectively) in research and development costs. Please see the section titled, "Management's Discussion and Analysis of Financial Condition and Results of Operations" in this Annual Report on Form 10-K for our planned research and development activities for the twelve months subsequent to August 31, 2010.

Compliance with Environmental Laws

We estimate the annual cost of compliance with environmental laws, comprised primarily of hazardous waste removal, will be approximately $5,000.

Employees

We presently have ten full time employees, including five executives, one scientist, one program director, one clinical operations director, one senior manager in our regulatory department and one senior manager in our finance department. We also have one part-time scientist. Based on our current plan, over the next 12 month period, we anticipate hiring one or two commercial operations specialists in preparation for the commercial launch of DR Cysteamine for cystinosis. We also plan to supplement our human resources needs through consultants and contractors as needed.

Facilities

Our primary offices are located at 9 Commercial Blvd., Suite 200, Novato, CA 94949. Our phone number is (415) 382-8111 and our facsimile number is (415) 382-1368. Our website is located at www.raptorpharma.com.

ITEM 1A: RISK FACTORS

An investment in our common stock involves a high degree of risk. Before investing in our common stock, you should consider carefully the specific risks detailed in this "Risk Factors" section before making a decision to invest in our common stock, together with all of the other information contained in this Annual Report on Form 10-K. If any of these risks occur, our business, results of operations and financial condition could be harmed, the price of our common stock could decline, and you may lose all or part of your investment.

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 consolidated financial statements as of August 31, 2010 have been prepared assuming that we will continue as a going concern. As of August 31, 2010, we had an accumulated deficit of approximately $40.8 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, 2010, 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 as of August 31, 2010 of $16.9 million will be sufficient to meet our obligations into December 2011. We are currently in the process of negotiating strategic partnerships, collaborations and potential equity sales to supplement the funding of our preclinical and clinical programs beyond December 2011. If we are unable to obtain such additional capital when needed, we may be forced to scale down our expenditures.

On August 9, 2010, we entered into the 2010 Private Placement Purchase Agreements with the 2010 Private Placement Investors for the private placement of units comprised of our common stock, and warrants to purchase our common stock, at a purchase price of $3.075 per unit, with each unit comprised of one share of common stock and a warrant to purchase one share of common stock. We issued and sold an aggregate of 4,897,614 units, comprised of an aggregate of 4,897,614 shares of common stock and warrants to purchase up to 4,897,614 shares of our common stock for gross proceeds of approximately $15.1 million. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share. As the placement agent to this private placement, JMP Securities LLC was issued one warrant to purchase 97,952 shares of our common stock, paid a cash commission of $978,911 and reimbursed for certain of its expenses incurred in connection with the 2010 Private Placement. Even with the 2010 Private Placement, in the future, we may need to sell equity or debt securities to raise 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 additional financing when needed, we may have to delay or terminate some or all of our research and development programs, our financial condition and operating results may be adversely affected and we may have to scale back our operations.

While we were restricted from selling additional shares of our common stock under the 2010 Private Placement Purchase Agreements until November 10, 2010, we may issue shares in connection with the exercise of warrants and/or stock options, and after the expiration of such "lock-up" period, we may draw on the equity line with LPC. The extent to which we rely on LPC as a source of funding will depend on a number of factors including, the prevailing market price of our common stock and the extent to which we are able to secure working capital from other sources. Specifically, LPC does not have the right nor the obligation to purchase any shares of our common stock on any business days that the purchase price of our common stock is less than $1.50 per share. If obtaining sufficient funding from LPC were to prove unavailable or prohibitively dilutive, and if other sources of funding are available to us, we may determine not to sell shares to LPC under the LPC Purchase Agreement.

If we obtain 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 launch and successfully commercialize our product
- candidates, once approved;
  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, for the purchase of intellectual property related to the 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 current good manufacturing practices, or cGMP, requirements enforced by the FDA, through its facilities inspection program. The FDA is likely to conduct inspections of our third party manufacturer and key supplier facilities as part of their review of any of our NDAs. If our third party manufacturers and key suppliers are not in compliance with cGMP requirements, it may result in a delay of approval, particularly if these sites are supplying single source ingredients required for the manufacture of any potential product. These cGMP requirements include quality control, quality assurance and the maintenance of records and documentation. Furthermore, regulatory qualifications of manufacturing facilities are applied on the basis of the specific facility being used to produce supplies. As a result, if one of the manufacturers that we rely on shifts production from one facility to another, the new facility must go through a complete regulatory qualification and be approved by regulatory authorities prior to being used for commercial supply. Our manufacturers may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. A failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall, or withdrawal of product approval. If the safety of any quantities supplied is compromised due to 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 control over financial reporting and disclosure controls and procedures. In particular, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. Our compliance with Section 404 will require that we incur substantial accounting and related expense and expend significant management efforts. In the future, we may need to hire additional accounting and financial staff to satisfy the ongoing requirements of Section 404. Moreover, if we are not able to comply with the requirements of Section 404, or we or our independent registered public accounting firm identifies deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by NASDAQ, the SEC or other regulatory authorities.

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 December 2009, we entered into a definitive securities purchase agreement or the Direct Offering Purchase Agreement, dated as of December 17, 2009, with 33 investors, collectively, the Direct Offering Investors, with respect to the offering of Units, whereby, on an aggregate basis, the Direct Offering Investors agreed to purchase 3,747,558 Units for a negotiated purchase price of $2.00 per Unit 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 Series A Warrants are exercisable during the period beginning on June 20, 2010 and ending on December 22, 2014. The Series B Warrants are exercisable during the period beginning on June 20, 2010 and ending on June 22, 2011. The Investor Warrants have a per share exercise price of $2.45. In connection with this offering we paid a placement agent cash compensation equaled to 6.5% of the gross proceeds or $487,183 plus a five-year warrant at an exercise price of $2.50

per share for the purchase of up to 74,951 shares of our common stock, on the same terms as the investor warrants described above.

In April 2010, we entered into a $15 million equity line facility with LPC, which allows us to sell shares of our common stock every two days if our selling price to LPC is over $1.50 per share. Cumulatively, as of November 5, 2010, we have sold approximately 2.2 million shares under the equity line raising approximately $4.9 million in gross proceeds to us. We plan to continue to utilize, when available and if needed, the equity line to fund our future cash needs which could create additional pressure on our common stock price as LPC resells its shares of our common stock into the market. On April 23, 2010, we filed a registration statement on Form S-1 registering the resale by LPC of up to 4.5 million shares of our common stock that have been issued or may be issued to LPC under the equity line. Such registration statement was declared effective by the SEC on May 7, 2010.

In August 2010, we entered into the 2010 Private Placement Purchase Agreements with the 2010 Private Placement Investors for the private placement of our common stock and warrants to purchase our common stock, at a purchase price of $3.075 per unit, with each unit comprised of one share of common stock and a warrant to purchase one share of common stock. We issued and sold an aggregate of 4,897,614 units, comprised of an aggregate of 4,897,614 shares of common stock and warrants to purchase up to 4,897,614 shares of our common stock for gross proceeds of approximately $15.1 million. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share.

Our Chief Executive Officer, our Chief Financial Officer and each of the members of our Board of Directors own, in the aggregate, 935,405 shares, or approximately 3% of our outstanding common stock as of November 5, 2010. Sales of a substantial number of shares of our common stock by such officers and directors in the public trading market, whether in a single transaction or a series of transactions, or the perception that these sales may occur, could also have a significant effect on volatility and the trading price of our common stock.

As of November 5, 2010, there were (i) outstanding warrants to purchase 10,236,609 shares of our common stock at a weighted average exercise price of $2.86 per share issued in connection with the transactions described above and other equity issuances, (ii) outstanding options to purchase 1,777,179 shares of our common stock outstanding under our 2010 and 2006 Raptor stock option plans at a weighted-average exercise price of $2.58, (iii) options to purchase 157,667 shares of our common stock outstanding under our TorreyPines Therapeutics stock option plans at a weighted-average exercise price of $106.89 and (iv) 2,153,670 shares of our common stock available for issuance under our 2010 Raptor Pharmaceutical stock option plan. The shares issuable under our stock option plans will be available for immediate resale in the public market. The shares issuable under the warrants are available for immediate resale in the public market. The market price of our common stock could decline as a result of such resales due to the increased number of shares available for sale in the market.

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

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.

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.

The sale of our common stock to LPC may cause dilution and the sale of the shares of common stock acquired by LPC could cause the price of our common stock to decline.

In connection with entering into the LPC Purchase Agreement, we authorized the sale to LPC of up to 4,137,418 shares of our common stock and the issuance of an additional 362,582 shares of our common stock as a commitment fee. The number of shares ultimately offered for sale by LPC is dependent upon the number of shares purchased by LPC under the LPC Purchase Agreement. The purchase price for the common stock to be sold to LPC pursuant to the

LPC Purchase Agreement will fluctuate based on the price of our common stock. All 4.5 million shares of our common stock which may be sold by us to LPC under the LPC Purchase Agreement are expected to be freely tradable. Depending upon market liquidity at the time, a sale of shares by LPC at any given time could cause the trading price of our common stock to decline. We can elect to direct purchases by LPC in our sole discretion but no sales to LPC may occur if the purchase price for our common stock under the Purchase Agreement is below $1.50 per share and therefore, LPC may ultimately purchase all or some of the 4,137,418 shares of common stock. As of November 5, 2010, we have sold approximately 2.2 million shares to LPC under the LPC Purchase Agreement. After LPC has acquired such shares, it may sell all, some or none of such shares. Therefore, sales to LPC by us under the LPC Purchase Agreement may result in substantial dilution to the interests of other holders of our common stock. The sale of a substantial number of shares of our common stock, or anticipation of such sales, by LPC could make it more difficult for us to sell equity or equity-related securities in the future at a time and at a price that we might otherwise wish to effect sales.

The sale of our common stock and common stock underlying warrants to the 2010 Private Placement Investors could cause the price of our common stock to decline.

In connection with the 2010 Private Placement, we issued and sold an aggregate of 4,897,614 units, comprised of an aggregate of 4,897,614 shares of common stock and warrants to purchase up to 4,897,614 shares of our common stock. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share. In connection with the 2010 Private Placement, the Placement Agent was issued one warrant, with an exercise price of $3.075 per share, to purchase 97,952 shares of our common stock. The warrant issued to the Placement Agent may not be exercised until the sixth month anniversary of the issuance date of August 12, 2010. The resale of all 9,893,180 shares which have been sold or upon exercise of the warrants may be sold by us to the 2010 Private Placement Investors and the Placement Agent has been registered on a Form S-1, which was declared effective by the SEC on August 31, 2010. Depending upon market liquidity at the time, a sale of shares under this offering at any given time could cause the trading price of our common stock to decline. Sales of our common stock to the 2010 Private Placement Investors and the Placement Agent upon exercise of the warrants they received in connection with 2010 Private Placement by us may result in substantial dilution to the interests of other holders of our common stock. The sale of a substantial number of shares of our common stock or anticipation of sales, by the 2010 Private Placement Investors and the Placement Agent could make it more difficult for us to sell equity or equity-related securities in the future at a time and at a price that we might otherwise wish to effect sales.

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 15g-9 under the Exchange Act 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.

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 1B: UNRESOLVED STAFF COMMENTS

None.

## ITEM 2: PROPERTIES

In March 2006, we entered into a lease for our 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, we paid $20,207 as a security deposit on this lease. Effective April 1, 2007, we leased additional office space adjoining the existing leased space, increasing our 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, our 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, we 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, we entered into a one year lease for administrative offices in San Mateo, California for $2,655 per month. During the years ended August 31, 2010 and 2009 and the cumulative period from September 8, 2005 (inception) to August 31, 2010, we paid $150,536, $128,830, and $518,947, respectively, in rent. We plan to continue to lease administrative offices in San Mateo, California and we plan to expand our Novato office space by approximately 3,100 square feet ($5,309 per month) in January 2011. We anticipate that the expanded space in Novato will be sufficient for the near future.

## ITEM 3: LEGAL PROCEEDINGS

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

## ITEM 4: (REMOVED AND RESERVED)

PART II

ITEM 5: MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Market Information

In connection with the closing of the 2009 Merger, our common stock commenced trading on the NASDAQ Capital Market on September 30, 2009, under the ticker symbol "RPTPD" with 18,822,162 shares outstanding. Effective October 27, 2009, our ticker symbol changed to "RPTP." There is no public trading market for our warrants. The closing price for our common stock on November 17, 2010 was $3.52.

The following table sets forth the range of high and low sales prices of our common stock for the quarterly periods indicated, as reported by NASDAQ. Such quotations represent inter-dealer prices without retail mark up, mark down or commission and may not necessarily represent actual transactions.

| | High | Low |
|---|---|---|
| Year Ended August 31, 2010: | | |
| First Quarter (through September 29)* | $ 7.14 | $ 3.23 |
| First Quarter (September 30 – November 30, 2009) | 4.90 | 1.16 |
| Second Quarter (December 1, 2009 – February 28, 2010) | 3.30 | 1.75 |
| Third Quarter (March 1 – May 31, 2010) | 3.88 | 1.41 |
| Fourth Quarter (June 1, 2010 – August 31, 2010) | 3.57 | 2.37 |
| Year Ended August 31, 2009: | | |
| First Quarter * | $ 11.73 | $ 2.72 |
| Second Quarter * | 5.95 | 2.72 |
| Third Quarter * | 7.65 | 2.55 |
| Fourth Quarter | 11.73 | 1.19 |

* Market prices reported have been adjusted to give retroactive effect to material changes resulting from thereverse stock split that occurred immediately prior to the consummation of the 2009 Merger on September 29,2009 by multiplying the reported sales prices for such periods by 17.

Holders of Record

As of November 5, 2010, there were approximately 83 holders of record of our common stock and 30,213,378 shares of our common stock outstanding, excluding shares held in book-entry form through The Depository Trust Company, and we estimate that the number of beneficial owners of shares of our common stock was approximately 5,400 as of such date. Additionally, on such date, options, held by 64 persons to acquire up to, in the aggregate, 1,934,846 shares, and warrants held by 53 persons to acquire up to, in the aggregate, 10,236,609 shares, of our common stock, were outstanding.

Dividends

We have never declared or paid cash dividends on our common stock and do not anticipate paying any cash dividends on our shares of common stock in the foreseeable future. We expect to retain future earnings, if any, for use in our development activities and the operation of our business. The payment of any future cash dividends will be subject to the discretion of our board of directors and will depend, among other things, upon our results of operations, financial condition, cash requirements, prospects and other factors that our board of directors may deem relevant. Additionally, our ability to pay future cash dividends may be restricted by the terms of any future financing.

Purchase of Equity Securities and Affiliated Purchasers

We have not repurchased any shares of our common stock since inception. For a discussion regarding our unregistered equity issuances during our fiscal year ended August 31, 2010, please refer to the shares issued pursuant to the LPC Purchase Agreement and to the 2010 Private Placement described under the heading, “Post-Merger Financings - Equity Line Facility with Lincoln Park Capital Fund, LLC, or LPC,” and “2010 Private Placement”, respectively, under Part I, Item 1 to this Annual Report on Form 10-K which discussion is incorporated herein by reference. The table below reflects all of the shares of our common stock issued pursuant to the LPC Purchase Agreement:

| Issuance Date | Number of Shares of Common Stock Issued |
|---|---|
| April 16, 2010 | 145,033 |
| May 7, 2010 | 190,576 |
| May 11, 2010 | 188,169 |
| May 13, 2010 | 183,844 |
| May 17, 2010 | 173,923 |
| May 19, 2010 | 163,702 |
| May 24, 2010 | 149,002 |
| May 25, 2010 | 143,363 |
| May 27, 2010 | 133,614 |
| June 1, 2010 | 113,466 |
| June 3, 2010 | 99,326 |
| June 7, 2010 | 101,614 |
| June 9, 2010 | 104,129 |
| June 11, 2010 | 104,239 |
| June 15, 2010 | 104,129 |
| June 29, 2010 | 41,449 |
| July 2, 2010 | 40,975 |
| July 7, 2010 | 42,266 |
| July 9, 2010 | 39,960 |
| July 13, 2010 | 39,960 |
| July 15, 2010 | 40,512 |
| July 17, 2010 | 43,644 |
| | 2,386,895 |

ITEM 6: SELECTED FINANCIAL DATA

Per Item 301(c) of Regulation S-K, information is not required.

ITEM 7: MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

## PLAN OF OPERATION

### Overview

You should read the following discussion in conjunction with our consolidated financial statements as of August 31, 2010, and the notes to such consolidated financial statements included elsewhere in this Annual Report on Form 10-K. 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 Annual Report on Form 10-K, particularly under the heading "Risk Factors."

Unless otherwise mentioned or unless the context requires otherwise (e.g., our consolidated financial statements as of August 31, 2010, and the notes to such consolidated financial statements included elsewhere in this Annual Report on Form 10-K, or a reference to an event or circumstance that occurred prior to the effective time of the 2009 Merger on September 29, 2009), all references in this Annual Report on Form 10-K to "we," "us," "our," the "Company," "Raptor" a similar references refer to the public company formerly known as TorreyPines Therapeutics, Inc. and now known as Raptor Pharmaceutical Corp., including its wholly-owned direct and indirect subsidiaries (which includes Raptor Pharmaceuticals Corp., Raptor Discoveries Inc., Raptor Therapeutics Inc. and Raptor Pharmaceuticals Europe BV), following the name change and completion of the 2009 Merger. On August 30, 2010, our former wholly-owned subsidiary, TPTX, Inc. was merged into Raptor Therapeutics Inc.

### Plan of Operation and Overview

We believe that we are building a balanced pipeline of drug candidates that may expand the reach and benefit of existing therapeutics. Our product portfolio includes both candidates from our proprietary drug targeting platforms and in-licensed and acquired product candidates.

Our current pipeline includes three clinical development programs which we are actively developing. We also have 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, an inherited neurodegenerative disorder.

Other Clinical-Stage Product Candidates

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

· 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 other liver diseases; and NeuroTrans™ to potentially deliver therapeutics across the blood-brain barrier for treatment of a variety of neurological diseases.

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

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 new 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. In addition, if we do not raise 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.

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

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

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

The EMA and FDA granted orphan drug designation for DR Cysteamine for the treatment of cystinosis in 2010 and 2006, respectively.

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

On June 28, 2010, we commenced our Phase 3 clinical trial, designed as a multi-center, randomized, crossover, outpatient study of the safety, tolerability, pharmacokinetics, or PK, and pharmacodynamics, or PD, of every 12-hour DR Cysteamine compared to immediate-release cysteamine bitartrate in cystinosis patients. The design of our Phase 3 clinical trial is a result of discussions with the FDA under a Special Protocol Assessment, or SPA, process by which the FDA provided significant guidance on trial protocol design, clinical endpoints, and statistical analyses. The primary endpoint of our study is the steady-state white blood cell, or WBC, cystine levels of patients taking DR Cysteamine compared to immediate-release cysteamine bitartrate. Secondary endpoints are the safety and tolerability of DR Cysteamine and the comparability of steady-state PK of DR Cysteamine and immediate-release cysteamine bitartrate in cystinosis patients. Our Phase 3 clinical trial is being conducted at nine sites in North America and Europe. We expect to enroll at least 30 patients. Patients who complete the nine-week clinical trial will be offered enrollment into our long-term follow-on study. We anticipate that our Phase 3 clinical trial enrollment will be completed in December 2010. If DR Cysteamine is approved by the FDA, we plan to commercialize DR Cysteamine in the U.S. by ourselves. However, we may enter into marketing partnerships for certain 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 May 2010, we presented positive Phase 2a clinical trial results from our pilot study of delayed-release cysteamine bitartrate in 11 adolescent patients with NASH, a progressive form of liver disease believed to affect 5% to 11% of the U.S. population. The results were presented at the Digestive Disease Week 2010 conference in New Orleans, Louisiana on May 2, 2010. Our open-label Phase 2a clinical trial was conducted under a collaboration agreement with UCSD at UCSD's General Clinical Research Center. Eligible patients with baseline levels of the liver enzymes alanine transaminase, or ALT, and aspartate aminotransferase, or AST, that were at least twice that of normal levels, were enrolled to receive twice-daily, escalating oral doses of up to 1,000 mg of delayed-release cysteamine bitartrate (a prototype of our DR Cysteamine) for six months, followed by a six-month post-treatment monitoring period.

Patients showed a marked decline in ALT levels during the treatment period with 7 of 11 patients achieving a greater than 50% reduction and 6 of 11 reduced to within normal range. AST levels also saw significant improvements with patients averaging 41% reduction by the end of the treatment phase. The reduction in liver enzymes was largely sustained during the 6 month post-treatment monitoring phase. Other important liver function markers showed positive trends. Levels of cytokeratin 18, a potential marker of disease activity in Non-alcoholic Fatty Liver Disease, or NAFLD, decreased by an average of 45%. Adiponectin levels increased by an average of 35% during the treatment period. Reduced adiponectin levels are thought to be a marker of the pathogenesis and progression of NASH. Body Mass Index, or BMI, did not change significantly during both the treatment and post-treatment phases. Delayed-release cysteamine bitartrate demonstrated a strong, favorable safety profile, with mean gastrointestinal symptom scores of 1.1 at baseline and 0.7 after 6 months of treatment using a rating system in which the maximum score of 14 indicates most severe gastrointestinal symptoms.

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

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

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

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

In June 2010, we acquired an exclusive worldwide license to intellectual property related to the potential treatment of Huntington's Disease from the Weizmann Institute of Science in Israel and Niigata University in Japan. The Weizmann and Niigata patents cover the use of transglutaminase inhibitors, a class of molecules chemically similar to cysteamine, in the potential treatment of Huntington's Disease and other neurological disorders. These patents add to our portfolio of intellectual property related to our programs utilizing DR Cysteamine.

Other Clinical-Stage Product Candidates

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 2a dose escalation clinical trial of oral 4-MP with ethanol in ALDH2 deficient patients. The study results demonstrated that the active ingredient in Convivia™ significantly reduced heart palpitations (tachycardia), which are commonly experienced by ALDH2 deficient people who drink, at all dose levels tested. The study also found that the 4-MP significantly reduced peak acetaldehyde levels and total acetaldehyde exposure in a subset of the study participants who possess specific genetic variants of the liver ADH and ALDH2 enzymes. We believe that this subset represents approximately one-third of East Asian populations.

In June 2010, we entered into an exclusive agreement with Uni Pharma Co., Ltd., or Uni Pharma to commercialize Convivia™ in Taiwan. Under terms of the agreement, we will grant to Uni Pharma an exclusive license under all relevant patent applications, trademarks and future patents controlled by us to market Convivia™ in Taiwan, with an option to expand the license to South Korea under the same terms. Uni Pharma will register Convivia™ for drug licensure for existing indications and will conduct a clinical trial and register ConviviaTM for acetaldehyde toxicity

resulting from ALDH2 deficiency. Uni Pharma will be responsible for marketing and sales activities for the commercialization of Convivia™ in the markets covered under the license agreement. We continue to seek potential partners in other Asian countries to continue clinical development of ConviviaTM in those countries.

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 2b 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 2 trial in which tezampanel has been shown to have analgesic activity. Based on a review of the Phase 2 data, the FDA has agreed that tezampanel may move forward into a Phase 3 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 or HCC and Other Liver Diseases

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

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

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

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

· RAP is captured by hepatocytes with efficiency, primarily on first-pass.

· Late-stage HCC is perfused exclusively by the hepatic artery, while the majority of the liver is primarily perfused through the portal vein.

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

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

NeuroTrans™ for the Potential Treatment of Diseases Affecting the Brain

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

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

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

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

In June 2009, we entered into a collaboration and licensing agreement with F. Hoffman — La Roche Ltd. and Hoffman—La Roche Inc., or Roche, to evaluate therapeutic delivery across the blood-brain barrier utilizing NeuroTrans™. Under the terms of the agreement, Roche has funded studies of select molecules attached to NeuroTrans™. The agreement provides Roche with an exclusive worldwide license to NeuroTrans™ for use in the delivery of diagnostic and therapeutic molecules across the blood-brain barrier. Roche's and our scientists are actively collaborating on the project. We have received an initial upfront payment for the collaboration to cover our portion of the initial studies, and may earn development milestone payments 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. The paper, titled, “LRP6 Overexpression Defines a Class of Breast Cancer Subtype and Is a Target for Therapy,” presented results that support the potential efficacy of WntTide™ as a targeted treatment for triple-negative breast cancers, a particularly aggressive and difficult-to-treat indication for recurrent and metastatic disease. Abnormal Wnt activation, found in 40% to 60% of breast cancers, is often associated with triple-negative breast cancers. We are currently evaluating WntTide™ in a preclinical breast cancer model to inhibit the Wnt-signaling pathway designed to block cancers dependent upon signaling through LRP6, as well as other IND enabling studies.

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 currently manufacturing drug product for a Phase 1 clinical trial in collaboration with a university hospital anticipated to commence in mid-calendar 2011.

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 Convivia™

In October 2007, prior to the 2009 Merger, Raptor Pharmaceuticals Corp. purchased certain assets of Convivia, Inc., or Convivia, including intellectual property, know-how and research reports related to a product candidate targeting liver ALDH2 deficiency, a genetic metabolic disorder. Raptor Pharmaceuticals Corp. hired Convivia's chief executive

officer and founder, Thomas E. (Ted) Daley, as the President of its clinical development division. In exchange for the assets related to the ALDH2 deficiency program, what we now call Convivia™, Raptor Pharmaceuticals Corp. issued to Convivia 46,625 shares of our common stock, an additional 46,625 shares of our common stock to a third party in settlement of a convertible loan between the third party and Convivia, and another 8,742 shares of our common stock in settlement of other obligations of Convivia. Mr. Daley, as the former sole stockholder of Convivia, may earn additional shares of our common stock based on certain triggering events or milestones related to the development of the Convivia assets. In addition, Mr. Daley may earn cash bonuses based on the same triggering events pursuant to his employment agreement. In January 2008, Mr. Daley earned a $30,000 cash bonus pursuant to his employment agreement as a result of the milestone of our execution of a formulation agreement for manufacturing Convivia™ with Patheon. In March 2008, Raptor Pharmaceuticals Corp. issued to Mr. Daley 23,312 shares of our common stock pursuant to the Convivia purchase agreement as a result of the milestone of our execution of an agreement to supply us with the active pharmaceutical ingredient for Convivia™ and two $10,000 cash bonuses pursuant to his employment agreement for reaching his six-month and one-year employment anniversaries. In October 2008, Raptor Pharmaceuticals Corp. issued to Mr. Daley 23,312 shares of our common stock valued at $27,000 and a $30,000 cash bonus as a result of fulfilling a clinical milestone. In July 2010, we issued 11,656 shares of our restricted common stock valued at $35,551 and paid a $10,000 cash bonus to Mr. Daley as result of the execution of the license agreement with Uni Pharma for the development of ConviviaTM in Taiwan.

Purchase of DR Cysteamine

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

As a result of the Encode merger, we received the exclusive worldwide license to DR Cysteamine, referred to herein as the License Agreement, developed by clinical scientists at the UCSD School of Medicine. In consideration of the grant of the license, we are obligated to pay an annual maintenance fee of $15,000 until we begin commercial sales of any products developed pursuant to the License Agreement. In addition to the maintenance fee, we are obligated to pay during the life of the License Agreement: milestone payments ranging from $20,000 to $750,000 for orphan indications and from $80,000 to $1,500,000 for non-orphan indications upon the occurrence of certain events, if ever; royalties on commercial net sales from products developed pursuant to the License Agreement ranging from 1.75% to 5.5%; a percentage of sublicense fees ranging from 25% to 50%; a percentage of sublicense royalties; and a minimum annual royalty commencing the year we begin commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, we are obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 years from the effective date of the License Agreement, depending on the indication. In addition, we are obligated, among other things, to spend annually at least $200,000 for the development of products (which we satisfied, as of August 31, 2010 and 2009 by spending approximately $6.2 million and $4.1 million, respectively, on such programs) pursuant to the License Agreement. To-date, we have accrued $470,000 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