

Raptor Pharmaceutical Corp
Form 10-K
November 14, 2011

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

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

For the fiscal year ended August 31, 2011

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
(State or other jurisdiction of incorporation or
organization)

86-0883978
(I.R.S. Employer Identification No.)

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

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

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

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Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes [] 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 [] 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 []

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. []

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

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

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of February 28, 2011 (the last business day of the registrant’s most recently completed second quarter) was \$103.6 million.

Indicate the number of shares outstanding of each of the registrant’s classes of common stock, as of the latest practicable date: 47,153,503 shares common stock, par value \$0.001, outstanding as of October 31, 2011

The documents incorporated by reference are as follows:

None.

DOCUMENTS INCORPORATED BY REFERENCE

Certain information of Part III of this Annual Report on Form 10-K incorporates information by reference from the registrant’s proxy statement for the registrant’s 2012 Annual Meeting of Stockholders.

RAPTOR PHARMACEUTICAL CORP.

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PART I
FORWARD-LOOKING STATEMENTS

In this Annual Report on Form 10-K, in our other filings with the Securities and Exchange Commission, or the SEC, and in press releases and other public statements by our officers throughout the year, we make or will make statements that plan for or anticipate the future. These “forward-looking statements,” within the meaning of the Private Securities Litigation Reform Act of 1995, include statements about our future business plans and strategies, as well as other statements that are not historical in nature. These forward-looking statements are based on our current expectations.

In some cases, these statements can be identified by the use of terminology such as “believes,” “expects,” “anticipates,” “plans,” “may,” “might,” “will,” “could,” “should,” “would,” “projects,” “anticipates,” “predicts,” “intends,” “continues,” “opportunity” or the negative of these terms or other comparable terminology. All such statements, other than statements of historical facts, including our financial condition, future results of operations, projected revenues and expenses, business strategies, operating efficiencies or synergies, competitive positions, growth opportunities for existing intellectual properties, technologies, products, plans, and objectives of management, markets for our securities, and other matters, are about us and our industry that involve substantial risks and uncertainties and constitute forward-looking statements for the purpose of the safe harbor provided by Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements, wherever they occur, are necessarily estimates reflecting the best judgment of our senior management on the date on which they were made, or if no date is stated, as of the date of the filing made with the SEC in which such statements were made. You should not place undue reliance on these statements, which only reflect information available as of the date that they were made. Our business’ actual operations, performance, development and results might differ materially from any forward-looking statement due to various known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled “Risk Factors” in Part I, Item 1A of this Annual Report on Form 10-K and including, but not limited to, the following:

- our need for, and our ability to obtain, additional funds;
- uncertainties relating to clinical trials and regulatory reviews;
- our dependence on a limited number of therapeutic compounds and formulations of these compounds;
- the early stage of the products we are developing;
- our ability to successfully launch and commercialize our future products;
- the acceptance of any of our future products by physicians and patients;
- our ability to adequately supply drug product to meet demand for our future products;
- 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” in Part I, Item 1A of this Annual Report on Form 10-K, 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 US federal securities laws and the rules and regulations of the SEC, we do not undertake any obligation and disclaim any intention to update or release publicly any revisions to these forward-looking statements after the filing of this 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

You should read the following discussion in conjunction with our consolidated financial statements as of August 31, 2011, and the notes to such consolidated financial statements included elsewhere in this Annual Report on Form 10-K. This “Business” 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, 2011, 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” and 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..

Overview

Our goal is to research, produce, and deliver medicines that improve life for patients with severe, rare disorders. Our product portfolio includes both candidates from our proprietary drug targeting platforms and in-licensed and acquired product candidates. Our current pipeline includes three clinical development programs, which we are actively developing. We also have two other clinical-stage product candidates, one of which we are seeking additional business development partners in Asia but are not internally developing, and we have three preclinical product candidates for which we are seeking development partners.

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, or RP103, for the potential treatment of nephropathic cystinosis, or cystinosis, a rare genetic disorder; and
- RP103 for the potential treatment of Huntington’s Disease, or HD, an inherited neurodegenerative disorder.

RP103 is our proprietary delayed-release formulation of cysteamine bitartrate microbeads in capsules, which may require less frequent dosing and reduce gastro-intestinal side effects compared to the current standard of care.

- RP104, for the potential treatment of non-alcoholic steatohepatitis, or NASH, a metabolic disorder of the liver.

RP104 is our proprietary delayed-release formulation of cysteamine bitartrate in tablets.

Other Clinical-Stage Product Candidates

Our other clinical-stage product candidates include:

- Convivia™ for the potential management of acetaldehyde toxicity due to alcohol consumption by individuals with aldehyde dehydrogenase, or ALDH2 deficiency, an inherited metabolic disorder; and
- Tezampanel, a glutamate receptor antagonist as a potential anti-platelet agent, and NGX 426 (oral tezampanel and together with tezampanel are referred to as tezampanel hereafter).

Preclinical Product Candidates

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

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

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Future Activities

Over the next 12 months, we plan to conduct research and development and general and administrative activities, including: pre-commercial preparation for the potential launch of RP103 for the treatment of cystinosis in the United States and Europe; supporting our ongoing extension study of RP103 in cystinosis; supporting the ongoing clinical trial of RP103 in HD; funding a potential collaboration of a clinical trial of RP104 in NASH; funding a potential Phase 1 clinical trial of tezampanel as a potential anti-platelet agent; continued business development of our preclinical product candidates; and supporting associated facilities and administrative functions. We plan to seek additional business development partners in Asia for our Convivia™ product candidate. We may also develop future in-licensed technologies and acquired technologies.

Company History

Corporate Structure; Historical Reverse Mergers

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 the corporate name to “TorreyPines Therapeutics, Inc.”

Raptor Pharmaceuticals Corp. 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 Raptor Pharmaceuticals Corp. 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 “Raptor Pharmaceuticals Corp.” 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.”

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

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

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

Our principal executive office is located at 9 Commercial Blvd., Suite 200, Novato, CA 94949. Our phone number is (415) 382-8111.

As of October 31, 2011, there were 47,153,503 shares of our common stock outstanding.

Exercises of Common Stock Options and Common Stock Warrants

During the cumulative period from September 8, 2005 (inception) through October 31, 2011, we received approximately \$16.0 million from the exercise of warrants in exchange for the issuance of an aggregate of 7.2 million shares of our common stock.

During the cumulative period from September 8, 2005 (inception) through October 31, 2011, we received \$168,609 from the exercise of stock options resulting in the issuance of 80,563 shares of our common stock.

Outstanding Common Stock Warrants

As of October 31, 2011, we had the following warrants outstanding related to the assumption of warrants from our Encode merger, issuance of warrants related to our May/June 2008 private placement, issuance of warrants related to our August 2009 private placement, the assumption of warrants pursuant to the 2009 Merger, issuance of warrants related to our December 2009 registered direct offering and issuance of warrants related to our August 2010 private placement. See Note 10 in our consolidated financial statements attached as an exhibit to this Annual Report on Form 10-K for further discussion regarding our common stock warrants.

	Number of shares exercisable	Exercise price	Expiration date
Issued in connection with Encode merger	233,309	\$ 2.87	12/13/2015
Issued to placement agents in May / June 2008	433,994	\$ 2.36	5/21/2013
Issued to placement agents in August 2009	65,000	\$ 1.50	8/21/2014
TorreyPines warrants assumed in 2009 Merger	8,140	\$ 80.86*	6/11/2013-9/26/2015
Issued to registered direct investors in Dec. 2009	1,556,250	\$ 2.45	12/23/2014
Issued to private placement investors in Aug. 2010	4,539,890	\$ 3.075	8/11/2015
Issued to placement agent in Aug. 2010	97,952	\$ 3.075	8/11/2015
Total warrants outstanding	6,934,535	\$ 2.94*	

* Average exercise price

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 was obligated to purchase from us up to \$15.0 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 to LPC under the LPC Purchase Agreement. The registration statement was declared effective on May 7, 2010. Post-effective amendments to such registration statement were filed on November 23, 2010 and December 1, 2010, which amended registration statement was declared effective by the SEC on December 1, 2010. Post-effective amendments to such amended registration statement were filed on October 11, 2011 and October 14, 2011 on Form S-3, which amended registration statement was declared effective by the SEC on October 21, 2011. After May 7, 2010, over approximately 25 months, generally we had 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 could also accelerate the amount of our common stock to be purchased under certain circumstances. The purchase price of the shares was based on the market prices of our shares at the time of sale as computed under the LPC Purchase Agreement without any fixed discount. Since inception, we have sold 4,186,038 shares to LPC at a weighted average price of \$2.78 and paid commitment fees to LPC in the form of 168,929 shares, valued at \$581,081, (in addition to the 145,033 shares, valued at \$246,556, issued as the initial commitment fee). We have issued an aggregate of 4.5 million shares (including shares issued to LPC as commitment fees) to LPC pursuant to the LPC Purchase Agreement, for aggregate gross proceeds to us of approximately \$11.6 million, and do not plan to issue or register additional shares under such agreement. We may at any time in our sole discretion terminate the LPC Purchase Agreement without fee, penalty or cost upon one business days notice.

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 US Investors) and a separate securities purchase agreement with a certain Canadian investor (or, the Canadian Investor and together with the US 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. As of October 31, 2011, warrants to purchase 357,724 shares were exercised for aggregate gross proceeds to us of approximately \$1.1 million. The balance of warrants to purchase 4,637,842 shares of our common stock remain outstanding as of October 31, 2011.

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. Post-effective amendments to such registration statement were filed on November 23, 2010 and December 1, 2010, which amended registration statement was declared effective by the SEC on December 1, 2010. Post-effective amendment to such amended registration statement was filed on October 11, 2011 on Form S-3, which amended registration statement was declared effective by the SEC on October 21, 2011.

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.

2011 Follow-on Public Offering

On September 13, 2011, we announced the closing of an underwritten public offering of shares of our common stock at a price to the public of \$4.00 per share. The shares sold in the offering included 10.0 million shares of our common stock plus an additional 1.5 million shares of our common stock pursuant to the exercise by JMP Securities LLC, Canaccord Genuity Inc. and Cowen and Company, LLC, the underwriters for the offering, of the over-allotment option we granted to them. Total gross proceeds to us in the offering (including in connection with the sale of the shares of common stock pursuant to the exercise of the over-allotment option) totaled \$46.0 million, before underwriting discounts and commissions. The offering resulted in net proceeds to us of approximately \$42.9 million after deduction of underwriting discounts and other offering expenses payable by us. We expect to use the net proceeds from the offering to fund our commercial and pre-commercial efforts, clinical and preclinical development programs and other general corporate activities.

Proprietary Rights

As of October 31, 2011, we have nine patent applications under prosecution in the US and internationally. Four of these relate to our Convivia™ program, three cover our RAP platform, one covers our WntTide™ program and one covers our thrombotic disorder program. We own the patents and patent applications used by us in the Convivia™ program and the RAP platform while we license the patents and patent applications used by us in the WntTide™ program and thrombotic disorder program. Four patents have been allowed in the US 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 WntTide™ Mesd program for the treatment of cancer and bone diseases, and we fund the prosecution of a patent application covering this technology, which entered national phase in the US and internationally in November 2009. In December 2007, we acquired an exclusive worldwide license agreement to pending patent applications for enterically coated cysteamine from UCSD relating to our RP103/RP104 programs. In March 2008, we amended our license with UCSD to add exclusive worldwide rights to develop RP103/RP104 for the potential treatment of NASH. As discussed below, both cysteamine patents have been issued in the US with expiration dates of 2027 and 2028 and are in the examination phase in Europe and other countries. Through the 2009 Merger, we have a license from Eli Lilly & Co. for the intellectual property related to tezampanel for pain indications and a license of tezampanel for the treatment of thrombotic disorder from Johns Hopkins University, or JHU, and we fund the prosecution of a patent covering this technology, which entered national phase in the US in August 2009. In March 2011, we acquired an exclusive worldwide license to two issued patents related to treatment of chronic pain with intrathecally-administered tezampanel from the University of Iowa (US Patents 7,091,249 and 7,439,270 "Drugs for Spinal Anesthesia"). In June 2010, we acquired an exclusive worldwide license to two issued patents related to the treatment of HD 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. In January 2011, we acquired an exclusive license to a pending patent related to the diagnosis of metabolic liver diseases, including NASH, from the University of Arizona and we are funding the prosecution of the patent.

On July 6, 2011, we announced that the United States Patent and Trademark Office, or USPTO, issued Notices of Allowance for two patents covering our delayed-release oral formulation of cysteamine bitartrate, or DR Cysteamine (being developed as RP103 and RP104), as well as other formulations of cystamine and cysteamine as described below. Subsequent to the announcement, on September 27, 2011, the USPTO issued patent number 8,026,284 as outlined below.

US Patent: 8,026,284 (Application No. 11/990,869)
Issued Notice of Allowance: June 27, 2011
Patent Title: "Enterically Coated Cystamine, Cysteamine and Derivatives Thereof."
Expected to Cover: Methods of administering DR Cysteamine to patients for any clinical indication, including nephropathic cystinosis, NASH and HD
Expected Initial Term: 20 years plus 239 days of patent term adjustment; expiring September 22, 2027

Patent application 11/990,869 covers the use of any composition of cysteamine or cystamine, regardless of the specific formulation, that provides increased delivery to the small intestine with pharmacokinetic benefits that allow for less than 4 times daily dosing.

US Patent Application No.: 12/745,504
Issued Notice of Allowance: June 24, 2011
Patent Title: "Methods of Treating Non-Alcoholic Steatohepatitis ("NASH") Using Cysteamine Products."
Expected to Cover: Methods of treating NASH by administering cysteamine or cystamine
Expected Initial Term: 20 years; expiring November 22, 2028

Patent application 12/745,504 covers the use of cysteamine or cystamine, in any formulation, for the treatment of NASH.

Regulatory Exclusivities

Orphan Drug Designation

We have been granted access to an Orphan Drug Designation from the US Food and Drug Administration, or FDA, for use of RP103 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 US 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 US for that indication. Equivalent European regulations may give us ten years of marketing exclusivity for that indication in Europe. RP103 has been granted Orphan Drug Designation by the FDA and the European Medicines Agency, or EMA.

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 US by Mylan Pharmaceuticals, and by Recordati and Swedish Orphan International in markets outside of the US. Cystagon® was approved by the FDA in 1994 and by the EMA in 1997 and is the standard of care for cystinosis treatment.

While we believe that our RP103 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 on the market and may compete with our product.

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 US 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. To our knowledge, our product candidate is the only compound in development which specifically targets the fundamental metabolic defect of the disease, with the goal of slowing disease progression.

Companies with HD product candidates in development include 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 terminated 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 US 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 potential 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.

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 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.

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 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.”

Government Regulations of the Biotechnology Industry

Regulation by governmental authorities in the US 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.

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 US, 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 US 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 Current Good Manufacturing Practices, or 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 cGMPs 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 US, the drug product candidate may be exported for sale outside of the US 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 recently joined the Department of Cancer Biology at the Dana Farber Cancer Institute.

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, or 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, 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.

Lawrence Steinman, M.D. is a leader in Multiple Sclerosis, or MS, research and currently serves as the George A. Zimmermann Professor of Neurology and Neurological Sciences, Pediatrics and Genetics at Stanford University. Dr. Steinman is the inaugural holder of the chair, funded to support MS research. He is also the chair of the Stanford University Program in Immunology. Dr. Steinman has received various awards for his scientific contributions to MS research, including the John M. Dystel Prize from the American Academy of Neurology and the National MS Society. He was also a two-time recipient of a Javits Neuroscience Award from the US Congress and the National Institutes of Health. Dr. Steinman's research focuses on what provokes relapses and remissions in MS, the nature of the genes that serve as a brake on brain inflammation, and the quest for a vaccine against MS. He has developed two antigen specific therapies using DNA vaccines for MS and type 1 diabetes. He was senior author on the seminal 1992 Nature article that reported the key role of a particular integrin in brain inflammation and led to the development of the drug Tysabri®. Dr. Steinman received his B.A. from Dartmouth College and his M.D. from Harvard University.

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, clinical and commercial development of our clinical drug candidates, RAP based platforms and complementary technologies to provide therapies that we believe will be safer, less intrusive, and more effective than current approaches in treating a wide variety of genetic disorders, neurodegenerative diseases, metabolic disorders and cancer. During the period from September 8, 2005 (inception of Raptor Pharmaceuticals Corp.) to August 31, 2011, we incurred approximately \$39.2 million (\$14.8 million and \$9.3 million for the years ended August 31, 2011 and 2010, 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, 2011.

Compliance with Environmental Laws

We estimate the annual cost of compliance with environmental laws, comprised primarily of hazardous waste removal, will be nominal.

Employees

We presently have 12 full time employees and two part-time employees. Of the 12 employees, 4 are in general and administrative and 8 are in research and development. Based on our current plan, over the next 12 month period, we plan to add several personnel in the areas of sales and marketing, regulatory, clinical, medical affairs, quality and finance. 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, 2011 have been prepared assuming that we will continue as a going concern. As of August 31, 2011, we had an accumulated deficit of approximately \$78.0 million. We expect to continue to incur losses for the foreseeable future and will have to raise substantial cash to fund our planned operations. Our recurring losses from operations and our stockholders’ deficit raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the year ended August 31, 2011, 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.

On September 13, 2011, we announced the closing of an underwritten public offering of shares of our common stock at a price to the public of \$4.00 per share. The shares sold in the offering included 10.0 million shares of our common stock plus an additional 1.5 million shares of our common stock pursuant to the exercise by JMP Securities LLC, Canaccord Genuity Inc. and Cowen and Company, LLC, the underwriters for the offering, of the over-allotment option we granted to them. Total gross proceeds to us in the offering (including in connection with the sale of the shares of common stock pursuant to the exercise of the over-allotment option) totaled \$46.0 million, before underwriting discounts and commissions. The offering resulted in net proceeds to us of approximately \$42.9 million after deduction of underwriting discounts and other offering expenses payable by us. We expect to use the net proceeds from the offering to fund our commercial and pre-commercial efforts, clinical and preclinical development programs and other general corporate activities.

We believe our cash and cash equivalents as of September 30, 2011 of approximately \$56.1 million, which include the net proceeds of approximately \$42.9 million from our September 2011 public offering, will be sufficient to meet our obligations at least through the first calendar quarter of 2013.

Even with the September 2011 offering, 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 are restricted from selling additional shares of our common stock under the September 2011 offering underwriting agreement until December 12, 2011, we may issue shares in connection with the exercise of warrants and/or stock options prior to that date. 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. Some of our drug product candidates are in the concept stage and have not undergone significant testing in preclinical studies or any testing in later-stage 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 US 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.

Although our lead product candidate is in a later stage of development, most 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.

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

Any regulatory approvals that we obtain for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials and surveillance to monitor the safety and efficacy of the product candidate. In addition, if the FDA approves a product candidate, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements may include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMPs or GCPs, and good laboratory practices. If we do not comply with the applicable regulations and requirements, the range of possible sanctions includes issuance of adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and enforcement actions, including injunctions and civil or criminal prosecution. The FDA and comparable international regulatory agencies can withdraw a product's approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient. If data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is delayed or withdrawn, the value of our Company and our operating results will be adversely affected.

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

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

If the purchase or licensing agreements we entered into are terminated, we will lose the right to use or exploit our owned and licensed technologies, in which case we will have to delay or terminate some or all of our research and

development programs, our financial condition and operating results will be adversely affected and we may have to cease our operations.

We entered into an asset purchase agreement with BioMarin Pharmaceutical Inc., or BioMarin, for the purchase of intellectual property related to the RAP, technology, a licensing agreement with Washington University for mesoderm development protein, or Mesd, a licensing agreement with UCSD for RP103/RP104, a licensing agreement with Yeda Research and Development Company Limited, or Yeda, for patents originating from Weizmann Institute of Technology and Niigata University, related to use of transglutaminase inhibitors to treat neurological diseases, and University of Arizona, or UA, for a pending patent related to a diagnostic technology for metabolic liver diseases. BioMarin, Washington University, UCSD, Yeda and UA 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, UCSD, Yeda and UA 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, UCSD, Yeda or UA 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, would lose our rights to RP103/RP104, in the case of UCSD, would lose our rights to the Weizmann and Niigata patents in the case of Yeda, and would lose our rights to the liver diagnostic technology in the case of UA. Under such circumstances, we would have no further right to use or exploit the patents, copyrights or trademarks in those respective technologies. If this happens, we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected, and we may have to cease our operations.

If we fail to compete successfully with respect to acquisitions, 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.

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

- conduct research, preclinical testing and human studies;

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- 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.

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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.

Market acceptance and sales of any of our product candidates that we may develop will depend in large part on global reimbursement policies and may be affected by future healthcare reform measures, both in the US and other key international markets. Successful commercialization of our products will depend in part on the availability of governmental and third-party payer reimbursement for the cost of our products. Government health administration authorities, private health insurers and other organizations generally provide reimbursement. In particular, in the US, private health insurers and other third-party payers often provide reimbursement for treatments based on the level at which the government (through the Medicare or Medicaid programs) provides reimbursement for such treatments. In the US, the European Union and other significant or potentially significant markets for our product candidates, government authorities and third-party payers are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Further, the increased emphasis on managed healthcare in the US and on country and regional pricing and reimbursement controls in the European Union will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our product sales, if approved, and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general. For our product candidates, we will not know what the reimbursement rates will be until we are ready to market the product and we actually negotiate the rates. If we are unable to obtain sufficiently high reimbursement rates for our products, they may not be commercially viable.

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

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

and expansion of the Medicaid program.

Several provisions of the new law, which have varying effective dates, may affect us, including our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. The PPACA also expands the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and includes a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or “donut hole.” The law also revised the definition of “average manufacturer price” for reporting purposes, which could increase the amount of the Medicaid drug rebates paid to states. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with health care practitioners.

The reforms imposed by the new law will significantly impact the pharmaceutical industry; however, the full effects of the PPACA cannot be known until these provisions are implemented and the Centers for Medicare & Medicaid Services and other federal and state agencies issue applicable regulations or guidance. Moreover, in the coming years, additional changes could be made to governmental healthcare programs that could significantly impact our product candidates. We will continue to evaluate the PPACA, as amended, the implementation of regulations or guidance related to various provisions of the PPACA by federal agencies, as well as trends and changes that may be encouraged by the legislation and that may potentially have an impact on our business over time.

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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 US

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

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

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

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

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

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

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

We do not currently manufacture our drug product candidates and do not currently plan to develop the capacity to do so. The manufacture of pharmaceutical products requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties

in production, particularly in scaling up initial production. These problems include difficulties with production costs and yields, quality control, including stability of the product candidate and quality assurance testing, shortages of qualified personnel, as well as compliance with strictly enforced federal, state and foreign regulations. Our third-party manufacturers and key suppliers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes, unstable political environments at foreign facilities or financial difficulties. If these manufacturers or key suppliers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to timely launch any potential product candidate, if approved, would be jeopardized.

In addition, all manufacturers and suppliers of pharmaceutical products must comply with cGMP requirements enforced by the FDA, through its facilities inspection program. The FDA is likely to conduct inspections of our third party manufacturer and key supplier facilities as part of their review of any of our NDAs. If our third party manufacturers and key suppliers are not in compliance with cGMP requirements, it may result in a delay of approval, particularly if these sites are supplying single source ingredients required for the manufacture of any potential product. These cGMP requirements include quality control, quality assurance and the maintenance of records and documentation. Furthermore, regulatory qualifications of manufacturing facilities are applied on the basis of the specific facility being used to produce supplies. As a result, if 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 US. 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 RP103 for the potential treatment of 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 priority review 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 intend to request six-month priority review from the FDA and EMA for our eligible drug product candidates, the FDA may not grant it. Without priority review, the FDA and EMA review timeline could be at least 10 to 12 months. Under the FDA policies, a drug candidate is eligible for priority review from the time a complete NDA is accepted for filing, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A lengthier review process will delay revenue from the sale of products and will increase the capital necessary to fund these product development programs.

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 RP103 targets diseases with small patient populations, including cystinosis and HD. If we are successful in developing RP103 and receive regulatory approval to market RP103 for a disease with a small patient population, the per-patient prices at which we could sell RP103 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 RP103 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 US, 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 any future collaborator/licensee will not be able to produce enough RAP drug product candidates for testing;
- the pharmacokinetics, or where the drug distributes in the body, of our RAP drug product candidates will preclude sufficient binding to the targeted receptors on the

blood-brain barrier;

- the targeted receptors are not transported across the blood-brain barrier;
- other features of the blood-brain barrier, apart from the cells, block access molecules to brain tissue after transport across the cells;
- the targeted receptors are expressed on the blood-brain barrier at densities insufficient to allow adequate transport of our RAP drug product candidates into the brain;
- targeting of the selected receptors induces harmful side-effects which prevent their use as drugs; or
- that we or our 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 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 US, our sales in the US may be reduced if our products are imported into the US from lower priced markets, whether legally or illegally. In the US, 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 US. 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 \$5.0 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, Dr. Patrice P. Rioux, Chief Medical Officer of our clinical development subsidiary, Patrick Reichenberger, Vice President, Commercial Operations of our clinical development subsidiary and Kathlene Powell, Vice President, Quality Operations 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.

We expect to continue to grow, which could strain our managerial, operational, financial and other resources. With the potential commercial launch of RP103 for cystinosis, the continued progress of our clinical-stage programs and with our plan to in-license and acquire additional clinical-stage product candidates, we will be required to retain experienced personnel in the commercial, 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 US 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 US.

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 were exercisable during the period beginning on June 20, 2010 and ended 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. As of October 31, 2011, the Series B Warrants expired and 100% of the Series B Warrants were exercised prior to expiration, generating aggregate gross proceeds of approximately \$4.6 million in exchange for the issuance of 1,873,779 shares of our common stock. In addition, in June 2011, placement agent warrants were exercised for gross proceeds of \$187,378 in exchange for the issuance of 74,951 shares of our common stock. As of October 31, 2011, there were Series A Warrants to purchase 1,556,250 shares of our common stock outstanding.

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. As of October 31, 2011, warrants to purchase 357,724 shares were exercised for aggregate gross proceeds of approximately \$1.1 million. The balance of warrants to purchase 4,637,842 shares of our common stock remain outstanding as of September 30, 2011.

Our executive officers and our board of directors own, in the aggregate, 1,744,628 shares, or approximately 3.7% of our outstanding common stock as of October 31, 2011. 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 October 31, 2011, there were (i) outstanding warrants to purchase 6,934,535 shares of our common stock at a weighted average exercise price of \$2.96 per share issued in connection with the transactions described above and other equity issuances, (ii) outstanding options to purchase 5,546,014 shares of our common stock outstanding under our 2010 and 2006 Raptor stock option plans at a weighted-average exercise price of \$3.84, (iii) options to purchase 154,446 shares of our common stock outstanding under our TorreyPines Therapeutics stock option plans at a weighted-average exercise price of \$86.42 and (iv) 1,753,508 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 (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.

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

Our common stock is a penny stock. The SEC has adopted Rule 15c-9 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 15.0 million shares of preferred stock with designations, rights and preferences determined from time-to-time by our board of directors. Accordingly, our board of directors is empowered, without stockholder approval, to issue preferred stock with dividend, liquidation, conversion, voting or other rights superior to those of stockholders of our common stock.

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law as currently in effect may make a change in control of our Company more difficult, even if a change in control would be beneficial to the stockholders. Our board of directors has the authority to issue up to 15.0 million shares of preferred stock, none of which are issued or outstanding. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Our charter contains provisions that may enable our management to resist an unwelcome takeover attempt by a third party, including: a prohibition on actions by written consent of our stockholders; the fact that stockholder meetings must be called by our board of directors; and provisions requiring stockholders to provide advance notice of proposals. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, the board of directors approves the transaction. Our board of directors may use these provisions to prevent changes in the management and control of our Company. Also, under applicable Delaware law, our board of directors may adopt additional anti-takeover measures in the future.

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

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 and amended the lease to expand the amount of leased space on April 1, 2007. Base monthly payments are subject to an annual rent increase of between 3% to 5%, based on the Consumer Price Index or CPI and annual adjustments to base operating expenses. In October 2010, we executed a lease addendum to the Novato lease for an additional 3,100 square feet (\$5,309 per month) starting in April 2011. Effective April 1, 2010, our monthly base rent, including base operating expenses, was \$10,826, and effective April 11, 2011, our monthly base rent (including the additional 3,100 square feet of rental space), plus base operating expenses, is \$16,135 with an adjustment for CPI and operating expenses in April 2012. The Novato lease expires in March 2013. In January 2010, we entered into a one year lease for administrative offices in San Mateo, California for \$2,655 per month. We anticipate continuing the San Mateo lease on a monthly basis. We also anticipate leasing office space in the Netherlands for our future European sales and marketing headquarters by mid-calendar 2012. We believe that our existing California facilities are adequate to meet our needs in the US at least through the end of calendar 2012.

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." As of October 31, 2011, there were 47,153,503 shares of our common stock outstanding. There is no public trading market for our warrants. The closing price for our common stock on October 28, 2011 was \$4.96.

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, 2011:		
First Quarter (September 1 – November 30, 2010)	\$4.00	\$2.76
Second Quarter (December 1, 2010 – February 28, 2011)	4.04	3.23
Third Quarter (March 1 – May 31, 2011)	5.75	3.10
Fourth Quarter (June 1 – August 31, 2011)	6.99	3.66
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

* 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 October 31, 2011, there were approximately 242 holders of record of our common stock and 47,153,503 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 68 persons to acquire up to, in the aggregate, 5,700,460 shares, and warrants held by 31 persons to acquire up to, in the aggregate, 6,934,535 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. We did not issue any unregistered equity securities during the fiscal quarter ended August 31, 2011.

ITEM 6: SELECTED FINANCIAL DATA

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

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

Overview

You should read the following discussion in conjunction with our consolidated financial statements as of August 31, 2011, 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."

Plan of Operation and Overview

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

Our current pipeline includes three clinical development programs, which we are actively developing. We also have two other clinical-stage product candidates, one of which we are seeking additional business development partners in Asia but are not internally developing, and we have three preclinical product candidates for which we are seeking development partners.

Clinical Development Programs

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

- DR Cysteamine, or RP103, for the potential treatment of cystinosis, a rare genetic disorder; and
- RP103 for the potential treatment of Huntington's Disease, or HD, an inherited neurodegenerative disorder.

RP103 is our proprietary delayed-release formulation of cysteamine bitartrate microbeads in capsules, which may require less frequent dosing and reduce gastro-intestinal side effects compared to the current standard of care.

- RP104, for the potential treatment of non-alcoholic steatohepatitis, or NASH, a metabolic disorder of the liver.

RP104 is our proprietary delayed-release formulation of cysteamine bitartrate in tablets.

Other Clinical-Stage Product Candidates

Our other clinical-stage product candidates include:

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

Preclinical Product Candidates

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

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

Future Activities

Over the next 12 months, we plan to conduct research and development and general and administrative activities including: pre-commercial preparation for the potential launch of RP103 for the treatment of cystinosis in the United States and Europe; supporting our ongoing extension study of RP103 in cystinosis; supporting the ongoing clinical trial of RP103 in HD; funding a potential collaboration in a clinical trial of RP104 in NASH; funding a potential clinical trial of tezampanel as a potential anti-platelet agent; continued business development of our preclinical product candidates; and supporting associated facilities and administrative functions. We plan to seek additional business development partners for our Convivia™ product candidate in Asia. We may also develop future in-licensed technologies and acquired technologies.

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 RP103 for the Potential Treatment of Nephropathic Cystinosis or Cystinosis

Our RP103 product candidate is a proprietary delayed-release, enteric-coated microbead formulation of cysteamine bitartrate contained in a gelatin capsule. We are investigating RP103 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 RP103 for the potential treatment of cystinosis is designed to mitigate these difficulties. It is expected to be dosed twice daily, compared to the current every-six-hour dosing schedule. In addition, RP103 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 RP103 for the treatment of cystinosis in calendar 2010 and 2006, respectively.

In June 2009, we commenced our Phase 2b clinical trial of RP103 in cystinosis, in which we enrolled nine cystinosis patients with histories of non-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 RP103 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 July 25, 2011, we announced that our Phase 3 clinical trial of RP103 for the treatment of cystinosis, met the sole primary endpoint of non-inferiority compared to Cystagon®, immediate-release cysteamine bitartrate. The comparison was based on white blood cell, or WBC, cystine levels, the established efficacy surrogate biomarker and sole primary endpoint in the clinical trial. There were no unexpected serious safety concerns experienced by patients in the trial attributable to RP103.

Our pivotal Phase 3 clinical trial was designed as an outpatient study of the pharmacodynamics, pharmacokinetics, safety and tolerability of RP103 compared to Cystagon® in cystinosis patients. The clinical trial was conducted at eight clinical research centers in the US and Europe.

Of 41 patients who completed the Phase 3 protocol, 38 were included in the evaluable data set, 3 not being fully compliant with the protocol due to the fact that their WBC cystine levels went above 2.0 while on Cystagon® during the trial. The age range of study participants was 6-26 years, with 87% of patients below 16 years old. On average, the peak WBC cystine level measured in patients treated with Cystagon® was 0.54 ± 0.05 nmol ½ cystine/mg protein, compared to an average peak value of 0.62 ± 0.05 nmol ½ cystine/mg protein for patients treated with RP103. The mean difference was 0.08 nmol ½ cystine/mg protein, with a 95.8% confidence interval of 0.00-0.16 (one sided $p=0.021$). As stipulated in our Statistical Analysis Plan, the non-inferiority endpoint of the clinical trial would be achieved when the upper end of the confidence interval around the mean difference of WBC cystine levels did not exceed an absolute value of 0.3. The upper end of the confidence interval in the Phase 3 clinical trial was determined to be 0.16, thus achieving the non-inferiority endpoint.

Additionally, the endpoint was achieved at a lower average daily dose of RP103, compared to Cystagon®. Patients enrolled in the study were required to be “well controlled” under the existing Cystagon® therapy. The starting dose of RP103 for patients in the Phase 3 clinical trial was initially set at 70% of their established dose of Cystagon®. The protocol allowed for a single RP103 dose increase of 25%, based on intermediate WBC cystine level results, to reflect the current standard of care in establishing appropriate dosing of Cystagon® in cystinosis patients. Approximately one-third of patients remained at 70% of their starting Cystagon® dose throughout the study. The remaining two-thirds of the patients had their RP103 dose increased. On average, the total daily, steady-state dose of RP103 in patients in the Phase 3 clinical trial was 82% of their established, incoming dose of Cystagon®.

In the course of the study, no unexpected safety issues were experienced. Seven serious adverse events, or SAEs, requiring a visit to the emergency room or hospital, were reported for seven individual patients. Of these seven SAEs, six were determined by the principal investigator to be unrelated to either RP103 or Cystagon®. One SAE, gastric intolerance, was graded as “possibly related” to RP103 and was subsequently resolved and the patient returned on RP103 treatment. That patient completed the RP103 study and continued on the extension study described below. The most frequently reported non-serious adverse events, or AEs, in the study were gastric intolerance symptoms. Fifty-three AEs were scored as “possibly” or “probably” related to either study drug, and forty-three of fifty-three of the drug related AEs were scored as gastric intolerance symptoms.

We are conducting an ongoing, extension study in which all patients completing the Phase 3 clinical trial may elect to continue on RP103 treatment and are monitored for WBC cystine levels and safety parameters. The extension study will provide at least six months of safety data for each patient and will be part of our New Drug Application filing. Forty out of forty-one patients who completed the Phase 3 clinical trial elected to enroll in the extension study. Thirty-eight of such patients remain in the extension study. Thirty-two patients have been on RP103 in the extension study for at least 6 months. We plan to submit our Phase 3 clinical trial data for publication in the coming months.

In a related clinical trial, we performed a bioequivalence study between RP103 administered as whole capsules and RP103 administered as capsule contents sprinkled onto applesauce. As a significant number of cystinosis patients are too young to take whole capsules, this result may enable us to expand enrollment in the extension study to patients who are too young to swallow whole capsules and were therefore ineligible for the pivotal Phase 3 clinical trial protocol.

With respect to RP103 for the treatment of cystinosis, we expect to file an NDA with the FDA and a marketing authorization application, or an MAA, with the EMA, in the first calendar quarter of 2012. In October and November 2011, we had a pre-NDA meeting with the FDA and a pre-MAA meeting with the EMA, respectively, resulting in no change to our expected NDA or MAA filing timelines. If RP103 is approved by the FDA or EMA for the treatment of cystinosis, we plan to commercialize RP103 in the US or Europe by ourselves. However, we may enter into marketing partnerships for certain markets outside of the US and Europe.

Development of RP104 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 RP104 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 US 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 RP104) 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. RP104 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 produce RP104 for our next potential clinical trial in NASH and are preparing an IND submission in late calendar 2011 in anticipation of such clinical trial. We are in late stages of discussions to collaborate with a national health organization for the clinical development of RP104 in juveniles with NASH. Assuming we receive the anticipated level of grant support from this organization, we anticipate initiating our Phase 2b clinical trial for RP104 in the first half of calendar 2012 and releasing the top-line Phase 2b clinical trial data in the second half of calendar 2013.

Development of RP103 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 US 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 RP103 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 RP103 and all non-placebo patients continuing on RP103 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 US 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.

We anticipate reaching full enrollment for our Phase 2 clinical trial for RP103 in patients with HD in the fourth quarter of calendar 2011 and we anticipate releasing the top-line Phase 2 clinical trial data in the middle of calendar 2013.

In June 2010, we acquired an exclusive worldwide license to intellectual property related to the potential treatment of HD 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 HD and other neurological disorders. These patents add to our portfolio of intellectual property related to our programs utilizing RP103/RP104.

Other Clinical-Stage Product Candidates

We have two other clinical-stage product candidates.

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 US and E.U. in an intravenous form as an anti-toxin. Convivia™ is designed to lower systemic levels of acetaldehyde (a carcinogen) and reduce symptoms, such as tachycardia and flushing, associated with alcohol consumption by ALDH2-deficient individuals.

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

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

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

Tezampanel for Anti-Platelet Therapy

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

In addition to such well-known platelet signaling molecules as thromboxane A2 (blocked by aspirin) and adenosine diphosphate (blocked by Plavix®), research conducted at JHU, by Dr. Craig Morrell and Dr. Charles Lowenstein demonstrated the importance of glutamate release in promoting platelet activation and thrombosis. This research showed that platelets treated with an AMPA/kainate receptor antagonist such as tezampanel are more resistant to glutamate-induced aggregation than untreated platelets. Glutamate release by a platelet acts to stimulate release of glutamate from other platelets, potentiating aggregation and the formation of the thrombus. Released glutamate acts by binding cell surface glutamate receptors expressed on platelets themselves. One particular type of the glutamate receptor is important in platelet activation, the AMPA receptor. Compounds that specifically activate the AMPA receptor can increase platelet activation. Conversely, compounds that inhibit the AMPA receptor decrease platelet activation.

This identifies the AMPA/kainate receptors on platelets targeted by tezampanel as a new antithrombotic target with a different mechanism of action than Plavix®, aspirin or tPA. Tezampanel is a molecule developed by Eli Lilly and licensed to us. Tezampanel has been shown to inhibit human platelet activation, subsequent human platelet aggregation, and thrombosis in mice. The inventors of this novel technology are Dr. Lowenstein and Dr. Morrell, currently at the University of Rochester in New York. The use of glutamate receptor antagonists as anti-platelet agents is covered by PCT/US08/00559, assigned to JHU and exclusively licensed to us.

Tezampanel has been extensively tested in Phase 1 clinical trials for safety in various indications. The drug candidate has been demonstrated to be safe over a wide range of doses, without any serious adverse events and without any major abnormal laboratory tests. Human pharmacokinetics of tezampanel are well characterized.

In collaboration with Dr. Lowenstein and Dr. Morrell, we are preparing to conduct a Phase 1 clinical trial in healthy volunteers to determine the efficacy of tezampanel in blocking platelet activation and aggregation. We anticipate this early-stage trial to commence by the end of 2011.

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™. Following some initial studies, Roche decided to terminate its agreement with us effective October 2011. We are currently reviewing potential out-licensing opportunities for NeuroTrans™ and continue to maintain the program's intellectual property portfolio.

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.

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 Convivia™ in Taiwan. In the aggregate, we have issued to Mr. Daley, 58,281 shares of our common stock valued at \$118,551 and paid \$70,000 in cash bonuses related to Convivia™ milestones along with another \$20,000 in cash bonuses related to employment milestones pursuant to Mr. Daley's employment agreement.

Purchase of RP103/RP104

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 RP103/RP104. 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 RP103/RP104, 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 RP103/RP104, 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, 2011, 2010 and 2009 by spending approximately \$11.3 million, \$6.2 million and \$4.1 million, respectively, on such programs) pursuant to the License Agreement. To date, we have accrued \$520,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, HD and in NASH. To the extent that we fail to perform any of our obligations under the License Agreement, UCSD may terminate the license or otherwise cause the license to become non-exclusive.

Application of Critical Accounting Policies

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

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

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Use of Estimates

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

Functional Currency

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

Fair Value of Financial Instruments

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

Cash and Cash Equivalents

We consider all highly liquid investments with original maturities of three months or less, when purchased, to be cash equivalents. We maintain cash and cash equivalents, which consist principally of money market funds with high credit quality financial institutions. Such amounts exceed Federal Deposit Insurance Corporation insurance limits. We have not experienced any losses on these investments. Restricted cash represents compensating balances required by our US and European banks as collateral for credit cards.

Deferred Offering Costs

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

Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine (currently developed as RP103 and RP104), to the RAP technology (written off as of August 31, 2011, but reflected in the August 31, 2010 balance sheet), to an out-license acquired in the 2009 Merger and the rights to tezampanel. The intangible assets related to RP103/RP104 and the RAP technology (prior to being written off) are amortized using the straight-line method over the estimated useful life of 20 years, which is the life of the intellectual property patents. The 20 year

estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. The intangible assets related to the out-license will be amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents. The intangible assets related to tezampanel, which has been classified as in-process research and development, will not be amortized until development is completed, but will be tested annually for impairment.

Goodwill

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

Fixed Assets

Fixed assets, which mainly consist of leasehold improvements, lab equipment, computer hardware and software and

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

Impairment of Long-Lived Assets

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

Common Stock Warrant Liabilities

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

Income Taxes

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

Research and Development

We are a development stage biotechnology company. Research and development costs are charged to expense as incurred. Research and development expenses include medical, clinical, regulatory and scientists' salaries and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated executive, human resources and facilities expenses. Research and development expenses are offset by contra-expenses which are reimbursements of research and development expenses received either from research collaborators or from government grants or tax rebates.

In-Process Research and Development

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

Comprehensive Loss

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

Stock-Based Compensation

In February 2010, our board of directors approved, and in March 2010 our stockholders approved, our 2010 Equity Incentive Plan, or the 2010 Plan, to grant up to an aggregate of 3,000,000 stock options or restricted stock or restricted stock units over the ten year life of the 2010 Plan. Our board of directors has determined not to make any new grants under any of our former plans, but rather under the 2010 Plan. The 2010 Plan's term is ten years and allows for the granting of options to employees, directors and consultants. On April 7, 2011, our stockholders passed amendments to the 2010 Plan which allow for an increase of the grant pool based upon 5% of our common stock outstanding as of April 7, 2011, August 31, 2011 and August 31, 2012 up to an aggregate maximum increase of 6,000,000 shares. The April 7, 2011 and August 31, 2011 increases added 1,629,516 and 1,778,459 shares, respectively, available for grant under the 2010 Plan. The amendments also allow for 50% accelerated vesting of unvested stock options upon a change of control as defined in the 2010 Plan, as amended. In September 2011, our board of directors approved an amended and restated form of award agreement to the 2010 Plan, which will be used for awards granted on or after September 22, 2011. The amended and restated award agreement, subject to the terms of any applicable employment agreement, extends the termination date of the awards granted under the 2010 Plan that are vested as of such termination date due to (a) an employee's or a non-employee director's retirement at age 62 or older which employee or non-employee director has at least five (5) years of continuous service with us prior to such retirement, (b) the termination of a non-employee director's board membership for reasons other than for cause or retirement and (c) an employee's or a non-employee director's death (during his or her continuous service with us or within 90 days' of such continuous service with us) or permanent disability, to eighteen (18) months from the date of termination of continuous service with us.

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

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

In March 2005, the FASB issued ASC Topic 718 (previously listed as Staff Accounting Bulletin No. 107), which offers guidance for what was previously referred to as SFAS 123(R). ASC Topic 718 was issued to assist preparers by simplifying some of the implementation challenges of SFAS 123(R) while enhancing the information that investors receive. ASC Topic 718 creates a framework that is premised on two overarching themes: (a) considerable judgment will be required by preparers to successfully implement SFAS 123(R), specifically when valuing employee stock options; and (b) reasonable individuals, acting in good faith, may conclude differently on the fair value of employee

stock options. Key topics covered by ASC Topic 718 include valuation models, expected volatility and expected term.

For the year ended August 31, 2011, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 1.6 to 2.4%; 6 year expected life; 88 to 116% volatility; 2.5% turnover rate; and 0% dividend rate.

We based our Black-Scholes inputs on the following factors: the risk-free interest rate was based upon our review of current constant maturity treasury bill rates for seven and five years (average); the expected life of six years was based upon our assessment of the ten-year term of the stock options issued along with the fact that we are a development-stage company and our anticipation that option holders will exercise stock options when we are at a more mature stage of development; the volatility was based on the actual volatility of our common stock price as quoted on NASDAQ since the closing of our 2009 Merger on September 30, 2009; the turnover rate was based on our assessment of our historical employee turnover; and the dividend rate was based on our current decision to not pay dividends on our stock at our current development stage. If factors change and different assumptions are employed in the application of ASC Topic 718, the compensation expense recorded in future periods may differ significantly from what was recorded in the current period. See Note 7 of our consolidated financial statements for a further discussion of our accounting for stock based compensation.

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

Results of Operations

Years ended August 31, 2011 and 2010

General and Administrative Expenses

General and administrative expenses include finance and executive compensation and benefits, pre-commercial expenses, such as reimbursement and marketing studies, corporate costs, such as legal and auditing fees, business development expenses, travel, board of director fees and expenses, investor relations expenses, intellectual property costs associated with filed (but not issued) patents, administrative consulting and allocated human resources and facilities costs. General and administrative expenses for the year ended August 31, 2011 increased by approximately \$2,457,000 compared to the prior fiscal year. The increase was primarily due to:

Reason for Increase (Decrease)	Increase (Decrease) in \$ Thousands
Stock option grants in FYE 2011 some with 25% up front vesting, non-cash expense	1,378
Additional commercial operations and business development consulting in FY 2011	654
Salary increases, new hires and increase in bonuses in FY 2011 including payroll tax effect	309
401(K) match for all employees in FY 2011	149
Increase in travel due to addition of new employees and new consultants in FY 2011	147
Increase in Delaware franchise taxes due to increase in Company value	111
Reduction in legal expenses related to clinical site agreements not repeated in FY 2011	(99)
Reduction in costs related to investor relations which were higher in FY 2010 due to the 2009 reverse merger	(137)
Increase in human resources costs allocated to R&D due to increase in 401(K) matching during FY 2011 and benefits attributable to new hires	(231)
Various other	176
General and Administrative increase year ended August 31, 2011 vs. August 31, 2010	2,457

Research and Development

Research and development expenses include medical, clinical, regulatory and scientists' compensation and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated executive, human resources and facilities expenses. Research and development expenses for the year ended August 31, 2011 increased by approximately \$5,454,000 over the prior fiscal year primarily due to:

Reason for Increase (Decrease)	Increase (Decrease) in \$ Thousands
Increase in clinical costs including materials, CRO fees and site fees related to RP103	5,832
Salary increases, new hires and increase in bonuses in FY 2011 including payroll tax effect	381
Stock option grants in FY 2011 some with 25% up front vesting, non-cash expense	361
Clinical materials related to thrombosis study	250
Increase in human resources costs allocated to R&D due to increase in 401(K) matching during FY 2011 and benefits attributable to new hires	231
Increase in regents due to additional assay testing in FY 2011	152
Write off of NeuroTrans™ net asset in FY 2011, non-cash	108
Milestone payments made in FY 2010 not repeated in FY 2011	(170)
Reduction in clinical consulting fees due to hiring in-house expertise in 2nd half of FY 2010	(658)
Tax grant and other program expense reimbursements received in FY 2011	(990)
Various other	(43)
Research and Development increase year ended August 31, 2011 vs. August 31, 2010	5,454

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

Major Program (stage of development)	Estimated next 12 months	Cumulative through August 31, 2011	Year ended August 31,	
			2011	2010
RP103/RP104 – All Indications (clinical/pre-commercial)	15.6	21.7	10.5	6.2

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Convivia™ (clinical)	(0.1)	2.5	0.1	0.1
HepTide™ (preclinical)	0.1	1.6	-	-
NeuroTrans™ (preclinical)	-	0.5	0.1	0.1
WntTide™ (preclinical)	0.2	0.4	-	0.1
Minor or Inactive Programs	0.1	1.1	0.3	0.1
R & D Personnel and Other Costs Not Allocated to Programs	5.2		3.8	2.7
		11.4		
Total Research & Development Expenses	21.1		14.8	9.3
		39.2		

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

Major Program (stage of development)	Estimated next 12 months	Cumulative through August 31, 2011	Year ended August 31,	
			2011	2010
RP103/RP104 – All Indications (clinical and pre-commercial)	5.91	1.12	0.78	0.14
Convivia™ (clinical)	0.05	0.27	0.10	0.08
HepTide™ (preclinical)	0.05	0.36	0.04	0.15
NeuroTrans™ (preclinical)	0.05	0.24	0.04	0.05
WntTide™ (preclinical)	0.06	0.17	0.04	0.07

Additional major program expenses include patent fees and patent expenses which were recorded as general and administrative expenses as these fees are to support patent applications (not issued patents) and expenses related to the preparation for commercial launch of RP103 for the treatment of cystinosis (approximately \$470,000, \$470,000 and \$5.8 million for the year ended August 31, 2011, the cumulative period from September 8, 2006 (inception) to August 31, 2011 and estimated for the next 12 months, respectively).

Any of our major programs could be partnered for further development and/or could be accelerated, slowed or ceased due to scientific results or challenges in obtaining funding. We anticipate that we will need additional funding in order to pursue our plans beyond the next 12 months. In addition, the timing and costs of development of our programs beyond the next 12 months is highly uncertain and difficult to estimate. See Part I, Item 1A of this Annual Report on Form 10-K titled “Risk Factors” for further discussion about the risks and uncertainties pertaining to drug development.

Current Status of Major Programs

Please refer to the prior provisions of this Item 7 of this Annual Report on Form 10-K for a detailed discussion of each of our major programs. In summary, RP103/RP104 is being developed in cystinosis, NASH and HD. In July 2011, we announced that our Phase 3 clinical trial of RP103 for the treatment of cystinosis met its sole primary clinical endpoint and in November 2009, we released data from our Phase 2b clinical trial. We plan to file for marketing approval of RP103 for cystinosis in the first calendar quarter of 2012. In May 2010, we presented the data from our NASH Phase 2a clinical trial and are reformulating the drug product candidate RP104 for a potential Phase 2b trial in early calendar 2012. In October 2010, our collaborator commenced a Phase 2a clinical trial of RP103 in HD patients and we anticipate full enrollment by the end of calendar 2011 with potential data by mid-calendar 2013.

Our Convivia™ product candidate completed its initial clinical study in 2008 and in June 2010, we licensed Convivia™ to Uni Pharma for further clinical development in Taiwan, with an option to develop Convivia™ in South Korea. We continue to seek other potential partners for Convivia™ in other Asian countries where its potential market exists. We are seeking to out-license our tezampanel product candidate and are preparing for a Phase 1 clinical trial for the potential treatment of thrombotic disorder. HepTide™ will be undergoing further preclinical proof of concept studies and WntTide™ and NeuroTrans™ are being considered for potential out-licensing for further development. All preclinical product candidates will require further study prior to potentially moving into a clinical phase of development.

Interest Income

Interest income for the years ended August 31, 2011 and 2010 was nominal.

Interest Expense

Interest expense for the years ended August 31, 2011 and 2010 was nominal.

Foreign Currency Transaction Loss

Foreign currency transaction gain (loss) for the years ended August 31, 2011 and 2010 was nominal.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of approximately \$(16.3) million for the year ended August 31, 2011 compared to a loss of approximately \$(5.9) million for the year ended August 31, 2010, an increase in loss of approximately \$10.4 million resulting from an increase in our common stock price of \$1.75 per share. These losses are non-cash.

Years ended August 31, 2010 and 2009

General and Administrative Expenses

General and administrative expenses include finance and executive compensation and benefits, corporate costs, such as legal and auditing fees, business development expenses, travel, board of director fees and expenses, investor relations expenses, intellectual property costs associated with filed (but not issued) patents, administrative consulting and allocated human resources and facilities costs. General and administrative expenses for the year ended August 31, 2010 increased by approximately \$1,032,000 compared to the prior fiscal year. The increase was primarily due to:

Reason for Increase (Decrease)	Increase (Decrease) in \$ Thousands
Legal expenses for clinical trial agreements, licenses and establishment of a European subsidiary	274
Additional investor relations costs relating to press releases and annual meeting costs in fiscal 2010 due to the 2009 Merger	258
Transfer agent and NASDAQ fees related to 2009 Merger and two post-2009 Merger financings in fiscal 2010 that did not occur in fiscal 2009	197
Cash bonuses paid or accrued in fiscal 2010 that did not occur in fiscal 2009. Fiscal 2009 bonuses were paid in March 2010.	196
Additional accounting and professional fees related to the 2009 Merger	165
Salary increases in fiscal 2010 retroactive to September 1, 2009	137
Increase in clinical patents application costs	122
Increase in administrative consulting related to business development and in support of the European subsidiary	115
Decrease in stock option expense due to options that were fully vested in fiscal 2009	(153)
Decrease in legal expenses incurred in fiscal 2009 for the 2009 Merger that did not occur in fiscal 2010	(215)
Increase in G&A costs allocated to R&D. G&A costs are allocated based upon headcount, which increased in R&D in fiscal 2010.	(348)
Various other	284
General and Administrative increase year ended August 31, 2010 vs. August 31, 2009	1,032

Research and Development

Research and development expenses include medical, clinical, regulatory and scientists' compensation and benefits, lab collaborations, preclinical studies, clinical trials, clinical trial materials, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated executive, human resources and facilities expenses. Research and development expenses for the year ended August 31, 2010 increased by approximately \$2,764,000 over the prior fiscal year primarily due to:

Reason for Increase (Decrease)	Increase (Decrease) in \$ Thousands
Clinical costs of preparing for and commencement of Phase 3 cystinosis trial	1,582
Manufacture of RP103 for cystinosis and HD clinical trials	1,188
Increase in executive costs attributable to salary increases and bonuses paid/accrued to executives which were allocated to R&D	348
Hiring of CMO in April 2009, salary increases retroactive to Sept. 1, 2009, addition of director of clinical operations in March 2010	312
Collaboration reimbursement received in fiscal 2009 not repeated in fiscal 2010	300
Cash bonuses paid/accrued in fiscal 2010 that did not occur in fiscal 2009. Fiscal 2009 bonuses were paid in March 2010.	113
Reduction of reagent purchases by preclinical development	(257)
Reduction of HepTide and WntTide preclinical studies	(306)
Reduction of R&D consultants replaced by CMO, Director of Program Mgmt. and Director of Clinical Operations	(602)
Various other	86
Research and Development increase year ended August 31, 2010 vs. August 31, 2009	2,764

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

Major Program (stage of development)	Cumulative through August 31, 2010	Year ended August 31,	
		2010	2009
RP103/RP104 – All		6.2	
Indications (clinical)	11.2		4.0
Convivia TM (clinical)	2.2	0.1	0.4
HepTide TM (preclinical)	1.6	-	0.4
NeuroTrans TM (preclinical)	0.4	0.1	(0.3)

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WntTide™ (preclinical)	0.4	0.1	0.1
Minor or Inactive Programs	0.8	0.1	0.1
R & D Personnel and Other Costs Not Allocated to Programs	7.6	2.7	1.9
Total Research & Development Expenses	24.2	9.3	6.6

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

Major Program (stage of development)	Cumulative through August 31, 2010	Year ended August 31,	
		2010	2009
RP103/RP104 – All		0.14	0.12
Indications (clinical)	0.34		
Convivia TM (clinical)	0.17	0.08	0.05
HepTide TM (preclinical)	0.32	0.15	0.07
NeuroTrans TM (preclinical)	0.20	0.05	0.05
WntTide TM (preclinical)	0.13	0.07	0.01

Additional major program expenses include patent fees and patent expenses which were recorded as general and administrative expenses as these fees are to support patent applications (not issued patents).

Interest Income

Interest income decreased by \$11,043 for the year ended August 31, 2010 compared to the prior fiscal year due to the reduction of interest rates.

Interest Expense

Interest expense for the years ended August 31, 2010 and 2009 was nominal.

Foreign Currency Transaction Loss

Foreign currency transaction loss increased by \$457 for the year ended August 31, 2010 compared to the prior fiscal year due to the addition of a Euro-denominated bank account and subsidiary in fiscal 2010 resulting from the creation of a European subsidiary to manage our European clinical trials.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants increased by \$(5.9) million resulting in an increase to our net loss for the year ended August 31, 2010 compared to the prior fiscal year due to the fact that there was no warrant liability recorded in the prior fiscal year.

Liquidity and Capital Resources

Capital Resource Requirements

As of August 31, 2011, we had approximately \$15.3 million in cash, cash equivalents and restricted cash, approximately \$26.7 million in current liabilities (of which \$23.6 million represented the non-cash common stock warrant liability) and approximately (\$11.0) million of net working capital deficit. Our forecasted average monthly cash expenditures for the next twelve months are approximately \$2.7 million.

We believe our cash and cash equivalents as of September 30, 2011 of approximately \$56.1 million (which includes approximately \$42.9 million of net proceeds in connection with our underwritten public offering of shares of our common stock that was announced on September 13, 2011) will be sufficient to meet our obligations at least through the first calendar quarter of 2013.

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

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

In December 2009, we entered into a definitive securities purchase agreement, or the Direct Offering Purchase Agreement, dated as of December 17, 2009, with 33 investors (collectively, the Direct Offering Investors) with respect to the sale of units, whereby, on an aggregate basis, the investors agreed to purchase 3,747,558 Units for a negotiated purchase price of \$2.00 per unit for aggregate gross proceeds of approximately \$7.5 million. Each unit consists of one share of our common stock, one Series A Warrant exercisable for 0.5 of a share of our common stock and one Series B Warrant exercisable for 0.5 of a share of our common stock. The shares of our common stock and the warrants were issued separately. The Series A Warrants exercisable for an aggregate 1,873,779 shares of our common stock were exercisable commencing on June 20, 2010 and ending December 22, 2014. The Series B Warrants exercisable for an aggregate 1,873,779 shares of our common stock were exercisable commencing on June 20, 2010 and ending June 22, 2011. The investor warrants have a per share exercise price of \$2.45. In connection with this offering we paid a placement agent cash compensation equal to 6.5% of the gross proceeds or \$487,183 plus a five-year warrant at an exercise price of \$2.50 per share for the purchase of up to 74,951 shares of our common stock. As of September 30, 2011, 317,529 shares of our common stock have been issued upon exercise of the Series A Warrants, 1,873,779 shares of our common stock have been issued upon exercise of the Series B Warrants and 74,951 shares of our common stock have been issued upon exercise of the placement agent warrants. As of September 30, 2011, Series A warrants to purchase up to 1,556,250 shares of our common stock were outstanding.

On August 9, 2010, we entered into the 2010 Private Placement Purchase Agreements with the 2010 Private Placement Investors for the private placement of our common stock and warrants to purchase our common stock, at a purchase price of \$3.075 per unit, with each unit comprised of one share of common stock and a warrant to purchase

one share of common stock. We issued and sold an aggregate of 4,897,614 units, comprised of an aggregate of 4,897,614 shares of common stock and warrants to purchase up to 4,897,614 shares of our common stock for gross proceeds of approximately \$15.1 million. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of \$3.075 per share. The placement agent for the 2010 Private Placement was issued one warrant to purchase 97,952 shares of our common stock at an exercise price of \$3.075 per share, paid a cash commission of \$978,911 and reimbursed for certain of its expenses incurred in connection with the 2010 Private Placement. As of September 30, 2011, 357,724 shares of our common stock have been issued upon exercise of the warrants. As of September 30, 2011, warrants to purchase up to 4,637,842 shares (including the placement agent warrants) of our common stock were outstanding.

On September 13, 2011, we announced the closing of an underwritten public offering of shares of our common stock at a price to the public of \$4.00 per share. The shares sold in the offering included 10.0 million shares of our common stock plus an additional 1.5 million shares of common stock pursuant to the exercise by the underwriters of the over-allotment option we granted to them. Total gross proceeds to us in the offering (including in connection with the sale of the shares of common stock pursuant to the exercise of the over-allotment option) were \$46.0 million, before underwriting discounts and commissions. The offering resulted in net proceeds to us of approximately \$42.9 million after deduction of underwriting discounts and other offering expenses payable by us. We expect to use the net proceeds from the offering to fund our commercial and pre-commercial efforts, clinical and preclinical development programs and other general corporate activities.

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

For the next 12 months we intend to expend a total of approximately \$31.9 million to implement our operating plan of preparing for commercial launch of RP103 for the treatment of cystinosis, continued research and development of our RP103 and RP104 clinical programs, our RAP based platform, our licensed technologies, as well as continuing business development efforts for our other clinical-stage product candidates. Specifically, we estimate our operating expenses and working capital requirements for the next 12 months to be as follows:

Estimated spending for the next 12 months:	In millions
Research and development activities	\$ 17.6
Research and development compensation and benefits	3.4
General and administrative and pre-commercial activities	7.3
General and administrative compensation and benefits	3.6
Capital expenditures	-
Total estimated spending for the next 12 months	\$ 31.9

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

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

Research and Development Activities

We plan to conduct further research and development, seek to support several clinical trials for RP103 and RP104, improve upon our RAP-based and in-licensed technology and continue business development efforts for our preclinical and other clinical-stage product candidates in the next 12 months. We plan to conduct research and development activities by our own laboratory staff and also by engaging contract research organizations, clinical research organizations and contract manufacturing organizations. We also plan to incur costs for the production of our clinical study drug candidates, RP103 and RP104, clinical trials, clinical and medical advisors and consulting and collaboration fees. We anticipate our research and development costs for the next 12 months, excluding in-house research and development compensation, will be approximately \$17.6 million.

Officer and Employee Compensation

We have seven executive officers, one permanent scientific staff member, three permanent clinical development staff members and one permanent finance staff member. We plan on adding several personnel in the areas of sales and marketing, regulatory, medical affairs, quality and finance. We anticipate spending up to approximately \$7.0 million in officer and employee compensation during the next 12 months, of which \$3.4 million is allocated to research and development expenses and \$3.6 million is allocated to general and administrative expenses.

General and Administrative

We anticipate spending approximately \$7.3 million on general and administrative costs in the next 12 months. These costs will consist primarily of pre-commercial activities in anticipation of approval and launch of RP103 for cystinosis, legal and accounting fees, patent legal fees, investor relations expenses, board fees and expenses, insurance, rent and facility support expenses, excluding finance and administrative compensation.

Capital Expenditures

We anticipate spending approximately \$20,000 in the next 12 months on capital expenditures for lab equipment and office furniture.

Contractual Obligations with BioMarin

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

- \$50,000 (paid by us in June 2006) within 30 days after we receive total aggregate debt or equity financing of at least \$2,500,000;
- \$100,000 (paid by us in June 2006) within 30 days after we receive total aggregate debt or equity financing of at least \$5,000,000;
- \$500,000 upon our filing and acceptance of an investigational new drug application for a drug product candidate based on our NeuroTrans™ product candidate;
- \$2,500,000 upon our successful completion of a Phase 2 human clinical trial for a drug product candidate based on our NeuroTrans™ product candidate;
- \$5,000,000 upon our successful completion of a Phase 3 human clinical trial for a drug product candidate based on our NeuroTrans™ product candidate;
- \$12,000,000 within 90 days of our obtaining marketing approval from the FDA or other similar regulatory agencies for a drug product candidate based on our NeuroTrans™ product candidate;
- \$5,000,000 within 90 days of our obtaining marketing approval from the FDA or other similar regulatory agencies for a second drug product candidate based on our NeuroTrans™ product candidate;
- \$5,000,000 within 60 days after the end of the first calendar year in which our aggregated revenues derived from drug product candidates based on our NeuroTrans™ product candidate exceed \$100,000,000; and
- \$20,000,000 within 60 days after the end of the first calendar year in which our aggregated revenues derived from drug product candidates based on our NeuroTrans™ product candidate exceed \$500,000,000.

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

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

Pursuant to the terms of the asset purchase agreement, or the Asset Purchase Agreement, that we entered into with Convivia, Inc. and Thomas E. Daley, pursuant to which we purchased intellectual property related to our 4-MP product candidate program, Mr. Daley will be entitled to receive the following, if at all, in such amounts and only to the extent certain future milestones are accomplished by us, as set forth below:

- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days after we enter into a manufacturing license or other agreement to produce any product that is predominantly based upon or derived from any assets purchased from Convivia, or Purchased Assets, in quantity, referred to as Product, if such license agreement is executed within one (1) year of execution of the Asset Purchase Agreement or, if thereafter, 11,656 shares of our restricted, unregistered common stock. Should we obtain a second such license or agreement for a Product, Mr. Daley will be entitled to receive 11,656 shares of our restricted, unregistered common stock within 30 days of execution of such second license or other agreement. In January 2008, Mr. Daley earned a \$30,000 cash bonus pursuant to his employment agreement for executing the Patheon formulation agreement for manufacturing ConviviaTM. On March 31, 2008, Raptor Pharmaceuticals Corp. issued 23,312 shares of our common stock valued at \$56,000 to Mr. Daley pursuant to this milestone reflecting the execution of an agreement to supply the active pharmaceutical ingredient for ConviviaTM, combined with the execution of a formulation agreement to produce the oral formulation of ConviviaTM. In July 2010, 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.

- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days after we receive our first patent allowance on any patents which constitute part of the Purchased Assets in any one of certain predetermined countries, or a Major Market.

- 11,656 shares of our restricted, unregistered common stock within fifteen (15) days after we receive our second patent allowance on any patents which constitute part of the Purchased Assets different from the patent referenced in the immediately preceding bullet point above in a Major Market.

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

- 11,656 shares of our restricted, unregistered common stock within fifteen (15) days of a Successful Completion in a Major Market by us or our licensee of the second phase 2 human clinical trial for a Product (other than the Product for which a distribution is made under the immediately preceding bullet point above).

- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days after we or our licensee applies for approval to market and sell a Product in a Major Market for the indications for which

approval is sought, or Marketing Approval.

- 11,656 shares of our restricted, unregistered common stock within fifteen (15) days after we or our licensee applies for Marketing Approval in a Major Market (other than the Major Market for which a distribution is made under the immediately preceding bullet point above).

- 46,625 shares of our restricted, unregistered common stock within fifteen (15) days after we or our licensee obtains the first Marketing Approval for a Product from the applicable regulatory agency in a Major Market.

- 23,312 shares of our restricted, unregistered common stock within fifteen (15) days after we or our licensee obtains Marketing Approval for a Product from the applicable regulatory agency in a Major Market (other than the Major Market for which a distribution is made under the immediately preceding bullet point above).

As discussed above, in aggregate, we issued to Mr. Daley, 58,281 shares of our common stock valued at \$118,551 and paid \$70,000 in cash bonuses related to Convivia™ milestones along with another \$20,000 in cash bonuses related to employment milestones pursuant to Mr. Daley's employment agreement.

Contractual Obligations with Former Encode Securityholders

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

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

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

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

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

Contractual Obligations with UCSD

As a result of the merger of our clinical subsidiary and Encode, we received the exclusive worldwide license to RP103/RP104, or the License Agreement for use in the field of human therapeutics for metabolic and neurologic disorders, developed by clinical scientists at the UCSD, School of Medicine. RP103/RP104 is a proprietary, delayed-release, enteric-coated formulation of cysteamine bitartrate, a cystine depleting agent currently approved by

the FDA. Cysteamine bitartrate is prescribed for the management of the genetic disorder known as cystinosis, a lysosomal storage disease. The active ingredient in RP103/RP104 has also demonstrated potential in studies as a treatment for other metabolic and neurodegenerative diseases, such as HD and NASH.

In consideration of the grant of the license, prior to the merger, Encode paid an initial license fee and we will be obligated to pay an annual maintenance fee of \$15,000 until we begin commercial sales of any products developed pursuant to the License Agreement. In addition to the maintenance fee, we will be obligated to pay during the life of the License Agreement: milestone payments ranging from \$20,000 to \$750,000 for orphan indications and from \$80,000 to \$1,500,000 for non-orphan indications upon the occurrence of certain events, if ever; royalties on commercial net sales from products developed pursuant to the License Agreement ranging from 1.75% to 5.5%; a percentage of sublicense fees ranging from 25% to 50%; a percentage of sublicense royalties; and a minimum annual royalty commencing the year we begin commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, we are obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 years from the effective date of the License Agreement, depending on the indication. In addition, we are obligated to, among other things, annually spend at least \$200,000 for the development of products—which as of August 31, 2011, 2010 and 2009 we satisfied by spending approximately \$11.3 million, \$6.2 million and \$4.1 million,

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respectively, on such programs —pursuant to the License Agreement. To date, we have accrued \$520,000 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.

Off-Balance Sheet Arrangements

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

Reverse Acquisition

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

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

Going Concern

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

New Accounting Pronouncements

In December 2010, the FASB issued Accounting Standards Update, or ASU, 2010-28, Intangibles – Goodwill and Other (Topic 350): When to Perform Step 2 of the Goodwill Impairment Test for Reporting Units with Zero or Negative Carrying Amounts (“ASU 2010-28”). ASU 2010-28 modifies Step 1 of the goodwill impairment test for reporting units with zero or negative carrying amounts and requires us to perform Step 2 if it is more likely than not that a goodwill impairment may exist. ASU 2010-28 is effective for fiscal years and interim periods within those years, beginning after December 15, 2010. Early adoption is not permitted. We adopted these standards on September 1, 2011 and are currently assessing the impact that ASU 2010-28 will have on our consolidated financial statements.

In December 2010, the FASB issued ASU 2010-29, Business Combinations (Topic 805): Disclosure of Supplementary Pro Forma Information for Business Combinations (“ASU 2010-29”). ASU 2010-29 is an update that addresses diversity in practice about the interpretation of the pro forma revenue and earnings disclosure requirements

for business combinations if the entity presents comparative financial statements and expands the required disclosures to include a description of the nature and amount of material, nonrecurring pro forma adjustments directly attributable to the business combination included in the reported pro forma revenue and earnings. This standard is effective prospectively for business combinations for which the acquisition dates are on or after the beginning of the first annual reporting period beginning on or after December 15, 2010. Early adoption is permitted. We adopted these standards on September 1, 2011, which will impact our financial disclosure for any business combinations entered into on or after September 1, 2011.

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

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for which fair value is disclosed, entities will be required to disclose the level within the fair value hierarchy that applies to the fair value measurement disclosed. ASU 2011-04 is effective for interim and annual periods beginning after December 15, 2011. We will adopt these standards on March 1, 2012 and are currently assessing the impact they will have on our consolidated financial statements.

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

In September 2011, the FASB issued ASU 2011-08, Intangibles – Goodwill and Other (Topic 350): Testing Goodwill for Impairment or ASU 2011-08, which permits an entity to first assess qualitative factors to determine whether it is more likely than not that the fair value of a reporting unit is less than its carrying amount as a basis for determining whether it is necessary to perform the two-step goodwill impairment test. We do not expect the provisions of ASU 2011-08 to have a material impact to our consolidated financial statements.

ITEM 7A: QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Per Item 305(e) of Regulation S-K, information is not required.

ITEM 8: FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The information required to be filed in this item appears on pages F-1 to F-41 of this Annual Report on Form 10-K.

Documents filed as part of this Annual Report on Form 10-K:

Financial Statements

	Page
Reports of Independent Registered Public Accounting Firm	F-1
Consolidated Balance Sheets as of August 31, 2011 and 2010	F-3
Consolidated Statements of Comprehensive Loss for the years ended August 31, 2011 and 2010 and for the cumulative period from September 8, 2005 (inception) to August 31, 2011	F-4
Consolidated Statements of Stockholders' Equity (Deficit) for period from September 8, 2005 (inception) to August 31, 2006 and the years ended August 31, 2007, 2008, 2009, 2010 and 2011	F-5
Consolidated Statements of Cash Flows for the years ended August 31, 2011 and 2010 for the cumulative period from September 8, 2005 (inception) to August 31, 2011	F-11
Notes to Consolidated Financial Statements	F-12

PART II – FINANCIAL INFORMATION

ITEM 9: CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A: CONTROLS AND PROCEDURES

As of August 31, 2011, we performed an evaluation of the effectiveness of our disclosure controls and procedures that are designed to ensure that the information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act, is accumulated and communicated to our management, including our principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Based on our evaluation, our management, including our Chief Executive Officer and Chief Financial Officer, has concluded that our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) of the Securities Exchange Act of 1934, as amended) as of August 31, 2011, are effective at a reasonable assurance level.

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

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

Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on our evaluation under the framework in Internal Control—Integrated Framework, our management concluded that our internal control over financial reporting was effective as of August 31, 2011.

Burr Pilger Mayer, Inc., our independent registered public accounting firm, has issued an attestation report on our internal control over financial reporting as of August 31, 2011, as stated in their report, which is included under Part IV below.

Changes in Internal Control Over Financial Reporting

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

ITEM 9B. OTHER INFORMATION

None.

PART III

ITEM 10: DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Directors

The following table sets forth the name, age and position of each of our directors as of October 31, 2011.

Name	Age	Position(s) Held with the Company
Raymond W. Anderson (2)(3)	69	Director
Suzanne L. Bruhn, Ph.D.(1)(3)	48	Director
Richard L. Franklin, M.D., Ph.D. (1)(2)	66	Director
Llew Keltner, M.D., Ph.D. (1)(2)	61	Director
Erich Sager	53	Director
Vijay B. Samant(1)(3)	58	Director
Christopher M. Starr, Ph.D.	59	Chief Executive Officer and Director
Timothy P. Walbert(2)(3)	44	Director

(1) Member of the Corporate Governance and Nominating Committee.

(2) Member of the Audit Committee.

(3) Member of the Compensation Committee.

Each of the current members of our board of directors has been elected to serve until our next annual meeting of stockholders or until their respective successors are duly elected and qualified.

Business Experience and Directorships

The following describes the background of our directors.

Raymond W. Anderson. Mr. Anderson has served as a director of Raptor Pharmaceutical Corp. since September 2009 and as a director of Raptor Pharmaceuticals Corp. since May 2006. Mr. Anderson worked at Dow Pharmaceutical Sciences, Inc. (a wholly-owned subsidiary of Valeant Pharmaceuticals International since December 31, 2008) from July 2003 until he retired in June 2010. He had been its Managing Director since January 2009 and was previously its Chief Financial Officer and Vice President, Finance and Administration. Mr. Anderson has more than 30 years of biopharmaceutical/medical technology sector experience, primarily focused in financial management. Prior to joining Dow in 2003, Mr. Anderson was Chief Financial Officer for Transurgical, Inc., a private medical technology company. Prior to that, Mr. Anderson served as Chief Operating Officer and Chief Financial Officer at BioMarin from June 1998 to January 2002. Prior to June 1998, Mr. Anderson held similar executive-level positions with other biopharmaceutical companies, including Syntex, Chiron, Glycomed and Fusion Medical Technologies. Mr. Anderson also served as an officer in the US Army Corps of Engineers, as a strategic planner and operational profit and loss

manager in General Electric and as a finance manager at Memorex. Mr. Anderson holds an M.B.A. from Harvard University, an M.S. in Administration from George Washington University and a B.S. in Engineering from the United States Military Academy. We nominated Mr. Anderson to the board of directors primarily due to his 30 years of healthcare experience in the areas of operations and finance.

Suzanne L. Bruhn, Ph.D. Dr. Bruhn has served as a director of Raptor Pharmaceutical Corp. since April 2011 and is Senior Vice President, Strategic Planning and Program Management for Shire Human Genetic Therapies (HGT), a division of Shire (NASDAQ: SHPGY) (LSE: SHP), specializing in the development and commercialization of treatments for orphan diseases. Since starting at Shire HGT in 1998, Dr. Bruhn has been responsible for establishing the program management function, driving strategic planning and portfolio management, and for global regulatory affairs. Dr. Bruhn has played a key role in the development, registration, and global expansion of Shire's products REPLAGAL®, ELAPRASE®, and VPRIV®, as well as Shire HGT's portfolio expansion through acquisitions, including FIRAZYR®. Prior to Shire HGT, Dr. Bruhn held various positions at Cytotherapeutics, Inc., a biotechnology company. Dr. Bruhn holds a Ph.D. in Chemistry from MIT and was a Postdoctoral Fellow in the Department of Human Genetics at Harvard Medical School. We nominated Dr. Bruhn to the board of directors due to her extensive healthcare experience in the orphan disease arena.

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Richard L. Franklin, M.D., Ph.D. Dr. Franklin has served as a director of Raptor Pharmaceutical Corp. since September 2009 and as a director of Raptor Pharmaceuticals Corp. since July 2008. Dr. Franklin served as Chairman of the board of directors of SyntheMed, Inc., a biomaterials company engaged in the development and commercialization of medical devices, from June 2003 to September 2011 and as a director of SyntheMed, Inc., from December 2000 to September 2011. Dr. Franklin has served as the Chief Executive Officer and Director of Tarix Pharmaceuticals, a drug development company, since 2004 and as Chairman of Pathfinder, LLC, a regenerative medicine company, since 2009. Pathfinder, LLC and SyntheMed merged in September 2011, at which point the combined companies were renamed Pathfinder Cell Therapy, Inc., and Dr. Franklin became the Chief Executive Officer and a director. Dr. Franklin received an M.A. in Mathematics from University of Wisconsin, a Ph.D. in Mathematics from Brandeis University and an M.D. from Boston University School of Medicine. We nominated Dr. Franklin to the board of directors due to his experience as a CEO and chairman of various healthcare companies.

Llew Keltner, M.D., Ph.D. Dr. Keltner has served as a director of Raptor Pharmaceutical Corp. since September 2009. Since May 2011, Dr. Keltner has been the chief executive officer of AgonOx, a biotech company developing OX40 agonists for use in cancer therapy. Dr. Keltner was the President of Novici Biotech, a privately-held gene and protein optimization firm, from 2010 to 2011. He is also Chief Executive Officer of EPISTAT, an international healthcare technology transfer, corporate risk management and healthcare strategy company that he founded in 1972. Dr. Keltner was Chief Executive Officer and President of Light Sciences Oncology, a privately-held biotechnology company developing a late stage, light-activated therapy for hepatocellular cancer and other solid tumors from 2001 to 2010. From 1997 to 2004, Dr. Keltner was Chief Executive Officer of Metastat, a development-stage biotech company focused on cancer metastasis. Dr. Keltner holds positions on the boards of Infostat, Oregon Life Sciences, and Goodwell Technologies. He is a previous director on the boards of Light Sciences Corporation, Vital Choice, Thesis Technologies, Oread Companies, and MannKind Corporation. He has also been a scientific advisory board member at Lifetime Corporation, ASB Meditest, Oread Laboratories, Hall-Kimbrell, and aai Pharma. He is currently a member of the American Society of Clinical Oncology, American Medical Association, International Association of Tumor Marker Oncology, American Association of Clinical Chemistry, and Drug Information Association. Dr. Keltner received an M.S. in Epidemiology and Biostatistics, a Ph.D. in Biomedical Informatics and an M.D. from Case Western Reserve University in Cleveland, Ohio. Dr. Keltner has also authored several research publications. We nominated Dr. Keltner to the board of directors due to his practical experience as a current chief executive officer of a life sciences company and due to his medical knowledge and network within the biotechnology industry.

Erich Sager. Mr. Sager has served as a director of Raptor Pharmaceutical Corp. since September 2009 and as a director of Raptor Pharmaceuticals Corp. since May 2006. He was a founding partner of Limetree Capital SA, a Swiss-based investment banking boutique, where he served as Chairman from 2006 to 2011. Mr. Sager also serves as Chairman and member of the board of directors at Calltrade Carrier Services AG, a European wholesale phone operator, and has held such position since 2004. He is also a current board member of Zecotek Medical Systems Inc. and Pulse Capital Corp. Mr. Sager served on the board of directors of BioMarin from November 1997 to March 2006 and as Chairman of LaMont Asset Management SA, a private investment management firm, from September 1996 until August 2004. Mr. Sager has held the position of Senior Vice President, Head of the Private Banking for Dresdner Bank (Switzerland) Ltd., Vice President, Private Banking, Head of the German Desk for Deutsche Bank (Switzerland) Ltd., and various positions at banks in Switzerland. Mr. Sager received a business degree from the School of Economics and Business Administration, Zurich, Switzerland. We nominated Mr. Sager to the board of directors due to his knowledge of healthcare fundraising in Europe including through his experience at BioMarin.

Vijay B. Samant. Mr. Samant has served as a director of Raptor Pharmaceutical Corp. since April 2011 and is President and Chief Executive Officer of Vical Inc. (NASDAQ: VICL), a leader in the development of DNA vaccines for infectious diseases and cancer therapeutics. Prior to Vical, Mr. Samant spent more than 20 years in diverse U.S and international sales, marketing, operations, and business development positions with Merck & Company (NYSE: MRK), including Chief Operating Officer of the Merck Vaccine Division, Vice President of Vaccine Operations in the

Manufacturing Division, Vice President of Business Affairs, and Executive Director of Materials Management. Mr. Samant has also been: a member of the Board of Trustees for the International Vaccine Institute (IVI, Seoul, Korea) since 2008; a member of the Board of Trustees for the National Foundation for Infectious Diseases (NFID, Bethesda, MD) since 2003; and a Director of the Aeras Global TB Vaccine Foundation from 2001 to 2010. Mr. Samant holds an S.M. from the Sloan School of Management at the Massachusetts Institute of Technology, as well as an M.S. in Chemical Engineering from Columbia University, and a B.S. in Chemical Engineering from the University of Bombay. We nominated Mr. Samant to the board of directors due to his experience in running a public healthcare company and due to his background in sales and marketing and business development.

Christopher M. Starr, Ph.D., Chief Executive Officer. Dr. Starr has served as the Chief Executive Officer and a director of Raptor Pharmaceutical Corp. since September 2009. Dr. Starr was a co-founder of Raptor Pharmaceuticals Corp. and has served as the Chief Executive Officer, President and director thereof since its inception in 2006. Dr. Starr has served as Chief Executive Officer of our wholly owned subsidiary, Raptor Pharmaceutical Inc., since its inception in September 2005. Dr. Starr co-founded BioMarin Pharmaceutical Inc. in 1997 where he last served as Senior Vice President and Chief Scientific Officer prior to joining us in 2006. As Senior Vice President at BioMarin, Dr. Starr was responsible for managing a Scientific Operations team

of 181 research, process development, manufacturing and quality personnel through the successful development of commercial manufacturing processes for its enzyme replacement products, and supervised the cGMP design, construction and licensing of BioMarin's proprietary biological manufacturing facility. From 1991 to 1998, Dr. Starr supervised research and commercial programs at BioMarin's predecessor company, Glyko, Inc., where he served as Vice President of Research and Development. Prior to his tenure at Glyko, Inc., Dr. Starr was a National Research Council Associate at the National Institutes of Health. Dr. Starr earned a B.S. from Syracuse University and a Ph.D. in Biochemistry and Molecular Biology from the State University of New York Health Science Center, in Syracuse, New York. We nominated Dr. Starr to the board of directors due to his extensive experience at BioMarin Pharmaceutical where he was directly involved in the successful approval of two drugs for orphan indications.

Timothy P. Walbert. Mr. Walbert has served as a director of Raptor Pharmaceutical Corp. since April 2011 and is Chairman, President and Chief Executive Officer of Horizon Pharma, Inc (NASDAQ: HZNP), a publicly traded biopharmaceutical company focused on developing and commercializing innovative medicines in arthritis, pain and inflammatory diseases. Prior to Horizon Pharma, Mr. Walbert was President, Chief Executive Officer and Director of IDM Pharma, Inc., a publicly traded oncology-focused biotechnology company. For more than 20 years, Mr. Walbert held executive positions in general management, corporate strategy, sales, US and international marketing and commercial operations at such biopharmaceutical industry leaders as Abbott Laboratories (NYSE: ABT), G.D. Searle/Pharmacia, Neopharm, Merck & Company (NYSE: MRK) and Wyeth. At Abbott, Mr. Walbert served as divisional vice president and general manager, immunology at Abbott, leading the global development and launch of HUMIRA, which attained over six billion in 2010 sales. Mr. Walbert serves on the board of directors of XOMA Ltd., the Biotechnology Industry Organization (BIO), the Illinois Biotechnology Industry Organization (iBIO) and the Greater Chicago Arthritis Foundation. Mr. Walbert holds a B.A. in Business and Marketing from Muhlenberg College. We nominated Mr. Walbert to the board of directors due to his experience in commercial operations, business strategy and his experience leading a publicly traded biopharmaceutical company.

Audit Committee

The audit committee of our board of directors, herein referred to as the Audit Committee, has been established in accordance with Section 3(a)(58)(A) of the Exchange Act. The Audit Committee is responsible for overseeing our accounting and financial reporting processes. In such capacity, our Audit Committee (a) has sole authority to appoint, replace and compensate our independent registered public accounting firm and is directly responsible for oversight of its work; (b) approves all audit fees and terms, as well as any permitted non-audit services performed by our independent registered public accounting firm; (c) meets and discusses directly with our independent registered public accounting firm its audit work and related matters; (d) oversees and performs investigations with respect to our internal and external auditing procedures, including the receipt, retention and treatment of complaints received by us regarding accounting, internal accounting controls or auditing matters and the confidential and anonymous submission by employees of concerns regarding questionable accounting or auditing matters and (e) undertakes such other activities as the Audit Committee deems necessary or advisable and as may be required by applicable law.

Our Audit Committee currently comprises Mr. Anderson, Dr. Franklin, Dr. Keltner and Mr. Walbert. Mr. Anderson has been designated as the "audit committee financial expert" as defined by the regulations promulgated by the SEC. Our board of directors has determined that each member of the Audit Committee is independent as defined by NASDAQ and SEC rules applicable to audit committee members.

Section 16(a) Beneficial Ownership Reporting Compliance

Section 16(a) of the Exchange Act requires our directors, executive officers and holders of more than ten percent of a registered class of our equity securities, or 10% Stockholders, to file reports of ownership and reports of changes in ownership of our common stock and other equity securities with the SEC. Directors, executive officers and 10%

Stockholders are required to furnish us with copies of all Section 16(a) forms they file. To our knowledge, based on a review of the copies of such reports furnished to us, we believe that during the fiscal year ended August 31, 2011, our directors, executive officers and 10% Stockholders timely filed all Section 16(a) reports applicable to them.

Code of Ethics

We have adopted a Code of Business Conduct and Ethics, which is applicable to our directors and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller or persons performing similar functions. Our Code of Business Conduct and Ethics is posted on the “Investors & Media—Corporate Governance” section of our website at www.raptorpharma.com and is reviewed and acknowledged by our directors and officers annually. If we make any substantive amendments to our Code of Business Conduct and Ethics or grant any waiver from a provision of the Code of Business Conduct and Ethics to any executive officer or director, we will promptly disclose the nature of the amendment or waiver in the “Investors & Media—Corporate Governance” section of our website at www.raptorpharma.com and/or in our public filings with the SEC.

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Executive Officers

The following table sets forth the name, age, date first appointed to serve as an executive officer, and position held by each of our executive officers as of October 31, 2011. Our executive officers are elected by our board of directors on an annual basis and serve at the discretion of our board of directors or until their successors have been duly elected and qualified.

Name	Age	Position(s) Held with the Company
Christopher M. Starr, Ph.D.	59	Chief Executive Officer and Director
Todd C. Zankel, Ph.D.	48	Chief Scientific Officer
Thomas (Ted) E. Daley	48	President, Raptor Therapeutics
Patrice P. Rioux, M.D., Ph.D.	60	Chief Medical Officer, Raptor Therapeutics
Kim R. Tsuchimoto	48	Chief Financial Officer, Treasurer and Secretary
Kathlene L. Powell	52	Vice President, Quality Operations, Raptor Therapeutics
Marc P. (Patrick) Reichenberger	47	Vice President, Commercial Operations, Raptor Therapeutics

The following describes the background of our executive officers except for Dr. Starr, whose background is described above under the heading “Business Experience and Directorships.”

Todd C. Zankel Ph.D. As of September 29, 2009, Dr. Zankel was appointed our Chief Scientific Officer. Prior to that, Dr. Zankel was a co-founder and has been Chief Scientific Officer of our wholly owned subsidiaries, Raptor Pharmaceutical Inc. and Raptor Pharmaceuticals Corp., since their inception in 2006. From 1997 to 2005, Dr. Zankel served as a Senior Director of Research at BioMarin. Prior to 1997, Dr. Zankel was a fellow for the National Institutes of Health at the Plant Gene Expression Center in Berkeley, California and at the Swiss Institute of Technology in Zurich, Switzerland. Dr. Zankel has been the author of a number of peer-reviewed articles in a variety of scientific areas. Dr. Zankel earned a B.A. from Reed College in Portland, Oregon and a Ph.D. from Columbia University.

Thomas (Ted) E. Daley. As of September 29, 2009, Mr. Daley joined us as President and a board member of Raptor Therapeutics Inc., a wholly-owned indirect subsidiary acquired in the 2009 Merger. Mr. Daley joined Raptor Therapeutics Inc. in September 2007 following the acquisition by it of Convivia, Inc., which Mr. Daley founded. Mr. Daley was co-founder, VP business development and chief operating officer of Instill Corporation, a leading electronic commerce services provider to the US foodservice industry. Between 1993 and 2001 Mr. Daley helped raise over \$50.0 million in venture capital and build Instill to a 150+ person operation with a nationwide customer base. After leaving Instill, from 2001 and 2007, Mr. Daley served in executive and consulting roles to a number of technology startup companies including MetricStream, Inc., PartsRiver and Certicom Security. Prior to that time, Mr. Daley worked in operations management for Anheuser-Busch, Inc., and consulted to Gordon Biersch Brewing Company and Lion Breweries (New Zealand). Mr. Daley received a BS in Fermentation Science from University of California at Davis, and an M.B.A. from Stanford University.

Patrice P. Rioux, M.D., Ph.D. As of September 29, 2009, Dr. Rioux joined us as Chief Medical Officer of Raptor Therapeutics Inc., a wholly-owned indirect clinical subsidiary acquired in the 2009 Merger. Prior to joining Raptor Therapeutics Inc. in April 2009, from November 2008 until March 2009, Dr. Rioux served as Chief Medical Officer of FerroKin Biosciences, an early-stage developer of iron chelator for treatment of anemias. From May 2005 to October 2008, he was Chief Medical Officer and Vice President Clinical/Regulatory for Edison Pharmaceuticals, which focused on developing drugs to treat inherited and acquired energy impairment diseases. From January 2004 through March 2006, Dr. Rioux was an independent clinical operations consultant. Dr. Rioux’ three-decade career includes positions at Repligen Corp., Arrow International, Variagenics, Inc., Biogen and GRP (Groupement de

Recherche en Pharmacologie). From 1975 to 1995, Dr. Rioux was a researcher in Clinical Research and Epidemiology at INSERM (Institut National de la Sante et de la Recherche Medicale), a French organization that supports national research in the medical field. Educated in France, Dr. Rioux has an M.D., a Ph.D. in Mathematical Statistics, and a Masters degree in Pharmacology.

Kim R. Tsuchimoto. As of September 29, 2009, Ms. Tsuchimoto was appointed our Chief Financial Officer, Treasurer and Secretary. Prior to that Ms. Tsuchimoto has served as the Chief Financial Officer, Treasurer and Secretary of our wholly owned subsidiaries, Raptor Pharmaceutical Inc. and Raptor Pharmaceuticals Corp., since their respective inceptions in 2006. Prior to this, Ms. Tsuchimoto served as Interim Controller at International Microcomputer Software, Inc., a software and Internet content company, from October 2005 to March 2006. From June 2005 to August 2005, Ms. Tsuchimoto served as Assistant Vice President, Controller at SpatiaLight Inc., a high technology company. From February 1997 to June 2005, Ms. Tsuchimoto served at BioMarin and its predecessor company, Glyko, Inc., most recently as Vice President, Treasurer for two years, Vice President, Controller for two years and prior to that, as Controller. Prior to her employment at BioMarin, Ms. Tsuchimoto served as Controller of a marketing consulting firm and an international venture capital firm and worked as a staff accountant in a local public accounting firm. Ms. Tsuchimoto is an inactive licensed California Certified Public Accountant and holds a B.S. in Business Administration with an emphasis in Accounting from San Francisco State University.

Kathlene L. Powell. As of April 2011, Ms. Powell was appointed our Vice President, Quality Operations of Raptor Therapeutics. Prior to that, Ms. Powell worked for Pacific BioDevelopment, LLC, a San Francisco-based biotechnology consulting group, where she most recently held the position of Vice President, Quality. During her nine years at Pacific BioDevelopment, Ms. Powell executed numerous quality and control initiatives for their client companies, including the development of CMC regulatory strategies and preparation of CMC sections of regulatory submissions; the selection and management of CMOs for the development, manufacture, and testing of drug substances and drug products; and the evaluation of quality control test methods, quality control data, stability programs, validation protocols and validation reports. Prior to Pacific BioDevelopment, Ms. Powell held quality management positions at Genentech and Covance Biotechnology Services, Inc. Ms. Powell received a B.A. in Chemistry from Doane College in Crete, Nebraska and an M.S. in Biochemistry from University of Missouri.

Patrick Reichenberger As of January 2011, Mr. Reichenberger was appointed our Vice President, Commercial Operations of Raptor Therapeutics. Mr. Reichenberger has over 20 years of experience in biotech/pharma sales and marketing including orphan product development and commercialization. Prior to joining Raptor, from September 2004 to May 2010, Mr. Reichenberger served as Senior Director at XOMA LLC. In this role Mr. Reichenberger led XOMA's commercial development department with particular emphasis on XOMA 052, an anti IL 1 antibody for diabetes, cardiovascular disease and Behcet's Uveitis, an orphan disease. Prior to XOMA, Mr. Reichenberger was an independent consultant to biopharma companies including XOMA. From October 2000 to July 2003, Mr. Reichenberger managed marketing, sales, reimbursement and distribution at Questcor Pharmaceuticals supporting the re-launch of H.P. Acthar® Gel for the treatment of infantile spasm, an ultra-orphan, pediatric disease. Prior to Questcor, he held positions of increasing responsibility at Genentech, Athena Neurosciences, a division of Elan, and Parke-Davis Pharmaceuticals. Mr. Reichenberger has an M.B.A. from Pepperdine University, where he graduated with Honors, and a B.S. in Biology from University of California, Los Angeles.

Relationships Among Executive Officers and Directors

There are no family relationships among any of our directors or executive officers.

ITEM 11: EXECUTIVE COMPENSATION

The information required by Item 11 is incorporated by reference to our Proxy Statement for our 2012 Annual Meeting of Stockholders, which will be filed with the SEC no later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K, or, alternatively, by amendment to this Annual Report on Form 10-K under cover of Form 10-K/A no later than the end of such 120 day period.

ITEM 12: SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

Equity Compensation Plan Information

The following table provides certain information with respect to all of our equity compensation plans in effect as of August 31, 2011:

Plan Category	Number of securities to be issued upon exercise of outstanding options, warrants and rights	Weighted average exercise price of outstanding options, warrants and rights	Number of securities remaining available for future issuance under equity compensation plans
Equity compensation plans approved by stockholders	3,580,555	6.64	3,873,413
Equity compensation plans not approved by stockholders	-	-	-
Total	3,580,555	6.64	3,873,413

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth, as of October 31, 2011, each beneficial owner (or group of affiliated beneficial owners) of more than five percent (5%) of any class of our voting securities, each of our named executive officers as of the end of the fiscal year ended August 31, 2011, each our directors and all of our executive officers and directors as a group. Except as otherwise indicated, each listed stockholder directly owned his or her shares and had sole voting and investment power. Unless otherwise noted, the address for each person listed below is Raptor Pharmaceutical Corp., 9 Commercial Blvd., Suite 200, Novato, CA 94949.

Name of Beneficial Owner and Address	Number of Shares of Common Stock Beneficially Owned	Number of Shares Subject to Options and Warrants (1)	Percentage of Outstanding Shares of Common Stock (2)
Columbia Management Investment Advisers, LLC (3) Entities affiliated with Deerfield Management Company, LP (4)	3,720,000	-	7.9%
Christopher M. Starr, Ph.D. (5)	1,017,577	318,208	2.1%
Ted Daley	246,857	141,953	*
Patrice P. Rioux, M.D, Ph.D.	123,549	123,549	*
Kim R. Tsuchimoto	164,488	163,906	*
Todd C. Zankel, Ph.D.	827,983	123,664	1.8%
Raymond W. Anderson	212,061	212,061	*
Suzanne L. Bruhn, Ph.D.	16,249	16,249	*
Richard L. Franklin, M.D., Ph.D.	102,054	102,054	*
Llew Keltner, M.D., Ph.D.	91,126	91,126	*
Erich Sager	564,077	328,623	1.1%
Vijay B. Samant	16,249	16,249	*
Timothy P. Walbert	16,249	16,249	*
All executive officers and directors as a group (14 persons)	3,442,684	1,698,056	3.7%

* Less than one percent.

- (1) Beneficial ownership is determined in accordance with SEC rules and generally includes voting or investment power with respect to securities. Shares of common stock subject to options, warrants and convertible preferred stock currently exercisable or convertible, or exercisable or convertible within sixty (60) days of October 31, 2011, are counted as outstanding for computing the percentage held by each person holding such options or

warrants but are not counted as outstanding for computing the percentage of any other person.

- (2) Based on 47,153,503 shares outstanding as of October 31, 2011.
- (3) Entities affiliated with Columbia Management Investment Advisers, LLC collectively hold an aggregate of 3,720,000 shares of our common stock. The principal business address for Columbia Management Investment Advisers, LLC is 227 West Monroe Street, Suite 3000, Chicago, IL 60606.
- (4) Includes 446,042 shares and warrants to purchase 643,976 shares held by Deerfield Special Situations Fund, LP, and 694,741 shares and warrants to purchase 1,007,244 shares held by Deerfield Special Situations Fund International, Limited, as of September 29, 2011. Deerfield Special Situations Fund, LP and Deerfield Special Situations Fund International, Limited, (or collectively, the "Deerfield Funds") are affiliated with Deerfield Management Company, LP. The Deerfield Funds were issued warrants to purchase an aggregate of 1,951,220 shares of common stock in the 2010 Private Placement. However, these warrants are exercisable only to the extent that the number of shares beneficially held by the entities affiliated with Deerfield Management Company, LP does not exceed 9.999% of our outstanding stock and therefore, a portion of those warrants have not been counted as outstanding for purposes of computing the percentage held by the entities affiliated with Deerfield Capital Management, LP. The principal business address of each of the Deerfield Funds is 780 3rd Avenue, 37th Floor, New York, NY 10017.
- (5) Includes 699,369 shares our common stock owned by the Christopher M. and S.L. Starr Trust of which Dr. Starr is a co-trustee and beneficiary and shares voting and investment power, and options to purchase 318,208 shares of our common stock by Dr. Starr directly.

ITEM 13: CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

Review, Approval or Ratification of Transactions with Related Persons

Since September 1, 2010, there has not been nor is there currently proposed any transaction or series of similar transactions to which we were or are to be a party in which the amount involved exceeds \$120,000 and in which any of our directors, executive officers, persons who we know hold more than 5% of our common stock, or any member of the immediate family of any of the foregoing persons had or will have a direct or indirect material interest other than: (i) compensation agreements and other arrangements, which are described elsewhere in this Annual Report on Form 10-K, and (ii) the transactions described below.

We have entered into indemnity agreements with certain of our officers and directors which provide, among other things, that we will indemnify such officer or director, under the circumstances and to the extent provided for therein, for expenses, damages, judgments, fines and settlements he or she may be required to pay in actions or proceedings which he or she is or may be made a party by reason of his or her position as a director, officer or other agent of us, and otherwise to the fullest extent permitted under Delaware law and our Bylaws.

Pursuant to the terms of an asset purchase agreement, we and our wholly-owned subsidiary, Raptor Therapeutics Inc. purchased certain assets of Convivia, Inc., which was as of such time wholly-owned by Ted Daley (currently the

President of Raptor Therapeutics Inc.). Pursuant to the terms of that asset purchase agreement and Mr. Daley's employment agreement, to date, in aggregate Mr. Daley has received 104,904 shares of our common stock and \$90,000 in cash bonuses and may receive additional common stock and cash bonuses based on the successful development of our Convivia™ development program. Mr. Daley was hired to develop the Convivia™ product candidate along with other clinical-stage programs at Raptor Therapeutics Inc.

In the ordinary course of business, our officers have loaned money to us by paying travel expenses and equipment and other costs from their personal funds on our behalf. We have promptly reimbursed the officers for such expenses and costs.

Independence of Our Board of Directors

Our board of directors has determined that all current members of our board of directors are independent (as independence is currently defined in Rule 5605(a)(2) of the NASDAQ listing standards), except for Dr. Starr and Mr. Sager. Our board of directors has also determined that each member of our Audit Committee, Compensation Committee and Corporate Governance and Nominating Committee is independent as defined by the SEC and NASDAQ rules.

ITEM 14: PRINCIPAL ACCOUNTING FEES AND SERVICES

Independent Registered Public Accounting Firm

Since June 15, 2006, Burr Pilger Mayer, Inc. has served as our independent registered public accounting firm.

The following is a summary of the fees and services provided for our years ended August 31, 2011 and 2010.

Description of Services Provided by Burr Pilger Mayer, Inc.	Year Ended August 31, 2011	Year Ended August 31, 2010
Audit Fees*	\$ 215,622	\$ 161,232
Audit Related Fees: These services relate to assurance and related services reasonably related to the performance of the audit or review of financial statements not included above.	82,160	133,036
Tax Compliance Fees: These services relate to the preparation of federal, state and foreign tax returns and other filings.	28,839	39,151
Tax Consulting and Advisory Services: These services primarily relate to the area of tax strategy and minimizing Federal, state, local and foreign taxes.	-	-
All Other Fees	-	-

* Audit Fees for August 31, 2011 includes unbilled audit fees for the year ended August 31, 2011, which is estimated to be \$165,000. Audit Fees for August 31, 2010 includes audit fees for the year ended August 31, 2010, billed and paid during the year ended August 31, 2011 totaling \$111,926.

As provided in the Audit Committee charter, the Audit Committee pre-approves all of the services provided by our independent registered public accounting firm. 100% of the above services and estimates of the expected fees were reviewed and approved by the Audit Committee before the respective services were rendered.

The Audit Committee has considered the nature and amount of the fees billed by Burr Pilger Mayer, Inc. and believes that the provision of the services for activities unrelated to the audit is compatible with maintaining Burr Pilger Mayer, Inc.'s independence.

PART IV

ITEM 15: EXHIBITS, FINANCIAL STATEMENT SCHEDULES

The information required to be filed in this item appears on pages F-1 to F-41 of this Annual Report on Form 10-K.

D o c u m e n t s f i l e d a s p a r t o f t h i s a n n u a l r e p o r t o n F o r m 10-K:

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Reports of Independent Registered Public Accounting Firm	F-1
Consolidated Balance Sheets as of August 31, 2011 and 2010	F-3
Consolidated Statements of Comprehensive Loss for the years ended August 31, 2011 and 2010 and for the cumulative period from September 8, 2005 (inception) to August 31, 2011	F-4
Consolidated Statements of Stockholders' Equity (Deficit) for period from September 8, 2005 (inception) to August 31, 2006 and the years ended August 31, 2007, 2008, 2009, 2011 and 2010	F-5
Consolidated Statements of Cash Flows for the years ended August 31, 2011 and 2010 and for the cumulative period from September 8, 2005 (inception) to August 31, 2011	F-11
Notes to Consolidated Financial Statements	F-12

Exhibits

The following exhibits are filed as part of, or incorporated by reference into this Annual Report on Form 10-K:

Exhibit Index

- | | |
|-----|--|
| 1.1 | Underwriting Agreement, dated September 8, 2011, by and between Raptor Pharmaceutical Corp. and JMP Securities LLC, as representative of the underwriters named therein (incorporated by reference to Exhibit 1.1 to the Registrant's Current Report on Form 8-K, filed on September 8, 2011). |
| 2.1 | Agreement and Plan of Merger and Reorganization, dated as of June 7, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Registration Statement No. 333-136018 filed on July 25, 2006). |
| 2.2 | Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated as of August 25, 2006, by and among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Annex A to Amendment No. 1 to Registration Statement No. 333-136018 filed on August 25, 2006). |
| 2.3 | Agreement and Plan of Merger and Reorganization, dated July 27, 2009, by and among Raptor Pharmaceuticals Corp., TorreyPines Therapeutics, Inc., a Delaware corporation, and ECP Acquisition, Inc., a Delaware corporation (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009). |
| 2.4 | Form of Voting Agreement between TorreyPines Therapeutics, Inc. and certain stockholders of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 99.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009). |
| 2.5 | Form of Voting Agreement between Raptor Pharmaceuticals Corp. and certain stockholders of TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009). |
| 3.1 | Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006). |
| 3.2 | Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006). |
| 3.3 | Certificate of Amendment filed with the Secretary of State of the State of Nevada effecting an 8-for-1 reverse stock of the Registrant's common stock and changing the name of the Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.3 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006). |
| 3.4 | Articles of Conversion filed with the Secretary of State of the State of Nevada changing the state of incorporation of the Registrant (incorporated by reference to Exhibit 3.4 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006). |
| 3.5 | Certificate of Conversion filed with the Secretary of State of the State of Delaware (incorporated by reference to Exhibit 3.5 to the Registrant's Current |

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- 3.6 Report on Form 8-K, filed on October 10, 2006).
Amendment to Bylaws of the Registrant (incorporated by reference to Exhibit 3.6 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 3.7 Charter Amendment for TorreyPines (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
- 3.8 Certificate of Merger between Raptor Pharmaceuticals Corp., ECP Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
- 4.1 Specimen common stock certificate of the Registrant (incorporated by reference to Exhibit 4.7 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
- 4.2 Form of Warrant to Purchase Common Stock issued to previous holders of TPTX, Inc. redeemable convertible preferred stock in connection with the business combination between TorreyPines Therapeutics, Inc. and Axonyx, Inc. (incorporated by reference to Exhibit 4.2 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.3 Form of Registration Rights Agreement 1999 (incorporated by reference to Exhibit 4.4 to the Registrant's Annual Report on Form 10-KSB, filed on March 13, 2000).
- 4.4 Registration Rights Agreement dated as of January 8, 2004 between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on January 12, 2004).
- 4.5 Registration Rights Agreement dated as of May 3, 2004, between Axonyx Inc. and certain investors (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on May 5, 2004).
- 4.6 Form of Warrant issued to Comerica Bank on July 1, 2003 (incorporated by reference to Exhibit 4.14 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.7 Form of Warrant issued to Silicon Valley Bank on December 8, 2000 (incorporated by reference to Exhibit 4.15 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.8 Form of Warrant issued to Oxford Financial and Silicon Valley Bank on September 27, 2005 (incorporated by reference to Exhibit 4.16 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).

- 4.9 Rights Agreement, dated as of May 13, 2005, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 99.2 to the Registrant's Current Report on Form 8-K, filed on May 16, 2005).
- 4.10 Amendment to Rights Agreement, dated as of June 7, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, filed on June 12, 2006).
- 4.11 Form of Warrant issued to Comerica Bank on June 11, 2008 (incorporated by reference to Exhibit 4.1 to the Registrant's Report on Form 8-K, filed on June 17, 2008).
- 4.12 Amendment to Rights Agreement, dated as of October 3, 2006, between the Registrant and The Nevada Agency and Trust Company, as Rights Agent (incorporated by reference to Exhibit 4.19 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 4.13 Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between TorreyPines and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company) (incorporated by reference to Exhibit 2.3 to the Registrant's Current Report on Form 8-K, filed on July 28, 2009).
- 4.14 Amendment to Rights Agreement, dated August 6, 2010, by and between the Registrant and American Stock Transfer & Trust Company, LLC (incorporated by reference to Exhibit 4.2 to the Registrant's Current Report on Form 8-K, filed on August 10, 2010).
- 4.15 * Warrant to purchase common stock dated December 14, 2007 issued to Flower Ventures, LLC (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A, filed on April 15, 2008).
- 4.16 * Warrant Agreement Amendment, dated December 17, 2009, between the Registrant and Flower Ventures, LLC (incorporated by reference to Exhibit 4.15 to Registrant's Quarterly Report on Form 10QSB, filed on April 9, 2010).
- 4.17 * Form of Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on May 22, 2008).
- 4.18 * Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K/A, filed on May 28, 2008).
- 4.19* Form of Placement Agent Warrant to purchase common stock of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 4.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on August 25, 2009).
- 4.20 Form of Senior Debt Indenture of the Registrant (incorporated by reference to Exhibit 4.10 to the Registrant's Registration Statement on Form S-3, filed on October 7, 2009).
- 4.21 Form of Subordinated Debt Indenture of the Registrant (incorporated by reference to Exhibit 4.11 to the Registrant's Registration Statement on Form S-3, filed on October 7, 2009).
- 4.22 Form of Investor Warrants (incorporated by reference to Exhibit 4.1 on Registrant's Current Report on Form 8-K filed on December 18, 2009).
- 4.23 Form of Investor Warrants (incorporated by reference to Exhibit 4.1 on Registrant's Current Report on Form 8-K filed on August 10, 2010).
- 4.24 Placement Agent Warrant (incorporated by reference to Exhibit 4.2 on Registrant's Current Report on Form 8-K filed on August 13, 2010).
- 4.27 Reference is made to Exhibits 3.1 through 3.8.
- 10.1# TorreyPines Therapeutics, Inc. 2006 Equity Incentive Plan (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed on October 4, 2006).
- 10.2# Form of Stock Option Agreement under TorreyPines Therapeutics, Inc. 2006 Equity Incentive Plan (incorporated by reference to Exhibit 10.9 to the Registrant's Current Report on

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Form 8-K, filed on October 14, 2006).

- 10.3** Development and License Agreement between TPTX, Inc. (formerly Neurogenetics, Inc.) and Eli Lilly and the Registrant, effective as of April 21, 2003 (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
- 10.4** Research and License Agreement by and between TPTX, Inc. and Life Science Research Israel Ltd. dated as of May 10, 2004 (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
- 10.5** License Agreement by and between TPTX, Inc. and University of Iowa Research Foundation dated as of May 10, 2006 (incorporated by reference to Exhibit 10.5 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
- 10.6 Form of Indemnity Agreement (incorporated by reference to Exhibit 10.13 to the Registrant's Current Report on Form 8-K, filed on October 10, 2006).
- 10.7# Form of Restricted Stock Unit Award Agreement under TorreyPines Therapeutics, Inc. 2006 Equity Incentive Plan (incorporated by reference to Exhibit 10.17 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
- 10.8# Employment Agreement between Raptor Pharmaceuticals Corp. and Dr. Christopher Starr dated May 1, 2006 (incorporated by reference to Exhibit 10.5 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on May 26, 2006).
- 10.9# First Amendment to the Employment Agreement between Raptor Pharmaceuticals Corp. and Dr. Christopher Starr dated January 1, 2009 (incorporated by reference to Exhibit 10.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on January 5, 2009).

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- 10.10# Employment Agreement between Raptor Pharmaceuticals Corp. and Dr. Todd Zankel dated May 15, 2006 (incorporated by reference to Exhibit 10.6 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K filed on May 26, 2006).
- 10.11# First Amendment to the Employment Agreement between Raptor Pharmaceuticals Corp. and Dr. Todd Zankel dated January 1, 2009 (incorporated by reference to Exhibit 10.3 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on January 5, 2009).
- 10.12# Employment Agreement between Raptor Pharmaceuticals Corp. and Ms. Kim Tsuchimoto dated May 1, 2006 (incorporated by reference to Exhibit 10.7 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K filed on May 26, 2006).
- 10.13# First Amendment to the Employment Agreement between Raptor Pharmaceuticals Corp. and Ms. Kim Tsuchimoto dated January 1, 2009 (incorporated by reference to Exhibit 10.2 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on January 5, 2009).
- 10.14# Employment Agreement between Raptor Therapeutics Inc. and Thomas E. Daley dated September 7, 2007 (incorporated by reference to Exhibit 10.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10-QSB filed on January 14, 2008).
- 10.15# First Amendment to the Employment Agreement between Raptor Pharmaceuticals Corp. and Thomas E. Daley dated January 1, 2009 (incorporated by reference to Exhibit 10.4 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K, filed on January 5, 2009).
- 10.16# Offer Letter from Raptor Therapeutics Inc. dated April 8, 2009 for Dr. Patrice Rioux (incorporated by reference to Exhibit 10.1 to Raptor Pharmaceuticals Corp.'s Current Report on Form 8-K filed on April 14, 2008).
- 10.17†#* Offer Letter from Raptor Therapeutics Inc. dated January 1, 2011 for Patrick Reichenberger.
- 10.18†#* Offer Letter from Raptor Therapeutics Inc. dated April 6, 2011 for Kathy Powell.
- 10.19# 2006 Equity Incentive Plan of Raptor Pharmaceuticals Corp., as amended (incorporated by reference to Exhibit 4.3 to Raptor Pharmaceuticals Corp.'s Registration Statement on Form S-8 filed on February 28, 2007).
- 10.20# 2008 Plan Amendment to 2006 Equity Incentive Plan of Raptor Pharmaceuticals Corp. (incorporated by reference to Exhibit 10.5 to Raptor Pharmaceuticals Corp.'s Annual Report on Form 10-K/A filed on December 23, 2008).
- 10.21 Asset Purchase Agreement between Raptor Therapeutics, Inc., Raptor Pharmaceuticals Corp. and Convivia, Inc. dated October 17, 2007 (incorporated by reference to Exhibit 10.3 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB filed on January 14, 2008).
- 10.22 Merger agreement between Raptor Therapeutics, Inc., Raptor Pharmaceuticals Corp. and Encode Pharmaceuticals, Inc. dated December 14, 2007 (incorporated by reference to Exhibit 10.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A filed on April 15, 2008).
- 10.23** Pharmaceutical development services agreement between Raptor Therapeutics Inc. and Patheon Pharmaceuticals Inc. dated January 7, 2008 (incorporated by reference to Exhibit 10.2 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A filed on April 15, 2008).
- 10.24** License agreement between Raptor Therapeutics Inc. and Regents of the University of California dated October 31, 2007 (incorporated by reference to Exhibit 10.3 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A filed on April 15, 2008).
- 10.25** Amendment No. 1 to License agreement between Raptor Therapeutics Inc. and Regents of the University of California dated February 29, 2008 (incorporated by reference to Exhibit 10.4 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB/A filed on April 15, 2008).
- 10.26 Securities Purchase Agreement, dated as of May 21, 2008, by and among Raptor Pharmaceuticals Corp. and the investors listed on the signature pages thereto (incorporated by

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reference to Exhibit 10.1 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB filed on July 9, 2008).

- 10.27 Amendment to Securities Purchase Agreement, dated as of May 21, 2008, by and among Raptor Pharmaceuticals Corp. and the investors listed on the signature pages thereto (incorporated by reference to Exhibit 10.2 to Raptor Pharmaceuticals Corp.'s Quarterly Report on Form 10QSB filed on July 9, 2008).
- 10.28** Collaboration and License Agreement, effective June 3, 2009, among Hoffmann-La Roche Ltd., Hoffmann-La Roche Inc. and the Registrant (incorporated by reference to Exhibit 10.19 on Raptor Pharmaceuticals Corp.'s Annual Report on Form 10-K filed on October 28, 2009).
- 10.29 First Amendment dated January 7, 2009 to Lease by and between TorreyPines Therapeutics, Inc. and HCP TPSP LLC dated July 18, 2005 (incorporated by reference to Exhibit 10.15 to the Registrant's Annual Report on Form 10-K, filed on March 27, 2009).
- 10.30** Amendment dated November 21, 2008 to Development and License Agreement by and between TPTX, Inc. and Eli Lilly and the Registrant, effective as of April 21, 2003 (incorporated by reference to Exhibit 10.16 to the Registrant's Annual Report on Form 10-K, filed on March 27, 2009).
- 10.31 Securities Purchase Agreement, dated as of August 21, 2009, by and among Raptor Pharmaceuticals Corp. and the investors listed on the signature pages thereto (incorporated by reference to Exhibit 10.19 on Raptor Pharmaceuticals Corp.'s Annual Report on Form 10-K filed on October 28, 2009).
- 10.32 Raptor Form Indemnity Agreement dated on December 9, 2009 (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K filed on December 15, 2009).

- 10.33 Placement Agent Agreement by and between the Registrant and Ladenburg Thalmann & Co. Inc. dated December 17, 2009 (incorporated by reference to Exhibit 1.1 on Registrant's Current Report on Form 8-K filed on December 18, 2009).
- 10.34 Securities Purchase Agreement, dated December 17, 2009, by and between the Registrant and the investors signatories thereto (incorporated by reference to Exhibit 10.1 on Registrant's Current Report on Form 8-K filed on December 18, 2009).
- 10.35# Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan (incorporated by reference to Appendix A to the Registrant's Revised Definitive Proxy Statement, filed on February 5, 2010).
- 10.36# 2011 Plan Amendments to the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan (incorporated by reference to Exhibit 4.15 to the Registrant's Registration Statement on Form S-8 (File No. 333-173719), filed on April 25, 2011).
- 10.37 Purchase Agreement, dated April 16, 2010, between the Registrant and Lincoln Park Capital Fund, LLC (incorporated by reference to Exhibit 10.1 on Registrant's Current Report on Form 8-K, filed on April 22, 2010).
- 10.38 Registration Rights Agreement, dated April 16, 2010, between the Registrant and Lincoln Park Capital Fund, LLC (incorporated by reference to Exhibit 10.2 on Registrant's Current Report on Form 8-K, filed on April 22, 2010).
- 10.39# Form of Award Agreement under the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan (incorporated by reference to Exhibit 4.13 to the Registrant's Registration Statement on Form S-8 (File No. 33-166813), filed on May 14, 2010).
- 10.40# Form of Award Agreement under the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan (incorporated by reference to Exhibit 10.1 of the Registrant's Current Report on Form 8-K filed on September 28, 2011).
- 10.41 Securities Purchase Agreement, dated August 9, 2010, by and among the Registrant and the Investors signatories thereto (incorporated by reference to Exhibit 10.1 on Registrant's Current Report on Form 8-K, filed on August 10, 2010).
- 10.42 Securities Purchase Agreement, dated August 9, 2010, by and among the Registrant and the Investor signatory thereto (incorporated by reference to Exhibit 10.2 of the Registrant's Current Report on Form 8-K filed on August 10, 2010).
- 10.43 Registration Rights Agreement, dated August 12, 2010, by and among the Registrant and the signatories thereto (incorporated by reference to Exhibit 10.3 of the Registrant's Current Report on Form 8-K, filed on August 13, 2010).
- 10.44** Manufacturing Services Agreement, dated as of November 15, 2010, by and between Patheon Pharmaceuticals Inc. and Raptor Therapeutics, Inc. (incorporated by reference to Exhibit 10.53 of the Registrant's Post-Effective Amendment No. 1 to the Registration Statement on Form S-1 filed on November 23, 2010 (File No. 333-168966)).
- 10.45** API Supply Agreement, dated November 15, 2010, by and between Raptor Therapeutics Inc. and Cambrex Profarmaco Milano (incorporated by reference to Exhibit 10.54 of the Registrant's Post-Effective Amendment No. 1 to the Registration Statement on Form S-1 filed on November 23, 2010 (File No. 333-168966)).
- 21.1 Subsidiaries of the Registrant (incorporated by reference to Exhibit 21.1 of the Registrant's Annual Report on Form 10-K, filed on November 22, 2010).
- 23.1† Consent of Burr Pilger Mayer, Inc. Independent Registered Public Accounting Firm to the Registrant
- 24.1† Power of Attorney (included in the signature page hereto).
- 31.1† Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director.
- 31.2† Certification of Kim R. Tsuchimoto, Chief Financial Officer, Secretary and Treasurer.

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- 32.1† Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director, and of Kim R. Tsuchimoto, Chief Financial Officer, Secretary and Treasurer.
- * The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.15 - 4.19 have been converted into warrants of the Registrant and the exercise price of such warrants and number of shares of common stock issuable thereunder have been converted as described in Item 1.01 (under the section titled, "Background") of the Registrant's Current Report on Form 8-K, filed on October 5, 2009.
- ** Certain information omitted pursuant to a request for confidential treatment filed separately with and granted by the SEC.
- # Indicates a management contract or compensatory plan or arrangement.
- † Filed herewith.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

RAPTOR PHARMACEUTICAL CORP.

Dated: November 14, 2011

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

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Christopher M. Starr, Ph.D. and Kim R. Tsuchimoto, his or her attorney-in-fact, with the power of substitution, for him or her in any and all capacities, to sign any amendments to the Annual Report on Form 10-K and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorneys-in-fact, or his or her substitute or substitutes, may do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated:

Signatures	Title	Date
/s/ Christopher M. Starr Christopher M. Starr, Ph.D.	Chief Executive Officer and Director (Principal Executive Officer)	November 14, 2011
/s/ Kim R. Tsuchimoto Kim R. Tsuchimoto	Chief Financial Officer, Secretary and Treasurer (Principal Financial Officer and Principal Accounting Officer)	November 14, 2011
/s/ Raymond William Anderson Raymond William Anderson	Director	November 14, 2011
/s/ Suzanne L. Bruhn Suzanne L. Bruhn, Ph.D.	Director	November 14, 2011
/s/ Richard L. Franklin Richard L. Franklin, M.D., Ph.D.	Director	November 14, 2011
/s/ Llew Keltner Llew Keltner, M.D., Ph.D.	Director	November 14, 2011
/s/ Erich Sager Erich Sager	Director	November 14, 2011

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/s/ Vijay B. Samant
Vijay B. Samant

Director

November 14, 2011

/s/ Timothy P. Walbert
Timothy P. Walbert

Director

November 14, 2011

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Financial Statements

The following consolidated financial statements of Raptor Pharmaceuticals Corp. and the Independent Registered Public Accounting Firm's Report issued thereon, are incorporated by reference in Part II, Item 8 of this Annual Report on Form 10-K:

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Consolidated Statements of Comprehensive Loss for the years ended August 31, 2011 and 2010 and for the cumulative period from September 8, 2005 (inception) to August 31, 2011	F-4
Consolidated Statements of Stockholders' Equity (Deficit) for period from September 8, 2005 (inception) to August 31, 2006 and the years ended August 31, 2007, 2008, 2009, 2010 and 2011	F-5
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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of
Raptor Pharmaceutical Corp.

We have audited the accompanying consolidated balance sheets of Raptor Pharmaceutical Corp. and its subsidiaries (the "Company") (a development stage enterprise) as of August 31, 2011 and 2010, and the related consolidated statements of comprehensive loss, stockholders' equity (deficit), and cash flows for the years ended August 31, 2011 and 2010 and the cumulative amounts from September 8, 2005 (inception) to August 31, 2011. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of Raptor Pharmaceutical Corp. and its subsidiaries as of August 31, 2011 and 2010, and the results of their operations and their cash flows for the years ended August 31, 2011 and 2010 and the cumulative amounts from September 8, 2005 (inception) to August 31, 2011 in conformity with accounting principles generally accepted in the United States of America.

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 2 to the consolidated financial statements, the Company's significant operating losses raise substantial doubt about its ability to continue as a going concern. These conditions raise substantial doubt about the Company's ability to continue as a going concern. Management's plans in regard to those matters are also described in Note 2. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

As discussed in Note 1 to the consolidated financial statements, effective September 29, 2009, the Company's wholly-owned subsidiary, ECP Acquisition, Inc., merged with and into Raptor Pharmaceuticals Corp.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the effectiveness of the Company's internal control over financial reporting as of August 31, 2011, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission, and our report dated November 14, 2011 expressed an unqualified opinion thereon.

/s/ Burr Pilger Mayer, Inc.
Burr Pilger Mayer, Inc.
San Francisco, California
November 14, 2011

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of
Raptor Pharmaceutical Corp.

We have audited the internal control over financial reporting of Raptor Pharmaceutical Corp. and its subsidiaries (the “Company”) (a development stage enterprise) as of August 31, 2011, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). The Company’s management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management’s Annual Report on Internal Control over Financial Reporting, appearing in Item 9A. Our responsibility is to express an opinion on the effectiveness of the Company’s internal control over financial reporting based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary under the circumstances. We believe that our audit provides a reasonable basis for our opinion.

A company’s internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company’s internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company’s assets that could have a material effect on the financial statements.

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

In our opinion, Raptor Pharmaceutical Corp. and its subsidiaries maintained, in all material respects, effective internal control over financial reporting as of August 31, 2011, based on the COSO criteria.

We have also audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of Raptor Pharmaceutical Corp. and its subsidiaries as of August 31, 2011 and 2010, and the related consolidated statements of comprehensive loss, stockholders’ equity (deficit), and cash flows for the years ended August 31, 2011 and 2010 and for the cumulative amounts from September 8, 2005 (inception) to

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August 31, 2011, and our report dated November 14, 2011 expressed an unqualified opinion on those consolidated financial statements.

/s/ Burr Pilger Mayer, Inc.
Burr Pilger Mayer, Inc
San Francisco, California
November 14, 2011

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

ASSETS	August 31,	
	2011	2010
Current assets:		
Cash and cash equivalents	\$ 15,172,086	\$ 16,953,524
Restricted cash	114,468	-
Prepaid expenses and other	415,944	285,898
Total current assets	15,702,498	17,239,422
Intangible assets, net	3,250,917	3,512,542
Goodwill	3,275,403	3,275,403
Fixed assets, net	76,997	93,249
Deposits	104,906	102,906
Deferred offering costs	151,783	166,015
Total assets	\$ 22,562,504	\$ 24,389,537
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Liabilities		
Current liabilities:		
Accounts payable	\$ 847,137	\$ 637,321
Accrued liabilities	2,249,254	1,129,810
Common stock warrant liability	23,575,294	15,780,216
Deferred rent	24,136	2,673
Capital lease liability – current	3,953	4,865
Total current liabilities	26,699,774	17,554,885
Capital lease liability - long-term	9,778	1,811
Total liabilities	26,709,552	17,556,696
Commitments and contingencies		
Stockholders' equity (deficit):		
Preferred stock, \$0.001 par value, 15,000,000 shares authorized, zero shares issued and outstanding	-	-
Common stock, \$0.001 par value, 150,000,000 shares authorized 35,569,188 and 30,076,758 shares issued and outstanding as at August 31, 2011 and 2010, respectively	35,569	30,077
Additional paid-in capital	73,817,083	47,617,449
Accumulated other comprehensive income (loss)	1,904	(7,854)
Deficit accumulated during development stage	(78,001,604)	(40,806,831)
Total stockholders' equity (deficit)	(4,147,048)	6,832,841
Total liabilities and stockholders' equity (deficit)	\$ 22,562,504	\$ 24,389,537

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

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

	For the year ended August, 31		For the period from September 8, 2005 (inception) to August 31, 2011
	2011	2010	
Revenues:	\$-	\$-	\$ -
Operating expenses:			
General and administrative	6,177,635	3,720,148	16,854,023
Research and development	14,788,301	9,334,080	39,237,290
Total operating expenses	20,965,936	13,054,228	56,091,313
Loss from operations	(20,965,936)	(13,054,228)	(56,091,313)
Interest income	44,836	25,701	372,440
Interest expense	(2,243)	(3,950)	(116,130)
Foreign currency transaction gain (loss)	29,319	(457)	28,862
Adjustment to fair value of common stock warrants	(16,300,749)	(5,894,714)	(22,195,463)
Net loss	(37,194,773)	(18,927,648)	(78,001,604)
Other comprehensive income (loss)			
Foreign currency translation adjustment	9,758	(7,854)	1,904
Comprehensive loss	\$ (37,185,015)	\$ (18,935,502)	\$ (77,999,700)
Net loss per share:			
Basic and diluted	\$ (1.15)	\$ (0.85)	
Weighted average shares outstanding used to compute:			

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Basic and diluted	32,327,411	22,227,198
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The accompanying notes are an integral part of these consolidated financial statements.

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Raptor Pharmaceutical Corp.

(A Development Stage Company)

Consolidated Statements of Stockholders' Equity (Deficit)

For the period from September 8, 2005 (inception) to August 31, 2006

	Common stock		Additional	Receivable	Deficit		Total
	Shares	Amount	paid-in Capital	from stockholders	accumulated during the development stage		
Balance at September 8, 2005, issuance of common stock to founders at \$0.004 per share, net of retirement of common stock upon reverse merger	1,398,740	\$ 1,399	\$ 8,601	\$ (10,000)	\$ —		—
Common stock issued in May 2006 at \$0.43 per share pursuant to a stock purchase agreement dated February 2006	233,123	233	99,767	(100,000)	—		—
Common stock issued in May 2006 at \$0.86 per share pursuant to a stock purchase agreement dated February 2006	233,123	233	199,767	—	—		200,000
Common stock issued on May 25, 2006 at \$2.57 per share, net of fundraising costs of \$217,534	1,942,695	1,943	4,780,523	—	—		4,782,466
Common stock and warrants issued for a placement fee in connection with May 25, 2006 financing	186,499	186	(186)	—	—		—

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Common stock issued in connection with reverse merger in May 2006	2,914,042	2,914	(2,914)	—	—	—
Warrant subscribed pursuant to a consulting agreement dated September 2005	—	—	60	—	—	60
Consultant stock-based compensation expense	—	—	23,500	—	—	23,500
Repayment of receivable from stockholders	—	—	—	110,000	—	110,000
Net loss	—	—	—	—	(969,250)	(969,250)
Balance at August 31, 2006	6,908,222	\$ 6,908	\$ 5,109,118	\$	—\$ (969,250)	\$ 4,146,776

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

Raptor Pharmaceutical Corp.

(A Development Stage Company)

Consolidated Statements of Stockholders' Equity (Deficit)

For the year ended August 31, 2007

	Common stock		Additional	Deficit		Total
	Shares	Amount	paid-in Capital	accumulated during the development stage		
Balance at September 1, 2006	6,908,222	\$ 6,908	\$ 5,109,118	\$ (969,250)	\$	4,146,776
Exercise of common stock warrants	765,422	766	1,969,234	—		1,970,000
Exercise of common stock options	3,380	3	8,697	—		8,700
Consultant stock-based compensation expense	—	—	95,731	—		95,731
Employee stock-based compensation expense	—	—	368,978	—		368,978
Net loss	—	—	—	(3,632,076)		(3,632,076)
Balance at August 31, 2007	7,677,024	\$ 7,677	\$ 7,551,758	\$ (4,601,326)	\$	2,958,109

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

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Raptor Pharmaceutical Corp.

(A Development Stage Company)

Consolidated Statements of Stockholders' Equity (Deficit)

For the year ended August 31, 2008

	Common stock		Additional paid-in capital	Deficit accumulated during the development stage	Total
	Shares	Amount			
Balance at September 1, 2007	7,677,024	\$ 7,677	\$ 7,551,758	\$ (4,601,326)	\$ 2,958,109
Exercise of common stock warrants	747,938	748	1,924,252	—	1,925,000
Consultant stock-based compensation expense	2,040	2	240,227	—	240,229
Employee stock-based compensation expense	23,312	23	491,532	—	491,555
Issuance of common stock for loan placement fee	46,625	47	101,953	—	102,000
Issuance of common stock for the purchase of Convivia, Inc. assets	101,992	102	240,523	—	240,625
Issuance of common stock for the merger with Encode Pharmaceuticals, Inc.	802,946	803	2,657,197	—	2,658,000
Issuance of common stock and warrants for the sale of units in a private placement at \$2.14 per unit, including placement	4,662,468	4,662	9,051,273	—	9,055,935

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agent warrants, net of
fundraising costs of
\$944,065

Net loss	—	—	—	(8,053,963)	(8,053,963)
Balance at August 31, 2008	14,064,345	\$ 14,064	\$ 22,258,715	\$ (12,655,289)	\$ 9,617,490

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

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Raptor Pharmaceutical Corp.

(A Development Stage Company)

Consolidated Statements of Stockholders' Equity (Deficit)

For the year ended August 31, 2009

	Common stock		Additional	Deficit		
	Shares	Amount	paid-in	accumulated		Total
			Capital	during the		
				development		
				stage		
Balance at August 31, 2008	14,064,345	\$ 14,064	\$ 22,258,715	\$ (12,655,289)	\$	9,617,490
Exercise of common stock warrants	2,031,671	2,032	2,612,468	—		2,614,500
Consultant stock-based compensation expense	—	—	48,094	—		48,094
Employee stock-based compensation expense	23,312	23	354,471	—		354,494
Issuance of common stock and warrants for the sale of units in a private placement at \$1.37 per unit, including placement agent warrants, net of fundraising costs of \$293,724	1,738,227	1,739	2,090,538	—		2,092,277
Net loss	—	—	—	(9,223,894)		(9,223,894)
Balance at August 31, 2009	17,857,555	\$ 17,858	\$ 27,364,286	\$ (21,879,183)	\$	5,502,961

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

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Raptor Pharmaceutical Corp.
(A Development Stage Company)

Consolidated Statements of Stockholders' Equity (Deficit)
For the year ended August 31, 2010

	Common stock		Additional	Accumulated	Deficit	
	Shares	Amount	paid-in	other	accumulated	Total
			capital	comprehensive	during	
				loss	development	
					stage	
Balance at August 31, 2009	7,857,555	\$ 17,858	\$ 27,364,286	\$ —	\$(21,879,183)	\$ 5,502,961
Exercise of common stock warrants	196,736	197	474,822	—	—	475,019
Exercise of common stock options	37,881	38	63,984	—	—	64,022
Consultant stock-based compensation expense	—	—	78,327	—	—	78,327
Employee stock-based compensation expense	11,656	12	216,719	—	—	216,731
Common stock issued and warrants/options assumed with 2009 Merger	940,863	940	4,416,106	—	—	4,417,046
Issuance of common stock to LPC pursuant to an equity line facility at a \$2.26 average per share purchase price, net of fundraising costs and commitment shares totaling	2,386,895	2,387	4,839,407	—	—	4,841,794

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\$533,294

Issuance of common stock and warrants in a registered direct financing at \$2.00 per unit, including placement agent warrants, net of fundraising costs of \$1,246,658	3,747,558	3,748	6,243,062	—	—	6,246,810
Initial value of warrants issued in a registered direct financing	—	—	(1,863,615)	—	—	(1,863,615)
Issuance of common stock and warrants for the sale of units in a private placement at \$3.075 per unit, including placement agent warrants, net of fundraising costs of \$1,457,687	4,897,614	4,897	13,597,578	—	—	13,602,475
Initial value of warrants issued in 2010 private placement	—	—	(7,813,227)	—	—	(7,813,227)
Comprehensive loss	—	—	—	(7,854)	(18,927,648)	(18,935,502)
Balance at August 31, 2010	30,076,758	\$ 30,077	\$ 47,617,449	\$ (7,854)	\$ (40,806,831)	\$ 6,832,841

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

Raptor Pharmaceutical Corp.
(A Development Stage Company)

Consolidated Statements of Stockholders' Equity (Deficit)
For the year ended August 31, 2011

	Common stock		Additional paid- in capital	Accumulated other comprehensive Income (loss)	Deficit accumulated during development stage	Total
	Shares	Amount				
Balance at August 31, 2010	30,076,758	\$ 30,077	\$ 47,617,449	\$ (7,854)	\$ (40,806,831)	\$ 6,832,841
Exercise of common stock warrants	3,340,023	3,340	8,909,640	—	—	8,912,980
Exercise of common stock options	39,302	39	95,849	—	—	95,888
Consultant stock-based compensation expense	—	—	197,381	—	—	197,381
Employee stock-based compensation expense	—	—	1,919,604	—	—	1,919,604
Reclassification of the fair value of warrant liabilities upon exercise	—	—	8,505,671	—	—	8,505,671
Issuance of common stock to LPC pursuant to an equity line facility at a \$3.35 average per share purchase price, net of fundraising costs and commitment shares	2,113,105	2,113	6,571,489	—	—	6,573,602

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totaling \$174,202

Foreign currency translation gain	—	—	—	9,758	—	9,758
Net loss	—	—	—	—	(37,194,773)	(37,194,773)
Balance at August 31, 2011	35,569,188	\$ 35,569	\$ 73,817,083	\$ 1,904	\$ (78,001,604)	\$ (4,147,048)

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

	For the year ended August 31,		For the cumulative period
	2011	2010	from September 8, 2005 (inception) to August 31, 2011
Cash flows from operating activities:			
Net loss	\$ (37,194,773)	\$ (18,927,648)	\$ (78,001,604)
Adjustments to reconcile net loss to net cash used in operating activities:			
Employee stock-based compensation expense	1,919,604	216,731	3,351,362
Consultant stock-based compensation expense	197,381	78,327	683,322
Fair value adjustment of common stock warrants	16,300,749		