Raptor Pharmaceutical Corp
Form 10-K
November 14, 2012

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K
[X] ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended August 31, 2012

[ ] TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from _____ to _____

Commission file number 000-50720

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

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

9 Commercial Blvd., Suite 200, Novato, CA 94949
(Address of principal executive offices) (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 Global Market
Preferred Share Purchase Rights	

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

None

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

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

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

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of February 29, 2012 (the last business day of the registrant's most recently completed second quarter) was $329.0 million.

Indicate the number of shares outstanding of each of the registrant's classes of common stock, as of the latest practicable date: 51,753,696 shares common stock, par value $0.001, outstanding as of October 18, 2012.

The documents incorporated by reference are as follows:

None.

DOCUMENTS INCORPORATED BY REFERENCE

None.

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," "estimates," "potential," "opportunity" or the negative of these terms or other comparable terminology. All such statements, other than statements of historical facts, including our financial condition, future results of operations, projected revenues and expenses, business strategies, operating efficiencies or synergies, competitive positions, growth opportunities for existing intellectual properties, technologies, products, plans, and objectives of management, markets for our securities, and other matters, are about us and our industry that involve substantial risks and uncertainties and constitute forward-looking statements for the purpose of the safe harbor provided by Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Such forward-looking statements, wherever they occur, are necessarily estimates reflecting the best judgment of our senior management on the date on which they were made, or if no date is stated, as of the date of the filing made with the SEC in which such statements were made. You should not place undue reliance on these statements, which only reflect information available as of the date that they were made. Our business' actual operations, performance, development and results might differ materially from any forward-looking statement due to various known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled "Risk Factors" 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 U.S. federal securities laws and the rules and regulations of the SEC, we do not undertake any obligation and disclaim any intention to update or release publicly any revisions to these forward-looking statements after the filing of this 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, 2012, 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.

Unless otherwise mentioned or unless the context requires otherwise (e.g., our consolidated financial statements as of August 31, 2012, 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 "the Company," "we," "our," "us" "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 Raptor Pharmaceuticals Corp. (which was merged into us as of December 7, 2011), Raptor Discoveries Inc., or Raptor Discoveries, Raptor Therapeutics Inc., or Raptor Therapeutics, Raptor European Products, LLC, RPTP European Holdings C.V. and Raptor Pharmaceuticals Europe B.V., 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.

Overview

We are an emerging biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. Our initial focus is on developing our first product candidate, RP103 (delayed-release oral cysteamine bitartrate), as a potential treatment for nephropathic cystinosis, or cystinosis, a rare genetic disorder. Cystinosis patients are at very high risk of experiencing life-threatening metabolic disorders, including kidney failure, severe gastrointestinal dysfunction and rickets as a result of an accumulation of the amino acid, cystine, in cells. As a result, cystinosis patients have a substantially reduced life span relative to unaffected individuals.

In July 2011, we announced that RP103 had met the sole primary endpoint in our Phase 3 clinical trial designed to evaluate RP103 as a potential treatment for cystinosis. In the first quarter of calendar 2012, we submitted a New Drug Application, or NDA, to the U.S. Food and Drug Administration, or FDA, requesting approval to market RP103 as a potential treatment for cystinosis. The FDA granted Standard Review designation for RP103 and has assigned the user fee goal date (by which we anticipate a response from the FDA) of January 30, 2013. Also in the first quarter of calendar 2012, we submitted a Marketing Authorization Application, or MAA, to the European Medicines Agency, or EMA, requesting approval to market RP103 as a potential treatment for cystinosis.

In addition to cystinosis, we are also testing RP103 for the potential treatment of non-alcoholic steatohepatitis, or NASH, a metabolic liver disorder and Huntington's Disease, or HD, a neurodegenerative disorder.

Clinical Development Programs

Our three active clinical development programs utilize the same active pharmaceutical ingredient cysteamine bitartrate. Cysteamine bitartrate was approved in 1994 as an orally available immediate-release powder in a capsule for the treatment of, and is the current standard of care for cystinosis. We are reformulating cysteamine bitartrate to potentially improve the dose administration, safety and/or efficacy compared to existing treatment and repurposing cysteamine bitartrate for potential applications in new disease indications. Our proprietary delayed-release formulation, RP103, is a capsule containing enteric coated micro-beads of cysteamine bitartrate. We believe RP103 will require less frequent dosing and could reduce gastro-intestinal and other side effects compared to immediate-release cysteamine bitartrate for cystinosis patients. In addition to cystinosis, we are also testing RP103 for the potential treatment of NASH and HD. RP104 is our enteric coated tablet formulation of cysteamine bitartrate in development. We obtained the exclusive worldwide license to delayed-release cysteamine, which is the basis for our proprietary formulations of cysteamine, through the 2007 merger of our clinical subsidiary and Encode Pharmaceuticals, Inc., or Encode, formerly a privately-held product development company.

RP103 for Cystinosis

Cystinosis is a rare, inherited error of metabolism that results in toxic cystine accumulation in all cells. Cystine accumulation causes widespread tissue and vital organ failure and death in late childhood if left untreated. Cystinosis is usually diagnosed in the first year of life after children present with symptoms including markedly increased

urination, thirst, dehydration, gastrointestinal distress, failure to thrive, rickets, photophobia and specific kidney symptoms (Fanconi syndrome). If patients survive to adolescence, they suffer from kidney malfunction, muscle wasting, myopathy, difficulty swallowing, respiratory problems, diabetes and hypothyroidism.

Normal protein turnover is absent in cystinosis patients, resulting in continuous cystine intracellular accumulation, which requires constant cystine depletion through aggressive therapeutic intervention. Studies have shown that cystine depleting therapy may delay and even prevent kidney transplant and lessen other clinical manifestations of the disease. The goal of cystine depletion therapy for cystinosis is to reduce cystine levels in cells to below toxic levels. Immediate-release cystine depleting treatment (cysteamine bitartrate), or Cystagon®, is the current standard of care. Cystagon has been available since 1994 in the U.S. and 1997 in EU, but requires strict adherence to an every 6-hour dosing schedule (including a middle-of-the-night dose) to maintain adequate therapeutic drug levels. Patients and care givers struggle to comply with the frequency of dosing and the drug side effects, which include severe gastrointestinal distress (nausea, vomiting) and an exhaled rotten egg smell and body odor.

In a recent survey of 37 cystinosis patients and caregivers conducted at the June 2011 Cystinosis Research Foundation (CRF) conference, 63% of patients rated the burden of nighttime dosing a 9 on a scale from 1 to 10 (10 being the worst burden). The requirement for middle-of-the-night dosing is the most significant compliance burden noted by patients. Inadequate disease control resulting from skipping this dose was the subject of a major publication (Levtchenko, Pediatr Nephrol 2006). In addition to the Cystagon dosing challenges, side effects associated with immediate release cysteamine treatment include gastrointestinal distress, nausea and vomiting, beyond those normally experienced as a result of the disease itself, and a socially difficult exhaled rotten egg smell and body odor soon after drug administration, which is especially a burden for children in the middle of the school day. Patients report frequent, concomitant and chronic use of proton-pump inhibitors, or PPIs, to reduce the gastrointestinal distress. We believe that the required dosing regimen coupled with these adverse side effects results in overall poor patient compliance within the cystinosis patient population, with approximately 70% to 80% of patients failing to comply with prescribed dosing.

Patients now in their 20's that have adhered strictly to cysteamine therapy from early diagnosis demonstrate that this therapy results in slowing the progression of disease to the point of delaying or potentially negating the need for a kidney transplant as well as reducing damage to other organs. Suboptimal drug handling and variable dose administration routines, the strict requirement of every 6 hour dosing, and unpleasant side effects that in some cases require temporary dosing suspensions, have all contributed to poor overall therapeutic disease control currently seen in the cystinosis population.

We are developing RP103 to address the compliance issues associated with Cystagon. Early reformulation clinical work supported by funding from cystinosis foundations and performed by treating physicians in cystinosis clinics at UCSD and other medical institutions worldwide highlights the need to address these tolerability and compliance problems by the cystinosis community. The primary goal of RP103 is to reduce the dosing frequency from once every 6 hours to once every 12 hours thereby eliminating the middle-of-the-night and the middle-of-the-school-day doses. We believe that via a reduced frequency dosing regimen, patients and their caregivers will be better able to adhere to the prescribed dosing schedule, and with improved adherence, patients and caregivers will be able to have a full uninterrupted night's sleep. Additionally parents and schools will not have to arrange for drug administration during school hours. We also believe RP103 will improve gastrointestinal tolerability potentially resulting in reduced PPI use, and in many patients, lessening of the rotten egg smell in breath and body odor.

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

Of the 43 patients randomized, 41 patients completed the Phase 3 protocol, of which 38 were included in the evaluable "per protocol" data set. Three patients not compliant with Cystagon dosing while on the Cystagon arm of the study, were dropped from the "per protocol" data set. The age range of study participants was 6 to 26 years, with 87% of patients below 16 years of age. On average, the peak WBC cystine level measured in patients treated with Cystagon was 0.54 +/- 0.05 nmol ½ cystine/mg protein, compared to an average peak 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). The pre-specified non-inferiority endpoint of the clinical trial would be achieved when the upper end of the confidence interval around the mean difference of WBC cystine levels did not exceed an absolute value of 0.3. The upper end of the confidence interval in the Phase 3 clinical trial was determined to be 0.16, thus achieving the non-inferiority endpoint.

In addition, RP103 patients in the study received a lower average daily dose compared to their baseline Cystagon dose. The starting dose of RP103 for patients in the Phase 3 clinical trial was initially set at 70% of their established dose of Cystagon. The protocol allowed for a single RP103 dose increase of 25%, based on intermediate WBC cystine results, to reflect the current standard of care in establishing appropriate dosing of Cystagon in cystinosis patients. 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, with one third of patients at 70% of their baseline Cystagon dose.

Extension Study. All patients who completed our pivotal Phase 3 clinical trial of RP103 for the potential treatment of cystinosis could voluntarily enroll in a planned extension study in which they would continue to be treated with RP103 and make regular clinic visits to monitor WBC cystine levels and collect long-term safety and quality of life data. Of the 40 patients who entered the extension study after completing the Phase 3 clinical trial, 38 are currently still enrolled. These 38 patients have now taken RP103 in the extension study for at least 15 months, with some patients having been in the extension study for as long as 27 months. We included a minimum of 12 months of safety data from the 38 Phase 3 completers who elected to enroll in the extension study with our NDA and MAA filings. We plan to keep the extension study open to all enrolled patients until RP103 becomes locally commercially available. Based on meeting the primary endpoint in our Phase 3 clinical trial and on the findings of our RP103 bioequivalence study, which demonstrated similar drug exposure whether administered in whole capsule or sprinkled onto applesauce, U.S. and EU regulatory agencies approved our expanded enrollment in the extension study to include patients who did not qualify for the Phase 3 clinical trial. Eighteen additional patients have enrolled, including 13 infants and children under six years old using RP103 sprinkled onto applesauce or administered through gastric tube, and 5 patients who have undergone a kidney transplant. Fifty-six cystinosis patients are currently taking RP103 treatment under a clinical protocol.

NDA/MAA Submission. Based on meeting the primary endpoint and other positive clinical data from our pivotal Phase 3 clinical study, the extension study and bioequivalence studies, we submitted applications for marketing approval of RP103 for the potential treatment of cystinosis with both the FDA and the European Medicines Agency, or EMA. In March 2012, the EMA validated our MAA for RP103 for the potential treatment of cystinosis. Validation of the MAA confirmed that the submission is sufficiently complete for the EMA to begin its formal review process. In June 2012, the FDA accepted for filing our RP103 NDA. The FDA granted Standard Review designation for RP103 and has assigned the user fee goal date (by which we anticipate a response from the FDA) of January 30, 2013. Future milestones payments of $500,000 and $750,000 will be payable to UCSD if the MAA and NDA for cystinosis are approved, respectively.

Preparation for Potential Commercial Launch. In anticipation of approval of RP103, we have begun preparing for launch of RP103 for the potential treatment of cystinosis in both the U.S. and the EU. In September 2012, we announced the appointment of Julie Anne Smith as our Executive Vice President, Strategy, and Chief Operating Officer, who will oversee the preparations of our pending launch in both the U.S. and the EU, along with managing the development of general corporate strategy.

Our near term launch goal is to rapidly convert cystinosis patients unsatisfied with or uncontrolled under their current cysteine depleting therapy to RP103, in accordance with all applicable local regulations and labeling. We anticipate FDA approval in the U.S. prior to EMA approval and subsequent introduction in certain EU countries. The EMA has approved, and the FDA has provisionally approved, the name PROCYSBI™ as our branded name for RP103 for the potential treatment of cystinosis. Regulatory and launch strategy for other international markets is being planned.

In the U.S. and EU, several key legal structures and operational activities have been or are being established. These include creating the EU legal entity and subsidiary structure, defining distribution strategies, and hiring personnel, including a European general manager of commercial operations, selected country managers, medical affairs staff and sales/marketing representatives. We anticipate additional hires before the potential launch of RP103. Several key pricing and reimbursement support activities are complete or underway including updating payor research, development of the global value dossier and establishment of a U.S. reimbursement hub, United BioSource Corporate, or UBC. Commercial demand planning and launch inventory build is underway. Our goal for commercial inventory

at launch is to have on hand sufficient drug quantities to meet a best-case demand scenario. FDA and EMA have granted conditional approval of our trademark PROCYSBI™ (delayed-release enteric coated microbead cysteamine bitartrate capsules).

The reformulation of cysteamine, started at UCSD and continued at our Company, has been a highly visible program in the cystinosis community for nearly a decade. We have been working with rare disease and cystinosis patient advocacy organizations in both the U.S. and the EU to establish a positive response to RP103's market introduction if it receives regulatory approval. Our medical team has been evaluating cystinosis disease and diagnostic awareness amongst potential treating physicians, identifying current Cystagon prescribers and evaluating potential future clinical studies to improve long term patient management and treatment with RP103. UBC has begun registering U.S. cystinosis patients interested in potential RP103 treatment and future assistance with benefits adjudication, co-pays, and disease and product information. The goal of early patient education and benefits investigation is to speed patient conversion from existing cystine-depleting therapy to RP103 treatment, if approved, in accordance with all local regulations and labeling.

RP103 for Huntington's Disease

Huntington's Disease is a rare hereditary condition caused by a defective gene. This gene makes an abnormal protein which leads to the degeneration of certain nerve cells in the brain. Adult-onset HD, the most common form of this disorder, usually appears in patients who are in their early 30's or 40's.

In 2008, we received FDA orphan drug designation for cysteamine formulations, including RP103, for the potential treatment of HD. We plan to apply for orphan drug designation in the EU pending clinical data.

The treatment options for HD patients are very limited with no drugs that address the underlying pathophysiology. Drugs that are available only help minimize certain of the disease symptoms such as the uncontrollable movements and mood swings associated with HD. HD patients are believed to be deficient in brain-derived neurotrophic factor, or BDNF. In preclinical studies, cysteamine has shown the potential to slow the progression of HD by increasing the levels and intracellular transport of BDNF in mice and non-human primates.

Centre Hospitalier Universitaire, or CHU, d'Angers, France, is currently conducting a Phase 2/3 clinical trial of RP103 designed to investigate potential mechanism of cysteamine in HD patients, using BDNF as a biomarker of potential efficacy. The trial commenced in October 2010, with full enrollment in June 2012. Eight clinical sites in France have enrolled 96 patients in a placebo-controlled, 18-month trial, followed by an open label trial with all placebo patients rolling onto RP103 and all non-placebo patients continuing on RP103 for up to another 18 months. The primary endpoint of the trial is change from baseline of the Unified Huntington's Disease Rating Scale, or UHDRS. Blood levels of BDNF are being measured as a secondary endpoint. Under the collaboration agreement with CHU d'Angers, we supply RP103 and placebo capsules for the clinical trial and open-label extension study in exchange for regulatory and commercial rights to the clinical trial results. Clinical expenses of the study are covered by a grant from the French government. Interim results of this study following 18 months of treatment are expected to be announced in the first half of calendar 2014.

RP103 for NASH

NASH, an advanced form of Non-alcoholic Fatty Liver Disease, or NAFLD, is a progressive liver disease, occurring in 25% of obese people. Approximately 2% to 5% of the U.S. population is afflicted with this disease, which can cause cirrhosis, liver failure and end-stage liver disease. The incidence of NASH is increasing in the U.S. adolescent population. Currently, we are not aware of any therapeutic options for NASH. The disease is generally managed, if at all, with lifestyle changes such as diet, exercise and weight reduction.

We believe cysteamine may operate in a number ways for the potential treatment of NASH. First, cysteamine is a potent anti-oxidant, and dietary anti-oxidants, like vitamin E, have been clinically tested in NASH studies. While the endpoint of this study was not achieved, the study provided useful data. Second, cysteamine is a precursor of the potent endogenous liver anti-oxidant glutathione, or GSH, and increasing GSH may have the potential to reverse NASH-related liver damage. GSH itself does not enter easily into cells, even when given in large doses. However, GSH precursors, such as cysteamine, enter into cells and have been shown to be effective in the treatment of certain conditions by preventing significant GSH depletion. Third, cysteamine has been shown to inhibit tissue transglutaminase activity, which is elevated in NASH, and may contribute to the formation of fibrotic tissue associate with advanced NASH.

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

The Phase 2a trial results were consistent with ALT and AST reductions seen in patients that achieve a 10% weight loss, although body Mass Index did not change significantly during both the treatment and post-treatment phases in our Phase 2a clinical trial. In this Phase 2a clinical trial, the prototype of RP103 demonstrated a favorable safety profile, with mean gastrointestinal symptom scores of 1.1 (the maximum score of 14 indicates the most severe gastrointestinal symptoms) at baseline and 0.7 after 6 months of treatment.

In June 2012, we announced the dosing of a first patient in our Phase 2b juvenile clinical trial evaluating the safety and efficacy of RP103 as a potential treatment of NASH. The clinical trial is being conducted pursuant to a Cooperative Research and Development Agreement, or CRADA, executed in December 2011 with the National Institute of Diabetes and Digestive and Kidney Diseases, or NIDDK, part of the National Institutes of Health.

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

Other Clinical-Stage Product Candidate

Convivia™ for ALDH2 Deficiency

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

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

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

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

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 collaborations. 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.

Corporate Information

We are incorporated under the laws of the State of Delaware and our business was founded in May 2006. 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 18, 2012, there were 51,753,696 shares of our common stock outstanding. Our common stock currently trades on the NASDAQ Global Market under the ticker symbol "RPTP."

Corporate History

In July 2009, our subsidiary merged with and into Raptor Pharmaceuticals Corp., a Delaware corporation, or RPC, pursuant to which the stockholders of RPC received shares of our common stock in exchange for their shares of stock and RPC survived as our wholly-owned subsidiary. This merger is referred to herein as the 2009 Merger.

Immediately prior to the 2009 Merger, we changed our corporate name from "TorreyPines Therapeutics, Inc." to "Raptor Pharmaceutical Corp." At the time of the 2009 Merger, RPC had two principal subsidiaries, Raptor Discoveries, a development-stage research and development company, and Raptor Therapeutics, which focuses on developing clinical-stage drug product candidates through to commercialization and, in connection with the 2009 Merger, these two subsidiaries became our subsidiaries. Due to the amount of our stock issued to the stockholders of RPC in the 2009 Merger, RPC was treated as the "accounting acquirer" in the merger, and its board of directors and officers manage and operate the combined company. In December 2011, we merged RPC with and into us and it has ceased to exist as a separate company. TorreyPines Therapeutics was incorporated in July 1997 under the name "Axonyx, Inc." and RPC was incorporated in May 2006 under the name "Highland Clan Creations Corp."

Exercises of Common Stock Options and Common Stock Warrants

During the cumulative period from September 8, 2005 (inception) through October 18, 2012, we received approximately $20.9 million from the exercise of warrants in exchange for the issuance of an aggregate of 8.9 million shares of our common stock.

During the cumulative period from September 8, 2005 (inception) through October 18, 2012, we received $0.7 million from the exercise of stock options resulting in the issuance of approximately 0.3 million shares of our common stock.

Outstanding Common Stock Warrants

As of October 18, 2012, 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 (in thousands)	Exercise price	Expiration Date
Issued in connection with Encode merger	233	$ 2.87	12/13/2015
Issued to placement agents in May / June 2008	433	$ 2.36	5/21/2013
Issued to placement agents in August 2009	65	$ 1.50	8/21/2014
TorreyPines warrants assumed in 2009 Merger	8	$ 80.86 *	6/11/2013 - 9/26/2015
Issued to registered direct investors in Dec. 2009	756	$ 2.45	12/22/2014
Issued to private placement investors in Aug. 2010	3,595	$ 3.075	8/12/2015
Issued to placement agent in Aug. 2010	98	$ 3.075	8/12/2015
Total warrants outstanding	5,188	$ 3.02 *	

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

The closing of this private placement occurred on August 12, 2010. We issued and sold an aggregate of 4,897,614 units, comprised of 4,897,614 shares of common stock and warrants to purchase up to 4,897,614 shares of our common stock for gross proceeds of approximately $15.1 million. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share. As the placement agent for the 2010 Private Placement, the Placement Agent was issued one warrant to purchase 97,952 shares of our common stock, was paid a cash commission of $978,911 and was reimbursed for certain of its expenses incurred in connection with the 2010 Private Placement. As of October 18, 2012, warrants to purchase 1.3 million shares were exercised for aggregate gross proceeds to us of approximately $3.9 million. The balance of warrants to purchase 3.7 million shares of our common stock remain outstanding as of October 18, 2012.

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.

Issuances of Common Stock in Connection with an At-the-Market Common Stock Sales Program

On April 30, 2012, we entered into an "At-the-Market" ("ATM") Sales Agreement, with Cowen and Company, LLC, or Cowen, under which we may, at our discretion, sell our common stock with a sales value of up to a maximum of $40 million through ATM sales on the NASDAQ Stock Market. Cowen acts as sole sales agent for any sales made under the ATM for a 3% commission on gross proceeds. The common stock is being sold at prevailing market prices at the time of the sale of common stock, and, as a result, prices will vary.

Sales in the ATM offering are being made pursuant to the prospectus supplement dated April 30, 2012, which supplements our prospectus dated February 3, 2012, filed as part of the shelf registration statement that was declared effective by the SEC on February 3, 2012. Through October 18, 2012, we sold approximately 2.7 million shares under the ATM at a weighted-average selling price of $5.05 per share for net proceeds of approximately $13.4 million.

Proprietary Rights

IP Protection for RP103 for Cystinosis and Other Indications

Our composition and method of use patents.

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

Our cysteamine intellectual property to treat metabolic and neurodegenerative conditions.

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

In May 2012, we acquired exclusive rights to U.S. patent application 13/277,942 related to cysteamine and related compounds in the potential treatment of parasitic diseases, including malaria, from McGill University, or McGill, in Montreal, Canada. The McGill patent covers the use of cysteamine and related compounds in the potential treatment of malaria in combination with artemisinin, the current standard of care. Researchers at McGill reported that, in mouse models of malaria, the combination reduced parasite levels in red blood cells and improved survival rates compared to artemisinin alone.

In June 2012, we acquired exclusive rights to international patent application PCT/CA 2012/050106, related to cysteamine and related compounds for the potential treatment of Parkinson's Disease from Université Laval, or Laval, Quebec, Canada. Our agreement with Laval provides exclusive rights to technology related to the use of cysteamine and related compounds to potentially modify the progression of Parkinson's Disease. Researchers at Laval reported that administration of cystamine (an oxidized form of cysteamine) in an animal model of Parkinson's Disease showed signs of preventing neuron loss and rescuing neurons undergoing a degenerative process. Signs of restoration of neuronal loss and partial reversal of behavioral impairments were also observed.

In July 2012, we announced the issuance of European Patent 1919458, which 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 twice daily dosing in the treatment of cystinosis. The European patent term will expire in January 2027.

In September 2012 we acquired exclusive world-wide rights to international patent application PCT/US11/57935, related to cysteamine and related compounds in the potential treatment of tissue fibrosis from the Seattle Children's Research Institute, or SCRI. Researchers at SCRI demonstrated in preclinical studies in mice that daily treatment with cysteamine attenuated renal fibrosis, with up to 25% reduction of extracellular fibrotic material observed over a 21-day study period.

Regulatory Exclusivity

Orphan Drug Designation

We have been granted Orphan Drug Designation from the FDA for use of RP103 to potentially treat cystinosis and the use of cysteamine to potentially treat HD and Batten Disease (although we are not currently working on the development of a drug product candidate for Batten Disease). The Orphan Drug Act of 1983 generally provides incentives, including marketing exclusivity and tax benefits, to companies that undertake development and marketing of products to treat relatively rare diseases, which are defined as diseases for which fewer than 200,000 persons in the U.S. would be likely to receive the treatment. A drug that receives orphan drug status may receive up to seven years of exclusive marketing in the U.S. for that indication. Our RP103 may receive orphan drug exclusivity if the Office of Orphan Products determines that our enteric formulation of cysteamine bitartrate has demonstrated comparable efficacy and is safer than the approved formulation.

RP103 has also been granted Orphan Drug Designation by the EMA. Equivalent European regulations may give ten years of marketing exclusivity for cystinosis in Europe.

Hatch-Waxman

Under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, our enteric formulation of RP103 is eligible for a 3-year regulatory exclusivity period as a reformulated version of a previously approved drug substance for which clinical studies that are essential for approval have been conducted.

Competition

Cystinosis

We are aware of only one pharmaceutical product currently approved by the FDA and the EMA to treat cystinosis, Cystagon (immediate-release cysteamine bitartrate capsules), is marketed in the U.S. by Mylan Pharmaceuticals, and by Recordati and Orphan Europe in markets outside of the U.S. Cystagon was approved by the FDA in 1994 and by 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 is reduced dose frequency and improved tolerability, if we receive marketing approval, we anticipate that Cystagon will remain on the market and will 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 (toxic cystine accumulation). Academic researchers in the U.S. and Europe are pursuing potential cures for cystinosis through gene therapy, stem cell therapy, pro-drug and pegylated drug approaches as alternatives to cysteamine bitartrate. We believe that the development timeline to an approved product for these approaches is many years with substantial uncertainty.

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, RP103, is the only compound in clinical development which specifically targets the fundamental metabolic defect of the disease

(deficient brain-derived neurotrophic factor), 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, Cystadane from RDT, Moexipril® from Univasc), insulin sensitizing agents (Actos® from Takeda Pharmaceuticals, 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 Bristol Myers Squib, in an ongoing Phase 2/3 study for NASH; and siliphos, or milk thistle, in a UCSD Phase 2 study for NASH.

ALDH2 Deficiency

We are not aware of any pharmaceutical products currently approved for ALDH2 deficiency, either in the U.S. or internationally. However, given the size of the potential patient population and the emerging awareness of this disorder as a serious health risk, we expect there are or will be other pharmaceutical companies, especially those with commercial operations in Asian countries, developing products to treat the symptoms of this condition. 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.

Government Regulations of the Biotechnology Industry

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

In particular, human therapeutic products are subject to rigorous preclinical and clinical testing and other approval procedures of the FDA and similar regulatory authorities in other countries. Various federal statutes and regulations also govern or influence testing, manufacturing, safety, labeling, storage, and record-keeping related to such products and their marketing. The process of obtaining these approvals and the subsequent compliance with the appropriate federal statutes and regulations requires substantial time and financial resources.

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

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

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

- completion of prerequisite 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 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 may result in increased costs and delays. 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 U.S., the drug product candidate may be exported for sale outside of the U.S. only if it has been approved in any one of the following: the European Union, Canada, Australia, New Zealand, Japan, Israel, Switzerland and South Africa. Specific FDA regulations govern this process.

In addition to the regulatory framework for product approvals, we and our collaborative partners must comply with federal, state, and local laws and regulations regarding occupational safety, laboratory practices, the use, handling and disposition of radioactive materials, environmental protection and hazardous substance control, and other local, state, federal and foreign regulations. All facilities and manufacturing processes used by third parties to produce our drug candidates for clinical use in the U S 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 or of manufacturing and sale of any of our products approved for sale. 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 U.S. 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 have an active research and development effort. 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) to August 31, 2012, we incurred approximately $60.7 million ($21.4 million, $14.8 million and $9.3 million for the years ended August 31, 2012, 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, 2012.

Operating Segment and Geographic Information

Operating segments are components of an enterprise for which separate financial information is available and are evaluated regularly by a company in deciding how to allocate its resources and to assess its performance. We have reviewed how we allocate our resources and analyze performance worldwide and have determined that, because employees work on integrated programs in the U.S. and in Europe, encompassing all stages of product development and commercialization, we operate in one segment.

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 36 full time employees and 2 part-time employees. Of the 38 employees, 22 are general and administrative (which includes 11 employees who are focused on preparation for the potential launch of RP103 in the U.S. and the EU) and 16 are involved in research and development. Based on our current plan, over the next 12 month period, we plan to add approximately 15 to 25 people in the following functions: field based sales and medical affairs, commercial, regulatory, clinical, manufacturing, program management, quality and finance. In addition, administrative, regulatory, clinical, commercial and human resources consultants will be used as appropriate.

Recent Executive Appointments

In September 2012, we hired a new Chief Financial Officer (principal financial officer and principal accounting officer), Secretary and Treasurer, Georgia Erbez, replacing Kim R. Tsuchimoto, who had served in these capacities from May 2006 to September 2012. Ms. Tsuchimoto remains as our VP, Finance.

Also in September 2012, we appointed Julie A. Smith as our Executive Vice President, Strategy, and Chief Operating Officer. Ms. Smith is responsible for directing our commercial, manufacturing, and program management organizations, and providing leadership in corporate and strategic development initiatives.

Facilities

Our primary offices are located at 9 Commercial Blvd., Suite 200, Novato, CA 94949. Our main phone number is (415) 382-8111 and our facsimile number is (415) 382-8002.

Website

Our website is located at www.raptorpharma.com. Any information contained in, or that can be accessed through, our website is not incorporated by reference into, nor is it in any way a part of, this Annual Report on Form 10-K.

Available Information

We are subject to the reporting requirements under the Exchange Act. Consequently, we are required to file reports and information with the SEC, including reports on the following forms: annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act. These reports and other information concerning us may be accessed through the SEC's website at www.sec.gov and on our website at www.raptorpharma.com. Such filings are placed on our website as soon as reasonably practicable after they are filed with the SEC. Information contained in, or that can be accessed through, our website is not part of this Annual Report on Form 10-K.

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 Associated with Product Development and Commercialization

We currently depend entirely on the success of our lead compound, RP103. We may not receive marketing approval for, or successfully commercialize, RP103 for any indication.

We currently have no drug products for sale. We are not permitted to market our lead compound, RP103, in the U.S. or any other market until we obtain necessary regulatory approvals. To market a new drug in the U.S., we must submit to the FDA and obtain FDA approval of an NDA for each individual disease indication. To market a new drug in Europe, we must submit to EMA or relevant regulatory authority in the designed Reference Member State and obtain approval of, an MAA for each individual disease indication. An NDA or MAA must be supported by extensive clinical and preclinical data, as well as extensive information regarding chemistry, manufacturing and controls, to demonstrate the safety and effectiveness of the applicable product candidate for the treatment of each individual disease indication.

In March 2012, we submitted an NDA to the FDA and an MAA to the EMA seeking approval to market our investigational drug candidate, Cysteamine Bitartrate Delayed-release Capsules (RP103) for the potential treatment of cystinosis. The FDA has assigned the user fee goal date of January 30, 2013 for the RP103 NDA. Our MAA for RP103 is under review by the EMA. We anticipate a decision from the EMA in the first half of calendar 2013. There is no assurance that we will obtain regulatory approval for RP103 for the potential treatment of cystinosis in the U.S. or the EU.

We have additional product development programs in the clinical testing stage for the use of RP103 in HD and in NASH. These product development programs have not advanced to the stage of a submission for marketing approval to the FDA or EMA or to any other regulatory body in any other jurisdiction.

Obtaining approval of an NDA or MAA or any other filing for approval in a foreign country, is an extensive, lengthy, expensive and uncertain process. The FDA or other regulatory authority may reject a filing or delay, limit or deny approval of RP103 for many reasons, including:

- the results of clinical trials may not meet the level of statistical significance or clinical significance required by the FDA and regulatory authorities outside the U.S. for approval;
- the FDA or regulatory authorities outside the U.S. may disagree with the number, design, size, conduct or implementation of our clinical trials; may not find the data from preclinical studies and clinical trials sufficient to demonstrate that RP103 has adequate clinical and other benefits and an adequate safety profile; or may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct one or more additional trials;
- the FDA or regulatory authorities outside the U.S. may not accept data generated at our clinical trial sites;
- the FDA may have difficulties scheduling an advisory committee meeting in a timely manner or the advisory committee may recommend against approval of our application or may recommend that the FDA require, as a condition of approval, additional preclinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;
- the FDA or regulatory authorities outside the U.S. may require additional preclinical or clinical studies or other data prior to granting approval, and we may not be able to generate the required data on a timely basis if at all;
- the FDA or regulatory authorities outside the U.S. may impose limitations on approved labeling of RP103 thus introducing reimbursement complications which may limit access for intended users;

- the FDA or EMA may identify deficiencies in the manufacturing processes or in the facilities of our third party contract manufacturers, or may require us to manufacture additional registration batches or change our process or specifications;
- we may not be able to validate our manufacturing process to the satisfaction of the FDA or EMA, or the FDA may not agree with our plan for concurrent validation; or
- the FDA or regulatory authorities outside the U.S. may change approval policies or adopt new regulations.

Despite regulatory guidelines, we cannot reliably predict if or when any of the drug product candidates we are developing or intend to develop will be approved for marketing. If we fail within a reasonable time period to gain approval for our lead drug product program, RP103 for the potential treatment of cystinosis, our financial results and financial condition will be adversely affected. In such a case, we will have to delay or terminate some or all of our research product development programs and may be forced to dramatically restructure or cease operations.

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.

Any regulatory approvals that we obtain for our product candidates will also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval. In addition, if the FDA approves a product candidate, the manufacturing processes, labeling, packaging, distribution, storage, adverse event reporting, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements will include submissions of safety and other post-marketing information and reports, ongoing maintenance of product registration, as well as continued compliance with cGMPs (good manufacturing practices) or GCPs (good clinical practices), and GLPs (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.

If we are unable to successfully commercialize RP103 for the potential treatment of cystinosis, if approved, or experience significant delays in doing so, our business will be materially harmed.

Our strategy is to build a biopharmaceutical company focused on the development of RP103 in multiple indications (with testing of additional applications of RP103) and a robust pipeline of other candidate compounds. We anticipate that, for at least the next several years, our ability to generate revenues will depend in large part upon U.S. and EU regulatory approval and successful commercialization of RP103 for the potential treatment of cystinosis, given that our other product candidates are currently in clinical or preclinical development. The successful commercialization of RP103 will depend on several factors, including:

- approval of RP103 for the potential treatment of cystinosis by applicable regulatory authorities;
- the successful launch of RP103 for the potential treatment of cystinosis in the U.S., the EU and other selected territories throughout the economically developed world;
- identification of potential physician prescribers and potential patients for, and obtaining sales of RP103 for the potential treatment of cystinosis;
- effective communication of the relative safety and efficacy of RP103 compared to competitive products or therapeutic alternative regimes
- obtaining acceptance of RP103 for the potential treatment of cystinosis by physicians, parents, patients and cystinosis research/advocacy organizations;
- obtaining and maintaining appropriate reimbursement for RP103 for the potential treatment of cystinosis from commercial health plans and government health programs, which we refer to collectively as third-party payors;
- maintaining compliance with regulatory requirements;
- provision of affordable out-of-pocket cost to patients and/or other programs to ensure patient access to RP103;
- approval by the FDA and other regulatory agencies of appropriate product labeling for RP103;
- establishing and maintaining agreements with wholesalers and distributors on commercially reasonable terms;
- manufacture and supply of adequate supplies of RP103 to meet commercial and clinical demand;
- development and maintenance of intellectual property protection for RP103 for the potential treatment of cystinosis and obtaining favorable drug listing treatment by the FDA to minimize generic competition; and
- execution of robust pre-launch, commercial launch and medical affairs activities in support of marketing and sales requirements.

If we fail to successfully commercialize RP103 for the potential treatment of cystinosis, if approved, at sufficient sales levels, we will be unable to generate sufficient sales revenue to sustain or grow our business and we may never become profitable, and our business, financial condition and results of operations will be materially affected.

If approved for marketing, pressure on drug product third-party coverage and reimbursement/pricing may impair our ability to be reimbursed for our products, at prices or on terms sufficient to provide a viable financial outcome. Market acceptance and sales of any product candidates that we may develop will depend in large part on global reimbursement policies and may be affected by future healthcare reform measures, both in the U.S. and other key international markets. The continuing efforts of governmental and third-party payors to contain, reduce or shift the costs of healthcare through various means may harm our business. Successful commercialization of our products will depend in part on the availability of governmental and third-party private payor reimbursement for the therapeutic value and price of our products. For example, in some foreign markets, the pricing or profitability of healthcare products is subject to

government control. In the U.S., there has been, and we expect there will continue to be, a number of federal and state proposals to implement similar government price control. If any of our product candidates become marketable, the implementation or even the announcement of any of these legislative or regulatory proposals or reforms could harm our business, by reducing the prices we are able to charge for our products, impeding our ability to achieve profitability, raise capital or form collaborations. In particular, in the U.S., private health insurers and other third-party payors often follow the coverage and reimbursement policies of government payors, including the Medicare or Medicaid programs. In many European countries, patients are unlikely to use prescription drugs that are not reimbursed by their governments. Further, the increased emphasis on managed healthcare in the U.S. and on country and regional pricing and reimbursement controls in the EU will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our product sales, and results of operations. In the U.S., EU and other significant or potentially significant markets for our product candidates, government authorities and third-party payors 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. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, drug coverage and reimbursement policies and pricing in general. For our product candidates, we will not know what the reimbursement rates will be until we obtain regulatory approval and then launch and enter into payor negotiations. If we are unable to obtain sufficiently high reimbursement rates for our products, they may not be commercially viable.

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

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

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

If RP103 for the potential treatment of cystinosis does not receive significant market acceptance among physicians, patients, healthcare payors or the healthcare community, our ability to generate revenues from this drug product will be severely affected.

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

Our clinical development of RP103 target diseases with small patient populations, including cystinosis and HD. A key component of the successful commercialization of a drug product for these indications includes identification of patients and a targeted prescriber base for the drug product. If we are successful in obtaining regulatory approval to

market RP103 for a disease with a small patient population and, in the case of HD, successful in developing this product candidate for that indication, we will need to identify patients and market RP103 for these indications in the U.S. and Europe, at a minimum, to achieve significant market penetration. In addition, the per-patient prices at which we sell RP103 for these indications will need to be relatively high in order for us to generate an appropriate return for the investment in these product development programs and achieve meaningful profitability. There can be no assurance that we will be successful in identifying patients and/or obtaining high per-patient prices for our product candidates that target diseases with small patient populations.

Even if we obtain regulatory approval for our product candidates, we will be subject to ongoing regulatory obligations, oversight and continued regulatory review, which may result in significant additional expense.

If we receive approval for any of our products, such approvals could contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials and extraordinary requirements for surveillance to monitor the safety and efficacy of the product candidate. Post-marketing studies and/or post-market surveillance may suggest that a product causes undesirable side effects which present an increased risk to the patient. If data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is delayed or withdrawn, the value of our Company and our operating results will be adversely affected.

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

Sales of our product candidates outside of the U.S. will be subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approvals. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries, including the EMA must also approve the manufacturing and marketing of our product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., including additional preclinical studies or clinical trials or manufacturing and control requirements. In many countries outside the U.S., a product candidate must be approved for reimbursement before it can be approved for sale in that country. In many cases, the price that we propose to charge for our products is also subject to approval by individual countries before we can launch our product candidates in those countries. Obtaining foreign regulatory approvals, compliance with foreign regulatory requirements and approved pricing could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries and regulatory approval in one country does not ensure approval in any other country, while a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others.

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

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

Several provisions of the new law, which have varying effective dates, may affect us, including our costs. For example, the PPACA increased the Medicaid rebate rate, revised the definition of "average manufacturer price" for reporting purposes, which could further increase the amount of the Medicaid drug rebates paid to states, and extended the rebate program to beneficiaries enrolled in Medicaid managed care organizations. The PPACA also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or "donut hole." The law also established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the U.S. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with healthcare practitioners. Although the U.S. Supreme Court recently upheld most of the PPACA, it remains unclear whether there will be any changes made to certain provisions of PPACA through acts of Congress at some point in the future. In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. Most recently, in August 2011, the President signed into law the Budget Control Act of 2011, which may result in such changes as aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. The full impact on our business of

PPACA and the Budget Control Act is uncertain. We cannot predict whether other legislative changes will be adopted, if any, or how such changes would affect the pharmaceutical industry generally.

We are or may be subject to various healthcare regulations, and if we fail to comply with such regulations, we could face substantial penalties.

The laws that may affect our ability to operate include:

- the federal healthcare programs' Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs;
- federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;

- federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information;
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; and
- State reporting requirements detailing interactions with and payments to healthcare providers.

If our operations are found to be in violation of any of the laws described above or any other governmental laws and regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, the exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to market our products after receiving regulatory approval and adversely impact our financial results.

If we fail to demonstrate safety or efficacy in our preclinical studies or clinical trials or keep to the terms of a product development program, our future business prospects for these drug product candidates will be materially adversely affected.

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

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. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful. The clinical trial process may fail to demonstrate that our drug product candidates are safe for humans and effective for indicated uses. This failure would cause us to abandon a drug product candidate and may delay development of other drug product candidates. Any delay in, or termination of, our preclinical testing or clinical trials will delay the filing of our investigational new drug application, or IND, and NDA as applicable, with the FDA and, ultimately, our ability to commercialize our drug product candidates and generate product revenues. In addition, some of our clinical trials will involve small patient populations. Because of the small sample size, the results of these early clinical trials may not be indicative of future results. The failure to demonstrate safety or efficacy in our clinical trials would have a material adverse effect on our future business prospects, financial condition and operating results.

We do not have a significant amount of manufacturing experience and expect to continue to rely on third-party manufacturers to produce drug products that adequately support our clinical trials and commercial sales of any approved products.

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, severe weather events, 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 our NDAs. If our third party manufacturers and key suppliers are not in compliance with cGMP requirements, it may result in a delay of approval, particularly if these sites are supplying single source ingredients required for the manufacture of any potential product. These cGMP requirements include quality control, quality assurance and the maintenance of records and documentation. Furthermore, regulatory qualifications of manufacturing facilities are applied on the basis of the specific facility being used to produce supplies. As a result, if one of the manufacturers that we rely on shifts production from one facility to another, the new facility must go through a complete regulatory qualification and be approved by regulatory authorities prior to being used for commercial supply. Our manufacturers may be unable to comply with these

cGMP requirements and with other FDA, state and foreign regulatory requirements. A failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall, or withdrawal of product approval. If the safety of any quantities supplied is compromised due to our third party manufacturer's or key supplier's failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products.

In addition, we rely on one exclusive supplier for the active pharmaceutical ingredient, or API, for RP103. While we work closely with this supplier to ensure continuity of supply while maintaining high quality and reliability, we cannot guarantee that these efforts will be successful. A reduction or interruption in our supply of API from this supplier, and efforts to identity and qualify alternative sources of supply, could result in significant additional operating costs and delays in developing and commercializing RP103. In addition, supply arrangements from alternative sources may not be available on acceptable economic terms.

Our success depends on our ability to manage our projected growth.

With the potential commercial launch of RP103 for the potential treatment of cystinosis, the continued progress of our clinical-stage programs and with our plan to in-license and acquire additional clinical-stage product candidates, we will be required to retain existing and add required new qualified and experienced personnel in the commercial, regulatory, manufacturing, program management, clinical and medical areas over the next several years. Also, as our preclinical pipeline diversifies through internal discoveries, or the acquisition or in-licensing of new molecules, we will need to hire additional scientists to supplement our existing scientific expertise over the next several years.

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

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

In connection with the potential commercial launch of RP103 for the potential treatment of cystinosis, we have expanded our operations in Europe and have added and expect to continue to add personnel. We may encounter difficulties successfully managing a substantially larger and internationally diverse organization and may encounter delays in drug development and commercialization if we are not successful in integrating our international operations. Challenges related to managing international operations include the following:

- the potential strain on our financial and managerial controls and reporting systems and procedures;
- potential miscommunication between U.S. personnel and European personnel due to cultural and language differences;
- ability to operate with in diverse individual country regulatory and statutory laws; and
- greater than anticipated costs of maintaining EU presence, in-country legal entities and related tax structures.

Credit risks from customers outside the U.S. may negatively affect our results of operations.

Any future sales of our potential products to government supported customers in various countries outside of the U.S. may be subject to significant payment delays due to government funding and reimbursement practices, which will result in an increase in the length of time that we may have accounts receivable outstanding. For example, many governments in Europe are facing significant liquidity crises. If government reimbursement for future sales of our potential products is delayed or becomes unavailable, we may not be able to collect on amounts payable to us from such customers and our results of operations would be adversely affected.

Our reliance on third parties may result in delays in completing, or a failure to complete, preclinical testing, clinical trials, or regulatory marketing submissions if they fail to perform under our agreements with them.

In the course of product development, we may engage or collaborate with a variety of external organizations to perform services essential to drug product development. The organizations which perform services can include, but are not limited to:

- governmental agencies, U.S. and international university laboratories;
- other biotechnology companies;
- contract manufacturing organizations;

- clinical research organizations;
- distribution and supply (logistics) service organizations;
- testing organizations;
- consultants or consulting organizations with specialized knowledge based expertise;
- intellectual property legal firms; and
- multiple other service organizations.

If we engage these organizations to help us with our product development programs, many important aspects of this process have been and will be out of our direct control. If any of these organizations we engage in the future fail to perform their obligations under our agreements with them or fail to perform in a satisfactory manner, we may face delays in completing our development and commercialization processes for any of our drug product candidates. Furthermore, any loss or delay in obtaining contracts with such entities may also delay the completion of our clinical trials, regulatory filings and the potential market approval of our drug product candidates.

Specifically, we have and will continue to 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. If third parties fail to perform or to meet the applicable standards, this will result in delays or failures to complete trials. A failure by us or such third parties to keep to the terms of a product development program for any particular product candidate or to complete the clinical trials for a product candidate in the anticipated time frame could have significant negative repercussions on our business and financial condition.

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

- collaborative arrangements might not be available on terms which are reasonably favorable to us;
- disagreements with partners may result in delays in the development and marketing of products, termination of collaboration agreements or time consuming and expensive legal action;
- outside of agreement terms (which may be different or costly to enforce, if enforceable), we cannot control the amount and timing of resources partners devote to product candidates or their prioritization of product candidates, and partners may not allocate sufficient funds or resources to the development, promotion or marketing of our product candidates, or may not perform their obligations as expected;
- partners may choose to develop, independently or with other companies, alternative products or treatments, including products or treatments which compete with ours;
- agreements with partners may expire or be terminated without renewal, or partners may breach collaboration agreements with us;
- business combinations or significant changes in a partner's business strategy might adversely affect that partner's willingness or ability to complete their obligations to us; and
- the terms and conditions of the relevant agreements may no longer be suitable.

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

Our business could be adversely affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates, foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to pass through price increases on to our future customers due to the process by which healthcare providers are reimbursed for our future products by the government.

The U.S. credit and capital markets have recently experienced historic dislocations and liquidity crisis which have caused financing to be unavailable in many cases and, even if available, have caused the cost of prospective financings to significantly increase. These circumstances have materially impacted liquidity in the debt and capital markets, making financing terms for borrowers or for companies seeking equity capital, for those companies that are able to find financing at all, less attractive. In many cases, financial conditions have resulted in the unavailability of certain types of debt or equity financing. Continued uncertainty in the debt and equity markets may negatively impact our ability to access financing on favorable terms or at all. Federal legislation to deal with the current disruptions in the financial markets could have an adverse effect on our ability to raise other types of financing. In addition, our

suppliers, manufacturers and other third parties important to our business also may be negatively impacted by market dislocations and disruptions, their business may be disrupted and this could adversely affect our business and results of operations.

If we do not obtain the support of new, and maintain the support of existing, key scientific and medical collaborators, it may be difficult to develop current and new products and establish those products as a standard of care for various indications.

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

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

Because the extent and scope of patent protection for some of our drug products is particularly limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the exclusivity period under Orphan Drug Act designation to maintain a competitive position. If we do not obtain orphan drug exclusivity for our enteric formulation of RP103, under the Hatch-Waxman Act, this formulation of RP103 is eligible for a 3-year regulatory exclusivity period as a reformulated version of a previously approved drug substance for which clinical studies that are essential for approval have been conducted. However, if we do not obtain orphan drug exclusivity for our drug products that do not have patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced.

Even though we have been granted orphan drug designation for RP103 for the potential treatment of cystinosis and RP103 for the potential treatment of HD, and even if we obtain orphan drug designation for our future drug product candidates, due to the uncertainties associated with developing pharmaceutical products, we may not be the first to obtain marketing approval for any orphan indication. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

The priority review for our drug product candidates, if obtained, may not actually lead to a faster review process.

In the future, we may request six month priority review from the FDA and EMA for RP103 for HD and our other drug product candidates; however, the FDA and EMA 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.

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

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

revenues could be reduced.

Our future international sales and operating expenses will be subject to fluctuations in currency exchange rates.

If RP103 is approved by regulatory authorities outside the U.S. and we sell RP103 in such jurisdictions, a portion of our business will be conducted in currencies other than our reporting currency, the U.S. dollar. We will recognize foreign currency gains or losses arising from our operations in the period in which we incur those gains or losses. As a result, currency fluctuations between the U.S. dollar and the currencies in which we do business will likely cause foreign currency translation gains and losses in the future. Because of the number of currencies that may be involved, the variability of currency exposures and the potential volatility of currency exchange rates, we may suffer significant foreign currency translation losses in the future due to the effect of exchange rate fluctuations.

The use of any of our drug product candidates in clinical trials or the commercialization of our drug products in the future may expose us to liability claims.

The nature of our business exposes us to potential liability risks inherent in the testing (including through human trials), manufacturing and marketing of our drug product candidates. While we are in clinical stage testing, our drug product candidates could potentially harm people or allegedly harm people and we may be subject to costly and damaging product liability claims. Many of the patients who participate in our current clinical trials are already critically ill or suffering from chronic debilitating diseases when they enter a trial. The waivers we obtain may not be enforceable and may not protect us from liability or the costs of product liability litigation. Although we currently carry a $5.0 million clinical product liability insurance policy, it may not be sufficient to cover future claims.

In addition, the product liability insurance that we will need to obtain in connection with the commercial sales of our product candidates, if and when they receive regulatory approval, may not be sufficient or available in meaningful amounts or at a reasonable cost. Furthermore, we may not be able to avoid significant liability if any product liability claim is brought against us. If a successful product liability claim is brought against us and the amount of liability exceeds our insurance coverage, we may incur substantial charges that would adversely affect our business, financial condition and results of operation. We currently do not have any clinical or product liability claims or threats of claims filed against us.

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

Our success is dependent in part upon the availability of our senior executive officers. 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 qualified employees are retained, or are not available via recruitment, 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 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.

If we do not achieve our projected development and commercialization goals in the time frames we expect and announce, the credibility of our management and our organizational competence may be adversely affected.

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

From time to time, we may publicly announce the estimated 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. For example, clinical trials may be delayed due to factors such as IRB approvals, qualification of clinical sites, scheduling conflicts with participating clinicians and clinical institutions and the rate of patient enrollment. In most circumstances, we rely on academic institutions, major medical institutions, governmental research organizations (U.S. or internationally based), clinical research organizations or contract manufacturing organizations to conduct, supervise or monitor some or all aspects of clinical trials involving our product candidates. We will have limited control over the timing and other aspects of these clinical trials.

If we do not meet the milestones as publicly announced (or as projected by various analysts who follow our Company), our stockholders or potential stockholders may lose confidence in our ability to meet overall product development and commercialization goals and, as a result, the price of our common stock may decline.

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

We face burdens relating to the recent trend toward stricter corporate governance and financial reporting standards. Legislation or regulations such as the Sunshine Act, Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the Dodd-Frank Wall Street Reform and Consumer Protection Act, as well as other rules implemented by the FDA, the SEC and NASDAQ, follow the trend of imposing stricter corporate governance and financial reporting standards have led to an increase in the costs of compliance for companies similar to us, including substantial 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.

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 contract manufacturers and our single-source suppliers of raw materials and critical services are also vulnerable to damage from other types of disasters, including fires, storms, floods, power losses and similar events. If such a disaster were to occur, our ability to continue our product development programs or product commercialization activities 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.

Risks Related to Intellectual Property and Competition

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

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

The patent positions of biopharmaceutical products are complex and uncertain.

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

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We do not know whether our patent applications will result in issued patents. For example, we may not have developed a method for treating a disease before others developed similar methods;

- Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us, or file patent applications before we do. Competitors may also claim that we are infringing on their patents and therefore cannot practice our technology as claimed under our patents, if issued. Competitors may also contest our patents, if issued, by showing the patent examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose that patent. As a company, we have no meaningful experience with competitors interfering with our patents or patent applications;

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

In addition, competitors also seek patent protection for their technology. Due to the number of patents in our field of technology, we cannot be certain that we do not infringe on those patents or that we will not infringe on patents granted in the future. If a patent holder believes our drug product candidate infringes on its patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe on their technology, we would face a number of issues, including the following:

- Defending a lawsuit takes significant time is typically very expensive;
- If a court decides that our drug product candidate infringes on the competitor's patent, we may have to pay substantial damages for past infringement;
- A court may prohibit us from selling or licensing the drug product candidate unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, we may have to pay substantial royalties or grant cross licenses to our patents; and
- Redesigning our drug product candidates so we do not infringe may not be possible or practical and could require substantial funds and time.

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

If we are limited in our ability to utilize acquired or licensed technologies, we may be unable to develop, out-license, market and sell our product candidates, which could prevent new product introductions and/or cause delayed new product introductions.

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

If the purchase or licensing agreements we entered into are terminated, we will lose the right to use or exploit our owned and licensed technologies.

We entered into: a licensing agreement with UCSD for RP103/RP104; a licensing agreement with Washington University for mesoderm development protein, or Mesd; and a licensing agreement with Yeda Research and Development Company Limited, or Yeda, for patents originating from Weizmann Institute of Technology and Niigata University, related to use of transglutaminase inhibitors to treat neurological diseases.

UCSD, Washington University and Yeda may terminate their respective agreements with us upon the occurrence of certain events, including if we enter into certain bankruptcy proceedings or if we materially breach our payment obligations and fail to remedy the breach within the permitted cure periods. Although we are not currently involved in any bankruptcy proceedings or in breach of these agreements, there is a risk that we may be in the future, giving UCSD, Washington University and Yeda the right to terminate their respective agreements with us. We have the right to terminate these agreements at any time by giving prior written notice. If the UCSD, Washington University or Yeda agreements are

terminated by either party, we would lose our rights to RP103/RP104 in the case of UCSD, would lose our rights to the Mesd technology in the case of the Washington University agreement, and would lose our rights to the Weizmann and Niigata patents in the case of Yeda. Under such circumstances, we would have no further right to use or exploit the patents, copyrights or trademarks in those respective technologies. If this happens, we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected, and we may have to cease our operations.

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

Licensing our product candidates from other companies, universities or individuals does not always prevent them from developing non-identical but competitive products for their own commercial or research purposes, or from pursuing patent protection in areas that are competitive with us. While we seek patent protection for all of our owned and licensed product candidates, our licensors or assignors or other research organizations who created these product candidates are experienced scientists and business people who may continue to do research and development and seek patent protection in the same areas that led to the discovery of the product candidates that are licensed or assigned to us. By virtue of the previous research that led to the discovery of the drugs or product candidates that they licensed or assigned to us, these companies, universities, or individuals may be able to develop and out-license or market competitive products in less time than might be required to develop a product with which they have no prior experience and may reduce our future revenues from such product candidates. In some instances, information published in the scientific literature can provide insights which could enable development of viable competitive product candidates on an accelerated time frame.

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. All of our large pharmaceutical competitors have greater capital resources, larger overall research and development staffs and facilities, and a longer history in drug discovery, development, regulatory approval, manufacturing and marketing than we do. With these additional resources and experience, our competitors may be able to respond to rapid and significant technological changes in the biotechnology and pharmaceutical industries faster than we can.

We expect that the technologies associated with biotechnology research and development will continue to develop rapidly. Our future success will depend in large part on our ability to maintain a competitive position with respect to these technologies. Rapid technological development, as well as new scientific developments, may result in our compounds, drug product candidates or processes becoming obsolete before we can recover any or all of the expenses incurred to develop them. For example, changes in our understanding of the appropriate population of patients who should be treated with a targeted therapy like we are developing may limit the drug's market potential if it is subsequently demonstrated that only certain subsets of patients should be treated with the targeted therapy.

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

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

Risks Related to Our Financial Position and Capital Requirements

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

Excluding RP103 for the potential treatment of cystinosis, it will take several years before we are able to develop our other drug product candidates into marketable drug products, if at all. The marketing and sales effort of our products, our ability to gain adequate reimbursement, if approved for sale, and our product development programs will require substantial additional capital to successfully complete them, arising from costs to:

·conduct research, preclinical testing and human studies and clinical trials;

·establish or contract for pilot scale and commercial scale manufacturing processes and facilities;

·market and distribute our products; and

- establish and develop quality control, regulatory, medical, manufacturing, distribution, marketing, sales, finance and administrative capabilities to support these programs.

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

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

We base our outlook regarding the need for funds on many uncertain variables. Such uncertainties include the success of our efforts to commercialize our products, if approved, the success of our research initiatives, regulatory approvals, the timing of events outside our direct control such as negotiations with payors, potential strategic partners and other factors. In additional, certain product programs may require collaborative agreements with corporate partners with substantial assets and organizations to help with the very substantial funds required and the complex organizational resources required. Such agreements may require substantial time to complete and may not be available in the time frame desired, with acceptable financial terms, if at all. Any of these uncertain events can significantly change our cash requirements as they determine such one-time events as the receipt or payment of major milestones and other payments.

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

If we fail to obtain the capital necessary to fund our operations, our financial results will be adversely affected.

As of August 31, 2012, we had an accumulated deficit of approximately $117 million. We expect to continue to incur losses for the foreseeable future and will have to raise substantial cash to fund our planned operations. Our recurring losses from operations to date raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the year ended August 31, 2012, with respect to this uncertainty. We will need to raise additional capital and/or generate significant revenue at profitable levels to continue to operate as a going concern. In addition, the perception that we may not be able to continue as a going concern may cause others to choose not to deal with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

As of August 31, 2012, our cash, cash equivalents and short term investments were approximately $39 million. We believe our cash, cash equivalents and short-term investments as of August 31, 2012 and cash we have generated from the proceeds of sales of our common stock under our at-the-market sales agreement to-date, will be sufficient to meet our projected operational requirements and obligations into the third quarter of calendar 2013.

There can be no assurance that we will be successful in raising sufficient funds when needed. If we are unable to obtain such additional capital when needed, we will be forced to reduce our expenditures or seek other corporate solutions.

In addition, in the future, we may need to sell equity or debt securities to raise additional funds to support, among other things, our development and commercialization programs. The sale of additional equity securities or convertible debt securities will result in additional dilution to our stockholders. Additional financing may not be available in

amounts or on terms satisfactory to us or at all. We may be unable to raise additional capital due to a variety of factors, including our financial condition, the status of our research and development programs, the status of regulatory reviews for marketing approvals, the status of our commercialization activities, the execution of our potential launch of RP103 for cystinosis and the general condition of the financial markets. If we fail to raise additional financing when needed, we may have to delay or terminate some or all of our research and development programs, scale back our operations and/or reduce our pre-launch/launch expenses for RP103. If such actions are required, our financial condition and operating results will be adversely affected and our current value and potential future value may be significantly reduced.

Risks Related to Our Common Stock

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

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

As of August 31, 2012, there were (i) outstanding warrants to purchase 5,187,772 shares of our common stock at a weighted-average exercise price of $3.02 per share, (ii) outstanding options to purchase 5,975,559 shares of our common stock outstanding under our 2010 and 2006 Raptor stock option plans at a weighted-average exercise price of $4.12, (iii) options to purchase 149,264 shares of our common stock outstanding under our TorreyPines Therapeutics stock option plans at a weighted-average exercise price of $75.87 and (iv) 3,691,901 shares of our common stock available for future stock option grants to be issued under our 2010 Raptor stock option plan. The shares issuable upon exercise of stock options granted under our stock option plans will be available for immediate resale in the public market. The shares issuable upon the exercise of our warrants will be available for immediate resale in the public market. The market price of our common stock could decline as a result of such exercises due to the increased number of shares available for sale in the market.

Our executive officers and our board of directors own, in the aggregate, 917,597 shares, or approximately 1.8% of our outstanding common stock as of October 18, 2012. Sales of a substantial number of shares of our common stock by such officers and directors in the public trading market, whether in a single transaction or a series of transactions, or the perception that these sales may occur, could also have a significant effect on volatility and the trading price of our common stock.

Future milestone payments, as more fully set forth under "Contractual Obligations with Thomas E. Daley (assignee of the dissolved Convivia, Inc.)" under Note 11 Commitments and Contingencies in our consolidated financial statements and "Contractual Obligations with Former Encode Security Holders" section located in this Annual Report on Form 10-K for the year ended August 31, 2012, in connection with our acquisition of the Convivia assets and merger with Encode will result in dilution. We may be required to make additional contingent payments of up to 699,369 shares of our common stock, in the aggregate, under the terms of our acquisition of Convivia assets and merger with Encode, based on milestones related to certain future marketing and development approvals obtained with respect to Convivia and Encode product candidates. The issuance of any of these shares will result in further dilution to our existing stockholders. In addition we have an at-the-market sales agreement with Cowen and Company which, as of October 18, 2012, allows us to sell up to an additional $26.2 million worth of stock, which, if utilized further, will create substantial dilution for our existing 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 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 should not invest in our common stock.

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

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

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

- the results and timing of regulatory reviews relating to the approval of our drug candidates;
- failure of any of our drug candidates, if approved, to achieve commercial success and, in particular, the rate of market penetration and sales growth in the launch period;
- the results of our current and any future clinical trials of our current drug candidates;
- issues in manufacturing our drug candidates or any approved products;
- the entry into, or termination of, key agreements, including key strategic alliance agreements;
- failure to meet security analysts' and investors' expectations;
- the results of ongoing preclinical studies and planned early stage clinical trials of our preclinical drug candidates;
- the initiation of, material developments in, or conclusion of litigation to enforce or defend any of our intellectual property rights;
- general and industry-specific economic conditions that may affect our product program expenditures;
- the results of clinical trials conducted by others on drugs that would compete with our drug candidates;
- the loss of key employees;
- the introduction by others of technological innovations or new commercial products or development of product programs which have a direct negative competitive impact on our products or product development programs;
- changes in estimates or recommendations by securities analysts, if any, who cover our common stock or influence the level of investor confidence in our sector of the equity market;
- future sales of our common stock or exercise of common stock warrants or options;
- changes in the structure of health care payment systems; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. The stock market also has periods during which industry segments, such as biotechnology, are in volatile swings of greater or lesser favor as investments. These swings in the investment in a sector (periods of net sales or purchases of equity securities) will directly affect the stock prices of many companies in the sector and, in particular, those companies that do not have conventional measures of financial and business health such as sales, earnings, growth rates, profitability and other measures.

These broad market fluctuations typically will adversely affect the trading price of our common stock. In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation can result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

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

Our certificate of incorporation authorizes us to issue up to 15.0 million shares of preferred stock with designations, rights and preferences determined from time-to-time by our board of directors. Accordingly, our board of directors is empowered, without stockholder approval, to issue preferred stock with dividend, liquidation, conversion, voting or other rights superior to those of stockholders of our common stock.

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law as currently in effect may make a change in control of our Company more difficult, even if a change in control may be beneficial to the stockholders.

Our board of directors has the authority to issue up to 15.0 million shares of preferred stock, none of which are issued or outstanding. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Our charter contains provisions that may enable our management to resist an unwelcome takeover attempt by a third party, including: a prohibition on actions by written consent of our stockholders; the fact that stockholder meetings must be called by our board of directors; and provisions requiring stockholders to provide advance notice of proposals. Delaware law also

prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, the board of directors approves the transaction. Our board of directors may use these provisions to prevent changes in the management and control of our Company. Also, under applicable Delaware law, our board of directors may adopt additional anti-takeover measures in the future.

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

ITEM 1B: UNRESOLVED STAFF COMMENTS

None.

ITEM 2: PROPERTIES

We lease office and laboratory space as our headquarters in Novato, California. Effective November 1, 2012, the monthly base rent and operating expenses will be $19,585. The Novato lease expires on March 31, 2013. In October 2012, we entered into a one-year lease for administrative offices (which we had been leasing for the past 20 months) in San Mateo, California. We also rent a small office in France for our country manager in France. For the fiscal year ending August 31, 2012, our total office/laboratory rental expense was approximately $241,000.

We may lease office space in the Netherlands as our European sales and marketing headquarters within the next 12 months. We will need to expand or relocate our Novato headquarters to accommodate future hiring prior to the expiration of our existing Novato lease.

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: MINE SAFETY DISCLOSURES

Not applicable.

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." Effective February 29, 2012, our common stock commenced trading on the NASDAQ Global Market. As of October 18, 2012, there were 51,753,696 shares of our common stock outstanding. There is no public trading market for our warrants. The closing price for our common stock on October 18, 2012 was $4.88.

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, 2012:		
First Quarter (September 1 – November 30, 2011)	$5.52	$3.92
Second Quarter (December 1, 2011 – February 29, 2012)	7.90	5.35
Third Quarter (March 1 – May 31, 2012)	7.31	5.17
Fourth Quarter (June 1 – August 31, 2012)	6.15	4.35
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

Holders of Record

As of October 18, 2012, there were approximately 233 holders of record of our common stock and 51,753,696 shares of our common stock outstanding. Additionally, on such date, options, held by 82 persons to acquire up to, in the aggregate, 7,519,084 shares, and warrants held by 28 persons to acquire up to, in the aggregate, 5,187,772 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, 2012.

Performance Graph

The following is not deemed "filed" with the Securities and Exchange Commission and is not to be incorporated by reference into any filing we make under the Securities Act of 1933, as amended, whether made before or after the date hereof and irrespective of any general incorporation by reference language in such filing.

The following graph shows the value of an investment of $100 on September 30, 2009 (date we effected our 2009 Merger) in our common stock, the NASDAQ Composite Index (U.S.) and the NASDAQ Biotechnology Index. All values assume reinvestment of the pretax value of dividends paid by companies included in these indices and are calculated as of August 31st of each year. Our common stock is traded on the NASDAQ Global Market. The comparisons shown in the graph are based upon historical data and we caution that the stock price performance shown in the graph is not indicative of, nor intended to forecast, the potential future performance of our stock.

	September 30, 2009	August 31, 2010	August 31, 2011	August 31, 2012
Raptor Pharmaceutical Corp.	$ 100.00	$90.30	$143.33	$150.61
NASDAQ U.S. Composite Index	100.00	100.54	124.79	152.73
NASDAQ Biotechnology Index	100.00	96.73	119.13	168.82

ITEM 6: SELECTED FINANCIAL DATA

The following table shows selected historical consolidated financial and operating information for, and as of the end of, each of the periods indicated and should be read in conjunction with the information in the sections titled, "Management's Discussion and Analysis of Financial Condition and Results of Operation" and "Business" and Raptor's consolidated financial statements and the corresponding notes to those consolidated financial statements included elsewhere in this Annual Report on Form 10-K. The following tables set forth Raptor's consolidated balance sheet data as of August 31, 2012, 2011, 2010, 2009 and 2008, and its consolidated statements of comprehensive loss data for the years ended August 31, 2012, 2011, 2010, 2009 and 2008, for the period from September 8, 2005 (inception) to August 31, 2012.

	For the period ending August 31,					
(in millions, except per share data)	2012	2011	2010	2009	2008	For the period September 8, 2005 (Inception) to August 31, 2012
Income Statement Data:						
Revenues	$–	$–	$–	$–	$–	$ –
Operating expenses:						
General and administrative	14.7	6.2	3.7	2.7	2.2	31.6
Research and development	21.4	14.8	9.3	6.5	5.8	60.6
Total operating expenses	36.1	21.0	13.0	9.2	8.0	92.2
Loss from operations	(36.1)	(21.0)	(13.0)	(9.2)	(8.0)	(92.2)
Interest income	0.3	0.1	–	–	0.1	0.7
Interest expense	0.0	–	–	–	(0.1)	(0.1)
Foreign currency transaction gain	0.2	–	–	–	–	0.2
Realized gain on short-term investments	0.2	–	–	–	–	0.2
Adjustment to fair value of common stock warrants	(3.2)	(16.3)	(5.9)	–	–	(25.4)
Net loss	(38.6)	(37.2)	(18.9)	(9.2)	(8.0)	(116.6)
Other comprehensive loss:						
Foreign currency translation adjustment	(0.1)	–	–	–	–	(0.1)
Comprehensive loss	$(38.7)	$(37.2)	$(18.9)	$(9.2)	$(8.0)	$ (116.7)
Net loss per share:						
Basic and diluted	$(0.80)	$(1.15)	$(0.85)	$(0.64)	$(0.81)	
Weighted average shares outstanding used to compute:						
Basic and diluted	48.1	32.3	22.2	14.4	9.9	

(in millions)	August 31, 2012	2011	2010	2009	2008
Balance Sheet Data:					
Cash, cash equivalents and short-term investments	$38.9	$15.2	$17.0	$3.7	$7.5
Working capital (deficit)	20.6	(11.0)	(0.3)	2.7	6.7
Total assets	48.3	22.6	24.4	6.6	10.6
Common stock warrant liability	17.3	23.6	–	–	–
Total liabilities	21.6	26.7	17.6	1.1	1.0
Accumulated deficit	(116.6)	(78.0)	(40.8)	(21.9)	(12.7)
Total stockholders' equity (deficit)	26.7	(4.1)	6.8	5.5	9.6

You should read the following tables presenting our unaudited quarterly results of operations in conjunction with the consolidated financial statements and related notes contained elsewhere in this Annual Report on Form 10-K. We have prepared this unaudited information on the same basis as our audited consolidated financial statements. Our quarterly operating results have fluctuated in the past and may continue to do so in the future as a result of a number of factors, including, but not limited to, the timing and nature of research and development activities.

	(In millions, except per share data, unaudited)			
	November 30,	February 28/29,	May 31,	August 31,
Quarterly Data				
2012:				
Net loss	$(11.4)	$ (14.0)	$(3.0)	$(10.2)
Net loss per share, basic and diluted	$(0.25)	$ (0.29)	$(0.06)	$(0.20)
2011:				
Net loss	$(10.1)	$ (3.0)	$(20.3)	$(3.8)
Net loss per share, basic and diluted	$(0.33)	$ (0.09)	$(0.62)	$(0.11)

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, 2012, and the notes to such consolidated financial statements included elsewhere in this Annual Report on Form 10-K. The "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

We are an emerging biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. Our initial focus is on developing our first product candidate, RP103, as a potential treatment for cystinosis, a rare genetic disorder. Cystinosis patients are at very high risk of experiencing life-threatening metabolic disorders, including kidney failure, severe gastrointestinal dysfunction and rickets as a result of an accumulation of the amino acid, cystine, in cells. As a result, cystinosis patients have a substantially reduced life span relative to unaffected individuals.

In July 2011, we announced that RP103 had met the sole primary endpoint in our Phase 3 clinical trial designed to evaluate RP103 as a potential treatment for cystinosis. In the first quarter of calendar 2012, we submitted an NDA to the FDA requesting approval to market RP103 as a potential treatment for cystinosis. The FDA granted Standard Review designation for RP103 and has assigned the user fee goal date (by which we anticipate a response from the FDA) of January 30, 2013. Also in the first quarter of calendar 2012, we submitted an MAA to the EMA requesting approval to market RP103 as a potential treatment for cystinosis.

In addition to cystinosis, we are also testing RP103 for the potential treatment of NASH, a metabolic liver disorder, and HD, a neurodegenerative disorder.

Clinical Development Programs

Our three active clinical development programs utilize the same active pharmaceutical ingredient cysteamine bitartrate. Cysteamine bitartrate was approved in 1994 as an orally available immediate-release powder in a capsule for the treatment of, and is the current standard of care for cystinosis. We are reformulating cysteamine bitartrate to potentially improve the dose administration, safety and/or efficacy compared to existing treatment and repurposing cysteamine bitartrate for potential applications in new disease indications. Our proprietary delayed-release formulation, RP103, is a capsule containing enteric coated micro-beads of cysteamine bitartrate. We believe RP103 will require less frequent dosing and could reduce gastro-intestinal and other side effects compared to immediate-release cysteamine bitartrate for cystinosis patients. In addition to cystinosis, we are also testing RP103 for the potential treatment of NASH and HD. RP104 is our enteric coated tablet formulation of cysteamine bitartrate in development. We obtained the exclusive worldwide license to delayed-release cysteamine, which is the basis for our proprietary formulations of cysteamine, through the 2007 merger of our clinical subsidiary and Encode, formerly a privately-held product development company.

Our other clinical-stage product candidate is Convivia, our proprietary oral formulation of 4-methylpyrazole, for the potential management of acetaldehyde toxicity due to alcohol consumption by individuals with aldehyde dehydrogenase, or ALDH2 deficiency, an inherited metabolic disorder.

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; and

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

Future Activities

Over the next fiscal year, we plan to conduct research and development and general and administrative activities including: pre-commercial launch preparation of RP103 in the U.S. and EU, (including preparing commercial materials and coordinating drug supply)

and, if approved, conducting a commercial launch of RP103 in the U.S. and EU; supporting our ongoing extension study of RP103 in cystinosis until patients are converted onto commercial drug; conducting other supporting clinical studies of RP103 in cystinosis; supplying clinical material for our ongoing clinical trial of RP103 in HD; funding the collaboration and supplying clinical material in our ongoing Phase 2b clinical trial of RP103 in NASH; continuing business development of our preclinical product candidates; conducting research and development activities for in-licensed and newly discovered preclinical assets; supporting potential clinical trials of RP103 in malaria, Rett Syndrome, fibrosis and Parkinson's Disease (subject to potential external funding); and supporting associated facilities and administrative functions.

We plan to seek additional business development partners for our Convivia product candidate in Asia. We may also develop new preclinical, clinical and or commercial opportunities, including proprietary targets discovered in-house and in-licensed and acquired technologies.

Application of Critical Accounting Policies

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

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

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 U.S. dollar. Raptor Pharmaceuticals Europe B.V. ("BV") and RPTP European Holdings C.V. ("CV"), our European subsidiary and Cayman-based subsidiary, respectively, use the European Euro as their functional currency. At each quarter end, BV's and the CV's balance sheets are translated into U.S. dollars based upon the quarter-end exchange rate, while their statements of comprehensive loss are translated into U.S. dollars based upon an average of the Euro's value between the beginning and end date of the reporting period. BV's and CV's equity are 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 U.S. and European banks as collateral for credit cards.

Short-term Investments

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

Prepaid expenses and other

Prepaid expenses consists primarily of advance payments to vendors that are due within one year. Other consists primarily of a receivable of $0.5 million resulting from a timing difference in the receipt of cash proceeds from the sale of common stock under our At-The-Market Sales Agreement as discussed in Note 9 in our consolidated financial statements attached as an exhibit to this Annual Report on Form 10-K.

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) and to an out-license acquired in the 2009 Merger. The intangible assets related to RP103/RP104 are amortized using the straight-line method over the estimated useful life of 20 years, which is the life of the intellectual property patents. The 20-year estimated useful life is also based upon the typical development, approval, marketing and life cycle management timelines of pharmaceutical drug products. The intangible assets related to the out-license will be amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents.

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.

As of August 31, 2012, we determined that the capitalized acquired in-process research and development cost of $0.9 million, representing the tezampanel and NGX 426 program acquired in the 2009 Merger, was impaired due to our decision to discontinue development for thrombosis due to regulatory hurdles that would require significant expenditures which we chose not to prioritize for funding. As such, we expensed $0.9 million as in-process research and development as part of research and development expense on our consolidated statements of comprehensive loss

for the year ended August 31, 2012.

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.

Our effective tax rate is 0% for income tax for the year ended August 31, 2012. Based on the weight of available evidence, including cumulative losses since inception and expected future losses, we have determined that it is more likely than not that the deferred tax asset amount will not be realized and therefore a full valuation allowance has been provided on our net deferred tax assets.

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

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

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

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

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, commercial manufacturing prior to drug approval, 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, August 31, 2011 and August 31, 2012 increases added 1,629,516, 1,778,459 and 2,528,407 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 retirement or a non-employee director's voluntary cessation of service 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 or cessation, (b) the termination of a non-employee director's board membership for reasons other than for cause or voluntary cessation of service 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.
In May 2006, RPC'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 RPC 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 the 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.

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, 2012, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 0.68% to 1.2%; 5-6 year expected life; 121 to 125% 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 five-to-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.

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, 2012 and 2011

General and Administrative Expenses (in thousands)

General and administrative expenses include finance, executive and sales and marketing 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, 2012 increased by approximately $8,545 compared to the prior fiscal year. The increase was primarily due to:

Reason for Increase (Decrease)	Increase (Decrease)
Expenses not in FY2011:	
Commercial operations requirements RP103 for the potential treatment of cystinosis:	
Pre-commercial consulting services	$ 1,996
Tax study and advisory fees related to EU headquarters	866
Salary and bonuses for commercial operations personnel	516
Salary, benefit and bonus increases and new finance and human resources personnel	1,182
Stock-based compensation expense, employees and directors (non-cash)	2,062
Legal fees due to in-licensing of intellectual property	575
Investor relations costs including proxy mailing and solicitation, press releases, webcasting, XBRL filing costs	492
Other, net	856
General and Administrative increase year ended August 31, 2012 vs. August 31, 2011	$ 8,545

Research and Development (in thousands)

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, 2012 increased by approximately $6,655 over the prior fiscal year primarily due to:

Reason for Increase (Decrease)	Increase (Decrease)
Increased product manufacture of RP103 and RP104 for the potential treatment of cystinosis, HD, NASH	$ 4,359
Tax grants and expense reimbursements for preclinical and clinical programs not available in FY2012	820
R&D compensation	
Salary, bonus and benefits increases and new hire compensation	621
Stock-based compensation expense, employees (non-cash)	505
Write-off of capitalized intangibles no longer being developed	792
Preclinical studies including research materials and lab services	479
Reduction in Phase 3 cystinosis trial expense partially offset by extension study and other smaller studies	(1,717)
Other, net	796
Research and development increase year ended August 31, 2012 vs. August 31, 2011	$ 6,655

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

		Year ended August 31,	
Major Program (stage of development)	Cumulative through August 31, 2012	2012	2011
RP103/RP104 – All Indications (clinical/pre-commercial)	$ 39.9	$18.2	$10.5
Minor and Inactive Programs	7.8	1.7	0.5
R & D Personnel and Other Costs Not Allocated to Programs	13.0	1.5	3.8
Total Research & Development Expenses	$ 60.7	$21.4	$14.8

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

	Cumulative through August 31,	Year ended August 31,	
		2012	
Major Program (stage of development)	2012		2011
RP103/RP104 – All Indications (clinical and pre-commercial)	$ 3.8	$2.7	$1.0
Minor and Inactive Programs	1.1	0.1	0.2

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 potential treatment of cystinosis.

Current Status of Major Programs

Please refer to the Item 1 of this Annual Report on Form 10-K for a detailed discussion of each of our major programs. In summary, RP103 is being developed in cystinosis, and RP103 is being developed in NASH and HD. In March 2012, we filed with the FDA and the EMA for marketing approval of RP103 for cystinosis and anticipate a decision by the FDA by January 30, 2013 (PDUFA date) and by the EMA by the first half of calendar 2013. In anticipation of marketing approval of RP103 for the potential treatment of cystinosis, we are preparing for potential launch in both the U.S. and the EU. In June 2012, we commenced a Phase 2b clinical trial of RP103 in NASH with the National Institutes of Health and anticipate full enrollment in the first quarter of calendar 2013 and the potential release of data in the first half of calendar 2014. In June 2012, we announced the full enrollment of our HD Phase 2/3 clinical trial of RP103 and the potential release of data in the first half of calendar 2014. We continue efforts to out-license Convivia and our preclinical programs.

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.

Interest Income

Interest income increased approximately $0.3 million for the year ended August 31, 2012 when compared to the prior year due to primarily to higher cash balances from our September 2011 $46 million follow-on offering and from sales of stock pursuant to our at-the-market sales agreement.

Interest Expense

Interest expense for the years ended August 31, 2012 and 2011 was nominal.

Foreign Currency Transaction Loss

Foreign currency transaction gains were approximately $0.1 million for the year ended August 31, 2012 compared to a nominal transaction loss in the prior year. The increase was due to more activity conducted in Europe (in Euro) in preparation for commercial launch and activities of our subsidiary, the CV, whose functional currency is the Euro.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of approximately $(3.2) million for the year ended August 31, 2012 compared to a loss of approximately $(16.3) million for the year ended August 31, 2011, a decrease in loss of approximately $13.1 million resulting primarily from the lower number of remaining warrants outstanding due to warrant exercises, as well as an increase of $0.24 per share in our common stock price from August 31, 2011. These losses are non-cash.

Years ended August 31, 2011 and 2010

General and Administrative Expenses (in thousands)

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 $2,458 compared to the prior fiscal year. The increase was primarily due to:

Reason for Increase (Decrease)	Increase (Decrease)
Stock option grants FY 2011, including catch-up grants, non-cash expense	$ 1,378
Additional commercial operations and business development consulting in FY 2011	654
Increased salaries, benefits (401K matching), bonuses including compensation for new hires	458
Various other, net	(32)
General and Administrative increase year ended August 31, 2011 vs. August 31, 2010	$ 2,458

Research and Development (in thousands)

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 over the prior fiscal year primarily due to:

Reason for Increase (Decrease)	Increase (Decrease)
Increase in clinical costs including materials, CRO fees and site fees related to RP103 for the potential treatment of cystinosis	$ 5,832
Increased salaries, benefits (increased 401K matching) bonuses, including compensation for new hires	381
Stock option grants in FY 2011, including catch-up grants, non-cash expense	361
Clinical materials related to thrombosis study	250
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, net	278
Research and Development increase year ended August 31, 2011 vs. August 31, 2010	$ 5,454

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

	Year ended August 31,	
Major Program (stage of development)	2011	2010
RP103/RP104 – All Indications (clinical/pre-commercial)	$10.5	$6.2
Minor or Inactive Programs	0.5	0.4
R & D Personnel and Other Costs Not Allocated to Programs	3.8	2.7
Total Research & Development Expenses	$14.8	$9.3

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

	Year ended August 31,	
Major Program (stage of development)	2011	2010
RP103/RP104 – All Indications (clinical and pre-commercial)	$1.0	$0.1
Minor or Inactive Programs	0.2	0.4

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 potential treatment of cystinosis (approximately $0.5 and zero for the year ended August 31, 2011 and 2010, respectively).

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.

Liquidity and Capital Resources
Capital Resource Requirements
As of August 31, 2012, we had approximately $39.1 million in cash, cash equivalents, short-term investments and restricted cash, approximately $21.5 million in current liabilities (of which $17.3 million represented the non-cash common stock warrant liability) and approximately $20.6 million of net working capital.

We believe our cash, cash equivalents, restricted cash and short term investments as of October 18, 2012 of approximately $41 million will be sufficient to meet our obligations into the third quarter of calendar 2013.
Our recurring losses from operations raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the year ended August 31, 2012 with respect to this uncertainty. We may need to generate significant revenue or sell equity or debt securities to raise additional funds to continue to operate as a going concern beyond the third quarter of calendar 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 equity securities will result in additional dilution to our stockholders. Additional financing, equity or debt, 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 will be adversely affected and we may have to scale back our operations.

As of October 18, 2012, approximately 1.1 million shares of our common stock have been issued upon exercise of the Series A Warrants, approximately 1.9 million shares of our common stock have been issued upon exercise of the Series B Warrants, approximately 0.1 million shares of our common stock have been issued upon exercise of the placement agent warrants, and Series A warrants to purchase up to approximately 0.8 million shares of our common stock were outstanding, all of which warrants were issued pursuant to a definitive securities purchase agreement, dated as of December 17, 2009. The outstanding Series A Warrants are exercisable until December 22, 2014, at a per share exercise price of $2.45.

As of October 18, 2012, approximately 1.3 million shares of our common stock had been issued upon exercise of the warrants, and warrants to purchase up to approximately 3.7 million shares (including the placement agent warrant described below) of our common stock were outstanding, all of which warrants were issued pursuant to private placement purchase agreements, dated as of August 9, 2010, with private placement investors for the private placement of our common stock and warrants. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share. The placement agent warrant is the warrant issued to the placement agent for this private placement to purchase 97,952 shares of our common stock at an exercise price of $3.075 per share.

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

On April 30, 2012, we entered into a Sales Agreement with Cowen and Company, LLC, or Cowen, to sell shares of our common stock, with aggregate gross sales proceeds of up to $40,000,000, from time to time, through an "at the market" equity offering program under which Cowen acts as sales agent. We pay a 3% commission to Cowen on any sales pursuant to this Sales Agreement. As of October 18, 2012, we sold an aggregate of approximately 2.7 million shares at a weighted-average sales price of $5.20 per share for net proceeds of approximately $13.4 million.

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

We will not be able to generate revenues from the sale of products until we obtain regulatory approval for RP103, our lead drug product candidate. Revenue generation may require several more months beyond the PDUFA date and potential approval, or substantially longer if there are further regulatory requirements and delays. Substantial revenues in addition to cystinosis revenues will depend on further development and regulatory approval of our other drug product candidates which will 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 including critical pricing, competition from well-funded competitors, and our ability to manage our expected growth. It is uncertain that for several years, we will be able to generate internal positive cash flow from the sales of our drug product candidates sufficient to meet all of our operating cash flow and capital expenditure requirements.

There is substantial doubt about our ability to continue as a going concern as the continuation of our business is dependent upon obtaining further long-term financing, the obtaining of regulatory approvals for our product candidates, the successful development of our drug product candidates and related technologies, the successful and sufficient market acceptance of any product offerings that we may introduce and, finally, the achievement of a

profitable level of operations. The issuance of additional equity securities by us is likely to result in a significant dilution in the equity interests of our current stockholders. Obtaining commercial loans, assuming those loans would be available, including on acceptable terms, will increase our liabilities and future cash commitments.

Research and Development Activities

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

General and Administrative Activities

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

Capital Expenditures

In the next 12 months, we anticipate to increase our capital expenditures on leasehold improvements, office equipment and computer software and hardware as we continue to increase our staff in anticipation of the potential commercial launch of RP103 in calendar 2013.

Contractual Obligations

Contractual Obligations With Former Encode Stockholders And UCSD Relating To The Acquisition Of The Dr Cysteamine (RP103 And RP104) License

As a result of the merger between our wholly owned subsidiary and Encode, the Encode security holders are eligible to receive up to approximately an additional 559,000 shares of our common stock, our stock options and our 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.

Also as a result of the merger, we are obligated to pay an annual maintenance fee to UCSD for the exclusive license to develop RP103/RP104 for certain indications of $15,000 until we begin commercial sales of any products developed pursuant to the License Agreement. In addition to the maintenance fee, we are obligated to pay during the life of the License Agreement: milestone payments ranging from $20,000 to $750,000 for orphan indications and from $80,000 to $1,500,000 for non-orphan indications upon the occurrence of certain events, if ever; royalties on commercial net sales from products developed pursuant to the License Agreement ranging from 1.75% to 5.5%; a percentage of sublicense fees ranging from 25% to 50%; a percentage of sublicense royalties; and a minimum annual royalty commencing the year we begin commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, we are obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 years from the effective date of the License Agreement, depending on the indication. In addition, we are obligated to, among other things, annually spend at least $200,000 for the development of products (which, as of our fiscal years ended August 31, 2012, 2011, 2010 and 2009 we satisfied by spending approximately $20.9 million, $11.3 million, $6.2 million and $4.1 million, respectively, on such programs) pursuant to the License Agreement. Cumulatively, we have expensed $910,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, HD and NASH and on regulatory filings in cystinosis. In March 2012, we filed our MAA with the EMA as well as our NDA with the FDA for RP103 for the potential treatment of cystinosis. In conjunction with the achievement of the MAA/NDA filing milestone, we paid $250,000 to UCSD pursuant to this license. Future

milestones of $500,000 and $750,000 will be payable if the RP103 MAA and NDA for cystinosis are approved, respectively.

To the extent that we fail to perform any of our obligations under the License Agreement, then UCSD may terminate the license or otherwise cause the license to become non-exclusive.

Other Contractual Obligations

We have contractual obligations under our operating leases and other obligations related to research and development activities, purchase commitments, and licenses. Information about these obligations as of August 31, 2012, with respect to fiscal years ending as noted in the table below, is presented in the table below (in thousands).

	Payments Due by Period					
	2013	2014	2015	2016	2017 and Thereafter	Total
Operating leases	$9	$9	$5	$0	$ 0	$23
Research and development and purchase commitments	7,329	2,254	320	444	244	10,591
Total	$7,338	$2,263	$325	$444	$ 244	$10,614

We maintain several contracts with contract manufacturers, clinical organizations and clinical sites, drug labelers and distributors, and research organizations, primarily to assist with clinical research and clinical manufacturing for our cystinosis and HD programs and our NASH clinical collaboration. The future commitments pursuant to these agreements are included in the table above as research and development and purchase commitments.

We are also subject to contingent payments related to various development activities totaling approximately $18.0 million, which are due upon achievement of certain development and commercial milestones if such milestones occur before certain dates in the future.

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, RPC 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 RPC (merged into Raptor Pharmaceutical Corp. effective December 7, 2011) consolidated with its subsidiaries and with us, its parent, Raptor Pharmaceutical Corp. (formerly TorreyPines Therapeutics, Inc.). Earnings per share for periods prior to the reverse merger have been restated to reflect the number of equivalent shares received by former stockholders.

Going Concern

Due to the uncertainty of our ability to meet our current operating and capital expenses, in their reports on our audited financial statements for the years ended August 31, 2012, 2011, 2010, 2009, 2008 and for the period September 8, 2005 (inception) to August 31, 2007, 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, or 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 was not permitted. We adopted these standards on September 1, 2011 and have determined that ASU 2010-28 had no material impact on our consolidated financial

statements for the fiscal period ended August 31, 2012, because there was no requirement to perform Step 2 due to our positive carrying amount.

In December 2010, the FASB issued ASU 2010-29, Business Combinations (Topic 805): Disclosure of Supplementary Pro Forma Information for Business Combinations, or 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 was permitted. We adopted these standards on September 1, 2011; however, since there were no business combinations during the fiscal period ended August 31, 2012, ASU 2010-29 had no material impact on our financial disclosure. However, the provision will impact the financial disclosures of any business combinations in the future.

In May 2011, the FASB issued ASU 2011-04, Fair Value Measurement (Topic 820): Amendments to Achieve Common Fair Value Measurement and Disclosure Requirements in GAAP and IFRSs, or ASU 2011-04. ASU 2011-04 is intended to result in convergence between 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 GAAP. Key provisions of the amendment include: a prohibition on grouping financial instruments for purposes of determining fair value, except when an entity manages market and credit risks on the basis of the entity's net exposure to the group; an extension of the prohibition against the use of a blockage factor to all fair value measurements (that prohibition currently applies only to financial instruments with quoted prices in active markets); and a requirement that for recurring Level 3 fair value measurements, entities disclose quantitative information about unobservable inputs, a description of the valuation process used and qualitative details about the sensitivity of the measurements. In addition, for items not carried at fair value but for which fair value is disclosed, entities will be required to disclose the level within the fair value hierarchy that applies to the fair value measurement disclosed. ASU 2011-04 is effective for interim and annual periods beginning after December 15, 2011. We adopted these standards on March 1, 2012 and determined that ASU 2011-04 did not have a material impact 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 was effective for interim and annual periods beginning after December 15, 2011. We early adopted these standards as of August 31, 2011. Because ASU 2011-05 impacts presentation only, it had no effect on our consolidated financial statements or on our financial condition for the fiscal period ended August 31, 2012.

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. Because we have only one reporting unit, which has a fair value higher than its carrying amount, adoption of ASU 2011-08 did not have a material impact on our consolidated financial statements for the fiscal period ended August 31, 2012.

In July 2012, the FASB issued ASU 2012-02, Intangibles – Goodwill and Other (Topic 350): Testing Indefinite-Lived Intangible Assets for Impairment, or ASU 2012-02. ASU 2012-02 simplifies how entities test indefinite-lived intangible assets, other than goodwill, for impairment and permits an entity to first assess qualitative factors to

determine whether it is more likely than not that the indefinite-lived intangible asset is impaired. The amendments are effective for annual and interim indefinite-lived intangible asset impairment tests performed for fiscal years beginning after September 15, 2012 (early adoption is permitted). We will adopt these standards on November 1, 2012. The implementation of the amended accounting guidance is not expected to have a material impact on our consolidated financial statements.

In October 2012, the FASB issued ASU 2012-04, Technical Corrections and Improvements, or ASU 2012-04, which makes certain technical corrections and "conforming fair value amendments" to the FASB Accounting Standards Codification. The amendments affect various codification topics and apply to all reporting entities within the scope of those topics. These provisions of the amendment are effective upon issuance, except for amendments that are subject to transition guidance, which will be effective for fiscal periods beginning after December 15, 2012. We will adopt these standards on November 1, 2012. The provisions of ASU 2012-04 are not expected to have a material impact on our consolidated financial statements.

ITEM 7A: QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

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

Foreign Exchange Risk

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

Interest Rate Risk

We are subject to interest rate risks associated with fluctuations in interest rates. As of August 31, 2012, we had $15.3 million invested in a short-term bond fund with the goal of increasing yield on our idle cash. Approximately $9.2 million remained in a money market account, yielding approximately 0.07% per year. The short-term bond fund invests the majority of its assets in high-quality securities issued or guaranteed by U.S. government agencies or Government Sponsored Enterprises, and has a historical annual yield of over 2%. This bond fund pays dividends and provides the net asset value of its assets on a daily basis with daily liquidity. The change in net asset value is recorded on our statements of comprehensive loss as unrealized gain or loss on short-term investments. We completed an evaluation of our investments and determined that we did not have any other-than-temporary impairments as of August 31, 2012. Our investments are placed in financial institutions with strong credit ratings and management expects full recovery of the carrying amounts. A hypothetical one percentage point decline in interest rates would not have materially affected our consolidated financial position, results of operations or cash flows as of August 31, 2012.

ITEM 8: FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The information required to be filed in this item appears on pages F-1 to F-49 of this Annual Report on Form 10-K.

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

Financial Statements

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

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

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

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

Changes in Internal Control over Financial Reporting

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

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 18, 2012.

Name	Age	Position(s) Held with the Company
Raymond W. Anderson (2)(3)	70	Director
Suzanne L. Bruhn, Ph.D. (1)(3)	49	Director
Richard L. Franklin, M.D., Ph.D. (1)(2)	67	Director
Llew Keltner, M.D., Ph.D. (1)(2)	62	Director
Erich Sager	54	Director
Vijay B. Samant (1)(3)	59	Director
Christopher M. Starr, Ph.D.	60	Chief Executive Officer and Director
Timothy P. Walbert (2)(3)	45	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. (Bill) Anderson. Mr. Anderson has served as a director of Raptor Pharmaceutical Corp. since September 2009 and as a director of RPC 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 U.S. 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 currently chief executive officer of Promedior, Inc., a privately held, clinical stage biotechnology company focused on the development of targeted therapeutics to treat diseases involving fibrosis. Immediately prior to her appointment as the chief executive officer of Promedior, Inc., Dr. Bruhn spent 13 years at Shire Human Genetic Therapies (HGT), a division of Shire (NASDAQ: SHPGY) (LSE: SHP), specializing in the development and commercialization of treatments for orphan diseases, most recently as Senior Vice President, Strategic Planning and Program Management. At Shire HGT, Dr. Bruhn was responsible for establishing the program management function, driving strategic planning and portfolio management, and for global regulatory affairs. Dr. Bruhn 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.

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 RPC 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 RPC 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, Inc. (NYSE: MRK), including Chief Operating Officer of the Merck Vaccine Division, and Vice President of Vaccine Operations, Vice President of Business Affairs, and Executive Director of Materials Management, all in the Merck Manufacturing Division. 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) from 2003 to 2012; 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, University Department of Chemical Technology. 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 RPC 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, which was acquired by Takeda Pharma Holdings in June 2009. For more than 20 years, Mr. Walbert held executive positions in general management, corporate strategy, sales, U.S. 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 eight billion in 2011 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, 2012, our directors, executive officers and 10% Stockholders timely filed all Section 16(a) reports applicable to them, except as noted below. We believe that during the fiscal year ended August 31, 2012, our directors, executive officers and 10% Stockholders timely filed all Section 16(a) reports, with the exception of one late Form 4 for Mr. Sager and two late Form 4s for Mr. Henk Doude van Troostwijk. As of September 25, 2012, Mr. Doude van Troostwijk was no longer classified as a Section 16 officer.

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.

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 18, 2012. 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.	60	Chief Executive Officer and Director
Julie Anne Smith	41	Chief Operations Officer, Raptor Therapeutics
Georgia Erbez	44	Chief Financial Officer, Treasurer and Secretary
Thomas (Ted) E. Daley	49	President, 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."

Julie Anne Smith. As of September 10, 2012, Ms. Smith joined us as our Executive Vice President, Strategy and Chief Operating Officer. Ms. Smith will be responsible for directing our commercial, manufacturing, and program management organizations, and providing leadership in corporate and strategic development initiatives. Prior to joining Raptor, from July 2008 to May 2012, Ms. Smith was Chief Commercial Officer of Enobia Pharma, Inc., a private, clinical stage orphan company acquired by Alexion (ALXN). From August 2006 to July 2008, as Vice President, Commercial at Jazz Pharmaceuticals, she led commercial functions. From December 2001 to August 2006, as Vice President, Global Marketing at Genzyme General in Cambridge MA, she led the worldwide commercialization and planning for Myozyme, an infused enzyme replacement therapy for an ultra-orphan genetic disease. In her nearly 20 years in biotech, Ms. Smith has served in executive management of both private and public biotech firms, mostly in orphan drug development and commercial product opportunities. She holds a B.S. in Biological and Nutritional Science from Cornell University, Ithaca, NY.

Georgia Erbez. As of September 10, 2012, Ms. Erbez joined us as our Chief Financial Officer. As of September 2012, Ms. Erbez also was appointed to serve as our Secretary and Treasurer. Ms. Erbez is responsible for directing our global financial strategy and organization and providing leadership in defining, communicating, and executing corporate and financial strategic initiatives. Prior to joining Raptor, from March 2008 to September 2012, Ms. Erbez has been a founder and managing director of Beal Advisors, a boutique investment bank that has provided advisory and capital acquisition services to emerging growth companies. Ms. Erbez also served as managing director and consultant at Collins Stewart LLC from April 2011 to January 2012. From 2005 to 2008, Ms. Erbez was a Senior Vice President in the life sciences investment banking group at Jefferies & Co. From 1998 to 2002, she was with the health care investment banking group at Cowen and Co., most recently as Director. From 1997 to 1998, Ms. Erbez was an associate at Hambrecht & Quist where she provided investment banking services to healthcare services and life sciences companies. From July 1989 to January 1997, Ms. Erbez was with Alex Brown & Sons in the healthcare investment banking group, where she focused on life sciences, medical technology and healthcare services companies. She holds a B.A. in International Relations with an emphasis in Economics from the University of California at Davis.

Thomas (Ted) E. Daley. As of September 29, 2009, Mr. Daley joined us as President and a board member of Raptor Therapeutics, a wholly-owned indirect subsidiary acquired in the 2009 Merger. Mr. Daley joined Raptor Therapeutics 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 U.S. 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.

Relationships Among Executive Officers and Directors

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

ITEM 11: EXECUTIVE COMPENSATION

Named Executive Officer Compensation
Compensation Discussion and Analysis

Overview

The Compensation Committee of our board of directors, herein referred to as the Compensation Committee, has overall responsibility for the compensation program for our executive officers. The Compensation Committee reviews, adopts and oversees our compensation strategy, policies, plans and programs, including:

(i) the establishment of corporate and individual performance goals and evaluation of performance relevant to the compensation of our executive officers and other senior management and staff;
(ii) the review and approval of the terms of employment or service, including severance and change in control arrangements, of our Chief Executive Officer and the other executive officers;
(iii) the review and recommendation to the board of directors of the compensation plans and programs advisable for the Company, including the type and amount of compensation to be paid or awarded to non-employee directors; and
(iv) the administration of our equity compensation plans, pension and profit-sharing plans, deferred compensation plans and other similar plans and programs.

In evaluating executive officer pay, the Compensation Committee may retain the services of an independent compensation consultant or research firm and consider recommendations from our Chief Executive Officer and persons serving in managerial positions over a particular executive officer with respect to goals and compensation of the executive officer. The executive officers are not present or involved in deliberations concerning their compensation. Our Compensation Committee assesses the information it receives in accordance with its business judgment. All decisions with respect to executive compensation, other than compensation for our Chief Executive Officer, are first approved by our Compensation Committee and then submitted, together with the Compensation Committee's recommendations, to our board of directors for final approval. Our Chief Executive Officer is not present for the discussion of and approval of his compensation. However, some compensation elements for our Chief Executive Officer are approved as an integral part of a company-wide action or program.

We choose to pay the various elements of compensation discussed in order to attract, retain and motivate our high quality executive talent, reward annual performance and provide incentive for the achievement of intermediate and long-term strategic goals.

We believe that the compensation of our executives (and their functional or business teams) should reflect their success in achieving key objectives and individual performance factors. The key objectives broadly include:

(1) establishing and executing on product development program milestones within planned budgetary expenditures and timelines;

(2) securing adequate funds to achieve program objectives, to maintain our solvency and to moderate financial risk;

(3) expanding our preclinical product pipeline through creation of novel proprietary products, by utilization of technology, or by acquiring/ in-licensing new preclinical or clinical products and technology;

(4) creating corporate partnerships, contracts, collaborations and in-licensing or out-licensing products and technologies to achieve strategic objectives;

(5) submitting and receiving satisfactory results from regulatory submissions and interactions with regulators;

(6) establishing long-term corporate expertise and competencies in key activities;

(7) developing a strong intellectual property position enhancing the value of our product candidates and technologies; and

(8) increasing short-term and long-term stockholder value.

Key individual factors for each executive include:

(1) the value of their unique skills and capabilities to support our short- and long-term performance;

(2) performance of their management responsibilities;

(3) leadership qualities in enhanced team performance;

(4) business judgment and execution skills;

(5) current compensation arrangements, especially in comparison to the compensation of other executives in similar positions in competitive companies within our industry and whether an increase in responsibilities and change in title is warranted;

(6) short- and long-term potential to enhance stockholder value; and

(7) contributions as a member of the executive management team.

Our allocation between currently paid cash compensation and longer term equity compensation is intended to balance the requirement for adequate base compensation to attract, retain and motivate highly skilled personnel, while providing equity incentives to maximize long-term value for our stockholders and thus for our employees. We provide cash compensation in the form of base salary and annual, discretionary incentive cash bonuses to reward performance against preset written goals and objectives (modified as needed due to changing circumstances). We provide non-cash compensation to reward performance against intermediate and long-term strategic goals and provide a basis for improved financial security for the employee if our stockholders and we have financial success.

The Role of Stockholder Say-on-Pay Votes

We provide our stockholders with the opportunity to cast a non-binding advisory vote on the compensation of our Named Executive Officers' and on the frequency with which this vote should be conducted in future years. During fiscal year 2012, our Named Executive Officers included our principal executive officer (Chief Executive Officer), our principal financial officer (Chief Financial Officer), and our three most highly compensated executive officers other than the principal executive officer and principal financial officer. In May 2012 at our Annual Meeting of Stockholders, based upon total shares voted, our stockholders approved our Named Executive Officers' compensation with a 94% affirmative vote and 89% of voters voted for a one year frequency for say-on-pay. Although the stockholder vote is non-binding, the Compensation Committee will consider the outcome of the vote when making future compensation decisions for Named Executive Officers. In addition, we will conduct future stockholder advisory votes on the compensation of our Named Executive Officers once every year, until the next required stockholder advisory vote on the frequency of future stockholder advisory votes on the compensation of our Named Executive Officers, which we will conduct no later than our 2018 Annual Meeting of Stockholders.

Compensation Risks

We believe that risks arising from our compensation policies and practices for our employees are not reasonably likely to have a material adverse effect on the Company. In addition, the Compensation Committee believes that the mix and design of the elements of executive compensation do not encourage management to assume excessive risks.

The Compensation Committee reviewed the elements of executive compensation to determine whether any portion of executive compensation encouraged excessive risk taking and concluded:

- significant weighting towards long-term incentive compensation discourages short-term risk taking;
- for most employees, base salary makes up a significant majority of cash compensation;
- goals are appropriately set to avoid targets that, if not achieved, result in a large percentage loss of compensation; and
- as a pharmaceutical product development company, we do not face the same level of short term risks associated with compensation for employees at other companies in rapidly changing markets.

Furthermore, compensation decisions include subjective considerations, which reduce the influence of formulae or objective factors on excessive risk taking.

Elements of Compensation

Elements of compensation for our executives generally include:

- base salary (typically subject to review and potential adjustment annually based on inflation factors, industry competitive salary levels, our ability to pay, and performance on corporate and individual goals);
- annual performance bonuses which are paid in cash and are based primarily on performance against preset written goals;
- equity compensation (which to date has been implemented using stock option awards);
- 401(k) plan Company matching contributions;
- health, disability and life insurance; and
- employment terms and conditions including severance and change in control provisions primarily delineated in individual employment contracts or employer offer letters and Company policies.

Base Salary

At hire, base salaries are set for our executives based on the scope of each executive's responsibilities, as well as their qualifications, breadth of experience, performance record in similar situations, depth and breadth of appropriate functional expertise and close match with position requirements. Competitive market compensation paid by similar companies in our industry for individuals with similar responsibilities is a fundamental consideration.

Shortly after the end of each fiscal year, the Compensation Committee conducts an annual review of base salaries and the overall compensation package as a basis for any adjustments. Annual adjustments, if any, are typically made effective retroactive to the first day of the new fiscal year. The basis for salary adjustments may include merit increases in the competitive marketplace, adjustments to move individuals toward our target penetration in the competitive salary range for similar positions, increased duties and responsibilities, and sustained superior performance against goals and in special assignments. Adjustments may be made during the fiscal year for promotions, for highly urgent competitive reasons, for sustained superior performance in new or special challenges or circumstances, and similar reasons (mid-year adjustments generally require unusual or special circumstances).

The Compensation Committee made recommendations and the board of directors approved base salary compensation for our executive officers for the fiscal year ended August 31, 2012 taking into account:

- our status as an early-stage product development company without revenues or meaningful cost sharing collaborative agreements;
- competitive levels of compensation; and
- our ability to pay at this stage of our funding capability.

In September 2011, following its usual practice, our Compensation Committee hired Virginia Keller, at the time an outside Human Resources consultant, to update the benchmark of our base salaries, annual incentive cash bonuses and equity compensation (stock option grants) against the Aon Radford Global Life Sciences survey, a well-established blinded industry compensation survey. In addition, our Compensation Committee considered individual performance and competitive salaries paid to executive officers of other biopharmaceutical/biotechnology companies similar in size, stage of development and other characteristics. In making its recommendations, the Compensation Committee took into account assessments and recommendations submitted by the person serving as the manager of a particular executive officer.

After consideration of competitive salary compensation factors, the board of directors acting on the recommendation of the Compensation Committee, increased the salaries of our Named Executive Officers.

Effective September 1, 2011, the base salary of Dr. Starr, our Chief Executive Officer, was increased to $356,807. This represents the 45th percentile for the comparative companies in the Aon Radford Global Life Sciences survey, a blinded industry compensation survey, grouping for companies with under 50 employees. This increase constituted a 3% increase in Dr. Starr's base salary.

Effective September 1, 2011, Ms. Tsuchimoto and Mr. Reichenberger both received 3% increases to their fiscal year 2011 base salaries, Mr. Daley received a 5.8% increase and Dr. Rioux received a 7.6% increase to equate their salaries to the 45th percentile for the comparative companies in the Radford survey grouping for companies with under 50 employees. These raises were to match merit increases among companies in the Radford survey and to maintain the relative position in the Radford survey grouping for companies with under 50 employees.

		Fiscal Year 2012 Annual Base Salary
Christopher M. Starr, Ph.D.	Chief Executive Officer and Director of Raptor Pharmaceutical Corp.	$ 356,807
Kim R. Tsuchimoto	Former Chief Financial Officer, Secretary and Treasurer (Currently VP, Finance)	$ 255,465
Ted Daley	President, Raptor Therapeutics	$ 265,458
Patrice P. Rioux., M.D., Ph.D.	Chief Medical Officer, Raptor Therapeutics	$ 321,041
Patrick Reichenberger	VP, Commercial Operations	$ 234,600

Annual Incentive Cash Bonus and Other Non-Equity Incentive Plan Compensation

All of our executive officers are eligible for annual and discretionary cash and stock option bonuses pursuant to their employment agreements.

Our Compensation Committee has implemented an annual performance program. Annual performance goals are determined and documented in writing at the beginning of each fiscal year for the Company as a whole (corporate goals) and for each executive (individual goals). Should there be a meaningful change in our situation, environment, or operating strategy, goals may be modified or new, more appropriate goals may be instituted upon the recommendation of our Compensation Committee and approval by our board of directors.

Performance against our corporate goals and the executive's individual goals is considered by our Compensation Committee in evaluating performance and as a significant contributing factor in determining all aspects of the compensation of our executives.

Goals are weighted in importance and are time-bound. When taken as a whole, goals are intended to be challenging goals which will have a meaningful impact on stockholder value, either immediately or as preparatory steps required for future achievements.

The achievement scores are desired to be measurable and quantifiable when appropriate. After judgmental evaluation of performance, achievement scores may be awarded which recognize partial performance of a goal or award additional score points for exceptional performance due to unanticipated challenges.

Fiscal Year 2012 Corporate Goals

Our corporate goals are separated by our major activities and are in the following order of importance.

Development of RP103 for Cystinosis

- File for marketing approval in the U.S. and the EU by March 2011;
- Respond to FDA/EMA comments to NDA/MAA filings within regulatory timeframes; and
- Establish a European headquarters with a General Manager hired in anticipation of EU launch by June 2011.

Development of RP103 for NASH and HD

- Commence our Phase 2b NASH clinical trial by March 2012; and
- Complete enrollment of our Phase 2/3 HD clinical trial in France by December 2011.

Finance

- Manage cash burn of $32 million and execute planned programs;
- Raise funds in order to end fiscal year 2012 with approximately $40 million to $42 million in cash; and
- Improve stockholder base by increasing stock ownership percentage of the target stockholder groups by 5 percentage points over the percentage at the start of fiscal year 2012.

An overall corporate achievement score of 78% was determined by our board of directors to reflect the achievement of the majority of the most important fiscal year 2012 goals as agreed in advance by the board of directors, including completion of key regulatory filings for our RP103 cystinosis program, commencement of our NASH Phase 2b clinical trial, complete enrollment of our Huntington's Disease Phase 2/3 clinical trial, and meeting critical financial objectives. Goals that were not met or only partially met were generally lower weighted goals, including a delay in the full enrollment of the HD clinical trial and the improvement of our stockholder base.

Fiscal Year 2012 Individual Goals

Our Chief Executive Officer's individual goals are identical to the corporate goals. Individual goals are proposed by each executive and reviewed by our Chief Executive Officer. After review and modification, if necessary, by our Compensation Committee, the goals are approved by our board of directors.

In fiscal year 2012, a significant percentage of the value of the individual executive officer's goals was based on our performance against his/her goals which directly support our corporate goals. During fiscal year 2012, each executive officer made a significant contribution to the achievement of our corporate goals. The remaining value is based on achievement of goals which are more focused on needed achievements within the executive's areas of responsibility.

Each organizational level in the Company has a target percentage of the annual base salary for annual incentive bonus awards. Such awards are granted at the sole discretion of our board of directors, and can be modified based on multiple factors including our available financial resources, our overall performance and others. Bonuses are pro-rated for the time of service within the year. An employee must still be in active service at the time of our board's determination to be eligible to be paid an annual incentive bonus.

Awards can vary up to 125% of the target percentage based on assessment of the achievement of meaningful additional goals or sustained superior performance in the conduct of duties and responsibilities in the employee's position.

The percentages are set at 100% achievement of applicable corporate and individual goals. The target bonus percentages for our Named Executive Officers are as follows: Dr. Starr 40% of his annual base salary; Ms. Tsuchimoto and Mr. Reichenberger 27.5% each; and Mr. Daley and Dr. Rioux 30% each.

Fiscal Year 2012 Goals for Christopher M. Starr, Ph.D., Our Chief Executive Officer

Dr. Starr's annual incentive bonus equals 40% of base salary (the base salary target percentage) times a corporate achievement score of 78% equaled an annual incentive bonus of $112,000. The corporate achievement score of 78% reflects the judgment of the board of directors that the majority of the fiscal year 2012 goals as agreed in advance were met, as described above.

Fiscal Year 2012 Goals for Kim R. Tsuchimoto, Our Former Chief Financial Officer, Secretary and Treasurer (Currently VP, Finance)

Ms. Tsuchimoto had a major role in the achievement of our corporate financial goals. In recognition of this factor, corporate financial goals account for 50% of Ms. Tsuchimoto's fiscal year 2012 annual incentive bonus, while individual goals account for the remaining 50%. Ms. Tsuchimoto's annual incentive bonus (which target was up to 27.5% of her base salary) for fiscal year 2012 totaled $65,000 and was based upon achievement of 90% of the corporate financial goals and on the achievement of 100% of the following individual goals: establishment of European tax structure; continued development of strategic financial model by major product programs in accordance with project plan; and merging two holding companies in order to reduce Delaware taxes by December 2011.

Fiscal Year 2012 Goals for Ted Daley, Our President of Raptor Therapeutics

Mr. Daley had a major role in the achievement of our corporate goals. In recognition of this factor, corporate program goals account for 85% of Mr. Daley's fiscal year 2012 annual incentive bonus, while individual goals account for the remaining 15%. Mr. Daley's annual incentive bonus (which target was up to 30% of his base salary) for fiscal year 2012 totaled $65,000 and was based upon achieving 88% of his corporate goals and 33% of the following individual goals (representing an achievement score percentage of 80%): continued development of strategic financial model by major product programs in accordance with project plan (achieved); and obtain a second out-license partner for Convivia (not achieved).

Fiscal Year 2012 Goals for Patrice Rioux, M.D., Ph.D., Our Chief Medical Officer of Raptor Therapeutics

Dr. Rioux had a major role in the achievement of our corporate goals. In recognition of this factor, corporate program goals account for 100% of Dr. Rioux's fiscal year 2012 annual incentive bonus. Dr. Rioux's annual incentive bonus (which target was up to 30% of his base salary) for fiscal year 2012 totaled $80,000 and was based upon achievement of 80% achievement of the corporate program goals (priority review was not obtained and HD full enrollment was

achieved later than the goal date of December 2011).

Fiscal Year 2012 Goals for Patrick Reichenberger, Our Vice President, Commercial Operations

Mr. Reichenberger had a minor role in the achievement of our corporate program goals but achieved significant individual goals. Corporate program goals account for 15% of Mr. Reichenberger's fiscal year 2012 annual incentive bonus, while individual goals account for 85%. Mr. Reichenberger's annual incentive bonus (which target was up to 27.5% of his base salary) for fiscal year 2012 totaled $50,000 and was based upon achieving 100% of his corporate program goal of establishing a European headquarters and hiring a European General Manager of European commercial operations and on achieving 100% of the following individual goals (representing an achievement score percentage of 85%): setting up a patient registry by August 2012; establishing a reimbursement HUB for RP103 (RaptorCares™) by August 2012; commencing implementation of early-access, named patient program for cystinosis patients; continuing development of strategic financial model by major product programs in accordance with project plan; completing a European distribution agreement.

In summary, after further qualitative discussion of the level of achievement for each Named Executive Officer, consideration of partial achievement of goals, assessments of adverse changes in environmental conditions which can change the difficulty of achievement of a goal, changes within the fiscal year of corporate operating strategy and priorities, and other factors, the board of directors awarded the following annual incentive bonuses to our Named Executive Officers:

Christopher M. Starr, Ph.D. Chief Executive Officer	$112,000
Kim R. Tsuchimoto, Former Chief Financial Officer	65,000
Ted Daley, President	65,000
Patrice P. Rioux, M.D., Ph.D., Chief Medical Officer	80,000
Patrick Reichenberger, VP, Commercial Operations	50,000

Equity Incentive Programs (Currently Based on Stock Options)

We believe that equity grants provided to our executive officers (and all members of our team) create a strong link to our long-term financial and equity market performance, create an ownership culture, and closely align the interests of our executive officers with the interests of our stockholders. Because of the direct relationship between the value of an equity award and the future market price of our common stock, we believe that granting equity awards is the best method of motivating executive officers to manage in a manner that is consistent with our stockholders' and our Company's interests. In addition, we believe that the continuous vesting feature of our equity grants promotes executive officer (and staff) retention because this feature provides an incentive of potentially increasing value to our executive officers during the vesting period.

In determining the size of equity grants to our executive officers, our Compensation Committee considered: our performance; the applicable executive officer's performance; comparative competitive levels of equity compensation for similar peer companies; the vesting of such awards; the number of shares available under our 2010 Equity Incentive Plan, or the 2010 Plan, and projected future needs to support future staff growth; the recommendations of management and consultants; and external data sources which support a comparative competitive analyses.

With respect to newly hired executives, our practice is to include equity compensation (currently based on stock option grants) as an integral part of the compensation package for inclusion in the executive's employment agreement. The compensation package, including the stock option grant, is approved by a unanimous written consent executed by our board of directors. The executive's stock option exercise price is based upon the closing price the day preceding the later of board approval or the executive's first day of employment.

Under the 2010 Plan, we were initially authorized 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. 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, August 31, 2011 and August 31, 2012 increases added 1,629,516, 1,778,459 and 2,528,407 shares, respectively, available for grant under the 2010 Plan. As of August 31, 2012, options to purchase 6,124,823 shares of our common stock were outstanding and 3,691,901 shares of our common stock remain available for future issuance under the 2010 Plan.

Stock Options. Stock options granted by us have an exercise price equal to the fair market value of our common stock on the day prior to grant, typically vest over a four-year period with 6/48ths vesting six months after the vesting commencement date and the remainder vesting ratably each month thereafter based upon continued employment or service, and generally expire ten years after the date of grant. Incentive stock options also include certain other terms necessary to assure compliance with the Internal Revenue Code of 1986, as amended, or the Code. In special, limited circumstances, we have granted stock options which vested 25% upon grant and 1/36th per month thereafter and expire 10 years from the grant date. Our annual grants to our non-employee directors vest 25% per quarter.

Restricted Stock and Restricted Stock Units. Our 2010 Plan authorizes us to grant restricted stock and restricted stock units. We have not issued restricted stock or restricted stock units under the 2010 Plan. The Compensation Committee reviews the relative advantages and disadvantages of restricted stock as a compensation alternative at each annual cycle and may issue restricted stock in the future depending on the analysis in the future.

In September 2011, our Compensation Committee analyzed our equity compensation program compared to equity compensation in comparative companies. This analysis was a continuation of analysis performed by our Compensation Committee and board in 2010 based on input from investors, from their own professional experience, and from our own comparative analysis, that our equity compensation program was not competitive in our segment of the biopharmaceutical industry.

A brief outline of the comparative equity compensation study follows: The study was based on equity compensation comparisons between a group of 23 comparative companies and us. The selected comparative companies were companies whose common stock is traded on the NASDAQ or the NYSE/AMEX stock exchanges, had market capitalizations between approximately $35 million and $350 million, and were in the biopharmaceutical industry at approximately the same stage of development (development stage with products in development but without a commercial product base) as us. Compensation and stock data was extracted from public records for the 23 comparative companies and for us. The data was used to calculate the fully diluted number of common shares outstanding for each company and for each company's equity compensation program. Additional data was extracted for chief executive officer cash compensation and for the accumulated deficit for each company.

The companies whose data was used in the fiscal year 2012 comparative company analysis were:

Acadia	Neurocrine Biosciences
Amicus Therapeutics	NeurogesX
Anadys Pharmaceuticals	Novavax
Arqule	Omeros
Avenir	Oncogenix Pharmaceuticals
Celidex Therapeutics	Oncothyreon
Cytokinetics	Peregrine Pharmaceuticals
CytRX Corporation	Stem Cells, Inc.
Dynavax Technologies	Sunesis
GenVec	Transcept Pharmaceuticals
Inovio Pharmaceuticals	Vical
	Zalicus

The primary measurement for each company was the percent calculated by the fully diluted equity compensation common share equivalents (primarily stock options, restricted stock and restricted stock units, and stock options exercised) divided by the total fully diluted common shares outstanding (primarily common stock outstanding, stock options outstanding and warrants outstanding).

In percent of equity compensation divided by fully diluted common stock equivalents outstanding, we ranked 20 of the 24 comparative companies (including us) in the study; our percentage was 6.9% and the median percent was 9.75%. In terms of the current market capitalization divided by the accumulated deficit, we ranked first in this approximate measurement of the efficiency of the company in creating stockholder value.

After consideration of this analysis, which reflects our less than competitive position compared to peer or comparative companies, our Compensation Committee recommended that actions should be initiated to improve over time our equity compensation position to approximate the median value of the equity compensation programs of our peers. In line with our equity award program initiated in October 2010, we continue our initiative of granting additional stock options (in a proportional manner based on the recently reviewed 2011 annual awards) to officers, directors and staff to increase the potential future value of their equity compensation.

Our Compensation Committee plans to review the competitive equity compensation position of our Company annually. Actions, if any, will be based on the performance of our Company and individuals, available stock options for equity compensation, an updated competitive analysis and other factors.

Fiscal Year 2012 Equity Compensation Award

On September 22, 2011, after a discussion of the equity compensation situation and alternatives, our board approved the recommendation of our Compensation Committee. With respect to our Named Executive Officers and our directors, our board awarded stock options to purchase up to the following number of shares at an exercise price of $5.13 per share, vesting 6/48ths on the 6 month anniversary of the grant date and 1/48th per month thereafter, with a 10 year expiry from grant date: Dr. Starr 345,048 shares; Ms. Tsuchimoto, Mr. Daley and Dr. Rioux 113,616 shares each; and Mr. Reichenberger 90,000 shares. In addition, our Named Executive Officers were awarded annual stock option grants to purchase up to the following number of shares: Dr. Starr 115,016 shares; Ms. Tsuchimoto, Mr. Daley and Dr. Rioux 37,872 shares each; and Mr. Reichenberger 20,000 shares. Dr. Starr did not participate in the discussion or approval of his option grant.

Also, the non-employee members of our board as of September 22, 2011 (Mr. Anderson, Dr. Bruhn, Dr. Franklin, Dr. Keltner, Mr. Sager, Mr. Samant and Mr. Walbert), were each granted options to purchase 90,000 shares with the same terms as executive officers, as outlined above. These grants were in addition to annual stock option grants to purchase up to the following number of shares: Dr. Franklin, Dr. Keltner and Mr. Sager 30,000 shares and Dr. Bruhn, Mr. Samant and Mr. Walbert 15,000 shares, all of which vest 25% upon grant and 1/36th per month thereafter.

The effect of the September 22, 2011 stock option grants was to make our equity compensation program more competitive by increasing the percentage of equity compensation to the fully diluted common share equivalents outstanding to 9.4% which was considered by the Company to be closer to the target of the 9.75% median percentage.

In summary, during the fiscal year ended August 31, 2012, our Named Executive Officers were awarded stock options in the amounts indicated below. All options granted to our Named Executive Officers are intended to be qualified stock options as defined under Section 422 of the Code to the extent possible.

Christopher M. Starr, Ph.D. Chief Executive Officer and Director	460,064
Kim R. Tsuchimoto, Former Chief Financial Officer, Secretary and Treasurer (currently VP, Finance)	151,488
Ted Daley, President	151,488
Patrice P. Rioux, M.D., Ph.D., Chief Medical Officer	151,488
Patrick Reichenberger, VP, Commercial Operations	110,000

Perquisites

Broad-based benefit plans are an integral component of competitive executive compensation packages. Our benefits include a 401(k) savings plan with the Company matching provisions (when such matching is financially viable), healthcare benefits such as medical, dental, and vision plans, and disability and life insurance benefits. We have no structured perquisite benefits, and do not provide any deferred compensation programs or supplemental pensions to any executives. In its discretion, our Compensation Committee may revise, amend or add to the executive's benefits if it deems it advisable.

During our fiscal year ended August 31, 2012, our executives did not receive any perquisites and were not entitled to benefits that are not otherwise available to all of our employees. In addition, we did not provide pension arrangements, post-retirement health coverage or similar benefits for our executives or employees.

Defined Contribution Plan

We maintain a qualified retirement plan pursuant to Code Sections 401(a) and 401(k) covering substantially all employees, subject to certain minimum age and service requirements, herein referred to as our 401(k) Plan. Our 401(k) Plan allows employees to make voluntary pre-tax contributions. The assets of the 401(k) plan are held in trust for participants and are distributed upon the retirement, disability, death or other termination of employment of the participant.

Employees who participate in our 401(k) Plan may contribute to their 401(k) account up to the maximum amount that varies annually in accordance with the Code. We also make available to 401(k) plan participants the ability to direct the investment of their 401(k) accounts in a well-balanced spectrum of various investment funds.

At our discretion, we provide for a 401(k) Company matching in the amount of 100% of the first 3% of salary that an employee defers and 50% of the next 2% of salary that an employee defers, in compliance with the Internal Revenue

Service's Safe Harbor rules.

Summary Compensation Table

The following table reports summary compensation information for the following individuals, referred to as our Named Executive Officers: (1) Raptor's principal executive officer (Chief Executive Officer) during our fiscal year ended August 31, 2012; (2) Raptor's principal financial officer (Chief Financial Officer) during our fiscal year ended August 31, 2012; and (3) our three most highly compensated executive officers other than the principal executive officer or principal financial officer who were serving as executive officers as of the end of our fiscal year ended August 31, 2012.

Name and Principal Position	Fiscal Year Ended August 31,	Salary ($)	Option Awards (1)($)	Non-Equity Incentive Plan Compensation ($)(2)	All Other Compensation ($)(3)	Total ($)
Christopher M. Starr, Ph.D.	2012	356,807	794,425	112,000	14,953	1,278,185
Chief Executive Officer and Director	2011	346,415	489,813	111,996	15,976	964,200
	2010	277,200	8,827	68,280	1,266	355,573
Kim R. Tsuchimoto	2012	255,465	278,325	65,000	13,739	612,529
Former Chief Financial Officer, Secretary and Treasurer (Currently, VP, Finance)	2011	248,024	166,982	52,404	11,816	479,226
	2010	240,800	11,968	68,100	1,286	322,154
Ted Daley	2012	265,458	286,623	65,000	14,848	631,929
President, Raptor Therapeutics	2011	250,432	182,800	61,839	12,013	507,084
	2010	240,800	25,992	78,100	1,234	346,126
.						
Patrice P. Rioux, M.D., Ph.D.	2012	321,041	278,390	80,000	15,738	695,169
Chief Medical Officer, Raptor Therapeutics	2011	298,480	160,437	67,681	13,436	540,034
	2010	283,208	47,074	25,000	1,678	356,960
Patrick Reichenberger (4)	2012	234,600	188,937	50,000	1,735	475,272
VP, Commercial Operations, Raptor Therapeutics	2011	153,333	49,289	47,969	553	223,465
	2010	–	–	–	–	–

(1) This column represents the dollar amount recognized for financial statement reporting purposes with respect to the fiscal years ended August 31, 2012, 2011 and 2010 for the fair value of the stock options granted to each of our Named Executive Officers since inception, in accordance with ASC Topic 718. For additional information on the valuation assumptions with respect to the fiscal years ended August 31, 2012, 2011 and 2010, please refer to the notes in our consolidated financial statements included elsewhere in the Form 10-K. These amounts reflect our accounting expense for these awards, and do not correspond to the actual value, if any, that will be realized by our Named Executive Officers.

(2) Cash bonuses for fiscal year 2012 include accruals of bonuses paid in October 2012 based upon milestones achieved by us for the fiscal year ended August 31, 2012. Cash bonuses for fiscal year 2011 include accruals of bonuses paid in September 2011 based upon milestones achieved by us for the fiscal year ended August 31, 2011. Cash bonuses for fiscal year 2010 include accruals of bonuses paid in October 2010 based upon milestones achieved by us for the period March 1, 2010 through August 31, 2010 of $41,580 for Dr. Starr, $30,100 for Ms. Tsuchimoto and $30,100 for Mr. Daley. Also in consideration of his agreement to cancel the last two potential milestone stock option bonuses in his April 2009 offer letter, Dr. Rioux was paid a $25,000 bonus in November 2010. Also included in the bonuses for fiscal year 2010 are bonuses paid in March 2010, based upon milestones achieved by us during the period from September 1, 2009 through February 28, 2010 of $26,700 to Dr. Starr, $38,000 to Ms. Tsuchimoto and $38,000 to Mr. Daley. In addition, Mr. Daley earned a $10,000 bonus in fiscal year 2010 resulting from the execution of a licensing agreement with Uni Pharma for the development of Convivia™ in Taiwan in July 2010 pursuant to his employment agreement.

(3) All Other Compensation includes 401(k) matching funded by us, life insurance premiums paid by us where the executive is the beneficiary and employee-taxable commuting benefits.

(4) Mr. Reichenberger commenced his employment on January 3, 2011.

Employment Agreements

Dr. Starr entered into an employment agreement with our wholly owned subsidiary, Raptor Discoveries, in May 2006. Dr. Starr's employment agreement described below is currently still in effect.

Dr. Starr's employment agreement had an initial term of three years commencing on May 1, 2006, and automatically renews for additional one year periods unless either party under such agreement notifies the other that the term will not be extended. Under his agreement, Dr. Starr is entitled to an annual salary of $150,000, which may be increased from time to time in the discretion of our board of directors, and stock options to purchase 58,281 shares of our common stock at an exercise price of $2.83 per share, which vested over three years with a six month cliff vest and expires 10 years from grant date. Dr. Starr's annual salary is subject to annual review and potential increase by our board of directors. In addition, he is eligible for annual bonuses based upon our annual bonus compensation program. Information regarding Dr. Starr's annual salary and bonus received during the year ended August 31, 2012 are described below under the headings "Annual Incentive Cash Bonuses" and "Equity Incentive Programs (Currently Based on Stock Options)" above. Dr. Starr's employment agreement was amended effective as of January 1, 2009 for purposes of bringing his employment agreement into compliance with the applicable provisions of Section 409A of the Code and the Treasury Regulations and interpretive guidance issued thereunder.

In September 2012, we appointed Georgia L. Erbez as our Chief Financial Officer and, we entered into an employment agreement with Ms. Erbez, or the Erbez Employment Agreement, dated September 10, 2012. The Erbez Employment Agreement has an initial term of three years commencing on September 10, 2012, and renews automatically for successive one year periods, unless either party provides notice to the other terminating the agreement. Under the Erbez Employment Agreement, Ms. Erbez is entitled to an annual salary of $330,000, the amount of which may be increased from time to time in the discretion of our board of directors, and stock options to purchase 190,000 shares of our common stock at the closing price on September 8, 2012, the business day preceding the date of grant. These stock options vest 6/48ths on the six-month anniversary of such grant and 1/48th per month thereafter and expire ten years from date of grant. In addition, Ms. Erbez is eligible for annual and discretionary cash bonuses as determined by our board of directors, provided, however, that Ms. Erbez must be employed on the date any such bonus actually is paid in order to be eligible to receive such bonus. The annual discretionary bonus has a target payment of 40% of Ms. Erbez's base salary for the year in question.

On September 10, 2012, our wholly-owned subsidiary, Raptor Therapeutics, entered into an employment agreement with Julie A. Smith naming her its Executive Vice President, Strategy, and Chief Operating Officer. The agreement provides for similar terms as the Erbez Employment Agreement, except for the following terms. Under the agreement, Ms. Smith is entitled to an annual salary of $350,000, the amount of which may be increased from time to time in the discretion of our board of directors, and stock options to purchase 190,000 shares of our common stock at the closing price on September 8, 2012, the business day preceding the date of grant. Further, Raptor Therapeutics will reimburse Ms. Smith for reasonable relocation expenses, not to exceed in the aggregate, $50,000, and commuting expenses related to the performance of Ms. Smith's duties until the earlier of August 31, 2013 or the date Ms. Smith moves her primary residence to the San Francisco Bay Area.

Kim R. Tsuchimoto entered into an employment agreement with our wholly owned subsidiary, Raptor Discoveries, in May 2006, or the Prior Tsuchimoto Employment Agreement. The Prior Tsuchimoto Employment Agreement was in effect throughout the fiscal year ended August 31, 2012. As of September 10, 2012, Ms. Tsuchimoto was appointed our Vice President, Finance, and we entered into a new employment agreement with Ms. Tsuchimoto, or the Current Tsuchimoto Employment Agreement.

The Prior Tsuchimoto Employment Agreement had an initial term of three years commencing on May 1, 2006, and automatically renewed for additional one year periods unless either party under such agreement notifies the other that the term will not be extended. Under the Prior Tsuchimoto Employment Agreement, Ms. Tsuchimoto was entitled to an annual salary of $160,000, which could be increased from time to time in the discretion of our board of directors, and stock options to purchase 58,281 shares of our common stock at an exercise price of $2.57 per share, which vested over three years with a six month cliff vest and expires 10 years from grant date. The Prior Tsuchimoto Employment Agreement provided that Ms. Tsuchimoto's annual salary was subject to annual review and potential increase by our

board of directors. In addition, Ms. Tsuchimoto was eligible for annual bonuses based upon our annual bonus compensation program. Information regarding Ms. Tsuchimoto's annual salary and bonus received during the year ended August 31, 2012 are described below under the headings "Annual Incentive Cash Bonuses" and "Equity Incentive Programs (Currently Based on Stock Options)" above. The Prior Tsuchimoto Employment Agreement was amended effective as of January 1, 2009 for purposes of bringing her employment agreement into compliance with the applicable provisions of Section 409A of the Code and the Treasury Regulations and interpretive guidance issued thereunder.

The Current Tsuchimoto Employment Agreement reflects Ms. Tsuchimoto's appointment as the Company's Vice President, Finance and provides for similar terms as the Prior Tsuchimoto Employment Agreement, except for the following terms. Under the Current Tsuchimoto Employment Agreement, Ms. Tsuchimoto is entitled to an annual salary of $255,465, the amount of which may be increased from time to time in the discretion of our board of directors. Ms. Tsuchimoto was paid a one-time bonus of $20,000 pursuant to the agreement. In addition, all of Ms. Tsuchimoto's remaining unexercised options granted on May 26, 2006, to purchase 47,021 shares of our common stock remain exercisable until expiration of the options on May 26, 2016, so long as Ms. Tsuchimoto remains employed with us through the six month anniversary of the agreement.

On September 7, 2007, our wholly-owned subsidiary, Raptor Therapeutics, entered into an employment agreement with Ted Daley for a term of 18 months which automatically renews for additional one year periods unless either party under such agreement notifies the other that the term will not be extended. Under Mr. Daley's agreement, Mr. Daley is entitled to an annual salary of $150,000 and stock options to purchase 34,969 shares of our common stock at an exercise price of $2.23 per share, which vest over four years with a six month cliff vest and expire 10 years from grant date. In August 2008, RPC's compensation committee recommended, and its full board of directors approved, a stock option grant to Mr. Daley for the purchase of 23,313 shares of our common stock at an exercise price of $1.88 per share, which vests 6/48ths upon the six-month anniversary of the grant date and 1/48th per month thereafter and expires ten years from the grant date. Mr. Daley's 2008 stock options were granted in order to increase his initial employment stock option grant to be equal to the stock option grants of our other executive officers. Mr. Daley's annual salary is subject to annual review and potential increase by our board of directors. Pursuant to Mr. Daley's employment agreement, Mr. Daley is eligible to receive certain cash bonuses based on triggering events related to the successful development of our Convivia™ product development program. In addition, Mr. Daley is eligible for annual bonuses based upon our annual bonus compensation program. Information regarding Mr. Daley's annual salary and bonuses received during the year ended August 31, 2012 are described below under the headings "Annual Incentive Cash Bonuses" and "Equity Incentive Programs (Currently Based on Stock Options)" above. Mr. Daley's employment agreement was amended effective as of January 1, 2009 for purposes of bringing his employment agreement into compliance with the applicable provisions of Section 409A of the Code and the Treasury Regulations and interpretive guidance issued thereunder.

In April 2009, Raptor Therapeutics executed an employment arrangement with Dr. Rioux with an annual base salary of $280,000 and stock options to purchase 34,969 shares of our common stock at an exercise price of $0.85 per share, which vest over four years with a six month cliff vest and expire 10 years from grant date. Dr. Rioux's annual salary is subject to annual review and potential increase by our board of directors. In addition, Dr. Rioux is eligible for annual bonuses based upon our annual bonus compensation program. Information regarding Dr. Rioux's annual salary and bonuses received during the year ended August 31, 2012 are described below under the headings "Annual Incentive Cash Bonuses" and "Equity Incentive Programs (Currently Based on Stock Options)" above.

In January 2011, Raptor Therapeutics executed an employment arrangement with Patrick Reichenberger with an annual base salary of $230,000. Mr. Reichenberger also earned a $10,000 sign-on bonus and is eligible for an annual bonus based upon our annual bonus compensation program. Mr. Reichenberger was granted stock options to purchase up to 120,000 shares of our common stock at an exercise price of $3.52 which vest over four years with a six month cliff vest and expire 10 years from grant date. Information regarding Mr. Reichenberger's annual salary and bonuses received during the year ended August 31, 2012 are described below under the headings "Annual Incentive Cash Bonuses" and "Equity Incentive Programs (Currently Based on Stock Options)" above.

If Dr. Starr's employment is constructively terminated or terminated by us without cause, including in the event of a change of control, then he will be entitled to continue to receive his base salary, bonus and other benefits for a period of 12 months from the date of termination. In addition, if the termination occurs after a change of control, all of Dr. Starr's vested and unvested options to purchase our stock are immediately exercisable in full. If Ms. Erbez's, Ms. Smith's or Ms. Tsuchimoto's employment is constructively terminated or terminated by us without cause not during the 12 months following a change in control, she will be entitled to continue to receive her base salary and other certain benefits for 12 months after such termination. In addition, all of her vested options or stock appreciation rights with respect to our common stock will remain exercisable until the first anniversary of the termination of her employment, and all shares of our common stock owned by her will immediately be released from any and all resale or repurchase rights restrictions. If Ms. Erbez, Ms. Smith or Ms. Tsuchimoto is terminated without cause or is constructively terminated by us within the 12 months following a change in control, in addition to the payments described in the preceding two sentences, all of her unvested equity and equity-based awards (including stock options) will vest immediately and will remain exercisable until the second anniversary of the termination of employment. Additionally, she will be entitled to a lump sum payment equal to the average of the annual bonus payments received by her in the two years preceding the year of termination. If Mr. Reichenberger's, Dr. Rioux's or Mr. Daley's employment is constructively terminated or terminated by us without cause, including in the event of a change of control, then such officer will be entitled to continue to receive his base salary and certain other benefits for a period of six months from the date of termination.

If any officer's employment is terminated for cause, by death or due to a voluntary termination, we shall pay to such officer, or in the case of termination due to death, his or her estate, the compensation and benefits payable through the date of termination or, in the case of Ms. Erbez, Ms. Smith and Ms. Tsuchimoto, if such officer's employment is terminated by death, through the third month anniversary of termination.

If any officer's employment is terminated due to disability, we shall pay to such officer the compensation and benefits payable through the date of termination. Except for Mr. Reichenberger and Dr. Rioux, we shall continue to pay such officer's salary for three months following such termination, at the end of which time such officer may be entitled to receive short-term and eventually long-term disability benefits, subject to the terms of and pursuant to our then current disability insurance plans. In addition, Dr. Starr is entitled a prorated bonus for three months following such termination.

Annual Incentive Cash Bonuses

In September 2012, our Compensation Committee recommended and our board approved cash bonuses for achievements during our fiscal year ended August 31, 2012. Corporate goal achievements included: Filing the NDA and MAA by March 2012, responding to FDA and EMA requests within regulatory deadlines, obtaining priority review from the FDA/EMA, establish European headquarters by May 2012, initiate the NASH Phase 2b trial by November 2011, complete HD Phase 2/3 enrollment by December 2011, manage cash burn of $32 million for planned operations for fiscal year 2012, finance the company in order to maintain a cash balance of approximately $40 to $42 million at end of fiscal year 2012 and improve stockholder base by increasing the ownership percentage of target stockholder groups by 5% over the percentage at the start of fiscal year 2012. Along with corporate goals, each officer (other than Dr. Starr who was only measured against corporate goals) was benchmarked against individual goal achievement. The following cash bonuses were approved and paid to our Named Executive Officers in October 2012: Dr. Starr $112,000; Ms. Tsuchimoto $65,000; Mr. Daley $65,000; Dr. Rioux $80,000; and Mr. Reichenberger $50,000.

Stock Option Grants and Exercises During our Fiscal Year Ended August 31, 2012

Grants of Plan-Based Awards Table

The following table sets forth information concerning stock option grants made during our fiscal year ended August 31, 2012 to our Named Executive Officers named in the "Summary Compensation Table" above. The fair value information in the far right column is for illustration purposes only and is not intended to predict the future price of our common stock. The actual future value of such stock options will depend on the market value of our common stock.

Name	Grant Date	Estimated Future Payouts Under Non-Equity Incentive Awards			Estimated Future Payouts Under Equity Incentive Plan Awards			All Other Stock Awards: Number of Shares of Stock Or Units (#)	All Other Option Awards: Number of Securities Underlying Options (#) (1)	Exercise or Base Price of Option Awards ($/Sh)	Grant Date Fair Value of Stock and Option Awards ($) (2)
		Threshold ($)	Target ($)	Maximum ($)	Threshold ($)	Target ($)	Maximum ($)				
Christopher M. Starr, Ph.D.	9/22/2011	—	—	—	—	—	—	—	460,064	5.13	457,213
Kim R. Tsuchimoto	9/22/2011	—	—	—	—	—	—	—	151,488	5.13	168,466
Ted Daley	9/22/2011	—	—	—	—	—	—	—	151,488	5.13	168,466
Patrice P. Rioux, M.D., Ph.D.	9/22/2011	—	—	—	—	—	—	—	151,488	5.13	168,466
Patrick Reichenberger	9/22/2011	—	—	—	—	—	—	—	110,000	5.13	113,468

(1) Stock options vest 6/48ths on the six-month anniversary of the grant date and 1/48th per month thereafter. All options expire 10 years from their respective grant dates.

(2) This column represents the dollar amount recognized for financial statement reporting purposes with respect to our year ended August 31, 2012 for the fair value of the stock options granted to each of our Named Executive Officers in the fiscal year ended August 31, 2012 in accordance with ASC Topic 718. These amounts reflect our accounting expense for these awards, and do not correspond to the actual value, if any, that will be realized by our Named Executive Officers.

Outstanding Equity Awards at August 31, 2012

The following table sets forth certain information with respect to outstanding stock option awards of our Named Executive Officers for the fiscal year ended August 31, 2012.

	Option Awards					
Name	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable	Equity Incentive Plan Awards: Number of Securities Underlying Unexercised Unearned Options (#)	Option Exercise Price ($)	Option Expiration Date
Christopher M. Starr, Ph.D.	38,111 (1)	–	–	2.83	5/26/2016
	34,089 (3)	12,661 (3)	–	2.02	3/9/2020
	82,666 (3)	89,858 (3)	–	2.97	10/12/2020
	237,224 (5)	107,830 (5)	–	3.54	11/22/2020
	105,429 (2)	354,635 (2)	–	5.13	9/22/2021
Kim R. Tsuchimoto	46,832 (1)	–	–	2.83	5/26/2016
	15,281 (2)	–	–	2.57	6/14/2017
	13,782 (3)	5,118 (3)	–	2.02	3/9/2020
	27,219 (3)	29,589 (3)	–	2.97	10/12/2020
	78,110 (5)	35,506 (5)	–	3.54	11/22/2020
	34,714 (2)	116,774 (2)	–	5.13	9/22/2021
Ted Daley	34,969 (2)	–	–	2.23	9/10/2017
	23,313 (2)	–	–	1.88	8/12/2018
	13,782 (3)	5,118 (3)	–	2.02	3/9/2020
	27,219 (3)	29,589 (3)	–	2.97	10/12/2020
	78,110 (5)	35,506 (5)	–	3.54	11/22/2020
	34,714 (2)	116,774 (2)	–	3.54	9/22/2021
Patrice P. Rioux, M.D., Ph.D.	3,643 (2)	5,829 (2)	–	0.85	4/16/2019
	11,656 (4)	–	–	1.66	3/30/2020
	11,656 (4)	–	–	3.05	6/28/2020
	27,219 (3)	29,589 (3)	–	2.97	10/12/2020
	78,110 (5)	35,506 (5)	–	3.54	11/22/2020
	34,714 (2)	116,774 (2)	–	5.13	9/22/2021
Patrick Reichenberger	47,499 (2)	72,501 (2)	–	3.52	1/4/2021
	25,207 (5)	84,793 (5)	–	5.13	9/22/2021

(1) Stock options vest 6/36ths

on the six month anniversary of grant date and 1/36th per month thereafter.

(2) Stock options vest 6/48ths on the six month anniversary of grant date and 1/48th per month thereafter.

(3) Stock options vest 6/48ths on grant date and1/48th per month thereafter.

(4) Stock options vest 100% upon grant date.

(3) Stock options vest 25% immediately and the remaining 75% vests 1/36th per month.

Options Exercised

The following table sets forth the number and value of options exercised during our fiscal year ended August 31, 2012 for each of the Named Executive Officers.

Name	Option Awards	
	Number of Shares Acquired on Exercise(#)	Value Realized on Exercise ($)(1)
Christopher M. Starr, Ph.D. (2)	20,170	67,199
Kim R. Tsuchimoto (2)	14,236	32,416
Patrice P. Rioux, M.D., Ph.D.	25,497	136,409

(1) The value realized upon exercise of stock options reflects the price at which shares acquired upon exercise of the stock options were sold or valued for income tax purposes, net of the exercise price for acquiring the shares.

(2) The transactions for each of Dr. Starr and Ms. Tsuchimoto were made pursuant to a Rule 10b5-1 trading plan adopted by the reporting person.

Executive Payments Upon Termination

Change in control arrangements are designed to retain executives and provide continuity of management in the event of a change in control. These agreements are described in more detail elsewhere in this Annual Report on Form 10-K, including the sections titled "Annual Incentive Cash Bonuses," "Employment Agreements," and "Equity Incentive Programs (Currently Based on Stock Options)" above.

The following table quantifies the amounts that we would owe each of our executive officers upon each of the termination triggers discussed above under "Employment Agreements," assuming a termination date of August 31, 2012:

Christopher M. Starr, Ph.D.
Chief Executive Officer and Director

Executive Benefits and Payments Upon Termination	Disability	Death	Termination Without Cause or Constructive Termination	CIC Termination Without Cause or Constructive Termination (1)
Base Salary	$89,202 (3)	$ –	$ 356,807 (2)	$ 356,807 (2)
Short-Term Incentive	28,000 (4)	–(4)	112,000 (5)	112,000 (5)
Value of Unvested Equity Awards and Accelerated Vesting Stock	–	–	–	1,841,393 (6)
Total	$117,202	$ –	$ 468,807	$ 2,310,200

(1) "CIC" means change in control, as defined in the officer's

employment agreement.

(2) 12 months base salary.

(3) 3 months base salary.

(4) Pro rata bonus.

(5) Full cash bonus otherwise payable.

(6) Vesting of all stock options granted in accordance with ASC Topic 718. This amount reflects our accounting expense for these awards, and does not correspond to the actual value, if any, that will be realized by the officer.

Kim R. Tsuchimoto
Former Chief Financial Officer, Secretary and Treasurer (Currently VP, Finance)

Executive Benefits and Payments Upon Termination	Disability	Death	Termination Without Cause or Constructive Termination	CIC Termination Without Cause or Constructive Termination (1)
Base Salary	$ 63,866 (3)	$ –	$ 255,455 (2)	$ 255,455 (2)
Short-Term Incentive	16,250 (4)	–(4)	65,000 (5)	65,000 (5)
Value of Unvested Equity Awards and Accelerated Vesting Stock	–	–	–	616,019 (6)
Total	$ 80,116	$ –	$ 320,455	$ 936,474

(1) "CIC" means change in control, as defined in the officer's employment agreement.
(2) 12 months base salary.
(3) 3 months base salary.
(4) Pro rata bonus.
(5) Full cash bonus otherwise payable.
(6) Vesting of all stock options granted in accordance with ASC Topic 718. This amount reflects our accounting expense for these awards, and does not correspond to the actual value, if any, that will

be realized by the officer.

Ted Daley
President, Raptor Therapeutics

Executive Benefits and Payments Upon Termination	Disability	Death	Termination Without Cause or Constructive Termination	CIC Termination Without Cause or Constructive Termination (1)
Base Salary	$ 66,365	$ –	$ 132,729 (2)	$ 132,729 (2)
Short-Term Incentive	16,250 (4)	–(4)	65,000 (5)	65,000 (5)
Value of Unvested Equity Awards and Accelerated Vesting Stock	–	–	–	616,019 (6)
Total	$ 82,615	$ –	$ 197,729	$ 813,748

(1) "CIC" means change in control, as defined in the officer's employment agreement.
(2) 6 months base salary.
(3) 3 months base salary.
(4) Pro rata bonus.
(5) Full cash bonus otherwise payable.
(6) Vesting of all stock options granted in accordance with ASC Topic 718. This amount reflects our accounting expense for these awards, and does not correspond to the actual value that will be recognized by the officer.

Patrice P. Rioux, M.D., Ph.D.
Chief Medical Officer, Raptor Therapeutics

Executive Benefits and Payments Upon Termination	Disability	Death	Termination Without Cause or Constructive Termination		CIC Termination Without Cause or Constructive Termination (1)	
Base Salary	$ –	$ –	$ 160,521	(2)	$ 160,521	(2)
Short-Term Incentive	–	–	–		–	
Value of Unvested Equity Awards and Accelerated Vesting Stock	–	–	–		613,086	(3)
Total	$ –	$ –	$ 160,521		$ 773,607	

(1) "CIC" means change in control, as defined in the officer's employment agreement.
(2) 6 months base salary.
(3) Vesting of all stock options granted in accordance with ASC Topic 718. This amount reflects our accounting expense for these awards, and does not correspond to the actual value that will be recognized by the officer.

Patrick Reichenberger
Vice President, Commercial Operations, Raptor Therapeutics

Executive Benefits and Payments Upon Termination	Disability	Death	Termination Without Cause or Constructive Termination		CIC Termination Without Cause or Constructive Termination (1)	
Base Salary	$ –	$ –	$ 117,300	(2)	$ 117,300	(2)
Short-Term Incentive	–	–	–		–	
Value of Unvested Equity Awards and Accelerated Vesting Stock	–	–	–		515,451	(3)
Total	$ –	$ –	$ 117,300		$ 632,751	

(1) "CIC" means change in control, as defined in the officer's employment agreement.
(2) 6 months base salary.
(3) Vesting of all stock options granted in accordance with ASC Topic 718. This amount reflects our accounting expense for these awards, and does not correspond to the actual value that will be recognized by the officer.

Director Compensation

Effective September 1, 2011 through August 31, 2012, non-employee members of our board of directors received the following cash compensation:

Director Position	Annual Cash Compensation(1)
Non-Employee Directors, Excluding Chairman of the Board of Directors	$ 36,600
Chairman of the Board of Directors	$ 68,000
Audit Committee Chair	$ 14,400
Audit Committee (Non-Chair members)	$ 8,400
Compensation Committee Chair	$ 12,400
Compensation Committee (Non-Chair members)	$ 7,400
Corporate Governance and Nominating Committee Chair	$ 12,000
Corporate Governance and Nominating Committee (Non-Chair members)	$ 7,000

The following table sets forth the total compensation paid by us to each of our non-employee directors during our fiscal year ended August 31, 2012.

Name	Fees Earned or Paid in Cash ($)	Option Awards($)(1)	Total($)
Raymond W. Anderson (2)	58,400	291,756	350,156
Suzanne L. Bruhn, Ph.D. (3)	56,000	197,055	253,055
Richard L. Franklin, M.D. Ph.D. (4)	52,000	304,446	356,446
Llew Keltner, M.D., Ph.D. (5)	57,000	315,095	372,095
Erich Sager (6)	68,000	291,756	359,756
Vijay B. Samant (7)	51,000	197,055	248,055
Timothy P. Walbert (8)	52,400	197,055	249,455

(1) Amounts shown do not reflect compensation actually received by a director, but reflect the dollar amount compensation cost recognized by us for financial statement reporting purposes for the fiscal year ended August 31, 2012, in accordance with ASC Topic 718, and thus may include amounts from awards granted in and prior to the fiscal year ended August 31, 2012. The assumptions underlying the calculations pursuant to ASC Topic 718 are set forth under Note 7 of the Notes to Consolidated Financial Statements, beginning on page F-30 of our Consolidated Financial Statements in the Form 10-K.

(2) Mr. Anderson had 380,619 options outstanding as of August 31, 2012, of which 255,929 were exercisable.

(3) Dr. Bruhn had 180,000 options outstanding as of August 31, 2012, of which 56,873 were exercisable.

(4) Dr. Franklin had 289,969 options outstanding as of August 31, 2012, of which 165,279 were exercisable.

(5) Dr. Keltner had 286,100 options outstanding as of August 31, 2012, of which 151,939 were exercisable.

(6) Mr. Sager had 478,483 options outstanding as of August 31, 2012, of which 353,793 were exercisable.

(7) Mr. Samant had 180,000 options outstanding as of August 31, 2012, of which 56,873 were exercisable.

(8) Mr. Walbert had 180,000 options outstanding as of August 31, 2012, of which 56,873 were exercisable.

For their services as members of our board of directors, each non-employee board member received stock options to purchase up to 30,000 shares of our common stock in September 2011, which vest 25% per quarter and expire 10 years from the date of grant. The exercise price of such options were $5.13 per share. In addition, in September 2011, based upon the research discussed in "Equity Incentive Programs (Currently Based on Stock Options)" above, each non-employee director received options to purchase 90,000 shares of our common stock, which vests 6/48ths upon the six month anniversary of the grant date and 1/48th per month thereafter with a ten year expiry. The exercise price of such options were $5.13 per share.

Compensation Committee Interlocks and Insider Participation

During our fiscal year ended August 31, 2012, our Compensation Committee consisted of Dr. Bruhn, Mr. Anderson, Mr. Samant and Mr. Walbert. No member of our Compensation Committee is currently or has been at any time one of our officers or employees, is or was a participant in a "related party" transaction under Item 404 of Regulation S-K promulgated by the SEC ("Regulation S-K") in the last completed fiscal year, or has served as a member of the board of directors, board of trustees or compensation committee of any entity that has one or more officers serving as a member of our board of directors or our Compensation Committee. None of our executive officers serves or in the past has served as a member of the board of directors or compensation committee of any entity that has one or more of its executive officers serving on our board of directors or our Compensation Committee. Prior to establishing the Compensation Committee, our full board of directors made decisions relating to compensation of our executive officers.

Compensation Committee Report

The Compensation Committee has reviewed and discussed the Compensation Discussion and Analysis required by Item 402(b) of Regulation S-K of the SEC's rules and regulations with management and, based on such review and discussions, the Compensation Committee recommended to the board of directors that the Compensation Discussion and Analysis be included in our Annual Report on Form 10-K for the fiscal year ended August 31, 2012.

Compensation Committee,
Suzanne Bruhn, Ph.D., Chair
Raymond W. Anderson
Vijay Samant
Timothy Walbert

This foregoing compensation committee report is not "soliciting material," is not deemed "filed" with the SEC, and shall not be deemed incorporated by reference by any general statement incorporating by reference this Annual Report on Form 10-K into any filing of ours under the Securities Act of 1933, as amended, or under the Exchange Act, except to the extent we specifically incorporate this report by reference.

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, 2012 (in thousands):

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	6,125	5.87	6,125
Equity compensation plans not approved by stockholders	–	–	–
Total	6,125	5.87	6,125

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth, as of October 18, 2012, 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, 2012, 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 Wanger Asset Management, LLC (3)	3,536,000	–	6.8%
Christopher M. Starr, Ph.D. (4)	1,265,482	566,112	2.4%
Ted Daley	329,889	234,984	*
Patrick Reichenberger	89,581	89,581	*
Patrice P. Rioux, M.D, Ph.D.	216,712	191,215	*
Kim R. Tsuchimoto	239,397	238,815	*
Raymond W. Anderson	278,428	278,428	*
Suzanne L. Bruhn, Ph.D.	72,498	72,498	*
Richard L. Franklin, M.D., Ph.D.	187,778	187,778	*
Llew Keltner, M.D., Ph.D.	176,623	176,623	*
Erich Sager	492,214	376,292	*
Vijay B. Samant	72,498	72,498	*
Timothy P. Walbert	72,498	72,498	*
All executive officers and directors as a	2,955,308	2,037,711	5.7%

group (11 persons)

* 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 18, 2012, 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 51,753,696 shares outstanding as of October 18, 2012.

(3) Entities affiliated with Columbia Wanger Asset Management, LLC collectively hold an aggregate of 3,536,000 shares of our common stock. The principal business address for Columbia Wanger Asset Management, LLC is 227 West Monroe Street, Suite 3000, Chicago, IL 60606.

(4) Includes 699,370 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 566,112 shares of our common stock held by Dr. Starr directly.									

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

Review, Approval or Ratification of Transactions with Related Persons

Our Audit Committee has primary responsibility for reviewing and approving in advance or ratifying all related party transactions. In conformance with SEC regulations, we define related persons to include our executive officers, our directors and nominees to become a director of our Company, any person who is known to us to be the beneficial owner of more than 5% of any class of our voting securities, any immediate family member of any of the foregoing persons, and any firm, corporation or other entity in which any of the foregoing persons is employed, is a general partner or in which such person has a 5% or greater beneficial ownership interest.

Our Audit Committee reviews, approves and oversees any related party transactions due to the potential for such transactions to create a conflict of interest. A conflict of interest occurs when an individual's private interest interferes, or appears to interfere, with our interests. It is our general policy to approve or ratify related person transactions only when our board of directors or a committee of our board of directors determines that the transaction is in, or is not inconsistent with, our and our stockholders' best interests, including situations where the Company may obtain products or services of a nature, quantity or quality, or on other terms, that are not readily available from alternative sources or when the transaction is on terms comparable to those that could be obtained in arm's length dealings with an unrelated third party.

Since September 1, 2011, 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.

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

Indebtedness of Directors and Executive Officers

None of our directors or executive officers or associates of any director or executive officer is or at any time since September 1, 2011 has been indebted to us.

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, our Chief Executive Officer. 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, 2012 and 2011.

	Year Ended August 31,	
Description of Services Provided by Burr Pilger Mayer, Inc.	2012	2011
Audit Fees*	$219,440	$222,222
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.	166,842	82,160
Tax Compliance Fees: These services relate to the preparation of federal, state and foreign tax returns and other filings.	38,631	28,839
Tax Consulting and Advisory Services: These services primarily relate to the area of tax strategy and minimizing Federal, state, local and foreign taxes.	259,113	–
All Other Fees	–	–

* Audit Fees for August 31, 2012 includes unbilled audit fees for the year ended August 31, 2012, which is estimated to be $166,400. Audit Fees for August 31, 2011 includes audit fees for the year ended August 31, 2011, billed and paid during the year ended August 31, 2012 totaling $171,600.

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-49 of this Annual Report on Form 10-K.

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

1) Index list to Consolidated Financial Statements:

2) Schedule II is included on F-49 of this report. All other schedules are omitted because they are not required or the required information is included in the financial statements or notes thereto.

Exhibits

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

Exhibit Index

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 Report on Form 8-K, filed on October 10, 2006).
3.6	Amendment to Bylaws of the Registrant (incorporated by reference to Exhibit 3.6 to the Registrant's Annual Report on Form 10-K, filed on March 29, 2007).
3.7	Charter Amendment for TorreyPines (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
3.8	Certificate of Merger between Raptor Pharmaceuticals Corp., ECP Acquisition, Inc. and TorreyPines Therapeutics, Inc. (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed on October 9, 2009).
3.9	Amendment to Bylaws of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed on May 14, 2012).
4.1	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).
412	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 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).

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 (incorporated by reference to Exhibit 10.17 to the Registrant's Annual Report on Form 10-K, filed on November 14, 2011).
10.18#**	Offer Letter from Raptor Therapeutics Inc. dated April 6, 2011 for Kathy Powell (incorporated by reference to Exhibit 10.18 to the Registrant's Annual Report on Form 10-K, filed on November 14, 2011).
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 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)).
10.46**	Cooperative Research and Development Agreement for Extramural-PHS Clinical Research dated December 15, 2011 between the U.S. Department of Health and Human Services, as represented by the National Institute of Diabetes and Digestive and Kidney Diseases, an institute or center of the National Institutes of Health, and Raptor Therapeutics Inc. (incorporated by reference to Exhibit 10.1of the Registrant's Quarterly Report on Form 10-Q filed on April 9, 2012).
10.47**#	Employment Agreement dated April 15, 2012 between Raptor Pharmaceuticals Europe B.V. and Henk Doude van Troostwijk (incorporated by reference to Exhibit 10.1of the Registrant's Quarterly Report on

	Form 10-Q filed on July 10, 2012).
10.48**	Intellectual Property Platform Contribution Transaction License Agreement, dated April 16, 2012, between RPTP European Holdings, C.V. and Raptor Therapeutics Inc. (incorporated by reference to Exhibit 10.2of the Registrant's Quarterly Report on Form 10-Q filed on July 10, 2012).
10.49+#	Employment Agreement, dated September 10, 2012, between the Registrant and Georgia Erbez (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed on September 12, 2012).
10.50+#	Employment Agreement, dated September 10, 2012, between Raptor Therapeutics and Julie A. Smith (incorporated by reference to Exhibit 10.2 to the Registrant's Current Report on Form 8-K, filed on September 12, 2012).
10.51+#	Employment Agreement, dated September 10, 2012, between the Registrant and Kim R. Tsuchimoto (incorporated by reference to Exhibit 10.3 to the Registrant's Current Report on Form 8-K, filed on September 12, 2012).
10.52#	Employment Agreement, dated September 25, 2012, between the Registrant and Kathy Powell (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed on October 1, 2012).
21.1†	Subsidiaries of the Registrant.
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 Georgia Erbez, Chief Financial Officer, Secretary and Treasurer.
32.1†	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director, and of Georgia Erbez, 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.

\+ Certain information omitted pursuant to a request for confidential treatment filed with 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 13, 2012

By: /s/ Georgia Erbez
Georgia Erbez
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 Georgia Erbez, 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 13, 2012
/s/ Georgia Erbez Georgia Erbez	Chief Financial Officer, Secretary and Treasurer (Principal Financial Officer and Principal Accounting Officer)	November 13, 2012
/s/ Raymond W. Anderson Raymond W. Anderson	Director	November 13, 2012
/s/ Suzanne L. Bruhn Suzanne L. Bruhn, Ph.D.	Director	November 13, 2012
/s/ Richard L. Franklin Richard L. Franklin, M.D., Ph.D.	Director	November 13, 2012
/s/ Llew Keltner Llew Keltner, M.D., Ph.D.	Director	November 13, 2012
/s/ Erich Sager Erich Sager	Director	November 13, 2012
/s/ Vijay B. Samant	Director	November 13, 2012

Vijay B. Samant		
/s/ Timothy P. Walbert Timothy P. Walbert	Director	November 13, 2012

Financial Statements

The following consolidated financial statements of Raptor Pharmaceutical 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:

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, 2012 and 2011, and the related consolidated statements of comprehensive loss, stockholders' equity (deficit), and cash flows for each of the three years in the period ended August 31, 2012, and the cumulative amounts from September 8, 2005 (inception) to August 31, 2012. Our audits also included the financial statement schedule listed in the Index to this Annual Report on Form 10-K at Part IV Item 15(a)(1). These consolidated financial statements and financial statement schedule 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, 2012 and 2011, and the results of their operations and their cash flows for each of the three years in the period ended August 31, 2012, and the cumulative amounts from September 8, 2005 (inception) to August 31, 2012 in conformity with accounting principles generally accepted in the United States of America. Also, in our opinion, the related financial statement schedule, when considered in relation to the consolidated financial statements taken as a whole, presents fairly, in all material respects, the information set forth therein.

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. 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.

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, 2012, 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 13, 2012 expressed an unqualified opinion thereon.

/s/ Burr Pilger Mayer, Inc.
San Francisco, California
November 13, 2012

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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, 2012, 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, 2012, 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, 2012 and

2011, and the related consolidated statements of comprehensive loss, stockholders' equity (deficit), and cash flows for each of the three years in the period ended August 31, 2012, and for the cumulative amounts from September 8, 2005 (inception) to August 31, 2012, and our report dated November 13, 2012 expressed an unqualified opinion on those consolidated financial statements and the related financial statement schedule.

/s/ Burr Pilger Mayer, Inc.
San Francisco, California
November 13, 2012

Raptor Pharmaceutical Corp.
(A Development Stage Company)
Consolidated Balance Sheets
(In thousands, except for per share data)

ASSETS	August 31, 2012	2011
Current assets:		
Cash and cash equivalents	$23,580	$15,172
Restricted cash	169	114
Short-term investments	15,307	0
Prepaid expenses and other	3,111	416
Total current assets	42,167	15,702
Intangible assets, net	2,205	3,251
Goodwill	3,275	3,275
Fixed assets, net	403	77
Deposits	105	105
Deferred offering costs	134	152
Total assets	$48,289	22,562
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Liabilities		
Current liabilities:		
Accounts payable	$1,601	$847
Accrued liabilities	2,652	2,249
Common stock warrant liability	17,266	23,575
Deferred rent	14	24
Capital lease liability – current	8	4
Total current liabilities	21,541	26,699
Capital lease liability - long-term	13	10
Total liabilities	21,554	26,709
Commitments and contingencies		
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value, 15,000 shares authorized, zero shares issued and outstanding	0	0
Common stock, $0.001 par value, 150,000 shares authorized 50,568 and 35,569 shares issued and outstanding as at August 31, 2012 and 2011, respectively	51	36
Additional paid-in capital	143,380	73,817
Accumulated other comprehensive income (loss)	(50)	2
Deficit accumulated during development stage	(116,646)	(78,002)
Total stockholders' equity (deficit)		