Raptor Pharmaceutical Corp
Form 10-K
March 17, 2014
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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 December 31, 2013

[ ] 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.)

5 Hamilton Landing, Suite 160, Novato, CA 94949
(Address of principal executive offices) (Zip Code)

(415) 408-6200
(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, $0.001 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 [X] No [ ]
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. [X]

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

Large accelerated filer [ ] Accelerated filer [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 June 30, 2013 (the last business day of the registrant's most recently completed second quarter) was $536.4 million.

Indicate the number of shares outstanding of each of the registrant's classes of common stock, as of the latest practicable date: 62,479,286 shares common stock, par value $0.001, outstanding as of February 28, 2014.

The documents incorporated by reference are as follows:

None.

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RAPTOR PHARMACEUTICAL CORP.

2013 Form 10-K Annual Report

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FORWARD-LOOKING STATEMENTS

Change in Fiscal Year End

On December 4, 2012, the board of directors of Raptor Pharmaceutical Corp., or the "Company", approved a change to the Company's fiscal year end from August 31 to December 31. This Annual Report on Form 10-K includes the financial information for 2013 which refers to the period from January 1 to December 31, 2013. The Company previously filed a report on Form 10-K/T, as amended, for the four-month period from September 1, 2012 to December 31, 2012, or the Transition Period. References in this Annual Report on Form 10-K to fiscal years prior to 2013 refer to the period from September 1 through August 31 of such year.

Forward-Looking Statement

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 statements regarding 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 prospective matters, 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, developments 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, 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 for any other reason.

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PART I

ITEM 1: BUSINESS

You should read the following discussion in conjunction with our consolidated financial statements as of December 31, 2013, and the notes to such consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Unless otherwise stated or the context requires otherwise, for the period from and after the effective time of the 2009 Merger (as described below under "Corporate Information"), all references in this Annual Report on Form 10-K to the "Company," "we," "our," "us," "Raptor" and similar references refer to the company formerly known as TorreyPines Therapeutics, Inc. and now known as Raptor Pharmaceutical Corp. and its direct and indirect wholly-owned subsidiaries Raptor Pharmaceuticals Inc., or Raptor Pharmaceuticals, Raptor European Products, LLC, RPTP European Holdings C.V., Raptor Pharmaceuticals Europe B.V., Raptor Pharmaceuticals France SAS and Raptor Pharmaceuticals Germany GmbH.

Overview

We are a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases.

Our first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules, or PROCYSBI, received marketing approval from the U.S. Food and Drug Administration, or FDA, on April 30, 2013 for the management of nephropathic cystinosis in adults and children six years and older. On September 6, 2013, the European equivalent, PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received a Community or EU marketing authorization from the European Commission, or EC, as an orphan medicinal product for the management of proven nephropathic cystinosis. The EU marketing authorization allows us to commercialize PROCYSBI in the 28 Member States of the EU plus Norway, Liechtenstein and Iceland (which are not EU Member States but are part of the European Economic Area, or EEA). PROCYSBI received seven years and 10 years of market exclusivity as an orphan drug in the U.S. and the EU, respectively. We commenced commercial sales of PROCYSBI in the U.S. in June 2013 and plan to launch PROCYSBI in the EU in the first half of 2014.

As of December 31, 2013, 165 U.S. commercial patients were taking PROCYSBI at a Wholesale Acquisition Cost, or WAC, price for PROCYSBI of $15,562.50 per bottle of 250 75-mg capsules and $3,735.00 per bottle of 60 25-mg capsules, resulting in an estimated average annualized price of $250,000 per patient in the U.S. In September 2013, we executed an agreement to participate in the U.S. State Medicare/Medicaid rebate program, which will be reflected in our net revenues in mandatory rebates on reimbursements for patients receiving state Medicaid insurance coverage.

Cysteamine Mechanism of Action

Cysteamine, or 2-aminoethanethiol, is a highly-reactive molecule generated in the cell during the metabolism of cysteine. Cysteamine is used to construct the key enzymatic cofactor involved in energy produced from sugars and lipids. Cysteamine's uniquely reactive properties result in many physiological effects when given exogenously in pharmaceutical doses.

- Antioxidation – Cysteamine is known to increase levels of a key cellular antioxidant, glutathione. Glutathione is composed of the amino acids gamma-glutamate, cysteine and glycine. The availability of cysteine is the major rate-limiting factor in glutathione production. Cysteamine may release cysteine in the circulation, or from within the cell. Cysteamine has been shown to activate the NRF2 pathway, which leads to the increased expression of a wide variety of proteins involved in antioxidant which may help to reduce oxidative stress in CNS and mitochondrial disorders..

- Heat shock response induction – Heat shock proteins, or HSPs, are chaperones that play an important role in protein-protein interactions such as folding and assisting in the establishment of proper protein conformation. Proper protein folding may also prevent unwanted protein aggregation. By helping to stabilize partially unfolded proteins, HSPs aid in transporting proteins across membranes within the cell. HSPs are typically produced by the cell in response to stress or injury, or other metabolic imbalance. HSPs are part of a cell's mechanism for protein

maintenance. The presence of cysteamine within a cell has been shown to increase transcription of certain HSPs that are key components to the cell's ability to maintain the integrity of proteins.

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- Anti-fibrosis – Cysteamine inhibits formation of three cross-links in collagen that exacerbate fibrotic pathology:
  - gamma-glutamyl peptide bonds, formed by transglutaminase
  - oxidized lysyl-lysine conjugates, formed by lysyl oxidase
  - inter-chain disulfide bonds

Cysteamine also inhibits transcription of a variety of collagens and basement membrane-related proteins.

- Metal chelation – In vitro studies have shown that cysteamine chelates metals, including copper, zinc and iron. High doses of cysteamine can lead to copper depletion, implying that chelation effects also occur in vivo.

- Induction of DNA repair mechanisms – Cysteamine, for over sixty years, has been known to mitigate the effects of radiation.

PROCYSBI®

PROCYSBI is an approved therapy for the management of nephropathic cystinosis, a rare, life-threatening metabolic lysosomal storage disorder that causes the rapid, toxic accumulation of cystine in all cells, tissues and organs in the body. PROCYSBI capsules contain cysteamine bitartrate in the form of innovative microspheronized beads that are individually coated to create delayed and extended-release properties, allowing patients to maintain consistent therapeutic systemic drug levels over a 12-hour dosing period. The enteric-coated beads are pH sensitive and bypass the stomach for dissolution and absorption in the more alkaline environment of the proximal small intestine. Randomized controlled clinical trials and extended treatment with PROCYSBI therapy demonstrated consistent cystine depletion as monitored by levels of the biomarker (and surrogate marker), white blood cell cystine.

About Nephropathic Cystinosis

There are an estimated 500 patients reported with cystinosis living in the U.S. and 2,000 worldwide. Nephropathic cystinosis comprises 95% of known cases of cystinosis. Elevated cystine leads to cellular dysfunction and death. Cystinosis is progressive, eventually causing irreversible tissue damage and multi-organ failure, including kidney failure, blindness, muscle wasting and premature death. Nephropathic cystinosis is usually diagnosed in infancy after children present with symptoms including markedly increased urination, thirst, dehydration, gastrointestinal distress, failure to thrive, rickets, photophobia and kidney symptoms specific to Fanconi syndrome. Management of cystinosis requires lifelong therapy. Untreated, the disease is usually fatal by the end of the first decade of life.

Cystine depletion is the only approved treatment for nephropathic cystinosis. Committed adherence and persistence to cystine depletion therapy is critical to achieve optimal clinical outcomes. Failure to adhere to prescribed dosing of cystine depletion therapy results in poor symptomatic control as cystine accumulates intracellularly and patients consequently experience disease progression, including kidney insufficiency leading to dialysis and kidney transplantation, muscle wasting and in most cases, premature death. Even brief interruptions in daily therapy can permit toxic accumulation of cystine, exposing tissues to renewed, progressive deterioration.

In October 2013, we executed a collaboration agreement with DaVita Clinical Research to screen blood samples from U.S. patients with end-stage renal disease in an effort to identify patients with unrecognized late-onset nephropathic cystinosis.

RP103 Clinical Development

Huntington's Disease

Huntington's disease, or HD, formerly called Huntington's chorea, is a rare, inherited neurodegenerative disorder. HD causes neuronal degeneration in the cerebral cortex and basal ganglia, which play a key role in movement and behavior control. The cumulative damage to these areas results in the hallmark symptoms of HD: chorea (jerky movements), neuropsychiatric symptoms, loss of executive functioning and dementia. HD is caused by an autosomal dominant mutation in a gene called huntingtin, which means any child of an affected person typically has a 50% chance of inheriting the disease. The huntingtin gene encodes a protein that is also called "huntingtin." Expansion of a CAG triplet repeat within the huntingtin gene results in a mutant form of the protein, which gradually damages cells in the brain. HD manifests as a triad of movement, cognitive and psychiatric symptoms which progress gradually in

severity over 15-20 years, eventually causing severe physical and mental disability and potentially early death. The symptoms of HD usually become evident between the ages 35-44 years, but the onset can also begin from childhood to late life (>75 years). The treatment options for HD patients are very limited, with no approved drugs that modify disease course. Recommended treatment strategies consist of drugs for symptomatic relief of chorea (with tetrabenazine, XENAZINE®, approved by FDA) and mood swings associated with HD as well as a variety of physical, occupational and dietary therapies.

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RP103 as a treatment for Huntington's disease

RP103, enteric-coated delayed-release cysteamine bitartrate, is currently being evaluated as a potentially disease modifying treatment for HD. Centre Hospitalier Universitaire, or CHU, d'Angers, France, is conducting the Phase 2/3 clinical trial of RP103. This 36-month randomized trial comprises an 18-month blinded, placebo-controlled phase followed by an 18-month open-label phase in which all patients transition to RP103. The primary endpoint of the trial is change from the baseline of the Total Motor Score, or TMS, of the Unified Huntington's Disease Rating Scale, or UHDRS. TMS, a validated rating scale, is comprised of approximately 15 different tests that evaluate gross and small motor function in patients with HD. Chorea is a single measurement included in the TMS. The trial commenced in October 2010, with full enrollment achieved in June 2012. The study enrolled primarily Stage 1 patients showing early disease symptoms with a UHDRS TMS, Score ≥ 5, Total Functional Capacity, or TFC, > 10 and a CAG repeat > 38. Due to the length of the study and the characteristic continuous progression of the disease, patients were allowed to continue their normal medication regime including taking antidepressants and tetrabenazine. Tetrabenazine is approved as a treatment for chorea associated with HD.

In February 2014, we announced top line results from the planned 18-month analysis of the study. A total of 96 patients with HD were randomized to treatment with RP103 or placebo. A total of 89 patients completed the initial 18-month phase. A mixed model analysis of all 96 patients enrolled in the trial showed slower progression of TMS in patients treated with RP103 versus those patients on placebo after 18 months treatment (4.51 vs. 6.68 respectively, p=0.19). While the results did not reach statistical significance, an overall positive trend was observed.

Patients were not randomized in the study based on concomitant medications. To assess whether the TMS results were impacted by the effect of tetrabenazine on chorea. In 66 patients not taking tetrabenazine (32 under placebo and 34 under RP103), the results showed a total motor score of 2.84 points of progression in the RP103 treatment arm versus 6.78 for the placebo group (p= 0.03). The lower change of the TMS score for patients treated with RP103 represents a clinically significant slower rate of decline of more than 50% compared to those patients receiving placebo.

There were no new or unusual variations from RP103's clinical safety profile with 48 of 52 patients experiencing at least one adverse event, or AE, during the 18-month interim evaluation versus 38 of 44 under placebo. There were slightly more patients under RP103 than under placebo reporting at least one gastrointestinal AE (61.5% RP103 versus 45.5% placebo), mostly nausea, vomiting, abdominal pain, constipation, headache and breath odor. There were five patients treated with RP103 who experienced serious adverse events, or SAEs, compared with four patients treated with placebo. As of the 18-month time point, seven patients discontinued treatment, six in the RP103 arm and one in placebo. Three patients receiving RP103 discontinued for serious adverse events including one for lymphopenia, one for repetitive faintness and one for elevated liver enzymes. One SAE in the placebo group, anxiety, resulted in discontinuation.

Under our amended collaboration agreement with CHU d'Angers, we supply RP103 and placebo capsules for the clinical trial and open-label extension study and fund the third-party statistical analysis of clinical trial data in exchange for regulatory and commercial rights to the clinical trial data. Clinical expenses of the study are covered by a grant from the Programme Hospitalier de Recherche Clinique, which is funded by the French government.

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 with these topline results.

RP103 Mechanism in HD

In HD, mutant Htt aggregate formation and processing leads to neuronal, mitochondrial and cellular dysfunction and death. Cysteamine may induce several beneficial stress responses, including the production of glutathione, that in aggregate reduce cellular oxidative stress. Through inhibition of several intracellular enzymes, such as transglutaminase, cysteamine inhibits protein aggregation, which are known to form in HD. Cysteamine also increases transcription and production of certain heat shock proteins, which may assist in clearing or repairing misfolded Htt and other proteins in neuronal cells. Cysteamine and its dimer cystamine have been shown in preclinical studies to increase levels of brain derived neurotropic factor, or BDNF, by assisting in the excretion and production of the protein. BDNF is induced by cortical neurons and helps support survival, growth and differentiation

of new neurons and synapses. Two master genes, huntingtin, or Htt, and huntingtin-associated protein, or Hap1, govern BDNF axonal transport and secretion. Expression of the Bdnf gene is reduced in both Alzheimer's and Huntington's disease patients and HD patients are believed to be deficient in BDNF. The Bdnf gene may play a role in the regulation of stress response and in the biology of mood disorders. Finally, cysteamine's metal-chelating properties may assist in removing excess copper, a metal that has shown increased accumulation in brains of people with HD as well as other neurodegenerative disorders.

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Non-alcoholic Fatty Liver Disease in Children

Non-alcoholic fatty liver disease, or NAFLD, is the hepatic component of metabolic syndrome and is associated with deposition of triglycerides in the hepatocytes in individuals who do not consume alcohol in amounts generally considered to be harmful to the liver. NAFLD is commonly associated with elements of metabolic syndrome, such as obesity, diabetes mellitus and hypertriglyceridemia. Additional factors include family history of diabetes and high blood lipids in people who are not obese. NAFLD refers to a spectrum of conditions ranging from simple fat accumulation in the liver to steatohepatitis, cirrhosis and hepatocellular carcinoma.

- Non-alcoholic fatty liver, or NAFL – A benign condition with simple fat accumulation within liver cells (hepatic steatosis).
- Non-alcoholic steatohepatitis, or NASH – An aggressive form of NAFLD characterized by hepatic steatosis and inflammation with hepatocyte injury (ballooning) with or without fibrosis.
- Cirrhosis – 15% to 25% of patients with NASH progress to cirrhosis with consequential complications over 10 to 20 years. Cirrhosis is characterized by the replacement of healthy liver tissue with fibrosis and scar tissue, leading to loss of liver function. NASH cirrhosis is a risk factor for development of hepatocellular carcinoma, or HCC.

NAFLD prevalence is increasing along with the rise of obesity. Advanced NAFLD is now among the most common reasons why patients are referred for liver transplantation.

According to the World Gastroenterology Organization Global Guidelines, the prevalence of NAFLD in children is about 15% in the U.S. and western countries. NAFLD is underdiagnosed in children due to lack of recognition, screening or appreciation of associated complications by healthcare providers. Children may not be recognized as obese during office visits and age-appropriate norms for body mass index may go unacknowledged. Liver disease is screened by measuring serum alanine aminotransferase, or ALT, and aspartate aminotransferase, or AST, starting at 10 years old in obese children and those with a body mass index of 85th to 94th percentile with other risk factors. Currently there are no drug treatment options for NAFLD. Disease management strategies include recommendations for lifestyle changes in diet, exercise and weight reduction.

RP103 as a treatment for NAFLD in children

In 2010, we conducted a Phase 2a clinical trial to examine RP103 as a treatment for NAFLD and NASH in children. Results of this trial with a prototype of RP103 showed that patients exhibited a marked decline in serum transaminase levels during the treatment period of 26 weeks. Seven of 11 juvenile NAFLD patients entering the study with elevated ALT and AST achieved more than 50% reduction in ALT and six of 11 reduced their ALT levels to normal range. AST levels were also improved, with patients averaging 41% reduction by the end of the 26-week treatment phase. This reduction in serum liver enzymes was largely sustained during the 6-month post-treatment monitoring phase. Other important liver function markers showed positive trends, suggesting improvements in hepatic histopathology. These markers included reduced levels of cytokeratin 18, or CK-18, a potential serum marker of disease activity in NASH and NAFLD, which decreased 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 and NAFLD.

The ALT and AST reductions achieved in the Phase 2a trial were consistent with ALT and AST reductions seen in patients who achieved a 10% weight loss, although body mass index did not change significantly during both the treatment and post-treatment phases in the 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 six months of treatment.

In June 2012, we announced the dosing of the first patient in a Phase 2b clinical trial – Cysteamine Bitartrate Delayed-Release for the Treatment of Non-alcoholic Fatty Liver Disease in Children, or CyNCh, which is evaluating the safety and efficacy of RP103 as a potential treatment of NAFLD in children. The clinical trial is being conducted under a Cooperative Research and Development Agreement, or CRADA, with the National Institute of Diabetes and Digestive and Kidney Diseases, or NIDDK, part of the National Institutes of Health, or NIH. Upon full enrollment in January 2014, 169 patients were enrolled at 10 U.S. centers in the NIDDK-sponsored NAFLD Clinical Research Network.

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Raptor and NIDDK share the costs of conducting 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 RP103 treatment reverses liver tissue damage caused by NAFLD as measured by changes in NAFLD Activity Score, or NAS, a histological rating scale of disease activity (based on scoring lobular inflammation, hepatocyte ballooning and steatosis from a liver biopsy), in conjunction with no worsening of liver tissue fibrosis. Secondary endpoints include blood markers for liver health including ALT and AST as well as safety and tolerability. Top line clinical trial results for this study are anticipated in the first half of 2015.

RP103 Mechanism in NAFLD

Cysteamine's potent antioxidative properties, including the production of glutathione, may reduce oxidative damage that results from excessive accumulation of fats in liver cells. In addition, cysteamine's anti-fibrotic activity, including inhibiting the production of transglutaminase, may play a role in stabilizing or even reducing the liver fibrosis that occurs in severe cases of NAFLD.

Mitochondrial disorders including Leigh syndrome

Leigh syndrome is a severe neurological disorder caused by genetic defects in mitochondrial or nuclear DNA affecting respiratory chain function that typically results in death within the first decade of life. The condition causes increased production of reactive oxygen species that disrupt mitochondrial electron transport and affect cellular function in a variety of tissues. Typically observed during the first year of life, Leigh syndrome is characterized by a failure to thrive, lack of coordination, involuntary and sustained muscle contraction, muscle wasting and multiple organ failure. The incidence of Leigh syndrome in the U.S. is estimated to be 1 in 40,000 newborns.

RP103 as a treatment for mitochondrial disorders including Leigh Syndrome

We have submitted an investigational new drug application, or IND, to the FDA for the clinical development of RP103 as a potential treatment for Leigh syndrome and other mitochondrial disorders. RP103 potentially increases mitochondrial glutathione which may act as a scavenging agent of reactive oxygen species and thereby reduce the mitochondrial oxidative stress typically associated with these disorders.

The clinical trial is designed to evaluate the safety, tolerability and efficacy of RP103 in patients with genetically confirmed Leigh syndrome as well as patients with other mitochondrial disorders. The clinical plan includes an open label, 24 week, Phase 2/3 study in 32 patients (up to a maximum of 64 patients). Patients with Leigh syndrome are expected to comprise two-thirds of the enrolled population in the study. Initiation of the clinical trial is planned for the first half of 2014 at four clinical sites in the U.S. Based on an adaptive design statistical plan, we will conduct interim analyses after 12 patients and again after 24 patients have completed the study to determine final sample size. The primary endpoint of the study will be the change from baseline in the Newcastle Pediatric Mitochondrial Disease Scale, or NPMDS, at 24 weeks. Secondary endpoints will include observations of myopathy, dystonia, seizures, motor development, dyskinesia, quality of life and activities of daily living. Interim results from the clinical trial are expected by the end of 2014.

Other Clinical-Stage Product Candidates

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.

We own the intellectual property portfolio pertaining to Convivia, including method of use and formulation patents. 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 programs include our cysteamine dioxygenase, or ADO, program, to improve treatment of diseases for which cysteamine is therapeutic and our HepTide™ program to treat hepatocellular carcinoma and other cancers susceptible to induced lysosomal storage.

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Future Activities

We expect that our near-term efforts will be focused on:

- Increasing sales of PROCYSBI and providing comprehensive reimbursement and adherence support to commercial patients in the U.S.;
- Negotiating pricing and reimbursement in specific European countries and launching PROCYSBI in the first EU country in the first half of 2014;
- Filing a New Drug Submission, or NDS, for PROCYSBI with Health Canada in the second half of 2014;
- Continuing a clinical trial to evaluate PROCYSBI in cystinosis patients that are cysteamine-naïve, as well as other supporting trials in underdeveloped markets;
- Developing select global markets with significant numbers of known cystinosis patients;
- Screening for undiagnosed and unidentified late-onset nephropathic cystinosis;
- Supporting clinical programs and developing regulatory strategies for the use of RP103 as a potential treatment of HD in adults;
- Supporting our clinical trials of RP103 for the potential treatment of NAFLD in children;
- Supporting clinical programs evaluating RP103 for the potential treatment of mitochondrial disorders;
- Supporting our novel preclinical programs and identifying promising in-licensing candidates; and
- Continuing the development of our RP103 clinical pipeline in other indications.

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 5 Hamilton Landing, Suite 160, Novato, CA 94949. Our phone number is (415) 408-6200.

As of February 28, 2014, there were 62,479,286 shares of our common stock outstanding. Our common stock currently trades on the NASDAQ Global Market under the ticker symbol "RPTP."

Corporate History

In September 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 focused on developing clinical-stage drug product candidates through to commercialization and, in connection with the 2009 Merger, these two subsidiaries became our subsidiaries. In December 2012, the two principal subsidiaries were merged and currently operate under the name Raptor Pharmaceuticals Inc. 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 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."

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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 the University of California, San Diego, or UCSD, to issued and pending patents covering composition of matter, or COM, method of use, or MOU, and composition for use, or CFU, 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 certain 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 certain 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 Patent 1919458 (expires 2027), represents a CFU patent and covers the use of any composition of enterically coated cysteamine or cystamine, regardless of the specific formulation, for treating cystinosis two times a day.

Our cysteamine intellectual property to treat metabolic and neurodegenerative conditions

We also have a worldwide exclusive license from UCSD to U.S. Patent Nos. 7,994,226 and 8,263,662 (expire 2028) and MOU patents which cover cysteamine and related compounds for the potential treatment of NASH and NAFLD, respectively. Additionally, we have a worldwide exclusive license from UCSD to international patent application PCT/U.S. 2012/66288, a MOU patent covering the use of cysteamine and related compounds to treat ischemic injury. Our exclusive worldwide license from the Weizmann Institute includes U.S. Patent Nos. 6,794,414 and 6,355,690, MOU patents which cover 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 application 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 September 2012, we acquired exclusive worldwide 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.

In May 2013, we acquired exclusive world-wide rights to international patent application PCT/EP2011/068576, an MOU patent covering use of cysteamine and related compounds to treat MECP-2 associated disorders including Rett Syndrome, from the Technology Transfer Accelerator of South Eastern France (SATT Sud Est) that represents the French medical research organizations where the technology was invented, including the Institut Curie, INSERM and Aix-Marseille Université.

Trademarks

The trademark "Raptor" is registered in the U.S. and the EU, and applied for in certain other countries. We also own applications and registrations for various other marks in the U.S. and certain countries throughout the world. All third party trademarks identified in this Annual Report on Form 10-K belong to their respective owners.

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Regulatory Exclusivity

Orphan Drug Designation

We have been granted Orphan Drug Designation from the FDA for use of PROCYSBI to manage cystinosis, and the use of RP103 to potentially treat HD, pancreatic cancer and Batten Disease. The Orphan Drug Act of 1983 generally provides incentives, including marketing exclusivity, user fee waivers and tax benefits, to companies that undertake development and marketing of products to treat relatively rare diseases, which are defined as diseases for which there is a patient population of fewer than 200,000 persons in the U.S. or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. A drug that receives orphan drug designation may receive up to seven years of exclusive marketing in the U.S. for that indication, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity. A drug may be entitled to an additional six months of exclusive marketing if it satisfies the requirements for pediatric exclusivity; we have applied for this additional six-month pediatric extension for PROCYSBI.

PROCYSBI has also been granted Orphan Drug Designation and awarded 10 years of marketing exclusivity by the European Medicines Agency, or EMA, for treatment of cystinosis. We plan to submit an application to EMA for Orphan Drug Designation for RP103 for potential treatment of HD in the first half of 2014.

Competition

Cystinosis

Other than PROCYSBI, we are aware of two pharmaceutical products currently approved to treat cystinosis. Cystagon® (immediate-release cysteamine bitartrate capsules), is marketed as a systemic cystine-depleting therapy for cystinosis in the U.S. by Mylan Pharmaceuticals, and by Orphan Europe in markets outside of the U.S. Cystagon was approved by the FDA in 1994 and by EMA in 1997. Cystaran® (cysteamine ophthalmic solution) was approved by FDA in 2012 for treatment of corneal crystal accumulation in patients with cystinosis.

While we believe that PROCYSBI will continue to be well received in the market, Cystagon remains on the market and we expect it will compete with PROCYSBI for the foreseeable future. We are not aware of any pharmaceutical company with an active program to develop an alternative therapy for cystinosis. Academic researchers in the U.S. and Europe are pursuing potential cures for cystinosis through gene therapy and stem cell therapy, as well as pro-drug 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 available treatments to slow the progression of HD. There is only one approved treatment available for specific symptoms of HD, Xenazine® to treat uncontrollable movements (chorea) that result from the disease. There are several pharmaceutical companies pursuing potential cures and disease modifying 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 deficient BDNF with the goal of slowing disease progression.

Companies with HD product candidates in development include Auspex, Prana, NeuroSearch, Omeros, Teva, Eli Lilly & Co. and Pfizer. Several other companies have drug candidates in preclinical development. Additionally, nutritional supplements including creatine 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 and NAFLD

We are not aware of any currently approved treatment options for NASH or NAFLD. Weight loss, healthy diet, abstinence from alcohol and increased physical activity are typically suggested to slow the progression of NASH and NAFLD. There are numerous therapies being studied for NASH, including obeticholic acid, a farnesoid X receptor (FXR) agonist (Intercept Pharmaceuticals), lysyl oxidase-like 2 inhibitor (Gilead), PPAR alpha and delta agonist

(Genfit), Diacylglycerol acyl transferase-1 inhibitor (Novartis) and galectin inhibitor (Galectin), as well as anti-oxidants.

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ALDH2 Deficiency

We are not aware of any pharmaceutical products currently approved for ALDH2 deficiency, either in the U.S. or internationally. There are several non-prescription, nutritional supplements available which purport to mitigate the side effects that result from drinking by people with ALDH2 deficiency. 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 would 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, or FDCA, 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 extensive preclinical studies performed in accordance with the FDA's Good Laboratory Practice, or GLP, regulations;
- the submission to the FDA of a request for authorization to conduct clinical trials in an investigational new drug application, or IND, which must become effective before clinical trials may commence;
- approval by an independent institutional review board, or IRB, or ethics committee representing each clinical site before each clinical study may be initiated;
- completion of adequate and well-controlled human clinical trials to establish and confirm the safety and efficacy of a drug candidate for the proposed indication;
- completion of process validation, quality product release and stability;
- submission to the FDA of a new drug application, or NDA, for the drug candidate for marketing approval;
- potential review of the product application by an FDA advisory committee, where appropriate and if applicable;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facilities where the proposed product is produced to assess compliance with the FDA's current Good Manufacturing Practice, or cGMP, requirements; and
- review and approval of the NDA by the FDA before the product may be sold commercially.

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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 GCP or good clinical practices, 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 FDA, the IRB or the clinical study sponsor may suspend or terminate a clinical study at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Additionally, some clinical studies are overseen by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study. We may also suspend or terminate a clinical study based on evolving business objectives and/or competitive climate. 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.

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, detailed investigational new drug product information is submitted to the FDA in the form of an NDA requesting approval to market the product for one or more indications. Under federal law, the submission of most NDAs is subject to a substantial application user fee. Applications for orphan drug products are exempted from the NDA user fees, unless the application includes an indication for other than a rare disease or condition.

An NDA must include all relevant data available from pertinent preclinical and clinical studies, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational new drug product to the satisfaction of the FDA.

Once an NDA has been submitted, the FDA's goal is to review the application within 10 months after it accepts the application for filing, or, if the application relates to an unmet medical need in a serious or life-threatening indication, six months after the FDA accepts the application for filing. The review process is often significantly extended by FDA requests for additional information or clarification. In addition, an application may be referred to an advisory committee, which is a panel of independent experts, to review, evaluate and provide a recommendation to the FDA as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it typically follows such recommendations and considers them carefully when making approval decisions.

Before obtaining FDA approval for each product, the FDA typically will inspect the facility or facilities where the product is manufactured and will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP requirements. Following approval, each product manufacturing establishment must be registered with the FDA and its quality control and manufacturing procedures must continue to conform and adhere at all times to the FDA's cGMP regulations. 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.

Even after initial FDA approval has been obtained, further studies, including a commitment to conduct post-market studies, would 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. Results of post-marketing programs, including Phase 4 clinical studies or post-market surveillance, 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, submission and approval of an NDA supplement might be required.

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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 GCPs, 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. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a Risk Evaluation and Mitigation Strategies, or REMS, program. The FDA strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

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 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.

Expedited Review and Accelerated Approval Programs

A sponsor may seek approval of its product candidate under programs designed to accelerate FDA's review and approval of NDAs. For example, Fast Track Designation may be granted to a drug intended for treatment of a serious or life-threatening disease or condition that has potential to address unmet medical needs for the disease or condition. The key benefits of fast track designation are the eligibility for priority review, rolling review (submission of portions of an application before the complete marketing application is submitted), and accelerated approval, if relevant criteria are met. Based on results of clinical studies submitted in an NDA, upon the request of an applicant, the FDA may grant the NDA priority review designation, which sets the target date for FDA action on the application at six months after the FDA accepts the application for filing. Priority review is granted where there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition. If criteria are not met for priority review, the application is subject to the standard FDA review period of 10 months after FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the accelerated approval program, the FDA may approve an NDA on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or

lack of alternative treatments. Post-marketing studies or completion of ongoing studies after marketing approval are generally required to verify the drug's clinical benefit in relationship to the surrogate endpoint or ultimate outcome in relationship to the clinical benefit. In addition, the Food and Drug Administration Safety and Innovation Act, or FDASIA, which was enacted and signed into law in 2012, established the new Breakthrough Therapy designation. A sponsor may seek FDA designation of its product candidate as a Breakthrough Therapy if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.

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European Union Drug Review and Approval

In the EEA (which is comprised of the 28 Member States of the European Union plus Norway, Iceland and Liechtenstein), medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. There are two types of MA:

The Community MA, which is issued by the European Commission through the Centralized Procedure, based on the opinion of the CHMP of the EMA, is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, and medicinal products containing a new active substance indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes and auto-immune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the European Union.

National MAs, which are issued by the competent authorities of the Member States of the EEA and only cover their respective territory, are available for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MA can be recognized in other Member States through the Mutual Recognition Procedure. If the product has not received a National MA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure.

Under the above described procedures, before granting the MA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

Orphan Designation and Exclusivity

The Orphan Drug Act provides incentives for the development of products intended to treat rare diseases or conditions. Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the U.S., or more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the cost of developing and making a drug product available in the U.S. for this type of disease or condition will be recovered from sales of the product. If a sponsor demonstrates that a drug is intended to treat rare diseases or conditions, the FDA will grant orphan designation for that product for the orphan disease indication. Orphan designation must be requested before submitting an NDA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA.

Orphan drug designation provides manufacturers with research grants, tax credits and eligibility for orphan drug exclusivity. If a product that has orphan drug designation subsequently receives the first FDA approval of the active moiety for that disease or condition for which it has such designation, the product is entitled to orphan drug exclusivity, which for seven years prohibits the FDA from approving another product with the same active ingredient for the same indication, except in limited circumstances. If a drug designated as an orphan product receives marketing approval for an indication broader than the orphan indication for which it received the designation, it will not be entitled to orphan drug exclusivity. Orphan exclusivity will not bar approval of another product under certain circumstances, including if a subsequent product with the same active ingredient for the same indication is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care, or if the company with orphan drug exclusivity is not able to meet market demand. Further, the FDA may approve more than one product for the same orphan indication or disease as long as the products contain different active ingredients. Moreover, competitors may receive approval of for the same product but for a different indication for which the orphan product has exclusivity. As a result, even if we obtain orphan exclusivity, we may still be subject to competition.

In the EU, the EMA's Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union Community and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would

be a significant benefit to those affected). Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the medicinal product. In the EU, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

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Pediatric Studies and Exclusivity

NDAs must contain data (or a proposal for post-marketing activity) to assess the safety and effectiveness of an investigational new drug product for the claimed indications in all relevant pediatric populations in order to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults or full or partial waivers if certain criteria are met. The requirements for pediatric data do not apply to any drug for an indication for which orphan designation has been granted.

Pediatric exclusivity is another type of non-patent exclusivity in the U.S. and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the five-year and three-year non-patent and orphan exclusivity. This six-month exclusivity may be granted if an NDA or Biologic License Application sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical study is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of FDA-requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application relying on the NDA sponsor's data.

Hatch-Waxman Amendments and Exclusivity

As an alternative path to FDA approval for modifications to formulations or uses of products previously approved by the FDA, an applicant may submit an NDA under Section 505(b)(2) of the FDCA. Section 505(b)(2) was enacted as part of the Hatch-Waxman Amendments and permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant. If the 505(b)(2) applicant can establish that reliance on FDA's previous findings of safety and effectiveness is scientifically appropriate, it may eliminate the need to conduct certain preclinical or clinical studies of the new product. The FDA may also require companies to perform additional studies or measurements, including clinical trials, to support the change from the approved branded reference drug. The FDA may then approve the new product candidate for all, or some, of the label indications for which the branded reference drug has been approved, as well as for any new indication sought by the 505(b)(2) applicant.

In seeking approval for a drug through an NDA, including a 505(b)(2) NDA, applicants are required to list with the FDA certain patents whose claims cover the applicant's product. Upon approval of an NDA, each of the patents listed in the application for the drug is then published in the Orange Book. Any applicant who files an abbreviated new drug application, or ANDA, seeking approval of a generic equivalent version of a drug listed in the Orange Book or a 505(b)(2) NDA referencing a drug listed in the Orange Book must certify to the FDA that (1) no patent information on the drug product that is the subject of the application has been submitted to the FDA; (2) such patent has expired; (3) the date on which such patent expires; or (4) such patent is invalid or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted. This last certification is known as a paragraph IV certification. A notice of the paragraph IV certification must be provided to each owner of the patent that is the subject of the certification and to the holder of the approved NDA to which the ANDA or 505(b)(2) application refers. The applicant may also elect to submit a "section viii" statement certifying that its proposed label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent.

If the reference NDA holder and patent owners assert a patent challenge directed to one of the Orange Book listed patents within 45 days of the receipt of the paragraph IV certification notice, the FDA is prohibited from approving the application until the earlier of 30 months from the receipt of the paragraph IV certification expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the applicant. The ANDA or 505(b)(2) application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the branded reference drug has expired. Specifically, the holder of the NDA for the listed drug may be entitled to a period of non-patent exclusivity, during which the FDA cannot approve an ANDA or 505(b)(2) application that relies

on the listed drug. For example, a pharmaceutical manufacturer may obtain five years of non-patent exclusivity upon NDA approval of a new chemical entity, or NCE, which is a drug that contains an active moiety that has not been approved by FDA in any other NDA. An "active moiety" is defined as the molecule or ion responsible for the drug substance's physiological or pharmacologic action. During the five-year exclusivity period, the FDA cannot accept for filing any ANDA seeking approval of a generic version of that drug or any 505(b)(2) NDA for the same active moiety and that relies on the FDA's findings regarding that drug, except that FDA may accept an application for filing after four years if the follow-on applicant makes a paragraph IV certification.

A drug, including one approved under Section 505(b)(2), may obtain a three-year period of exclusivity for a particular condition of approval, or change to a marketed product, such as a new formulation for a previously approved product, if one or more new clinical studies (other than bioavailability or bioequivalence studies) was essential to the approval of the application and was conducted/sponsored by the applicant. Should this occur, the FDA would be precluded from approving any ANDA or 505(b)(2) application for the protected modification until after that three-year exclusivity period has run. However, unlike NCE exclusivity, the FDA can accept an application and begin the review process during the exclusivity period.

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Coverage and Reimbursement

Sales of our products will depend, in part, on the extent to which our products will be covered by third-party payors, such as government health care programs, commercial insurance and managed healthcare organizations. These third-party payors are increasingly limiting coverage and reducing reimbursements for medical products and services. In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. Decreases in third-party reimbursement for PROCYSBI and our drug candidates or a decision by a third-party payor to not cover PROCYSBI and our drug candidates could reduce physician utilization of our products and have a material adverse effect on our sales, results of operations and financial condition.

Other Healthcare Laws

We are also subject to healthcare regulation and enforcement by the federal government and the states and foreign governments in which we conduct our business. These laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, privacy and security and physician sunshine laws and regulations.

The federal Anti-Kickback Statute prohibits, among other things, any person from knowingly and willfully offering, soliciting, receiving or providing remuneration, directly or indirectly, to induce either the referral of an individual, for an item or service or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs. The Anti-Kickback Statute is subject to evolving interpretations. In the past, the government has enforced the Anti-Kickback Statute to reach large settlements with healthcare companies based on sham consulting and other financial arrangements with physicians. Further, the recently enacted Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively, the Health Care Reform Law, among other things, amends the intent requirement of the federal Anti-Kickback Statute and the criminal statute governing healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them. In addition, the Health Care Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal False Claims Act or federal civil money penalties statute. The majority of states also have anti-kickback laws which establish similar prohibitions and in some cases may apply to items or services reimbursed by any third-party payor, including commercial insurers.

Additionally, the civil False Claims Act prohibits knowingly presenting or causing the presentation of a false, fictitious or fraudulent claim for payment to the U.S. government. Actions under the False Claims Act may be brought by the Attorney General or as a qui tam action by a private individual in the name of the government. Violations of the False Claims Act can result in very significant monetary penalties and treble damages. The federal government is using the False Claims Act, and the accompanying threat of significant liability, in its investigation and prosecution of pharmaceutical and biotechnology companies throughout the country, for example, in connection with the promotion of products for unapproved uses and other sales and marketing practices. The government has obtained multi-million and multi-billion dollar settlements under the False Claims Act in addition to individual criminal convictions under applicable criminal statutes. Given the significant size of actual and potential settlements, it is expected that the government will continue to devote substantial resources to investigating healthcare providers' and manufacturers' compliance with applicable fraud and abuse laws.

In addition, there has been a recent trend of increased federal and state regulation of payments made to physicians and other healthcare providers. The Health Care Reform Law, among other things, imposes new reporting requirements on drug manufacturers for payments made by them to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Failure to submit required information may result in civil monetary penalties of up to an aggregate of $150,000 per year (or up to an aggregate of $1 million per year for "knowing failures"), for all payments, transfers of value or ownership or investment interests that are not

timely, accurately and completely reported in an annual submission. Drug manufacturers were required to begin collecting data on August 1, 2013 and submit reports to the government by March 31, 2014 and the 90th day of each subsequent calendar year. Certain states also mandate implementation of commercial compliance programs, impose restrictions on drug manufacturer marketing practices and/or require the tracking and reporting of gifts, compensation and other remuneration to physicians.

The shifting commercial compliance environment and the need to build and maintain robust and expandable systems to comply with different compliance and/or reporting requirements in multiple jurisdictions increase the possibility that a healthcare company may violate one or more of the requirements. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, civil and criminal penalties, damages, fines, the curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs and imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

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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 research and development efforts in the discovery, research, preclinical and clinical development of our clinical drug candidates in order to provide therapies that we believe will be safer, less intrusive and more effective than current approaches in treating a wide variety of disorders. During the year ended December 31, 2013, the four-month transition period ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011, we incurred approximately $29.2 million, $8.9 million, $21.4 million and $14.8 million, respectively, in research and development expenses.

Operating Segment and Geographic Information

Operating segments are components of an enterprise for which separate financial information is available and is 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

As of December 31, 2013, we had 70 full time employees (65 and 5 in the U.S. and EU, respectively) and 2 U.S. part-time employees. Of the 70 employees, 40 are sales and marketing and general and administrative personnel and 30 are in manufacturing, quality control and assurance and research and development. Based on our current plan, over the next 12-month period we intend to expand our U.S. and EU employee base across all functions in the Company.

Facilities

Our primary offices are located at 5 Hamilton Landing, Suite 160, Novato, CA 94949. Our main phone number is (415) 408-6200 and our facsimile number is (415) 382-8002.

Website

Our corporate 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.

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ITEM 1A: RISK FACTORS

Investing in our common stock involves a high degree of risk. Before deciding whether to invest in our common stock, you should consider carefully the risk factors described below, together with the other information contained in this Annual Report on Form 10-K. If any of these risks actually occur, it may materially harm our business, financial condition, operating results or cash flow. As a result, the market price of our common stock could decline, and you could lose all or part of your investment. Additional risks and uncertainties that are not yet identified or that we think are immaterial may also materially harm our business, operating results and financial condition and could result in a complete loss of your investment.

Risks Associated with Commercialization and Product Development

We currently depend entirely on the commercial success of our lead drug, PROCYSBI, for the management of nephropathic cystinosis.

PROCYSBI is our only product currently approved for marketing and, as a result, our operating results are substantially dependent on the commercial success of PROCYSBI, for which we commenced marketing in the U.S. in June 2013. In the U.S., we are permitted to market PROCYSBI only for the management of nephropathic cystinosis in adults and children six years and older. In September 2013, we received marketing authorization from the European Commission, which allows us to commercialize PROCYSBI in the 28 Member States of the EU plus Norway, Liechtenstein and Iceland (which are not EU Member States but are part of the European Free Trade Association, or EFTA) for the treatment of proven nephropathic cystinosis; however, we have not yet commercially launched PROCYSBI in the EU. We believe that the trading price of our common stock will be substantially affected by our results of operations and, in particular, net product sales of PROCYSBI. We do not have prior experience in commercializing therapeutics. If PROCYSBI sales do not meet expectations, our stock price may not increase or could significantly decrease. The successful commercialization of PROCYSBI will depend on several factors, including:

- growing sales of PROCYSBI in the U.S.;
- the negotiation and agreement on an acceptable prices in EU countries and other select territories, and reimbursement at the country-specific price;
- the successful commercial launch of PROCYSBI in the EU and other select territories;
- acceptance of PROCYSBI by physicians, parents, patients and cystinosis research/advocacy organizations including the conversion from the existing standard of care to PROCYSBI;
- coverage and reimbursement for PROCYSBI from commercial health plans and government health programs, which we refer to collectively as third-party payors;
- compliance with regulatory requirements including fulfilling any FDA and EC required post-approval commitments;
- provision of affordable out-of-pocket cost to patients and/or other programs to ensure patient access to PROCYSBI in the U.S.;
- approval by other country regulatory agencies of appropriate product labeling for PROCYSBI;
- agreements with wholesalers, distributors and pharmacies on commercially reasonable terms;
- manufacture and supply of adequate quantities of PROCYSBI to meet commercial demand; and
- development and maintenance of intellectual property protection for PROCYSBI.

If we fail to grow sales of PROCYSBI in the U.S. or successfully commercialize PROCYSBI in the EU within a reasonable time period, we may never become profitable and may be unable to sustain our business, and our business, financial condition and results of operations will be adversely affected.

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Our ability to generate significant product sales from PROCYSBI is dependent upon market acceptance among physicians, patients, patient families, third-party payors and the healthcare community.

PROCYSBI may not attain or maintain market acceptance among physicians, patients, patient families, third-party payors or the healthcare community compared to the current standard of care. We believe that the degree of market acceptance and our ability to generate significant product sales of PROCYSBI will depend on a number of factors, including:

- availability and relative efficacy, safety and ease of administration of alternative treatments;
- the price of our product, both in absolute terms and relative to alternative treatments;
- timing of market introduction of our product as well as competitive drugs;
- efficacy, safety and prevalence and severity of any side effects of PROCYSBI;
- identification of currently diagnosed and undiagnosed patients and continued growth of the cystinosis market;
- acceptance by patients, patient families and primary care and other specialists including conversion from the existing standard of care;
- continued patient adherence to therapy;
- the effect of current and future healthcare laws;
- availability of coverage and adequate reimbursement and pricing from third-party payors; and
- breadth of product labeling or product insert requirements of the FDA, EC or other regulatory authorities.

If PROCYSBI does not achieve and maintain significant market acceptance among physicians, patients, patient families, third-party payors or the healthcare community, our ability to generate revenues from PROCYSBI will be materially and adversely affected.

The amount of our product sales of PROCYSBI in the EU is dependent upon the pricing and reimbursement guidelines adopted in each of the various countries in the EU, which levels may be below our current expectations.

We have not yet priced PROCYSBI in any countries in the EU. While we are developing estimates of anticipated pricing, one or more EU countries may not support our anticipated pricing and reimbursement for PROCYSBI, particularly in light of the budget crises faced by a number of countries in the EU, which would negatively affect anticipated revenue from PROCYSBI. The pricing and reimbursement process in the EU can be lengthy and involved, and we do not have significant experience with this process. Failure to timely complete the pricing and reimbursement process in the EU will delay our ability to market PROCYSBI in the EU and derive product sales in that region.

If we are unable to expand the use of RP103 and receive regulatory approval for any other indication, we may delay or cease some of our product development activities, which would adversely affect the long term value of RP103 and our growth prospects.

We must obtain and maintain appropriate pre-market approvals from regulatory agencies in each of the markets in which we intend to market our products. Once approved, we may only market our products for the specific uses that are reflected in the product's approved labeling. In the U.S., we are permitted to market RP103 only for the management of nephropathic cystinosis in adults and children six years and older under the brand name PROCYSBI. We are permitted to market PROCYSBI in the EU as an orphan medicinal product for the treatment of proven nephropathic cystinosis. We do not have approval of RP103 in any other market nor for any other disease indication. A new drug application, or NDA, submitted to the FDA or marketing authorization application, or MAA, submitted to the EMA must be supported by extensive clinical and preclinical data, as well as extensive information regarding chemistry, manufacturing and controls, to demonstrate the safety and efficacy of the applicable product candidate for the management of each individual indication to the satisfaction of the applicable regulatory authority.

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Obtaining approval of an NDA, MAA or any other filing for marketing authorization in a foreign country is an extensive, expensive and uncertain process. The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. The FDA, EC or other regulatory authorities may delay, limit or deny approval of RP103 or our future drug product candidates for many reasons, including:

- the results of clinical trials may not meet the level of statistical or clinical significance required by regulatory authorities for approval;
- regulatory authorities 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 a product candidate has adequate clinical and other benefits or an adequate safety profile; or may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct additional trials;
- regulatory authorities may not accept data generated at our clinical trial sites;
- regulatory authorities may have difficulties scheduling an advisory committee meeting (or equivalent, if required) in a timely manner or the advisory committee may recommend against approval of our application or may recommend that the regulatory agency require, as a condition of approval, additional preclinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;
- regulatory authorities 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;
- regulatory authorities may impose limitations on approved labeling, thus introducing reimbursement complications which may limit access for intended uses or limit the commercial profile of the drug;
- regulatory authorities may identify deficiencies in the manufacturing processes or in the facilities of our third-party suppliers and/or contract manufacturers, or may require us to manufacture additional validation batches or change our process, specifications or third-party suppliers or contract manufacturers;
- we may not be able to validate our manufacturing process to the satisfaction of the regulatory authorities, or they may not agree with our plan for potential retrospective validation; or
- regulatory authorities may change approval policies or adopt new regulations.

If we fail to gain regulatory approval for RP103 for other indications, we will have to delay or terminate some or all of our research product development programs and our business, financial condition and results of operations will be adversely affected.

We do not have internal manufacturing capabilities. During 2014 and throughout most of 2015, we expect to continue to rely on a single supplier for the active pharmaceutical ingredient and a single third-party manufacturer for the conversion to finished drug product. If we are unable to obtain an adequate supply of our drugs, our reputation will be harmed, our revenues will be delayed or diminished and our financial results will be adversely affected.

Using external CMOs under contract, we currently manufacture commercial and clinical quantities of PROCYSBI and RP103 for the indications under development. We rely on single manufacturing sources for our cysteamine active pharmaceutical ingredient, or API, and finished products. Our ability to obtain sufficient quantities of PROCYSBI and RP103 is constrained by limited supplies of raw materials and capacity and output of these manufacturers, which may have a material adverse impact on sales of PROCYSBI and the availability of product for our clinical trials. While we have entered into an agreement with a second tablet manufacturer, we expect that this second manufacturer will not be able to produce finished products for commercial sale until the latter part of 2015. Furthermore, any reduction or interruption in our supply of API from the single source supplier and of finished goods from our contract manufacturer, and efforts to identify and qualify alternative sources of API supply, could result in significant additional operating costs, interruptions in product supply and delays in sales of PROCYSBI and in developing RP103 for HD, NAFLD and Leigh's syndrome. In addition, supply arrangements from alternative sources may not be available on acceptable economic terms, if at all.

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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 to commercial requirements. Difficulties may arise related to production costs and yields, quality control, including stability of the product or product candidates and quality control testing, sourcing scarcities, resource constraints, equipment problems, shortages of qualified personnel, labor disputes, severe weather events, unstable political environments or financial difficulties at foreign facilities, as well as compliance with strictly enforced federal, state and foreign regulations. In addition, due to our small patient population, the manufacture of our drug may be given lower prioritization on the production line if manufacturing is decided by scale.

We depend on our third-party supplier and manufacturers for compliance with the FDA's cGMP requirements and other FDA requirements, Drug Enforcement Administration's regulations and other rules and regulations prescribed by applicable non-U.S. regulatory authorities. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with good manufacturing practices, or cGMP, requirements. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our product candidates or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to market PROCYSBI and to develop, obtain regulatory approval for or market our product candidates, if approved. If we or our third-party suppliers and manufacturers fail to comply with applicable regulatory requirements, a regulatory agency may issue warning letters or untitled letters; seek an injunction or impose civil or criminal penalties or monetary fines; suspend or withdraw regulatory approval; suspend any ongoing clinical trials; refuse to approve pending applications or supplements to applications; suspend or impose restrictions on operations, including costly new manufacturing requirements; seize or detain products; or request that we initiate a product recall.

If any of these events were to occur, our reputation would be harmed, revenues from sales of our products would be delayed or diminished and our business, financial condition and results of operations would be adversely affected.

PROCYSBI is, and any other future product candidates, if approved, will be, subject to extensive and ongoing regulatory requirements and continued regulatory review, which will result in significant expense. Additionally, PROCYSBI and our future product candidates, if approved, may be subject to labeling and other restrictions or potential market withdrawal, and we may be subject to penalties and litigation if we fail to comply with regulatory requirements or experience problems with our products.

Our manufacturing processes, labeling, packaging, distribution, storage, adverse event reporting, dispensation, distribution, advertising, promotion and recordkeeping are subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, ongoing maintenance of product registration, as well as continued compliance with cGMPs, GCPs, good distribution practices, or GDPs, and good laboratory practices, or GLPs. If we do not comply with applicable regulations and requirements, the range of possible sanctions includes adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, withdrawal of a product's approval and enforcement actions, including injunctions and civil or criminal prosecution. In addition, if we or a regulatory agency discover previously unknown problems with PROCYSBI, such as adverse events of unanticipated severity or frequency, or identify data that suggest that PROCYSBI may present a risk to safety, the regulatory 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 withdrawn, our growth prospects and our operating results will be adversely affected.

Moreover, any regulatory approvals that we obtain will be subject to limitations on the approved indicated uses for which the product may be marketed or the conditions of approval, or contain requirements for potentially costly

post-market testing and surveillance to monitor the safety and efficacy of the product. The FDA and EC strictly regulate the promotional claims that may be made about prescription products and our product labeling, advertising and promotion is subject to continuing regulatory review. Physicians nevertheless may prescribe our product to their patients in a manner that is inconsistent with the approved label, or that is off-label. The FDA, the Competent Authorities of the Member States of the Economic European Area, or EEA, and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and if we are found to have improperly promoted off-label uses we may be subject to significant sanctions, civil and criminal fines and injunctions prohibiting us from engaging in specified promotional conduct.

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In addition, engaging in improper promotion of our products for off-label uses in the U.S. can subject us to false claims litigation under federal and state statutes, which can lead to consent decrees, civil money penalties, restitution, criminal fines and imprisonment, and exclusion from participating in Medicare, Medicaid and other federal and state health care programs. These false claims statutes in the U.S. include the federal False Claims Act, which allows any individual to bring a lawsuit against a pharmaceutical company on behalf of the federal government alleging submission of false or fraudulent claims, or causing to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government prevails in the lawsuit, the individual will share in any fines or settlement funds. Growth in false claims litigation has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, agree to comply with burdensome reporting and compliance obligations, and be excluded from Medicare, Medicaid and other federal and state healthcare programs.

In the EEA, the advertising and promotion of pharmaceuticals is strictly regulated, and the direct-to-consumer promotion of prescription pharmaceuticals is not permitted. The Member States of the EEA have also adopted laws against misleading and unfair advertising. In addition, some Member States require the notification and/or prior authorization of promotional or advertising materials directed at health care professionals. Failure to comply with these regulations can lead to the imposition of administrative fines and criminal penalties, civil litigation leading to injunctive relief to stop the advertising, corrective statements, or damages.

If serious adverse side effects become associated with PROCYSBI, our business will be harmed.

The prescribing information for PROCYSBI includes several warnings relating to observed adverse reactions of cysteamine bitartrate usage. These adverse reactions were not observed in our clinical trials supporting PROCYSBI's NDA and MAA, but were required on our label due to our submission of a 505(b)(2) application in the U.S. and a hybrid application in the EU. The FDA may require products approved under Section 505(b)(2) of the FDCA to bear the same or similar warning statements as the reference product. We expect to update adverse reactions listed in the prescribing information based on continued commercial use and additional clinical trials. If additional adverse reactions emerge, or if there is a pattern of severe or persistent previously observed side effects in the relevant patient populations, the FDA, the EMA or other regulatory agencies could modify or revoke our marketing approval, require us to modify our label, or require us to suspend production, or we may choose to withdraw PROCYSBI from the market. If this were to occur, we may be unable to obtain marketing approval in other indications. In addition, patients or their representatives may bring claims against us alleging serious adverse side effects or harm suffered as a result of use of PROCYSBI. Any such side effects or related claims could have a material adverse effect on our business, financial condition and results of operations.

See also the risk factor titled "We may be subject to product liability claims."

Pressure on drug product third-party payor coverage, reimbursement and pricing may impair our ability to be reimbursed for PROCYSBI and our other future product candidates at prices or on terms sufficient to provide a viable financial outcome.

Market acceptance and sales of PROCYSBI and any product candidates that we may develop will depend in large part on third-party payor coverage and reimbursement policies and may be affected by future healthcare reform measures in the U.S. as well as the EU 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, including an increased emphasis on managed care and attempts to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, may result in downward pressure on product pricing, reimbursement and utilization, which may adversely affect our product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, drug coverage and reimbursement policies and pricing in general. Moreover, private health insurers and other third-party payors in the U.S. often follow the coverage and reimbursement policies of government payors, including the Medicare or Medicaid programs. In the U.S., third-party payors are shifting their cost containment measures to specialty products and high-cost drugs and PROCYSBI may be a target of such measures.

Beginning April 1, 2013, Medicare payments for all items and services under Part A and B, including drugs and biologicals, and most payments to plans under Medicare Part D were reduced by 2% under the automatic spending reductions, or sequestration, required by the Budget Control Act of 2011, or BCA, as amended by the American Taxpayer Relief Act, or ATRA. The BCA required sequestration for most federal programs, excluding Medicaid, Social Security and certain other programs, because Congress failed to enact legislation by January 15, 2012 to reduce federal deficits by $1.2 trillion over 10 years. As long as BCA cuts remain in effect, they could adversely impact payment for PROCYSBI. In addition, other recent legislative changes that increase manufacturer liability for rebates and other payments under the 340B drug pricing program, the Medicaid Drug Rebate Program and the Medicare Part D prescription drug benefit also could impact our revenues. See the risk factor titled "Enacted and future legislation may increase the difficulty and cost for us to commercialize PROCYSBI or any other product candidate that we develop and affect the prices we may obtain."

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Further, payors also are increasingly considering new metrics as the basis for reimbursement rates, such as average sales price, or ASP, average manufacturer price, or AMP, or actual acquisition cost, or AAC. Although the intent of the changes to reimbursement methodologies generally is to limit payment increases, it is difficult to project the impact of these and other alternative reimbursement methodologies on the willingness of payors to reimburse PROCYSBI and any product candidates that we may develop. Although to date PROCYSBI has been reimbursed, we do not know whether third-party payors will continue to reimburse PROCYSBI in the U.S. and whether third-party payors will reimburse RP103 and our future products for future commercial indications until we enter into payor negotiations. If coverage and reimbursement are not available or available only to limited levels, we may not be able to generate sufficient revenue to meet our operating costs or to achieve our revenue, cash flow breakeven or profitability goals in the timeframe that we expect, or at all.

Enacted and future legislation may increase the difficulty and cost for us to commercialize PROCYSBI or any other product candidate that we develop and affect the prices we may obtain.

In the U.S., there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that restrict or regulate post-approval activities, which may affect our ability to profitably sell PROCYSBI or any other product candidate for which we obtain marketing approval.

The Medicare Prescription Drug Improvement and Modernization Act of 2003, or MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for outpatient drug purchases by those covered by Medicare under a new Part D and introduced a new reimbursement methodology based on average sales prices for Medicare Part B physician-administered drugs. In addition, this legislation authorized Medicare Part D prescription drug plans to use formularies whereby they can limit the number of drugs that will be covered in any therapeutic class. As a result of this legislation and the expansion of federal coverage of drug products, there is additional pressure to contain and reduce costs. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates, and any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors. These cost reduction initiatives and other provisions of the MMA could decrease the coverage and reimbursement that we receive for any approved products, and could seriously harm our business. Manufacturers' contributions to this area, including donut hole coverage (as described below) or potential excise taxes, are increasing and are subject to additional changes in the future.

In 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (together, the "Health Care Reform Law"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Health Care Reform Law, among other things, revised the definition of AMP for reporting purposes, which could increase the amount of Medicaid drug rebates to states and extended the rebate program to beneficiaries enrolled in Medicaid managed care organizations. The Health Care Reform Law also imposed a significant annual fee on companies that manufacture or import branded prescription drug products and established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the U.S. The Health Care Reform Law 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 Health Care Reform Law includes a provision to increase the Medicaid rebate for line extensions or reformulated drugs, which depending on how this provision is implemented could substantially increase our Medicaid rebate rate (in effect limiting reimbursement for these patients). These and other new provisions are likely to continue the pressure on pharmaceutical pricing, may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs. See the risk factor titled "Failure to comply with healthcare regulations may subject us to substantial penalties."

Legislative and regulatory proposals also have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. In addition, increased scrutiny by the U.S. Congress of the FDA's

approval process may subject us to more stringent product labeling and post-marketing testing and other requirements. Delays in feedback from the FDA may affect our ability to quickly update or adjust our label in the interest of patient adherence and tolerability. We cannot predict whether other legislative changes will be adopted or how such changes would affect the pharmaceutical industry generally and specifically the commercialization of PROCYSBI.

Failure to comply with healthcare regulations may subject us to substantial penalties.

Although we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, federal and state healthcare laws and regulations pertaining to fraud and abuse, physician payment transparency and privacy and security laws and regulations apply to us and our arrangements with healthcare providers, customers and other entities, including our marketing practices, educational programs and pricing policies. The laws that may affect our ability to operate as a commercial organization include:

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- the federal 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 federal third-party payors that are false or fraudulent;
- federal criminal laws that prohibit executing a scheme to defraud any federal 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;
- the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or other "transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and applicable manufacturers and group purchasing organizations to report annually to CMS ownership and investment interests held by physicians (as defined above) and their immediate family members and payments or other "transfers of value" to such physician owners and their immediate family members. Manufacturers were required to begin data collection on August 1, 2013 and to report aggregate data to the government by March 31, 2014 with more detailed reports due by August 1, 2014;
- in the EU, in various Member States, including France, the UK, the Netherlands, Italy, or Spain, the legislator or self-regulatory industry bodies have adopted rules requiring the notification and/or publication of certain transfers of value from pharmaceutical companies to health care professionals. For example, France has recently adopted legislation (Law No. 2011-2012, or the "French Sunshine Act," and Decree no. 2013-414 which implements it) requiring pharmaceutical companies to disclose and publish agreements with or transfers of value to health care professionals; and
- analogous state and foreign 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.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. In addition, the Health Care Reform Law further strengthened these laws by amending the intent requirement of the federal anti-kickback and criminal health care fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the Health Care Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the false claims statutes. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business.

Moreover, there has been a recent trend of increased federal and state regulation of payments made to physicians for marketing. Certain states mandate implementation of compliance programs and/or the tracking and reporting of gifts, compensation, and other remuneration to physicians. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increase the possibility that a healthcare company may violate one or more of the requirements.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available under such laws, it is possible that some of our business activities, including our relationships with physicians and other health care providers, some of whom recommend, purchase and/or prescribe our products, could be subject to challenge under one or more of such laws. While these activities are structured to comply with all applicable laws, if

our operations are found to be in violation of any of the laws described above or any other 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 PROCYSBI, RP103 and other future drug candidates and adversely impact our financial results. See also the risk factor titled "If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and future business prospects."

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If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and future business prospects.

We participate in the Medicaid Drug Rebate Program and other Federal and state government pricing programs in the U.S., and we may participate in additional government pricing programs in the future. These programs generally require us to pay rebates or provide discounts to government payors in connection with drugs, including PROCYSBI, that are dispensed to beneficiaries of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing and rebate calculations that we report on a monthly and quarterly basis to the government agencies that administer the programs. The terms, scope and complexity of these government pricing programs change frequently. Responding to current and future changes may increase our costs and the complexity of compliance will be time-consuming, and could have a material adverse effect on our results of operations.

Pricing and rebate calculations vary among products and programs. The calculations are complex and are often subject to interpretation by governmental or regulatory agencies and the courts.

In addition, the Office of Inspector General has recently increased its focus on the methodologies used by manufacturers to calculate AMP and best price, or BP, to assess manufacturer compliance with reporting requirements under the Medicaid Drug Rebate Program. We are liable for errors associated with our submission of pricing data and for overcharging government payors. For example, failure to submit monthly/quarterly AMP and BP data on a timely basis could result in a civil monetary penalty of $10,000 per day for each day the submission is late beyond the due date. Failure to make necessary disclosures and/or to identify overpayments could result in allegations against us under the Federal False Claims Act and other laws and regulations.

Unexpected refunds to the U.S. government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition and results of operations. In the event that CMS were to terminate our rebate agreement, no federal payments would be available under Medicaid or Medicare for our covered outpatient drugs.

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

PROCYSBI and our clinical development of RP103 target diseases with small patient populations, including cystinosis and HD, respectively. 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. Due to small patient populations, we believe that we would need to have significant market penetration to achieve meaningful revenues and identifying patients and targeting the prescriber base are key to achieving significant market penetration. In addition, the per-patient prices at which we sell PROCYSBI (currently an average of $250,000 per year prior to rebates, discounts, distribution fees and not adjusted for patient compliance in the U.S.) and 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 gross margins. There can be no assurance that we will be successful in achieving a sufficient degree of market penetration and/or obtaining or maintaining high per-patient prices for PROCYSBI and RP103 for diseases with small patient populations. Further, even if we obtain significant market share for PROCYSBI and RP103, if approved, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share. Furthermore, because the potential target populations are very small, even if we do obtain significant market share for PROCYSBI and RP103, if approved, we may never achieve profitability. Additionally, patients who discontinue therapy or do not fill prescriptions are not easily replaced by new patients, given the limited patient population.

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

As part of our business strategy, we intend to develop RP103 for additional indications and other 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., or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. In the U.S., orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. 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 six months 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, a subsequent product is deemed clinically superior, or if the manufacturer is unable to deliver sufficient quantity of the drug.

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In the EU, the EMA's Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU Community and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the medicinal product. An EU orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Because the extent and scope of patent protection for some of our drug products may be 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 orphan exclusivity period to maintain a competitive position. However, if we do not obtain orphan drug exclusivity for RP103 for the potential treatment of HD or other potential indications, or our future relevant drug products do not have strong patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced. Also, without strong patent protection, competitors may sell a generic version upon the expiration of orphan exclusivity, if our patent position is not upheld.

Even though we have been granted orphan drug designation in the U.S. prior to the approval of RP103 for the potential treatment of HD, and even if we obtain orphan drug designation for our future drug product candidates, we may not fulfill the criteria for exclusivity or we may not be the first to obtain marketing approval for any orphan indication. Further, even if we obtain orphan drug exclusivity for a particular 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 a 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. The FDA can discontinue Orphan Drug exclusivity after it has been granted if the orphan drug cannot be manufactured in sufficient quantities to meet demand. Positive clinical trial results in any of our RP103 programs increase the risk that Cystagon may be used off-label in those indications in certain geographic areas due to Cystagon's lower cost and our 505(b)(2) filing status.

A breakthrough designation or fast track designation for our drug product candidates, if obtained, may not actually lead to a faster review process.

Under the Prescription Drug User Fee Act, the FDA has a goal of responding to NDAs within 10 months of submission the filing date for standard review, but this timeframe is also often extended. In the future, we may seek approval of our drug candidates under programs designed to accelerate the FDA's review and approval of NDAs. For example, a sponsor may seek FDA designation of a drug candidate as a "fast track product." Fast track products are those products intended for the treatment of a serious or life-threatening disease or condition and which demonstrate the potential to address unmet medical needs for such disease or condition. If fast track designation is obtained, the FDA may initiate review of sections of an NDA before the application is complete. This "rolling review" is available if the applicant provides and the FDA approves a schedule for the remaining information. In some cases, a product may be approved on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Approvals of this kind typically include requirements for appropriate post-approval Phase 4 clinical trials to validate the surrogate endpoint or otherwise confirm the effect of the clinical endpoint. In addition, the Food and Drug Administration Safety and Innovation Act, or FDASIA, which was enacted and signed into law in 2012, established a new category of drugs

referred to as "breakthrough therapies," which are defined as drugs intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. In the future, we may request breakthrough designation or fast track designation from the FDA for our other drug product candidates, but we cannot assure that we will obtain such designations. Moreover, even if we obtain breakthrough designation or fast track designation from the FDA, the designations do not guarantee FDA approval of our NDA, that the development program or review timeline will ultimately be shorter than if we had not obtained the designations, or that the FDA will not request additional information, including requesting additional clinical studies (although potentially a post-marketing requirement), during its review. Any request for additional information or clinical data could delay the FDA's timely review of our NDA.

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

In connection with the anticipated European commercial launch of PROCYSBI, we have expanded our operations in Europe where we expect to continue to add personnel. We may encounter difficulties successfully managing remotely a substantially larger and internationally diverse organization and may encounter delays in commercialization if we are not successful in integrating our international operations. Challenges related to managing international operations include the following:

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- 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;
- the small size of our company and our intention to grow at a consistent but measured pace;
- ability to operate within diverse individual country regulatory and statutory laws; and
- the costs of maintaining EU presence, in-country legal entities and related tax structures.

If we fail to obtain and maintain approval from regulatory authorities in international markets for PROCYSBI, 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 products outside of the U.S. are subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approvals. Even if the FDA and EC grant marketing approval for a product candidate, comparable regulatory authorities of foreign countries 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, complying with foreign regulatory requirements and gaining approved pricing and reimbursement 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.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome and results of earlier studies and trials may not be predictive of future trial 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 safety and efficacy in preclinical studies and clinical trials. Preclinical studies involve testing in appropriate multiple non-human disease models to demonstrate efficacy and safety. Regulatory agencies evaluate these data carefully before they will authorize clinical testing in humans. If certain preclinical data reveal 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 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. There are many potential preclinical models to test for different disease states, and we could fail to choose the best preclinical model to determine proof of concept, safety and efficacy of our drug product candidates.

Following successful preclinical testing, drug product candidates must be tested in a clinical development program to provide data on safety and efficacy in humans 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. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. The clinical trial process may fail to demonstrate with statistical significance 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 relevant marketing applications with the regulatory agencies and, ultimately, our ability to commercialize our drug product candidates and generate product revenues.

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:

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- 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.

In addition, many of our clinical trials involve small patient populations. Patient enrollment is a significant factor in the timing of clinical trials, and is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. Moreover, because of the small sample size, the results of these early clinical trials may not be indicative of future results. Any delay in our preclinical or clinical programs or 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 results of operations.

We may be subject to product 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 drugs. PROCYSBI and 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 and U.S. and EU cystinosis patients who may purchase PROCYSBI commercially are already critically ill or suffering from chronic debilitating diseases. The waivers we obtain from patients participating in clinical trials may not be enforceable and may not protect us from liability or the costs of product liability litigation.

We may not be able to avoid significant liability if any product liability claim is brought against us. Although we currently carry product liability insurance, it may not be sufficient to cover future claims. 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 operations.

We rely on third parties for the distribution and pharmaceutical services of PROCYSBI in the U.S. and the EU.

We rely on a third-party logistics provider and specialty pharmacy to distribute PROCYSBI to patients and to collect from insurance companies and government agencies in the U.S. and in the EU. Our ability to collect from the logistics provider is not only subject to such provider's credit worthiness but is also dependent, in part, on its ability to arrange for full reimbursement from third-party payors. The outsourcing of our distribution function is complex, and we may experience difficulties that could reduce, delay or stop shipments of PROCYSBI. If we encounter such distribution problems, and we are unable to quickly enter into a similar agreement with another specialty distributor on substantially similar terms, if at all, the distribution of PROCYSBI could become disrupted, resulting in reduced revenues, healthcare provider dissatisfaction and/or patient dissatisfaction which may harm our reputation and financial condition.

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Our reliance on third parties may result in delays in completing, or a failure to complete, preclinical testing, clinical trials or regulatory marketing submissions.

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

- governmental agencies and university laboratories;
- other biotechnology and pharmaceutical companies;
- contract manufacturing organizations;
- clinical research organizations;
- distribution and supply (logistics) service organizations;
- contract testing organizations;
- consultants or consulting organizations with specialized knowledge based expertise;
- intellectual property legal firms; and
- multiple other service organizations.

As a result of our engagement of these types of organizations to help us with our product development programs, many important aspects of our business are and will be out of our direct control. Nevertheless, we are responsible for ensuring that each of our product development programs complies with applicable regulatory requirements, and our reliance on these organizations does not relieve us of our regulatory responsibilities. If any such organizations we engage in the future fail to perform their obligations under our agreements with them or fail to perform in a satisfactory manner in compliance with applicable regulatory requirements, we may face delays in completing our development and commercialization processes for any of our drug product candidates and could be required to repeat testing or clinical trials, which would delay the regulatory approval process. 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 in or failures to complete trials. A failure by us or such third parties to observe 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.

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

- collaborative arrangements might not be available on terms which are reasonably favorable to us, or at all;
- 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;
- agreement terms may be difficult or costly to enforce;
- 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 agreements with us;
- business combinations or significant changes in a partner's business strategy or financial resources might adversely affect that partner's willingness or ability to fulfill its obligations to us; and
- the terms and conditions of the relevant agreements may no longer be suitable.

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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.

We depend on the support of key scientific and medical collaborators.

We must establish and maintain relationships with key opinion leaders, leading scientists and research institutions. We believe that such relationships are pivotal to establishing products using our technologies as a standard of care for their approved indications. Although we have various medical and scientific advisors and research collaborations, there is no assurance that our advisors 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 clinical and scientific relationships to assist in our commercialization and research and development, we may not be able to successfully develop PROCYSBI, RP103 or our other drug product candidates.

We will continue to incur increased costs as a result of corporate governance and financial reporting laws and regulations and our management will continue to be required to devote substantial time to comply with such laws and regulations.

We face burdens relating to increased corporate governance and financial reporting standards. Legislation or regulations, such as the Physician Payment Sunshine Act, 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 stricter corporate governance and financial reporting standards and have led to an increase in the costs of compliance, including substantial increases in consulting, auditing and legal fees. Our management and other personnel will need to devote a substantial amount of time to these requirements. 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, on our board committees or as executive officers. Failure to comply with these new laws and regulations may impact our financial condition and could materially harm our business.

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 control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. Our compliance with Section 404 requires that we incur substantial accounting and related expense and expend significant management efforts. 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 control 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 success depends on our ability to manage our projected growth.

Continued commercial sales of PROCYSBI in the U.S., the EU commercial launch of PROCYSBI, expansion of our commercial operations into other markets, the continuation of our clinical-stage programs and our current plans to in-license and acquire additional clinical-stage product candidates will require us to retain existing and add required new qualified and experienced personnel in all functional areas over the next several years. Also, if 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.

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Our loan agreement with HealthCare Royalty Partners II, L.P., or HC Royalty, contains a number of restrictive covenants and other provisions, which, if violated, could result in the acceleration of the payment terms of our outstanding indebtedness, which could have an adverse impact on our business and financial condition.

In December 2012, we entered into a loan agreement with HC Royalty as lender, under which we agreed to borrow $50.0 million in two $25.0 million tranches, or the HC Royalty loan agreement. We drew down the first tranche in the amount of $25.0 million in December 2012 upon signing the HC Royalty loan agreement and we drew down the second tranche of $25.0 million in May 2013 as a result of our achievement of the milestone of U.S. approval of PROCYSBI. The HC Royalty loan agreement includes a number of affirmative and negative covenants, including the use of commercially reasonable efforts to exploit PROCYSBI and RP103 in specific markets and compliance with laws, as well as restrictions on mergers and sales of assets, incurrence of liens, incurrence of indebtedness and transactions with affiliates and other requirements. To secure the performance of our obligations under the HC Royalty loan agreement, we granted a security interest to HC Royalty in substantially all of our assets, the assets of our domestic subsidiaries and a pledge of stock of certain of our domestic subsidiaries. Our failure to comply with the terms of the HC Royalty loan agreement and related documents, the occurrence of a change of control of our Company or the occurrence of an uncured material adverse effect on our Company, or our wholly-owned subsidiary Raptor Pharmaceuticals, or the occurrence of certain other specified events, could result in an event of default under the HC Royalty loan agreement that, if not cured or waived, could result in the acceleration of the payment of all of our indebtedness to HC Royalty and interest thereon. Under the terms of the security agreement, in an event of default, the lender could potentially take possession of, foreclose on, sell, assign or grant a license to use our pledged collateral and assign and transfer the pledged stock of certain of our subsidiaries. A default or material adverse effect or change of control would also trigger a prepayment penalty which would require us to pay a substantially higher amount due than the current balance of our loan.

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

Any future sales of our products to government supported customers outside of the U.S. are likely to 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 PROCYSBI or our potential products in the EU is delayed or becomes unavailable, we may not be able to collect on amounts payable to us in reasonable time frames from such customers and our capital requirements will increase and our results of operations would be adversely affected.

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 conditions in the global financial markets and business and economic conditions. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to increase the price of PROCYSBI or other future products due to the process by which healthcare providers are reimbursed.

In the recent past, the U.S. credit and capital markets experienced historic dislocations and a massive liquidity crisis which caused financing to be unavailable in many cases, or caused the cost of financing to significantly increase. Any similar disruption in the financial markets may increase uncertainty in the debt and equity markets, which may negatively impact our ability to access financing on favorable terms in the future. In addition, our suppliers, manufacturers and other third parties important to our business also may be negatively affected by such potential market dislocations and disruptions, and their businesses may be disrupted which could adversely affect our business and results of operations.

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

Even though we have FDA approval of PROCYSBI, our recognized product sales in the U.S. may be reduced if PROCYSBI is 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 and when we launch PROCYSBI in the EU and in other countries outside the U.S., 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 and transaction losses in the future due to the effect of exchange rate fluctuations.

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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, including Christopher M. Starr, Ph.D., Chief Executive Officer; Julie Anne Smith, Chief Operating Officer; Georgia Erbez, Chief Financial Officer and Ted Daley, Chief Business Officer. 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 results of operations. We do not have key-man insurance on any of our employees.

There is no assurance that we will be able to retain key employees or consultants. 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. 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.

In addition to our employees, we rely and will continue to 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.

If we do not achieve our projected development and commercialization goals in the time frames we expect and announce, the price of our common stock may decline.

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 include the completion of reimbursement and pricing negotiations and a commercial launch in the EU and other territories, 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 institutional review board, or 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. Furthermore, our ability to launch commercial sales of PROCYSBI in the EU is subject to the timely completion of reimbursement and pricing negotiations with various governmental entities in the EU, which process can be lengthy and uncertain. See also the risk factors titled "The amount of our product sales of PROCYSBI in the EU is dependent upon the pricing and reimbursement guidelines adopted in each of the various countries in the EU, which levels may be below our current expectations" and "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 we do not meet the milestones as publicly announced, or as projected by various security 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.

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.

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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:

- 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 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.

Our trade secrets may not be 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.

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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 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 adversely affect our business, financial condition and results of operations. 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, financial condition and results of operations.

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

Most of our patent portfolio pertaining to PROCYSBI and RP103 for cystinosis and other therapeutic indications has been licensed from academic institutions. Our license agreements with these institutions include termination clauses which permit the licensor to terminate our license under certain circumstances, including if we materially breach our obligations and fail to remedy the breach within permitted cure periods. If one or more of our licenses is terminated, we would have no further right to use or exploit the patents, know-how and other intellectual property rights relating to those respective technologies and it could impact our ability to market PROCYSBI or continue our development programs of RP103 in other clinical indications. If this happens our financial condition and results of operations will be adversely affected, and we may have to cease our operations.

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 PROCYSBI or our drug product candidates. Many of our pharmaceutical competitors who are in areas competitive with us have greater capital resources, larger overall research and development staff 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 products, 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, suppliers 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 commercialization efforts and clinical development programs will require substantial future funding which will impact our operational and financial condition.

Excluding PROCYSBI for cystinosis, it will take a substantial period of time before we are able to develop our other drug product candidates into marketable drug products, if at all. The marketing and sales efforts for PROCYSBI and any future approved products, obtaining adequate reimbursement for products and our product development programs will require substantial additional capital, arising from costs to:

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- 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 PROCYSBI and any future approved products; and
- establish and develop quality control, manufacturing, regulatory, medical, 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 commercial sales of PROCYSBI in the U.S. and EU, our efforts to commercialize any future approved products, the success of our research initiatives, regulatory approvals, the timing of events outside our direct control, such as negotiations with healthcare payors and potential strategic partners and other factors. In addition, certain product programs may require collaborative agreements with corporate partners with greater financial and organizational resources than we have. Such agreements may require substantial time to complete and may not be available in the time frame desired or with acceptable financial terms, if at all. Any of these factors may significantly change the timing and amount of our cash requirements as they determine such one-time events as the receipt or payment of milestone-based and other payments.

Significant additional funds from outside financing sources will be required to support our operations. If we are unable to obtain them on acceptable terms, we may be required to cease or reduce further development of PROCYSBI and our other 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 operational and financial results will be adversely affected.

As of December 31, 2013, we had an accumulated deficit of approximately $205.4 million. We may need to raise additional capital and/or generate significant revenue at profitable levels to fund our development and commercialization programs in accordance with our plans.

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 commercial expenses for PROCYSBI, which would have a material adverse effect on our financial condition and operating results.

While we believe that based upon our projected PROCYSBI sales and planned operations, our cash and cash equivalents as of December 31, 2013 of approximately $83.1 million will be sufficient to meet our projected operational requirements and obligations through at least through the first half of 2015, 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 on a timely basis, 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, sales of PROCYSBI in the U.S., the execution of our launch of PROCYSBI in Europe 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 commercial expenses for PROCYSBI. If such actions are required, our financial condition and operating results will be adversely affected and our future value may be significantly reduced.

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Our cash flows and capital resources may be insufficient to make required payments on our indebtedness.

The required payments of principal and interest on our indebtedness under the HC Royalty loan agreement may require a substantial portion, or all, of our available cash to be dedicated to the service of these debt obligations. The loan bears interest at an annual fixed rate of 10.75% and a synthetic royalty based on the amount of PROCYSBI and other future approved product net revenues in a calendar year, and such royalty is payable quarterly. Principal payments under the HC Royalty loan agreement will become due beginning in June 2015.

There is no assurance that our business will generate sufficient cash flow or that we will have capital resources in an amount sufficient to enable us to pay our indebtedness to HC Royalty. If our cash flows and capital resources are insufficient to fund these debt service obligations, we may be forced to reduce or delay product development, sales and marketing, and capital and other expenditures, and we may be forced to restructure our indebtedness or raise additional capital through the issuance of equity or debt instruments. We cannot ensure that we will be able to refinance any of our indebtedness or raise additional capital on a timely basis, in sufficient amounts, on satisfactory terms or at all. In addition, the terms of the HC Royalty loan agreement may limit our ability to pursue any of these financing alternatives and these alternatives may not enable us to meet our scheduled debt service obligations. Failure to meet our debt service obligations may result in an event of default under the HC Royalty loan agreement, which would permit the lender to accelerate the payment of all of our indebtedness to HC Royalty and interest thereon, take possession of, foreclose on, sell, assign or grant a license to use, our pledged collateral and assign and transfer the pledged stock of our subsidiaries. A default or material adverse effect or change of control would also trigger a prepayment penalty which would require us to pay a substantially higher amount due than the current balance of our loan. This could have a material adverse impact on our financial condition and results of operations.

Risks Related to Our Common Stock

We may fail to meet publicly announced financial guidance or other expectations about our business, which would cause our stock to decline in value.

There are a number of reasons why we might fail to meet financial guidance or other expectations about our business, including, but not limited to, the following:

- unexpected difficulties in the commercialization of PROCYSBI in the U.S. or in the EU;
- the effectiveness of our sales, marketing and distribution efforts and overall success of our commercialization efforts in the U.S. and in the EU;
- lower than expected pricing and reimbursement levels, or no reimbursement at all, for PROCYSBI;
- current and future competitive products that have or obtain greater acceptance in the market than PROCYSBI;
- negative publicity about the results of our clinical trials, or those of others with similar or related products;
- if only a subset of or no affected patients respond to therapy with PROCYSBI or future products, if any;
- the inability to sell a product at the price we expect; or
- the inability to supply enough product to meet demand.

If we fail to meet our revenue and/or expense projections and/or other financial guidance for any reason, our stock price could decline.

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, affects and will continue to significantly affect the market price of our common stock. We face a number of risks including those described in this Risk Factors section, which may negatively impact the price of our common stock.

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The market price of our common stock also may be adversely impacted by broad market and industry fluctuations including general economic and technology trends, regardless of our operating performance. The NASDAQ Global Market has, from time to time, experienced extreme price and trading volume fluctuations, and the market prices of securities of pharmaceutical, biotechnology and other life sciences companies in a comparable stage to us have historically been particularly volatile and trading volume in such securities has often been relatively small. 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 may affect in particular the stock prices of companies such as ours that do not have conventional measures of financial and business health such as sales, earnings, profitability and related measures.

These broad market fluctuations, during which our stage of company and our industry may experience a stronger degree of market sensitivity, will adversely affect the trading price of our common stock. In the past, following periods of volatility in the market resulting in substantial price declines 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.

A substantial number of shares of our common stock are 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 raising capital to fund our operations, 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, including pursuant to the exercise of our currently outstanding warrants and stock options, or issuances of 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, exercises of our currently outstanding warrants and stock options and the subsequent sale of the shares acquired thereunder or the sale by us of shares of our common stock or securities convertible or exchangeable into our common stock for capital raising purposes 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.

We have entered into an Amended and Restated Sales Agreement with Cowen and Company, which, if utilized further, will create substantial dilution for our existing stockholders. The original Sales Agreement provided for at-the-market sales of our common stock with aggregate gross proceeds of up to $40.0 million. On July 3, 2013, we entered into an Amended and Restated Sales Agreement to increase the aggregate gross sales proceeds that may be raised pursuant to the agreement to $100.0 million. Sales in the at-the-market offering were made pursuant to our prospectus supplement dated April 30, 2012, as amended by Amendment No. 2 dated July 3, 2013, 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. As of December 31, 2013, an aggregate of approximately $46.2 million remained available for future sales of our common stock under the Amended and Restated Sales Agreement.

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 earnings 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.

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.

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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 certificate of incorporation 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 21,330 square feet of office and laboratory space as our headquarters in Novato, California. We plan to move to an adjacent facility of 30,989 square feet when it becomes available approximately May 2014.

In addition, we lease small office spaces in Paris, France for our French general manager and future French personnel; in Frankfurt, Germany for our German general manager and his field-based staff; and plan to lease office space in the Netherlands for our European sales, marketing and administrative headquarters within the next several months.

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.

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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 February 28, 2014, there were 62,479,286 shares of our common stock outstanding. There is no public trading market for our warrants. The closing price for our common stock on February 28, 2014 was $15.83 per share.

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
Fiscal Year Ended December 31, 2013:		
First Quarter (January 1 – March 31, 2013)	$6.28	$4.71
Second Quarter (April 1 – June 30, 2013)	10.47	5.40
Third Quarter (July 1 – September 30, 2013)	15.00	9.26
Fourth Quarter (October 1 – December 31, 2013)	15.29	11.09
Four Months Ended December 31, 2012:		
First Quarter (September 1 – November 30, 2012)	5.74	4.35
December 1, 2012 – December 31, 2012	6.04	5.06
Fiscal 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
Fiscal 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 February 28, 2014, there were approximately 139 holders of record of our common stock. Additionally, on such date, options held by 128 persons to acquire up to, in the aggregate, 9,532,948 shares and warrants held by 7 persons to acquire up to, in the aggregate, 334,764 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 earnings 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 upon, among other things, 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.

Recent Sales of Unregistered Securities

We did not issue any unregistered equity securities during the year ended December 31, 2013.

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Purchase of Equity Securities and Affiliated Purchasers

We have not repurchased any shares of our common stock since inception.

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.), NASDAQ Benchmark TR 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 31, 2010, 2011, 2012, for the 4 months ended December 31, 2012 and as of our year ended December 31, 2013. Our common stock is traded under the ticker symbol RPTP 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	2011	2012	Four months ended December 31, 2012	December 31, 2013
Raptor Pharmaceutical Corp.	$ 100	$90.30	$143.33	$150.61	$177.27	$394.55
NASDAQ U.S. Composite Index	100	100.54	124.79	152.68	151.02	210.35
NASDAQ U.S. Benchmark TR Index	100	101.96	120.68	141.52	145.47	194.18
NASDAQ Biotechnology Index	100	96.73	119.13	168.82	170.41	282.22

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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 our 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 our consolidated balance sheet data as of December 31, 2013 and 2012 and as of August 31, 2012, 2011, 2010 and 2009 and its consolidated statements of operations and comprehensive loss data for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012, 2011, 2010 and 2009.

	For the fiscal year ending December 31,	For the four months ending December 31,	For the fiscal years ending August 31,			
(In millions, except per share data)	2013	2012	2012	2011	2010	2009
Statements of operations and comprehensive loss data:						
Revenues	$ 16.9	$ 0	$0	$0	$0	$0
Operating expenses:						
Cost of sales	1.7	0	0	0	0	0
Research and development	29.2	8.9	21.4	14.8	9.3	6.5
Selling, general and administrative	37.9	9.0	14.7	6.2	3.7	2.7
Total operating expenses	68.8	17.9	36.1	21.0	13.0	9.2
Loss from operations	(51.9)	(17.9)	(36.1)	(21.0)	(13.0)	(9.2)
Interest income	0.1	0.2	0.3	0.1	0	0
Interest expense	(6.8)	(0.1)	0	0	0	0
Foreign currency transaction gains	0	0.1	0.2	0	0	0
Gains (losses) on short-term investments	(0.1)	(0.1)	0.2	0	0	0
Adjustment to fair value of common stock warrants	(10.7)	(1.5)	(3.2)	(16.3)	(5.9)	0
Net loss	(69.4)	(19.3)	(38.6)	(37.2)	(18.9)	(9.2)
Other comprehensive loss:						
Foreign currency translation adjustment	(0.3)	(0.1)	(0.1)	0	0	0
Comprehensive loss	$ (69.7)	$ (19.4)	$(38.7)	$(37.2)	$(18.9)	$(9.2)
Net loss per share:						
Basic and diluted	$ (1.20)	$ (0.37)	$(0.80)	$(1.15)	$(0.85)	$(0.64)
Weighted-average shares outstanding:	57.9	51.7	48.1	32.3	22.2	14.4

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(In millions)	12/31/13	12/31/12	8/31/12	8/31/11	8/31/10	8/31/09
Balance Sheet Data:						
Cash, cash equivalents and short-term investments	$ 83.1	$ 58.4	$ 38.9	$ 15.2	$ 17.0	$ 3.7
Working capital (deficit)	66.2	37.0	20.6	(11.0)	(0.3)	2.7
Total assets	108.7	68.1	48.3	22.6	24.4	6.6
Common stock warrant liability	7.1	16.4	17.3	23.6	15.8	0
Note payable	50.0	25.0	0	0	0	0
Total liabilities	80.2	48.2	21.6	26.7	17.6	1.1
Accumulated deficit	(205.4)	(135.9)	(116.6)	(78.0)	(40.8)	(21.9)
Total stockholders' equity (deficit)	28.6	19.9	26.7	(4.1)	6.8	5.5

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

Overview

You should read the following discussion in conjunction with our consolidated financial statements as of December 31, 2013, 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."

Change in Fiscal Year End

On December 4, 2012, our board of directors approved a change in our fiscal year end from August 31 to December 31. The change became effective at the end of the four months ended December 31, 2012. All references to "fiscal years" or "years" prior to this change refer to the twelve-month fiscal period covering September 1 through August 31, and each year after December 31, 2012, the fiscal year covers January 1 through December 31.

Plan of Operation and Overview

We are a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. On April 30, 2013, our first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules, or PROCYSBI, received marketing approval from the U.S. Food and Drug Administration, or FDA, for the management of nephropathic cystinosis in adults and children six years and older. On September 6, 2013, our European equivalent, PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received a Community or EU marketing authorization from the European Commission, or EC, as an orphan medicinal product for the management of proven nephropathic cystinosis. The EU marketing authorization allows us to commercialize PROCYSBI in the 28 Member States of the EU plus Norway, Liechtenstein, and Iceland (which are not EU Member States but are part of the EEA). PROCYSBI received 7 years and 10 years of market exclusivity as an orphan drug in the U.S. and the EU, respectively. We commenced commercial sales of PROCYSBI in the U.S. in mid-June 2013 and plan to launch PROCYSBI in the EU in the first half of 2014. With FDA approval of PROCYSBI and the commencement of commercial sales, we are no longer considered to be in the development stage.

Clinical Development Programs

Our three active clinical development programs utilize the same active pharmaceutical ingredient, cysteamine bitartrate, or RP103, our proprietary extended and delayed-release formulation capsule containing enteric coated micro-beads of cysteamine bitartrate. Cysteamine bitartrate was approved in the U.S. in 1994 and the EU in 1997 as an orally available immediate-release powder in a capsule for the management of, and is the current standard of care for, cystinosis. We have an exclusive worldwide license to delayed-release cysteamine bitartrate from UCSD which is the basis for our proprietary formulation of cysteamine. Our proprietary extended and delayed-release formulation, RP103, is a capsule containing enteric-coated microbeads of cysteamine bitartrate. We currently have product candidates in clinical development designed to potentially treat Huntington's disease, or HD, non-alcoholic fatty liver disease, or NAFLD, Leigh syndrome and other mitochondrial disorders.

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 programs, for which we are seeking development partners include our cysteamine dioxygenase, or ADO, program and our HepTide™ program, for the potential treatment of hepatocellular carcinoma and other cancers susceptible to induced lysosomal storage.

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Future Activities

Over the next fiscal year, our efforts will be focused on increasing sales of PROCYSBI in the U.S. and successfully launching PROCYSBI in the EU in the first half of 2014; filing a New Drug Submission, or NDS, for PROCYSBI with Health Canada in the second half of 2014; conducting a clinical trial to evaluate PROCYSBI in cystinosis patients that are cysteamine-naïve, as well as other supporting trials in underdeveloped markets; developing select global markets with significant numbers of known cystinosis patients; screening for undiagnosed and unidentified late-onset nephropathic cystinosis patients; initiating and supporting our clinical trials of RP103 for the potential treatment of Leigh syndrome and mitochondrial disorders; supporting our novel preclinical programs; identifying promising in-licensing candidates; and continuing the development of our RP103 clinical pipeline in new indications including Rett Syndrome,.

We plan to seek additional business development partners in Asia for our Convivia™ product candidate. We may also develop new preclinical, clinical and or commercial opportunities, including proprietary molecules 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 and results of operations.

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

Revenue Recognition and Accounts Receivable

We recognize revenue in accordance with the Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC 605, Revenue Recognition, when the following criteria have been met: persuasive evidence of an arrangement exists; delivery has occurred and risk of loss has passed; the seller's price to the buyer is fixed or determinable and collectability is reasonably assured. We determine that persuasive evidence of an arrangement exists based on written contracts that define the terms of the arrangements. Pursuant to the contract terms, we determine when title to products and associated risk of loss has passed onto the customer. We assess whether the fee is fixed or determinable based on the payment terms associated with the transaction and whether the sales price is subject to refund or adjustment. We assess collectability based primarily on the customer's payment history and creditworthiness.

PROCYSBI is currently only available for distribution from our U.S. specialty pharmacy partner, the Accredo Health Group, Inc., or Accredo, which is currently our only customer and ships directly to patients. Our distributor in the EU will be the Almac Group, Ltd. for the commercial launch in the EU anticipated to occur in the first half of 2014. PROCYSBI is not available in retail pharmacies. Prior authorization of coverage level by patients' private insurance plans, our patient assistance program, or PAP, or government payors is a prerequisite to the shipment of PROCYSBI to patients. Revenue is recognized once the product has been shipped by the specialty pharmacy to patients because at this time, we are unable to reasonably estimate rebate percentages based upon our lack of sufficient historical data. Billings to our distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenue by us until such shipments to patients occur.

We record revenue net of expected discounts, distributor fees, returns and rebates, including those paid to Medicare and Medicaid in the U.S. Allowances are recorded as a reduction of revenue at the time product sales are recognized. Allowances for government rebates and discounts are established based on the actual payor information, which is known at the time of shipment to patients, and the government-mandated discount rates applicable to government-funded programs. The allowances are adjusted to reflect known changes in the factors that may impact such allowances in the quarter the changes are known.

Trade accounts receivable are recorded net of product sales allowances for prompt-payment discounts and chargebacks. Estimates for chargebacks and prompt-payment discounts are based on contractual terms and our expectations regarding the utilization rates.

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Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price, with cost determined on a first-in, first-out basis. Inventories are reviewed periodically to identify slow-moving inventory based on sales activity, both projected and historical, as well as product shelf-life. Prior to the approval of PROCYSBI by the FDA on April 30 2013 and in Europe, prior to EMA approval on September 6, 2013, we recorded the purchase of raw materials and the manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to approval, we began capitalizing these costs as commercial inventory. Cost of sales includes the cost of inventory sold or reserved, manufacturing and supply chain costs, product shipping and handling costs, amortization of licensing approval milestone payments and licensing royalties payable to the University of California, San Diego, or UCSD.

Note Payable

Note payable consists of our loan agreement with HealthCare Royalty Partners II, L.P., or HC Royalty, as lender, under which we borrowed $50.0 million in two $25.0 million tranches received in December 2012 and May 2013. The loan bears interest at an annual fixed rate of 10.75% of outstanding principal and includes a synthetic royalty component based on net product sales, including PROCYSBI, in a calendar year. With respect to the first $25.0 million tranche, for each calendar year, the loan bears a royalty rate of 6.25% of the first $25.0 million of product net revenues, 3.0% of product net revenues for such calendar year in excess of $25.0 million and below $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly. With respect to the second $25.0 million tranche, for each calendar year, the loan bears a royalty rate of 6.0% of the first $25.0 million of net revenues for such calendar year, 3.0% of product net revenues for such calendar year in excess of $25.0 million and below $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly. The fixed and royalty interest are recognized as interest expense as incurred. The revenue royalty related interest may lead to significant fluctuations in interest expense from period to period.

Impairment of Goodwill and Intangible Assets

Goodwill represents the excess of the purchase price over the fair value of tangible and identified intangible net assets of businesses acquired. Goodwill is not amortized, but is evaluated for impairment on an annual basis or more often when impairment indicators are present. We have one reporting unit. Therefore, our consolidated net assets, including existing goodwill and other intangible assets, are considered to be the carrying value of the reporting unit. If the carrying value of the reporting unit is in excess of its fair value, an impairment may exist, and we must perform the second step of the analysis, in which the implied fair value of the goodwill is compared to its carrying value to determine the impairment charge, if any. If the estimated fair value of the reporting unit exceeds the carrying value of the reporting unit, goodwill is not impaired and no further analysis is required. We performed our goodwill impairment test as of December 31, 2013 and noted no impairment.

We make judgments about the recoverability of purchased intangible assets with finite lives whenever events or changes in circumstances indicate that impairment may exist. Recoverability of purchased intangible assets with finite lives is measured by comparing the carrying amount of the asset to the future undiscounted cash flows the asset is expected to generate. Impairment, if any, is measured as the amount by which the carrying value exceeds the fair value of the impaired asset.

Assumptions and estimates about future values and remaining useful lives of our purchased intangible assets are complex and subjective. They can be affected by a variety of factors, including external factors such as industry and economic trends and internal factors such as changes in our business strategy and our internal forecasts.

Common Stock Warrant Liabilities

The common stock warrants we issued in connection with certain fiscal year 2010 equity financings contain conditional obligations that may require us to transfer cash to settle the warrants upon the occurrence of certain fundamental transactions. Therefore, we have classified the warrants as liabilities. We re-measure the liability at the end of every reporting period with the change in value reported in our consolidated statements of operations and comprehensive loss. At the exercise date, the fair values of these warrants are re-measured and reclassified to equity. We use the Black-Scholes option pricing model as our method of valuation for warrants that are subject to warrant liability accounting. The determination of the fair value as of the reporting date is affected by our stock price as well

as assumptions regarding a number of highly complex and subjective variables. These variables include, but are not limited to, expected stock price volatility over the term of the security and risk-free interest rate. In addition, the Black-Scholes option pricing model requires the input of an expected life for the securities for which we have estimated based upon the stage of our development. The fair value of the warrant liability is revalued each balance sheet date utilizing Black-Scholes valuation model computations with the decrease or increase in fair value being reported in the statement of operations and comprehensive loss as other income or expense, respectively. The primary factors affecting the fair value of the warrant liability are our stock price and volatility. In addition, the Black-Scholes option pricing model requires the input of highly subjective assumptions, and other reasonable assumptions could provide differing results.

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We reported a net loss of $69.4 million for the year ended December 31, 2013. If our December 31, 2013 closing stock price had been 10% lower, our net loss would have been approximately $0.9 million lower. If our December 31, 2013 closing stock price had been 10% higher, our net loss would have been approximately $0.9 million higher

If our December 31, 2013 volatility assumption had been 10% lower, our net loss would have been approximately $0.1 million lower. If our December 31, 2012 volatility assumption had been 10% higher, our net loss would have been approximately $0.1 million higher.

We reported a net loss of $19.3 million for the four months ended December 31, 2012. If our December 31, 2012 closing stock price had been 10% lower, our net loss would have been approximately $2.0 million lower. If our August 31, 2012 closing stock price had been 10% higher, our net loss would have been approximately $2.0 million higher.

If our December 31, 2012 volatility assumption had been 10% lower, our net loss would have been approximately $0.7 million lower. If our December 31, 2012 volatility assumption had been 10% higher, our net loss would have been approximately $0.7 million higher.

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. 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. We intend to maintain the valuation allowance until sufficient positive evidence exists to support the reversal of the valuation allowance. Any decision to reverse part or all of the valuation allowance would be based on our estimate of future profitability.

We identify uncertain tax positions and record or disclose any resulting potential tax liability based upon whether the position is more likely than not sustainable upon examination. We consider proposed assessments by tax authorities, changes in facts and circumstances, issuance of new regulations or new case law and negotiations between tax authorities of different countries concerning our transfer prices or intellectual property transfers. As of December 31, 2013, we have identified no uncertain tax positions.

We file U.S. Federal, California, various other state income and other tax returns and various foreign country income tax returns. We are currently not subject to any income tax examinations. Due to our net operating losses, all tax years generally remain open in each jurisdiction.

Stock-Based Compensation

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.

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 five years; the expected life of five 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 a combination of the actual annualized volatility of our common stock price as quoted on NASDAQ since the closing of our 2009 Merger on September 30, 2009 and of annualized volatility of peer companies; 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 corporate stage of development. If actual results differ significantly from these estimates, stock-based compensation expense and results of operations could be materially impacted. See Note 8 of our consolidated financial statements for a further discussion of our accounting for stock-based compensation.

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Results of Operations
For the year ended December 31, 2013

(In millions)	For the year ended December 31, 2013
Revenues	$ 16.9
Operating expenses:	
Cost of sales	1.7
Research and development	29.2
Selling, general and administrative	37.9
Total operating expenses	68.8
Loss from operations	(51.9)
Interest expense	(6.9)
Adjustment to the fair value of common stock warrants	(10.7)
Other	0.1
Net loss	$ (69.4)

Revenue
We recognized $10.2 million in PROCYSBI net product sales for the fourth quarter of 2013. Net product sales for the year 2013 totaled $16.9 million. There were no product sales for the comparable prior periods as PROCYSBI became commercially available in the U.S. in June 2013.
Cost of Sales
Prior to the approval of PROCYSBI by the FDA on April 30, 2013 and in Europe, prior to EMA approval on September 6, 2013, we recorded manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to approval, we began capitalizing these costs as commercial inventory. PROCYSBI became commercially available in mid-June 2013, and we began recognizing manufacturing costs as capitalized inventory. Costs capitalized as inventory are expensed as cost of sales as product is sold. During the year ended December 31, 2013, we recorded a $0.4 million reserve as cost of sales representing commercial inventory that was capitalized subsequent to FDA approval but written off due to an unanticipated minor change in the finished product presentation. Cost of sales includes the cost of inventory sold, inventory and reserve costs, manufacturing and supply chain costs, product shipping and handling costs, amortization of licensing approval milestone payments and licensing royalties payable to UCSD.
Research and Development
For the year ended December 31, 2013, our research and development expenses consisted primarily of costs associated with the manufacturing and testing of clinical and commercial materials in anticipation for our potential launch of RP103 for cystinosis, clinical trial research expenses and employee compensation. The increase in research and development expenses relates primarily to increased clinical product manufacture of RP103 for the potential treatment of cystinosis (prior to the capitalization of such amounts upon FDA and EMA approval); HD, NASH, cystinosis extension, and other supporting study expenses and related employee compensation, offset by a reduction in Phase 3 cystinosis clinical trial expenses.

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Major Program expenses recorded as research and development:

Major Program (stage of development) (In millions)	For the year ended December 31, 2013
RP103:	
Cystinosis (pre-commercial and extension)	$ 14.8
HD (clinical)	0.8
NASH (clinical)	2.0
Preclinical programs	1.1
Other programs	0.8
R & D personnel and other costs not allocated to programs	9.7
Total research and development expenses	$ 29.2

Selling, General and Administrative Expenses

For the year ended December 31, 2013, our selling, general and administrative expenses consisted primarily of employee compensation, marketing and reimbursement studies, consulting, accounting, legal and patent fees. The increase in selling, general and administrative expenses relates primarily to increased expenses for pre-commercial operations requirements for RP103 for the potential treatment of cystinosis, employee compensation, stock-compensation for employees and directors, legal fees and investor relations costs.

Major Program expenses recorded as selling, general and administrative expenses:

For the year ended December 31, 2013, our program expenses in selling, general and administrative expenses consisted primarily of pre-commercial and commercial launch expenses for PROCYSBI.

Major Program (stage of development) (In millions)	For the year ended December 31, 2013
RP103:	
Cystinosis (pre-commercial and commercial)	$ 9.8
HD (clinical)	0.5
NASH (clinical)	0.1
Other programs	0.2
Total selling, general and administrative expenses related to programs	$ 10.6

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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. We currently have product candidates in clinical development as potential treatments for HD, NAFLD, Leigh syndrome and other mitochondrial disorders and ALDH2. Our preclinical programs are based upon bioengineered novel drug candidates that are designed to target cancer and other diseases. 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 18 months. In addition, the timing and costs of development of our programs beyond the next 18 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 Expense

Interest expense for the year ended December 31, 2013 was approximately $6.8 million, which primarily represented interest on our $50 million note payable to HC Royalty including the "synthetic" royalty interest on sales of PROCYSBI.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of approximately $10.7 million for the year ended December 31, 2013.

For the four months ended December 31, 2012 and 2011

(In millions)	For the four months ended December 31, 2012	2011 (Unaudited)
Revenues	$0	$ 0
Operating expenses:		
Research and development	8.9	6.3
General and administrative	9.0	3.2
Total operating expenses	17.9	9.5
Loss from operations	(17.9)	(9.5)
Adjustment to the fair value of common stock warrants	(1.5)	(5.0)
Other	0.1	(1.6)
Net loss	$(19.3)	$ (16.1)

Research and Development

For the four months ended December 31, 2012, our research and development expenses consisted primarily of costs associated with the manufacturing and testing of clinical and commercial materials in anticipation for our approval and commercial launch of RP103 for cystinosis, clinical trial research expenses and employee compensation. The increase in research and development expenses for the four months ended December 31, 2012 compared to the four months ended December 31, 2011 relates primarily to increased product manufacture of RP103 for the potential

treatment of cystinosis, HD and NASH, additional cystinosis extension and other supporting study expenses, employee compensation, offset by a reduction in Phase 3 cystinosis clinical trial expenses.

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Major Program expenses recorded as research and development:

Major Program (stage of development) (In millions)	For the four months ended December 31, 2012
RP103:	
Cystinosis (pre-commercial)	$ 3.9
HD (clinical)	0.1
NASH (clinical)	1.1
Preclinical programs	0.2
Other programs	0.2
R & D personnel and other costs not allocated to programs	3.4
Total research and development expenses	$ 8.9

General and Administrative Expenses

For the four months ended December 31, 2012, our general and administrative expenses consisted primarily of employee compensation, marketing and reimbursement studies, consulting fees and legal and patent fees. The increase in general and administrative expenses for the four months ended December 31, 2012 compared to the four months ended December 31, 2011 relates primarily to increased expenses for pre-commercial operations requirements for RP103 for the potential treatment of cystinosis, employee compensation, stock-compensation for employees and directors, legal fees and investor relations costs.

Major Program expenses recorded as general and administrative expenses:

For the four months ended December 31, 2012, our program expenses in general and administrative expenses consisted primarily of pre-commercial launch expenses for RP103, such as market research and market access studies.

Major Program (stage of development) (In millions)	For the four months ended December 31, 2012
RP103:	
Cystinosis (pre-commercial)	$ 3.2
HD (clinical)	0
NASH (clinical)	0
Preclinical programs	0
Other programs	0.2
Total general and administrative expenses related to programs	$ 3.4

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 pre-commercial launch of RP103 for the potential treatment of cystinosis.

Adjustment to the Fair Value of Common Stock Warrants

Adjustment to the fair value of common stock warrants was a loss of approximately $1.5 million for the four months ended December 31, 2012 compared to a loss of approximately $5.0 million for the four months ended December 31, 2011 (unaudited), a decrease in loss of approximately $3.5 million resulting primarily from a smaller increase in stock price, shorter remaining term and the lower number of remaining warrants outstanding due to warrant exercises as of December 31, 2012.

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Years ended August 31, 2012 and 2011

(In millions)	For the year ended August 31, 2012	2011
Revenues	$0	$0
Operating expenses:		
Research and development	14.7	6.2
General and administrative	21.4	14.8
Total operating expenses	36.1	21.0
Loss from operations	(36.1)	(21.0)
Adjustment to the fair value of common stock warrants	(3.2)	(16.3)
Other	0.7	0.1
Net loss	$(38.6)	$(37.2)

Research and Development

Research and development expenses include medical, clinical, regulatory and scientists' compensation and benefits, lab collaborations, preclinical studies, clinical trials, amortization of intangible assets and allocated human resources and facilities expenses. Research and development expenses for the year ended August 31, 2012 increased by approximately $6.7 million over the prior fiscal year primarily due to:

Reason for Increase (Decrease) (In millions)	Increase (Decrease)
Increased product manufacture of RP103 for the potential treatment of cystinosis, HD, NASH	$ 4.4
Tax grants and expense reimbursements for preclinical and clinical programs not available in fiscal year 2012	0.8
R&D compensation	
Salary, bonus and benefits increases and new hire compensation	0.6
Stock-based compensation expense, employees (non-cash)	0.5
Write-off of capitalized intangibles no longer being developed	0.8
Preclinical studies including research materials and lab services	0.5
Reduction in Phase 3 cystinosis trial expense partially offset by extension study and other smaller studies	(1.7)
Other, net	0.8
Research and development increase	$ 6.7

Research and development expenses include the following:

Major Program (stage of development) (In millions)	Year ended August 31, 2012	2011
RP103 – All indications (clinical/pre-commercial)	$18.2	$10.5

Minor and inactive programs	1.7	0.5
R & D personnel and other costs not allocated to programs	1.5	3.8
Total research and development expenses	$21.4	$14.8

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General and Administrative Expenses

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 $8.6 million compared to the prior fiscal year. The increase was primarily due to:

Reason for Increase (Decrease) (In millions)	Increase (Decrease)
Increase in General and Administrative expenses:	
Pre-commercial operations requirements RP103 for the potential treatment of cystinosis:	
Pre-commercial consulting services	$ 2.0
Tax study and advisory fees related to EU headquarters	0.9
Salary, benefits and bonuses for commercial operations personnel	0.5
Salary, benefit and bonus increases and new finance and human resources personnel	1.2
Stock-based compensation expense, employees and directors	2.1
Legal fees due to in-licensing of intellectual property	0.6
Investor relations costs including proxy mailing and solicitation, press releases, webcasting, XBRL filing costs	0.5
Other, net	0.8
General and administrative increase	$ 8.6

Major Program expenses recorded as general and administrative expenses:

	Year ended August 31,	
Major Program (stage of development) (In millions)	2012	2011
RP103 – All indications (clinical and pre-commercial)	$2.7	$1.0
		0.2
Minor and inactive programs	0.1	

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 which resulted primarily from the lower number of remaining warrants outstanding due to warrant exercises.

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Liquidity and Capital Resources

Capital Resources

As of December 31, 2013, we had $83.1 million in cash and cash equivalents, of which $4.2 million is held by our foreign subsidiaries, $30.1 million in current liabilities (of which $7.1 million represented the common stock warrant liability, and is expected to be settled in shares) and $66.2 million of net working capital. During the year ended December 31, 2013, we raised $23.7 million of net proceeds from the second tranche of financing under our loan agreement with HC Royalty Partners, $38.8 million in proceeds after commissions under our at-the-market (ATM) common stock sales agreement, $10.3 million net proceeds from warrant exercises and $2.5 million net proceeds from stock option exercises. We believe that our cash balance will be sufficient to meet our projected operational requirements and obligations at least through the first half of 2015.

Under the terms of the HC Royalty loan agreement executed on December 20, 2012, we received $23.4 million in net proceeds from the first tranche of the loan at closing in December 2012. We received an additional $23.7 million in net proceeds in May 2013 from the second tranche upon FDA approval of RP103 for the management of cystinosis. The loan matures on March 31, 2020, bears interest at an annual fixed rate of 10.75% and has a synthetic royalty, tiered down, based on a percentage of net product sales. The loan is interest-only until May 2015. The proceeds from the loans are being used primarily to fund the commercialization of PROCYSBI for the management of cystinosis, advance our development programs and for general corporate purposes.

On April 30, 2012, we entered into a Sales Agreement with Cowen and Company, or Cowen, to sell shares of our common stock, with aggregate gross sales proceeds of up to $40 million, 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.

On July 3, 2013, we amended and restated the Sales Agreement to increase the aggregate gross sales proceeds that may be raised to $100 million. Cumulatively through December 31, 2013, we sold 7,599,474 shares under the ATM offerings at a weighted-average selling price of $7.08 per share for net proceeds of approximately $52.1 million.

As of February 28, 2014, 334,764 shares (including the placement agent warrant described below) of our common stock warrants were outstanding, all of which warrants were issued pursuant to private placement purchase agreements, dated as of August 9, 2010.

Future Funding Requirements

We will need to raise additional capital either through the sale of equity or debt securities (including convertible debt securities) to fund our operations and to, among other activities, develop and commercialize RP103 for the management of cystinosis and other indications. Our future capital requirements may be substantial, and will depend on many factors, including:

- the success of our U.S. commercial launch of PROCYSBI, including patient uptake and revenue;
- the cost of establishing the sales and marketing capabilities in the EU necessary to launch PROCYSBI in the EU in the first half of 2014;
- our ability to negotiate reimbursement and pricing of PROCYSBI in the EU;
- the successful launch of PROCYSBI in the EU;
- the cost of our manufacturing-related activities in support of PROCYSBI and RP103;
- the cost of activities related to the regulatory submission of PROCYSBI in Canada;
- the cost of additional clinical trials in order to obtain regulatory approvals for PROCYSBI in non-U.S. and non-EU countries;
- the timing and cost of our ongoing clinical programs for RP103, including: evaluating PROCYSBI in treatment-naïve cystinosis patients, and other supportive studies; evaluating RP103 as a potential treatment for Huntington's disease; and evaluating RP103 as a potential treatment for NAFLD;
- the cost of regulatory submissions, as well as the preparation, initiation and execution of clinical trials in potential new clinical indication using RP103;
- the cost of evaluating and potentially acquiring or in-licensing new drug compound(s) for potential clinical development;

- the cost of business development activities to identify, test and potentially license or acquire new therapeutic drug candidates; and
- the cost of filing, prosecuting and enforcing patent claims.

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There can be no assurance that we will be successful in raising sufficient funds when needed. Additional financing may not be available in amounts or on terms satisfactory to us or at all.

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 candidate RP103 for the potential treatment of HD and NASH; 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 addition of new studies in support of cystinosis, HD, NAFLD, Leigh syndrome and other indications.

Selling, General and Administrative Activities

Selling, general and administrative costs in the next 12 months will consist primarily of sales activities surrounding the sale of PROCYSBI in the U.S. and the commercial launch of PROCYSBI in the EU, 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 selling, general and administrative expenses will continue to increase in support of PROCYSBI sales growth, as well as an increase in facilities and administrative expenses to support our rapid growth.

Capital Expenditures

In the next 12 months, we expect to increase our capital expenditures on leasehold improvements on new facilities, laboratory and office equipment and computer software and hardware as we continue to increase our staff in calendar 2014.

Contractual Obligations

Contractual Obligations With UCSD Relating To The Acquisition Of The DR Cysteamine (RP103) License

We are obligated to pay an annual maintenance fee to UCSD for the exclusive license to develop RP103 for certain indications 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 milestone payments (ranging in size for orphan and non-orphan indications) upon the occurrence of certain events during the life of the License Agreement. These include a royalty on commercial net sales from products developed pursuant to the License Agreement - a percentage of sublicense fees - a percentage of sublicense royalties - and a minimum annual royalty commencing the year we begin commercially selling any products pursuant to the License Agreement. Under the License Agreement, we are obligated to fulfill predetermined milestones within a specified number of years from the effective date of the License Agreement, depending on the indication. Cumulatively, we have expensed approximately $0.9 million in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, Huntington's disease and NASH and on regulatory filings in cystinosis. In March 2012, we filed an MAA with the EMA, as well as an NDA with the FDA for RP103 for the potential treatment of cystinosis. In conjunction with the achievement of MAA/NDA filing milestone, we paid additional milestone payments to UCSD pursuant to this license. Based on approval of RP103 by the FDA on April 30, 2013 we paid a milestone license of $0.75 million which was capitalized as commercial IP and is being amortized as expense in cost of sales over the life of the patent. Based on approval by the EMA of RP103 on September 6, 2013, we paid a milestone license of $0.5 million which was capitalized as commercial IP and is being amortized as expense in cost of sales over the life of the patent. In 2013, we began paying UCSD royalties based upon our net sales of PROCYSBI. Other future milestones will be payable based on other approvals of cystinosis throughout the rest of the world.

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Other Contractual Obligations

We have contractual obligations under our capital and operating leases and other obligations related to research and development activities, purchase commitments and licenses. Information about these obligations as of December 31, 2013 is presented in the table below:

	Payments Due by Period				
(In thousands)	< 1 Year	1 - 3 Years	3 - 5 Years	> 5 Years	Total
Debt principal	$0	$17,500	$20,000	$12,500	$50,000
Capital lease obligations	18	36	8	0	62
Operating lease obligations	710	1,567	1,868	2,497	6,642
Purchase commitments and research and development/clinical	7,902	3,483	140	180	11,705
Total	$8,630	$22,586	$22,016	$15,177	$68,409

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 NAFLD clinical collaboration. The future commitments pursuant to these agreements, some of which include estimates of amounts or timing of payments, 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 $14.9 million, which are primarily due upon the achievement of certain development and commercial milestones if such milestones occur before certain dates in the future. These contingent payments are not included in the table above as we cannot reliably predict their timing or occurrence.

In conjunction with our loan agreement, we have contractual interest payments that began in December 2012 at a fixed rate of 10.75% plus a percentage of product revenue. The fixed interest amount that remains committed through the term of loan is approximately $22.1 million.

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.

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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, SAS, GmbH and CV, which use the 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 December 31, 2013. We do not currently hedge our foreign currency transactions.

Interest Rate Risk

As of December 31, 2013, we had approximately $70.6 million in cash equivalent money market accounts, yielding approximately 0.05% per year. 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 December 31, 2013.

ITEM 8: FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The information required to be filed in this item appears on pages 91 to 120 of this Annual Report on Form 10-K.

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

Financial Statements

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PART II – FINANCIAL INFORMATION

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

None.

ITEM 9A: CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

As of December 31, 2013, 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. Our disclosure controls and procedures were designed to provide reasonable assurance of achieving our control objectives. 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) were effective at a reasonable assurance level as of December 31, 2013.

Management's Report on Internal Control Over Financial Reporting

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.

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 the 1992 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 December 31, 2013.

Limitations on the Effectiveness of Controls

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.

Attestation Report of Grant Thornton LLP

Grant Thornton LLP, an independent registered public accounting firm, has audited the consolidated financial statements included in this Annual Report on Form 10-K and, as part of the audit, has issued a report, included herein, on the effectiveness of our internal control over financial reporting as of December 31, 2013.

Changes in Internal Control over Financial Reporting

During the quarter ended December 31, 2013, 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.

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ITEM 9B: OTHER INFORMATION

On February 7, 2014, the Board of Directors approved increasing the 2014 base salaries for Dr. Christopher Starr, our Chief Executive Officer, and Julie Anne Smith, our Chief Operations Officer, Executive Vice President, Strategy, by 15% to $471,500 and $402,500, respectively.

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PART III

ITEM 10: DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Directors

For each of our directors, the following table sets forth their name, age as of March 17, 2014 and position.

Name	Age	Position(s) Held with the Company
Raymond W. Anderson (2)(3)	72	Director
Suzanne L. Bruhn, Ph.D. (1)(3)	50	Director
Richard L. Franklin, M.D., Ph.D. (1)(2)	68	Director
Llew Keltner, M.D., Ph.D. (1)	64	Chairman of the Board of Directors
Erich Sager (2)	56	Director
Vijay B. Samant (1)(3)	61	Director
Christopher M. Starr, Ph.D.	61	Chief Executive Officer and Director
Timothy P. Walbert (2)(3)	46	Director

(1) Member of the Corporate Governance and Nominating Committee.
(2) Member of the Audit Committee.
(3) Member of the Compensation Committee.

Business Experience and Directorships

The following describes the background of our directors.

Raymond W. (Bill) Anderson. Mr. Anderson has served as a director of the Company since September 2009 and has more than 30 years of biopharmaceutical/medical technology sector experience, primarily focused in financial management. Mr. Anderson worked at Dow Pharmaceutical Sciences, Inc. (now a wholly owned subsidiary of Valeant Pharmaceuticals International) from July 2003 until he retired in June 2010. He most recently served as Dow's Managing Director since January 2009 and previously served as Chief Financial Officer and Vice President, Finance and Administration. 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 Pharmaceutical Inc. from June 1998 to January 2002. Prior to June 1998, Mr. Anderson held similar executive-level positions with other biopharmaceutical companies, including Syntex Laboratories, Chiron Corporation, Glycomed Incorporated and Fusion Medical Technologies. Mr. Anderson also served as an officer in the United States Army Corps of Engineers, as a strategic planner and operational profit and loss manager at 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 the Company since April 2011. She is currently President and 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 Promedior's Chief Executive Officer, Dr. Bruhn spent 13 years at Shire Human Genetic Therapies (HGT), a division of Shire plc, specializing in the development and commercialization of treatments for orphan diseases. Dr. Bruhn's most recent position at Shire HGT was 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 HGT's products REPLAGAL®, ELAPRASE® and VPRIV®. She also played a key role in Shire HGT's portfolio expansion through acquisitions, including the acquisition of FIRAZYR®. Prior to her time at HGT, Dr. Bruhn held various positions at Cytotherapeutics, Inc., a biotechnology company. Dr. Bruhn holds a Ph.D. in Chemistry from Massachusetts Institute of Technology and was a

Postdoctoral Fellow in the Department of Human Genetics at Harvard Medical School. We nominated Dr. Bruhn to the Board of Directors due to her extensive healthcare experience in the orphan disease arena.

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Richard L. Franklin, M.D., Ph.D. Dr. Franklin has served as a director of the Company since September 2009. Dr. Franklin has served as the Chief Executive Officer and a director of Tarix Pharmaceuticals, a drug development company, since 2004. He has also served as the Chairman of Pathfinder, LLC, a regenerative medicine company, since 2009. 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 that company from December 2000 to September 2011. Pathfinder, LLC and SyntheMed, Inc. 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 of the surviving entity. 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 Chairman of the Board of the Company since July 2013 and as a director since September 2009. Dr. Keltner is Chief Executive Officer of EPISTAT, an international healthcare technology transfer, corporate risk management and healthcare strategy company that he founded in 1972. Dr. Keltner also served as the Chief Executive Officer of AgonOx, a biotech company developing OX40 agonists for use in cancer therapy, from 2011 to 2013. From 2010 until 2011, Dr. Keltner was the President of Novici Biotech, a privately held gene and protein optimization firm, and from 2001 to 2010, he 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 1997 to 2004, Dr. Keltner was Chief Executive Officer of Metastat, Inc., a development-stage biotech company focused on cancer metastasis. Dr. Keltner holds positions on the boards of Infostat, BioQuiddity, Oregon Life Sciences and Goodwell Technologies. He previously served as a director of Light Sciences Corporation, Vital Choice, Thesis Technologies, Oread Companies and MannKind Corporation. Dr. Keltner has also been a scientific advisory board member at Lifetime Corporation, ASB Meditest, Oread Laboratories, Hall-Kimbrell and AAIPharma. Dr. Keltner is an Associate Professor at Case Western Reserve School of Medicine and a Guest Lecturer and Director in the Bioethics Program at Columbia University School of Medicine. He is currently a member of the American Society of Clinical Oncology, the American Medical Association, the International Association of Tumor Marker Oncology, the American Association of Clinical Chemistry and the 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 many research publications. We nominated Dr. Keltner to the Board of Directors due to his practical experience as a 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 the Company since September 2009 and served as the Chairman of our Board of Directors from September 2009 through July 2013. Mr. Sager 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 currently serves as Chairman and a 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 Photonics 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 also held various positions at banks in Switzerland, including Senior Vice President, Head of the Private Banking for Dresdner Bank (Switzerland) Ltd. and Vice President, Private Banking, Head of the German Desk for Deutsche Bank (Switzerland) Ltd. 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, as well as his experience while at BioMarin.

Vijay B. Samant. Mr. Samant has served as a director of the Company since April 2011. He currently serves as President and Chief Executive Officer of Vical Inc., a publicly traded company focused on the development of DNA vaccines for infectious diseases and cancer therapeutics. Prior to his time at 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., including Vice President of Vaccine Operations, Vice President of Business Affairs and Executive Director of Materials Management, all in the Merck Manufacturing Division, and Chief Operating Officer of the Merck Vaccine Division. Mr. Samant served as a member of the Board of Trustees for the International Vaccine Institute (IVI, Seoul, Korea) from 2008 to 2012, 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.

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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 the Company since April 2011. He is currently the Chairman, President and Chief Executive Officer of Horizon Pharma, Inc., a publicly traded biopharmaceutical company focused on developing and commercializing innovative medicines in arthritis, pain and inflammatory diseases. Prior to his time at Horizon Pharma, Mr. Walbert served as President, Chief Executive Officer and a 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 biopharmaceutical companies such as Abbott Laboratories, G.D. Searle/Pharmacia, Neopharm, Merck & Company and Wyeth. At Abbott, Mr. Walbert served as Divisional Vice President and General Manager, Immunology, leading the global development and launch of HUMIRA, which attained over $8.0 billion in sales in 2011. 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 and 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 consists of Mr. Anderson (Chair), Dr. Franklin, Mr. Sager 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. Based solely on a review of the copies of such reports furnished to us, we believe that during the year ended December 31, 2013, our directors, executive officers and 10% stockholders timely filed all Section 16(a) reports applicable to them.

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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 in the Corporate Governance section of our website at www.raptorpharma.com and is acknowledged by our executive officers and directors on an annual basis. We intend to satisfy the disclosure requirements under Item 5.05 of the SEC Form 8-K regarding an amendment to, or waiver from, a provision of our Code of Business Conduct and Ethics by posting such information on our website at the website address and location specified above.

Executive Officers

For each of our executive officers, the following table sets forth their name, age as of March 17, 2014 and position. 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. Below is a current list of the executive officers of our Company.

Name	Age	Position(s) Held with the Company
Christopher M. Starr, Ph.D.	61	Chief Executive Officer and Director
Julie Anne Smith	43	Chief Operations Officer, Executive Vice President, Strategy
Georgia Erbez	47	Chief Financial Officer, Treasurer and Secretary
Thomas (Ted) E. Daley	51	Chief Business Officer

Julie Anne Smith. Ms. Smith has served as our Executive Vice President, Strategy and Chief Operations Officer since September 2012. Ms. Smith is responsible for directing our commercial, manufacturing and program management organizations and providing leadership in corporate and strategic development initiatives. In her nearly 20 years in biotechnology, Ms. Smith has served in executive management of both privately held and publicly held biotechnology firms, mostly in orphan drug development and commercial product opportunities. Prior to joining us, from July 2008 to May 2012, Ms. Smith was Chief Commercial Officer of Enobia Pharma, Inc., a privately held, clinical-stage orphan drug company later acquired by Alexion. From August 2006 to July 2008, she led commercial functions as Vice President, Commercial at Jazz Pharmaceuticals. 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. Ms. Smith holds a B.S. in Biological and Nutritional Science from Cornell University, Ithaca, New York.

Georgia Erbez. Ms. Erbez has served as our Chief Financial Officer, Treasurer and Secretary since September 2012. Ms. Erbez is responsible for directing our global financial strategy and organization and provides leadership in defining, communicating and executing corporate and financial strategic initiatives. Prior to joining us, from March 2008 to September 2012, Ms. Erbez was a founder and Managing Director of Beal Advisors, a boutique investment bank providing 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 healthcare 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 life sciences companies and healthcare services. 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. Ms. Erbez holds a B.A. in International Relations with an emphasis in Economics from the University of California at Davis.

Thomas (Ted) E. Daley. Mr. Daley has served as our Chief Business Officer since January 2013. Mr. Daley first joined us in September 2007, following our acquisition of Convivia, Inc., which Mr. Daley founded. Since that time, he has held various positions, including President of one of our wholly owned, indirect subsidiaries. Previously, Mr. Daley was co-founder, VP Business Development and Chief Operating Officer of Instill Corporation, a leading electronic commerce services provider for the U.S. food service industry. Between 1993 and 2001, Mr. Daley helped

raise over $50.0 million in venture capital and grew Instill to an operation of more than 150 people with a nationwide customer base. After leaving Instill, from 2001 and 2007, Mr. Daley served in executive and consulting roles for 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 in New Zealand. Mr. Daley received a B.S. 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.

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ITEM 11: EXECUTIVE COMPENSATION

Named Executive Officer Compensation

Compensation Discussion and Analysis

Overview

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;

(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 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. 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.

The Compensation Committee's philosophy is to:

- provide a total executive compensation program that is competitive with other companies in the pharmaceutical and biotechnology industries with which we compete for executive talent;
- place a significant portion of executive compensation at risk by linking cash incentive compensation to the achievement of pre-established corporate financial performance objectives and other key objectives within the executive's area of responsibility, and by using equity as a key component of our executive compensation program;
- provide long-term incentive compensation that focuses executives' efforts on building stockholder value by aligning their interests with those of our stockholders; and
- promote stability and retention of our senior 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. We provide non-cash equity compensation to reward performance against current, 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.

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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 the year ended December 31, 2013, our named executive officers included our principal executive officer (Chief Executive Officer), our principal financial officer (Chief Financial Officer), our Chief Operations Officer and our Chief Business Officer. In July 2013 at our Annual Meeting of Stockholders, based upon total shares voted, our stockholders approved our named executive officers' compensation with a 98.9% affirmative vote. 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 at our 2015 Annual Meeting of Stockholders.

Approach for Determining Form and Amount of Compensation

Use of External Compensation Consultant

The Compensation Committee works with an external, independent compensation consultant to assist the Compensation Committee in its duties, including providing advice regarding market trends relating to the form and amount of compensation. Compensia, Inc. ("Compensia") was engaged for 2013 as the compensation consultant for the Compensation Committee. The Compensation Committee has taken great care to ensure that the advice provided by its external compensation consultant is objective and unbiased. Compensia performs no work for us other than its work providing executive and director compensation consulting services to the Compensation Committee and reports directly to the Compensation Committee through its chairperson. In addition, Compensia annually provides a certification to the Compensation Committee regarding its independence and provision of services. The Compensation Committee has assessed the independence of Compensia and concluded that no conflict of interest exist that would prevent Compensia from providing independent and objective advice to the Compensation Committee. Compensia provides the Compensation Committee with third-party data and analyses, advice and expertise on competitive practices and trends and executive compensation plan design. During 2013, Compensia provided the Compensation Committee with:

·market survey data; and

·a comprehensive review of our peer group companies.

Comparison to Market Practices

The Compensation Committee annually compares the levels and elements of compensation that we provide to our executive officers with the levels and elements of compensation provided to their counterparts in the biotechnology, specialty pharma and pharmaceutical industries as a guideline in its review and determination of base salaries, annual performance incentive awards and long-term incentive compensation.

The levels and elements of total compensation that we provide are compared to a "market composite" of data that includes, where available, proxy information for all of the companies in our peer group as well as industry-specific published survey data from Aon Radford Global Life Sciences survey, a well-established blinded industry compensation survey. The survey data and the peer group data are complementary to one another. The survey data provides a broader industry-wide component and matches are made based on job and functional responsibility, while the peer group data provides information regarding companies most directly comparable to us.

The peer companies selected by our Compensation Committee, with Compensia's input, in September 2012 consisted of the following:

Aegerion Pharmaceuticals	Dyax	Progenics Pharmaceutical
Affymax	Dynavax Technologies	Sangamo BioSciences
Amicus Therapeutics	MAP Pharmaceuticals	Synageva BioPharma
ArQule	Neurocrine Biosciences	Transcept Pharmaceuticals
Avanir Pharmaceuticals	Novavax	Vical
Corcept Therapeutics	Omeros	Zalicus
Curis	Oncothyreon	
Depomed	Orexigen Therapeutics	

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As the time, these peer companies were determined to be appropriate from a market capitalization and from a corporate progress/stage of development and corporate strategy perspective. At the time of the peer review in September 2012, the Company's market capitalization was below the median of the peer group market capitalization (200-day average market capitalization as of September 5, 2012).

In November 2013, on the recommendation of Compensia, the Compensation Committee significantly revised this peer group to remove companies that were no longer appropriate from a market capitalization perspective (Affymax, Amicus Therapeutics, Corcept Therapeutics, OncoGenex Pharmaceuticals, Oncothyreon) or not appropriate from a stage of development perspective (ArQule, Dynavax Technologies, Neurocrine Biosciences, Novavax, Omeros, Progenics Pharmaceutical, Sangamo BioSciences and Vical). Additionally, MAP Pharmaceuticals was removed because it was acquired. The peer group approved in November 2013 consisted of the following companies:

Aegerion Pharmaceuticals	Hyperion Therapeutics	Portola Pharmaceuticals
Arena Pharmaceuticals	Intercept Pharma	Sarepta Therapeutics
Avanir Pharmaceuticals	InterMune	Synageva BioPharma
Curis	Ironwood Pharmaceuticals	VIVUS
Depomed	Keryx Biopharmaceuticals	
Dyax	KYTHERA Biopharmaceuticals	
Exelixis	Momenta Pharmaceuticals	
Halozyme Therapeutics	Pacira Pharmaceuticals	

These peer companies were generally appropriate from a strategic/stage of development, revenues and market capitalization perspective. At the time of the peer review in December 2013, our market capitalization was below the median of the peer group market capitalization (60 day average market capitalization as of December 10, 2013).

In January 2014, Compensia reviewed each named executive officer's 2013 compensation as compared to proxy data from this peer group, where available, and a blend of proxy data and survey data for executives without a peer match. Compensia found that total target cash compensation levels for the executives, on average, approximated the market 25th percentile, with Dr. Starr, Ms. Erbez and Ms. Smith at or below the 25th percentile and Mr. Daley at the 60th percentile. Dr. Starr's September 2012 annual option grant also fell below the 25th percentile, while Mr. Daley's annual option grant was at the 60th percentile.

The Role of Our Chief Executive Officer

While the Compensation Committee has overall responsibility for establishing the elements, level and administration of our executive compensation programs, our Chief Executive Officer routinely participates in this process, as does the Compensation Committee's external, independent compensation consultant. Our Chief Executive Officer conducts in-depth performance reviews of each of the other executive officers and provides a summary of this review to the Compensation Committee. Our Chief Executive Officer also makes recommendations to the Compensation Committee regarding adjustments to these executives' base salaries, target bonus opportunities and equity awards, as required and based on their performance and market considerations. Our Chief Executive Officer's recommendations are one of several important factors considered by the Compensation Committee in making its determinations regarding our executive compensation programs. The Chief Executive Officer also provides a self-assessment to the Compensation Committee and full Board of Directors for their consideration.

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 with multiple year vesting terms and up to ten year expiration periods);
- 401(k) plan Company matching contributions;
- health, disability and life insurance; and

- severance and change in control provisions primarily delineated in individual employment contracts or employer offer letters and Company policies.

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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).

There were no changes made to the base salary compensation of our named executive officers during the 16-month period ended December 31, 2013 as the Compensation Committee had recommended and the Board of Directors had approved the following base salaries effective September 2012:

Name	Position	Effective September 1, 2012* Annual Base Salary
Christopher M. Starr, Ph.D.	Chief Executive Officer and Director	$410,000
Julie Anne Smith	Executive Vice President, Strategy, Chief Operations Officer	350,000
Georgia Erbez	Chief Financial Officer, Treasurer and Secretary	330,000
Ted Daley	Chief Business Officer	292,000

* For Ms. Smith and Ms. Erbez, salaries were negotiated in connection with and effective upon their hire date of September 10, 2012.

In February 2014, the Compensation Committee recommended and the Board of Directors approved 15% base salary increases for Dr. Starr and Ms. Smith given their strong performance during 2013 and, in the case of Dr. Starr, to bring his pay closer to the market median. The Board approved 5% base salary increases for the other named executive officers in light of the Company's strong performance during 2013 and to provide for a cost of living adjustment. The adjusted 2014 base salaries for the named executive officers are as follows.

Name	Position	Effective January 1, 2014 Annual Base Salary
Christopher M. Starr, Ph.D.	Chief Executive Officer and Director	$471,500
Julie Anne Smith	Executive Vice President, Strategy, Chief Operations Officer	402,500
Georgia Erbez	Chief Financial Officer, Treasurer and Secretary	346,000
Ted Daley	Chief Business Officer	306,600

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

All of our executive officers are eligible for annual cash 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. Given that we changed our fiscal year in 2012 from August 31 to December 31, the cash incentive program covered the 16-month period from September 1, 2012 through December 31, 2013.

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. Performance against goals is the primary factor in determining annual cash incentive bonuses.

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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. After 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 or superior performance.

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, include subjective aspects of performance, 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 our Board of Directors determines 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 annual target bonus percentages for our named executive officers effective September 2012 were as follows: Dr. Starr 50% of his annual base salary; Ms. Erbez and Ms. Smith, 40% each; Mr. Daley 35%. These target bonus percentages were increased (or were set for new executive officers) after a review of the practices of our peer companies, which indicated that our previous percentages were not competitive with our peer company compensation practices and could contribute unfavorably to the competitive compensation position for key executives of our Company. Given that the performance period covered 16 months instead of 12 months, the annual target bonuses were increased by 133% to account for the longer performance period.

Corporate Goals

Our corporate goals for the period September 2012 through December 31, 2013 were grouped into our major activities with the weighting shown.

Development of RP103 for Cystinosis (weighted 70%; achieved 60%)
- NDA approval on PDUFA date;
- MAA approval Q3 calendar 2013;
- Meet patient enrollment goals for RaptorCares™ in U.S. and EU;
- Launch in the U.S. as per schedule and achieve meaningful net revenue goals; and
- Obtain orphan exclusivity from FDA/EMA.

Finance (weighted 25%; achieved 25%)
- End 2013 with cash balance of $84 million; and
- Meet or exceed EBITDA goal of -$54 million for calendar 2013.

RP103 – Other (weighted 5%; achieved 5%)
- Contract with second source supplier.

The Company achieved each of the above corporate goals at 100% except that the PDUFA date was delayed and the patient enrollment goal in RaptorCares was only partially achieved, resulting is a combined corporate score of 90%.

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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.

For the period beginning September 2012 through December 31, 2013, a significant percentage of each executive officer's individual goals directly supported our corporate goals. The remaining goals were based on achievements within the executive's areas of responsibility.

As discussed above, Dr. Starr's individual goals were 100% of the corporate goals. Ms. Smith's individual goals included the enrollment goals for RaptorCares™ in U.S. and EU listed above plus goals related to our revenues, patient identification, drug supply and program management. Ms. Erbez's individual goals included meeting or exceeding our EBITDA goal for calendar 2013 plus metrics related to financial management measurement systems, financial projections and needs, internal financial reporting, valuation analyses, internal controls, spending controls, audits, investors and financings. Mr. Daley's individual goals included NDA approval, MAA approval and orphan exclusivity from FDA/EMA goals, listed above, plus metrics related to WBC cystine testing, NASH and certain business development milestone. Corporate and individual goals were weighted based upon importance and impact to Raptor.

Fiscal Year 2013 Goal Achievements for our Executive Officers

Dr. Starr's annual target incentive bonus was equal to 50% of his base salary (the base salary target percentage). Following consideration of the corporate achievement score of 90% and given the Company's strong shareholder returns during 2013, the Compensation Committee recommended and the Board approved an annual incentive bonus of $250,000.

Ms. Smith's annual target incentive bonus was equal to 40% of her base salary (the base salary target percentage). Corporate program goals account for 70% of Ms. Smith's annual incentive bonus, while individual goals account for the remaining 30%. Following consideration of the corporate achievement score of 90%, Ms. Smith's individual performance and the Company's strong shareholder returns during 2013, the Compensation Committee recommended and the Board approved an annual incentive bonus of $200,000.

Ms. Erbez's annual target incentive bonus was equal to 40% of her base salary (the base salary target percentage). Corporate program goals account for 70% of Ms. Erbez's annual incentive bonus, while individual goals account for the remaining 30%. Following consideration of the corporate achievement score of 90%, Ms. Erbez's individual performance and given the Company's strong shareholder returns during 2013, the Compensation Committee recommended and the Board approved an annual incentive bonus of $165,000.

Mr. Daley's annual target incentive bonus was equal to 35% of his base salary (the base salary target percentage). Corporate program goals account for 70% of Mr. Daley's annual incentive bonus, while individual goals account for the remaining 30%. Following consideration of the corporate achievement score of 90%, Ms. Daley's individual performance and given the Company's strong shareholder returns during 2013, the Compensation Committee recommended and the Board approved an annual incentive bonus of $120,000.

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 four-year 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 considers: 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 approval by the Board of Directors or the executive's first day of employment.

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Under the 2010 Plan, we were initially authorized to grant up to an aggregate of 3,000,000 shares 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. On July 23, 2013, our stockholders approved an amendment to the 2010 Plan to increase the share reserve by an additional 3,000,000 shares. As of December 31, 2013, options to purchase 8,217,674 shares of our common stock were outstanding and 3,861,729 shares of our common stock remained 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.

Equity Compensation Award

Following the changes in our fiscal year end from August 31 to December 31, there were no annual stock options granted to our named executive officers during the year ended December 31, 2013. Subsequent to the year ended December 31, 2013, in February 2014, our Board of Directors approved, on the recommendation of the Compensation Committee, to award stock options to our named executive officers, as shown in the table 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. The options granted included an adjustment for the 16-month period of service. Dr. Starr did not participate in the discussion or approval of his option grant.

Name and Position	February 2014 Grants
Christopher M. Starr, Ph.D. Chief Executive Officer and Director	195,615
Julie Anne Smith, EVP, Chief Operations Officer	107,065
Georgia Erbez, Chief Financial Officer, Secretary and Treasurer	83,800
Ted Daley, Chief Business Officer	46,529

Perquisites and Other Benefits

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. At its discretion, our Compensation Committee may revise, amend or add to the executive's benefits if it deems it advisable.

Other than as disclosed below, during our fiscal year ended December 31, 2013, our executives did not receive any perquisites and were not entitled to benefits that are not otherwise available to all of our employees. In connection with Ms. Smith's hire, we agreed to reimburse her for reasonable relocation expenses, not to exceed in the aggregate $50,000, and provided that if her employment with the Company terminates, other than in connection with a "constructive termination" or "termination without cause" (each, as defined in her employment agreement), prior to

the second anniversary of her first day of employment with the company, she will promptly reimburse the Company for the full amount of payment received for such relocation expenses. Ms. Smith was also entitled to reimbursement of commuting expenses related to the performance of her duties until the earlier of August 31, 2013 or the date Ms. Smith moves her primary residence to the San Francisco Bay Area.

During the fiscal year ended December 31, 2013, we did not provide pension arrangements, post-retirement health coverage or similar benefits for our executives or employees.

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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

Year Ended December 31, 2013

The following table reports summary compensation information for the following individuals, referred to as our named executive officers during the year ended December 31, 2013: (1) Raptor's principal executive officer (Chief Executive Officer); (2) Raptor's principal financial officer (Chief Financial Officer); and (3) our two other executive officers other than the principal executive officer or principal financial officer who were serving as executive officers.

Name and Principal Position	Salary	Non-Equity Incentive Plan Compensation (1)	All Other Compensation (2)		Total
Christopher M. Starr, Ph.D. Chief Executive Officer and Director	$410,000	$ 250,000	$ 10,200		$670,200
Georgia Erbez Chief Financial Officer, Secretary and Treasurer	330,000	165,000	10,200		505,200
Julie Anne Smith EVP, Strategy, Chief Operations Officer	350,000	200,000	42,173	(3)	592,173
Ted Daley Chief Business Officer	292,000	120,000	10,200		422,200

(1) Represents cash incentive awards earned for 16-month period ended December 31, 2013 and paid in February 2014.

(2) All Other Compensation includes 401(k) matching funded by us.

(3) Raptor is headquartered in Novato, CA. This total amount includes $31,973 which represents expenses reimbursed to Ms. Smith based on commuting to Novato from her residence in Nevada.

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Four-month transition period from September 1, 2012 through December 31, 2012

The following table reports summary compensation information for our named executive officers during the four-month transition period from September 1 to December 31, 2012.

Name and Principal Position	Period	Salary	Option Awards (1)	Non-Equity Incentive Plan Compensation (2)	All Other Compensation (3)	Total
Christopher M. Starr, Ph.D. Chief Executive Officer and Director	September 1 to December 31, 2012	$136,667	$560,378	$ –	$ 4,017	$701,062
Georgia Erbez (4) Chief Financial Officer, Secretary and Treasurer	September 10 to December 31, 2012	103,125	681,366	–	1,844	786,335
Julie Anne Smith (4) EVP, Strategy, Chief Operations Officer	September 10 to December 31, 2012	109,375	681,366	–	13,876 (5)	804,617
Ted Daley Chief Business Officer	September 1 to December 31, 2012	97,333	250,301	–	6,558	354,192

(1) This column represents the grant date fair value of the stock options granted during the period to each of our named executive officers, in accordance with ASC Topic 718. For additional information on the valuation assumptions with respect to the four months ended December 31, 2012, please refer to the notes in our consolidated financial statements included elsewhere in the Form 10-KT. 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) With the change in fiscal year from August 31 to December 31 in 2012, the new bonus period covered the September 1, 2012 - December 31, 2013 period. As such, no bonuses were paid during the transition period.

(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) Ms. Erbez and Ms. Smith commenced employment on September 10, 2012.

(5) Raptor is headquartered in Novato, CA. Amount includes $11,596 in expenses reimbursed to Ms. Smith based on commuting to Novato from her residence in Nevada.

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Fiscal Year Ended August 31, 2012 and August 31, 2011

The following table reports summary compensation information for our named executive officers who served at the Company during 2012 and 2011.

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. Chief Executive Officer and Director	2012	$356,807	$1,941,447	$ 112,000	$ 14,953	$2,425,207
	2011	346,415	1,232,990	111,996	15,976	1,707,377
Ted Daley President, Raptor Therapeutics (Currently Chief Business Officer)	2012	265,458	639,267	65,000	14,848	984,573
	2011	250,432	406,348	61,839	12,013	730,632

(1) This column represents the grant date fair value of the stock options granted during each period to each of our named executive officers, in accordance with ASC Topic 718. For additional information on the valuation assumptions with respect to the fiscal years ended August 31, 2012 and 2011, please refer to the notes in our

consolidated financial statements included elsewhere in the Form 10-KT. 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.

(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.

Employment Agreements

Dr. Starr entered into an employment agreement with us 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 an initial stock option grant 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 December 31, 2013 are described in the Compensation Discussion and Analysis section of this annual report. 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 an initial stock option grant to purchase 190,000 shares of our common stock at the closing price on September 7, 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.

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On September 10, 2012, we 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 an initial stock option grant to purchase 190,000 shares of our common stock at the closing price on September 7, 2012, the business day preceding the date of grant. Further, Raptor will reimburse Ms. Smith for reasonable relocation expenses, not to exceed in the aggregate $50,000, provided that if her employment with the Company terminates, other than in connection with a "constructive termination" or "termination without cause" (each, as defined in her employment agreement), prior to the second anniversary of her first day of employment with the Company, she will promptly reimburse the Company for the full amount of payment received for such relocation expenses. Ms. Smith was also entitled to reimbursement of 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.

On September 7, 2007, we 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 an initial stock option grant 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, the compensation committee recommended, and its 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.

Under Dr. Starr's employment agreement, if Dr. Starr's employment is constructively terminated or terminated by us without cause, then he will be entitled to continue to receive his base salary, other benefits for a period of 12 months from the date of termination and his annual cash bonus, which will be paid on the expiration of the 12 months following the date of termination. If such termination occurs after a Change in Control (as defined in his employment agreement), Dr. Starr will receive a lump sum amount equal to one year of his base salary, his annual cash bonus as described in the preceding sentence, and all of Dr. Starr's vested and unvested options to purchase our stock will be immediately exercisable in full. Dr. Starr will also receive a tax gross-up of any taxes, including any excise tax imposed by Section 4999 of the Code, for any payments and benefits he receives under his employment agreement in the event of a Change in Control. If Dr. Starr's employment is terminated due to disability, he will be entitled to receive: (i) continued payment of base salary and receipt of benefits for three months; (ii) his annual cash bonus pro rata for three months, payable in a lump sum on the expiration of the three months following his termination; and (iii) continued vesting of the stock options granted under his employment agreement for three months following termination.

Under Mr. Daley's employment agreement, if Mr. Daley's employment is constructively terminated or terminated by us without cause, including in the event of a Change in Control (as defined in his employment agreement), then he will be entitled to receive: (i) a lump sum payment equal to six months of his base salary; (ii) a lump sum payment equal to 50% of his actual bonus earned in the prior fiscal year; and (iii) payment of his COBRA premiums for six months. In the event such termination occurs within six months following a change of control, all of Mr. Daley's

vested and unvested options will be exercisable in full and all shares of common stock owned by Mr. Daley pursuant to such options shall immediately be released from all vesting restrictions. Payment of any of the foregoing cash amounts is subject to Mr. Daley's execution of a general release of claims that becomes effective and irrevocable within 60 days following termination and his continued compliance with certain representations, warranties or covenants for six months following the date of termination. In addition, in the event of a termination for disability, Mr. Daley is entitled to receive: (i) continued payment of his base salary for three months; (ii) any target bonuses he would otherwise have earned in the three months following his termination; and (iii) continued vesting of the stock options granted under his employment agreement for three months following termination.

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If Ms. Erbez's or Ms. Smith's employment is constructively terminated or terminated by us without cause other than during the 12 months following a Change in Control (as defined in the applicable employment agreement), they will be entitled to receive (i) continued payment of base salary for 12 months after such termination; (ii) payment or reimbursement of health plan coverage under COBRA for up to twelve months; (iii) continued exercisability of all of their vested options or stock appreciation rights with respect to our common stock until the first anniversary of the termination of their employment; and (iii) the release from any and all resale or repurchase rights restrictions of all shares of our common stock owned by them. If Ms. Erbez or Ms. Smith 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 sentence, all of their 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, they will be entitled to a lump sum payment equal to (i) the average of the annual bonus payments received by them in the two years preceding the year of termination; or (ii) if two annual bonus payment dates have not occurred (regardless of whether they received any annual bonus on such dates) prior to termination, the annual bonus they received with respect to the year preceding the year of termination; or (iii) if an annual bonus payment date has not occurred prior to their termination of employment, 40% of their base salary. If Ms. Erbez or Ms. Smith is terminated for disability, they will continue to receive their base salary and reimbursement or payment of health plan coverage under COBRA for three months following the date of termination. Each of the foregoing payments and benefits is subject to the applicable officer's execution of a general release of claims that becomes effective and irrevocable within 60 days following 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 or Ms. Smith, if such officer's employment is terminated by death, a lump sum amount equal to three months of base salary.

Grants of Plan-Based Awards Table

There were no equity awards granted to our named executive officers during the fiscal year ended December 31, 2013.

	Estimated Future Payouts Under Non-Equity Incentive Awards		
Name	Threshold ($)	Target ($)	Maximum ($)
Christopher M. Starr, Ph.D.	–	$272,650	$340,813
Georgia Erbez	–	$175,560	$219,450
Julie Anne Smith	–	$186,200	$232,750
Ted Daley	–	$135,926	$169,908

(1) Amounts represent the target and maximum cash awards payable to our named executive officers under our cash incentive program for the 16 month period from September 1, 2012 through December 31, 2013.

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Outstanding Equity Awards
Year Ended December 31, 2013
The following table sets forth certain information with respect to outstanding stock option awards of our named executive officers for the year ended December 31, 2013.

		Option Awards					
Name	Grant Date	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.	3/9/10	4,688	–	(2)	–	$ 2.02	3/9/2020
	10/12/10	130,173	32,351	(2)	–	2.97	10/12/2020
	11/22/10	315,054	–		–	3.54	11/22/2020
	9/22/11	258,782	201,282	(1)	–	5.13	9/22/2021
	9/25/12	46,874	103,126	(1)	–	5.49	9/25/2022
Georgia Erbez	9/10/12	59,374	130,626	(1)	–	5.27	9/10/2022
Julie Anne Smith	9/10/12	59,374	130,626	(1)	–	5.27	9/10/2022
Ted Daley	9/10/07	24,969	–		–	2.23	9/10/2017
	8/12/08	23,313	–		–	1.88	8/12/2018
	3/9/10	18,900	–		–	2.02	3/9/2020
	10/12/10	46,155	10,653	(2)	–	2.97	10/12/2020
	11/22/10	113,616	–		–	3.54	11/22/2020
	9/22/11	85,210	66,278	(1)	–	5.13	9/22/2021
	9/25/12	20,937	46,063	(1)	–	5.49	9/25/2022

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

1/48th per month thereafter.

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Options Exercised

Year ended December 31, 2013

The following table sets forth the number and value of options exercised during our year ended December 31, 2013 for each of the named executive officers.

	Option Awards	
Name	Number of Shares Acquired on Exercise (2)	Value Realized on Exercise (1)
Christopher M. Starr, Ph.D.	100,000	$1,051,470
Ted E. Daley	10,000	120,972

(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 Mr. Daley 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 under the section titled "Employment Agreements" above.

Year Ended December 31, 2013

The following table quantifies the amounts that we would owe each of our named executive officers upon each of the termination triggers discussed above under "Employment Agreements," assuming a termination date of December 31, 2013:

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	$102,500	(2)	$102,500	$ 410,000	(3)	$ 410,000	(3)
Short-Term Incentive	62,500	(4)	–	250,000	(5)	250,000	(5)
Value of Unvested Equity Awards Vesting Continuation/Acceleration	542,973	(6)	–	–		2,689,781	(7)
Total	$707,973		$102,500	$ 660,000		$ 3,349,781	

(1) "CIC" means change in control, as defined in the officer's employment agreement.
(2) 3 months base salary.
(3) 12 months base salary.
(4) Annual cash bonus pro rata for three months.
(5) Annual cash bonus, payable upon the expiration of the twelve month period following termination.

(6) 3 months continued vesting of the stock options granted under his employment agreement.

(7) Assumes accelerated vesting of unvested equity awards at December 31, 2013. This amount reflects the intrinsic value for these awards, and does not correspond to the actual value, if any, that will be realized by the officer.

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Georgia Erbez
Chief Financial Officer, Secretary and Treasurer

Executive Benefits and Payments Upon Termination	Disability		Death		Termination Without Cause or Constructive Termination		CIC Termination Without Cause or Constructive Termination (1)	
Base Salary	$82,500	(2)	$82,500	(2)	$ 330,000	(3)	$ 330,000	(3)
Short-Term Incentive	–		–		–		165,000	(4)
COBRA Continuation	7,416	(5)	–		29,664	(6)	29,664	(6)
Value of Unvested Equity Awards Vesting Continuation/Acceleration	29,664		–		–		1,012,352	(7)
Total	$119,580		$82,500		$ 359,664		$ 1,537,016	

(1) "CIC" means change in control, as defined in the officer's employment agreement.
(2) 3 months base salary.
(3) 12 months base salary.
(4) 40% of base salary.
(5) 3 months COBRA continuation.
(6) 12 months COBRA continuation.
(7) Assumes accelerated vesting of unvested equity awards at December 31, 2013. This amount reflects the intrinsic value for these awards, and does not correspond to the actual value, if any, that will be realized by the officer.

Julie Anne Smith
EVP, Strategy, Chief Operations Officer

Executive Benefits and Payments Upon Termination	Disability		Death		Termination Without Cause or Constructive Termination		CIC Termination Without Cause or Constructive Termination (1)	
Base Salary	$87,500	(2)	$87,500	(2)	$ 350,000	(2)	$ 350,000	(2)
Short-Term Incentive	–		–		–		200,000	(4)
COBRA Continuation	9,032	(5)	–		36,127	(5)	36,127	(6)
Value of Unvested Equity Awards Vesting Continuation/Acceleration	36,127		–		–		1,012,352	(7)
Total	$132,659		$87,500		$ 386,127		$ 1,598,479	

(1) "CIC" means change in control, as defined in the officer's employment agreement.
(2) 3 months base salary.
(3) 2 months base salary.
(4) 40% of base salary.
(5) 3 months COBRA continuation.
(6) 12 months COBRA continuation.
(7) Assumes accelerated vesting of unvested equity awards at December 31, 2013. This amount reflects the intrinsic value for these awards, and does not correspond to the actual value, if any, that will be realized by the officer.

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Ted Daley
Chief Business Officer

Executive Benefits and Payments Upon Termination	Disability		Death		Termination Without Cause or Constructive Termination		CIC Termination Without Cause or Constructive Termination (1)	
Base Salary	$73,000	(2)	$73,000		$ 146,000	(3)	$ 146,000	(3)
Short-Term Incentive	25,550	(4)	–		60,000	(5)	60,000	(5)
COBRA Continuation	–		–		21,088	(6)	21,088	(6)
Value of Unvested Equity Awards Continued Vesting/Acceleration	141,908	(7)	–		–		976,850	(8)
Total	$240,458		$73,000		$ 227,088		$ 1,203,938	

(1) "CIC" means change in control, as defined in the officer's employment agreement.
(2) 3 months base salary.
(3) 6 months base salary.
(4) The target bonus that otherwise would have been earned within the three months following termination.
(5) 50% of the actual bonus earned for the prior fiscal year.
(6) 6 months COBRA continuation.
(7) 3 months continued vesting of the stock options granted under his employment agreement.
(8) Assumes accelerated vesting of unvested equity awards at December 31, 2013. This amount reflects the intrinsic value for these awards, and does not correspond to the actual value, if any, that will be realized by the officer.

Director Compensation
Year ended December 31, 2013
Effective July 16, 2013, non-employee members of our Board of Directors received the following cash compensation:

Director Position	Annual Cash Compensation
Non-Employee Directors, (Non-Chair members)	$ 40,000
Chairman of the Board of Directors	75,000
Audit Committee Chair	20,000
Audit Committee (Non-Chair members)	10,000
Compensation Committee Chair	13,000
Compensation Committee (Non-Chair members)	7,500
Corporate Governance and Nominating Committee Chair	12,000
Corporate Governance and Nominating Committee (Non-Chair members)	7,000

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For fiscal 2014, our Compensation Committee chair fee was increased to $15,000. The following table sets forth the total compensation paid by us to each of our non-employee directors during our year ended December 31, 2013.

Name	Fees Earned or Paid in Cash
Raymond W. Anderson (2)	$67,500
Suzanne L. Bruhn, Ph.D. (3)	60,000
Richard L. Franklin, M.D. Ph.D. (4)	55,250
Llew Keltner, M.D., Ph.D. (5)	67,958
Erich Sager (6)	62,000
Vijay B. Samant (7)	55,750
Timothy P. Walbert (8)	57,500

(2) Mr. Anderson had 394,619 options outstanding as of December 31, 2013, of which 349,617 were exercisable.
(3) Dr. Bruhn had 218,960 options outstanding as of December 31, 2013, of which 154,583 were exercisable.
(4) Dr. Franklin had 318,539 options outstanding as of December 31, 2013, of which 273,537 were exercisable.
(5) Dr. Keltner had 324,164 options outstanding as of December 31, 2013, of which 279,162 were exercisable.
(6) Mr. Sager had 495,531 options outstanding as of December 31, 2013, of which 450,529 were exercisable.
(7) Mr. Samant had 220,000 options outstanding as of December 31, 2013, of which 155,623 were exercisable.
(8) Mr. Walbert had 218,959 options outstanding as of December 31, 2013, of which 154,582 were exercisable.

There were no stock option or stock grants issued to any non-employee member of our Board of Directors during the 2013 calendar year. For their services as members of our Board of Directors, in February 2014, each non-employee member of our Board of Directors received stock options to purchase 50,000 shares of our common stock, which vest 25% per quarter and expire 10 years from the date of grant. The exercise price of such options was $14.74 per share.

Compensation Committee Interlocks and Insider Participation

No member of our Compensation Committee has served as one of our officers or employees at any time. None of our executive officers serves, or has served during the last fiscal year, as a member of the compensation committee or a member of the board of directors of any other company that has an executive officer serving as a member of our

Compensation Committee or our Board of Directors.

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.

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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 equity compensation (with multiple year vesting schedules and long expiration terms) discourages short-term risk taking;
- for key decision-making officers, base salary makes up a significant majority of cash compensation even with full achievement of annual incentive (cash) awards;
- goals are appropriately set to avoid targets that, if not achieved, result in a large percentage loss of compensation;
- as a pharmaceutical product development company with industry standard long development timelines, prior to U.S. drug approval in April 2013, we did not face the same level of short-term risks associated with compensation for employees at other companies in rapidly changing markets; the short-term risks in the capital markets faced by us were largely in the approval of and related market exclusivity actions for our product by the FDA and were not under the direct control of our management; and
- with a U.S. drug approved in April 2013, as a company with an ultra-orphan product in the critical phase of market launch, the capital market risk has increased due to the short term focus on the emerging product performance in the pharmaceutical marketplace. This potentially affects both short-term cash and long-term equity compensation.

Furthermore, compensation decisions include subjective considerations, which moderate the influence of formulaic or objective factors which may encourage excessive risk taking.

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 and proxy statement for the 2014 annual meeting of stockholders.

2013 Compensation Committee,
Suzanne Bruhn, Ph.D., Chair
Raymond W. Anderson
Vijay Samant
Timothy P. 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.

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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 December 31, 2013:

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	8,217,674	$ 5.77	3,861,729
Equity compensation plans not approved by stockholders	–	–	–
Total	8,217,674	$ 5.77	3,861,729

Security Ownership of Certain Beneficial Owners, Directors and Management

The following table sets forth, as of February 28, 2014, any person or group known by us to be the beneficial owner of more than five percent (5%) of any class of our voting securities, each of our named executive officers, each of our directors and all of our current 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., 5 Hamilton Landing, Suite 160, Novato, CA 94949.

Name of Beneficial Owner and Address	Number of Shares of Common Stock Beneficially Owned(1)	Number of Shares Beneficially Owned Underlying Convertible Securities (1)(2)	Percentage of Outstanding Shares of Common Stock (3)
5% Stockholders: (4)			
BlackRock, Inc. (5)	4,337,767		6.9 %
Credit Suisse AG (6)	5,520,600		8.8
FMR LLC (7)	3,332,230		5.3
Named Executive Officers and Directors:			
Christopher M. Starr, Ph.D. (8)	1,467,030	767,660	2.4
Julie Anne Smith	75,207	75,207	*
Georgia Erbez (9)	78,707	75,207	*
Thomas (Ted) E. Daley	401,174	354,645	*
Raymond W. Anderson	349,617	349,617	*
Suzanne L. Bruhn, Ph.D.	162,813	162,813	*
Richard L. Franklin, M.D., Ph.D.	272,822	272,822	*

Llew Keltner, M.D., Ph.D.	279,162	279,162	*
Erich Sager	544,826	460,529	*
Vijay B. Samant	164,373	164,373	*
Timothy P. Walbert	162,812	162,812	*
All executive officers and directors as a group (11 persons)	3,958,543		6.3%

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* 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 stock options, warrants and convertible preferred stock that may be acquired within sixty (60) days of February 28, 2014 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) The shares reported in this column represent shares of common stock underlying stock options, warrants and convertible preferred stock exercisable or convertible for shares of our common stock within sixty (60) days of February 28, 2014.

(3) Based on 62,479,286 shares outstanding as of February 28, 2014.

(4) Beneficial ownership shares as reported on Form SC 13G as of December 31, 2013.

(5) The principal business address for BlackRock, Inc. is 40 East 52nd

	Street, New York, NY 10022.
(6)	The principal business address for Credit Suisse AG is Uetlibergstrasse 231, CH 8070, Zurich, Switzerland.
(7)	The principal business address for FMR LLC is 245 Summer Street, Boston, MA 02210.
(8)	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 767,660 shares of our common stock held by Dr. Starr directly as of February 28, 2014.
(9)	Includes 3,500 shares our common stock held in trust for Ms. Erbez's children of which Ms. Erbez is a co-trustee and beneficiary and shares voting and investment power, and options to purchase 75,207 shares of our common stock held by Ms. Erbez directly as of February 28, 2014.

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

Transactions with Related Persons

Our Audit Committee approves and oversees any transaction between the Company and any related person (as defined in Item 404 of Regulation S-K) on an ongoing basis and maintains policies and procedures for the Audit Committee's approval of related party transactions.

Raptor's policy is on related party transactions is covered in the company's code of conduct policy. Pursuant to the direction in our Code of Conduct and Business Ethics, authorization from the Audit Committee is required for a director or an officer or their affiliates to enter into a related party transaction or similar transaction which could result in a conflict of interest. Conflicts of interest are prohibited unless specifically authorized in accordance with the Code of Conduct and Business Ethics. Our Audit Committee is responsible for reviewing, reporting and the approval or ratification of each related party transaction. The Audit Committee is responsible for determining if a related party transaction is in the best interest of the Company. The scope of related party transactions or other relationships

includes those that would be required to be disclosed in the Proxy Statement as a related party transaction pursuant to applicable NASDAQ or SEC rules.

In general, these transactions and relationships are defined as those involving a direct or indirect material interest of any of our executive officers, directors, nominees for director and 5% stockholders, as well as specified members of the family or households of these individuals or stockholders, including entities in which the related party has a material interest, indebtedness, guarantees of indebtedness or employment by us of a related person, where we or any of our affiliates have participated in the transaction (either as a direct party or by arranging the transaction) and the transactions or series of transactions involves more than $120,000.

Our general practice is that we may not enter into a related party transaction unless our Corporate Counsel has specifically confirmed in writing that no further reviews are necessary, or that all requisite corporate approvals have been obtained. Our practice excludes transactions, among others, involving compensation of our executive officers or directors that the Board or our Compensation Committee has expressly approved. Indemnification agreements between the Company and our executive officers and directors are outside the scope of related party transactions processes. In the ordinary course of business, our officers occasionally utilize their personal credit cards or cash to pay for expenses on behalf of the Company and the Company reimburses the officers within 30 days.

Since January 1, 2013, 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, nominees for director, 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, and (ii) the transactions described below.

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We have entered into indemnity agreements with all 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 or our subsidiaries, and otherwise to the fullest extent permitted under the General Corporation Law of the State of Delaware and our Bylaws.

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 as of December 31, 2013, 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 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

On January 22, 2014, our Audit Committee appointed the firm of Grant Thornton LLP, or Grant Thornton, an independent registered public accounting firm, to replace Burr Pilger Mayer, Inc., or BPM, as our independent registered public accounting firm. Accordingly, our Audit Committee has engaged Grant Thornton as our independent registered public accounting firm for the year ending December 31, 2013.

For the audit of our year ended December 31, 2013 and re-audit of our transition period from September 1, 2012 through December 31, 2012, Grant Thornton's estimated audit fees, excluding overhead expenses, would be approximately $580,000. We have not engaged Grant Thornton to perform audit-related, tax compliance or tax consulting and advisory services.

The following is a summary of the fees and services provided by BPM during the year ended December 31, 2013, our transition period from September 1, 2012 through December 31, 2012 and fiscal years ended August 31, 2012 and 2011.

			Year ended August 31,	
Description of Services Provided by BPM	Year ended December 31, 2013*	Four months ended December 31, 2012	2012	2011
Audit Fees: These services relate to the audit and review of financial statements, assurance and other related services.	$261,237	$194,480	$386,282	$304,382
Tax Compliance Fees: These services relate to the preparation of federal, state and foreign tax returns and other filings.	86,060	0	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.	5,252	68,726	259,113	0

* Audit Fees during the year ended December 31, 2013 include amounts which have been billed but not paid as of the date of this report of approximately $62,000.

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 Grant Thornton and believes that the provision of the services for activities is compatible with maintaining Grant Thornton's independence.

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PART IV

ITEM 15: EXHIBITS, FINANCIAL STATEMENT SCHEDULES

The information required to be filed in this item appears on pages 91 to 120 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 page 121 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.

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Exhibits

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

Exhibit Number	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Exhibit Number	
2.1	Agreement and Plan of Merger and Reorganization, dated June 7, 2006, among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc.	S-4	7/25/2006	Annex A	
2.2	Amendment No. 1 to Agreement and Plan of Merger and Reorganization, dated August 25, 2006, among Axonyx Inc., Autobahn Acquisition, Inc. and TorreyPines Therapeutics, Inc.	S-4/A	8/25/2006	Annex A	
2.3	Agreement and Plan of Merger and Reorganization, dated July 27, 2009, among ECP Acquisition, Inc., Raptor Pharmaceuticals Corp. and TorreyPines Therapeutics, Inc.	8-K	7/28/2009	2.1	
3.1	Certificate of Incorporation of the Registrant.	8-K	10/10/2006	3.1	
3.2	Amended and Restated Bylaws of the Registrant.	8-K	2/26/2014	3.1	
3.3	Certificate of Amendment to the Articles of Incorporation of Axonyx Inc., filed with the Secretary of State of the State of Nevada, effecting an 8-for-1 reverse split of the Registrant's common stock and changing the name of the Registrant from Axonyx Inc. to TorreyPines Therapeutics, Inc.	8-K	10/10/2006	3.3	
3.4	Articles of Conversion of TorreyPines Therapeutics, Inc., filed with the Secretary of State of the State of Nevada, changing the state of incorporation of the Registrant.	8-K	10/10/2006	3.4	
3.5	Certificate of Conversion of TorreyPines Therapeutics, Inc., filed with the Secretary of State of the State of Delaware.	8-K	10/10/2006	3.5	
3.6	Certificate of Amendment to Certificate of Incorporation of TorreyPines Therapeutics, Inc.	8-K	10/5/2009	3.1	
3.7	Certificate of Merger of ECP Acquisition, Inc. with and into Raptor Pharmaceuticals Corp.	8-K	10/5/2009	3.2	
4.1	Specimen common stock certificate of the Registrant.	8-K	10/5/2009	4.7	
4.2(a)	Rights Agreement, dated as of May 13, 2005, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	8-K	5/16/2005	99.2	
4.2(b)	Amendment to Rights Agreement, dated as of June 7, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	8-K	6/12/2006	4.1	
4.2(c)	Amendment to Rights Agreement, dated as of October 3, 2006, between Registrant and The Nevada Agency and Trust Company, as Rights Agent	10-K	3/29/2007	4.19	
4.2(d)	Rights Agreement Amendment, dated as of July 27, 2009, to the Rights Agreement dated May 13, 2005 between Registrant and American Stock Transfer and Trust Company (replacing The Nevada Agency and Trust Company)	8-K	7/28/2009	2.3	

4.2(e)	Amendment to Rights Agreement, dated August 6, 2010, by and between Registrant and American Stock Transfer & Trust Company, LLC	8-K	8/10/10	4.2
4.3	Form of Warrant, dated September 27, 2005, issued to Oxford Financial and Silicon Valley Bank.	10-K	3/29/2007	4.16
4.5*	Warrant, dated December 14, 2007, issued to Flower Ventures, LLC.	10QSB**	4/15/2008	4.1
4.6*	Warrant Agreement Amendment, dated December 17, 2009, between Flower Ventures, LLC and the Registrant.	10-Q	4/9/2010	4.15

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10.1#	TorreyPines Therapeutics, Inc. 2006 Equity Incentive Plan.	8-K	10/4/2006	10.1
10.2#	Form of Stock Option Agreement under TorreyPines Therapeutics, Inc. 2006 Equity Incentive Plan.	8-K	10/4/2006	10.2
10.3#	2006 Equity Incentive Plan of Raptor Pharmaceuticals Corp., as amended.	S-8**	2/28/2007	4.3
10.4#	Amendment to 2006 Equity Incentive Plan of Raptor Pharmaceuticals Corp.	10-K/A**	12/23/2008	10.5
10.5#	Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan.	DEF 14A	2/5/2010	Appendix A
10.6#	Amendments to the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan.	S-8	4/26/2011	4.15
10.7#	Form of Award Agreement under the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan.	8-K	9/28/2011	10.1
10.8#	Amendment to the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan	8-K	7/25/2013	10.1
10.9	Securities Purchase Agreement, dated December 17, 2009, between the investors signatories thereto and the Registrant.	8-K	12/18/2009	10.1
10.10	Securities Purchase Agreement, dated August 9, 2010, among the investors signatories thereto and the Registrant.	8-K	8/10/2010	10.1
10.11	Securities Purchase Agreement, dated August 9, 2010, among the investors signatories thereto and the Registrant.	8-K	8/10/2010	10.2
10.12	Registration Rights Agreement, dated April 16, 2010, between Lincoln Park Capital Fund, LLC and the Registrant.	8-K	4/22/2010	10.2
10.13	Registration Rights Agreement, dated August 12, 2010, among the signatories thereto and the Registrant.	8-K	8/13/2010	10.3
10.14#	Employment Agreement, dated May 1, 2006, between Dr. Christopher Starr and Raptor Pharmaceuticals Corp.	8-K**	5/26/2006	10.5
10.15#	First Amendment to Employment Agreement, dated January 1, 2009, between Dr. Christopher Starr and Raptor Pharmaceuticals Corp.	8-K**	1/5/2009	10.1
10.16#	Employment Agreement, dated May 15, 2006, between Dr. Todd Zankel and Raptor Pharmaceuticals Corp.	8-K**	5/26/2006	10.6
10.17#	First Amendment to Employment Agreement, dated January 1, 2009, between Dr. Todd Zankel and Raptor Pharmaceuticals Corp.	8-K**	1/5/2009	10.3
10.18#	Employment Agreement, dated September 7, 2007, between Thomas E. Daley and Raptor Therapeutics Inc.	10QSB**	1/14/2008	10.1
10.19#	First Amendment to Employment Agreement, dated January 1, 2009, between Thomas E. Daley and Raptor Pharmaceuticals Corp.	8-K**	1/5/2009	10.4
10.20#	Offer Letter, dated April 8, 2009, between and Dr. Patrice Rioux and Raptor Therapeutics Inc.	8-K**	4/14/2009	10.1
10.21+#	Offer Letter, dated January 1, 2011, between Patrick Reichenberger and Raptor Therapeutics Inc.	10-K	11/14/2011	10.17
10.22++#	Employment Agreement, dated April 15, 2012, between Henk Doude van Troostwijk and Raptor Pharmaceuticals Europe B.V.	10-Q	7/10/2012	10.1
10.23++#	Employment Agreement, dated September 10, 2012, between Georgia Erbez and the Registrant.	8-K	9/12/2012	10.1
10.24++#	Employment Agreement, dated September 10, 2012, between Julie A. Smith and Raptor Therapeutics.	8-K	9/12/2012	10.2
10.25++#		8-K	9/12/2012	10.3

	Employment Agreement, dated September 10, 2012, between Kim R. Tsuchimoto and the Registrant.			
10.26#	Employment Agreement, dated September 25, 2012, between Kathy Powell and the Registrant.	8-K	10/1/2012	10.1
10.27+	Research and License Agreement, dated May 10, 2004, between TPTX, Inc. and Life Science Research Israel Ltd.	8-K	10/10/2006	10.2
10.28	Asset Purchase Agreement, dated October 17, 2007, between Convivia, Inc., Raptor Therapeutics, Inc. and Raptor Pharmaceuticals Corp.	10QSB**	1/14/2008	10.3
10.29	Merger Agreement, dated December 14, 2007, between Encode Pharmaceuticals, Inc., Raptor Therapeutics, Inc. and Raptor Pharmaceuticals Corp.	10QSB/A**	4/15/2008	10.1
10.30+	Pharmaceutical Development Services Agreement, dated January 7, 2008, between Raptor Therapeutics Inc. and Patheon Pharmaceuticals Inc.	10QSB/A**	4/15/2008	10.2
10.31	Form Indemnity Agreement, dated on December 9, 2009.	8-K	12/15/2009	10.1
10.32++	Manufacturing Services Agreement, dated November 15, 2010, between Patheon Pharmaceuticals Inc. and Raptor Therapeutics, Inc.	POS AM	11/23/2010	10.53

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10.33++	API Supply Agreement, dated November 15, 2010, between Cambrex Profarmaco Milano and Raptor Therapeutics Inc.	POS AM	11/23/2010	10.54	
10.34++	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.	10-Q	4/9/2012	10.1	
10.36	Sales Agreement, dated April 30, 2012, between Cowen and Company, LLC and the Registrant.	8-K	5/1/2012	1.1	
10.37++	Second Amendment to License Agreement, effective October 30, 2012, between The Regents of the University of California and Raptor Therapeutics, Inc.	10-KT	3/14/2013	10.37	
10.38++	Loan Agreement, dated December 20, 2012, among HealthCare Royalty Partners II, L.P., the Guarantors party thereto and the Registrant.	10-KT/A	6/19/2013	10.1	
10.39++	Wholesale Product Purchase Agreement, dated April 3, 2013, between Accredo Health Group, Inc. and Raptor Pharmaceuticals Inc.	10-Q	8/9/2013	10.1	
10.40++	Pharmacy Services Agreement, dated April 3, 2013, between Accredo Health Group, Inc. and Raptor Pharmaceuticals Inc.	10-Q	8/9/2013	10.2	
10.41	Office Lease, dated April 18, 2013, between Hamilton Marin, LLC and Raptor Pharmaceuticals Corp.	10-Q	8/9/2013	10.3	
10.42	First Amendment to Lease, dated June 10, 2013, between Hamilton Marin, LLC and Raptor Pharmaceuticals Corp.	10-Q	8/9/2013	10.4	
10.43++	Amendment to Manufacturing Services Agreement, dated April 5, 2012, between Patheon Pharmaceuticals Inc. and Raptor Therapeutics, Inc.	10-Q	8/9/2013	10.5	
10.44++	Second Amendment to Manufacturing Services Agreement, dated June 21, 2013, between Patheon Pharmaceuticals Inc. and Raptor Therapeutics, Inc.	10-Q	8/9/2013	10.6	
21.1	Subsidiaries of the Registrant.				X
23.1	Consent of Grant Thornton LLP, Independent Registered Public Accounting Firm to the Registrant				X
23.2	Consent of Burr Pilger Mayer, Inc., Former Independent Registered Public Accounting Firm to the Registrant				X
24.1	Power of Attorney (included in the signature page hereto).				X
31.1	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director.				X
31.2	Certification of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer.				X
32.1	Certification of Christopher M. Starr, Ph.D., Chief Executive Officer and Director, and of Georgia Erbez, Chief Financial Officer, Secretary and Treasurer.				X

*	The Raptor Pharmaceuticals Corp. warrants set forth in Exhibits 4.6 - 4.9 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.
**	Incorporated by reference from the indicated filing of Raptor Pharmaceuticals Corp. rather than that of the Registrant.
#	Indicates a management contract or compensatory plan or arrangement.
+	Certain information omitted pursuant to a request for confidential treatment filed with the SEC.
++	Certain information omitted pursuant to a request for confidential treatment filed separately with and granted by the SEC.

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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: March 17, 2014

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 this 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)	March 17, 2014
/s/ Georgia Erbez Georgia Erbez	Chief Financial Officer, Secretary and Treasurer (Principal Financial Officer and Principal Accounting Officer)	March 17, 2014
/s/ Raymond W. Anderson Raymond W. Anderson	Director	March 17, 2014
/s/ Suzanne L. Bruhn Suzanne L. Bruhn, Ph.D.	Director	March 17, 2014
/s/ Richard L. Franklin Richard L Franklin, M.D., Ph.D.	Director	March 17, 2014
/s/ Llew Keltner Llew Keltner, M.D., Ph.D.	Director	March 17, 2014
/s/ Erich Sager		March 17, 2014

Erich Sager	Director	
/s/ Vijay B. Samant Vijay B. Samant	Director	March 17, 2014
/s/ Timothy P. Walbert Timothy P. Walbert	Director	March 17, 2014

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Explanatory Note

As previously disclosed by the Company in its current report on Form 8-K filed with the U.S. Securities and Exchange Commission on January 22, 2014, the Company has engaged Grant Thornton LLP ("Grant Thornton") to replace Burr Pilger Mayer, Inc. ("BPM") to serve as the Company's independent registered public accounting firm for the fiscal year ended December 31, 2013 and to re-audit the four-month transition period ended December 31, 2012 (the "Transition Period"). The audit report of Grant Thornton with respect to the Company's financial statements for the Transition Period included in this Annual Report on Form 10-K is in addition to, and not in replacement of, the audit report previously issued by BPM for the same period. (The audit report previously issued by BPM for the Transition Period is not included in this Annual Report on Form 10-K.)

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:

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

We have audited the accompanying consolidated balance sheets of Raptor Pharmaceutical Corp. (a Delaware corporation) and subsidiaries (the "Company") as of December 31, 2013 and 2012, and the related consolidated statements of operations and comprehensive loss, stockholders' equity (deficit), and cash flows for the year ended December 31, 2013 and for the four month period ended December 31, 2012. Our audits of the basic consolidated financial statements included the financial statement schedule listed in the index appearing under Item 15(a)(2). These financial statements and financial statement schedule are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements and financial statement schedule 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 subsidiaries as of December 31, 2013 and 2012, and the results of their operations and their cash flows for the year ended December 31, 2013 and for the four month period ended December 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 basic consolidated financial statements taken as a whole, presents fairly, in all material respects, the information set forth therein.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the Company's internal control over financial reporting as of December 31, 2013, based on criteria established in the 1992 Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), and our report dated March 17, 2014 expressed an unqualified opinion thereon.

/s/ GRANT THORNTON LLP
San Francisco, California
March 17, 2014

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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. (a Delaware corporation) and subsidiaries (the "Company") as of December 31, 2013, based on criteria established in the 1992 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 Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on 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 in 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, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2013, based on criteria established in the 1992 Internal Control—Integrated Framework issued by COSO.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated financial statements of the Company as of and for the year ended December 31, 2013, and our report dated March 17, 2014 expressed an unqualified opinion on those financial statements.

/s/ GRANT THORNTON LLP
San Francisco, California
March 17, 2014

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

We have audited the accompanying consolidated statements of comprehensive loss, shareholders' equity (deficit), and cash flows of Raptor Pharmaceutical Corp. and its subsidiaries (the "Company") (a development stage enterprise) for each of the two years in the period ended August 31, 2012. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits.

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

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the results of operations and cash flows of Raptor Pharmaceutical Corp. and its subsidiaries for each of the two years in the period ended August 31, 2012, in conformity with accounting principles generally accepted in the United States of America.

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

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

	December 31,	
	2013	2012
Assets		
Current assets:		
Cash and cash equivalents	$83,052	$36,313
Restricted cash	500	163
Short-term investments	0	22,096
Accounts receivable	6,181	0
Inventories	3,000	0
Prepaid expenses and other	3,566	1,610
Total current assets	96,299	60,182
Intangible assets, net	3,213	2,156
Goodwill	3,275	3,275
Fixed assets, net	1,810	416
Other assets	4,129	2,094
Total assets	$108,726	$68,123
Liabilities and Stockholders' Equity (Deficit)		
Liabilities		
Current liabilities:		
Accounts payable	$5,264	$4,599
Accrued liabilities	12,767	2,150
Deferred revenue	4,698	0
Common stock warrant liability	7,066	16,405
Deferred rent	302	6
Capital lease liability - current	18	8
Total current liabilities	30,115	23,168
Note payable	50,000	25,000
Capital lease liability - long-term	41	11
Total liabilities	80,156	48,179
Commitments and contingencies		
Stockholders' equity:		
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, 61,615 and 52,425 shares issued and outstanding at December 31, 2013 and December 31, 2012, respectively	62	52

Additional paid-in capital	234,286	155,945
Accumulated other comprehensive loss	(423)	(115)
Accumulated deficit	(205,355)	(135,938)
Total stockholders' equity	28,570	19,944
Total liabilities and stockholders' equity	$108,726	$68,123

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

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Raptor Pharmaceutical Corp.
Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except per share data)

	For the year ended December 31, 2013	For the four months ended December 31, 2012	For the year ended August 31, 2012	2011
Revenues	$16,872	$0	$0	$0
Operating expenses:				
Cost of sales	1,653	0	0	0
Research and development	29,177	8,963	21,443	14,788
Selling, general and administrative	37,948	8,971	14,723	6,178
Total operating expenses	68,778	17,934	36,166	20,966
Loss from operations	(51,906)	(17,934)	(36,166)	(20,966)
Interest income	188	160	340	45
Interest expense	(6,832)	(83)	(3)	(2)
Foreign currency transaction gains	8	113	145	29
Gain (loss) on short-term investments	(128)	(64)	213	0
Adjustment to the fair value of common stock warrants	(10,747)	(1,484)	(3,173)	(16,301)
Net loss	(69,417)	(19,292)	(38,644)	(37,195)
Other comprehensive income (loss)				
Foreign currency translation adjustment	(308)	(65)	(52)	10
Comprehensive loss	(69,725)	(19,357)	(38,696)	(37,185)
Net loss per share:				
Basic and diluted	$(1.20)	$(0.37)	$(0.80)	$(1.15)
Weighted-average shares outstanding used to compute:				
Basic and diluted	57,860	51,737	48,085	32,327

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

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Raptor Pharmaceutical Corp.
Consolidated Statements of Stockholders' Equity (Deficit)
(In thousands, except for per share data)

	Common stock		Additional paid-in	Accumulated other comprehensive income	Accumulated	
	Shares	Amount	capital	(loss)	deficit	Total
Balance at August 31, 2010	30,077	$ 30	$47,618	$ (8)	$ (40,807)	$6,833
Exercise of common stock warrants	3,340	4	8,909	0	0	8,913
Exercise of common stock options	39	0	96	0	0	96
Consultant stock-based compensation expense	0	0	197	0	0	197
Employee stock-based compensation expense	0	0	1,920	0	0	1,920
Reclassification of the fair value of warrant liabilities upon exercise	0	0	8,506	0	0	8,506
Issuance of common stock pursuant to an equity line facility at a $3.35 average per share purchase price, net of fundraising costs and commitment shares of $174	2,113	2	6,571	0	0	6,573
Foreign currency translation gain	0	0	0	10	0	10
Net loss	0	0	0	0	(37,195)	(37,195)
Balance at August 31, 2011	35,569	36	73,817	2	(78,002)	(4,147)
Exercise of common stock warrants	1,831	2	5,011	0	0	5,013
Exercise of common stock options	160	0	366	0	0	366
Consultant stock-based compensation expense	0	0	72	0	0	72
Employee stock-based compensation expense	0	0	4,487	0	0	4,487
Reclassification of the fair value of warrant liabilities upon exercise	0	0	9,482	0	0	9,482
Issuance of common stock in a follow-on public offering at $4.00 per share, net of fundraising costs of $3,168	11,500	12	42,822	0	0	42,834
Issuance of common stock pursuant to an at-the-market financing facility at an average per share purchase price of $5.10, net of fundraising costs of $360	1,508	1	7,323	0	0	7,324
Foreign currency translation loss	0	0	0	(52)	0	(52)
Net loss	0	0	0	0	(38,644)	(38,644)
Balance at August 31, 2012	50,568	$ 51	$143,380	$ (50)	$ (116,646)	$26,735

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

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Raptor Pharmaceutical Corp.
Consolidated Statements of Stockholders' Equity (Deficit) (Continued)
(In thousands, except for per share data)

	Common stock		Additional paid- in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total
	Shares	Amount				
Balance at August 31, 2012	50,568	$ 51	$ 143,380	$ (50)	$ (116,646)	$ 26,735
Exercise of common stock warrants	625	0	1,843	0	0	1,843
Exercise of common stock options	79	0	192	0	0	192
Consultant stock-based compensation expense	0	0	9	0	0	9
Employee stock-based compensation expense	0	0	2,230	0	0	2,230
Reclassification of the fair value of warrant liabilities upon exercise	0	0	2,345	0	0	2,345
Issuance of common stock pursuant to an at-the-market financing facility at an average per share purchase price of $5.34, net of fundraising costs of $185	1,153	1	5,946	0	0	5,947
Foreign currency translation loss	0	0	0	(65)	0	(65)
Net loss	0	0	0	0	(19,292)	(19,292)
Balance at December 31, 2012	52,425	52	155,945	(115)	(135,938)	19,944
Exercise of common stock warrants	3,600	4	10,322	0	0	10,326
Exercise of common stock options	651	1	2,474	0	0	2,475
Consultant stock-based compensation expense	0	0	4	0	0	4
Employee stock-based compensation expense	0	0	7,026	0	0	7,026
Reclassification of the fair value of warrant liabilities upon exercise	0	0	20,086	0	0	20,086
Issuance of common stock pursuant to an at-the-market financing facility at an average per share purchase price of $8.09 net of fundraising costs of $1,570	4,939	5	38,389	0	0	38,394
Foreign currency translation loss	0	0	40	(308)	0	(268)
Net loss	0	0	0	0	(69,417)	(69,417)
Balance at December 31, 2013	61,615	$ 62	$ 234,286	$ (423)	$ (205,355)	$ 28,570

The accompanying notes are an integral part of these consolidated financial statements.
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Raptor Pharmaceutical Corp.
Consolidated Statements of Cash Flows
(In thousands, except for per share data)

	For the year ended December 31, 2013	For the four months ended December 31, 2012	For the year ended August 31, 2012	2011
Cash flows from operating activities:				
Net loss	$(69,417)	$(19,292)	$(38,644)	$(37,195)
Adjustments to reconcile net loss to net cash used in operating activities:				
Employee stock-based compensation expense	7,026	2,230	4,487	1,920
Consultant stock-based compensation expense	4	9	72	197
Fair value adjustment of common stock warrants	10,747	1,484	3,173	16,301
Amortization of intangible assets	193	49	146	153
Depreciation of fixed assets	244	42	65	78
Gain on sale of fixed assets	(12)	0	0	0
Loss on sale of short-term investments	128	64	0	0
Write-off of intangible assets and other intellectual property	0	0	900	108
Amortization of debt issuance costs	433	0	0	0
Changes in assets and liabilities:				
Accounts receivable, net	(6,181)	0	0	0
Deferred revenue	4,698	0	0	0
Inventories	(3,000)	0	0	0
Prepaid expenses and other	(1,956)	1,501	(2,695)	(130)
Deposits	(72)	79	0	(2)
Accounts payable	(114)	3,081	754	210
Accrued liabilities	10,387	(585)	403	1,119
Deferred rent	296	(8)	(10)	22
Net cash used in operating activities	(46,596)	(11,346)	(31,349)	(17,219)
Cash flows from investing activities:				
Purchase of fixed assets	(1,603)	(55)	(378)	(50)
Sale of fixed assets	27	0	0	0
Purchase of short-term investments	(147)	(6,853)	(45,307)	0
Sale of short-term investments	22,114	0	30,000	0
Intangible assets	(1,250)	0	0	0
Change in restricted cash	(337)	6	(54)	(114)
Net cash provided by (used in) investing activities	18,804	(6,902)	(15,739)	(164)
Cash flows from financing activities:				
Proceeds from the sale of common stock, net	0	0	42,834	0
Proceeds from the sale of common stock under an equity line	0	0	0	6,740
Proceeds from the sale of common stock under an At-the-Market agreement, net	38,394	5,947	7,324	0
Proceeds from the exercise of common stock warrants	10,326	1,843	5,013	8,913

Proceeds from the exercise of common stock options	2,475	192	366	96
Proceeds from note payable	25,000	25,000	0	0
Offering costs	(126)	25	18	(152)
Debt issuance costs	(1,260)	(1,959)	0	0
Principal payments on capital lease obligations	(10)	(2)	(7)	(5)
Net cash provided by financing activities	74,799	31,046	55,548	15,592
Effect of exchange rates on cash and cash equivalents	(268)	(65)	(52)	10
Net increase (decrease) in cash and cash equivalents	46,739	12,733	8,408	(1,781)
Cash and cash equivalents, beginning of period	36,313	23,580	15,172	16,953
Cash and cash equivalents, end of period	$83,052	$36,313	$23,580	$15,172

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Supplemental cash flow information:				
Interest paid	$5,142	$83	$3	$2
Income taxes paid	$2	$0	$0	$0
Supplemental disclosure of non-cash financing activities:				
Common stock issued as fee for equity line	$0	$0	$0	$519
Acquisition of equipment in exchange for capital lease	$60	$0	$13	$14
Fair value of warrant liability reclassified to equity upon exercise	$20,086	$2,345	$9,482	$8,506

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

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RAPTOR PHARMACEUTICAL CORP.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except for per share data, or unless otherwise specified)

(1) NATURE OF OPERATIONS AND BUSINESS RISKS

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

Raptor is a biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. The Company's first product, PROCYSBI® (cysteamine bitartrate) delayed-release capsules ("PROCYSBI"), received marketing approval from the U.S. Food and Drug Administration ("FDA"), on April 30, 2013 for the management of nephropathic cystinosis in adults and children six years and older. The European equivalent, PROCYSBI gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate), received marketing authorization on September 6, 2013 from the European Commission ("EC"), as an orphan medicinal product for the management of proven nephropathic cystinosis for marketing in the European Union ("EU"). PROCYSBI received 7 years and 10 years of market exclusivity as an orphan drug in the U.S. and the EU, respectively. The Company commenced commercial sales of PROCYSBI in the U.S. in mid-June 2013 and plan to launch PROCYSBI in the EU in the first half of 2014. Prior to the second quarter of 2013, the Company had been in the development stage. With FDA approval of PROCYSBI and the commencement of commercial sales in the second quarter of 2013, the Company is no longer considered to be in the development stage. In the near-term, the Company's ability to generate revenues is entirely dependent upon sales of PROCYSBI in the U.S. for the management of nephropathic cystinosis in adults and children six years and older and the EU for the management of nephropathic cystinosis.

Raptor's pipeline includes its proprietary delayed-release form of cysteamine, or RP103. Raptor currently has product candidates in clinical development designed to potentially treat Huntington's disease ("HD"), Non-alcoholic fatty liver disease ("NAFLD"), Leigh syndrome and other mitochondrial disorders and aldehyde dehydrogenase deficiency ("ALDH2"). Raptor's preclinical programs are based upon bioengineered novel drug candidates that are designed to target cancer and other diseases.

The Company is subject to a number of risks, including: the level of commercial sales of PROCYSBI in the U.S. and the ability to launch PROCYSBI in the EU; the uncertainty of whether the Company's research and development efforts will result in expanded indications for PROCYSBI or additional commercial products; competition from larger organizations; reliance on licensing the proprietary technology of others; dependence on key personnel; uncertain patent protection; and the need to raise capital through equity and/or debt financings. Funding may not be available when needed if at all or on terms acceptable to us. If the Company exhausts its cash reserves and is unable to obtain adequate financing, it may be required to curtail planned operating expenditures, including its development programs.

Change in Fiscal Year End

On December 4, 2012, Raptor's Board of Directors approved a change in its fiscal year end from August 31 to December 31. The change became effective at the end of the four months ended December 31, 2012. All references to "fiscal years" or "years" prior to this change refer to the twelve-month fiscal period covering September 1 through August 31, and each year after December 31, 2012, the fiscal year covers January 1 through December 31.

Basis of Presentation

The Company's consolidated financial statements include the accounts of the Company's direct and indirect wholly owned subsidiaries, Raptor Pharmaceuticals Inc., formerly known as Raptor Therapeutics Inc. which merged with Raptor Discoveries Inc. in December 2012 prior to changing its name and Raptor European Products, LLC, such subsidiaries incorporated in Delaware on August 1, 2007, and February 14, 2012, respectively, and Raptor Pharmaceuticals Europe B.V., Raptor Pharmaceuticals France SAS, Raptor Pharmaceuticals Germany GmbH and RPTP European Holdings C.V., domiciled in the Netherlands on December 15, 2009, in France on October 30, 2012, in Germany on October 16, 2013 and in the Cayman Islands on February 16, 2012, respectively. All inter-company

accounts have been eliminated.

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RAPTOR PHARMACEUTICAL CORP.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except for per share data, or unless otherwise specified)

(2) SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Use of Estimates

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

Functional Currency

The Company's consolidated functional currency is the U.S. dollar. Raptor Pharmaceuticals Europe B.V. ("BV"), Raptor Pharmaceuticals France SAS ("SAS"), Raptor Pharmaceuticals Germany GmbH ("GMBH") and RPTP European Holdings C.V. ("CV"), the Company's Dutch subsidiary, French subsidiary, German Subsidiary and Cayman-based subsidiary, respectively, use the European Euro as their functional currency. At each quarter end, each foreign subsidiary's balance sheets are translated into U.S. dollars based upon the quarter-end exchange rate, while their statements of operations and comprehensive loss are translated into U.S. dollars based upon an average exchange rate during the period.

Revenue Recognition and Accounts Receivable

The Company recognizes revenue in accordance with the FASB ASC 605, Revenue Recognition, when the following criteria have been met: persuasive evidence of an arrangement exists; delivery has occurred and risk of loss has passed; the seller's price to the buyer is fixed or determinable and collectability is reasonably assured. The Company determines that persuasive evidence of an arrangement exists based on written contracts that define the terms of the arrangements. Pursuant to the contract terms, the Company determines when title to products and associated risk of loss has passed onto the customer. The Company assesses whether the fee is fixed or determinable based on the payment terms associated with the transaction and whether the sales price is subject to refund or adjustment. The Company assesses collectability based primarily on the customer's payment history and creditworthiness.

PROCYSBI is currently only available for distribution from the Company's U.S. specialty pharmacy partner, the Accredo Health Group, Inc., or Accredo, which is currently the Company's only customer and ships directly to patients. The Company's distributor in the EU will be the Almac Group, Ltd. for the commercial launch in the EU anticipated to occur in the first half of 2014. PROCYSBI is not available in retail pharmacies. Prior authorization of coverage level by patients' private insurance plans, Raptor's patient assistance program ("PAP") or government payors is a prerequisite to the shipment of PROCYSBI to patients. Revenue is recognized once the product has been shipped by the specialty pharmacy to patients because at this time, the Company is unable to reasonably estimate rebate percentages based upon its lack of sufficient historical data. Billings to the Company's distributor in advance of product shipment and delivery by the specialty pharmacy to patients are recorded as deferred revenue by the Company until such shipments to patients occur.

The Company records revenue net of expected discounts, distributor fees, returns and rebates, including those paid to Medicare and Medicaid in the U.S. Allowances are recorded as a reduction of revenue at the time product sales are recognized. Allowances for government rebates and discounts are established based on the actual payor information, which is known at the time of shipment to patients, and the government mandated discount rates applicable to government-funded programs. The allowances are adjusted to reflect known changes in the factors that may impact such allowances in the quarter the changes are known.

Trade accounts receivable are recorded net of product sales allowances for prompt-payment discounts and chargebacks. Estimates for chargebacks and prompt-payment discounts are based on contractual terms and the Company's expectations regarding the utilization rates.

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Inventories and Cost of Sales

Inventories are stated at the lower of cost or market price, with cost determined on a first-in, first-out basis. Inventories are reviewed periodically to identify slow-moving inventory based on sales activity, both projected and historical, as well as product shelf-life. Prior to the approval of PROCYSBI by the FDA on April 30, 2013 and in Europe, prior to EMA approval on September 6, 2013, the Company recorded the purchase of raw materials and the manufacturing costs relating to PROCYSBI as research and development expense. Subsequent to approval, the Company began capitalizing these costs as commercial inventory. On December 31, 2013, net inventories were approximately $3.0 million, which consisted of $2.6 million of raw materials and $0.4 million of finished goods. Upon launching PROCYSBI in mid-June 2013, the Company began recognizing cost of sales. Cost of sales includes the cost of inventory sold or reserved, manufacturing and supply chain costs, product shipping and handling costs, amortization of licensing approval milestone payments and licensing royalties payable to the University of California, San Diego ("UCSD").

Fair Value of Financial Instruments

The carrying amounts of certain of the Company's financial instruments including cash equivalents, restricted cash, accounts payable, accrued liabilities, note payable and capital lease liability approximate fair value due either to length of maturity or interest rates that approximate prevailing market rates. 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

The Company considers all highly liquid investments with original maturities of three months or less, when purchased, to be cash equivalents. The Company maintains cash and cash equivalents, which consist principally of money market funds with high credit quality financial institutions. Such amounts exceed Federal Deposit Insurance Corporation insurance limits. Restricted cash represents compensating balances required by the Company's U.S. and European banks as collateral for credit cards. As of December 31, 2013, the Company had $83.1 million in cash and cash equivalents, of which $4.2 million was held by its foreign subsidiaries.

Short-term Investments

The Company invests in short-term investments in high credit-quality funds in order to obtain higher yields on its idle cash. Short-term investments consisted of a short-term duration government fund in the amount of $22.1 million at December 31, 2012. Such investments were not insured by the Federal Deposit Insurance Corporation. The Company completed an evaluation of its investments and determined that it did not have any other-than-temporary impairments as of December 31, 2012. The Company did not hold investments as of December 31, 2013.

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Prepaid Expenses and Other

Prepaid expenses consists primarily of advance vendor payments which will be expensed within one year from the balance sheet date, including $1.0 million prepaid to the National Institute of Diabetes and Digestive and Kidney Diseases, or NIDDK, which is part of the National Institutes of Health. Such amounts relate to a clinical trial being conducted under a Cooperative Research and Development Agreement, or CRADA, with the NIDDK, and are being recorded to research and development expense over the estimated term of the trial. Future payments due under the CRADA are included in the commitments table under research and development and purchase commitments in Note 12.

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.

Note Payable and Debt Issuance Costs

Note payable consists of the Company's loan agreement with HealthCare Royalty Partners II, L.P. ("HC Royalty"), as lender, under which Raptor borrowed $50.0 million in two $25.0 million tranches received in December 2012 and May 2013. The loan bears interest at an annual fixed rate of 10.75% of outstanding principal and includes a synthetic royalty component based on net product sales, including PROCYSBI, in a calendar year. The fixed and royalty interest are recognized as interest expense as incurred. Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan to interest expense using the effective interest method.

Intangible Assets

Intangible assets primarily include the intellectual property and other rights relating to DR Cysteamine (currently developed as RP103) and to an out-license acquired in a 2009 merger. The intangible assets related to RP103 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.

Fixed Assets

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

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Segment Information

The Company has determined that it operates in only one segment, as it only reports profit and loss information on an aggregate basis to its chief operating decision maker. The Company's long-lived assets maintained outside the U.S. are not material.

Impairment of Goodwill and Intangible Assets

Goodwill represents the excess of the purchase price over the fair value of tangible and identified intangible net assets of businesses acquired. Goodwill is not amortized, but is evaluated for impairment on an annual basis or more often when impairment indicators are present. The Company has one reporting unit. Therefore, the Company's consolidated net assets, including existing goodwill and other intangible assets, are considered to be the carrying value of the reporting unit. If the carrying value of the reporting unit is in excess of its fair value, an impairment may exist, and the Company must perform the second step of the analysis, in which the implied fair value of the goodwill is compared to its carrying value to determine the impairment charge, if any. If the estimated fair value of the reporting unit exceeds the carrying value of the reporting unit, goodwill is not impaired and no further analysis is required.

The Company makes judgments about the recoverability of purchased intangible assets with finite lives whenever events or changes in circumstances indicate that impairment may exist. Recoverability of purchased intangible assets with finite lives is measured by comparing the carrying amount of the asset to the future undiscounted cash flows the asset is expected to generate. Impairment, if any, is measured as the amount by which the carrying value exceeds the fair value of the impaired asset.

Common Stock Warrant Liabilities

The Company issued warrants that contain conditional obligations that may require the Company to transfer cash to settle the warrants upon the occurrence of certain fundamental transactions. Therefore, the Company has classified the warrants as liabilities. The Company re-measures the liability at the end of every reporting period with the change in value reported in the Company's consolidated statements of operations and comprehensive loss. At the exercise date, the fair values of these warrants are re-measured and reclassified to equity.

Research and Development

Research and development costs are charged to expense as incurred. Research and development expenses primarily include salaries and benefits for medical, clinical and regulatory personnel, preclinical studies, clinical trials and commercial drug manufacturing expenses prior to obtaining marketing approval.

Advertising Expenses

The Company expenses advertising costs, including promotional expenses, as incurred. For the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal year ended August 31, 2012, advertising expenses were $3.7 million, $1.3 million and $0.6 million, respectively. The Company did not incur any advertising expenses for the fiscal year ended August 31, 2011.

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Net Loss per Share

Net loss per share is calculated by dividing net loss by the weighted-average shares of common stock outstanding during the period. Diluted net loss per share is calculated by dividing net loss by the weighted-average shares of common stock outstanding and potential shares of common stock during the period. For all periods presented, potentially dilutive securities are excluded from the computation of fully diluted net loss per share as their effect is anti-dilutive. Potentially dilutive securities include:

	December 31,		August 31	
	2013	2012	2012	2011
Warrants to purchase common stock	946	4,563	5,188	7,019
Options to purchase common stock	8,218	7,791	6,125	3,581
Total potentially dilutive securities	9,164	12,354	11,313	10,600

Net loss per share, basic and diluted, was $(1.20), $(0.37), $(0.80) and $(1.15) for the year ended December 31, 2013, the four months ended December 31, 2012 and for the fiscal years ended August 31, 2012 and 2011, respectively.

Comprehensive Loss

The components of comprehensive loss include net loss and foreign currency translation adjustments.

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. Based on the weight of available evidence, including cumulative losses since inception and expected future losses, the Company has 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 the Company's net deferred tax assets.

The Company identifies uncertain tax positions and discloses any potential tax liability on their financial statements. The Company recognizes interest and/or penalties related to income tax matters as a component of income tax expense. As of December 31, 2013, there was no accrued interest and penalties related to uncertain tax positions. The Company files U.S. Federal, California, various other state income and other tax returns and various foreign country income tax returns. The Company is currently not subject to any income tax examinations. Due to the Company's net operating losses ("NOLs"), generally all tax years remain open.

Stock Option Plan

Compensation cost related to the Company's stock option plans is measured at the grant date based on the fair value of the equity instruments awarded and is recognized over the period during which an employee is required to provide service in exchange for the award, or the requisite service period, which is usually the vesting period. The Company recognizes expense associated with stock options issued to third parties, including consultants based upon the fair value of such awards on the date the options vest.

Reclassifications

Certain amounts previously reported under specific financial statement captions have been reclassified to be consistent with the current period presentation.

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RAPTOR PHARMACEUTICAL CORP.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(In thousands, except for per share data, or unless otherwise specified)

(3) INTANGIBLE ASSETS AND GOODWILL

MERGER OF RAPTOR'S CLINICAL DEVELOPMENT SUBSIDIARY AND ENCODE PHARMACEUTICALS, INC.

On December 14, 2007, the Company acquired the intellectual property and other rights to develop RP103 to treat various clinical indications from UCSD by way of a merger with Encode Pharmaceuticals, Inc., a privately held development stage company ("Encode"), which held the intellectual property license with UCSD.

The fair value of the intangible assets at the time of acquisition was approximately $2.6 million.

Pursuant to the license agreement with UCSD, the Company is obligated to pay an annual maintenance fee until the commencement of commercial sales of any licensed products developed. The Company is also obligated to pay milestone payments upon the occurrence of certain events, royalties on net sales from products developed pursuant to the license agreement and a percentage of sublicense fees or royalties, if any. The Company is obligated to fulfill predetermined milestones within a specified number of years from the effective date of the license agreement, depending on the indication. To the extent that the Company fails to perform any of the obligations, UCSD may terminate the license or otherwise cause the license to become non-exclusive.

In May 2013, the Company announced that the FDA has approved PROCYSBI (cysteamine bitartrate) delayed release capsules for the management of nephropathic cystinosis in adults and children 6 years and older. Subsequently, the Company announced that the European Commission (EC) has approved PROCYSBI® gastro-resistant hard capsules of cysteamine (as mercaptamine bitartrate) as an orphan medicinal product for the management of proven nephropathic cystinosis for marketing in the European Union (EU). In conjunction with these approvals, the Company paid milestone payments to UCSD during the second and third quarters of 2013 of $0.8 million and $0.5 million, respectively, pursuant to this license, which were capitalized as intangible assets.

A summary of intangibles acquired is as follows:

	Useful Life (years)	December 31, 2013	2012
Intangible asset (IP license for RP103) related to the Encode merger	20.0	$2,620	$2,620
Intangible assets (UCSD license FDA and EC approval milestones)	20.0	1,250	0
Other intangible assets	16.0	240	240
Total intangible assets	19.8	4,110	2,860
Less accumulated amortization		(897)	(704)
Intangible assets, net		$3,213	$2,156

The intangible assets related to RP103 are being amortized over an 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. Other intangible assets are being amortized using the straight-line method over an estimated useful life of 16 years, which is the life of the intellectual property patents.

As of August 31, 2012, the Company had determined that is acquired in-process research and development asset was impaired and wrote off the $0.9 million carrying amount to research and development expense. During the year ended December 31, 2013, the four months ended December 31, 2012, and the year ended August 31, 2011, there was no intangible asset impairment recognized.

During the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011, the Company amortized $193, $49, $146 and $153, respectively, of intangible assets to research and development expense.

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Amortization expense for intangible assets for each of the next five years is as follows:

Year ending December 31,	Amortization expense
2014	$ 238
2015	238
2016	238
2017	238
2018	238

The Company tested the carrying value of goodwill for impairment as of December 31, 2013 and determined that there was no impairment.

(4) FIXED ASSETS

Fixed assets consisted of:

	December 31,		
Category	2013	2012	Estimated useful lives
Leasehold improvements	$0	$146	Shorter of life of asset or lease term
Office furniture	605	35	7 years
Laboratory equipment	1,132	593	5 years
Manufacturing equipment	102	0	5 years
Computer hardware and software	578	204	3 years
Capital lease equipment	68	27	Shorter of life of asset or lease term
Total at cost	2,485	1,005	
Less: accumulated depreciation	(675)	(589)	
Total fixed assets, net	$1,810	$416	

Depreciation expense for the year ended December 31, 2013, the four months ended December 31, 2012 and the years ended August 31, 2012 and 2011 was $244, $42, $65 and $78, respectively. Accumulated depreciation on capital lease equipment was $10 and $8 as of December 31, 2013 and December 31, 2012, respectively.

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RAPTOR PHARMACEUTICAL CORP.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
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(5) FAIR VALUE MEASUREMENT

The Company uses a fair-value approach to value certain assets and liabilities. Fair value is the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. The Company uses a fair value hierarchy, which distinguishes between assumptions based on market data (observable inputs) and an entity's own assumptions (unobservable inputs). The hierarchy consists of three levels:

- Level one - Quoted market prices in active markets for identical assets or liabilities;
- Level two - Inputs other than level one inputs that are either directly or indirectly observable; and
- Level three - Unobservable inputs developed using estimates and assumptions, which are developed by the reporting entity and reflect those assumptions that a market participant would use.

Determining which category an asset or liability falls within the hierarchy requires significant judgment. The Company evaluates its hierarchy disclosures each reporting period. Assets and liabilities measured at fair value on a recurring basis at December 31, 2013 and December 31, 2012 are summarized as follows:

Assets	Level 1	Level 2	Level 3	December 31, 2013
Cash equivalents	$70,627	$ 0	$0	$70,627
Total	$70,627	$ 0	$0	$70,627
Liabilities				
Common stock warrants	$0	$ 0	$7,066	$7,066
Total	$0	$ 0	$7,066	$7,066

Assets	Level 1	Level 2	Level 3	December 31, 2012
Cash equivalents	$35,069	$ 0	$0	$35,069
Short-term investments	22,096	0	0	22,096
Total	$57,165	$ 0	$0	$57,165
Liabilities				
Common stock warrants	$0	$ 0	$16,405	$16,405
Total	$0	$ 0	$16,405	$16,405

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RAPTOR PHARMACEUTICAL CORP.

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Cash equivalents and short-term investments represent the fair value of the Company's investments in money market funds and a short-term bond fund as of December 31, 2013 and December 31, 2012.
Certain of the Company's common stock warrants are classified as liabilities and are, therefore, re-measured using the Black-Scholes option valuation model at the end of each reporting period with the change in value reported in the Company's consolidated statements of operations and comprehensive loss.
The following table presents a reconciliation of the Company's recurring fair value measurements categorized within level three of the fair value hierarchy (common stock warrants):

Fair value as of September 1, 2012	$17,266
Change in fair value recognized in earnings	1,484
Exercises	(2,345)
Fair value as of December 31, 2012	16,405
Change in fair value recognized in earnings	10,747
Exercises	(20,086)
Fair value as of December 31, 2013	$7,066

Effect of Raptor's Stock Price and Volatility Assumptions on the Calculation of Fair Value of Warrant Liabilities
As discussed above, the Company uses the Black-Scholes option pricing model as its method of valuation for warrants that are subject to warrant liability accounting. The determination of the fair value as of the reporting date is affected by Raptor's stock price as well as assumptions regarding a number of highly complex and subjective variables. These variables include, but are not limited to, expected stock price volatility over the term of the security and risk-free interest rate. The primary factors affecting the fair value of the warrant liability are the Company's stock price and volatility.

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(6) NOTE PAYABLE

On December 20, 2012, the Company entered into a loan agreement with HealthCare Royalty Partners ("HC Royalty"), as lender, under which it agreed to borrow $50.0 million in two $25.0 million tranches (the "HC Royalty Loan"). The Company received $23.4 million in net proceeds from the first tranche of the loan at closing in December 2012 and an additional $23.7 million in net proceeds in May 2013 from the second tranche upon FDA approval of RP103 for the management of cystinosis. The Company's loan agreement with HC Royalty includes a variety of affirmative and negative covenants, including the use of commercially reasonable efforts to exploit RP103 in specific markets and compliance with laws, as well as restrictions on mergers and sales of assets, incurrence of liens, incurrence of indebtedness and transactions with affiliates and other requirements. To secure the performance of the Company's obligations under the HC Royalty Loan, the Company granted a security interest to HC Royalty in substantially all of its assets, the assets of its domestic subsidiaries and a pledge of stock of certain of its domestic subsidiaries. The Company's failure to comply with the terms of the HC Royalty Loan agreement and related documents, the occurrence of a change of control of the Company or the occurrence of an uncured material adverse effect on the Company, or Raptor Pharmaceuticals, or the occurrence of certain other specified events, could result in an event of default under the HC Royalty Loan agreement that, if not cured or waived, could result in the acceleration of the payment of all of its indebtedness, as well as prepayment penalties, to HC Royalty and interest thereon. Under the terms of the security agreement, in an event of default, the lender could potentially take possession of, foreclose on, sell, assign or grant a license to use, the Company's pledged collateral and assign and transfer the pledged stock of certain of its subsidiaries.

The loan bears interest at an annual fixed rate of 10.75%, payable quarterly. The loan also contains a synthetic royalty component based on net product revenues, including PROCYSBI, in a calendar year, and such royalty is payable quarterly. With respect to the first $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.25% of the first $25.0 million of product net revenues, 3.0% of product net revenues for such calendar year in excess of $25.0 million and below $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly. With respect to the second $25.0 million tranche, for each calendar year (prorated for any portion thereof), the loan bears a royalty rate of 6.0% of the first $25.0 million of net revenues for such calendar year, 3.0% of product net revenues for such calendar year in excess of $25.0 million and below $50.0 million, and 1.0% of product net revenues for such calendar year in excess of $50.0 million, payable quarterly.

The Company received marketing approval of PROCYSBI from the FDA on April 30, 2013 and commenced shipment of PROCYSBI during June 2013, and as a result, royalties became payable to HC Royalty based upon net revenues of PROCYSBI. Interest expense on the loan for the year ended December 31, 2013 and the four months ended December 31, 2012 was approximately $6.8 million and $0.1 million, respectively.

The loan and the Company's obligation to make any payments shall terminate immediately when all payments received by HC Royalty equal $97.5 million. If, by December 20, 2014, net revenue for the immediately preceding four fiscal quarters exceed $100.0 million, then the loan and the Company's obligation to make any payments shall terminate immediately when all payments received by HC Royalty from the Company equal $90.0 million. Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan to interest expense using the effective interest method.

Unamortized debt issuance costs totaled $2.8 million as of December 31, 2013. Amortization expense was $0.4 million for the year ended December 31, 2013 and a nominal amount for the four months ended December 31, 2012.

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(7) ACCRUED LIABILITIES

Accrued liabilities consisted of:

	December 31,	
	2013	2012
Clinical trial and related costs	$1,661	$641
Personnel-related costs	4,443	1,244
Rebates and other sales deductions	2,325	0
Royalty based interest payable	1,255	0
License royalty payable	564	0
Other	2,519	265
Total accrued liabilities	$12,767	$2,150

(8) STOCK OPTION PLANS

2010 Stock Incentive Plan

In February 2010, the Company's Board of Directors approved, and in March 2010 Raptor's stockholders approved, the Raptor Pharmaceutical Corp. 2010 Stock Incentive Plan, as subsequently amended and approved by its stockholders in March 2011 ("Amended Plan"). On July 23, 2013, the Company, held its 2013 Annual Meeting of Stockholders (the "Annual Meeting"). At the Annual Meeting, Raptor's stockholders approved an amendment to the Amended Plan, which among other things, increased the authorized share reserve by 3,000,000 shares to an aggregate of 11,936,383 shares. As of December 31, 2013, there were 3,861,728 shares remaining available for issuance. Stock options are granted to recognize the contributions made by its employees, independent contractors, consultants and directors, to provide those individuals with additional incentive to devote themselves to the Company's future success and to improve its ability to attract, retain and motivate individuals upon whom its growth and financial success depends. Employee stock options generally vest over four years with a six-month clift-vesting period. In general, all options are exercisable over a period not to exceed the contractual term of ten years from the date the stock options are granted at prices not less than the fair market value of the Company's common stock on the grant date. The Company has and may grant options with different vesting terms from time to time.

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The Company recorded employee stock-based compensation expense as follows:

	For the year ended December 31, 2013	For the four months ended December 31, 2012	For the year ended August 31, 2012	2011
Research and development	$ 1,546	$ 453	$926	$421
Selling, general and administrative	5,480	1,777	3,561	1,499
Total stock-based compensation expense	$ 7,026	$ 2,230	$4,487	$1,920

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

Period*	Risk-free interest rate	Expected life of stock option	Annual volatility
Year ended August 31, 2011	1.6 to 2.4%	6 years	88 to 116%
Year ended August 31, 2012	0.68 to 1.2%	5 to 6 years	121 to 125%
Four months ended December 31, 2012	0.68 to 0.7%	5 years	95%
Year ended December 31, 2013	0.71 to 1.51%	5 years	66 to 100%

*Dividend rate is 0% for all periods presented.
The compensation expense for stock-based compensation awards includes an estimate for forfeitures and is recognized over the requisite service period of the options, which is typically the period over which the options vest, using the straight-line method.
The Company also recognizes as an expense the fair value of options granted to persons who are neither employees nor directors. The fair value of expensed options was based on the Black-Scholes option-pricing model assuming the same factors shown in the stock-based compensation expense table above. Stock-based compensation expense for consultants for the year ended December 31, 2013, the four months ended December 31, 2012 and the years ended August 31, 2012 and 2011, was $4, $9, $72 and $197, respectively.

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A summary of the activity in the 2010 Equity Incentive Plan, the 2006 Equity Compensation Plan, as amended, and the Company's other stock option plans, is as follows:

	Option shares	Weighted-average exercise price	Weighted-average fair value of options granted
Outstanding at December 31, 2012	7,791	$ 5.79	3.48
Granted	1,187	9.21	5.33
Exercised	(651)	3.80	
Canceled	(109)	34.89	
Outstanding at December 31, 2013	8,218	6.05	5.77

The number of options outstanding, vested and expected to vest as of December 31, 2013 was 8,109 and the weighted-average remaining contractual life was 7.6 years. The aggregate intrinsic value and the weighted-average intrinsic value of stock options outstanding, vested and expected to vest as of December 31, 2013 was $71.8 million and $8.85 per option, respectively. The number of options outstanding, vested and expected to vest as of December 31, 2012 was 7,644 and the weighted-average remaining contractual life was 8.21 years. The aggregate intrinsic value and the weighted-average intrinsic value of stock options outstanding, vested and expected to vest as of December 31, 2012 was $10.3 million and $1.34 per option, respectively.
As of December 31, 2013, the options outstanding under all of the Company's stock option plans consisted of the following (in thousands, except per share data):

	Options outstanding			Options vested and exercisable		
Range of exercise prices	Number of options outstanding	Weighted-average remaining contractual life	Weighted-average exercise price	Number of options exercisable	Weighted-average remaining contractual life	Weighted-average exercise price
($)	(#)	(years)	($)	(#)	(years)	($)
$0 to $3.00	1,174	5.06	2.65	1,069	4.89	$ 2.62
$3.01 to $4.00	1,613	6.90	3.51	1,471	6.87	3.52
$4.01 to $5.00	296	7.87	4.79	138	6.81	4.74
$5.01 to $6.00	3,917	8.23	5.29	1,935	8.12	5.26
$6.01 to $7.00	487	8.74	6.45	138	8.48	6.46
$7.01 to $8.00	165	9.03	7.69	29	8.78	7.70
$8.01 to $965.00	566	9.26	25.49	36	2.17	217.52
	8,218	7.62	6.05	4,816	6.95	5.77

The aggregate intrinsic value of stock options outstanding as of December 31, 2013 was $72.5 million. The aggregate intrinsic value of stock options exercisable as of December 31, 2013 was $46.9 million.

At December 31, 2013, the total unrecognized compensation cost was approximately $14.8 million. The weighted-average period over which it is expected to be recognized is approximately 2.64 years.

(In thousands, except for per share data)	For the year ended December 31, 2013	For the four months ended December 31, 2012	For the year ended August 31, 2012	2011
Weighted-average fair value per share of options granted	$ 5.33	$ 3.84	$4.62	$2.54
Aggregate intrinsic value of options exercised	5,979	228	602	131

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(9) INCOME TAXES

The Company had losses before income taxes for domestic and foreign operations as follows:

	For the year ended December	For the four months ended December	For the year ended August 31,	
(In thousands)	31, 2013	31, 2012	2012	2011
Domestic	$ 33,966	$ 12,510	$26,642	$37,415
Foreign	35,451	6,782	12,002	(220)
Loss before income taxes	$ 69,417	$ 19,292	$38,644	$37,195

The provision for income taxes differs from the amount estimated by applying the statutory federal income tax rate to loss before taxes as follows:

	For the year ended December 31,		For the four month period ended December 31,		August 31,			
(In thousands)	2013		2012		2012		2011	
Federal tax (benefit) at statutory rate	$(23,595)	-34.00%	$(6,536)	-34.00%	$(13,111)	-34.00%	$(12,679)	-34.00%
State tax (benefit) at statutory rate, net of federal tax benefit	497	0.72	(715)	-3.72	(1,069)	-2.77	(2,247)	-6.03
Change in valuation allowance	14,475	20.86	2,349	12.22	4,775	12.38	19,278	51.70
Research and development credits	(10,354)	-14.92	0	0	(1,034)	-2.68	(1,831)	-4.91
Fair market value of warrants	3,654	5.27	505	2.62	1,079	2.80	5,543	14.86
Qualified Therapeutic Discovery Project Grant income	0	0	0	0	0	0	(297)	-0.80
Intangible asset basis allocation	0	0	1,670	8.69	2,952	7.66	(8,633)	-23.15
Stock based compensation – ISO	748	1.08	755	3.93	1,525	3.96	493	1.32
Tax attributes not benefited	4,215	6.07	0	0	0	0	0	0
Foreign losses not benefited	10,368	14.94	1,995	10.38	3,848	9.98	0	0
Other	(8)	-0.02	(23)	-0.12	1,035	2.67	373	1.01
Provision for income taxes	$0	0 %	$0	0 %	$0	0 %	$0	0 %

Deferred tax assets consist of the following as of December 31,:

	2013	2012
Deferred Tax Assets		
Net operating loss carryforwards	$18,797	$19,514
Capitalized start-up costs	11,256	11,160
Stock option expense	1,909	425
Research credits	19,281	7,970
Fixed assets and intangible assets	4,813	3,786
Accruals	1,224	330
Inventory	186	0
Other	65	(50)
Valuation allowance	(57,531)	(43,135)
Deferred tax assets, net	$0	$0

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As of December 31, 2013, the Company had net operating loss carryforwards for U.S. federal, U.S. state and foreign income tax purposes of approximately $38.3 million, $66.8 million and $10.7 million, respectively, which expire beginning after the year 2022, 2016 and 2021, respectively. As of December 31, 2013 the Company had federal and state research and development credits of $18.6 million and $1.1 million respectively. The federal credits expire beginning after the year 2026 and the state credits have no expiration.

As of December 31, 2013, the Company's net operating loss carryforwards for federal and state income tax purposes include approximately $1.5 million and $0.4 million on a gross basis, respectively, of losses attributable to stock option tax expense deductions.

The valuation allowance increased approximately $14.4 million during the period ending December 31, 2013, primarily as a result of current year losses and tax credits.

Utilization of the Company's net operating loss may be subject to substantial annual limitation due to the ownership change limitations provided by the Internal Revenue Code and similar state provisions. Such an annual limitation could result in the expiration of the net operating loss before utilization.

The Company has analyzed its tax positions in all of the federal, state and foreign jurisdictions where it is required to file income tax returns, as well as all open tax years in these jurisdictions.

As of December 31, 2013, the Company had no unrecognized tax benefits and has recorded no liability related to uncertain tax positions. The Company did not record a change in its unrecorded tax benefits during the year ended December 31, 2013, and expects no change in its unrecorded tax benefits in the next 12 months.

Due to net operating loss and research credit carryforwards, substantially all of the Company's tax years, from 2001 through 2013, remain open to U.S. federal and state tax examinations.

The Company is not aware of any pending income tax audits. Significant components of the Company's deferred tax assets for income tax purposes are net operating loss carryforwards, capitalized start-up costs, and stock-based compensation and research credits. Due to the Company's lack of earning history, any deferred assets recorded have been fully offset by a valuation reserve.

The Company's practice is to recognize interest and/or penalties related to income tax matters as a component of income tax expense. As of December 31, 2013, there were no accrued interest and penalties related to uncertain tax positions.

(10) ISSUANCE OF COMMON STOCK

As of December 31, 2013, there were 61,615 shares of the Company's common stock outstanding.

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

On August 21, 2009, Raptor entered into a securities purchase agreement pursuant to which the Company issued shares and warrants for aggregate gross proceeds of approximately $2.4 million. The warrants, which were exercisable for two years from the closing, entitled the investors to purchase, in the aggregate, up to 869 shares of Raptor's common stock and have an exercise price of either $2.57 until the first anniversary of issuance or $3.22 per share after the first anniversary of issuance.

In connection with the August 2009 private placement, the Company also issued warrants to its sole placement agent to compensate it for placing investors into the financing. The placement agent was issued a five-year warrant to purchase 130 shares of Raptor's Common Stock at an exercise price of $1.50 per share.

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2009 MERGER AND NASDAQ LISTING

On September 29, 2009, the Company, formerly known as TorreyPines Therapeutics, Inc. ("TorreyPines") and Raptor Pharmaceutical Corp. ("RPC") completed a reverse merger. The Company changed its name to "Raptor Pharmaceutical Corp." and commenced trading on September 30, 2009 on the NASDAQ Capital Market under the ticker symbol "RPTP". Effective February 29, 2012, the Company's common stock commenced trading on the NASDAQ Global Market. In connection with the merger, the Company assumed all of the TorreyPines stock options and warrants outstanding at the time of the merger. The warrants are exercisable at $80.86 per share and expire on various dates through September 2015.

ISSUANCES OF COMMON STOCK AND WARRANTS IN CONNECTION WITH THE SALE OF UNITS IN A REGISTERED DIRECT OFFERING

On December 17, 2009, the Company entered into a Placement Agent Agreement (the "2009 Placement Agent"), pursuant to a registered direct offering (the "Direct Offering") of up to 3,748 units (the "Units"), consisting of (i) 3,748 shares of the Company's common stock, (ii) warrants to purchase an aggregate of up to 1,874 shares of the Company's common stock (and the shares of common stock issuable from time to time upon exercise of such warrants) (the "Series A Warrants"), and (iii) warrants to purchase an aggregate of up to 1,874 shares of the Company's common stock (and the shares of common stock issuable from time to time upon exercise of such warrants) (the "Series B Warrants," and collectively with the Series A Warrants, the "Investor Warrants").

The Series A Warrants were exercisable during the period beginning one hundred eighty (180) days after the date of issue and ending on the fifth (5th) anniversary of the date of issue. The Series B Warrants were exercisable during the period beginning one hundred eighty (180) days after the date of issue and ending on the eighteen (18) month anniversary of the date of issue. The Investor Warrants have a per share exercise price of $2.45. Based on the underlying terms of the Investor Warrants and Placement Agent Warrants, these warrants are classified as a liability, as discussed further below in Note 11.

ISSUANCES OF COMMON STOCK IN CONNECTION WITH AN EQUITY LINE

On April 16, 2010, the Company signed a purchase agreement with Lincoln Park Capital Fund, LLC ("LPC"), together with a registration rights agreement, whereby LPC agreed to purchase up to $15.0 million of the Company's common stock over a 25 month period.

The purchase price of the shares issued to LPC under the purchase agreement was based on the prevailing market prices of the Company's shares at the time of sale without any fixed discount. The Company controlled the timing and amount of any sales of shares to LPC. LPC did not have the right or the obligation to purchase any shares of the Company's common stock on any business day that the purchase price of the Company's common stock was below $1.50 per share.

2010 PRIVATE PLACEMENT

On August 9, 2010, the Company entered into a securities purchase agreement for the private placement (the "2010 Private Placement") of the Company's common stock and warrants to purchase its common stock, at a purchase price of $3.075 per unit, with each unit comprised of one share of common stock and a warrant to purchase one share of common stock. Each warrant, exercisable for 5 years from August 12, 2010, has an exercise price of $3.075 per share.

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2011 FOLLOW-ON PUBLIC OFFERING

On September 13, 2011, the Company closed an underwritten public offering of shares of the Company's 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 common stock plus an additional 1.5 million shares of common stock pursuant to the exercise by the underwriters of the over-allotment option the Company granted to them. Total gross proceeds to the Company 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 the Company of approximately $42.8 million after deduction of underwriting discounts of 6% and other offering expenses paid by the Company.

ISSUANCES OF COMMON STOCK IN CONNECTION WITH AN AT-THE-MARKET COMMON STOCK SALES PROGRAM

On April 30, 2012, the Company entered into an "At-the-Market" ("ATM") Sales Agreement, with Cowen and Company, LLC ("Cowen"), under which the Company may, at its discretion, sell its 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 may vary.

On July 3, 2013, the Company and Cowen amended and restated the Sales Agreement (the "Amended and Restated Sales Agreement") to increase the aggregate gross sales proceeds that may be raised to $100 million.

Sales in the ATM offerings are being made pursuant to the prospectus supplement dated April 30, 2012, as amended by Amendment No. 2 dated July 3, 2013, which supplements the Company's prospectus dated February 3, 2012, filed as part of the shelf registration statement that was declared effective by the Securities and Exchange Commission ("SEC") on February 3, 2012. During the year ended December 31, 2013, the Company sold approximately 4.9 million shares under ATM offerings at a weighted-average selling price of $8.09 per share for proceeds of approximately $38.8 million after commissions. During the four month period ended December 31, 2012 and fiscal year ended August 31, 2012, the Company sold approximately 1.2 million shares and 1.5 million shares, respectively, at a weighted-average selling price of $5.10 and $5.34 per share, respectively, for net proceeds of approximately $7.4 million and $6.0 million, net of commissions, respectively. As of December 31, 2013, the Company had approximately $46.2 million remaining available under the ATM for future sales of the Company's common stock.

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(11) WARRANTS

The table reflects the number of common stock warrants outstanding as of December 31, 2013:

	Number of shares exercisable	Exercise price	Expiration date
Issued in connection with Encode merger	233	$ 2.87	12/13/2015
Issued to placement agents in August 2009	30	$ 1.50	8/21/2014
TorreyPines warrants assumed in 2009 Merger	4	$ 80.86	*9/26/2015
Issued to registered direct investors in December 2009	12	$ 2.45	12/22/2014
Issued to private placement investors in August 2010	569	$ 3.075	8/12/2015
Issued to placement agent in August 2010	98	$ 3.075	8/12/2015
Total warrants outstanding	946	$ 3.03	*

*Weighted-average exercise price

The warrants issued by the Company in the August 2010 private placement and the December 2009 equity financing contain a conditional obligation that may require the Company to transfer assets to repurchase the warrants upon the occurrence of potential future events. Under ASC 480, a financial instrument that may require the issuer to settle the obligation by transferring assets is classified as a liability. Therefore, the Company has classified the warrants from both financings as liabilities and marks them to fair value at each period end.

A Black-Scholes option-pricing model was used to obtain the fair value of the warrants issued in the December 2009 and August 2010 equity financings using the following assumptions at December 31, 2013 and December 31, 2012:

	December 2009 equity financing		August 2010 private placement Investors and placement agent	
	December 31, 2013	December 31, 2012	December 31, 2013	December 31, 2012
Fair value ($ millions)	$0.1	$ 2.6	$6.9	$ 13.8
Black-Scholes inputs:				
Stock price	$13.02	$ 5.85	$13.02	$ 5.85
Exercise price	$2.45	$ 2.45	$3.075	$ 3.075
Risk free interest rate	0. %	0.25 %	0. %	0.31 %
Volatility	95 %	100 %	95 %	112 %
Expected term (years)	1.00	2.00	1.75	2.50
Dividend	0	0	0	0

For the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011, the Company recorded losses of approximately $10.7 million, $1.5 million, $3.2 million and $16.3 million, respectively, in its consolidated statements of operations and comprehensive loss from changes in the fair values of warrants.

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(12) COMMITMENTS AND CONTINGENCIES

CONTRACTUAL OBLIGATIONS WITH UCSD RELATING TO THE ACQUISITION OF THE DR CYSTEAMINE (RP103) LICENSE

Pursuant to the license agreement with UCSD, the Company is obligated to pay an annual maintenance fee until the commencement of commercial sales of any licensed products developed. The Company is also obligated to pay milestone payments upon the occurrence of certain events, royalties on net sales from products developed pursuant to the license agreement and a percentage of sublicense fees or royalties, if any The Company is obligated to fulfill predetermined milestones within a specified number of years from the effective date of the license agreement, depending on the indication. Cumulatively, the Company has expensed $910 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis, Huntington's disease and NASH and on regulatory filings in cystinosis. To the extent that the Company fails to perform any of its obligations under the license agreement, then UCSD may terminate the license or otherwise cause the license to become non-exclusive.

LEASES

On April 25, 2013, the Company executed a seven-year lease for its corporate office facilities in Novato, California. The Company took occupancy of such facilities at the end of June 2013. For the period June 2013 through May 2014, the Company is obligated to make lease payments of $19,460 per month. On June 10, 2013, the Company amended the lease to add space to accommodate its research laboratory. The Company is obligated to make additional lease payments of $1,870 per month for the period June 2013 through May 2014 under this amendment. Under the lease, the Company will relocate to an adjacent facility when it becomes available approximately mid-2014. In conjunction with the move, excluding certain operating expenses and taxes, the initial base rent will increase to $71,275 per month and is subject to annual increases. The Company records such rent on a straight-line basis.

Rent expense for the Company's current and previous facilities was approximately $0.6 million, $0.1 million, $0.2 million and $0.2 million for the year ended December 31, 2013, the four months ended December 31, 2012 and the fiscal years ended August 31, 2012 and 2011, respectively.

The Company is also subject to contingent payments related to various development activities which are primarily due upon the achievement of certain development and commercial milestones. The Company maintains several contracts with suppliers, contract manufacturers, research organizations, clinical organizations, drug labelers and distributors and clinical sites, primarily to assist with clinical research, clinical and commercial manufacturing and distribution of PROCYSBI and clinical manufacturing of drug product for the Company's HD and NAFLD clinical collaborations. The future commitments pursuant to these agreements are included in the table below as research and development and purchase commitments.

The Company has contractual obligations as of December 31, 2013, which are presented in the table below:

	Payments due by period						
(In thousands)	2014	2015	2016	2017	2018	Thereafter	Total
Debt principal	$ 0	$ 7,500	$ 10,000	$ 10,000	$ 10,000	$ 12,500	$ 50,000
Capital lease obligations	18	18	18	8	0	0	62
Operating lease obligations	710	673	894	920	948	2,497	6,642
Research and development and purchase commitments	7,902	3,222	261	70	70	180	11,705
Total	$ 8,630	$ 11,413	$ 11,173	$ 10,998	$ 11,018	$ 15,177	$ 68,409

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(13) QUALIFYING THERAPEUTIC DISCOVERY PROJECT GRANT

In October 2010, the Company was awarded a tax grant under the U.S. Government's Qualifying Therapeutic Discovery Project for five of its research programs including its cystinosis, Huntington's disease and NASH clinical programs and its HepTide™ and WntTide™ preclinical cancer research programs. The Company was granted an aggregate of approximately $1.1 million for all five programs of which, as of August 31, 2011, it had received approximately $0.9 million. The Company recorded the $0.8 million of proceeds as a contra-research and development expense during the first two quarters of fiscal year 2011. During the fiscal year ended August 31, 2012, the Company received approximately $162 pursuant to the government program funding guidelines and the remaining balance of approximately $36 was drawn but was returned to the government in March 2012 along with an additional $28 as recapture tax because the Company had not incurred the amount originally estimated as qualified expenses for its WntTide™ program, which was the basis for the program funding. The Company recorded the contra-expense upon receipt of the grant proceeds.

(14) QUARTERLY RESULTS OF OPERATIONS (UNAUDITED)

The following table presents selected 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. These unaudited results were prepared on the same basis as the Company's audited consolidated financial statements. The Company's 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 amounts of its revenues and the timing and nature of research and development activities.

	(In millions, except per share data, unaudited)			
Quarterly Data 2013:	March 31, 2013	June 30, 2013	September 30, 2013	December 31, 2013
Net sales	$0	$ 0	$ 6.7	$ 10.2
Net loss	$(15.9)	$ (24.1)	$ (17.3)	$ (12.1)
Net loss per share, basic and diluted	$(0.30)	$ (0.43)	$ (0.29)	$ (0.20)

Quarterly Data for the Four Months Ended December 31, 2012:	November 30, 2012 (1)
Net loss	$(13.4)
Net loss per share, basic and diluted	$(0.26)

Quarterly Data 2012:	November 30, 2011	February 29, 2012	May 31, 2012	August 31, 2012
Net loss	$(11.4)	$ (14.0)	$ (3.0)	$ (10.2)
Net loss per share, basic and diluted	$(0.25)	$ (0.29)	$ (0.06)	$ (0.21)

Quarterly Data 2011:	November 30, 2010	February 28, 2011	May 31, 2011	August 31, 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)

(1) The Company changed its fiscal year end to December; the four month transition period included one quarterly report on Form 10-Q for the three months ended November 30, 2012.

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RAPTOR PHARMACEUTICAL CORP.

Schedule II: Valuation and Qualifying Accounts
(in millions)

Valuation allowance for deferred tax assets	Balance at beginning of year	Additions charged to expenses/other accounts	Net (deductions) recoveries	Balance at end of year
2013	$ 43	$ 15	$ 0	$ 58
Four months ended December 31, 2012	$ 41	$ 2	$ 0	$ 43
2012	$ 36	$ 5	$ 0	$ 41
2011	$ 17	$ 19	$ 0	$ 36