Raptor Pharmaceutical Corp
Form 10-KT/A
May 12, 2014

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K/A
(Amendment No. 2)

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

For the fiscal year ended _____

x TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from September 1, 2012 to December 31, 2012

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, $.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 o

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

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

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

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

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

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act.) Yes o 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: 62,616,859 shares common stock, par value $0.001, outstanding as of April 30, 2014.

The documents incorporated by reference are as follows:

None.

EXPLANATORY NOTE

As previously disclosed by Raptor Pharmaceutical Corp., or the "Company," in its current report on Form 8-K filed with the U.S. Securities and Exchange Commission (the "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.

The Company is filing this Amendment No. 2 (the "Form 10-KT/A") to its Transition Report on Form 10-KT for the four-month transition period ended December 31, 2012, filed with the Commission on March 14, 2013 (the "Form 10-KT"), as previously amended by Amendment No. 1 to Form 10-KT filed with the Commission on June 19, 2013, to include the audit report of Grant Thornton.

In addition to the changes to the consolidated financial statements and notes thereto included in this Form 10-KT/A, which are to conform to the presentation of the 2013 financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2013 (filed with the Commission on March 17, 2014), the Company has amended (i) Part I, Item 7 to delete its previous disclosure relating to an explanatory paragraph regarding the Company's ability to continue as a going concern because this explanatory paragraph was removed from BPM's reissued reports for such periods due to changes in the Company's circumstances, (ii) Part II, Item 9A to include a reference to the attestation report of Grant Thornton and make other changes included therein, (iii) Part III, Item 14 to include the fees of Grant Thornton for re-audit of the Company's four-month transition period ended December 31, 2012, and (iv) Part IV, Item 15 to make changes to the exhibits and to include Grant Thornton's consent and its reports with respect to the Company's consolidated financial statements and internal control over financial reporting included in the Form 10-KT/A.

Except as specifically noted above, this Form 10-KT/A does not modify or update disclosures in the Form 10-KT, and there have been no other material changes to the disclosures made in the Form 10-KT as of the filing of the Form 10-KT. Accordingly, except as specifically noted above, this Form 10-KT/A does not reflect events occurring after the filing of the Form 10-KT or modify or update any related or other disclosures.

RAPTOR PHARMACEUTICAL CORP.

Table of Contents

Table of Contents

PART I

FORWARD-LOOKING STATEMENTS

(In $ thousands, except as noted or per share data and percentages)

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. As a result of this change, this Transition Report on Form 10-KT includes the financial information for the four month transition period from September 1, 2012 to December 31, 2012, or "Transition Period". References in this Transition Report on Form 10-KT to fiscal year 2012 or fiscal 2012 refer to the period from September 1, 2011 through August 31, 2012 and references to fiscal year 2011 or fiscal 2011 refer to the period from September 1, 2010 through August 31, 2011. Prior to this Transition Report on Form 10-KT, our Annual Reports on Form 10-K cover the fiscal year from September 1 to August 31.

Forward-Looking Statement

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

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

Although we believe that the expectations reflected in the forward-looking statements are reasonable, the factors discussed in this Transition Report on Form 10-KT, in other filings with the SEC and in press releases and other public statements by our officers throughout the year, could cause actual results or outcomes to differ materially and/or adversely from those expressed in any forward-looking statements made by us or on our behalf, and therefore we cannot guarantee future results, levels of activity, performance or achievements and you should not place undue reliance on any such forward-looking statements. We cannot give you any assurance that such forward-looking statements will prove to be accurate and such forward-looking events may not occur. In light of the significant uncertainties inherent in such forward-looking statements, you should not regard the inclusion of this information as a

representation by us or any other person that the results or conditions described in those statements or our objectives and plans will be achieved.

All subsequent forward-looking statements attributable to us or any person acting on our behalf are expressly qualified in their entirety by the cautionary statements contained or referred to herein. Unless required by U.S. federal securities laws and the rules and regulations of the SEC, we do not undertake any obligation and disclaim any intention to update or release publicly any revisions to these forward-looking statements after the filing of this Transition Report on Form 10-KT to reflect later events or circumstances or to reflect the occurrence of unanticipated events or any other reason.

Table of Contents

ITEM 1: BUSINESS

You should read the following discussion in conjunction with our consolidated financial statements as of December 31, 2012, and the notes to such consolidated financial statements included elsewhere in this Transition Report on Form 10-KT. This "Business" section contains forward-looking statements.

Unless otherwise mentioned or unless the context requires otherwise (e.g., our consolidated financial statements as of December 31, 2012, and the notes to such consolidated financial statements included elsewhere in this Transition Report on Form 10-KT, or a reference to an event or circumstance that occurred prior to the effective time of the 2009 Merger on September 29, 2009), all references in this Transition Report on Form 10-KT to "the Company," "we," "our," "us" "Raptor" and similar references refer to the public company formerly known as TorreyPines Therapeutics, Inc. and now known as Raptor Pharmaceutical Corp. and its direct and indirect wholly-owned subsidiaries, Raptor Pharmaceuticals Corp. (which was merged into us as of December 7, 2011), Raptor Discoveries Inc. (which was merged into Raptor Therapeutics Inc. as of December 28, 2012), or Raptor Discoveries, Raptor Therapeutics Inc. (which changed its name to Raptor Pharmaceuticals Inc. as of December 28, 2012), or Raptor Pharmaceuticals, Raptor European Products, LLC, RPTP European Holdings C.V., Raptor Pharmaceuticals Europe B.V. and Raptor Pharmaceuticals France SAS.

Overview

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

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

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

Clinical Development Programs

Our current active clinical development programs involve the clinical evaluation of RP103, a delayed and extended release formulation of cysteamine bitartrate. Cysteamine bitartrate was approved in 1994 as an oral, immediate-release powder in a capsule for the treatment of, and is the current standard of systemic care for, cystinosis. We reengineered cysteamine bitartrate in an effort to improve the dose administration, safety and/or efficacy compared to the existing treatment for cystinosis and we are studying cysteamine bitartrate for potential applications in new disease indications. Our proprietary delayed- and extended-release formulation, RP103, is a capsule containing enteric coated micro-beads of cysteamine bitartrate. We believe we have demonstrated that RP103 requires less frequent dosing and can be taken with substantially fewer antacid medications without increasing gastro-intestinal and other side effects compared to immediate-release cysteamine bitartrate for cystinosis patients. In addition to cystinosis, we are also testing RP103 for the potential treatment of NASH and HD. We have an exclusive worldwide

license to delayed-release cysteamine from the University of California, San Diego, or UCSD, which is the basis for our proprietary formulation of cysteamine bitartrate.

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.

RP103 for Cystinosis

Cystinosis is a rare, life-threatening error of metabolism that results in toxic cystine accumulation in all cells. Cystine accumulation causes widespread tissue and vital organ failure and death in late childhood if left untreated. Cystinosis is usually diagnosed in the first year of life after children present with symptoms including markedly increased urination, thirst, dehydration, gastrointestinal distress, failure to thrive, rickets, photophobia and specific kidney symptoms (Fanconi syndrome). If patients survive to adolescence, they suffer from kidney malfunction, muscle wasting, myopathy, difficulty swallowing, respiratory problems, diabetes and hypothyroidism.

Table of Contents

Normal cystine protein turnover is absent in cystinosis patients, resulting in continuous intracellular cystine accumulation, which requires constant cystine depletion through aggressive therapeutic intervention. Studies have shown that cystine depleting therapy may delay and even prevent kidney transplant and lessen other clinical manifestations of the disease. The goal of cystine depletion therapy for cystinosis is to reduce cystine levels in cells to below toxic levels (generally recognized as 1nmol/mg protein in white blood cells). Immediate-release cystine depleting treatment (cysteamine bitartrate), or Cystagon®, is the current standard of care. Cystagon has been available since 1994 in the U.S. and 1997 in the EU, but, due to its pharmacokinetics, requires to an every 6-hour dosing schedule, including a middle-of-the-night dose to maintain adequate therapeutic drug levels. The dosing schedule for Cystagon requires strict adherence to every six-hour administration which is a challenge for patients and caregivers along with the drug side effects of immediate release of cysteamine bitartrate, which include severe gastrointestinal distress (nausea, vomiting) and a strong exhaled rotten egg smell and body odor. In a recent survey of 37 cystinosis patients and caregivers conducted at the June 2011 Cystinosis Research Foundation, or CRF, conference, 63% of patients rated the burden of nighttime dosing a 9 on a scale from 1 to 10 (10 being the worst burden). The requirement for middle-of-the-night dosing is the most significant compliance burden noted by patients. Inadequate disease control resulting from skipping this nocturnal dose was the subject of a major publication (Levtchenko, Pediatr Nephrol 2006). In addition to the Cystagon dosing challenges, side effects associated with immediate release cysteamine treatment include gastrointestinal distress, nausea and vomiting, beyond those normally experienced as a result of the disease itself, and a socially difficult exhaled rotten egg smell and body odor soon after drug administration, which is especially a burden for children in the middle of the school day. Patients report frequent, concomitant and chronic use of proton-pump inhibitors, or PPIs, to reduce the gastrointestinal distress. We believe that the required dosing regimen coupled with these adverse side effects results in overall poor patient compliance within the cystinosis patient population, with approximately 70% to 80% of patients failing to comply with prescribed dosing.

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

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

Extension Study. All patients who completed our pivotal Phase 3 clinical trial of RP103 for the potential treatment of cystinosis could voluntarily enroll in a planned extension study in which they would continue to be treated with RP103 and make regular clinic visits to monitor white blood cell, or WBC, cystine levels and collect long-term safety and quality of life data. Of the 40 patients who entered the extension study after completing the Phase 3 clinical trial, 38 are currently still enrolled. These 38 patients have now taken RP103 in the extension study for at least 18 months, with some patients having been in the extension study for as long as 30 months. We included a minimum of 12 months of safety data from the 38 Phase 3 completers who elected to enroll in the extension study with our NDA and

MAA filings. We plan to keep the extension study open to all enrolled patients until RP103 becomes commercially available locally.

Based on meeting the primary endpoint in our Phase 3 clinical trial and on the findings of our RP103 bioequivalence study, which demonstrated similar drug exposure whether administered in whole capsule or sprinkled onto applesauce, U.S. and EU regulatory agencies approved our expanded enrollment in the extension study to include patients who did not qualify for the Phase 3 clinical trial. Twenty additional patients have enrolled, including 13 infants and children under six years old using RP103 sprinkled onto applesauce or administered through gastric tube, and 7 patients who have undergone a kidney transplant. Fifty-four cystinosis patients remain in this clinical study.

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

Table of Contents

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

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

In the U.S. and EU, several key legal structures and operational activities have been or are being established. These include creating the EU legal entity and subsidiary structure, defining supply chain strategies, and hiring personnel. Personnel hired include a European general manager of commercial operations, select country managers, medical affairs staff, sales/marketing representatives, market analytics and other health services managers. We anticipate additional hires before the potential launch of RP103. Several key pricing and reimbursement support activities are complete or underway including obtaining feedback from U.S. and EU payors on RP103's value proposition, development of the global value dossier, establishment of a U.S. reimbursement hub (United BioSource Corporation, or UBC) and contracting with national account and reimbursement managers. Commercial demand planning and launch inventory build is underway at our contract manufacturing organization, Patheon Pharmaceuticals, Inc. Our goal for commercial inventory at launch is to have on hand sufficient drug quantities to meet a best-case demand scenario.

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

RP103 for Huntington's disease

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

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

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

Centre Hospitalier Universitaire, or CHU, d'Angers, France, is currently conducting a Phase 2/3 clinical trial of RP103 designed to investigate potential mechanism of cysteamine in HD patients, using BDNF as a biomarker of potential efficacy. The trial commenced in October 2010, with full enrollment in June 2012. Eight clinical sites in France have enrolled 96 patients in a placebo-controlled, 18-month trial, followed by an open label trial with all placebo patients rolling onto RP103 and all non-placebo patients continuing on RP103 for up to another 18 months. The primary endpoint of the trial is change from the baseline of the motor score of the Unified Huntington's Disease Rating Scale, or UHDRS. Blood levels of BDNF are being measured as a secondary endpoint. Under the collaboration agreement with CHU d'Angers, we supply RP103 and placebo capsules for the clinical trial and open-label extension study in exchange for regulatory and commercial rights to the clinical trial results. Clinical expenses of the study are covered by a grant from the French government. Interim results of this study following the first18 months of treatment are expected to be announced in the first half of calendar 2014.

RP103 for NASH

NASH, an advanced form of Non-alcoholic Fatty Liver Disease, or NAFLD, is a progressive liver disease, occurring in 25% of obese people. Approximately 2% to 5% of the U.S. population is afflicted with this disease, which can cause cirrhosis, liver failure and end-stage liver disease. The incidence of NASH is increasing in the U.S. adolescent population. Currently, there is no FDA approved therapeutic options for NASH. The disease is generally managed, if at all, with lifestyle changes such as diet, exercise and weight reduction.

Table of Contents

We believe cysteamine may exert a number of effects for the potential treatment of NASH. First, cysteamine is a potent anti-oxidant, and dietary anti-oxidants, like vitamin E, have been clinically tested in NASH studies. While the endpoint of the vitamin E study was not met, the study provided useful data. Second, cysteamine, through the formation of a cysteamine-cysteine disulfide complex, increases the production of the potent endogenous liver anti-oxidant glutathione, or GSH, and increasing GSH may have the potential to reverse NASH-related liver damage. GSH itself does not enter easily into cells, even when given in large doses. However, the cysteamine-cysteine complex easily enters cells through the lysine transporter and has been shown to be effective in treating certain conditions by preventing significant GSH depletion. Third, cysteamine has been shown to inhibit tissue transglutaminase activity, which is elevated in NASH and may contribute to the formation of fibrotic tissue associated with advanced NASH.

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

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

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

The trial, called Cysteamine Bitartrate Delayed-Release for the Treatment of Non-alcoholic Fatty Liver Disease in Children, or CyNCh, is expected to enroll a total of 160 pediatric participants at ten U.S. centers in the NIDDK-sponsored NASH Clinical Research Network. NIDDK and we are sharing the costs to conduct the CyNCh clinical trial. The primary objective of this randomized, multicenter, double-blind, placebo-controlled Phase 2b clinical trial is to evaluate whether 52 weeks of treatment with RP103 in children reverses damage caused by NASH as measured by changes in NAFLD Activity Score, or NAS, a histological rating scale of disease activity, in conjunction with no worsening of liver fibrosis. Secondary endpoints will include blood markers for liver health including ALT and AST as well as safety and tolerability. We anticipate full enrollment by the second half of 2013 and potential data release in connection with the Phase 2b clinical trial in the second half of calendar 2014.

Other Clinical-Stage Product Candidate

Convivia™ for ALDH2 Deficiency

We are developing Convivia, our proprietary oral formulation of 4-methylpyrazole, or 4-MP, for the potential treatment of acetaldehyde toxicity resulting from ALDH2 deficiency. Sometimes referred to as ethanol intolerance or "Asian flush," ALDH2 deficiency is an inherited metabolic disorder affecting 40% to 50% of East Asian populations. The association of this metabolic disorder with serious health risks, including liver diseases and digestive tract

cancers, has been documented in numerous peer-reviewed studies over the last 10 years. ALDH2 deficiency impairs the activity of the liver enzyme ALDH2, the second enzyme of the primary metabolic pathway for ethanol and other alcohols. The result is an accumulation of acetaldehyde, a carcinogenic intermediate in the metabolism of ethanol, in blood and tissues of affected persons who drink alcoholic beverages. In recurrent drinkers, this disorder has been associated with increased risks of digestive tract cancers and other serious health problems. In addition to these long-term serious health risks, elevated acetaldehyde levels lead to immediate and unpleasant symptoms including facial flushing, tachycardia, or rapid heart rate, headache, nausea and dizziness. We are developing Convivia to potentially lower systemic acetaldehyde levels and reduce symptoms associated with alcohol intake by ALDH2-deficient individuals.

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

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

Table of Contents

Preclinical Product Candidates

Our preclinical programs, for which we are seeking development partners for these 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. In December 2012, we decided to terminate our WntTide™ program based upon recent preclinical study results.

Other Development Areas

Securing Additional and Complementary Technology Licenses from Others

We plan to establish additional research collaborations with prominent universities and research labs and to secure licenses from these universities and labs for technology resulting from the collaborations. No assurances can be made regarding our ability to establish such collaborations over the next 12 months, or at all. We intend to focus our in-licensing and product candidate acquisition activities on identifying complementary therapeutics, therapeutic platforms that offer a number of therapeutic targets, and clinical-stage therapeutics based on existing approved drugs in order to create proprietary reformulations to improve safety and efficacy or to expand such drugs' clinical indications through additional clinical trials. We may obtain these products through collaborations, joint ventures or through merger and/or acquisitions with other biotechnology companies.

Corporate Information

We are incorporated under the laws of the State of Delaware and our business was founded in May 2006. Our principal executive office is located at 9 Commercial Blvd., Suite 200, Novato, CA 94949. Our phone number is (415) 382-8111.

As of February 22, 2013, there were 53,506,604 shares of our common stock outstanding. Our common stock currently trades on the NASDAQ Global Market under the ticker symbol "RPTP."

Corporate History

In July 2009, our subsidiary merged with and into Raptor Pharmaceuticals Corp., a Delaware corporation, or RPC, pursuant to which the stockholders of RPC received shares of our common stock in exchange for their shares of stock and RPC survived as our wholly-owned subsidiary. This merger is referred to herein as the 2009 Merger. Immediately prior to the 2009 Merger, we changed our corporate name from "TorreyPines Therapeutics, Inc." to "Raptor Pharmaceutical Corp." At the time of the 2009 Merger, RPC had two principal subsidiaries, Raptor Discoveries, a development-stage research and development company, and Raptor Therapeutics, which focuses on developing clinical-stage drug product candidates through to commercialization and, in connection with the 2009 Merger, these two subsidiaries became our subsidiaries. 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."

Exercises of Common Stock Options and Common Stock Warrants

During the cumulative period from September 8, 2005 (inception) through February 22, 2013, we received approximately $24.6 million from the exercise of warrants in exchange for the issuance of an aggregate of

approximately 10.1 million shares of our common stock.

During the cumulative period from September 8, 2005 (inception) through February 22, 2013, we received approximately $0.7 million from the exercise of stock options resulting in the issuance of 319,489 shares of our common stock.

Outstanding Common Stock Warrants

As of February 22, 2013, we had the following warrants outstanding related to the assumption of warrants from our Encode merger, issuance of warrants related to our May/June 2008 private placement, issuance of warrants related to our August 2009 private placement, the assumption of warrants pursuant to the 2009 Merger, issuance of warrants related to our December 2009 registered direct offering and issuance of warrants related to our August 2010 private placement. See Note 11 in our consolidated financial statements attached as an exhibit to this Transition Report on Form 10-KT for further discussion regarding our common stock warrants.

Table of Contents

	Number of shares exercisable (in thousands)	Exercise price	Expiration Date
Issued in connection with Encode merger	233	$ 2.87	12/13/2015
Issued to placement agents in May / June 2008	433	$ 2.36	5/21/2013
Issued to placement agents in August 2009	65	$ 1.50	8/21/2014
TorreyPines warrants assumed in 2009 Merger	8	$ 80.86	*6/11/2013 - 9/26/2015
Issued to registered direct investors in Dec. 2009	631	$ 2.45	12/22/2014
Issued to private placement investors in Aug. 2010	2,495	$ 3.075	8/12/2015
Issued to placement agent in Aug. 2010	98	$ 3.075	8/12/2015
Total warrants outstanding	3,963	$ 3.03	*

* Weighted average exercise price

2011 Follow-on Public Offering

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

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

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

Sales in the ATM offering are being made pursuant to the prospectus supplement dated April 30, 2012, as amended by Amendment No. 1 dated June 28, 2012, which supplements our prospectus dated February 3, 2012, filed as part of the shelf registration statement that was declared effective by the SEC on February 3, 2012. Through February 22, 2013 we sold approximately 3.1 million shares under the ATM at a weighted-average selling price of $5.24 per share for net proceeds of approximately $15.9 million.

Proprietary Rights

IP Protection for RP103 for Cystinosis and Other Indications

Our composition and method of use patents.

We have an exclusive worldwide license from UCSD to issued and pending patents covering composition of matter, or COM, method of use, or MOU, and composition 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 European and other countries, represents a COM patent, which covers the composition comprising cysteamine and any material that provides increased delivery to the small intestine and composition comprising enterically coated cysteamine. U.S. Patent No. 8,026,284 (expires 2027), for which applications are pending in European and other countries, represents a MOU patent, which covers method of administering cysteamine composition that increases delivery to small intestine, at a dosing schedule less than four times daily, including two times daily and contains pharmacokinetic claims. European 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.

Table of Contents
Our cysteamine intellectual property to treat metabolic and neurodegenerative conditions.

In addition, our UCSD license includes U.S. Patent No. 7,994,226 and 8,263,662 (expire 2028), MOU patents which covers cysteamine and related compounds for the potential treatment of NASH and NAFLD, respectively. 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 world-wide rights to international patent application PCT/US11/57935, related to cysteamine and related compounds in the potential treatment of tissue fibrosis from the Seattle Children's Research Institute, or SCRI. Researchers at SCRI demonstrated in preclinical studies in mice that daily treatment with cysteamine attenuated renal fibrosis, with up to 25% reduction of extracellular fibrotic material observed over a 21-day study period.

Regulatory Exclusivity

Orphan Drug Designation

We have been granted Orphan Drug Designation from the FDA for use of RP103 to potentially treat cystinosis and the use of cysteamine to potentially treat HD and Batten Disease (although we are not currently working on the development of a drug product candidate for Batten Disease). The Orphan Drug Act of 1983 generally provides incentives, including marketing exclusivity and tax benefits, to companies that undertake development and marketing of products to treat relatively rare diseases, which are defined as diseases for which fewer than 200,000 persons in the U.S. would be likely to receive the treatment. A drug that receives orphan drug status may receive up to seven years of exclusive marketing in the U.S. for that indication (with an additional half year if for a pediatric indication). Our RP103 may receive orphan drug exclusivity if the Office of Orphan Products determines that our enteric formulation of cysteamine bitartrate is "clinically superior" to the approved product by means of greater effectiveness, greater safety, or that it provides a major contribution to patient care; or if the Review Division determines RP103 has comparable efficacy and/or is safer than the approved formulation or provides a major contribution to clinical care.

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

Hatch-Waxman Act

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

Table of Contents

Competition

Cystinosis

We are aware of only one pharmaceutical product currently approved by the FDA and the EMA to treat cystinosis, Cystagon (immediate-release cysteamine bitartrate capsules), is marketed in the U.S. by Mylan Pharmaceuticals, and by Recordati and Orphan Europe in markets outside of the U.S. Cystagon was approved by the FDA in 1994 and by EMA in 1997 and is the standard of care for cystinosis treatment.

While we believe that our RP103 formulation will be well received in the market due to what we believe is reduced dose frequency and improved tolerability, if we receive marketing approval, we anticipate that Cystagon will remain on the market and will compete with our product. We are not aware of any pharmaceutical company with an active program to develop an alternative therapy for cystinosis. There are companies developing and/or marketing products to treat symptoms and conditions related to, or resulting from cystinosis, but none developing products to treat the underlying metabolic disorder (toxic cystine accumulation). Academic researchers in the U.S. and Europe are pursuing potential cures for cystinosis through gene therapy, stem cell therapy, pro-drug and pegylated drug approaches as alternatives to cysteamine bitartrate. We believe that the development timeline to an approved product for these approaches is many years with substantial uncertainty.

Huntington's Disease

We are not aware of any currently available treatment alternatives for HD, although there are products available such as Haldol®, Klonopin® and Xenazine® to treat uncontrollable movements and mood swings that result from the disease. There are several pharmaceutical companies pursuing potential cures and treatments for HD, as well as numerous academic and foundation sponsored research efforts. To our knowledge, our product candidate, RP103, is the only compound in clinical development which specifically targets the fundamental metabolic defect of the disease (deficient brain-derived neurotrophic factor), with the goal of slowing disease progression.

Companies with HD product candidates in development include Eli Lilly & Co. and Pfizer. Several other companies have drug candidates in preclinical development. Additionally, nutritional supplements including creatinine and coenzyme Q10 have been investigated as potential treatments for HD. The Huntington Study Group sponsors numerous studies of potential therapies for HD, including coenzyme Q10 and the antibiotic minocycline.

NASH

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

ALDH2 Deficiency

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

commercial operations in Asian countries, developing products to treat the symptoms of this condition.

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

Government Regulations of the Biotechnology Industry

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

Table of Contents

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

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

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

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

- completion of prerequisite preclinical studies;
- the submission to the FDA of a request for authorization to conduct clinical trials on an investigational new drug application, or IND, which must become effective before clinical trials may commence;
- adequate and well-controlled Phase 1, Phase 2 and Phase 3 clinical trials to establish and confirm the safety and efficacy of a drug candidate;
- submission to the FDA of a new drug application, or NDA, for the drug candidate for marketing approval; and
- review and approval of the NDA by the FDA before the product may be sold commercially.

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

Preclinical testing includes laboratory evaluation and characterization of the safety and efficacy of a drug and its formulation. Preclinical testing results are submitted to the FDA as a part of an IND which must become effective prior to commencement of clinical trials. Clinical trials are typically conducted in three sequential phases following submission of an IND. Phase 1 represents the initial administration of the drug to a small group of humans, either patients or healthy volunteers, typically to test for safety (adverse effects), dosage tolerance, absorption, distribution, metabolism, excretion and clinical pharmacology, and, if possible, to gain early evidence of effectiveness. Phase 2 involves studies in a small sample of the actual intended patient population to assess the efficacy of the drug for a specific indication, to determine dose tolerance and the optimal dose range and to gather additional information relating to safety and potential adverse effects. Once an investigational drug is found to have some efficacy and an

acceptable safety profile in the targeted patient population, Phase 3 studies are initiated to further establish clinical safety and efficacy of the therapy in a broader sample of the general patient population, in order to determine the overall risk-benefit ratio of the drug and to provide an adequate basis for any physician labeling. During all clinical studies, we must adhere to Good Clinical Practice, or GCP, standards. The results of the research and product development, manufacturing, preclinical studies, clinical studies and related information are submitted in an NDA to the FDA.

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

Table of Contents

The rate of completion of any clinical trials will be dependent upon, among other factors, the rate of patient enrollment. Patient enrollment is a function of many factors, including the size of the patient population, the nature of the trial, the availability of alternative therapies and drugs, the proximity of patients to clinical sites and the eligibility criteria for the study. Delays in planned patient enrollment may result in increased costs and delays. We do not know whether our IND for future products or the protocols for any future clinical trials will be accepted by the FDA. We do not know if our clinical trials will begin or be completed on schedule or at all. Even if completed, we do not know if these trials will produce clinically meaningful results sufficient to support an application for marketing approval. The commencement of our planned clinical trials could be substantially delayed or prevented by several factors, including:

- a limited number of, and competition for, suitable patients with particular types of disease for enrollment in clinical trials;
- delays or failures in obtaining regulatory clearance to commence a clinical trial;
- delays or failures in obtaining sufficient clinical materials;
- delays or failures in reaching agreement on acceptable clinical trial agreement terms or clinical trial protocols with prospective sites; and
- delays or failures in obtaining Institutional Review Board, or IRB, approval to conduct a clinical trial at a prospective site.

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

- slower than expected rates of patient recruitment and enrollment;
- failure of patients to complete the clinical trial;
- unforeseen safety issues;
- lack of efficacy during clinical trials;
- inability or unwillingness of patients or medical investigators to follow our clinical trial protocols;
- inability to monitor patients adequately during or after treatment; and
- regulatory action by the FDA for failure to comply with regulatory requirements.

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

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

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

In addition to the regulatory framework for product approvals, we and our collaborative partners must comply with federal, state and local laws and regulations regarding occupational safety, laboratory practices, the use, handling and

disposition of radioactive materials, environmental protection and hazardous substance control, and other local, state, federal and foreign regulations. All facilities and manufacturing processes used by third parties to produce our drug candidates for clinical use in the U.S. must conform with cGMPs. These facilities and practices are subject to periodic regulatory inspections to ensure compliance with cGMP requirements. Their failure to comply with applicable regulations could extend, delay, or cause the termination of clinical trials conducted for our drug candidates or of manufacturing and sale of any of our products approved for sale. We cannot accurately predict the extent of government regulation that might result from future legislation or administrative action.

Table of Contents

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 period from the four months ended December 31, 2012 and September 8, 2005 (inception) to December 31, 2012, we incurred approximately $9.0 million and $69.6 million, respectively, in research and development costs ($21.4 million, $14.8 million and $9.3 million during the fiscal years ended August 31, 2012, 2011 and 2010, respectively).

Operating Segment and Geographic Information

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

Compliance with Environmental Laws

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

Employees

We presently have 41 full time employees and 1 part-time employee. Of the 41 employees, 26 are general and administrative (which includes 13 employees who are focused on preparation for the potential launch of RP103 in the U.S. and the EU) and 15 are involved in research and development. Based on our current plan, over the next 12 month period, we plan to add approximately 15 to 25 people in the following functions: field based sales and medical affairs, commercial, regulatory, clinical, manufacturing, program management, quality and finance. In addition, administrative, regulatory, clinical, commercial and human resources consultants will be used as appropriate.

Facilities

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

Website

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

Available Information

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

Table of Contents
ITEM 1A: RISK FACTORS

(In $ thousands, except as noted or per share data and percentages)

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

Risks Associated with Product Development and Commercialization

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

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

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

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

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

- the results of clinical trials may not meet the level of statistical significance or clinical significance required by the FDA, EMA and/or other regulatory authorities for approval;
- the FDA, EMA or other 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 RP103 has adequate clinical and other benefits and an adequate safety profile; or may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct one or more additional trials;
- the FDA, EMA or other regulatory authorities may not accept data generated at our clinical trial sites;
- the FDA, EMA or other regulatory authorities may have difficulties scheduling an advisory committee meeting (if required) in a timely manner or the advisory committee may recommend against approval of our application or may

recommend that the FDA require, as a condition of approval, additional preclinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;

- the FDA, EMA or other 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;
- the FDA, EMA or other regulatory authorities may impose limitations on approved labeling of RP103 thus introducing reimbursement complications which may limit access for intended users;
- the FDA, EMA or other regulatory authorities may identify deficiencies in the manufacturing processes or in the facilities of our third party contract manufacturers, or may require us to manufacture additional validation batches or change our process or specifications;
- we may not be able to validate our manufacturing process to the satisfaction of the FDA, EMA, or other regulatory authorities, or they may not agree with our plan for concurrent validation; or
- the FDA, EMA or other regulatory authorities may change approval policies or adopt new regulations.

Table of Contents

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

Any of our product candidates, if approved by the FDA, EMA or other regulatory authorities could be subject to labeling and other restrictions or market withdrawal, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with our products.

Any regulatory approvals that we obtain for our product candidates will also be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval. In addition, if the FDA, EMA or other regulatory authorities approve a product candidate, the manufacturing processes, labeling, packaging, distribution, storage, adverse event reporting, dispensation, distribution, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements will include submissions of safety and other post-marketing information and reports, ongoing maintenance of product registration, as well as continued compliance with cGMPs (good manufacturing practices), GCPs (good clinical practices), and GLPs (good laboratory practices). If we do not comply with the applicable regulations and requirements, the range of possible sanctions includes issuance of adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and enforcement actions, including injunctions and civil or criminal prosecution. The FDA and comparable international regulatory agencies can withdraw a product's approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues.

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

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

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

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

Table of Contents

If approved for marketing and sale, pressure on drug product third-party coverage and reimbursement/pricing may impair our ability to be reimbursed for our products, at prices or on terms sufficient to provide a viable financial outcome.

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

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

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

- availability and relative efficacy and safety of therapeutic alternatives;
- timing of market introduction of our products as well as competitive drugs;
- efficacy and safety and real-world patient and physician experience with RP103 for cystinosis;
- identification of currently diagnosed and undiagnosed patients and continued projected growth of the cystinosis market;
- prevalence and severity of any side effects;
- acceptance by patients, patient families, primary care specialists and key specialists;
- potential or perceived advantages or disadvantages of our products compared to alternative treatments, including safety, efficacy, cost of treatment and relative convenience and ease of administration;
- strength of sales, marketing, market access, medical affairs and distribution support;
- the price of our products, both in absolute terms and relative to alternative treatments;

- the effect of current and future healthcare laws;
- availability of coverage and adequate reimbursement and pricing from government and other third-party payors; and
- breadth of product labeling or product insert requirements of the FDA, EMA or other regulatory authorities.

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

Table of Contents

Because the target patient populations for 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 profitability.

Our clinical development of RP103 targets diseases with small patient populations, including cystinosis and HD. A key component of the successful commercialization of a drug product for these indications includes identification of patients and a targeted prescriber base for the drug product. If we are successful in developing RP103 for certain diseases with a small patient population, such as cystinosis or HD, and in obtaining regulatory approval to market RP103 for such diseases, we will need to identify patients and market RP103 for these indications in the U.S. and Europe, at a minimum, to achieve significant market penetration. In addition, the per-patient prices at which we sell RP103 for these indications will need to be relatively high in order for us to generate an appropriate return for the investment in these product development programs and achieve meaningful profitability. There can be no assurance that we will be successful in identifying patients and/or obtaining high per-patient prices for our product candidates that target diseases with small patient populations.

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

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

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

Sales of our product candidates outside of the U.S. will be subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approvals. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries, including the EMA must also approve the manufacturing and marketing of our product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the U.S., including additional preclinical studies or clinical trials or manufacturing and control requirements. In many countries outside the U.S., a product candidate must be approved for reimbursement before it can be approved for sale in that country. In many cases, the price that we propose to charge for our products is also subject to approval by individual countries before we can launch our product candidates in those countries. Obtaining foreign regulatory approvals, 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.

Table of Contents

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

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

Several provisions of the new law, which have varying effective dates, may affect us, including our costs. For example, the PPACA increased the Medicaid rebate rate, revised the definition of "average manufacturer price" for reporting purposes, which could further increase the amount of the Medicaid drug rebates paid to states, and extended the rebate program to beneficiaries enrolled in Medicaid managed care organizations. The PPACA also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or "donut hole." The law also established an annual non-deductible fee on entities that sell branded prescription drugs or biologics to specified government programs in the U.S. The PPACA includes a provision to increase the Medicaid rebate for line extensions or reformulated drugs (NDA Type 3) priced higher than the original drug. Depending on the final regulations this could substantially increase our Medicaid rebate rate (in effect limiting reimbursement for these patients) if we participate in the Medicaid Rebate Program. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with healthcare practitioners. Although the U.S. Supreme Court recently upheld most of the PPACA, it remains unclear whether there will be any changes made to certain provisions of PPACA through acts of Congress at some point in the future. In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, the President signed into law the Budget Control Act of 2011, which may result in such changes as aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. The full impact on our business of PPACA and the Budget Control Act is uncertain. We cannot predict whether other legislative changes will be adopted, if any, or how such changes would affect the pharmaceutical industry generally.

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

The laws that may affect our ability to operate include:

- the federal healthcare programs' Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs;
- federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;
- federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information;
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; and
- State reporting requirements detailing interactions with and payments to healthcare providers.

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

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

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

Table of Contents

Following successful preclinical testing, drug product candidates will need to be tested in a clinical development program to provide data on safety and efficacy prior to becoming eligible for product approval and licensure by regulatory agencies. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful. The clinical trial process may fail to demonstrate 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 our investigational new drug application, or IND, and NDA as applicable, with the FDA and, ultimately, our ability to commercialize our drug product candidates and generate product revenues. In addition, some of our clinical trials will involve small patient populations. Because of the small sample size, the results of these early clinical trials may not be indicative of future results. The failure to demonstrate safety or efficacy in our clinical trials would have a material adverse effect on our future business prospects, financial condition and operating results.

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

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

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

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

be available on acceptable economic terms, if at all.

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

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

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

Table of Contents

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

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

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

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

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

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

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

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

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

Specifically, we have and will continue to rely on third parties, such as contract research organizations and/or co-operative groups, to assist us in overseeing and monitoring clinical trials as well as to process the clinical results.

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

Table of Contents

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

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

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

Our business could be adversely affected by macroeconomic conditions.

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

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

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

We will need to establish relationships with additional leading scientists and research institutions. We believe that such relationships are pivotal to establishing products using our technologies as a standard of care for various

indications. Although we have established a Medical and Scientific Advisory Board and research collaborations, there is no assurance that our Advisory Board members and our research collaborators will continue to work with us or that we will be able to attract additional research partners. If we are not able to maintain existing or establish new scientific relationships to assist in our research and development, we may not be able to successfully develop our drug product candidates.

Table of Contents

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

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

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

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

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

In the future, we may request six month priority review from the FDA and EMA for RP103 for HD and our other drug product candidates; however, the FDA and EMA may not grant it. Without priority review, the FDA and EMA review timeline could be at least 10 to 12 months. Under the FDA policies, a drug candidate is eligible for priority review from the time a complete NDA is accepted for filing, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A lengthier review process will delay revenue from the sale of products and will increase the capital necessary to fund these product development programs.

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

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

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

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

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

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

Table of Contents

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

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

Our success is dependent in part upon the availability of our senior executive officers, including Christopher M. Starr, Ph.D., chief executive officer; Julie Anne Smith, chief operating officer; Georgia Erbez, chief financial officer; Ted Daley, chief business officer and Patrice Rioux, M.D., Ph.D., chief medical 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 operating results. We have no key-man insurance on any of our employees.

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

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

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

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

If we do not meet the milestones as publicly announced (or as projected by various 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.

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

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

Table of Contents

In addition, the Sarbanes-Oxley Act requires, among other things, that we maintain effective internal control over financial reporting and disclosure controls and procedures. In particular, we must perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. Our compliance with Section 404 will require that we incur substantial accounting and related expense and expend significant management efforts. In the future, we may need to hire additional accounting and financial staff to satisfy the ongoing requirements of Section 404. Moreover, if we are not able to comply with the requirements of Section 404, or we or our independent registered public accounting firm identifies deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by NASDAQ, the SEC or other regulatory authorities.

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

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

Our loan agreement with HC Royalty contains a number of restrictive covenants and other provisions, which, if violated, could result in the acceleration 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 HealthCare Royalty Partners, or HC Royalty, as lender, under which we agreed to borrow $50 million in two $25 million tranches, or the HC Royalty Loan, and we have drawn the first tranche in the amount of $25 million. Our 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 our obligations under the HC Royalty Loan, we granted a security interest to HC Royalty in substantially all of our assets, the assets of our subsidiaries and a pledge of stock of certain of our 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 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. Further, HC Royalty may terminate its commitment to fund the second $25 million tranche upon the occurrence of any such event prior to the funding of such tranche.

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.

Table of Contents

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 much practical protection. If we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent.

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

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

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

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

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

Table of Contents

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

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

UCSD and Yeda may terminate their respective agreements with us upon the occurrence of certain events, including if we enter into certain bankruptcy proceedings or if we materially breach our payment obligations and fail to remedy the breach within the permitted cure periods. Although we are not currently involved in any bankruptcy proceedings or in breach of these agreements, there is a risk that we may be in the future, giving UCSD and Yeda the right to terminate their respective agreements with us. We have the right to terminate these agreements at any time by giving prior written notice. If the UCSD or Yeda agreements are terminated by either party, we would lose our rights to RP103 in the case of UCSD and would lose our rights to the Weizmann and Niigata patents in the case of Yeda. Under such circumstances, we would have no further right to use or exploit the patents, copyrights or trademarks in those respective technologies. If this happens, we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected, and we may have to cease our operations.

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

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

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

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

We expect that the technologies associated with biotechnology research and development will continue to develop rapidly. Our future success will depend in large part on our ability to maintain a competitive position with respect to these technologies. Rapid technological development, as well as new scientific developments, may result in our

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

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

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

Table of Contents

Risks Related to Our Financial Position and Capital Requirements

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

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

- conduct research, preclinical testing and human studies and clinical trials;
- establish or contract for pilot scale and commercial scale manufacturing processes and facilities;
- market and distribute our products; and
- establish and develop quality control, 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 efforts to commercialize our products, if approved, the success of our research initiatives, regulatory approvals, the timing of events outside our direct control such as negotiations with healthcare payors, potential strategic partners and other factors. In additional, certain product programs may require collaborative agreements with corporate partners with substantial assets and organizations to help with the very substantial funds required and the complex organizational resources required. Such agreements may require substantial time to complete and may not be available in the time frame desired, with acceptable financial terms, if at all. Any of these uncertain events can significantly change our cash requirements as they determine such one-time events as the receipt or payment of major milestones and other payments.

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

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

As of December 31, 2012, we had an accumulated deficit of $135.9 million. We expect to continue to incur losses for the foreseeable future and will have to raise substantial cash to fund our planned operations. Our recurring losses from operations to date raise substantial doubt about our ability to continue as a going concern and, as a result, our

independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the four month period ended December 31, 2012, with respect to this uncertainty. We will need to raise additional capital and/or generate significant revenue at profitable levels to continue to operate as a going concern. In addition, the perception that we may not be able to continue as a going concern may cause others to choose not to deal with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

We believe our cash, cash equivalents and short-term investments as of December 31, 2012 of $58.4 million, including $23.4 million we received with respect to the first tranche amount drawn down under our HC Royalty Loan in December 2012 will be sufficient to meet our projected operational requirements and obligations into the fourth quarter of calendar 2013.

In addition, under the HC Royalty Loan, HC Royalty has agreed to lend us $25 million in a second tranche, provided that we have received FDA approval for RP103 for the treatment of cystinosis and other funding conditions are satisfied. There can be no assurance that we will receive such FDA approval or that such other conditions will be satisfied and, accordingly, there can be no assurance that we will be able to draw on the second $25 million tranche under the HC Royalty Loan.

Table of Contents

In the future, we may need to sell equity or debt securities to raise additional funds to support, among other things, our development and commercialization programs. The sale of additional equity securities or convertible debt securities will result in additional dilution to our stockholders. Additional financing may not be available in amounts or on terms satisfactory to us or at all. We may be unable to raise additional capital due to a variety of factors, including our financial condition, the status of our research and development programs, the status of regulatory reviews for marketing approvals, the status of our commercialization activities, the execution of our potential launch of RP103 for cystinosis and the general condition of the financial markets. If we fail to raise additional financing when needed, we may have to delay or terminate some or all of our research and development programs, scale back our operations and/or reduce our pre-launch/launch expenses for RP103. If such actions are required, our financial condition and operating results will be adversely affected and our current value and potential future value may be significantly reduced.

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 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 variable rate based on the amount of included product payments in a calendar year, and such interest is payable quarterly. Included product payments are the net revenues of our Company and our subsidiaries from existing and future products. Principal payments under the HC Royalty Loan will become due beginning on the ninth quarterly payment date occurring after the date the second $25 million tranche is funded (if at all) and, in the case of the first tranche loan, in no event later than March 31, 2017.

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, on satisfactory terms or at all. In addition, the terms of the HC Royalty Loan may limit our ability to pursue any of these alternatives and these alternative measures may not be successful and 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, 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. This could have a material adverse impact on our financial condition and results of operations.

Risks Related to Our Common Stock

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

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

raise additional capital at all.

As of December 31, 2012, there were (i) outstanding warrants to purchase 4,562,772 shares of our common stock at a weighted-average exercise price of $3.03 per share, (ii) outstanding options to purchase 7,641,585 shares of our common stock outstanding under our 2010 and 2006 Raptor stock option plans at a weighted-average exercise price of $4.42 (of which 44% was vested), (iii) options to purchase 149,209 shares of our common stock outstanding under our TorreyPines Therapeutics stock option plans at a weighted-average exercise price of $75.83 and (iv) 1,947,420 shares of our common stock available for future stock option grants to be issued under our 2010 Raptor stock option plan and would be available for exercise when vesting conditions in the grants are satisfied. The shares issuable upon exercise of stock options granted under our stock option plans will be available for immediate resale in the public market. The shares issuable upon the exercise of our warrants will be available for immediate resale in the public market. The market price of our common stock could decline as a result of such exercises due to the increased number of shares available for sale in the market.

Our named executive officers and our board of directors own, in the aggregate, 925,247 shares, and approximately 2.3 million vested stock options representing approximately 6% beneficial ownership of our outstanding common stock as of December 31, 2012. Sales of a substantial number of shares of our common stock by such officers and directors in the public trading market, whether in a single transaction or a series of transactions, or the perception that these sales may occur, could also have a significant effect the trading price of our common stock.

Table of Contents

In addition, we have an at-the-market sales agreement with Cowen and Company which, as of December 31, 2012, allows us to sell up to an additional $26.2 million worth of stock, which, if utilized further, will create substantial dilution for our existing stockholders.

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

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

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

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

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

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

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 biopharmaceutical development stage companies such as ours have been extremely volatile. Market prices for securities of pre-commercial pharmaceutical, biotechnology and other life sciences companies have historically been particularly volatile and trading volume in such securities has often been relatively small. Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. The stock market also has periods during which industry segments, such as biotechnology, are in volatile swings of greater or lesser favor as investments. These swings in the investment in a sector (periods of net sales or purchases of equity securities) will directly affect the stock prices of many companies in the sector and, in particular, those companies that do not have conventional measures of financial and business health such as sales, earnings, growth rates, profitability and other measures.

Table of Contents

These broad market fluctuations during which our stage of company and our industry is not in favor in the markets or equity investments are relatively less favorable, 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.

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

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

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law as currently in effect may make a change in control of our Company more difficult, even if a change in control may be beneficial to the stockholders. Our board of directors has the authority to issue up to 15,000,000 shares of preferred stock, none of which are issued or outstanding. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Our charter contains provisions that may enable our management to resist an unwelcome takeover attempt by a third party, including: a prohibition on actions by written consent of our stockholders; the fact that stockholder meetings must be called by our board of directors; and provisions requiring stockholders to provide advance notice of proposals. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, the board of directors approves the transaction. Our board of directors may use these provisions to prevent changes in the management and control of our Company. Also, under applicable Delaware law, our board of directors may adopt additional anti-takeover measures in the future.

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

ITEM 1B: UNRESOLVED STAFF COMMENTS

None.

ITEM 2: PROPERTIES

We lease office and laboratory space as our headquarters in Novato, California. Effective November 1, 2012, the monthly base rent and operating expenses will be $20. The Novato lease expires on March 31, 2013, after which we will continue leasing on a month-to-month basis. In October 2012, we entered into a one-year lease for administrative offices (which we had been leasing for the past 20 months) in San Mateo, California. We also rent a small office in France for our French country manager. For the four months ended December 31, 2012 and the fiscal year ended

August 31, 2012, our total office/laboratory rental expense was approximately $86 and $241, respectively.

We are currently in negotiations to expand or relocate our Novato headquarters to accommodate current and future hiring needs prior to the expiration of our existing Novato lease. In addition, we may lease office space in the Netherlands for our European sales and marketing headquarters within the next 12 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.

Table of Contents

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 22, 2013, there were 53,506,604 shares of our common stock outstanding. There is no public trading market for our warrants. The closing price for our common stock on February 22, 2013 was $5.16.

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
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 22, 2013, there were approximately 228 holders of record of our common stock and 53,506,604 shares of our common stock outstanding. Additionally, on such date, options held by 89 persons to acquire up to, in the aggregate, 7,805,794 shares and warrants held by 23 persons to acquire up to, in the aggregate, 3,962,772 shares of our common stock, were outstanding.

Dividends

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

Purchase of Equity Securities and Affiliated Purchasers

We have not repurchased any shares of our common stock since inception. We did not issue any unregistered equity securities during the four months ended December 31, 2012.

Table of Contents
Performance Graph

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

The following graph shows the value of an investment of $100 on September 30, 2009 (date we effected our 2009 Merger) in our common stock, the NASDAQ Composite Index (U.S.) and the NASDAQ Biotechnology Index. All values assume reinvestment of the pretax value of dividends paid by companies included in these indices and are calculated as of August 31st of each year and for the 4 months ended December 31, 2012. Our common stock is traded on the NASDAQ Global Market. The comparisons shown in the graph are based upon historical data and we caution that the stock price performance shown in the graph is not indicative of, nor intended to forecast, the potential future performance of our stock.

	September 30, 2009	August 31, 2010	2011	2012	December 31, 2012
Raptor Pharmaceutical Corp.	$ 100	$90.3	$143.33	$150.61	$177.27
NASDAQ U.S. Composite Index	100	100.54	124.79	152.73	151.07
NASDAQ Biotechnology Index	100	96.73	119.13	168.82	170.41

Table of Contents

ITEM 6: SELECTED FINANCIAL DATA

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

		For the period ending August 31,					
(in millions, except per share data)	For the four months ended December 31, 2012	2012	2011	2010	2009	2008	For the period September 8, 2005 (Inception) to December 31, 2012
Income Statement Data:							
Revenues	$ 0	$ 0	$ 0	$ 0	$ 0	$ 0	$ 0
Operating expenses:							
General and administrative	9.0	14.7	6.2	3.7	2.7	2.2	40.6
Research and development	8.9	21.4	14.8	9.3	6.5	5.8	69.5
Total operating expenses	17.9	36.1	21.0	13.0	9.2	8.0	110.1
Loss from operations	(17.9)	(36.1)	(21.0)	(13.0)	(9.2)	(8.0)	(110.1)
Interest income	0.2	0.3	0.1	0	0	0.1	0.9
Interest expense	(0.1)	0	0	0	0	(0.1)	(0.2)
Foreign currency transaction gain	0.1	0.2	0	0	0	0	0.3
Realized loss on short-term investments	0.0	0.2	0	0	0	0	0.2
Unrealized gain on short-term investments	(0.1)	0	0	0	0	0	(0.1)
Adjustment to fair value of common stock warrants	(1.5)	(3.2)	(16.3)	(5.9)	0	0	(26.9)
Net loss	(19.3)	(38.6)	(37.2)	(18.9)	(9.2)	(8.0)	(135.9)
Other comprehensive loss:							
Foreign currency translation adjustment	(0.1)	(0.1)	0	0	0	0	(0.2)
Comprehensive loss	$ (19.4)	$ (38.7)	$ (37.2)	$ (18.9)	$ (9.2)	$ (8.0)	$ (136.1)
Net loss per share:							
Basic and diluted	$ (0.4)	$ (0.80)	$ (1.15)	$ (0.85)	$ (0.64)	$ (0.81)	
Weighted-average shares outstanding used to compute:							
Basic and diluted	51.7	48.1	32.3	22.2	14.4	9.9	
(in millions)	12/31/12	8/31/12	8/31/11	8/31/10	8/31/09	8/31/08	
Balance Sheet Data:							
	$ 58.4	$ 38.9	$ 15.2	$ 17.0	$ 3.7	$ 7.5	

Cash, cash equivalents and short-term investments						
Working capital (deficit)	37.0	20.6	(11.0)	(0.3)	2.7	6.7
Total assets	68.1	48.3	22.6	24.4	6.6	10.6
Common stock warrant liability	16.4	17.3	23.6	15.8	0	0
Note payable	25.0	0	0	0	0	0
Total liabilities	48.2	21.6	26.7	17.6	1.1	1.0
Accumulated deficit	(135.9)	(116.6)	(78.0)	(40.8)	(21.9)	(12.7)
Total stockholders' equity (deficit)	19.9	26.7	(4.1)	6.8	5.5	9.6

Table of Contents

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

	(In millions, except per share data, unaudited)	
	November 30, 2012	Fiscal Year changed to 12/31
Quarterly Data 2013:		
Net loss	$(13.4)	n/a
Net loss per share, basic and diluted	$(0.26)	n/a

	November 30, 2011	February 29, 2012	May 31, 2012	August 31, 2012
Quarterly Data 2012:				
Net loss	$(11.4)	$(14.0)	$(3.0)	$(10.2)
Net loss per share, basic and diluted	$(0.25)	$(0.29)	$(0.06)	$(0.20)

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

	November 30, 2009	February 28, 2010	May 31, 2010	August 31, 2010
Quarterly Data 2010:				
Net loss	$(2.9)	$(4.2)	$(7.5)	$(4.4)
Net loss per share, basic and diluted	$(0.16)	$(0.19)	$(0.33)	$(0.17)

Table of Contents

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, 2012, and the notes to such consolidated financial statements included elsewhere in this Transition Report on Form 10-KT. 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 Transition Report on Form 10-KT, particularly under the heading "Risk Factors."

Change in Fiscal Year End

On December 4, 2012, our board of directors approved a change to 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 an emerging biopharmaceutical company focused on developing and commercializing life-altering therapeutics that treat debilitating and often fatal diseases. Our initial focus is on developing our first product candidate, RP103, as a potential treatment for cystinosis, a rare genetic disorder. Cystinosis patients are at very high risk of experiencing life-threatening metabolic disorders, including kidney failure, severe gastrointestinal dysfunction and rickets as a result of an accumulation of the amino acid, cystine, in cells. As a result, cystinosis patients have a substantially reduced life span relative to unaffected individuals.

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

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

Clinical Development Programs

Our three active clinical development programs utilize the same active pharmaceutical ingredient, cysteamine bitartrate. Cysteamine bitartrate was approved in the U.S. in 1994 and the EU in 1997 as an orally available immediate-release powder in a capsule for the treatment of, and is the current standard of care for, cystinosis. We are reformulating cysteamine bitartrate to potentially improve the dose administration, safety and/or efficacy compared to existing treatment and repurposing cysteamine bitartrate for potential applications in new disease indications. Our proprietary extended and delayed-release formulation, RP103, is a capsule containing enteric coated micro-beads of cysteamine bitartrate. We believe RP103 will require less frequent dosing and could reduce gastro-intestinal and other side effects compared to immediate-release cysteamine bitartrate for cystinosis patients. In addition to cystinosis, we

are also testing RP103 for the potential treatment of NASH and HD. We have an exclusive worldwide license to delayed-release cysteamine bitartrate from the University of California, San Diego, or UCSD, which is the basis for our proprietary formulation of cysteamine.

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 for these programs include our cysteamine dioxygenase, or ADO, program, to improve treatment of diseases for which cysteamine is therapeutic and our HepTide™ program, for the potential treatment of hepatocellular carcinoma and other cancers susceptible to induced lysosomal storage. In December 2012, we decided to terminate our WntTide™ program based upon recent preclinical study results.

Table of Contents

Future Activities

Over the next fiscal year, we plan to conduct research and development and general and administrative activities including: pre-commercial launch preparation of RP103 for the treatment of cystinosis in the U.S. and EU (including preparing commercial materials and coordinating drug supply) and, if approved by applicable regulatory authorities, conducting a commercial launch of RP103 in the U.S. and EU; supporting our ongoing extension study of RP103 in cystinosis until patients are converted onto commercial drug; conducting other supporting clinical studies of RP103 in cystinosis; supplying clinical material for our ongoing clinical trial of RP103 in HD; funding the collaboration and supplying clinical material in our ongoing Phase 2b clinical trial of RP103 in NASH; continuing business development of our preclinical product candidates; conducting research and development activities for in-licensed and newly discovered preclinical assets; supporting potential clinical trials of RP103 in malaria, fibrosis and Parkinson's disease (subject to potential external funding); and supporting associated facilities and administrative functions.

We plan to seek additional business development partners 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.

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

Use of Estimates

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

Functional Currency

Our consolidated functional currency is the U.S. dollar. Raptor Pharmaceuticals Europe B.V., or BV, Raptor Pharmaceuticals France SAS, or SAS, and RPTP European Holdings C.V., or CV, our European subsidiary, French subsidiary and Cayman-based subsidiary, respectively, use the European Euro as their functional currency. At each quarter end, balance sheets of BV, SAS and CV are translated into U.S. dollars based upon the quarter-end exchange rate, while their statements of comprehensive loss are translated into U.S. dollars based upon an average of the Euro's value between the beginning and end date of the reporting period. Additionally, the equity accounts for BV, SAS and CV are adjusted for any translation gain or loss.

Fair Value of Financial Instruments

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

length of maturity or interest rates that approximate prevailing market rates unless otherwise disclosed in our consolidated financial statements. The warrant liability is carried at fair value which is determined using the Black-Scholes option valuation model at the end of each reporting period.

Cash and Cash Equivalents

We consider all highly liquid investments with original maturities of three months or less, when purchased, to be cash equivalents. We maintain cash and cash equivalents, which consist principally of money market funds with high credit quality financial institutions. Such amounts exceed Federal Deposit Insurance Corporation insurance limits. Restricted cash represents compensating balances required by our U.S. and European banks as collateral for credit cards.

Short-term Investments

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

Table of Contents

Prepaid Expenses and Other

Prepaid expenses and other consists primarily of advance payments to vendors that are due within one year.

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, which consists of our loan agreement with HealthCare Royalty Partners, or HC Royalty, as lender, under which we agreed to borrow $50 million in two $25 million tranches, or the HC Royalty Loan, and we have drawn the first tranche in the amount of $25 million, is stated at the borrowed amount as of December 31, 2012. The loan bears interest at an annual fixed rate of 10.75%, and quarterly interest payments are included in interest expense in our Consolidated Statements of Comprehensive Loss for the four month period ended December 31, 2012. Principal payments, when made, reduce our note payable balance. There is a synthetic royalty component based on sales of products in a calendar year, and such royalty is payable quarterly. As of December 31, 2012, there were no royalty payments since we had no approved products that generate revenue. Upon regulatory approval, if at all, of RP103 for cystinosis, such synthetic royalty will be due to HC Royalty.

Debt issuance costs, which were capitalized and included in other long-term assets, are being amortized over the life of the loan using the interest method. The amortization of debt issuance costs is included in interest expense in our Consolidated Statements of Comprehensive Loss for the four month period ended December 31, 2012.

Intangible Assets

Intangible assets include the intellectual property and other rights relating to DR Cysteamine (currently developed as RP103) and to an out-license acquired in the 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. The intangible assets related to the out-license are amortized using the straight-line method over the estimated useful life of 16 years, which is the life of the intellectual property patents.

Goodwill

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

Fixed Assets

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

Impairment of Long-Lived Assets

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

As of August 31, 2012, we determined that the capitalized acquired in-process research and development cost of $900, representing the tezampanel and NGX 426 program acquired in our 2009 Merger, was impaired due to our decision to discontinue development of this product candidate for thrombosis due to regulatory hurdles that would require significant expenditures which we chose not to prioritize for funding. As such, we expensed $900 as in-process research and development as part of research and development expense on our consolidated statements of comprehensive loss for the year ended August 31, 2012. During the four month period ended December 31, 2012, we did not identify any such impairment losses.

Table of Contents

Common Stock Warrant Liabilities

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

Income Taxes

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

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

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

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

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

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

Research and Development

We are a development stage biotechnology company. Research and development costs are charged to expense as incurred. Research and development expenses include medical, clinical, regulatory and scientists' salaries and benefits,

lab collaborations, preclinical studies, clinical trials, clinical trial materials, commercial drug manufacturing prior to obtaining marketing approval, regulatory and clinical consultants, lab supplies, lab services, lab equipment maintenance and small equipment purchased to support the research laboratory, amortization of intangible assets and allocated administrative expenses. Research and development expenses are offset by contra-expenses, which are reimbursements of research and development expenses received either from research collaborators or from government grants or tax rebates.

In-Process Research and Development

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

Table of Contents

Comprehensive Loss

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

Stock-Based Compensation

In February 2010, our board of directors approved, and in March 2010 our stockholders approved, our 2010 Equity Incentive Plan, as amended, or the 2010 Plan, to grant up to an aggregate of 3,000,000 stock options or restricted stock or restricted stock units over the ten year life of the 2010 Plan which aggregate number was increased up to 6,000,000 stock options through an amendment to the 2010 Plan in April 2011. Our board of directors has determined not to make any new grants under any of our former plans, but rather under the 2010 Plan. The 2010 Plan allows for the granting of options to employees, directors and consultants. As of December 31, 2012, options to purchase 7,790,794 shares of our common stock were outstanding and 1,947,420 shares of our common stock remain available for future issuance under the 2010 Plan. The 2010 Plan allows for 50% accelerated vesting of unvested stock options upon a change of control as defined in the 2010 Plan. The award agreement, subject to the terms of any applicable employment agreement, extends the termination date of the awards granted under the 2010 Plan that are vested as of such termination date due to (a) an employee's or a non-employee director's retirement at age 62 or older which employee or non-employee director has at least five (5) years of continuous service with us prior to such retirement, (b) the termination of a non-employee director's board membership for reasons other than for cause or retirement and (c) an employee's or a non-employee director's death (during his or her continuous service with us or within 90 days' of such continuous service with us) or permanent disability, to eighteen (18) months from the date of termination of continuous service with us.

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

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

In March 2005, the FASB issued ASC Topic 718 (previously listed as Staff Accounting Bulletin No. 107), which offers guidance for what was previously referred to as SFAS 123(R). ASC Topic 718 was issued to assist preparers by simplifying some of the implementation challenges of SFAS 123(R) while enhancing the information that investors receive. ASC Topic 718 creates a framework that is premised on two overarching themes: (a) considerable judgment

will be required by preparers to successfully implement SFAS 123(R), specifically when valuing employee stock options; and (b) reasonable individuals, acting in good faith, may conclude differently on the fair value of employee stock options. Key topics covered by ASC Topic 718 include valuation models, expected volatility and expected term.

For the one and four month periods ended December 31, 2012, stock-based compensation expense was based on the Black-Scholes option-pricing model assuming the following: risk-free interest rate of 0.68% and 0.7%, respectively; five year expected life; 95% volatility; 2.5% turnover rate; and 0% dividend rate.

We based our Black-Scholes inputs on the following factors: the risk-free interest rate was based upon our review of current constant maturity treasury bill rates for 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 factors change and different assumptions are employed in the application of ASC Topic 718, the compensation expense recorded in future periods may differ significantly from what was recorded in the current period. See Note 8 of our consolidated financial statements for a further discussion of our accounting for stock-based compensation.