

Raptor Pharmaceutical Corp  
Form 8-K  
February 05, 2010

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K  
CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): February 5, 2010

RAPTOR PHARMACEUTICAL CORP.

(Exact name of registrant as specified in its charter)

Delaware	000-25571	86-0883978
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

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

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

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))



EXPLANATORY NOTE #1

As discussed in certain of our reports, proxy statements, prospectuses and other documents filed with or furnished to the Securities and Exchange Commission, or the SEC, the first of which was our Current Report on Form 8-K filed with the SEC on July 28, 2009, we and ECP Acquisition, Inc., a Delaware corporation, our then-wholly-owned subsidiary, herein referred to as merger sub, entered into an Agreement and Plan of Merger and Reorganization, herein referred to as the 2009 Merger Agreement, with Raptor Pharmaceuticals Corp., a Delaware corporation on July 27, 2009. On September 29, 2009, on the terms and subject to the conditions set forth in the 2009 Merger Agreement, pursuant to a stock-for-stock reverse triangular merger, herein referred to as the Merger or the 2009 Merger, merger sub was merged with and into Raptor Pharmaceuticals Corp. and Raptor Pharmaceuticals Corp. survived such 2009 Merger as our wholly-owned subsidiary. Immediately prior to such 2009 Merger and in connection therewith, we effected a 1-for-17 reverse stock split of our common stock and changed our corporate name from “TorreyPines Therapeutics, Inc.” to “Raptor Pharmaceutical Corp.”

As a result of the 2009 Merger and in accordance with the 2009 Merger Agreement, each share of Raptor Pharmaceuticals Corp.’s common stock outstanding immediately prior to the effective time of the 2009 Merger was converted into the right to receive 0.2331234 shares of our common stock, on a post 1-for-17 reverse-split basis. Each option and warrant to purchase Raptor Pharmaceuticals Corp.’s common stock outstanding immediately prior to the effective time of the 2009 Merger was assumed by us at the effective time of the 2009 Merger, with each share of such common stock underlying such options and warrants being converted into the right to receive 0.2331234 shares of our common stock, on a post 1-for-17 reverse split basis, rounded down to the nearest whole share of our common stock. Following the 2009 Merger, each such option or warrant has an exercise price per share of our common stock equal to the quotient obtained by dividing the per share exercise price of such common stock subject to such option or warrant by 0.2331234, rounded up to the nearest whole cent.

Immediately following the effective time of the 2009 Merger, Raptor Pharmaceuticals Corp.’s stockholders (as of immediately prior to such 2009 Merger) owned approximately 95% of our outstanding common stock and our stockholders (as of immediately prior to such 2009 Merger) owned approximately 5% of our outstanding common stock.

Raptor Pharmaceuticals Corp., our wholly-owned subsidiary, was the “accounting acquirer,” and for accounting purposes, we were deemed as having been “acquired,” in the 2009 Merger. The board of directors and officers that managed and operated Raptor Pharmaceuticals Corp. immediately prior to the effective time of the 2009 Merger became our board of directors and officers. Additionally, following the effective time of the 2009 Merger, the business conducted by Raptor Pharmaceuticals Corp. immediately prior to the effective time of the 2009 Merger became primarily the business conducted by us.



EXPLANATORY NOTE #2

We are filing this Current Report on Form 8-K which will be used as the Company's 2010 Annual Report to stockholders in order to disclose information appropriate for an annual report with respect to both of the Company and Raptor Pharmaceuticals Corp., herein referred to as RPC.

Unless otherwise mentioned or unless the context requires otherwise, subject to the Notes in the immediately subsequent paragraphs, all references in this Current Report on Form 8-K to "we," "us," "our," the "Company," "Raptor" and similar references refer to the public company formerly known as TorreyPines Therapeutics, Inc. and now known as Raptor Pharmaceutical Corp., including its wholly-owned direct and indirect subsidiaries (which includes RPC, TPTX, Inc., Raptor Discoveries Inc. (f/k/a Raptor Pharmaceutical Inc.) and Raptor Therapeutics Inc. (f/k/a Bennu Pharmaceutical Inc.)), following the name change and completion of the 2009 Merger. All references to "TorreyPines" refer to TorreyPines Therapeutics, Inc., prior to the name change and the completion of the 2009 Merger. Unless otherwise mentioned or unless the context requires otherwise, all discussions in this Current Report on Form 8-K regarding our business includes the programs of the combined business of Raptor Pharmaceutical Corp., including its wholly-owned direct and indirect subsidiaries. Unless otherwise mentioned or unless the context requires otherwise, all discussions in this Current Report on Form 8-K regarding our common stock, our stock price, and our stock options and warrants to purchase our common stock have been converted to their equivalent post-2009 Merger number of shares and equivalent post-2009 Merger stock prices and exercises prices.

Note to Items 6 (Selected Financial Data) and 8 (Financial Statements and Supplementary Data) of Part II:

Notwithstanding the immediately preceding paragraph, and although references in Items 6 and 8 of Part II of this Current Report on Form 8-K to "we," "us," "our," the "Company," "Raptor" and similar references and words of similar import with respect to (i) the unaudited financials statements (and notes thereto) refer to the Company and (ii) the audited financials statements (and notes thereto) refer to Raptor Pharmaceuticals Corp., because Raptor Pharmaceuticals Corp. was the "accounting acquirer," and for accounting purposes, the Company was deemed as having been "acquired," in the 2009 Merger, the consolidated financial information set forth in Items 6 and 8 of Part II of this Current Report on Form 8-K does reflect the consolidated financial information of the combined company, and all references herein to "condensed consolidated financial statements," "consolidated financial statements," "consolidated position" and similar references and words of similar import does reflect the consolidated information of the combined company.

Note to Item 7 (Management's Discussion and Analysis of Financial Condition and Results of Operations) of Part II:

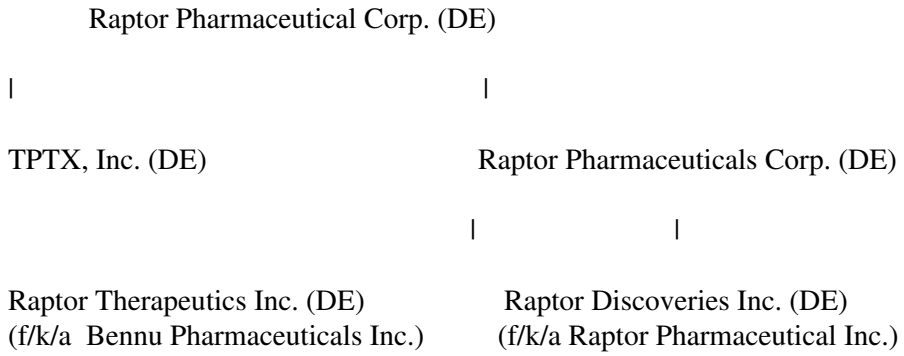
Although the section titled, "Results of Operations – Years ended August 31, 2009 and 2008" and "Results of Operations – Years ended August 31, 2008 and 2007" in Item 7 of Part II of this Current Report on Form 8-K refers to events, circumstances, dates and periods of time that occurred prior to the effective time of the 2009 Merger, and therefore refer to Raptor Pharmaceuticals Corp., such information is generally applicable as supporting the consolidated information of the combined company.

Note to Part III:

Certain sections of Part III of this Current Report on Form 8-K refer to events, circumstances, dates and periods of time that occurred prior to the effective time of the 2009 Merger and refer to each of the Company and to RPC. Such information is generally applicable as supporting the consolidated information of the combined company.

Current Structure Chart:

The following reflects our current, post 2009 Merger corporate structure:



Item 8.01 Other Events.

We are filing this Current Report on Form 8-K which will be used as the Company's 2010 Annual Report to stockholders in order to disclose information appropriate for an annual report with respect to both of the Company and Raptor Pharmaceuticals Corp., herein referred to as RPC.

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RAPTOR PHARMACEUTICAL CORP.

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## PART I

### FORWARD-LOOKING STATEMENTS

This Current Report on Form 8-K contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. 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,” “continues,” “estimates,” “potential,” “opportunity” or the negative of these terms or other comparable terminology. All statements, other than statements of historical facts, included in this Current Report on Form 8-K, including our financial condition, future results of operation, 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 this Current Report on Form 8-K. You should not place undue reliance on these statements, which only reflect information available as of the date that they were made. Our business’ actual operations, performance, development and results might differ materially from any forward-looking statement due to various known and unknown risks, uncertainties, assumptions and contingencies, including those described in the section titled “Risk Factors,” and including, but not limited to, the following:

- our need for, and our ability to obtain, additional funds;
- uncertainties relating to clinical trials and regulatory reviews;
- our dependence on a limited number of therapeutic compounds;
- the early stage of the products we are developing;
- the acceptance of any of our future products by physicians and patients;
- competition and dependence on collaborative partners;
- loss of key management or scientific personnel;
- our ability to obtain adequate intellectual property protection and to enforce these rights;
- our ability to avoid infringement of the intellectual property rights of others; and

- the other factors and risks described under the section captioned “Risk Factors” as well as other factors not identified therein.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, the factors discussed in this Current Report on Form 8-K 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 the forward-looking statements included in this Current Report on Form 8-K will prove to be accurate and the forward-looking events discussed in this Current Report on Form 8-K may not occur. In light of the significant uncertainties inherent in the forward-looking statements included in this Current Report on Form 8-K, 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.

## ITEM 1: BUSINESS

### Overview

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

Our current pipeline includes three clinical development programs which we are actively developing. We also have three other clinical-stage product candidates, for which we are seeking business development partners but are not actively developing, and we have four preclinical product candidates we are developing, three of which are based upon our proprietary drug-targeting platforms.

### Clinical Development Programs

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

- DR Cysteamine for the potential treatment of nephropathic cystinosis, or cystinosis, a rare genetic disorder;
- DR Cysteamine for the potential treatment of non-alcoholic steatohepatitis, or NASH, a metabolic disorder of the liver; and
- DR Cysteamine for the potential treatment of Huntington's Disease, or HD.

### Other Clinical-Stage Product Candidates

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

- Convivia™ for the potential management of acetaldehyde toxicity due to alcohol consumption by individuals with aldehyde dehydrogenase, or ALDH2 deficiency, an inherited metabolic disorder; and
- Tezampanel and NGX426, non-opioids for the potential treatment of migraine, acute pain, and chronic pain.

### Preclinical Product Candidates

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Our preclinical platforms consist of targeted therapeutics, which we are developing for the potential treatment of multiple indications, including liver diseases, neurodegenerative diseases and breast cancer:

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

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

We are also examining our glutamate receptor antagonists, tezampanel and NGX426, for the potential treatment of thrombosis disorder.

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## Future Activities

Over the next 12 months, we plan to conduct research and development activities based upon our DR Cysteamine clinical programs and continued development of our preclinical product candidates. We also plan to seek business development partners for our Convivia<sup>TM</sup> product candidate and Tezampanel and NGX426. We may also develop future in-licensed technologies and acquired technologies. A brief summary of our primary objectives in the next 12 months for our research and development activities is provided below. There can be no assurances that our research and development activities will be successful. Our plans for research and development activities over the next 12 months can only be implemented if we are successful in raising significant funds during this period. If we do not raise significant additional funds, we may not be able to continue as a going concern.

## Clinical Development Programs

We develop clinical-stage drug product candidates which are: internally discovered therapeutic candidates based on our novel drug delivery platforms and in-licensed or purchased clinical-stage products which may be new chemical entities in mid-to-late stage clinical development, currently approved drugs with potential efficacy in additional indications, and treatments that we could repurpose or reformulate as potentially more effective or convenient treatments for a drug's currently approved indications.

### Development of DR Cysteamine for the Potential Treatment of Nephropathic Cystinosis or Cystinosis

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

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

The FDA granted orphan drug designation for DR Cysteamine for the treatment of cystinosis in 2006.

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

commence in the first quarter of 2010. While we plan to commercialize DR Cysteamine in the U.S. by ourselves, we are actively negotiating a potential development partner for DR Cysteamine for markets outside of the U.S.

#### Development of DR Cysteamine for the Potential Treatment of Non-Alcoholic Steatohepatitis or NASH

In October 2008, we commenced a clinical trial in collaboration with UCSD to investigate a prototype formulation of DR Cysteamine for the treatment of NASH in juvenile patients. In October 2009, we announced positive findings from the completed treatment phase of this open-label Phase IIa clinical trial. At the completion of the initial six-month treatment phase, the study achieved the primary endpoint: mean blood levels of alanine aminotransferase, or ALT, a common biomarker for NASH, were reduced by over 50%. Additionally, over half of the study participants had achieved normalized ALT levels by the end of the treatment phase.

There are no currently approved drug therapies for NASH, and patients are limited to lifestyle changes such as diet, exercise and weight reduction to manage the disease. DR Cysteamine may provide a potential treatment option for patients with NASH. Although NASH is most common in insulin-resistant obese adults with diabetes and abnormal serum lipid profiles, its prevalence is increasing among juveniles as obesity rates rise within this patient population. Although most patients are asymptomatic and feel healthy, NASH causes decreased liver function and can lead to cirrhosis, liver failure and end-stage liver disease.

The NASH trial entails six months of treatment followed by a six-month post-treatment monitoring period. Eligible patients with baseline ALT and aspartate aminotransferase or AST measurements at least twice that of normal levels were enrolled to receive twice-daily, escalating oral doses of up to 1,000 mg of DR Cysteamine. The trial currently has enrolled eleven NASH patients between 11-18 years old. No major adverse events were reported during the six-month treatment phase. Trial subjects continue to be monitored during the six-month post-treatment period currently underway. Full results are being submitted for peer review by UCSD and us and are expected to be presented in 2010.

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

Huntington's Disease, or HD, is a fatal, inherited degenerative neurological disease affecting about 30,000 people in the U.S. and a comparable number of people in Europe. We are not aware of any treatment for HD other than therapeutics that minimize symptoms such as the uncontrollable movements and mood swings resulting from HD. We are collaborating with a French institution, CHU d' Angers, on a Phase II clinical trial investigating DR Cysteamine in HD patients, anticipated to begin in early 2010. We are providing the clinical trial materials for the study, which is sponsored by CHU d' Angers and funded in part by a grant from the French government. We were granted Orphan Drug Designation in the U.S. by the FDA for cysteamine as a potential treatment for HD in 2008.

#### Other Clinical-Stage Product Candidates

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

#### Convivia™ for Liver Aldehyde Dehydrogenase Deficiency

Convivia™ is our proprietary oral formulation of 4-methylpyrazole, or 4-MP, intended for the potential treatment of acetaldehyde toxicity resulting from alcohol consumption in individuals with ALDH2 deficiency, which is an inherited disorder of the body's ability to breakdown ethanol, commonly referred to as alcohol intolerance. 4-MP is presently marketed in the U.S. and E.U. in an intravenous form as an anti-toxin. Convivia™ is designed to lower systemic levels of acetaldehyde (a carcinogen) and reduce symptoms, such as tachycardia and flushing, associated with alcohol consumption by ALDH2-deficient individuals.

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

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

#### Tezampanel and NGX426 for the Potential Treatment of Migraine and Pain



Tezampanel and NGX426, the oral prodrug of tezampanel, are what we believe to be first-in-class compounds that may represent novel treatments for both pain and non-pain indications. Tezampanel and NGX426 are receptor antagonists that target and inhibit a specific group of receptors—the AMPA and kainate glutamate receptors—found in the brain and other tissues. While normal glutamate production is essential, excess glutamate production, either through injury or disease, has been implicated in a number of diseases and disorders. Published data show that during a migraine, increased levels of glutamate activate AMPA and kainate receptors, result in the transmission of pain and, in many patients, the development of increased pain sensitivity. By acting at both the AMPA and kainate receptor sites to competitively block the binding of glutamate, tezampanel and NGX426 have the potential to treat a number of diseases and disorders. These include chronic pain, such as migraine and neuropathic pain, muscle spasticity and a condition known as central sensitization, a persistent and acute sensitivity to pain.

Results of a Phase IIb clinical trial of tezampanel were released in October 2007. In the trial, a single dose of tezampanel given by injection was statistically significant compared to placebo in treating acute migraine headache. This was the sixth Phase II trial in which tezampanel has been shown to have analgesic activity. Based on a review of the Phase II data, the FDA previously agreed that tezampanel may move forward into a Phase III program for acute migraine.

In December 2008, results of NGX426 in a human experimental model of cutaneous pain, hyperalgesia and allodynia demonstrated a statistically significant reduction in spontaneous pain, hyperalgesia and allodynia compared to placebo following injections of capsaicin (i.e., chili oil) under the skin. In February 2009, results from a Phase 1 multiple dose trial of NGX426 showed that the compound is safe and well-tolerated in healthy male and female subjects when dosed once daily for five consecutive days.

In November 2009, we announced the presentation of clinical trial data on NGX426 at the 12th International Conference on the Mechanisms and Treatment of Neuropathic Pain. The results of the study led by Mark Wallace, M.D., Professor of Clinical Anesthesiology at the Center for Pain Medicine of the University of California at San Diego, suggested that NGX426 has the potential to be effective in a variety of neuropathic pain states, which are caused by damage to or dysfunction of the peripheral or central nervous system rather than stimulation of pain receptors.

We are currently seeking program funding, development collaborations or out-licensing partners for the migraine and pain programs.

#### Preclinical Product Candidates

We are also developing a drug-targeting platform based on the proprietary use of RAP and Mesd. We believe that these proteins may have therapeutic applications in cancer, infectious diseases and neurodegenerative diseases, among others.

These applications are based on the assumption that our targeting molecules can be engineered to bind to a selective subset of receptors with restricted tissue distribution under particular conditions of administration. We believe these selective tissue distributions can be used to deliver drugs to the liver or to other tissues, such as the brain.

In addition to selectively transporting drugs to specific tissues, selective receptor binding constitutes a means by which receptor function might be specifically controlled, either through modulating its binding capacity or its prevalence on the cell surface. Mesd is being engineered for this latter application.

#### HepTide™ for Hepatocellular Carcinoma and Hepatitis C

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

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

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

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

- RAP is captured by hepatocytes with efficiency, primarily on first-pass.
- Late-stage HCC is perfused exclusively by the hepatic artery, while the majority of the liver is primarily perfused through the portal vein.

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

We are evaluating conjugates between HepTide™ and a chemotherapeutic for testing in vitro and in appropriate preclinical models for the potential treatment of HCC.

We are also evaluating conjugates between HepTide™ and an antiviral agent for testing in vitro and in appropriate preclinical models for the potential treatment of hepatitis C.

#### NeuroTrans™ for the Potential Treatment of Diseases Affecting the Brain

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

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

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

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

In June 2009, we entered into a collaboration and licensing agreement with F. Hoffman — La Roche Ltd. and Hoffman—La Roche Inc., or Roche, to evaluate therapeutic delivery across the blood-brain barrier utilizing NeuroTrans™. Under terms of the agreement, Roche has funded studies of select molecules attached to NeuroTrans™. The agreement provides Roche with an exclusive worldwide license to NeuroTrans™ for use in the delivery of diagnostic and therapeutic molecules across the blood-brain barrier. Roche's and our scientists will actively collaborate on the project. We have received an initial upfront payment for the collaboration to cover our portion of the initial studies, and may earn development milestone payments and royalties in exchange for the licensing of NeuroTrans™ to Roche.

#### WntTide™ for the Potential Treatment of Cancer

Human Mesd is a natural inhibitor of the receptor LRP6. LRP6 has recently been shown to play a role in the progression of some breast tumors. Studies in the laboratory of Professor Guojun Bu, one of our scientific advisors, at the Washington

University in St. Louis Medical School support the potential of Mesd and related peptides to target these tumors. These molecules and applications are licensed to us from Washington University.

WntTide™ is our proprietary, Mesd-based peptide that we are developing as a potential therapeutic to inhibit the growth and metastasis of tumors over-expressing LRP5 or LRP6. We have licensed the use of Mesd from Washington University in St. Louis for the potential treatment of cancer and bone density disorders.

In April 2009, Washington University conducted a preclinical study of WntTide™ in a breast cancer model which showed tumor inhibition. The results of this study were presented at the 2nd Annual Wnt Conference in Washington, D.C., in June 2009 and will likely be published in the first quarter of 2010. We are currently planning another breast tumor preclinical model study with researchers at Washington University in the continued development of WntTide™.

#### Tezampanel and NGX426 for the Potential Treatment of Thrombotic Disorder

Research conducted at Johns Hopkins University, or JHU, by Craig Morrell, D.V.M., Ph.D., and Charles Lowenstein, M.D. demonstrated the importance of glutamate release in promoting platelet activation and thrombosis. Research

shows that platelets treated with an AMPA/kainate receptor antagonist such as tezampanel or NGX426 are more resistant to glutamate-induced aggregation than untreated platelets. This identifies the AMPA/kainate receptors on platelets targeted by tezampanel or NGX426 as a new antithrombotic target with a different mechanism of action than Plavix®, aspirin or tPA. We have licensed the intellectual property of Tezampanel and NGX 426 for the treatment of thrombotic disorder from JHU and are in discussions with potential collaborators regarding the development of this product candidate.

#### Other Development Areas

##### Securing Additional and Complementary Technology Licenses from Others

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

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## Strategic Acquisitions

### Reverse Merger with Raptor Pharmaceuticals Corp.

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

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

### Purchase of ConviviaTM

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

Thomas E. (Ted) Daley, as the President of its clinical development division. In exchange for the assets related to the ALDH2 deficiency program, what we now call ConviviaTM, Raptor Pharmaceuticals Corp. issued to Convivia 200,000 shares of its common stock, an additional 200,000 shares of its common stock to a third party in settlement of a convertible loan between the third party and Convivia, and another 37,500 shares of its common stock in settlement of other obligations of Convivia. Mr. Daley, as the former sole stockholder of Convivia, may earn additional shares of our common stock based on certain triggering events or milestones related to the development of the Convivia assets. In addition, Mr. Daley may earn cash bonuses based on the same triggering events pursuant to his employment agreement. In January 2008, Mr. Daley earned a \$30,000 cash bonus pursuant to his employment agreement as a result of the milestone of our execution of a formulation agreement for manufacturing ConviviaTM with Patheon. In March 2008, Raptor Pharmaceuticals Corp. issued to Mr. Daley 100,000 shares of its common stock pursuant to the Convivia purchase agreement as a result of the milestone of our execution of an agreement to supply us with the active pharmaceutical ingredient for ConviviaTM and two \$10,000 cash bonuses pursuant to his employment agreement for reaching his six-month and one-year employment anniversaries. In October 2008, Raptor Pharmaceuticals Corp. issued to Mr. Daley 100,000 shares of its common stock valued at \$27,000 and a \$30,000 cash bonus as a result of fulfilling a clinical milestone. Due to the 2009 Merger, the 200,000, 200,000, 37,500, 100,000 and 100,000 shares Raptor Pharmaceuticals Corp., respectively, described above, became 46,625, 46,625, 8,742, 23,312 and 23,312 shares of our common stock, respectively.

### Purchase of DR Cysteamine

In December 2007, prior to the 2009 Merger, through a merger between Encode Pharmaceuticals, Inc., or Encode, and Raptor Therapeutics, Raptor Pharmaceuticals Corp. purchased certain assets, including the clinical development rights to DR Cysteamine. Under the terms of and subject to the conditions set forth in the merger agreement, Raptor Pharmaceuticals Corp. issued 3,444,297 shares of its common stock to the stockholders of Encode, or Encode Stockholders, options, or Encode Options, to purchase up to, in the aggregate, 357,427 shares of its common stock to the optionholders of Encode, or Encode Optionholders, and warrants, or Encode Warrants, to purchase up to, in the aggregate, 1,098,276 shares of its common stock to the warrantholders of Encode, or Encode Warrantholders, and together with the Encode Stockholders and Encode Optionholders, referred to herein collectively as the Encode Securityholders, as of the date of such agreement. Due to the 2009 Merger, the 3,444,296 shares of Raptor Pharmaceuticals Corp.'s common stock, the 357,427 Encode Options and 1,098,276 Encode Warrants, respectively, became 802,946 shares of our common stock, Encode Options to purchase 83,325 shares of our common stock and Encode Warrants to purchase 256,034 shares of our common stock, respectively. The Encode Securityholders are eligible to receive up to an additional 559,496 shares of our common stock, Encode Options and Encode Warrants to purchase our common stock in the aggregate based on certain triggering events related to regulatory approval of DR Cysteamine, an Encode product program, if completed within the five year anniversary date of the merger agreement.

As a result of the Encode merger, we received the exclusive worldwide license to DR Cysteamine, referred to herein as the License Agreement, developed by clinical scientists at the UCSD, School of Medicine. In consideration of the grant of the license, we are obligated to pay an annual maintenance fee of \$15,000 until we begin commercial sales of any products developed pursuant to the License Agreement. In addition to the maintenance fee, we are obligated to pay during the life of the License Agreement: milestone payments ranging from \$20,000 to \$750,000 for orphan indications and from \$80,000 to \$1,500,000 for non-orphan indications upon the occurrence of certain events, if ever; royalties on commercial net sales from products developed pursuant to the License Agreement ranging from 1.75% to 5.5%; a percentage of sublicense fees ranging from 25% to 50%; a percentage of sublicense royalties; and a minimum annual royalty commencing the year we begin commercially selling any products pursuant to the License Agreement, if ever. Under the License Agreement, we are obligated to fulfill predetermined milestones within a specified number of years ranging from 0.75 to 6 years from the effective date of the License Agreement, depending on the indication. In addition, we are obligated, among other things, to spend annually at least \$200,000 for the development of products (which we satisfied, as of August 31, 2009 by spending approximately \$4.1 million on such programs) pursuant to the License Agreement. To-date, we have paid \$270,000 in milestone payments to UCSD based upon the initiation of clinical trials in cystinosis and in NASH. To the extent that we fail to perform any of our obligations under the License Agreement, UCSD may terminate the license or otherwise cause the license to become non-exclusive.

## Company History

### Corporate Structure

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

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

Raptor Pharmaceuticals Corp. was incorporated in the State of Nevada on April 1, 2002 under the name of Highland Clan Creations Corp., or HCCC. On June 9, 2006, HCCC merged with Raptor Pharmaceuticals Corp. which was incorporated on May 5, 2006 in Delaware. As a result, HCCC was reincorporated from the State of Nevada to the State of Delaware and changed its corporate name to “Raptor Pharmaceuticals Corp.” HCC was a publicly traded company quoted on the OTC Bulletin Board and upon such merger, its common stock traded on the OTC Bulletin Board under the ticker “RPTP.”

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



Financing History of Raptor Pharmaceuticals Corp.

Initial Investors

On May 25, 2006, in exchange for all of the outstanding common stock of Raptor Pharmaceutical Inc., Raptor Pharmaceuticals Corp. issued 8,000,000 shares of common stock to the-then Raptor Pharmaceutical Inc. stockholders including 3,000,000 shares of its common stock to each of Christopher M. Starr, Ph.D., and Todd C. Zankel, Ph.D., our Chief Executive Officer and Chief Scientific Officer, respectively, 1,000,000 shares of its common stock to Erich Sager, a member of our board of directors and 1,000,000 shares of its common stock to an unrelated third party. These initial stockholders of Raptor Pharmaceutical Inc. purchased common stock of Raptor Pharmaceutical Inc. when it was a privately held company for the following amounts of proceeds: Dr. Starr \$5,000; Dr. Zankel \$5,000; Mr. Sager \$100,000 and the unrelated third party \$200,000. Due to the 2009 Merger, the 3,000,000, 3,000,000, 1,000,000 and 1,000,000 shares of common stock of Raptor Pharmaceuticals Corp., respectively, described above, became 699,370, 699,370, 233,123 and 233,123 shares of our common stock, respectively.

#### \$5 Million Financing and the 2006 Reverse Merger

Pursuant to an agreement dated March 8, 2006, with HCCC, on May 25, 2006, Raptor Pharmaceuticals Corp. closed a \$5 million financing concurrent with a reverse merger. As part of that agreement, HCCC loaned Raptor Pharmaceuticals Corp. \$0.2 million to be repaid with accrued interest upon the earlier of six months or the closing of the financing. Also, the agreement stated that pending the closing of at least a \$3.5 million financing, HCCC would be obligated to issue 800,000 units as fees to a placement agent and \$30,000 in commissions to an investment broker. In the financing HCCC sold 8,333,333 units of Raptor Pharmaceuticals Corp. at \$0.60 per unit. Each such unit consisted of one share of Raptor Pharmaceuticals Corp.'s common stock and one common stock purchase warrant exercisable for one share of Raptor Pharmaceuticals Corp.'s common stock at \$0.60 per share. The warrants were exercisable for 18 months and expired on November 25, 2007. Gross proceeds from the financing were \$5 million and net proceeds after the repayment of the \$0.2 million loan plus interest and the deduction of commissions and legal fees totaled approximately \$4.6 million. Prior to the warrants expiring, Raptor Pharmaceuticals Corp. received \$3,895,000 in gross proceeds from the exercise of warrants in exchange for 6,491,667 shares of its common stock. Due to the 2009 Merger, each such share of common stock of Raptor Pharmaceuticals Corp. (including such common stock issued pursuant to the exercise of warrants) issued pursuant to such financing and reverse merger outstanding as of immediately prior to the 2009 Merger, was exchanged for 0.2331234 shares of our common stock.

#### Issuance of Common Stock Pursuant to Stock Option Exercises

Since inception, Raptor Pharmaceuticals Corp. has received \$8,700 from the exercise of stock options resulting in the issuance of 14,500 shares of its common stock. Due to the 2009 Merger, such 14,500 shares of common stock became 3,380 shares of our common stock.

#### Raptor Pharmaceuticals Corp.'s 2008 and 2009 Private Placements and Warrant Exchange

During May and June 2008, prior to the 2009 Merger, Raptor Pharmaceuticals Corp., issued an aggregate of 20,000,000 units of its securities, each unit comprised of one share of its common stock and one warrant to purchase one half of one share of its common stock, at a unit purchase price of \$0.50 per unit, in a private placement with various accredited investors. The warrants, exercisable for two years from closing of such private placement, as initially issued, entitled such investors to purchase up to an aggregate of 10,000,000 shares of Raptor Pharmaceuticals Corp.'s common stock at an exercise price of \$0.75 per share during the first year and \$0.90 per share during the second year. In connection with this private placement, Raptor Pharmaceuticals Corp. issued placement agents warrants to purchase in the aggregate 2,100,000 shares of its common stock at an exercise price of \$0.55 per share for a five year term and it paid to such placement agents cash fees totaling \$700,000. Such placement agent warrants contained a cashless (net exercise) feature that allows its holders, under certain circumstances, to exercise such warrants without making any cash payment. Of the placement agents compensated, Limetree Capital was issued warrants to purchase 1,882,650 shares of Raptor Pharmaceuticals Corp.'s common stock and was paid cash commissions of \$627,550. Erich Sager, one of our board members, serves on the board of directors of Limetree Capital and is a founding partner thereof.

In July 2009, prior to the 2009 Merger, Raptor Pharmaceuticals Corp. closed a warrant exchange offer with those investor-warrant holders who were holders of the warrants to purchase its common stock issued in connection with its May and June 2008 private placement, as described above, of the right to exchange such warrants and subscribe for new warrants to purchase shares of Raptor Pharmaceuticals Corp.'s common stock at an exercise price of \$0.30 per share (to the extent such new warrants were exercised (in whole or in part) on or before July 17, 2009). Pursuant to such warrant exchange, new warrants were exercised for an aggregate amount of 8,715,000 shares of Raptor

Pharmaceuticals Corp.'s common stock which resulted in aggregate proceeds to Raptor Pharmaceuticals Corp. of \$2,614,500.

In August 2009, prior to the 2009 Merger, Raptor Pharmaceuticals Corp., issued an aggregate of 7,456,250 units of its securities, each unit comprised of one share of its common stock and one warrant to purchase one half of one share of its common stock, at a unit purchase price of \$0.32 per unit, in a private placement with various accredited investors. The warrants, exercisable for two years from closing of such private placement, as initially issued, entitled such investors to purchase up to an aggregate of 3,728,125 shares of Raptor Pharmaceuticals Corp.'s common stock at an exercise price of \$0.60 per share during the first year and \$0.75 per share during the second year. In connection with this private placement, Raptor Pharmaceuticals Corp. issued Limetree Capital, the placement agent in such private placement, warrants to purchase in the aggregate 556,500 shares of its common stock at an exercise price of \$0.35 per share for a five year term and it paid to such placement agent cash fees totaling \$59,360. Such placement agent warrants contained a cashless (net exercise) feature that allows its holders, under certain circumstances, to exercise such warrants without making any cash payment.

As a result of the 2009 Merger, (i) the 20,000,000 shares of Raptor Pharmaceuticals Corp.'s common stock issued in the 2008 private placement, the 8,715,000 shares of Raptor Pharmaceuticals Corp.'s common stock issued as a result of the warrant exchange, and the 7,456,250 shares of Raptor Pharmaceuticals Corp.'s common stock issued in the 2009 private placement, were converted into the right to receive an aggregate of 8,432,364 shares of our common stock, (ii) the warrants issued in the 2008 private placement to investors to purchase 10,000,000 shares of Raptor Pharmaceuticals Corp.'s common stock at exercise prices of \$0.75 and \$0.90 per share, depending on when exercised, which, after the warrant exchange, were reduced to warrants to purchase 1,285,000 shares of Raptor Pharmaceuticals Corp.'s common stock, and the warrants issued in the 2009 private placement to investors to purchase 3,728,125 shares of Raptor Pharmaceuticals Corp.'s common stock at exercise prices of \$0.60 and \$0.75 per share, depending on when exercised, were converted into the right to receive warrants to purchase 299,563 shares of our common stock at exercise prices of \$3.21 and \$3.86 per share, depending on when exercised, and warrants to purchase 869,114 shares of our common stock at exercise prices of \$2.57 and \$3.21 per share, depending on when exercised, respectively, and (iii) the warrants issued in the 2008 private placement to such placement agents to purchase 2,100,000 shares of Raptor Pharmaceuticals Corp.'s common stock at an exercise price of \$0.55 per share (after the exercise by a certain placement agent of a warrant to purchase 101,850 shares of Raptor Pharmaceuticals Corp.'s common stock but prior to the effective time of the 2009 Merger), and the warrants issued in the 2009 private placement to such placement agent to purchase 556,500 shares of Raptor Pharmaceuticals Corp.'s common stock at an exercise price of \$0.35 per share, were converted into the right to receive warrants to purchase 465,816 shares of our common stock at an exercise price of \$2.36, 23,744 shares of our common stock, and warrants to purchase 129,733 shares of our common stock at an exercise price of \$1.50, respectively. Other than as described herein, none of the other provisions of such warrants were changed, including, with respect to the placement agent warrants, the cashless (net exercise) feature.

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

#### Proprietary Rights

We purchased from BioMarin the intellectual property owned by BioMarin for the research and development of the RAP technologies, including two patents, two pending patent applications and two provisional patent applications in review in the U.S., and countries in Europe and Asia and two trademarks for NeuroTrans™. Subsequent to the purchase from BioMarin, we have filed four additional patent applications for our RAP technologies. As of October 23, 2009, we have eight patent applications under prosecution in the U.S. and internationally. Two of these applications relate to cysteamine and the remaining six cover the RAP platform. Of the six RAP platform patents, two have been allowed in the U.S., as of July 14, and August 4, 2009, and another was allowed in Japan, Australia and Europe during the first half of 2009. All other applications are awaiting examination in a variety of countries. We also entered into an exclusive worldwide license agreement with Washington University for our Mesd program for the treatment of cancer and bone diseases. We fund the prosecution of a patent application covering this technology, entering national phase in the U.S. and internationally in November, 2009. In October 2007, we acquired intellectual property assets from Convivia, Inc., a privately held pharmaceutical company, including four filed patents for 4-MP as a potential treatment for ALDH2 deficiency. Since the acquisition of Convivia, Inc. assets, we filed a provisional patent for trans-dermal formulation of 4-MP, a provisional patent for genotype specific methods for treating human subjects using 4-methylpyrazole and a patent based on botanically derived compound for treatment of ALDH2 deficiency. In December 2007, we acquired an exclusive worldwide license agreement to pending patent applications from UCSD relating to our DR Cysteamine program. In March 2008, we amended our license with UCSD to add exclusive worldwide rights to develop DR Cysteamine for the potential treatment of NASH. We also have a license from Eli

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Lilly & Co. for the intellectual property related to tezampanel and NGX426 for pain indications and a license of tezampanel and NGX 426 for the treatment of thrombotic disorder from JHU. We fund the prosecution of a patent covering this technology, which entered national phase in the U.S. in August, 2009.

## Regulatory Exclusivities

### Orphan Drug Designation

We have been granted access to an Orphan Drug Designation from the FDA for use of DR Cysteamine to potentially treat cystinosis and the use of Cysteamine to potentially treat HD and Batten Disease. The Orphan Drug Act of 1983 generally provides incentives, including marketing exclusivity and tax benefits, to companies that undertake development and marketing of products to treat relatively rare diseases, which are defined as meaning 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 is entitled to up to seven years of exclusive marketing in the U.S. for that indication. Equivalent European regulations would give us ten years of marketing exclusivity for that indication in Europe. DR Cysteamine has already been granted Orphan Drug Designation by the FDA and we plan to submit an orphan drug application in Europe. We cannot be sure that we will be granted orphan drug status or that it would prove advantageous. In addition, the testing and approval process will likely require a significant commitment of time, effort, and expense on our part. If we fail to obtain or maintain orphan drug exclusivity for some of our drug product candidates, our competitors may sell products to treat the same conditions and our results of operations and revenues will be affected.

### Facilities

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

### Competition

#### Cystinosis

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

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

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

#### Huntington's Disease

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

academic- and foundation-sponsored research efforts.

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

## NASH

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

## ALDH2 Deficiency

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

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

## Migraine

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

## Pain

In the neuropathic pain market, we would compete with companies such as Pfizer, marketing Neurontin and Lyrica® , and Eli Lilly, marketing Cymbalta ® in addition to opioids approved for treating neuropathic pain, off-label uses of products to treat neuropathic pain and generic products. Given the size of the neuropathic pain market, approximately \$3.5 billion in 2006 and expected to double by 2016, it is likely that most of the large pharmaceutical companies as well as many biotechnology companies will look to develop compounds to treat neuropathic pain. Since the licensing



of tezampanel, Eli Lilly has continued development of more potent and specific molecules (e.g., iGluR5 antagonists) targeting the same receptors as tezampanel and NGX424 and based on the same chemistry (i.e., tetrahydroisoquinoline moiety) as tezampanel and NGX424. Eli Lilly's third generation candidate is currently in phase II studies for osteoarthritis and peripheral neuropathy.

#### Primary Liver Cancer

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

## Hepatitis

It has been estimated that approximately 3% of the world's population is chronically infected with hepatitis C, which translates to nearly 200 million people infected worldwide. Due to the latency of hepatitis C virus, or HCV, infection and slow disease progression, along with a lag in awareness of the disease, the number of patients with HCV is increasing and expected to peak in the next 20-30 years. Over 50,000 people die of HCV infection every year. Up to 75% of chronically infected individuals carry the genotype I strain of HCV. The most effective current treatment for chronic HCV infection is Interferon, but nearly 60% of patients infected with genotype 1 do not show a sustained viral reduction with Interferon treatment, and the remaining 40% of such genotype 1 HCV cases are without any therapy.

The significant number of interferon non-responders has created a need for second generation therapies and a large number of pharmaceutical companies have active therapeutic programs to meet the requirements of this large and growing market. There are currently 28 compounds in clinical development for the treatment of chronic HCV infection. A large number of these clinical compounds are small molecule antivirals being developed by pharmaceutical companies including Novartis, Kemin, Vertex and Migenix. In addition, over a dozen non-interferon immunomodulators are currently under clinical development by companies including SciClone, Schering-Plough, Chiron and Innogenetics. These compounds are designed to attack different parts of the Hepatitis C virus and its ability to replicate or enhance the body's immune system to better recognize and destroy the virus. Most clinicians now believe that eventually these and future drugs will be used in combination to treat chronic HCV.

## Brain Delivery

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

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

- Neurosurgery or invasive techniques.
- Pharmacological techniques, which include less than 2% of currently available drugs.
- Physiologically based techniques, such as transcytosis.

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

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

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

## Thrombotic Disorder

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

Because, many of our competitors have greater capital resources and larger overall research and development staffs and facilities, than us, there can be no assurances that we will be successful in competing in the areas discussed above. See the section under “Risk Factors” titled, “If our competitors succeed in developing products and technologies that are more effective than ours, or if scientific developments change our understanding of the potential scope and utility of our drug product candidates, then our technologies and future drug product candidates may be rendered less competitive.”

## Government Regulations of the Biotechnology Industry

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

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

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

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

product candidates or result in marketable products.

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

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

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

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

Preclinical testing includes laboratory evaluation and characterization of the safety and efficacy of a drug and its formulation. Preclinical testing results are submitted to the FDA as a part of an IND which must become effective prior to commencement of clinical trials. Clinical trials are typically conducted in three sequential phases following submission of an IND. Phase I 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 II 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 III studies are initiated to further establish clinical safety and efficacy of the therapy in a broader sample of the general patient population, in order to determine the overall risk-benefit ratio of the drug and to provide an adequate basis for any physician labeling. During all clinical studies, we must adhere to Good Clinical Practice, or GCP, standards. The results of the research and product

development, manufacturing, preclinical studies, clinical studies and related information are submitted in an NDA to the FDA.

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

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

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

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

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

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

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



product approval applications, withdrawal of approved applications, and prosecution.

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

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

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

#### Scientific Advisory Board

The following describes the background of our Scientific Advisory Board.

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

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

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

Children's Hospital in Vancouver, Canada and at St. Bartholemew and The London Medical School.

William C. Mobley, M.D. , Ph.D. After completing undergraduate training in Chemistry and Zoology at the University of Nebraska at Lincoln, William C. Mobley, M.D., Ph.D., received his M.D. and Ph.D. in Neuroscience from Stanford University. Dr. Mobley trained in Pathology and Pediatrics at the Stanford University Hospital and completed a residency and fellowship in Neurology at Johns Hopkins University Hospital, where he also was Chief Resident in Pediatric Neurology. In 1985, he joined the faculty of the University of California, San Francisco School of Medicine where he rose to the rank of Professor of Neurology, Pediatrics and the Neuroscience Program and served as the Director of Child Neurology. In 1991, he was named Derek Denny Brown Scholar of the American Neurological Association. From 1997 to 2005, he served as the Chair of the Department of Neurology and Neurological Sciences at Stanford University, and he held the John E. Cahill Family Endowed Chair. He was appointed Director of the Neuroscience Institute at Stanford. While at Stanford his laboratory studied the signaling biology of neurotrophic factors in the normal nervous system and in animal models of neurological disorders, including Alzheimer's disease, Down's syndrome and peripheral neuropathy. He is the recipient of both the Zenith Award and the Temple Award from the Alzheimer's Association and is a Fellow of the Royal College of Physicians. He was chosen to receive the Cotzias Award of the American Academy of Neurology for 2004. Dr. Mobley is Past President of the Association of University Professors of Neurology and is President of The Professors of Child Neurology. He was recently elected to the Institute of Medicine of the National Academy of Sciences. Dr Mobley now serves as the chair of the department of neurosciences at the University of California, San Diego School of Medicine since April 2009.

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

Sam Teichman, M.D., FACC, FACP Sam Teichman, M.D., is an independent consultant in the area of strategic drug discovery and development. He has worked on over 40 medical products in various stages of development from the earliest identification of leads in research to supporting commercial-stage products. Most recently, Dr. Teichman served as Vice President and Chief Development Officer at ARYx Therapeutics, where he was involved in identifying and advancing three products from the research stage into clinical development. During the past 20 years, Dr. Teichman has held senior level executive positions at Genentech, Medco Research (now part of King Pharmaceuticals), Glycomed (now part of Ligand Pharmaceuticals), and Mimetix. He has provided scientific advisory services and has acted in an interim executive role for numerous early-stage and established biotechnology companies. Dr. Teichman holds an M.D. from Columbia University and a B.S. in Chemistry from Columbia College, Columbia University. He is board certified in Internal Medicine and Cardiology. Dr. Teichman is a Fellow of the American College of Cardiology (FACC) and the American College of Physicians (FACP). Dr. Teichman served as Associate Clinical Professor of Medicine at University of California in San Francisco from 1990 to 2001. He has more than 40 original publications, reviews and abstracts published in peer-reviewed and invited medical journals.

#### Legal Proceedings

Several lawsuits were filed against us in February 2005 in the U.S. District Court for the Southern District of New York asserting claims under Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 thereunder on behalf of a class of purchasers of our common stock during the period from June 26, 2003, through and including February 4, 2005, referred to as the class period. Dr. Marvin S. Hausman, M.D., a former director and a former Chief Executive Officer, and Dr. Gosse B. Bruinsma, M.D., also a former director and a former Chief Executive Officer, were also named as defendants in the lawsuits. These actions were consolidated into a single class action lawsuit in January 2006. On April 10, 2006, the class action plaintiffs filed an amended consolidated complaint. We filed our answer to that complaint on May 26, 2006. Our motion to dismiss the consolidated amended complaint was filed on May 26, 2006 and was submitted to the court for a decision in September 2006. On March 31, 2009 the U.S. District Court for the Southern District of New York dismissed the proceedings. On April 24, 2009, an appeal was filed with the United States Court of Appeals for the Second Circuit by the class action plaintiffs. Our response to such appeal was filed on October 23, 2009. The Second Circuit heard the plaintiffs' appeal of the order dismissing the complaint on January 14, 2010. We do not anticipate that this claim, if successful, would burden the Company with any additional liability above and beyond the insurance coverage provided under the insurance policy that we currently maintain.

Other than as described above, we know of no material, active or pending legal proceedings against us, nor are we involved as a plaintiff in any material proceedings or pending litigation. There are no proceedings in which any of our

directors, officers or affiliates, or any registered or beneficial stockholders are an adverse party or have a material interest adverse to us.

#### Research and Development

We are a research and development company and our plan is to focus our efforts in the discovery, research, preclinical and clinical development of our RAP based platforms, complementary technologies and clinical drug candidates to provide therapies that we believe will be safer, less intrusive, and more effective than current approaches in treating a wide variety of brain disorders and neurodegenerative diseases, genetic disorders and cancer. During the period from September 8, 2005 (inception of Raptor Pharmaceuticals Corp.) to August 31, 2009, we incurred approximately \$14.9 million (\$6.6 million and \$5.6 million for the years ended August 31, 2009 and 2008, respectively) in research and development costs.

Please see the section titled, "Management's Discussion and Analysis of Financial Condition and Results of Operations" in this Current Report on Form 8-K for our planned research and development activities for the twelve months subsequent to November 30, 2009.

#### Compliance with Environmental Laws

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

#### Employees

We presently have twelve full time employees, including eight executives, one scientist, one program director and one clinical development assistant in our research and development department and one senior manager in our finance department. Nine of these employees were retained as part of the 2009 Merger, including our Chief Executive Officer, Dr. Christopher M. Starr, our Chief Scientific Officer, Dr. Todd C. Zankel, our Chief Financial Officer, Kim R. Tsuchimoto, Ted Daley, the President of our clinical development subsidiary and Dr. Patrice P. Rioux, Chief Medical Officer of our clinical development subsidiary. Based on our current plan, over the next 12 month period, we anticipate hiring a regulatory director. We also plan to supplement our human resources needs through consultants and contractors as needed.

#### ITEM 1A: RISK FACTORS

An investment in our common stock involves a high degree of risk. Before investing in our common stock, you should consider carefully the specific risks detailed in this "Risk Factors" section before making a decision to invest in our common stock, together with all of the other information contained in this Current Report on Form 8-K. If any of these risks occur, our business, results of operations and financial condition could be harmed, the price of our common stock could decline, and you may lose all or part of your investment.

#### Risks Related to Our Business

If we fail to obtain the capital necessary to fund our operations, our financial results, financial condition and our ability to continue as a going concern will be adversely affected and we will have to delay or terminate some or all of our product development programs.

Our (i) condensed consolidated financial statements as of November 30, 2009 and (ii) consolidated financial statements as of August 31, 2009, have been prepared assuming that we will continue as a going concern. As of November 30, 2009, we had an accumulated deficit of approximately \$24.8 million. We expect to continue to incur losses for the foreseeable future and will have to raise substantial cash to fund our planned operations. Our recurring losses from operations and our stockholders' deficit raise substantial doubt about our ability to continue as a going concern and, as a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the year ended August 31, 2009, with respect to this uncertainty. We will need to generate significant revenue or raise additional capital to continue to operate as a going concern. In addition, the perception that we may not be able to continue as a going concern may cause others to choose not to deal

with us due to concerns about our ability to meet our contractual obligations and may adversely affect our ability to raise additional capital.

We believe that our cash and cash equivalents at November 30, 2009 along with the net funds raised subsequent to quarter-end in December 2009 of approximately \$6.9 million (see the subsequent event Note 12 to the condensed consolidated financial statements) will be sufficient to meet our obligations into the third calendar quarter of 2010. This estimate is based on assumptions that may prove to be wrong. We are currently in the process of negotiating strategic partnerships and collaborations in order to fund our preclinical and clinical programs into 2011. If we are not able to close a strategic transaction, we anticipate raising additional capital in the second calendar quarter of 2010 for the continued development of our drug development programs.. We will need to sell equity or debt securities to raise significant additional funds. The sale of additional securities is likely to result in additional dilution to our stockholders. Additional financing may not be available in amounts or on terms satisfactory to us or at all. We may be unable to raise additional financing due to a variety of factors, including our financial condition, the status of our research and development programs, and the general condition of the financial markets. If we fail to raise significant additional financing, we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected and we may have to cease our operations.

If we obtain significant additional financing, we expect to continue to spend substantial amounts of capital on our operations for the foreseeable future. The amount of additional capital we will need depends on many factors, including:

- the progress, timing and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the time and cost necessary to respond to technological and market developments; and
- any changes made or new developments in our existing collaborative, licensing and other corporate relationships or any new collaborative, licensing and other commercial relationships that we may establish.

Moreover, our fixed expenses such as rent, collaboration and license payments and other contractual commitments are substantial and will likely increase in the future. These fixed expenses are likely to increase because we expect to enter into:

- additional licenses and collaborative agreements;
- contracts for manufacturing, clinical and preclinical research, consulting, maintenance and administrative services; and
- financing facilities.

We are an early development stage company and have not generated any revenues to date and have a limited operating history. Many of our drug product candidates are in the concept stage and have not undergone significant testing in preclinical studies or any testing in clinical trials. Moreover, we cannot be certain that our research and development efforts will be successful or, if successful, that our drug product candidates will ever be approved for sale or generate commercial revenues. We have a limited relevant operating history upon which an evaluation of our performance and prospects can be made. We are subject to all of the business risks associated with a new enterprise, including, but not limited to, risks of unforeseen capital requirements, failure of drug product candidates either in preclinical testing or in clinical trials, failure to establish business relationships, and competitive disadvantages against larger and more established companies.



The current disruptions in the financial markets could affect our ability to obtain financing on favorable terms (or at all).

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

Even if we are able to develop our drug product candidates, we may not be able to receive regulatory approval, or if approved, we may not be able to generate significant revenues or successfully commercialize our products, which would adversely affect our financial results and financial condition and we would have to delay or terminate some or all of our research product development programs.

All of our drug product candidates are at an early stage of development and will require extensive additional research and development, including preclinical testing and clinical trials, as well as regulatory approvals, before we can market them. Since our inception in 1997, and since Raptor Pharmaceuticals Corp. began operations in 2005, both companies have dedicated substantially all of their resources to the research and development of their technologies and related compounds. All of our compounds currently are in preclinical or clinical development, and none have been submitted for marketing approval. Our preclinical compounds may not enter human clinical trials on a timely basis, if at all, and we may not develop any product candidates suitable for commercialization. We cannot predict if or when any of the drug product candidates we intend to develop will be approved for marketing. There are many reasons that we may fail in our efforts to develop our drug product candidates. These include:

- the possibility that preclinical testing or clinical trials may show that our drug product candidates are ineffective and/or cause harmful side effects;
- our drug product candidates may prove to be too expensive to manufacture or administer to patients;
- our drug product candidates may fail to receive necessary regulatory approvals from the FDA or foreign regulatory authorities in a timely manner, or at all;
- our drug product candidates, if approved, may not be produced in commercial quantities or at reasonable costs;
- our drug product candidates, if approved, may not achieve commercial acceptance;
- regulatory or governmental authorities may apply restrictions to our drug product candidates, which could adversely affect their commercial success; and
- the proprietary rights of other parties may prevent us or our potential collaborative partners from marketing our drug product candidates.

If we fail to develop our drug product candidates, our financial results and financial condition will be adversely affected, we will have to delay or terminate some or all of our research product development programs and may be forced to cease operations.

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

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

If the purchase or licensing agreements we entered into are terminated, we will lose the right to use or exploit our owned and licensed technologies, in which case we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected and we may have to cease our operations.

We entered into an asset purchase agreement with BioMarin Pharmaceutical Inc., or BioMarin, for the purchase of intellectual property related to the receptor-associated protein, or RAP, technology, a licensing agreement with Washington University for mesoderm development protein, or Mesd, and a licensing agreement with UCSD for DR Cysteamine. BioMarin, Washington University and UCSD may terminate their respective agreements with us upon the occurrence of certain events, including if we enter into certain bankruptcy proceedings or if we materially breach our payment obligations and fail to remedy the breach within the permitted cure periods. Although we are not currently involved in any bankruptcy proceedings or in breach of these agreements, there is a risk that we may be in the future, giving BioMarin, Washington University and UCSD the right to terminate their respective agreements with us. We have the right to terminate these agreements at any time by giving prior written notice. If the BioMarin, Washington University or UCSD agreements are terminated by either party, we would be forced to assign back to BioMarin, in the case of the BioMarin agreement, all of our rights, title and interest in and to the intellectual property related to the RAP technology, would lose our rights to the Mesd technology, in the case of the Washington University agreement and would lose our rights to DR Cysteamine, in the case of UCSD. Under such circumstances, we would have no further right to use or exploit the patents, copyrights or trademarks in those respective technologies. If this happens, we will have to delay or terminate some or all of our research and development programs, our financial condition and operating results will be adversely affected, and we may have to cease our operations. If we lose our rights to the intellectual property related to the RAP technology purchased by us from BioMarin, our agreement with Roche regarding the evaluation of therapeutic delivery across the blood-brain barrier utilizing NeuroTrans™ would likely be terminated and any milestone or royalty payments from Roche to us would thereafter cease to accrue.

If we fail to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop our drug product candidates.

Our competitors compete with us to attract established biotechnology and pharmaceutical companies or organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. Collaborations include licensing proprietary technology from, and other relationships with, academic research institutions. If our competitors successfully enter into partnering arrangements or license agreements with academic research institutions, we will then be precluded from pursuing those specific opportunities. Since each of these opportunities is unique, we may not be able to find a substitute. Other companies have already begun many drug development programs, which may target diseases that we are also targeting, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities.

Universities and public and private research institutions also compete with us. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our drug product candidates. We will attempt to license this proprietary technology, if available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to compete

successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products.

If we do not achieve our projected development goals in the time frames we announce and expect, the credibility of our management and our technology may be adversely affected and, as a result, the price of our common stock may decline.

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

From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones will be based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. If we do not meet these milestones as publicly announced, our stockholders may lose confidence in our ability to meet these milestones and, as a result, the price of our common stock may decline.

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

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

- conduct research, preclinical testing and human studies;
- establish pilot scale and commercial scale manufacturing processes and facilities; and
- establish and develop quality control, regulatory, marketing, sales, finance and administrative capabilities to support these programs.

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

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

We base our outlook regarding the need for funds on many uncertain variables. Such uncertainties include the success of our research initiatives, regulatory approvals, the timing of events outside our direct control such as negotiations with potential strategic partners and other factors. Any of these uncertain events can significantly change our cash requirements as they determine such one-time events as the receipt or payment of major milestones and other payments.

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

Uncertainties regarding healthcare reform and third-party reimbursement may impair our ability to raise capital, form collaborations and if any of our product candidates become marketable, sell such products.

The continuing efforts of governmental and third-party payers to contain or reduce the costs of healthcare through various means may harm our business. For example, in some foreign markets, the pricing or profitability of healthcare products is subject to government control. In the United States, there have been, and we expect there will continue to be, a number of federal and state proposals to implement similar government control. The implementation or even the announcement of any of these legislative or regulatory proposals or reforms could harm our business if any of our product candidates become marketable by reducing the prices we or our partners are able to charge for our products (if marketable), impeding our ability to achieve profitability, raise capital or form collaborations. In addition, the availability of reimbursement from third-party payers determines, in large part, the demand for healthcare products in the United States and elsewhere. Examples of such third-party payers are government and private insurance plans. Significant uncertainty exists as to the reimbursement status of newly approved healthcare products and third-party payers are increasingly challenging the prices charged for medical products and services. If we succeed in bringing one or more products to the market, reimbursement from third-party payers may not be available or may not be sufficient to allow us to sell such products on a competitive or profitable basis.

If we fail to demonstrate efficacy in our preclinical studies and clinical trials our future business prospects, financial condition and operating results will be materially adversely affected.

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

Moreover, success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and we cannot be sure that the results of later clinical trials will replicate the results of prior clinical trials and preclinical testing. The clinical trial process may fail to demonstrate that our drug product candidates are safe for humans and effective for indicated uses. This failure would cause us to abandon a drug product candidate and may delay development of other drug product candidates. Any delay in, or termination of, our preclinical testing or clinical trials will delay the filing of our investigational new drug application, or IND, and new drug application, or NDA, as applicable, with the FDA and, ultimately, our ability to commercialize our drug product candidates and generate product revenues. In addition, some of our clinical trials will involve small patient populations. Because of the small sample size, the results of these early clinical trials may not be indicative of future results. Following successful preclinical testing, drug product candidates will need to be tested in a clinical development program to provide data on safety and efficacy prior to becoming eligible for product approval and licensure by regulatory agencies. From first clinical trial through product approval can take at least eight years, on average in the U.S.

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

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



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

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

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

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

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

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

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

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

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

Because the extent and scope of patent protection for some of our drug products is particularly limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the exclusivity period under Orphan Drug Act designation to maintain a competitive position. If we do not obtain orphan drug exclusivity for our drug products that do not have patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced.

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

The fast-track designation for our drug product candidates, if obtained, may not actually lead to a faster review process and a delay in the review process or in the approval of our products will delay revenue from the sale of the products and will increase the capital necessary to fund these product development programs.

Although we have received Orphan Drug Designations from the FDA as described above, our drug product candidates may not receive an FDA fast-track designation or priority review. Without fast-track designation, submitting an NDA and getting through the regulatory process to gain marketing approval is a lengthy process. Under fast-track designation, the FDA may initiate review of sections of a fast-track drug's NDA before the application is complete. However, the FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, the fast-track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process. Under the FDA policies, a drug candidate is eligible for priority review, or review within a six-month time frame from the time a complete NDA is accepted for filing, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A fast-track designated drug candidate would ordinarily meet the FDA's criteria for priority review. The fast-track designation for our drug product candidates, if obtained, may not actually lead to a faster review process and a delay in the review process or in the approval of our products will delay revenue from the sale of the products and will increase the capital necessary to fund these product development programs.

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

Our clinical development of DR Cysteamine targets diseases with small patient populations, including nephropathic cystinosis and HD. If we are successful in developing DR Cysteamine and receive regulatory approval to market DR Cysteamine for a disease with a small patient population, the per-patient prices at which we could sell DR Cysteamine for these indications are likely to be relatively high in order for us to recover our development costs and achieve profitability. We believe that we will need to market DR Cysteamine for these indications worldwide to achieve significant market penetration of this product.

We may not be able to market or generate sales of our products to the extent anticipated.

Assuming that we are successful in developing our drug product candidates and receive regulatory clearances to market our products, our ability to successfully penetrate the market and generate sales of those products may be limited by a number of factors, including the following:

- Certain of our competitors in the field have already received regulatory approvals for and have begun marketing similar products in the U.S., the EU, Japan and other territories, which may result in greater physician awareness of their products as compared to ours.
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Information from our competitors or the academic community indicating that current products or new products are more effective than our future products could, if and when it is generated, impede our market penetration or decrease our future market share.

- Physicians may be reluctant to switch from existing treatment methods, including traditional therapy agents, to our future products.
- The price for our future products, as well as pricing decisions by our competitors, may have an effect on our revenues.
- Our future revenues may diminish if third-party payers, including private healthcare coverage insurers and healthcare maintenance organizations, do not provide adequate coverage or reimbursement for our future products.

There are many difficult challenges associated with developing proteins that can be used to transport therapeutics across the blood-brain barrier.

Our RAP technology has a potential clinical use as a drug transporter through the blood-brain barrier. However, we do not know that our technology will work or work safely. Many groups and companies have attempted to solve the critical medical challenge of developing an efficient method of transporting therapeutic proteins from the blood stream into the brain. Unfortunately, these efforts to date have met with little success due in part to a lack of adequate understanding of the biology of the blood-brain barrier and to the enormous scientific complexity of the transport process itself. In the research and development of our RAP technology, we will certainly face many of the same issues that have caused these earlier attempts to fail. It is possible that:

- We or our collaborator/licensee will not be able to produce enough RAP drug product candidates for testing;
- the pharmacokinetics, or where the drug distributes in the body, of our RAP drug product candidates will preclude sufficient binding to the targeted receptors on the blood-brain barrier;
- the targeted receptors are not transported across the blood-brain barrier;
- other features of the blood-brain barrier, apart from the cells, block access molecules to brain tissue after transport across the cells;
- the targeted receptors are expressed on the blood-brain barrier at densities insufficient to allow adequate transport of our RAP drug product candidates into the brain;
- targeting of the selected receptors induces harmful side-effects which prevent their use as drugs; or
- that we or our collaborator/licensee's RAP drug product candidates cause unacceptable side-effects.

Any of these conditions may preclude the use of RAP or RAP fusion compounds from potentially treating diseases affecting the brain.

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

We face significant competition from industry participants that are pursuing similar technologies that we are pursuing and are developing pharmaceutical products that are competitive with our drug product candidates. Nearly all of our industry competitors have greater capital resources, larger overall research and development staffs and facilities, and a

longer history in drug discovery and development, obtaining regulatory approval and pharmaceutical product manufacturing and marketing than we do. With these additional resources, our competitors may be able to respond to the rapid and significant technological changes in the biotechnology and pharmaceutical industries faster than we can. Our future success will depend in large part on our ability to maintain a competitive position with respect to these technologies. Rapid technological development, as well as new scientific developments, may result in our compounds, drug product candidates or processes becoming obsolete before we can recover any of the expenses incurred to develop them. For example, changes in our understanding of the appropriate population of patients who should be treated with a targeted therapy like we are developing may limit the drug's market potential if it is subsequently demonstrated that only certain subsets of patients should be treated with the targeted therapy.

Our reliance on third parties, such as collaborators, university laboratories, contract manufacturing organizations and contract or clinical research organizations, may result in delays in completing, or a failure to complete, preclinical testing or clinical trials if they fail to perform under our agreements with them.

In the course of product development, we may engage university laboratories, other biotechnology or companies or contract or clinical manufacturing organizations to manufacture drug material for us to be used in preclinical and clinical testing and collaborators and contract or clinical research organizations to conduct and manage preclinical studies and clinical trials. If we engage these organizations to help us with our preclinical and clinical programs, many important aspects of this process have been and will be out of our direct control. If any of these organizations we may engage in the future fail to perform their obligations under our agreements with them or fail to perform preclinical testing and/or clinical trials in a satisfactory manner, we may face delays in completing our clinical trials, as well as commercialization of any of our drug product candidates. Furthermore, any loss or delay in obtaining contracts with such entities may also delay the completion of our clinical trials, regulatory filings and the potential market approval of our drug product candidates.

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

Licensing our product candidates from other companies, universities or individuals does not always prevent them from developing non-identical but competitive products for their own commercial purposes, nor from pursuing patent protection in areas that are competitive with us. While we seek patent protection for all of our owned and licensed product candidates, our licensors or assignors who created these product candidates are experienced scientists and business people who may continue to do research and development and seek patent protection in the same areas that led to the discovery of the product candidates that they licensed or assigned to us. By virtue of the previous research that led to the discovery of the drugs or product candidates that they licensed or assigned to us, these companies, universities, or individuals may be able to develop and market competitive products in less time than might be required to develop a product with which they have no prior experience and may reduce our future revenues from such product candidates.

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

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

The use of any of our drug product candidates in clinical trials may expose us to liability claims.

The nature of our business exposes us to potential liability risks inherent in the testing, manufacturing and marketing of our drug product candidates. While we are in clinical stage testing, our drug product candidates could potentially harm people or allegedly harm people and we may be subject to costly and damaging product liability claims. Some of the patients who participate in clinical trials are already critically ill when they enter a trial. The waivers we obtain may not be enforceable and may not protect us from liability or the costs of product liability litigation. Although we currently carry a \$3 million clinical product liability insurance policy, it may not be sufficient to cover future claims. We currently do not have any clinical or product liability claims or threats of claims filed against us.

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

Our success is dependent in part upon the availability of our senior executive officers, including our Chief Executive Officer, Dr. Christopher M. Starr, our Chief Scientific Officer, Dr. Todd C. Zankel, our Chief Financial Officer, Kim R. Tsuchimoto, Ted Daley, the President of our clinical development subsidiary and Dr. Patrice P. Rioux, Chief Medical Officer of our clinical development subsidiary. The loss or unavailability to us of any of these individuals or key research and development personnel, and particularly if lost to competitors, could have a material adverse effect on our business, prospects, financial condition, and operating results. We have no key-man insurance on any of our employees. There is intense competition for qualified scientists and managerial personnel from numerous pharmaceutical and biotechnology companies, as well as from academic and government organizations, research institutions and other entities. In addition, we will rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development strategy. All of our consultants and advisors will be employed by other employers or be self-employed, and will have commitments to or consulting or advisory contracts



with other entities that may limit their availability to us. There is no assurance that we will be able to retain key employees and/or consultants. If key employees terminate their employment, or if insufficient numbers of employees are retained to maintain effective operations, our development activities might be adversely affected, management's attention might be diverted from managing our operations to hiring suitable replacements, and our business might suffer. In addition, we might not be able to locate suitable replacements for any key employees that terminate, or that are terminated from, their employment with us and we may not be able to offer employment to potential replacements on reasonable terms, which could negatively impact our product candidate development timelines and may adversely affect our future revenues and financial condition.

Our success depends on our ability to manage our growth.

If we are able to raise significant additional financing, we expect to continue to grow, which could strain our managerial, operational, financial and other resources. With the addition of our clinical-stage programs and with our plan to in-license and acquire additional clinical-stage product candidates, we will be required to retain experienced personnel in the regulatory, clinical and medical areas over the next several years. Also, as our preclinical pipeline diversifies through the acquisition or in-licensing of new molecules, we will need to hire additional scientists to supplement our existing scientific expertise over the next several years.

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

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

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

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

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

In addition, the Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. In particular, we must perform system and process

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

We may be required to suspend, repeat or terminate our clinical trials if they do not meet regulatory requirements, the results are negative or inconclusive, or if the trials are not well designed, which may result in significant negative repercussions on our business and financial condition.

Before regulatory approval for any potential product can be obtained, we must undertake extensive clinical testing on humans to demonstrate the tolerability and efficacy of the product, both on our own terms, and as compared to the other principal drugs on the market that have the same therapeutic indication. We cannot provide assurance that we will obtain authorization to permit product candidates that are already in the preclinical development phase to enter the human clinical testing phase. In addition, we cannot provide assurance that any authorized preclinical or clinical testing will be completed successfully within any specified time period by us, or without significant additional resources or expertise to those originally expected to be necessary. We cannot provide assurance that such testing will show potential products to be safe and efficacious or that any such product will be approved for a specific indication. Further, the results from preclinical studies and early clinical trials may not be indicative of the results that will be obtained in later-stage clinical trials. In addition, we or regulatory authorities may suspend clinical trials at any time on the basis that the participants are being exposed to unacceptable health risks.

Completion of clinical tests depends on, among other things, the number of patients available for testing, which is a function of many factors, including the number of patients with the relevant conditions, the nature of the clinical testing, the proximity of patients to clinical testing centers, the eligibility criteria for tests as well as competition with other clinical testing programs involving the same patient profile but different treatments. We will rely on third parties, such as contract research organizations and/or co-operative groups, to assist us in overseeing and monitoring clinical trials as well as to process the clinical results and manage test requests, which may result in delays or failure to complete trials, if the third parties fail to perform or to meet the applicable standards. A failure by us or such third parties to keep to the terms of a product program development for any particular product candidate or to complete the clinical trials for a product candidate in the envisaged time frame could have significant negative repercussions on our business and financial condition.

If we fail to establish and maintain collaborations or if our partners do not perform, we may be unable to develop and commercialize our product candidates, which may adversely affect our future revenues and financial condition.

We have entered into collaborative arrangements with third parties to develop and/or commercialize product candidates. Additional collaborations might be necessary in order for us to fund our research and development activities and third-party manufacturing arrangements, seek and obtain regulatory approvals and successfully commercialize existing and future product candidates. If we fail to maintain the existing collaborative arrangements held by us or fail to enter into additional collaborative arrangements, the number of product candidates from which we could receive future revenues would decline.

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

- collaborative arrangements might not be on terms favorable to us;
-

disagreements with partners may result in delays in the development and marketing of products, termination of collaboration agreements or time consuming and expensive legal action;

- we cannot control the amount and timing of resources partners devote to product candidates or their prioritization of product candidates, and partners may not allocate sufficient funds or resources to the development, promotion or marketing of our product candidates, or may not perform their obligations as expected;
- partners may choose to develop, independently or with other companies, alternative products or treatments, including products or treatments which compete with ours;
- agreements with partners may expire or be terminated without renewal, or partners may breach collaboration agreements with us;
- business combinations or significant changes in a partner's business strategy might adversely affect that partner's willingness or ability to complete their obligations to us; and
- the terms and conditions of the relevant agreements may no longer be suitable.

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

We may not complete our clinical trials in the time expected, which could delay or prevent the commercialization of our products, which may adversely affect our future revenues and financial condition.

Although for planning purposes we forecast the commencement and completion of clinical trials, the actual timing of these events can vary dramatically due to factors such as delays, scheduling conflicts with participating clinicians and clinical institutions and the rate of patient enrollment. Clinical trials involving our product candidates may not commence nor be completed as forecasted. In certain circumstances we will rely on academic institutions or clinical research organizations to conduct, supervise or monitor some or all aspects of clinical trials involving our product candidates. We will have less control over the timing and other aspects of these clinical trials than if we conducted them entirely on our own. These trials may not commence or be completed as we expect. They may not be conducted successfully. Failure to commence or complete, or delays in, any of our planned clinical trials could delay or prevent the commercialization of our product candidates and harm our business and may adversely affect our future revenues and financial condition.

If we fail to keep pace with rapid technological change in the biotechnology and pharmaceutical industries, our product candidates could become obsolete, which may adversely affect our future revenues and financial condition.

Biotechnology and related pharmaceutical technology have undergone and are subject to rapid and significant change. We expect that the technologies associated with biotechnology research and development will continue to develop rapidly. Our future will depend in large part on our ability to maintain a competitive position with respect to these technologies. Any compounds, products or processes that we develop may become obsolete before we recover any expenses incurred in connection with developing such products, which may adversely affect our future revenues and financial condition.

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

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

The patent positions of biopharmaceutical products are complex and uncertain.

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

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

developed similar methods.

- Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us. Competitors may also claim that we are infringing on their patents and therefore cannot practice our technology as claimed under our patents, if issued. Competitors may also contest our patents, if issued, by showing the patent examiner that the invention was not original, was not novel or was obvious. In litigation, a competitor could claim that our patents, if issued, are not valid for a number of reasons. If a court agrees, we would lose that patent. As a company, we have no meaningful experience with competitors interfering with our patents or patent applications.
- Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing drug product candidates, which could increase our operating expenses and delay product programs.
- Receipt of a patent may not provide much practical protection. If we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent.

- In addition, competitors also seek patent protection for their technology. Due to the number of patents in our field of technology, we cannot be certain that we do not infringe on those patents or that we will not infringe on patents granted in the future. If a patent holder believes our drug product candidate infringes on its patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe on their technology, we would face a number of issues, including the following:
  - Defending a lawsuit takes significant time and can be very expensive.
  - If a court decides that our drug product candidate infringes on the competitor's patent, we may have to pay substantial damages for past infringement.
- A court may prohibit us from selling or licensing the drug product candidate unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, we may have to pay substantial royalties or grant cross licenses to our patents.
- Redesigning our drug product candidates so we do not infringe may not be possible or could require substantial funds and time.

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

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

We have taken steps to protect our intellectual property and proprietary technology, by entering into confidentiality agreements and intellectual property assignment agreements with our employees, consultants, advisors and corporate



partners. Such agreements may not be enforceable or may not provide meaningful protection for our trade secrets or other proprietary information in the event of unauthorized use or disclosure or other breaches of the agreements, and we may not be able to prevent such unauthorized disclosure. Monitoring unauthorized disclosure is difficult, and we do not know whether the steps we have taken to prevent such disclosure are, or will be, adequate. Furthermore, the laws of some foreign countries may not protect our intellectual property rights to the same extent as do the laws of the United States.

#### Risks Related to Our Common Stock

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

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

In addition, future sales of substantial amounts of our currently outstanding common stock in the public market, or the perception that such sales could occur, could adversely affect prevailing trading prices of our common stock, and could impair our ability to raise capital through future offerings of equity or equity-related securities. We cannot predict what effect, if any, future sales of our common stock, or the availability of shares for future sales, will have on the trading price of our common stock.

In May and June 2008, prior to the 2009 Merger, pursuant to a securities purchase agreement for a private placement of units, Raptor Pharmaceuticals Corp. issued to investors in such private placement, 20,000,000 shares of its common stock and two-year warrants to purchase up to, in the aggregate, 10,000,000 shares of its common stock and to placement agents in such private placement, five-year warrants to purchase up to, in the aggregate, 2,100,000 shares of its common stock. On a post-merger basis, the 20,000,000 shares of Raptor Pharmaceuticals Corp.'s common stock, the two-year warrants to purchase up to, in the aggregate, 10,000,000 shares of Raptor Pharmaceuticals Corp.'s common stock and the five-year warrants to purchase up to, in the aggregate, 2,100,000 shares of Raptor Pharmaceuticals Corp.'s common stock, respectively, would be 4,662,468 shares of our common stock, two-year warrants to purchase up to, in the aggregate, 2,331,234 shares of our common stock and the five-year warrants to purchase up to, in the aggregate, 489,559 shares of our common stock, respectively.

In April 2009, in order to reflect then-current market prices, Raptor Pharmaceuticals Corp. notified the holders of warrants purchased in the May/June 2008 private placement that it was offering, in exchange for such warrants, new warrants to purchase its common stock at an exercise price of \$0.30 per share, but only to the extent such exchange of the original warrants and exercise of the new warrants, including the delivery of the exercise price, occurred on or prior to July 17, 2009. The warrants that were not exchanged prior to or on July 17, 2009 retained their original exercise prices of \$0.90 per share and original expiration date of May 21, 2010. On a post-merger basis, the warrants that were not exchanged prior to or on July 17, 2009 would be warrants to purchase shares of our common stock at an exercise price of \$3.86 per share and would continue to have an expiration date of May 21, 2010. Raptor Pharmaceuticals Corp. received approximately \$2.6 million of proceeds from warrant exercises that resulted in the issuance of 8,715,000 shares of its common stock pursuant to the exchange described above. On a post-merger basis, the 8,715,000 shares of Raptor Pharmaceuticals Corp.'s common stock would be 2,031,670 shares of our common stock.

In August 2009, pursuant to a securities purchase agreement for a private placement of units, Raptor Pharmaceuticals Corp. issued to investors in such private placement, 7,456,250 shares of its common stock and two-year warrants to purchase up to, in the aggregate, 3,728,125 shares of its common stock and to placement agents in such private placement, a five-year warrant to purchase up to, in the aggregate, 556,500 shares of its common stock. On a post-merger basis, the 7,456,250 shares of Raptor Pharmaceuticals Corp.'s common stock, the two-year warrants to purchase up to, in the aggregate, 3,728,125 shares of Raptor Pharmaceuticals Corp.'s common stock and the five-year warrants to purchase up to, in the aggregate, 556,500 shares of Raptor Pharmaceuticals Corp.'s common stock, respectively, would be 1,738,226 shares of our common stock, two-year warrants to purchase up to, in the aggregate, 869,113 shares of our common stock and the five-year warrants to purchase up to, in the aggregate, 129,733 shares of our common stock, respectively.

In December 2009, we entered into a definitive securities purchase agreement or the Purchase Agreement, dated as of December 17, 2009, with 33 investors set forth on the signature pages thereto, collectively, the Investors, with

respect to the offering of Units, whereby, on an aggregate basis, the Investors purchased 3,747,558 Units for a negotiated purchase price of \$2.00 per Unit for aggregate gross proceeds of approximately \$7.5 million. Each Unit consists of one share of our common stock, one Series A Warrant exercisable for 0.5 of a share of our common stock and one Series B Warrant exercisable for 0.5 of a share of our common stock. Units were not issued or certificated. The shares of our common stock and the Warrants were issued separately. The Series A Warrants will be exercisable during the period beginning one hundred eighty (180) days after the date of issue and ending on the fifth (5th) anniversary of the date of issue. The Series B Warrants will be exercisable during the period beginning one hundred eighty (180) days after the date of issue and ending on the eighteen (18) month anniversary of the date of issue. The Investor Warrants have a per share exercise price of \$2.45. In connection with this offering we paid a placement agent cash compensation equaled to 6.5% of the gross proceeds or \$487,183 plus a five-year warrant at an exercise price of \$2.50 for the purchase of up to 74,951 shares of our common stock, on the same terms as the investor warrants described above.

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

As of December 23, 2009, after the financing described in the previous paragraph, there were 22,579,515 shares of our common stock outstanding (or issuable). We also had outstanding as of December 23, 2009 warrants that are exercisable to purchase an aggregate of 5,843,302 shares of our common stock at a weighted average exercise price of \$2.66 per share. On October 13, 2009, we filed a registration statement registering the resale of up to an aggregate of 5,557,865 shares of our common stock (including common stock issuable under warrants). Such registration statement was declared effective by the SEC on November 12, 2009.

In addition to our outstanding warrants, as of December 23, 2009, there were (i) options to purchase 1,037,688 shares of our common stock outstanding under our 2006 Raptor Pharmaceutical Equity Incentive Plan at a weighted-average exercise price of \$2.47, (ii) options to purchase 158,475 shares of our common stock outstanding under our 2006 TorreyPines Therapeutics Equity Incentive Plan at a weighted-average exercise price of \$114.12, (iii) 355,557 shares of our common stock available for issuance under our 2006 Raptor Pharmaceutical Equity Incentive Plan (of which all such shares are subject to approval by our stockholders at our 2010 Annual Meeting of stockholders) and (iv) 852,547 shares of our common stock available for issuance under our 2006 TorreyPines Therapeutics Equity Incentive Plan. The shares issuable under our equity incentive plans will be available for immediate resale in the public market. The shares issuable under the warrants are available for immediate resale in the public market. The market price of our common stock could decline as a result of such resales due to the increased number of shares available for sale in the market.

Our executive officers and directors are subject to lock-up agreements pursuant to the Purchase Agreement executed in our December 2009 financing. Each lock-up agreement is for a period of 90 days commencing on December 18, 2009, and represent 1,728,022 shares, or 7.7% of our outstanding common stock as of December 23, 2009 (taking into account the 3,747,558 shares of common stock sold in the December 2009 financing). Following the termination of this lock-up period, these stockholders will have the ability to sell a substantial number of shares of common stock in the public market in a short period of time. Sales of a substantial number of shares of common stock in the public trading market, whether in a single transaction or a series of transactions, or the perception that these sales may occur, could also have a significant effect on volatility and the trading price of our common stock.

Future milestone payments, as more fully set forth under “Contractual Obligations with Thomas E. Daley (as assignee of the dissolved Convivia, Inc.)” and “Contractual Obligations with Former Encode Securityholders” discussed in certain of our periodic filings with the SEC relating to our acquisition of the Convivia assets and merger with Encode will result in dilution. We may be required to make additional contingent payments of up to 664,400 shares of our common stock, in the aggregate, under the terms of our acquisition of Convivia assets and merger with Encode, based on milestones related to certain future marketing and development approvals obtained with respect to Convivia and Encode product candidates. The issuance of any of these shares will result in further dilution to our existing stockholders.

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

We have not declared or paid any cash dividends on our common stock since our inception. We anticipate that we will retain our future earnings, if any, to support our operations and to finance the growth and development of our business and do not expect to pay cash dividends in the foreseeable future. As a result, the success of an investment in

our common stock will depend upon any future appreciation in the value of our common stock. There is no guarantee that our common stock will appreciate in value or even maintain its current price. Investors seeking dividend income or liquidity should not invest in our common stock.

Our stock price is volatile, which could result in substantial losses for our stockholders, and the trading in our common stock may be limited.

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

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

- the results of our current and any future clinical trials of our drug candidates;
- the results of ongoing preclinical studies and planned clinical trials of our preclinical drug candidates;
- the entry into, or termination of, key agreements, including key strategic alliance agreements;
- the results and timing of regulatory reviews relating to the approval of our drug candidates;
- the initiation of, material developments in, or conclusion of litigation to enforce or defend any of our intellectual property rights;
- failure of any of our drug candidates, if approved, to achieve commercial success;
- general and industry-specific economic conditions that may affect our research and development expenditures;
- the results of clinical trials conducted by others on drugs that would compete with our drug candidates;
- issues in manufacturing our drug candidates or any approved products;
- the loss of key employees;
- the introduction of technological innovations or new commercial products by our competitors;
- changes in estimates or recommendations by securities analysts, if any, who cover our common stock;

- future sales of our common stock;
- changes in the structure of health care payment systems; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock. In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation can result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

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

Our common stock is a penny stock. The SEC has adopted Rule 15c-9 which generally defines "penny stock" to be any equity security that has a market price less than \$5.00 per share or an exercise price of less than \$5.00 per share, subject to certain exceptions. Our securities are covered by the penny stock rules, which impose additional sales practice requirements on broker-dealers who sell to persons other than established customers and institutional accredited investors. The penny stock rules require a broker-dealer, prior to a transaction in a penny stock not otherwise exempt from the rules, to deliver a standardized risk disclosure document in a form prepared by the SEC which provides information about penny stocks and the nature and level of risks in the penny stock market. The broker-dealer also must provide the customer with current bid and offer quotations for the penny stock, the compensation of the broker-dealer and its salesperson in the transaction and monthly account statements showing the market value of each penny stock held in the customer's account. The bid and offer quotations, and the broker-dealer and salesperson compensation information, must be given to the customer orally or in writing prior to effecting the transaction and must be given to the customer in writing before or with the customer's confirmation. In addition, the penny stock rules require that prior to a transaction in a penny stock not otherwise exempt from these rules, the broker-dealer must make a special written determination that the penny stock is a suitable investment for the purchaser and receive the purchaser's written agreement to the transaction. These disclosure requirements may have the effect of reducing the level of trading activity in the secondary market for the stock that is subject to these penny stock rules. Consequently, these penny stock rules may affect the ability of broker-dealers to trade our securities. We believe that the penny stock rules discourage investor interest in and limit the marketability of our common stock.

In addition to the "penny stock" rules promulgated by the SEC, the Financial Industry Regulatory Authority, or FINRA, has adopted rules that require that in recommending an investment to a customer, a broker-dealer must have reasonable grounds for believing that the investment is suitable for that customer. Prior to recommending speculative low priced securities to their non-institutional customers, broker-dealers must make reasonable efforts to obtain information about the customer's financial status, tax status, investment objectives and other information. Under interpretations of these rules, the FINRA believes that there is a high probability that speculative low priced securities will not be suitable for at least some customers. The FINRA requirements make it more difficult for broker-dealers to recommend that their customers buy our common stock, which may limit your ability to buy and sell our stock.

An adverse determination, if any, in the class action suit in which we are a defendant, or our inability to obtain or maintain directors' and officers' liability insurance, could have a material adverse affect on us.

A class action securities lawsuit was filed against us, as described in the section titled, "Legal Proceedings" in certain of our periodic reports that we file with the SEC. We are defending against this action vigorously; however, we do not know what the outcome of the proceedings will be and, if we do not prevail, we may be required to pay substantial damages or settlement amounts. Furthermore, regardless of the outcome, we may incur significant defense costs, and the time and attention of our key management may be diverted from normal business operations. If we are ultimately required to pay significant defense costs, damages or settlement amounts, such payments could materially and adversely affect our operations and results. We have purchased liability insurance, however, if any costs or expenses associated with the litigation exceed the insurance coverage, we may be forced to bear some or all of these costs and expenses directly, which could be substantial and may have an adverse effect on our business, financial condition, results of operations and cash flows. In any event, publicity surrounding the lawsuits and/or any outcome unfavorable to us could adversely affect our reputation and stock price. The uncertainty associated with substantial unresolved



lawsuits could harm our business, financial condition and reputation. We have certain obligations to indemnify our officers and directors and to advance expenses to such officers and directors. Although we have purchased liability insurance for our directors and officers, if our insurance carriers should deny coverage, or if the indemnification costs exceed the insurance coverage, we may be forced to bear some or all of these indemnification costs directly, which could be substantial and may have an adverse effect on our business, financial condition, results of operations and cash flows. If the cost of the liability insurance increases significantly, or if this insurance becomes unavailable, we may not be able to maintain or increase our levels of insurance coverage for our directors and officers, which could make it difficult to attract or retain qualified directors and officers.

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

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

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

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

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



## ITEM 2: PROPERTIES

Effective April 1, 2006, we entered into a three year lease for 2,892 square feet of combined laboratory and office space with an additional three year option. Our original monthly base rent was \$5,206. Effective April 1, 2007, we leased an additional 3,210 square feet in order to expand our office space and our base rent increased to \$9,764 per month. In June 2008, our rent increased to \$10,215 reflecting a Consumer Price Index increase of 3% plus an increase in operating costs for the period from April 1, 2008 to March 31, 2009. In September 2008, we executed a lease addendum replacing the one three-year extension with two two-year extensions commencing on April 1, 2009 and renegotiated the first two-year extension base rent to \$10,068 with an adjustment after the first year for CPI between 3% (minimum) and 5% (maximum). The facility is located in an industrial park at 9 Commercial Blvd, Suite 200, Novato, California 94949. We also store cell line back ups at an off site cell bank, a commercial facility specifically licensed for such purpose. Our current facility is expected to be adequate for the foreseeable future.

## ITEM 3: LEGAL PROCEEDINGS

Several lawsuits were filed against us in February 2005 in the U.S. District Court for the Southern District of New York asserting claims under Sections 10(b) and 20(a) of the Exchange Act and Rule 10b-5 thereunder on behalf of a class of purchasers of our common stock during the period from June 26, 2003, through and including February 4, 2005, referred to as the class period. Dr. Marvin S. Hausman, M.D., a former director and a former Chief Executive Officer, and Dr. Gosse B. Bruinsma, M.D., also a former director and a former Chief Executive Officer, were also named as defendants in the lawsuits. These actions were consolidated into a single class action lawsuit in January 2006. On April 10, 2006, the class action plaintiffs filed an amended consolidated complaint. We filed our answer to that complaint on May 26, 2006. Our motion to dismiss the consolidated amended complaint was filed on May 26, 2006 and was submitted to the court for a decision in September 2006. On March 31, 2009 the U.S. District Court for the Southern District of New York dismissed the proceedings. On April 24, 2009, an appeal was filed with the United States Court of Appeals for the Second Circuit by the class action plaintiffs. Our response to such appeal was filed on October 23, 2009. The Second Circuit heard the plaintiffs' appeal of the order dismissing the complaint on January 14, 2010. We do not anticipate that this claim, if successful, would burden the Company with any additional liability above and beyond the insurance coverage provided under the insurance policy that we currently maintain.

Other than as described above, 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.

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## PART II

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

## Market Information

In connection with the closing of the 2009 Merger, our common stock commenced trading on The NASDAQ Capital Market on September 30, 2009, under the ticker symbol "RPTPD" with 18,822,162 shares outstanding. Effective October 27, 2009, our ticker symbol changed to "RPTP." There is no public trading market for our warrants. As of January 28 2010, there were approximately 335 holders of record of the Company's common stock and 22,455,365 shares of our common stock outstanding.

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

	High	Low
Year Ended August 31, 2010:		
First Quarter (through September 29) *	\$ 7.14	\$ 3.23
First Quarter (September 30 – November 30, 2009)	4.90	1.16
Year Ended August 31, 2009:		
First Quarter *	\$ 11.73	\$ 2.72
Second Quarter *	5.95	2.72
Third Quarter *	7.65	2.55
Fourth Quarter	11.73	1.19
Year Ended August 31, 2008:		
First Quarter *	\$ 115.09	\$ 43.52
Second Quarter *	50.15	30.60
Third Quarter *	33.83	21.25
Fourth Quarter *	27.03	8.84

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

## 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.

## Securities Authorized for Issuance Under Equity Compensation Plans

## The Company

The following table provides information as of August 31, 2009 with respect to shares of common stock that may be issued under the (i) TorreyPines 2006 Equity Incentive Plan and (ii) 2000 Stock Option Plan (formerly the Axonyx 2000 Stock Option Plan), taking into effect the 1 for 17 reverse stock split in September 2009.

## Equity Compensation Plan Information \*

Plan category	Number of securities to be issued upon exercise of outstanding options, warrants and rights (a)	Weighted-average exercise price of outstanding options, warrants and rights (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by security holders	258,553	\$ 129.51	50,301
Equity compensation plans not previously approved by security holders	0	0	0
Total	258,553	\$ 129.51	50,301





## Raptor Pharmaceuticals Corp.

The following table provides information as of August 31, 2009 with respect to shares of common stock that may be issued under the Raptor Pharmaceuticals Corp. 2006 Equity Incentive Plan, as amended, or the Plan, which was assumed by the Company in connection with the 2009 Merger. Our stockholders have not approved the Plan. RPC's stockholders approved the Plan in May 2006, and RPC's board of directors approved Amendment No. 1 in February 2007 and Amendment No. 2 in December 2008. As discussed elsewhere in this Current Report on Form 8-K, the relevant options are options to purchase common stock of Raptor Pharmaceutical Corp., and the number of securities underlying such options as well as the option exercise prices have been converted to their equivalent post-2009 Merger number of securities and equivalent post-2009 Merger exercise prices, respectively.

## Equity Compensation Plan Information

Plan category	Number of securities to be issued upon exercise of outstanding options, warrants and rights (a)	Weighted-average exercise price of outstanding options, warrants and rights (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by security holders	989,213	\$ 2.42	406,147
Equity compensation plans not previously approved by security holders	0	0	0
Total	989,213	\$ 2.42	406,147

The Plan's life is ten years and allows for the granting of options to employees, directors and consultants. Typical option grants are for ten years at or above market price based on the last closing price as of the date of grant and vests over four years as follows: 6/48ths on the six month anniversary of the date of grant and 1/48th per month thereafter.



## ITEM 6: SELECTED FINANCIAL DATA

The following table shows selected historical, condensed consolidated financial and operating information for, and as of the end of, each of the periods indicated and should be read in conjunction with sections titled "Management's Discussion and Analysis of Financial Condition and Results of Operation" and "Business" and our consolidated financial statements and the corresponding notes to those consolidated financial statements included elsewhere in this Current Report on Form 8-K. The following tables set forth the Company's unaudited, condensed, consolidated statements of operations data for the three month periods ended November 30, 2009 and 2008 and the cumulative period from September 8, 2005 (inception) to November 30, 2009, and our condensed, consolidated balance sheet data as of November 30, 2009 (unaudited) and August 31, 2009.

## Unaudited Financial Statements of the Company

	For the three month periods from September 1, to November 30,	
	2009	2008
Revenues:	\$ -	\$ -
Operating expenses:		
General and administrative	1,010,076	659,689
Research and development	1,930,836	1,820,400
Total operating expenses	2,940,912	2,480,089
Loss from operations	(2,940,912)	(2,480,089)
Interest income	3,265	21,777
Interest expense	(1,025)	(686)
Net loss	\$ (2,938,672)	\$ (2,458,998)
Net loss per share:		
Basic and diluted	\$ (0.16)	\$ (0.17)
Weighted average shares outstanding used to compute:		
Basic and diluted	18,520,579	14,074,849
		For the cumulative period from September 8, 2005 (inception) to November 30, 2009
Revenues:		\$ -
Operating expenses:		
General and administrative		7,966,316
Research and development		16,805,120
In-process research and development		240,625
Total operating expenses		25,012,061
Loss from operations		(25,012,061)

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Interest income		305,168
Interest expense		(110,962)
Net loss	\$	(24,817,855)

	November 30,	2009
Balance Sheet Data:		
Cash and cash equivalents	\$	1,164,808
Working capital deficit		(554,501)
Total assets		8,530,910
Long-term portion of capital lease obligations		5,535
Total liabilities		1,956,802
Total stockholders' equity		6,574,108

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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 sections titled “Management’s Discussion and Analysis of Financial Condition and Results of Operation” and “Business” and our consolidated financial statements and the corresponding notes to those consolidated financial statements included elsewhere in this Current Report on Form 8-K. The following tables set forth our consolidated statements of operations data for the years ended August 31, 2009 and 2008 and for the cumulative period from September 8, 2005 (inception) to August 31, 2009, and our consolidated balance sheet data as of August 31, 2009 and 2008, each which were audited by Burr, Pilger & Mayer, LLP, an independent registered public accounting firm.

Audited Financial Statements of Raptor Pharmaceuticals Corp.

	For the year ended August 31, 2009	For the year ended August 31, 2008	For the period from September 8, 2005 (inception) to August 31, 2009
Revenues:	\$	\$	\$
Operating expenses:			
General and administrative	2,687,993	2,229,140	6,956,240
Research and development	6,570,119	5,558,871	14,874,284
In-process research and development	—	240,625	240,625
Total operating expenses	9,258,112	8,028,636	22,071,149
Loss from operations	(9,258,112)	(8,028,636)	(22,071,149)
Interest income	36,744	77,871	301,903
Interest expense	(2,526)	(103,198)	(109,937)
Net loss	\$ (9,223,894)	\$ (8,053,963)	\$ (21,879,183)
Net loss per share:			
Basic and diluted	\$ (0.64)	\$ (0.81)	
Weighted average shares outstanding used to compute:			
Basic and diluted	14,440,254	9,893,612	

Balance Sheet Data: 2009 August 31,  
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